

REFINITIV

DELTA REPORT

10-Q

RIGL - RIGEL PHARMACEUTICALS INC

10-Q - MARCH 31, 2024 COMPARED TO 10-Q - SEPTEMBER 30, 2023

The following comparison report has been automatically generated

TOTAL DELTAS 7143

█ CHANGES 210

█ DELETIONS 2209

█ ADDITIONS 4724

**UNITED STATES
SECURITIES AND EXCHANGE COMMISSION**
WASHINGTON, D.C. 20549

FORM 10-Q

(Mark One)

QUARTERLY REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934

FOR THE QUARTERLY PERIOD ENDED **SEPTEMBER 30, MARCH 31, 2023** **2024**

OR

TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934

FOR THE TRANSITION PERIOD FROM **TO**

Commission File Number **0-29889**

Rigel Pharmaceuticals, Inc.

(Exact name of registrant as specified in its charter)

Delaware

94-3248524

(State or other jurisdiction of incorporation or
organization)

(I.R.S. Employer Identification No.)

611 Gateway Boulevard, Suite 900,

94080

South San Francisco, CA

(Zip Code)

(Address of principal executive offices)

(650) 624-1100

(Registrant's telephone number, including area code)

Securities registered pursuant to Section 12(b) of the Act:

Title of each class:

Common Stock, par value \$0.001 per share

Trading Symbol

RIGL

Name of each exchange on which registered:

The Nasdaq Stock Market LLC

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes No

Indicate by check mark whether the registrant has submitted electronically, every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit such files). Yes No

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, a smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer

Accelerated filer

Non-accelerated filer

Smaller reporting company

Emerging Growth Company

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act.

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). Yes No

As of November 2, 2023 May 2, 2024, there were 174,369,503 175,406,146 shares of the registrant's Common Stock outstanding.

[Table of Contents](#)

RIGEL PHARMACEUTICALS, INC.
QUARTERLY REPORT ON FORM 10-Q
FOR THE QUARTERLY PERIOD ENDED SEPTEMBER 30, 2023 MARCH 31, 2024

INDEX

	Page
PART I	FINANCIAL INFORMATION
Item 1.	Financial Statements
Condensed Balance Sheets — <u>September 30, 2023</u> <u>March 31, 2024</u> (Unaudited) and <u>December 31, 2022</u> <u>December 31, 2023</u>	3
Condensed Statements of Operations (Unaudited) — three and nine months ended <u>September 30, 2023</u> <u>March 31, 2024</u> and <u>2022</u> <u>2023</u>	4
Condensed Statements of Comprehensive Loss (Unaudited) — three and nine months ended <u>September 30, 2023</u> <u>March 31, 2024</u> and <u>2022</u> <u>2023</u>	5
Condensed Statements of Stockholders' (Deficit) Equity Deficit (Unaudited) — three and nine months ended <u>September 30, 2023</u> <u>March 31, 2024</u> and <u>2022</u> <u>2023</u>	6
Condensed Statements of Cash Flows (Unaudited) — nine three months ended <u>September 30, 2023</u> <u>March 31, 2024</u> and <u>2022</u> <u>2023</u>	7
Notes to Condensed Financial Statements (Unaudited)	8
Item 2.	Management's Discussion and Analysis of Financial Condition and Results of Operations
Item 3.	Quantitative and Qualitative Disclosures About Market Risk
Item 4.	Controls and Procedures
PART II	OTHER INFORMATION
Item 1.	Legal Proceedings
Item 1A.	Risk Factors

Item 2.	Unregistered Sales of Equity Securities and Use of Proceeds	92 94
Item 3.	Defaults Upon Senior Securities	92 94
Item 4.	Mine Safety Disclosures	92 94
Item 5.	Other Information	93 95
Item 6.	Exhibits	93 96
Signatures		95 97

[Table of Contents](#)

PART I. FINANCIAL INFORMATION

Item 1. Financial Statements

RIGEL PHARMACEUTICALS, INC.
CONDENSED BALANCE SHEETS
(In thousands)

	As of		
	December 31, 2022		
	September 30, 2023	(1)	(unaudited)
Assets			
Current assets:			
Cash and cash equivalents	\$ 45,414	\$ 24,459	
Short-term investments	16,937	33,747	
Accounts receivable, net	23,546	40,320	
Inventories	6,112	9,118	
Prepaid and other current assets	3,015	8,259	
Total current assets	95,024	115,903	
Property and equipment, net	190	857	
Intangible asset, net	14,146	14,949	
Operating lease right-of-use assets	1,008	1,930	
Other assets	4,956	640	
Total assets	\$ 115,324	\$ 134,279	
Liabilities and stockholders' deficit			
Current liabilities:			
Accounts payable	\$ 7,999	\$ 22,508	
Accrued compensation	8,093	8,866	
Accrued research and development	5,754	7,708	
Revenue reserves and refund liability	14,543	12,145	
Other accrued liabilities	5,030	6,485	
Lease liabilities, current portion	673	1,133	

Deferred revenue	1,355	1,369
Other long-term liabilities, current portion	4,826	4,997
Total current liabilities	48,273	65,211
Long-term portion of lease liabilities	466	972
Loans payable, net of discount	59,659	39,448
Other long-term liabilities	38,760	42,264
Total liabilities	147,158	147,895
Commitments		
Stockholders' deficit:		
Preferred stock	—	—
Common stock	175	174
Additional paid-in capital	1,376,293	1,368,822
Accumulated other comprehensive loss	(15)	(153)
Accumulated deficit	(1,408,287)	(1,382,459)
Total stockholders' deficit	(31,834)	(13,616)
Total liabilities and stockholders' deficit	\$ 115,324	\$ 134,279

	As of			
	March 31, 2024	December 31, 2023 (1)		
	(unaudited)			
Assets				
Current assets:				
Cash and cash equivalents	\$ 25,574	\$ 32,786		
Short-term investments	23,976	24,147		
Accounts receivable, net	27,512	30,550		
Inventories	6,579	5,522		
Prepaid and other current assets	8,845	6,261		
Total current assets	92,486	99,266		
Property and equipment, net	140	165		
Intangible assets, net	28,863	13,878		
Operating lease right-of-use assets	712	861		
Other assets	4,318	3,055		
Total assets	<u><u>\$ 126,519</u></u>	<u><u>\$ 117,225</u></u>		
Liabilities and stockholders' deficit				
Current liabilities:				
Accounts payable	\$ 9,276	\$ 7,142		
Accrued compensation	5,246	8,676		
Accrued research and development	4,127	3,513		
Acquisition-related liabilities	15,222	—		
Revenue reserves and refund liability	16,045	15,684		
Loans payable, net, current portion	14,780	7,229		
Other accrued liabilities	5,435	5,334		
Deferred revenue	1,355	1,355		
Lease liabilities, current portion	709	692		
Other long-term liabilities, current portion	1,003	3,642		
Total current liabilities	73,198	53,267		
Long-term portion of lease liabilities	100	285		
Long-term portion of loans payable, net	44,910	52,373		
Other long-term liabilities	39,982	39,944		
Total liabilities	<u><u>\$ 158,190</u></u>	<u><u>\$ 145,869</u></u>		

Commitments		
Stockholders' deficit:		
Preferred stock	—	—
Common stock	175	175
Additional paid-in capital	1,383,956	1,378,723
Accumulated other comprehensive (loss) income	(5)	8
Accumulated deficit	(1,415,797)	(1,407,550)
Total stockholders' deficit	(31,671)	(28,644)
Total liabilities and stockholders' deficit	\$ 126,519	\$ 117,225

(1) The balance sheet as of December 31, 2022 December 31, 2023 has been derived from the audited financial statements included in Rigel's Annual Report on Form 10-K for the year ended December 31, 2022 December 31, 2023 filed with the Securities and Exchange Commission (SEC) on March 7, 2023 March 5, 2024.

See Accompanying Notes to Condensed Financial Statements

3

[Table of Contents](#)

RIGEL PHARMACEUTICALS, INC.
CONDENSED STATEMENTS OF OPERATIONS
(In thousands, except per share amounts)
(unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,		Three Months Ended March 31,	
	2023	2022	2023	2022	2024	2023
Revenues:						
Product sales, net	\$ 27,129	\$ 19,188	\$ 74,755	\$ 53,935	\$ 26,003	\$ 23,745
Contract revenues from collaborations	1,005	722	5,335	12,529	3,531	2,325
Government contract	—	2,500	1,000	2,500		
Total revenues	28,134	22,410	81,090	68,964	29,534	26,070
Costs and expenses:						
Cost of product sales	1,268	250	3,320	1,407	2,025	977
Research and development	6,475	14,666	21,336	44,907	6,026	10,089
Selling, general and administrative	24,856	25,897	78,891	80,279	28,449	27,729
Total costs and expenses	32,599	40,813	103,547	126,593	36,500	38,795
Loss from operations	(4,465)	(18,403)	(22,457)	(57,629)	(6,966)	(12,725)
Interest income	672	192	1,594	255	593	393
Interest expense	(1,899)	(826)	(4,965)	(2,600)	(1,874)	(1,204)
Net loss	\$ (5,692)	\$ (19,037)	\$ (25,828)	\$ (59,974)	\$ (8,247)	\$ (13,536)
Net loss per share, basic and diluted	\$ (0.03)	\$ (0.11)	\$ (0.15)	\$ (0.35)	\$ (0.05)	\$ (0.08)

Weighted average shares used in computing net loss per share, basic and diluted	174,364	172,836	173,897	172,256	175,203	173,568
---	---------	---------	---------	---------	---------	---------

See Accompanying Notes to Condensed Financial Statements

4

[Table of Contents](#)

RIGEL PHARMACEUTICALS, INC.
CONDENSED STATEMENTS OF COMPREHENSIVE LOSS
 (In thousands)
 (unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,		Three Months Ended March 31,	
			2023	2022	2024	2023
	\$ (5,692)	\$ (19,037)	\$ (25,828)	\$ (59,974)	\$ (8,247)	\$ (13,536)
Net loss						
Other comprehensive gain (loss):						
Net unrealized gain (loss) on short-term investments	10	152	138	(184)		
Other comprehensive (loss) gain:						
Net unrealized (loss) gain on short-term investments					(13)	126
Comprehensive loss	\$ (5,682)	\$ (18,885)	\$ (25,690)	\$ (60,158)	\$ (8,260)	\$ (13,410)

See Accompanying Notes to Condensed Financial Statements

5

[Table of Contents](#)

RIGEL PHARMACEUTICALS, INC.
CONDENSED STATEMENTS OF STOCKHOLDERS' (DEFICIT) EQUITY DEFICIT
 (In thousands, except share amounts)
 (unaudited)

		Additional	Accumulated	Other	Total			Additional	Accumulated	Other
Common Stock		Paid-in	Comprehensive	Accumulated	Stockholders'	Common Stock	Paid-in	Comprehensive	Accumulated	Deficit
Shares	Amount	Capital	Loss	Deficit	Deficit	Shares	Amount	Capital	Income (Loss)	Deficit

Balance as of												
January 1,												
2023	173,398,645	\$ 174	\$1,368,822	\$ (153)	\$ (1,382,459)	\$ (13,616)						
Balance as of												
January 1,												
2024							174,825,610	\$ 175	\$1,378,723	\$ 8	\$ (1,407)	
Net loss	—	—	—	—	(13,536)	(13,536)	—	—	—	—	—	(8)
Net change in unrealized gain on short-term investments	—	—	—	126	—	126	—	—	—	—	—	(13)
Net change in unrealized loss on short-term investments	—	—	—	—	—	—	—	—	—	—	—	—
Issuance of common stock upon exercise of options	952	—	1	—	—	1	90,544	—	89	—	—	
Issuance of common stock upon vesting of restricted stock units (RSUs)	266,256	—	—	—	—	—	489,992	—	—	—	—	
Stock-based compensation expense	—	—	2,768	—	—	2,768	—	—	5,144	—	—	
Balance as of												
March 31,												
2023	173,665,853	174	1,371,591	(27)	(1,395,995)	(24,257)						
Net loss	—	—	—	—	(6,600)	(6,600)						
Net change in unrealized gain on short-term investments	—	—	—	2	—	2	—	—	—	—	—	—
Issuance of common stock upon exercise of options and participation in Purchase Plan	523,795	1	553	—	—	554	—	—	—	—	—	
Issuance of common stock upon vesting of RSUs	168,750	—	—	—	—	—	—	—	—	—	—	

Stock-based compensation expense	—	—	2,186	—	—	—	2,186
Balance as of June 30, 2023	174,358,398	\$ 175	\$1,374,330	\$ (25)	\$(1,402,595)	\$ (28,115)	
Net loss	—	—	—	—	(5,692)	(5,692)	
Net change in unrealized gain on short-term investments	—	—	—	10	—	10	
Issuance of common stock upon exercise of options	8,830	—	8	—	—	8	
Stock-based compensation expense	—	—	1,955	—	—	—	1,955
Balance as of September 30, 2023	174,367,228	\$ 175	\$1,376,293	\$ (15)	\$(1,408,287)	\$ (31,834)	
Balance as of March 31, 2024	175,406,146	\$ 175	\$1,383,956	\$ (5)	\$(1,415)		

	Additional Accumulated Other Total						Additional Accumulated Other					
	Common Stock		Paid-in	Comprehensive	Accumulated	Stockholders' Equity (Deficit)	Common Stock		Paid-in	Comprehensive	Accumulated	Deficit
	Shares	Amount	Capital	Loss	Deficit	Equity (Deficit)	Shares	Amount	Capital	(Loss)	Deficit	
Balance as of January 1, 2022	171,602,226	\$ 172	\$1,354,190	\$ (102)	\$(1,323,886)	\$ 30,374	173,398,645	\$ 174	\$1,368,822	\$ (153)	\$(1,383,956)	
Net loss	—	—	—	—	(27,445)	(27,445)	—	—	—	—	—	(1,415)
Net unrealized loss on short-term investments	—	—	—	(314)	—	(314)	—	—	—	—	—	126
Net change in unrealized gain on short-term investments	—	—	—	—	—	—	—	—	—	—	—	
Issuance of common stock upon exercise of options	420,521	—	940	—	—	940	952	—	1	—	—	

Issuance of common stock upon vesting of RSUs	22,500	—	—	—	—	—	266,256	—	—	—
Stock-based compensation expense	—	—	3,243	—	—	—	3,243	—	—	2,768
Balance as of March 31, 2022	172,045,247	172	1,358,373	(416)	(1,351,331)	6,798				
Net loss	—	—	—	—	(13,492)	(13,492)				
Net unrealized loss on short-term investments	—	—	—	(22)	—	(22)				
Issuance of common stock upon exercise of options and participation in Purchase Plan	609,839	1	598	—	—	599				
Issuance of common stock upon vesting of RSUs	181,250	—	—	—	—	—	—	—	—	—
Stock-based compensation expense	—	—	2,440	—	—	—	2,440	—	—	—
Balance as of June 30, 2022	172,836,336	\$ 173	\$ 1,361,411	\$ (438)	\$ (1,364,823)	\$ (3,677)				
Net loss	—	—	—	—	(19,037)	(19,037)				
Net unrealized gain on short-term investments	—	—	—	152	—	152				
Stock-based compensation expense	—	—	2,728	—	—	—	2,728	—	—	—
Balance as of September 30, 2022	172,836,336	\$ 173	\$ 1,364,139	\$ (286)	\$ (1,383,860)	\$ (19,834)				
Balance as of March 31, 2023							173,665,853	\$ 174	\$ 1,371,591	\$ (27) \$(1,39

See Accompanying Notes to Condensed Financial Statements

[Table of Contents](#)

RIGEL PHARMACEUTICALS, INC.
CONDENSED STATEMENTS OF CASH FLOWS
(In thousands)
(unaudited)

	Nine Months Ended September 30,	
	2023	2022
Operating activities		
Net loss	\$ (25,828)	\$ (59,974)
Adjustments to reconcile net loss to net cash used in operating activities:		
Stock-based compensation expense	6,873	8,305
Loss (gain) on sale and disposal of fixed assets	376	(465)
Depreciation and amortization	945	714
Non-cash interest expense	—	682
Net amortization and accretion of discount on short-term investments and term loan	(219)	116
Changes in assets and liabilities:		
Accounts receivable, net	16,774	(53)
Inventories	(1,359)	(394)
Prepaid and other current assets	5,244	1,255
Other assets	85	490
Right-of-use assets	922	6,712
Accounts payable	491	(999)
Accrued compensation	(773)	(1,917)

Accrued research and development	(1,954)	(2,810)
Revenue reserves and refund liability	2,398	3,274
Other accrued liabilities	(1,455)	1,637
Lease liability	(966)	(7,337)
Deferred revenue	(14)	(1,227)
Other current and long-term liabilities	(1,043)	142
Net cash provided by (used in) operating activities	497	(51,849)
Investing activities		
Maturities of short-term investments	35,650	80,062
Purchases of short-term investments	(18,222)	(26,049)
Purchases of intangible asset	(15,000)	—
Proceeds from sale of property and equipment	149	543
Purchases of property and equipment	—	(377)
Net cash provided by investing activities	2,577	54,179
Financing activities		
Net proceeds from term loan financing	19,950	19,542
Net proceeds from issuances of common stock upon exercise of options and participation in Purchase Plan	563	1,539
Cost share payments to a collaboration partner	(2,632)	(12,435)
Net cash provided by financing activities	17,881	8,646
Net increase in cash and cash equivalents	20,955	10,976
Cash and cash equivalents at beginning of period	24,459	18,890
Cash and cash equivalents at end of period	\$ 45,414	\$ 29,866
Supplemental disclosure of cash flow information		
Interest paid	\$ 4,167	\$ 1,549
	Three Months Ended March 31,	
	2024	
	2023	
Operating activities		
Net loss	\$ (8,247)	\$ (13,536)
Adjustments to reconcile net loss to net cash used in operating activities:		
Stock-based compensation expense	5,134	2,758
Loss on sale and disposal of fixed assets	—	347
Depreciation and amortization	400	357
Net amortization of discount on short-term investments and term loan	(205)	(68)
Changes in assets and liabilities:		
Accounts receivable, net	3,038	10,954
Inventories	(2,310)	(1,949)
Prepaid and other current assets	(2,584)	392
Other assets	—	98
Right-of-use assets	149	807
Accounts payable	2,134	(1,213)
Accrued compensation	(3,430)	(3,205)
Accrued research and development	614	(768)
Revenue reserves and refund liability	361	1,943
Other accrued liabilities	101	(1,389)
Lease liability	(168)	(854)
Other current and long-term liabilities	—	1,252
Net cash used in operating activities	(5,013)	(4,074)
Investing activities		
Maturities of short-term investments	8,250	15,650
Purchases of short-term investments	(7,799)	—

Payments for acquisition of intangible assets	(138)	(15,000)
Proceeds from sale of property and equipment	—	127
Net cash provided by investing activities	313	777
Financing activities		
Net proceeds from term loan financing	—	19,950
Net proceeds from issuances of common stock upon exercise of options	89	1
Cost share payments to a collaboration partner	(2,601)	(828)
Net cash (used in) provided by financing activities	(2,512)	19,123
Net (decrease) increase in cash and cash equivalents	(7,212)	15,826
Cash and cash equivalents at beginning of period	32,786	24,459
Cash and cash equivalents at end of period	<u>\$ 25,574</u>	<u>\$ 40,285</u>
Supplemental disclosure of cash flow information		
Interest paid	\$ 1,684	\$ 1,011
Intangible assets included within acquisition-related liabilities	<u>\$ 15,222</u>	<u>\$ —</u>

See Accompanying Notes to Condensed Financial Statements

[Table of Contents](#)

Rigel Pharmaceuticals, Inc.
Notes to Condensed Financial Statements
(unaudited)

In this report, "Rigel," "we," "us" and "our" refer to Rigel Pharmaceuticals, Inc.

1. Organization and Summary of Significant Accounting Policies

Description of Business

We are a biotechnology company dedicated to **discovering**, developing and providing novel therapies that significantly improve the lives of patients with hematologic disorders and cancer. We focus on products that address signaling pathways that are critical to disease mechanisms.

Our first product approved by the US Food and Drug Administration (FDA) is TAVALISSE® (fostamatinib disodium hexahydrate) tablets, the only approved oral spleen tyrosine kinase (SYK) inhibitor for the treatment of adult patients with chronic immune thrombocytopenia (ITP) who have had an insufficient response to a previous treatment. The product is also commercially available in Europe and the United Kingdom (UK) (as TAVLESSE), and in Canada, Israel and Japan (as TAVALISSE) for the treatment of chronic ITP in adult patients.

Our second **FDA approved** **FDA-approved** product is REZLIDHIA® (olutasidenib) capsules for the treatment of adult patients with relapsed or refractory (R/R) acute myeloid leukemia (AML) with a susceptible isocitrate dehydrogenase-1 (IDH1) mutation as detected by an FDA-approved test. We began our commercialization of REZLIDHIA in December 2022. We in-licensed olutasidenib from Forma Therapeutics, Inc. (**now**, **now Novo Nordisk** Novo Nordisk (Forma), with exclusive, worldwide rights for its development, manufacturing and commercialization.

In February 2024, we entered into an Asset Purchase Agreement with Blueprint Medicines Corporation (Blueprint) to purchase certain assets comprising the right to research, develop, manufacture and commercialize GAVRETO® (pralsetinib) in the US. GAVRETO (pralsetinib) is a once daily, small molecule, oral, kinase inhibitor of wild-type rearranged during transfection (RET) and oncogenic RET fusions. GAVRETO is approved by the FDA for the treatment of adult patients with metastatic RET fusion-positive non-small cell lung cancer (NSCLC) as detected

by an FDA-approved test. GAVRETO is also approved under accelerated approval based on overall response rate and duration response rate, for the treatment of adult and pediatric patients 12 years of age and older with advanced or metastatic RET fusion-positive thyroid cancer who require systemic therapy and who are radioactive iodine-refractory (if radioactive iodine is appropriate). We intend to distribute and market GAVRETO for approved indications in RET fusion-positive NSCLC and advanced thyroid cancers, and we expect to complete the transition of the asset and start recognizing product sales in July of 2024.

We continue to advance the development of our interleukin receptor-associated kinase (IRAK) 1/kinases 1 and 4 (IRAK1/4) inhibitor program, in an open-label, Phase 1b trial to determine the tolerability and preliminary efficacy of the drug in patients with lower-risk myelodysplastic syndrome (MDS) who are refractory or resistant to prior therapies.

We have strategic development collaborations with the University of Texas MD Anderson Cancer Center (MDACC) to expand our evaluation of REZLIDHIA (olutasidenib) in AML and other hematologic cancers, and with Collaborative Network for Neuro-Oncology Clinical Trials (CONNECT) to conduct a Phase 2 clinical trial to evaluate REZLIDHIA (olutasidenib) in combination with temozolamide in patients with high-grade glioma (HGG) harboring an IDH1 mutation.

We have a receptor-interacting serine/threonine-protein kinase 1 (RIPK1) inhibitor program in clinical development with our partner Eli Lilly and Company (Lilly). We also have product candidates in clinical development with partners BerGenBio ASA (BerGenBio) and Daiichi Sankyo (Daichi).

Basis of Presentation

Our accompanying unaudited condensed financial statements have been prepared in accordance with United States generally accepted accounting principles (US GAAP), for interim financial information and pursuant to the

[Table of Contents](#)

instructions to Form 10-Q and Article 10 of Regulation S-X of the Securities Act of 1933, as amended (Securities Act). Accordingly, they do not include all the information and notes required by US GAAP for complete financial statements. These unaudited condensed financial statements include only normal and recurring adjustments that we believe are necessary to fairly state our financial position and the results of our operations and cash flows. Interim-period results are not necessarily indicative of results of operations or cash flows for a full-year or any subsequent interim period. The balance sheet as of **December 31, 2022** **December 31, 2023** has been derived from audited financial statements at that date but does not include all disclosures required by US GAAP for complete financial statements. Because certain disclosures required by US GAAP for complete financial statements are not included herein, these interim unaudited condensed financial statements and the notes accompanying them should be read in conjunction with our audited financial statements and the notes thereto included in our Annual Report on Form 10-K for the year ended **December 31, 2022** **filed with the SEC on March 7, 2023** **December 31, 2023**.

[Table of Contents](#)

Use of Estimates

The preparation of financial statements in conformity with US GAAP requires management to make estimates and assumptions that affect the amounts reported in the financial statements and accompanying notes. We base our estimates on historical experience and on various other assumptions that we believe to be reasonable under the circumstances. Actual results could differ from these estimates.

Significant Accounting Policies

Our significant accounting policies are described in "Note 1 – Description of Business and Summary of Significant Accounting Policies" to our "Notes to Financial Statements" contained in "Part II, Item 8, Financial Statements and Supplementary Data" of our Annual Report on Form 10-K for the year ended December 31, 2022 December 31, 2023. There have been no material changes to these accounting policies, except for the accounting consideration related to the Asset Purchase Agreement with Blueprint as discussed in "Note 5 – In-licensing and Acquisition."

Liquidity

As of September 30, 2023 March 31, 2024, we had approximately \$62.4 million \$49.6 million in cash, cash equivalents and short-term investments. Since inception, we have financed our operations primarily through sales of equity securities, debt financing, contract payments under our collaboration agreements and from product sales.

Based on our current operating plan, we believe that our existing cash, cash equivalents, and short-term investments will be sufficient to fund our expenses and capital expenditure requirements for at least the next 12 months from the date of issuance of this Form 10-Q.

Recently Issued Accounting Standards

Recently In November 2023, FASB issued ASU 2023-07, *Segment Reporting (Topic 280): Improvements to Reportable Segment Disclosures*. This update expands public entities' segment disclosures, among others, requiring disclosure of significant segment expenses that are regularly provided to the chief operating decision maker and included within each reported measure of segment profit or loss; an amount and description of its composition for other segment items; and interim disclosures of a reportable segment's profit or loss and assets. All disclosure requirements under this update are also required for public entities with a single reportable segment. This update is effective for our Annual Report on Form 10-K for the fiscal year ending December 31, 2024, and interim periods thereafter. Early adoption is permitted. The update should be applied retrospectively to all periods presented in the financial statements. We are currently evaluating the impact of adopting this update on our financial statements and disclosures.

In December 2023, FASB issued ASU 2023-09, *Improvements to Income Tax Disclosures*, which enhance the annual disclosure requirements regarding the tax rate reconciliation and incomes taxes paid information. This update is effective for our fiscal year ending December 31, 2025, and maybe adopted on a prospective or retrospective basis. Early adoption is permitted. We are currently assessing the impact of adopting this guidance but does not expect to have a significant impact to our financial statements and disclosures.

Other recently issued accounting guidance is not discussed in this Quarterly Report on Form 10-Q are either not applicable or did not have, or is not expected to have, a material impact to us.

[Table of Contents](#)

2. Net Loss Per Share

Basic net loss per share is computed by dividing net loss by the weighted-average number of shares of common stock outstanding during the period. Diluted net loss per share is computed by dividing net loss by the weighted-average number of shares of common stock outstanding during the period and the number of additional shares of common stock that would have been outstanding if potentially dilutive securities had been issued. Potentially dilutive securities include stock options, RSUs and shares issuable under our Employee Stock Purchase Plan (Purchase Plan). The dilutive effect of these potentially dilutive securities is reflected in diluted earnings per share using the treasury stock method. Under the treasury stock method, an increase in the fair market value of our common stock can result in a greater dilutive effect from potentially dilutive securities.

The potential shares of common stock that were excluded from the computation of diluted net loss per share for the periods presented because including them would have been antidilutive are as follows (in thousands):

	Three Months Ended March 31,					
	Three Months Ended September 30,		Nine Months Ended September 30,			
	2023	2022	2023	2022	2024	2023
Outstanding stock options	34,284	32,687	34,284	32,687	37,135	35,909
RSUs	1,937	1,174	1,937	1,174	4,072	1,988
Purchase Plan	309	398	309	398	246	345
Total	36,530	34,259	36,530	34,259	41,453	38,242

[Table of Contents](#)

3. Revenues

Revenues disaggregated by category were as follows (in thousands):

	Nine Months Ended September 30,					
	Three Months Ended September 30,		30,		Three Months Ended March 31,	
	2023	2022	2023	2022	2024	2023
Product sales:						
Gross product sales	\$ 38,585	\$ 26,977	\$ 105,239	\$ 76,022	\$ 38,426	\$ 33,198
Discounts and allowances	(11,456)	(7,789)	(30,484)	(22,087)	(12,423)	(9,453)
Total product sales, net	27,129	19,188	74,755	53,935	26,003	23,745
Revenues from collaborations:						
License revenues	—	—	—	2,545		
Development milestones	75	—	75	5,000		
Royalty, delivery of drug supplies and others	930	722	5,260	4,984		
Delivery of drug supplies, royalty and others					3,531	2,325
Total revenues from collaborations	1,005	722	5,335	12,529	3,531	2,325
Government contract	—	2,500	1,000	2,500		
Total revenues	\$ 28,134	\$ 22,410	\$ 81,090	\$ 68,964	\$ 29,534	\$ 26,070

Revenue from product sales are related to sales of our commercial products, TAVALISSE and REZLIDHIA, to our specialty distributors. For detailed discussions of our revenues from collaboration and government contract, collaborations, see "Note 4 – Sponsored Research, and License Agreements and Government Contract, Contracts."

Our net product sales include gross product sales, net of chargebacks, discounts and fees, government and other rebates and returns. Of the total discounts and allowances from gross product sales for the nine three months ended September 30, 2023 March 31, 2024 and 2022, \$29.7 million \$12.3 million and \$19.7 million \$9.2 million, respectively, was accounted for as additions to revenue reserves and refund liability, and \$0.8 million \$0.1 million and \$2.4 million \$0.3 million, respectively, as reductions in accounts receivable (as it relates to allowance for prompt pay discount) and prepaid and other current assets (as it relates to certain chargebacks and other fees that were prepaid) in the condensed balance sheet. The following tables summarize the activities in chargebacks, discounts and fees, government and other rebates and returns that were accounted for within revenue reserves and refund liability, for each of the periods presented (in thousands):

	Chargebacks, Government				Chargebacks, Government			
	Discounts and and Other				Discounts and and Other			
	Fees	Rebates	Returns	Total	Fees	Rebates	Returns	Total
Balance as of January 1, 2023	\$ 6,213	\$ 2,636	\$ 3,296	\$ 12,145				
Balance as of January 1, 2024					\$ 8,236	\$ 3,517	\$ 3,931	\$ 15,684
Provision related to current period sales	23,150	5,932	623	29,705	9,763	2,280	224	12,267
Credit or payments made during the period	(21,504)	(5,631)	(172)	(27,307)	(10,255)	(1,609)	(42)	(11,906)
Balance as of September 30, 2023	\$ 7,859	\$ 2,937	\$ 3,747	\$ 14,543				
Balance as of March 31, 2024					\$ 7,744	\$ 4,188	\$ 4,113	\$ 16,045

10

	Chargebacks, Government			
	Discounts and and Other			
	Fees	Rebates	Returns	Total
Balance as of January 1, 2022	\$ 3,404	\$ 2,494	\$ 2,017	\$ 7,915
Provision related to current period sales	14,475	4,130	1,045	19,650
Credit or payments made during the period	(11,920)	(4,257)	(199)	(16,376)
Balance as of September 30, 2022	\$ 5,959	\$ 2,367	\$ 2,863	\$ 11,189

[Table of Contents](#)

	Chargebacks, Government			
	Discounts and and Other			
	Fees	Rebates	Returns	Total
Balance as of January 1, 2023	\$ 6,213	\$ 2,636	\$ 3,296	\$ 12,145
Provision related to current period sales	7,231	1,795	197	9,223
Credit or payments made during the period	(5,777)	(1,422)	(81)	(7,280)
Balance as of March 31, 2023	\$ 7,667	\$ 3,009	\$ 3,412	\$ 14,088

The following table summarizes the percentages of revenues from each of our customers who individually accounted for 10% or more (wherein * denotes less than 10%) of the total net product sales and revenues from collaborations:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2023	2022	2023	2022
McKesson Specialty Care Distribution Corporation	49%	44%	46%	38%
Cardinal Healthcare	28%	27%	27%	22%
ASD Healthcare and Oncology Supply	18%	25%	20%	21%
Kissei	*	*	*	11%

10

[Table of Contents](#)

	Three Months Ended March 31,	
	2024	2023
McKesson Specialty Care Distribution Corporation	42%	45%
Cardinal Healthcare	23%	24%
Cencora Inc. (formerly ASD Healthcare)	23%	22%

4. Sponsored Research, and License Agreements and Government Contract Contracts

Sponsored Research and License Agreements

We conduct research and development programs independently and in connection with our corporate collaborators. As of **September 30, 2023** **March 31, 2024**, we are a party to collaboration agreements with Lilly to develop and commercialize R552, a RIPK1 inhibitor, for the treatment of non-central nervous system (non-CNS) diseases and collaboration aimed at developing additional RIPK1 inhibitors for the treatment of central nervous system (CNS) diseases; with Grifols S.A. (Grifols) to commercialize fostamatinib for human diseases in all indications including chronic ITP and autoimmune hemolytic anemia (AIHA), in Grifols territory which includes Europe, the UK, Turkey, the Middle East, North Africa and Russia (including Commonwealth of Independent States); with Kissei Pharmaceutical Co., Ltd. (Kissei) to develop and commercialize fostamatinib in Kissei territory which includes Japan, China, Taiwan and the Republic of Korea; with Medison Pharma Trading AG (Medison Canada) and Medison Pharma Ltd. (Medison Israel and, together with Medison Canada, Medison) to commercialize fostamatinib in all indications, including chronic ITP and AIHA, in Medison territory which includes Canada and Israel; and with Knight Therapeutics International SA (Knight) to commercialize fostamatinib in all indications, including chronic ITP and AIHA, in Knight territory which includes Latin America, consisting of Mexico, Central and South America, and the Caribbean (Knight territory).

Further, we are also a party to collaboration agreements, but do not have ongoing performance obligations with BerGenBio for the development and commercialization of AXL receptor tyrosine kinase (AXL) inhibitors in oncology, and with Daiichi to pursue research related to murine double minute 2 (MDM2) inhibitors, a novel class of drug targets called ligases.

Under the above existing agreements that we entered into in the ordinary course of business, we received or may be entitled to receive upfront cash payments, payments contingent upon specified events achieved by such partners and royalties on any net sales of products sold by such partners under the agreements. As of **September 30, 2023** **March 31, 2024**, total future contingent payments to us under all of the above existing agreements, excluding terminated agreements, could exceed \$1.3 billion if all potential product candidates achieved all of the payment triggering events under all of our current agreements. Of this amount, \$279.5 million relates to the achievement of development events, \$263.1 million relates to the achievement of regulatory events and \$796.0 million relates to the achievement of certain commercial events. This estimated future contingent amount does not include any estimated royalties that could be due to us if the partners successfully commercialize any of the licensed products. Future events that may trigger payments to us under the agreements are based solely on our partners' future efforts and achievements of specified development, regulatory and/or commercial events.

We account for the milestone payments when such milestones are considered probable of being achieved, and estimate the amount to be included in the transaction price using the most likely amount method. If it is probable that a significant revenue reversal would not occur, the associated milestone value is included in the transaction price. Milestone payments that are not within our or the licensee's control, such as regulatory approvals, are not considered.

[Table of Contents](#)

probable of being achieved until uncertainty associated with the approvals has been resolved. The transaction price is then allocated to each performance obligation, on a relative standalone selling price basis, for which we recognize revenue as or when the performance obligations under the contract are satisfied. At the end of each subsequent reporting period, we re-evaluate the probability of achieving such milestones and any related constraint, and if necessary, adjust our estimate of the overall transaction price. Any such adjustments are recorded on a cumulative catch-up basis, and recorded as part of contract revenues from collaborations during the period of adjustment.

Global Exclusive License Agreement with Lilly

We have a global exclusive license agreement and strategic collaboration with Lilly (Lilly Agreement) entered in February 2021, which became effective on March 27, 2021, upon clearance under the Hart-Scott-Rodino Antitrust Improvements Act of 1976, to develop and commercialize R552 for the treatment of non-CNS diseases. In addition, the collaboration is aimed at developing additional RIPK1 inhibitors for the treatment of CNS diseases. Pursuant to the terms of the Lilly Agreement, we granted Lilly the exclusive rights to develop and commercialize R552 and related RIPK1 inhibitors in all indications worldwide. The parties' collaboration is governed through a joint governance committee and appropriate subcommittees.

Under the terms of the Lilly Agreement, we were entitled to receive a non-refundable and non-creditable upfront cash payment amounting to \$125.0 million, which we received in April 2021. We are also entitled to additional milestone payments for non-CNS disease products consisting of up to \$330.0 million in milestone payments upon the achievement of specified development, regulatory and commercial milestones, and up to \$100.0 million in sales milestone payments on a product-by-product basis. In addition, depending on the extent of our co-funding of R552 development activities, we would be entitled to receive tiered royalty payments on net sales of non-CNS disease products at percentages ranging from the mid-single digits to high-teens, subject to certain standard reductions and offsets. We are also eligible to receive milestone payments for CNS disease products consisting of up to \$255.0 million in milestone payments upon the achievement of specified development, regulatory and commercial milestones, and up to \$150.0 million in sales milestone payments on a product-by-product basis. We would be entitled to receive tiered royalty

11

[Table of Contents](#)

payments on net sales of CNS disease products up to low-double digits, subject to certain standard reductions and offsets.

Under the Lilly Agreement, we are responsible for performing and funding initial discovery and identification of CNS disease development candidates. Following candidate selection, Lilly will be responsible for performing and funding all future development and commercialization of the CNS disease development candidates. We are responsible for 20% of development costs for R552 in the US, Europe, and Japan, up to a specified cap, and Lilly is responsible for funding the remainder of all development activities for R552 and other non-CNS disease development candidates. Pursuant to the terms of the Lilly Agreement, we have the right to opt-out of co-funding the R552 development activities in the US, Europe and Japan at two different specified times and as a result receive lesser royalties from sales. Prior to us providing our first opt-out notice as discussed below, under the Lilly Agreement, we were required to fund our share of the R552 development activities up to a maximum funding commitment of \$65.0 million through April 1, 2024.

On September 28, 2023, we entered into an amendment to the Lilly Agreement which provides, among others that if we exercise our first opt-out right, we have the right to opt-in to the co-funding of R552 development, upon us providing notice to Lilly within 30 days of certain events as specified in the Lilly Agreement, and as a result receive greater royalties from sales. Following the amendment to the Lilly Agreement, on September 29, 2023, we provided the first opt-out notice to Lilly. We continued to fund our share of the R552 development activities up to \$22.6 million through April 1, 2024 as provided for in the amended Lilly Agreement. If we decide to exercise our opt-in right, we will be required to continue to share in global development costs, and if we later exercise our second opt-out right (no later than April 1, 2025), our share in global development costs will be up to a specified cap through December 31, 2025, as provided for in the Lilly Agreement.

On September 29, 2023, we provided the first opt-out notice to Lilly. We will continue to fund our share of the R552 development activities up to \$22.6 million through April 1, 2024 as provided for in the amended Lilly Agreement.

We accounted for this agreement under ASC 606 and identified the following distinct performance obligations at inception of the agreement: (a) granting of the license rights over the non-CNS penetrant intellectual property (IP), and (b) granting of the license rights over the CNS penetrant IP which will be delivered to Lilly upon completion of the additional research and development efforts specified in the agreement. We concluded that each of these performance obligations is distinct. We based our assessment on the assumption that Lilly can benefit from each of the licenses on its own by developing and commercializing the underlying product using its own resources.

[Table of Contents](#)

At the inception of the Lilly Agreement, given our rights to opt-out from the development of R552, we believed at the minimum, we had a commitment to fund the development costs up to \$65.0 million as discussed above. We considered this commitment to fund the development costs as a significant financing component of the contract, which we accounted for as a reduction of the upfront fee to derive the transaction price. This financing component was recorded as a liability at its net present value of approximately \$57.9 million using a 6.4% discount rate. Interest expense is accreted on such liability over the expected commitment period, adjusted for timing of expected cost share payments. No interest was accreted during the **nine** three months ended **September 30, 2023**, **March 31, 2024** and **\$0.7 million** of interest was accreted during **the nine months ended September 30, 2022**, **2023**. Through **September 30, 2023**, **March 31, 2024**, Lilly billed us **\$17.7 million** **\$20.3 million** for our share of development costs **incurred** as of the fourth quarter of 2023, and the amount was fully paid as of **September 30, 2023**, **March 31, 2024**. As of **September 30, 2023**, **March 31, 2024** and **December 31, 2022**, **December 31, 2023**, the outstanding liability to Lilly was **\$43.6 million** **\$41.0 million** and **\$46.2 million** **\$43.6 million**, respectively, and included within other long-term liabilities, current portion, and other long-term liabilities in the condensed balance sheet. As discussed above, following the amendment to the Lilly Agreement, and us providing the first opt-out notice to Lilly, our cumulative share of the R552 development cost is now capped at \$22.6 million through April 1, 2024. Although our cumulative share of the development cost is now at the specified cap that is less than our outstanding recorded liability at the balance sheet date, such excess amount has not been recognized as revenue because we cannot conclude that it is probable that a significant reversal of the amount of revenue, if recognized, will not occur until the likelihood of us exercising our opt-in right becomes remote, or when the opt-in right period lapses.

At the inception, we allocated the net transaction price of \$67.1 million to each performance obligation based on our best estimate of its relative standalone selling price using the adjusted market assessment approach. The transaction price allocated to the non-CNS penetrant IP of \$60.4 million was recognized as revenue upon delivery of the non-CNS penetrant IP to Lilly during the first quarter of 2021. The transaction price allocated to the CNS penetrant IP of \$6.7 million was recognized as revenue from the effective date of the Lilly Agreement through the eventual acceptance by Lilly using the input method, since we were required to perform additional research and development efforts before the final acceptance of the license by Lilly. In June 2022, Lilly provided notice of continuance pursuant to the terms of the Lilly Agreement, whereby Lilly elected its option to lead the identification and selection of CNS penetrant lead

[Table of Contents](#)

candidate. As such, we recognized the remaining outstanding deferred revenue in the second quarter of 2022. For the three and nine months ended September 30, 2022, we recognized **There was no outstanding deferred revenue** and **\$0.5 million** of revenue, respectively, associated with the delivery of CNS penetrant IP. No such revenue was recognized in the three and nine months ended September 30, 2023.

The remaining future variable consideration related to **future milestone payments** **Lilly Agreement** as discussed above were fully constrained because we cannot conclude that it is probable that a significant reversal of the amount of cumulative revenue recognized will not occur, given the inherent uncertainty of success with these future milestones. For sales-based milestones **March 31, 2024** and **royalties**, we determined that the license is the predominant item to which the royalties or sales-based milestones relate. Accordingly, we will recognize revenue at the later of (i) when the related sales occur, or (ii) when the performance obligation to which some or all of the royalty has been allocated has been satisfied (or partially satisfied) **December 31, 2023**. We will re-evaluate the transaction price in each reporting period and as uncertain events are resolved or other changes in circumstances occur.

Grifols License Agreement

We have an exclusive commercialization license agreement with Grifols entered in January 2019 with exclusive rights to commercialize fostamatinib for human diseases, including chronic ITP and AIHA, and non-exclusive rights to develop fostamatinib in Grifols territory. Under the agreement, we received an upfront payment of \$30.0 million, with the potential for \$297.5 million in total regulatory and commercial milestones. We are also entitled to receive stepped double-digit royalty payments based on tiered net sales which may reach 30% of net sales. The agreement also required us to continue to conduct our long-term open-label extension study on patients with ITP through European Medicines Agency (EMA) approval of ITP in Europe or until the study ends as well as conduct the Phase 3 trial of fostamatinib in AIHA, autoimmune hemolytic anemia (AIHA).

In January 2020, the European Commission (EC) granted a centralized Marketing Authorization (MA) for fostamatinib valid throughout the European Union (EU) and in the UK after the departure of the UK from the EU for the treatment of chronic ITP in adult patients who are refractory to other treatments. With this approval, in February 2020, we received \$20.0 million non-refundable payment, composed of a \$17.5 million payment due upon Marketing Authorization Application (MAA) approval by the EMA of fostamatinib for the first indication and a \$2.5 million creditable advance royalty payment, based on the terms of our collaboration agreement with Grifols. The above milestone payment was allocated to the distinct performance obligations in the collaboration agreement with Grifols.

We accounted for this agreement under ASC 606 and identified the following distinct performance obligations at inception of the agreement: (a) granting of the license, (b) performance of research and regulatory services related to our long-term open-label extension study on patients with ITP, and (c) performance of research services related to our Phase 3 study in AIHA. We allocated the transaction price to the distinct performance obligations in our collaboration agreement based on our best estimate of the relative standalone selling price, and recognized the corresponding revenue in the periods we satisfied the performance obligations. As of September 30, 2023, there was no outstanding deferred revenue. In the three and nine months ended September 30, 2022, we recognized \$0.2 million and \$0.7 million, respectively, of revenue associated with such remaining performance obligation.

The remaining variable consideration related to future regulatory Grifols license agreement as of March 31, 2024 and commercial milestones were fully constrained because we cannot conclude that it is probable that a significant reversal in the amount of cumulative revenue recognized will not occur, given the inherent uncertainty of success with these future milestones. We are recognizing revenues related to the research and regulatory services throughout the term of the respective clinical programs using the input method. For sales-based milestones and royalties, we determined that the license is the predominant item to which the royalties or sales-based milestones relate. Accordingly, we will recognize revenue at the later of (i) when the related sales occur, or (ii) when the performance obligation to which some or all of the royalty has been allocated has been satisfied (or partially satisfied) December 31, 2023. We will re-evaluate the transaction price in each reporting period and as uncertain events are resolved or other changes in circumstances occur.

[Table of Contents](#)

We entered into a Commercial Supply Agreement with Grifols in October 2020 to supply and sell our drug product priced at a certain markup specified in the agreement, in quantities Grifols order from us pursuant to and in accordance with the agreement. Prior to the Commercial Supply Agreement, we had a Drug Product Purchase Agreement with Grifols entered in December 2019. During For the three and nine months ended September 30, 2023, we recognized March 31, 2024 and 2023, no revenue and \$2.8 million \$1.6 million of revenue, respectively, was recognized related to delivery of drug supply to Grifols. During the three and nine months ended September 30, 2022, we recognized \$0.4 million and \$1.6 million of revenue, respectively, related to delivery of drug supply to Grifols.

We began recognizing royalty revenue from Grifols included within contract revenues from collaboration beginning in the third quarter of 2022. For the three and nine months ended September 30, 2023, March 31, 2024 and 2023, we recognized \$0.8 royalty revenue of \$1.1 million and \$2.3 million \$0.7 million, respectively, of royalty revenue from Grifols. For the three and nine months ended September 30, 2022, we recognized \$0.1 million of royalty revenue from Grifols, respectively.

We have an exclusive license and supply agreement with Kissei entered in October 2018, to develop and commercialize fostamatinib in all current and potential indications in Kissei's territory. Kissei is responsible for performing and funding all development activities for fostamatinib in the above-mentioned territories. We received an upfront cash payment of \$33.0 million, with the potential for up to an additional \$147.0 million in development, regulatory and commercial milestone payments, and will receive mid- to upper twenty percent, tiered, escalated net sales-based payments for the supply of fostamatinib. Under the agreement, we granted Kissei the license rights to fostamatinib in Kissei's territory and are obligated to supply Kissei with drug product for use in clinical trials and pre-commercialization activities. We are also responsible for the manufacture and supply of fostamatinib for all future development and commercialization activities under the agreement.

We accounted for this agreement under ASC 606 and identified the following distinct performance obligations at inception of the agreement: (a) granting of the license, (b) supply of fostamatinib for clinical use and (c) material right associated with discounted fostamatinib that is supplied for use other than clinical or commercial. In addition, we will provide commercial product supply if the product is approved in the licensed territory. We concluded that each of these performance obligations is distinct. We determined that the upfront fee of \$33.0 million represented the transaction price and was allocated to the performance obligations based on our best estimate of the relative standalone selling price and recognized the corresponding revenue in the period we satisfied the performance obligations. As of **September 30, 2023**, **March 31, 2024** and **December 31, 2022**, the remaining deferred revenue was related to the material right associated with discounted fostamatinib supply which amounted to \$1.4 million. **No revenue was recognized during the three months ended March 31, 2024 and 2023 associated with the remaining performance obligation.**

For the three **and nine** months ended **September 30, 2022**, we recognized an immaterial amount **March 31, 2024 and 2023**, \$2.3 million of revenue, and \$2.6 million of **no** revenue, respectively, **was recognized** related to the delivery of fostamatinib supply to Kissei mainly for commercial use. **No such revenue was recognized during the three and nine months ended September 30, 2023.**

In April 2022, Kissei announced that an NDA was submitted to Japan's Pharmaceuticals and Medical Devices Agency (PMDA) for fostamatinib in chronic ITP. With this milestone event, we received \$5.0 million non-refundable and non-creditable payment from Kissei pursuant to the terms of our collaboration agreement, and such amount was recognized as revenue in the second quarter of 2022. In December 2022, Kissei announced that Japan's PMDA approved the NDA for fostamatinib in chronic ITP. With this milestone event, we were entitled to receive \$20.0 million non-refundable and non-creditable payment from Kissei pursuant to the terms of our collaboration agreement, which we recognized as revenue in the fourth quarter of 2022. The amount was subsequently collected in January 2023.

The remaining variable consideration related to future development and regulatory milestones was fully constrained because we cannot conclude that it is probable that a significant reversal in the amount of cumulative revenue recognized will not occur, given the inherent uncertainty of success with these future milestones. For sales-based milestones and tiered, escalated net sales-based payments for the supply of fostamatinib, we determined that the license is the predominant item to which the sales-based milestones relate to. Accordingly, we will recognize revenue at the later of (i) when the related sales occur, or (ii) when the performance obligation to which some or all of the allocated costs for the tiered, escalated net sales-based payments has been satisfied (or partially satisfied). We will re-evaluate the transaction price in each reporting period and as uncertain events are resolved or other changes in circumstances occur.

[Table of Contents](#)

Medison Commercial and License Agreements

We have two exclusive commercial and license agreements with Medison entered in October 2019 for the commercialization of fostamatinib for chronic ITP in Medison territory, pursuant to which, we received a \$5.0 million upfront payment with respect to the agreement in Canada. We accounted for this agreement under ASC 606 and identified the following combined performance obligations at inception of the agreement: (a) granting of the license and (b) obtaining regulatory approval in Canada of fostamatinib in ITP. **We determined that the non-refundable upfront fee of \$5.0 million represented the transaction price.** However, under the agreement, we have the option to buy back all rights to the product in Canada within six months from obtaining regulatory approval for the treatment of AIHA in Canada. **The We determined that the non-refundable upfront fee represented the transaction price, however, due to the buyback option precludes us from transferring**

control of the license to Medison provision, we accounted this upfront payment as financing arrangement under ASC 606. We believed In 2022, management concluded that the buyback provision, if exercised, will require us to repurchase the license at an amount equal to or more than the upfront \$5.0 million. As such, this arrangement was accounted for as a financing arrangement. Interest expense was accreted on such liability over the expected buyback period. We also billed Medison for the delivery likelihood of drug supplies for clinical use which we previously deferred and included within the outstanding financing liability considering the buy-back provision.

The decision to exercise exercising the buyback option is dependent on many factors including management's cost and benefit assessments and the success of obtaining regulatory approval for the treatment of AIHA in Canada. In June 2022, we reported right was remote considering the top-line results from our Phase 3 trial of fostamatinib in warm autoimmune auto immune hemolytic anemia anemian (wAIHA) which showed that the trial did not demonstrate statistical significance in the primary efficacy endpoint, in the overall study population. We also announced in early October 2022 that we will not file a supplemental new drug application (sNDA) for wAIHA indication considering the top-line data results and the guidance received from the FDA. With these developments, we assessed our options path forward, including our buyback option right with regards to the Medison license agreement. Based on management's assessment, the likelihood

[Table of exercising the buy-back option right was remote. Contents](#)

FDA. As such, during the fourth quarter of 2022, in accordance with ASC 606, we relieved the outstanding financing liability to Medison amounting to \$5.7 million which includes the upfront payment and accrued interest, and recognized such amount as collaboration revenue in accordance with ASC 606. 2022. There was no outstanding financing liability deferred revenue related to Medison license agreement as of September 30, 2023 March 31, 2024 and December 31, 2022 December 31, 2023.

During For the three and nine months ended September 30, 2023 March 31, 2024, we recognized \$0.2 million \$0.1 million of revenue from Medison related to the delivery of drug supplies and royalty revenue. For the three months ended March 31, 2023, we recognized \$0.1 million of revenue related to the delivery of drug supplies to Medison and a milestone pursuant to the commercial and license agreement. No such revenue was recognized during the three and nine months ended September 30, 2022 supplies.

Knight Commercial License and Supply Agreement

We have commercial license and supply agreements with Knight entered in May 2022 for the commercialization of fostamatinib for approved indications in Knight territory. Pursuant to such commercial license agreement, we received a \$2.0 million one-time, non-refundable, and non-creditable upfront payment, with potential for up to an additional \$20.0 million in regulatory and sales-based commercial milestone payments, and will receive twenty- to mid-thirty percent, tiered, escalated net-sales based royalty payments for products sold in the Knight territory. We accounted for this agreement under ASC 606 and identified that the upfront payment was a consideration for granting Knight the license to commercialize fostamatinib for approved indication in the Knight territory, and no further material deliverables associated to such upfront payment. As such, we recognized the upfront payment as revenue during the second quarter of 2022. Variable consideration related to future regulatory milestones was fully constrained because we cannot conclude that it is probable that a significant reversal in the amount of cumulative revenue recognized will not occur, given the inherent uncertainty of success with these future milestones. For sales-based milestones and royalties, we determined that the license is the predominant item to which the royalties or sales-based milestones relate to. Accordingly, we will recognize revenue at the later of (i) when the related sales occur, or (ii) when the performance obligation to which some or all the royalty has been allocated has been satisfied (or partially satisfied). We will re-evaluate the transaction price in each reporting period and as uncertain events are resolved or other changes in circumstances occur. We are also responsible for the exclusive manufacture and supply of fostamatinib for all future development and commercialization activities under the agreement.

[Table of Contents](#)

Government Contract - Contracts

US Department of Defense's JPEO-CBRND Defense (DOD)

In January 2021, we were awarded up to \$16.5 million by the US Department of Defense DOD to support our ongoing Phase 3 clinical trial to evaluate the safety and efficacy of fostamatinib for the treatment of hospitalized high-risk patients with COVID-19. The amount of award we will receive from No revenue was recognized during the US Department of Defense is subject to submission of proper documentation as evidence of completion of certain clinical trial events or milestones as specified in the agreement, and approval by the US Department of Defense that such events or milestones have been met. We record government contract revenue in the statement of operations in the period when it is probable that we will receive the award, which is when we comply with the conditions associated with the award and obtain approval from the US Department of Defense that such conditions have been met. We recognized no revenue and \$1.0 million of revenue for three and nine months ended September 30, 2023, respectively, March 31, 2024 and \$2.5 million of revenue for the three and nine months ended September 30, 2022, related to this grant upon achievement of certain milestones. Through September 30, 2023 March 31, 2024, we received \$16.0 million of the award which award.

Biomedical Advanced Research and Development (BARDA)

In August 2023, we recognized as revenue in were awarded up to \$0.8 million by BARDA, part of the respective periods, with remaining \$0.5 million available, subject to us meeting a certain milestone Office of the Assistant Secretary for the Preparedness and approval by Response at the US Department of Defense that Health and Human Services (DHHS), for our evaluation of fostamatinib in mitigating the impact of long-term respiratory distress. No revenue was recognized during the three months ended March 31, 2024 and 2023. Through March 31, 2024, we received \$0.1 million of the award.

Strategic Development Collaborations with MDACC and CONNECT

In December 2023, we entered into a Strategic Collaboration Agreement with MDACC, a comprehensive cancer research, treatment, and prevention center. The collaboration will expand our evaluation of REZLIDHIA (olutasidenib) in AML and other hematologic cancers. Under the collaboration, we will provide MDACC the study materials and \$15.0 million in time-based milestone payments as compensation for services to be provided for the studies, over the five-year collaboration term, unless terminated earlier as provided for in the agreement. Through March 31, 2024, we provided \$2.0 million funding to MDACC.

In January 2024, we announced our collaboration with CONNECT, an international collaborative network of pediatric cancer centers, to conduct a Phase 2 clinical trial to evaluate REZLIDHIA (olutasidenib) in glioma. Under the collaboration, we will provide funding up to \$3.0 million and study material over the four-year collaboration.

We account for the funding we provide under the above research collaboration agreements as prepaid research and development in the balance sheet to the extent the payment is made in advance of services being rendered, and recognize such amount as research and development expense within the statements of operations as the collaborative partners render the services under the respective agreement.

Table of Contents

5. In-licensing and Acquisition

Asset Purchase Agreement with Blueprint

On February 22, 2024, we acquired the US rights to research, develop, manufacture and commercialize GAVRETO (pralsetinib) from Blueprint pursuant to an Asset Purchase Agreement. The acquired assets include, among other things, applicable intellectual property related to pralsetinib in the US, including patents, copyrights and trademarks, as well as clinical regulatory and commercial data and records. Pursuant

to the Asset Purchase Agreement, we agreed to pay a purchase price of \$15.0 million, \$10.0 million of which is payable upon our first commercial sale of GAVRETO (pralsetinib) and an additional \$5.0 million of which is payable on the first anniversary of the closing date of the agreement, subject to certain conditions. Blueprint is also eligible to receive up to \$97.5 million in future commercial milestone payments and up to \$5.0 million in future regulatory milestone payments. The potential regulatory milestones include full regulatory approval of pralsetinib (or related compounds) for the treatment of adult RET-fusion positive thyroid cancer, and maintenance of the current regulatory approval of pralsetinib for the treatment of adult RET-fusion positive thyroid cancer during the period beginning on February 22, 2024 and ending on the third anniversary of the first commercial sale of pralsetinib subject to certain conditions. Subject to the terms and conditions of the Asset Purchase Agreement, Blueprint would be entitled to tiered royalty payments on net sales of products containing pralsetinib (or related compounds) ranging from 10% to 30%, subject to certain reductions and offsets.

In accordance with ASC 805 *Business Combinations* (ASC 805), the transaction was accounted for as an asset acquisition, because substantially all of the fair value of the gross assets acquired is concentrated in a single asset, which is the GAVRETO product rights. The GAVRETO product rights comprised developed technology, customers, trademarks and trade name, and are considered a single asset as they are inextricably linked. ASC 805 provides for a screen test, wherein if substantially all of the fair value of the gross assets acquired is concentrated in a single identifiable asset or group of similar identifiable assets, the assets acquired are not considered to be a business.

The following table summarizes the total purchase consideration in connection with the asset acquisition (in thousands):

Closing purchase price	\$ 15,000
Transaction costs	360
Total purchase consideration	<u>\$ 15,360</u>

The closing purchase price was recorded within acquisition-related liabilities in the condensed balance sheet. The transaction cost of \$0.2 million was also recorded within acquisition-related liabilities, and the remaining \$0.1 million has been met, paid in cash as specified of March 31, 2024. The contingent considerations relating to future commercial and regulatory milestones were not included in the agreement total purchase price consideration, and will be accounted for when the contingency is resolved and the consideration becomes payable. Royalties will be recognized within cost of sales, as revenue from GAVRETO product sales is recognized.

In an asset acquisition, the acquiring entity should recognize the assets acquired at cost to the acquiring entity which includes transaction costs and consideration given, allocated based on a relative fair value of the assets acquired measured at acquisition date. The fair value of the developed technology, customers, trademarks and trade name was estimated using a multi-period excess earnings income approach that discounts expected cash flows to present value by applying discount rate that represents the estimated rate that market participants would use to value such assets. The relative fair value are based on estimates that required judgement and certain assumptions, categorized as Level 3 in the fair value hierarchy. Since we acquired a single asset, the total purchase consideration was recorded as intangible assets. The related intangible assets is being amortized on a straight-line basis over the estimated useful life of 12 years, and the related amortization is recorded within cost of sales.

Simultaneously and in connection with entering into the Asset Purchase Agreement, we also entered into certain supporting agreements, including a customary transition agreement, pursuant to which, during the transition period, Blueprint will transition regulatory and distribution responsibility for GAVRETO (pralsetinib) to us. We also agreed to purchase certain drug product inventories from Blueprint amounting to approximately \$7.0 million under a Material Transfer Agreement. As of March 31, 2024, we received inventories amounting to approximately \$3.1 million.

We have a license and transition services agreement with Forma (now Novo Nordisk) entered in July 2022, for an exclusive license to develop, manufacture and commercialize olutasidenib, a proprietary inhibitor of mutated IDH1 (mIDH1), for any uses worldwide, including for the treatment of AML and other malignancies. Forma became a wholly owned subsidiary of Novo Nordisk following the closing of its acquisition by Novo Nordisk in October 2022. Pursuant to the terms of the license and transition services agreement, we paid an upfront fee of \$2.0 million, with the potential to pay up to \$67.5 million of additional payments upon achievement of specified development and regulatory milestones and up to \$165.5 million of additional payments upon achievement of certain commercial milestones. In addition, subject to the terms and conditions of the license and transition services agreement, Forma (now Novo Nordisk) would be entitled to tiered royalty payments on net sales of licensed products at percentages ranging from low-teens to mid-thirties, as well as certain portion of our sublicensing revenue, subject to certain standard reductions and offsets.

The transaction was accounted for as an acquisition of asset under ASC 730, *Research and Development*. In accordance with the guidance, in a transaction accounted for as an asset acquisition, any acquired IPR&D that does not have alternative future use is charged to expense at the acquisition date. At the acquisition date, the acquired license asset was accounted for as IPR&D, and we anticipated no other economic benefit to be derived from such acquired licensed asset other than the primary indications. As such, we accounted for the upfront fee of \$2.0 million as IPR&D and recorded such cost within research and development expense in the **condensed** statements of operations in the **three and nine months ended September 30, 2022, 2022**.

Under the accounting guidance, we account for contingent cash payments when **it a contingency is probable that a liability is incurred resolved**, and the **amount can be reasonably estimated** **consideration becomes payable**. We account for milestone payment obligations incurred at development stage and prior to a regulatory approval of an indication associated with the acquired licensed asset as research and development expense when the event requiring payment of the milestone occurs. Milestone payment obligations incurred upon and after a regulatory approval of an indication associated with the acquired licensed asset, and at the commercial stage, are recorded as **intangible asset assets** when the event requiring payment of the milestones occurs. **The amount recorded as intangible asset is amortized over the estimated useful life of the acquired licensed asset. Royalty payments related to the acquired licensed asset is recorded as cost of sales when incurred.** Prior to the FDA approval of REZLIDHIA in December 2022, **we achieved a certain regulatory milestone was met** which entitled Forma (now Novo Nordisk) to receive a \$2.5 million milestone payment. Because such milestone payment obligation was incurred prior to a regulatory approval of an indication associated with the acquired licensed asset, we recorded such amount as research and development expense in the fourth quarter of 2022. On December 1, 2022, the FDA approved REZLIDHIA capsules for the treatment of adult patients with R/R AML with susceptible IDH1 mutations as detected by an FDA-approved test. Following the FDA approval, we launched REZLIDHIA and made first shipments of the product to our customers in December 2022. With this FDA approval and first commercial sale of the product, Forma (now Novo Nordisk) was entitled to receive a total of \$15.0 million milestone payments. Since such milestone payment obligations were incurred upon and after regulatory approval of the product, we recorded such amount as intangible **asset assets** on our condensed balance sheet in the fourth quarter of 2022. **The \$15.0 million No new milestone was met in milestone payment obligation was outstanding as of**

[Table of Contents](#)

December 31, 2022 2023 and **included within accounts payable in our condensed balance sheet. Such amount was paid in the first quarter of 2023.**

During during the three and nine months ended September 30, 2023, we recognized amortization of March 31, 2024.

The amount recorded as intangible asset is being amortized on a straight-line basis over the estimated useful life of \$0.3 million 14 years, and \$0.8 million, respectively, and royalty expense of \$0.4 million and \$1.0 million, respectively, the related to the license and transition services agreement as discussed above. Such costs were included amortization is recorded within cost of sales in our condensed statements. Royalties are recognized within cost of operations. No such expenses were recognized during the three and nine months ended September 30, 2022, sales, as revenue from REZLIDHIA product sales is recognized.

5.6. Stock-Based Compensation

Stock-based compensation for the periods presented was as follows (in thousands):

							Three Months Ended March 31,	
	Three Months Ended September 30,		Nine Months Ended September 30,		2024		2023	
	2023	2022	2023	2022	2024	2023	2024	2023
Selling, general and administrative	\$ 1,596	\$ 2,119	\$ 5,127	\$ 6,791	\$ 4,484	\$ 1,735		
Research and development	347	588	1,746	1,514	650	1,023		
Total stock-based compensation expense	\$ 1,943	\$ 2,707	\$ 6,873	\$ 8,305	\$ 5,134	\$ 2,758		

Stock-based compensation expense within research and development in [17](#)

[Table of Contents](#)

During the [nine](#) [three](#) months ended September 30, 2023 include an incremental charge of approximately \$0.5 million from stock option modifications recorded in the first quarter of 2023 related to the acceleration of vesting and extension of exercise period of vested stock option grants made to a former officer whose employment ended in March 2023. Stock-based compensation expense within selling, general and administrative in the nine months ended September 30, 2022 include an incremental charge of approximately \$0.8 million from stock option modifications recorded in the first quarter of 2022 related to the extension of the exercise period of the stock option grants made to our two former Board of Directors whose terms expired in May 2022.

During the nine months ended September 30, 2023 [March 31, 2024](#), we granted stock options to purchase [3,186,000](#) [5,410,890](#) shares of common stock with weighted-average grant-date fair value of [\\$1.34](#) \$0.97 per share, and [24,837](#) [90,544](#) stock options were exercised. The stock options granted during the three months ended March 31, 2024 generally vest over 3 years. As of [September 30, 2023](#) [March 31, 2024](#), there were [34,283,826](#) [37,135,396](#) stock options outstanding, of which, [2,870,000](#) [1,322,500](#) are outstanding performance-based stock options wherein the achievement of the corresponding corporate-based milestones were [assessed](#) not [considered](#) probable as of [September 30, 2023](#) [March 31, 2024](#). Accordingly, none of the [\\$5.3 million](#) \$2.5 million grant date fair value for these awards has been recognized as stock-based compensation expense as of [September 30, 2023](#) [March 31, 2024](#).

The fair value of each option award is estimated on the date of grant using the Black-Scholes option pricing model. The following table summarizes the weighted-average assumptions relating to options granted pursuant to our Equity Incentive Plans ([2018](#) our 2018 Equity Incentive Plan and Inducement Plan) [Plan, as amended](#)) for the periods presented:

	Three Months Ended September 30,		Nine Months Ended September 30,		Three Months Ended March 31,	
	2023	2022	2023	2022	2024	2023
Risk-free interest rate	4.3 %	3.0 %	3.8 %	1.9 %	4.1 %	3.7 %
Expected term (in years)	6.0	6.1	6.9	6.5	6.2	7.0
Dividend yield	0.0 %	0.0 %	0.0 %	0.0 %	0.0 %	0.0 %
Expected volatility	86.7 %	80.9 %	83.4 %	70.5 %	87.4 %	82.8 %

During the [nine](#) [three](#) months ended [September 30, 2023](#) [March 31, 2024](#), we granted [1,387,600](#) [2,763,979](#) RSUs with a grant-date weighted-average fair value of [\\$1.80](#) \$1.27 per share, and [435,006](#) [489,992](#) RSUs were released. The RSUs granted during the three months ended March 31, 2024 generally vest over [4](#) [3](#) years. As of [September 30, 2023](#) [March 31, 2024](#), there were [1,937,064](#) [4,071,854](#) RSUs outstanding.

As of [September 30, 2023](#) [March 31, 2024](#), there was approximately [\\$12.6 million](#) \$16.2 million of unrecognized stock-based compensation cost which is expected to be recognized over a remaining weighted-average period of [2.45](#) [2.49](#) years, related to time-based

stock options, performance-based stock options wherein achievement of the corresponding corporate-based milestones was considered as probable, and RSUs.

[Table of Contents](#)

In April 2023 and July 2023, March 2024, our Board of Directors approved additional 356,000 375,000 shares of common stock reserved reseved for issuance under our Inducement Plan. In May 2023, our stockholders approved an amendment to our 2018 Plan, to, among other items, add an additional 4,000,000 shares to the number of shares of common stock authorized for issuance under our 2018 Plan. As of September 30, 2023 March 31, 2024, there were 13,145,511 7,129,161 shares of common stock available for future grant under our Equity Incentive Plans.

Employee Stock Purchase Plan

Our Purchase Plan provides for a 24-month offering period comprises four six-month purchase periods with a look-back option. A look-back option is a provision in our Purchase Plan under which eligible employees can purchase shares of our common stock at a price per share equal to the lesser of 85% of the fair market value on the first day of the offering period or 85% of the fair market value on the purchase date. Our Purchase Plan also includes a feature that provides for a new offering period to begin when the fair market value of our common stock on any purchase date during an offering period falls below the fair market value of our common stock on the first day of such offering period. This feature is called a "reset." Participants are automatically enrolled in the new offering period.

Our previous 24-month offering period under our Purchase Plan ended on June 30, 2022, and a new 24-month offering period started on July 1, 2022. The fair value of awards under our Purchase Plan is estimated on the date of our new offering period using the Black-Scholes option pricing model, which is being amortized over the requisite service periods. As of September 30, 2023 March 31, 2024, unrecognized stock-based compensation cost related to our Purchase Plan amounted to \$0.3 million \$0.1 million, which is expected to be recognized over the remaining weighted average period of 0.49 0.24 years.

During the nine three months ended September 30, 2023 March 31, 2024, there were 509,190 no shares purchased under the Purchase Plan. As of September 30, 2023 March 31, 2024, there were 2,928,443 2,495,835 shares reserved for future issuance under the Purchase Plan.

6. Inventories

Inventories for the periods presented consist of the following (in thousands):

	As of	
	September 30, 2023	December 31, 2022
Raw materials	\$ 5,701	\$ 4,555
Work in process	3,587	2,659
Finished goods	1,225	1,904
Total	<u>\$ 10,513</u>	<u>\$ 9,118</u>
Reported as:		
Inventories	\$ 6,112	\$ 9,118
Other assets	4,401	—
Total	<u>\$ 10,513</u>	<u>\$ 9,118</u>

Inventories as of September 30, 2023 and December 31, 2022 include inventories acquired from Forma (now Novo Nordisk) pursuant to the license and transition agreement. As of September 30, 2023 and December 31, 2022, zero and \$0.8 million, respectively, of advance payments to the manufacturer of our raw materials were included within prepaid and other current assets in the condensed balance sheet.

We provide reserves for potential excess, dated or obsolete inventories based upon assumptions about future demand and market conditions, as well as product shelf life. There were no material inventory reserves as of September 30, 2023 and December 31, 2022. Inventories that are not expected to be consumed beyond our normal operating cycle are classified as non-current inventories and included within other assets in the condensed balance sheet. Non-current inventories primarily consist of active pharmaceutical ingredient classified as raw materials, which have multi-year shelf life.

[Table of Contents](#)

7. Other Balance Sheet Components

Inventories

Inventories for the periods presented consist of the following (in thousands):

	As of	
	March 31, 2024	December 31, 2023
Raw materials	\$ 3,918	\$ 4,609
Work in process	5,120	1,876
Finished goods	1,275	1,508
Total	\$ 10,313	\$ 7,993
Reported as:		
Inventories	\$ 6,579	\$ 5,522
Other assets	3,734	2,471
Total	\$ 10,313	\$ 7,993

Inventories as of March 31, 2024 and December 31, 2023 include inventories acquired from Forma pursuant to the license and transition services agreement. Inventories as of March 31, 2024 also include inventories acquired from Blueprint pursuant to a Material Transfer Agreement as discussed in Note 5 – In-licensing and Acquisition. As of March 31, 2024, advance payments to the manufacturer of our raw materials were included within prepaid and other current assets in the condensed balance sheet amounted to \$0.7 million. No such advance payment was included within prepaid and other current assets as of December 31, 2023.

Non-current inventories consist of active pharmaceutical ingredient classified as raw materials which have multi-year shelf life, as well as certain work in process and finished goods inventories that are not expected to be consumed beyond our normal operating cycle.

Intangible assets

Intangible assets consist of the following (in thousands):

	As of	
	March 31, 2024	December 31, 2023
Intangible asset cost	\$ 30,360	\$ 15,000
Accumulated amortization	(1,497)	(1,122)

Intangible asset, net	\$ 28,863	\$ 13,878
-----------------------	-----------	-----------

See "Note 5 – In-licensing and Acquisition" for related discussions of capitalized intangible assets. For the three months ended March 31, 2024 and 2023, amortization expense recorded within cost of sales in the statements of operations were \$0.4 million and \$0.3 million, respectively.

The following table presents the estimated future amortization expense of intangible assets as of March 31, 2024 (in thousands):

Remainder of 2024	\$ 1,764
2025	2,351
2026	2,351
2027	2,351
2028	2,351
Thereafter	17,695
	<u>\$ 28,863</u>

19

[Table of Contents](#)

7.8.Cash, Cash Equivalents and Short-Term Investments

Cash, cash equivalents and short-term investments for the periods presented consist of the following (in thousands):

	As of		As of	
	September 30, 2023	December 31, 2022	March 31, 2024	December 31, 2023
Cash	\$ 9,133	\$ 6,264	\$ 5,843	\$ 8,247
Money market funds	15,165	4,155	9,767	9,685
US treasury bills	12,955	5,225	12,906	12,594
Government-sponsored enterprise securities	14,758	15,796	5,469	11,233
Corporate bonds and commercial paper	10,340	26,766	15,565	15,174
	<u>\$ 62,351</u>	<u>\$ 58,206</u>	<u>\$ 49,550</u>	<u>\$ 56,933</u>
Reported as:				
Cash and cash equivalents	\$ 45,414	\$ 24,459	\$ 25,574	\$ 32,786
Short-term investments	16,937	33,747	23,976	24,147
	<u>\$ 62,351</u>	<u>\$ 58,206</u>	<u>\$ 49,550</u>	<u>\$ 56,933</u>

Cash equivalents and short-term investments include the following securities with gross unrealized gains and losses (in thousands):

	Gross				Gross			
	Amortized	Unrealized	Unrealized	Fair Value	Amortized	Unrealized	Unrealized	Fair Value
As of September 30, 2023	Cost	Gains	Losses	Fair Value	Cost	Gains	Losses	Fair Value
As of March 31, 2024								
US treasury bills	\$ 12,955	\$ 1	\$ (1)	\$ 12,955	\$ 12,907	\$ —	\$ (1)	\$ 12,906
Government-sponsored enterprise securities	14,768	1	(11)	14,758	5,468	2	(1)	5,469
Corporate bonds and commercial paper	10,345	1	(6)	10,340	15,570	1	(6)	15,565
Total	<u>\$ 38,068</u>	<u>\$ 3</u>	<u>\$ (18)</u>	<u>\$ 38,053</u>	<u>\$ 33,945</u>	<u>\$ 3</u>	<u>\$ (8)</u>	<u>\$ 33,940</u>

	Gross				Gross			
	Amortized	Unrealized	Unrealized		Amortized	Unrealized	Unrealized	
As of December 31, 2022	Cost	Gains	Losses	Fair Value	Cost	Gains	Losses	Fair Value
As of December 31, 2023								
US treasury bills	\$ 5,251	\$ —	\$ (26)	\$ 5,225	\$12,591	\$ 3	\$ —	\$12,594
Government-sponsored enterprise securities	15,882	1	(87)	15,796	11,230	7	(4)	11,233
Corporate bonds and commercial paper	26,807	—	(41)	26,766	15,172	5	(3)	15,174
Total	\$ 47,940	\$ 1	\$ (154)	\$ 47,787	\$38,993	\$ 15	\$ (7)	\$39,001

As of **September 30, 2023** **March 31, 2024** and **December 31, 2022** **December 31, 2023**, our cash equivalents and short-term investments had a weighted-average time to maturity of approximately **82** **58** days and **89** **82** days, respectively. Our short-term investments are classified as available-for-sale securities. Accordingly, we have classified **certain** **these** securities as short-term investments on our condensed balance sheets as they are available for use in the current operations. As of **September 30, 2023** **March 31, 2024**, we had no investments that had been in a continuous unrealized loss position for more than 12 months. As of **September 30, 2023** **March 31, 2024**, a total of **19** **31** individual securities had been in an unrealized loss position for 12 months or less, and the losses were determined to be temporary. No significant facts or circumstances have arisen to indicate that there has been any significant deterioration in the creditworthiness of the issuers of the securities held by us. Based on our review of these securities, including the assessment of the duration and severity of the unrealized losses, we have not recognized any credit losses on these securities as of **September 30, 2023** **March 31, 2024** and **December 31, 2022** **December 31, 2023**.

20

[Table of Contents](#)

The following table shows the fair value and gross unrealized losses of our investments in individual securities that are in an unrealized loss position, aggregated by investment category (in thousands):

	Unrealized			
	Fair Value	Losses	Unrealized	
As of September 30, 2023	Fair Value	Losses	Fair Value	Losses
As of March 31, 2024				
US treasury bills	\$ 1,695	\$ (1)	\$11,919	\$ (1)
Government-sponsored enterprise securities	7,141	(11)	3,986	(1)
Corporate bonds and commercial paper	10,340	(6)	12,085	(6)
Total	\$ 19,176	\$ (18)	\$27,990	\$ (8)

19

[Table of Contents](#)

8.9. Fair Value

The table below summarizes the fair value of our cash equivalents and short-term investments measured at fair value on a recurring basis, and are categorized based upon the lowest level of significant input to the valuations (in thousands):

	Assets at Fair Value as of September 30, 2023				Assets at Fair Value as of March 31, 2024			
	Level 1	Level 2	Level 3	Total	Level 1	Level 2	Level 3	Total
Money market funds	\$ 15,165	\$ —	\$ —	\$ 15,165	\$ 9,767	\$ —	\$ —	\$ 9,767
US treasury bills	—	12,955	—	12,955	—	12,906	—	12,906
Government-sponsored enterprise securities	—	14,758	—	14,758	—	5,469	—	5,469
Corporate bonds and commercial paper	—	10,340	—	10,340	—	15,565	—	15,565
Total	\$ 15,165	\$ 38,053	\$ —	\$ 53,218	\$ 9,767	\$ 33,940	\$ —	\$ 43,707

	Assets at Fair Value as of December 31, 2022				Assets at Fair Value as of December 31, 2023			
	Level 1	Level 2	Level 3	Total	Level 1	Level 2	Level 3	Total
Money market funds	\$ 4,155	\$ —	\$ —	\$ 4,155	\$ 9,685	\$ —	\$ —	\$ 9,685
US treasury bills	—	5,225	—	5,225	—	12,594	—	12,594
Government-sponsored enterprise securities	—	15,796	—	15,796	—	11,233	—	11,233
Corporate bonds and commercial paper	—	26,766	—	26,766	—	15,174	—	15,174
Total	\$ 4,155	\$ 47,787	\$ —	\$ 51,942	\$ 9,685	\$ 39,001	\$ —	\$ 48,686

9.10. Debt

We have a The following table summarizes loans payable, net (in thousands):

	As of	
	March 31, 2024	December 31, 2023
Principal outstanding	\$ 60,000	\$ 60,000
Unamortized debt issuance costs	(310)	(398)
Principal outstanding, net of unamortized debt issuance costs	<u>\$ 59,690</u>	<u>\$ 59,602</u>
Reported as:		
Loans payable, net, current portion	\$ 14,780	\$ 7,229
Long-term portion of loans payable, net	44,910	52,373
	<u>\$ 59,690</u>	<u>\$ 59,602</u>

The outstanding loans payable as of the periods presented was related to our Credit and Security Agreement (Credit Agreement) with MidCap Financial Trust (MidCap) entered on September 27, 2019 (Closing Date) and amended on March 29, 2021 (First Amendment), February 11, 2022 (Second Amendment) and July 27, 2022 (Third Amendment). On April 11, 2024, we entered into Fourth Amendment to the Credit Agreement. See "Note 12 – Subsequent Events" for further discussions.

[Table of Contents](#)

The Credit Agreement provides for a \$60.0 million term loan credit facility. At the Closing Date, \$10.0 million was funded (Tranche 1), in May 2020, an additional \$10.0 million was funded (Tranche 2), at the Second Amendment, an additional \$10.0 million was funded (Tranche 3), at the Third Amendment, an additional \$10.0 million was funded (Tranche 4), and in March 2023, an additional \$20.0 million was funded (Tranche 5). As of **September 30, 2023** **March 31, 2024**, the outstanding principal balance of the loan was \$60.0 million, and no remaining funds are available for draw under the term loan credit facility.

The First Amendment to the Credit Agreement extended the period through which Tranche 3 was available to us. The Second Amendment to the Credit Agreement, among other things, amended the applicable funding conditions, applicable commitments and certain other terms relating to available credit facilities (Tranches 3 and 4), added additional term loan credit facility (Tranche 5), and revised certain terms related to the financial covenants.

Following the Third Amendment but prior to the **maturity date for Fourth Amendment to the Credit Agreement in April 2024** as discussed in "Note 12 – Subsequent Events", the term loans **is** **would mature** on September 1, 2026, and the interest-only period **is** **was** through October 1, 2024. The **interest rate applicable to the term loans under the amended Credit Agreement is** **bore interest equal to the sum of one-month Secured Overnight Financing Rate (SOFR)**, plus an adjustment of 0.11448%, subject to 1.50% applicable floor, plus applicable margin of 5.65%. **A**, and a final payment fee of 2.5% of principal **is** **due at maturity date of the term loans**. Prior to the Third Amendment, the **outstanding principal balance of the loan bore interest at an annual rate of one-month London Interbank Offered Rate (LIBOR)**, or a **comparable applicable index rate**, plus applicable margin of 5.65%, subject to a **LIBOR floor of 1.50%** and **is payable monthly in arrears**. **date**.

We may make voluntary prepayments, in whole or in part, subject to certain prepayment premiums and additional interest payments. The Credit Agreement also contains certain provisions, such as event of default and change in control provisions, which, if triggered, would require us to make mandatory prepayments on the term loan, which are subject to certain prepayment premiums and additional interest payments. The obligations under the amended Credit Agreement are secured by a perfected security interest in all of our assets including our intellectual property.

Debt issuance costs are recorded as a direct deduction from the outstanding principal balance of the term loan. As of September 30, 2023 and December 31, 2022, the unamortized issuance costs and debt discounts amounted to \$0.3 million and \$0.6 million, respectively.

20

[Table of Contents](#)

Interest expense, including amortization of the debt discount and accretion of the final fees related to the Credit Agreement for the three months ended **September 30, 2023** **March 31, 2024** and **2022** **2023** was \$1.9 million and \$0.8 million, respectively, and for the nine months ended **September 30, 2023** and **2022** was \$5.0 million and \$1.9 million \$1.2 million, respectively. Accrued interest of **\$1.4 million** \$1.6 million was included within other accrued liabilities in the condensed balance sheet as of **September 30, 2023** **March 31, 2024**.

The following table presents the future minimum principal payments of the outstanding loan as of **September 30, 2023** **March 31, 2024** (in thousands):

Remainder of 2023	\$	—
2024		7,500
Remainder of 2024		\$ 7,500
2025		30,000 30,000
2026		22,500 22,500
Principal amount (Tranches 1, 2, 3 and 4)	\$	60,000 \$60,000

The amended Credit Agreement contains certain covenants which, among others, require us to deliver financial reports at designated times of the year and maintain minimum unrestricted cash and trailing net revenues. As of **September 30, 2023** **March 31, 2024**, we were not in violation of any covenants.

10.11. Leases

We have a sublease agreement with Atara Biotherapeutics, Inc. (Atara) entered in October 2022 to sublease an office space located in South San Francisco, California. Subject to the terms of the sublease agreement, the lease term commenced in November 2022 and shall expire in May 2025. This leased facility is currently held as our new **headquarters** Headquarters following the expiration of our previously leased facility in January 2023. The weighted average remaining term of our leases as of **September 30, 2023** **March 31, 2024** was **1.67** **1.17** years.

We previously leased our prior headquarter space located in South San Francisco, California with Healthpeak Properties, Inc. (formerly known as HCP BTC, LLC), and had a sublease agreement with an unrelated third-party to sublet a portion of the leased facility. Both leases expired in January 2023.

The components of our operating lease expense were as follows (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2023	2022	2023	2022
Fixed operating lease expense	\$ 166	\$ 1,340	\$ 944	\$ 4,020
Variable operating lease expense	75	211	105	602
Total operating lease expense	\$ 241	\$ 1,551	\$ 1,049	\$ 4,622

Supplemental information related to our operating lease were as follow (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2023	2022	2023	2022
Cash payments included in the measurement of operating lease liabilities	\$ 178	\$ 2,630	\$ 1,352	\$ 7,856

Supplemental information related to our operating sublease was as follow (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2023	2022	2023	2022
Fixed sublease expense	\$ —	\$ 1,095	\$ 365	\$ 3,285
Variable sublease expense	—	232	77	682
Sublease income	—	(1,327)	(442)	(3,967)
Net	\$ —	\$ —	\$ —	\$ —

2122

Table of Contents

The components of our operating lease expense were as follows (in thousands):

	Three Months Ended March 31,	
	2024	2023
Fixed operating lease expense	\$ 166	\$ 612
Variable operating lease expense	28	72
Total operating lease expense	\$ 194	\$ 684

Supplemental information related to our operating lease were as follow (in thousands):

	Three Months Ended March 31,	
	2024	2023
Cash payments included in the measurement of operating lease liabilities	\$ 184	\$ 996

Supplemental information related to our operating sublease was as follow (in thousands):

	Three Months Ended March 31,	
	2024	2023
Fixed sublease expense	\$ —	\$ 365
Variable sublease expense	—	77
Sublease income	—	(442)
Net	\$ —	\$ —

The following table presents the future lease payments as of **September 30, 2023** **March 31, 2024** (in thousands):

Remainder of 2023	\$ 182	
2024	739	
Remainder of 2024	\$ 555	
2025	301	301
Total minimum payments required	\$ 1,222	\$ 856

12. Subsequent Events

Fourth Amendment to the Credit Agreement with MidCap

On April 11, 2024, we entered into Fourth Amendment to the Credit Agreement with MidCap, pursuant to which the parties agreed to, among other things, (i) extend the maturity date for the term loans to September 1, 2027, (ii) extend the interest only period for the term loans to October 1, 2025, (iii) revise the interest rate payable on the term loans to SOFR plus an adjustment of 0.11448%, subject to 4.00% applicable floor, plus applicable margin of 6.50%, (iv) reset the prepayment fee applicable to the term loans, (v) increase the exit fee payable on the term loans to 4.25%, and (vi) update certain financial covenants in connection with the new maturity date.

23

[Table of Contents](#)

Item 2. Management's Discussion and Analysis of Financial Condition and Results of Operations

This discussion and analysis should be read in conjunction with our financial statements and the accompanying notes included in this report and the audited financial statements and accompanying notes included in our Annual Report on Form 10-K for the year ended December 31, 2022 December 31, 2023 filed with the SEC on March 7, 2023 March 5, 2024. Our financial results for the three and nine months ended September 30, 2023 March 31, 2024 are not necessarily indicative of results that may occur in future interim periods or for the full fiscal year.

This Quarterly Report on Form 10-Q contains statements indicating expectations about future performance and other forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended (Securities Act) and Section 21E of the Securities

Exchange Act of 1934, as amended (Exchange Act), and the Private Securities Litigation Reform Act of 1995, that involve risks and uncertainties. We usually use words such as "may," "will," "would," "should," "could," "expect," "plan," "anticipate," "might," "believe," "estimate," "predict," "intend," or the negative of these terms or similar expressions to identify these forward-looking statements. These statements appear throughout this Quarterly Report on Form 10-Q and are statements regarding our current expectations, beliefs or intent, primarily with respect to our operations and related industry developments. Examples of these statements include, but are not limited to: our expectations regarding the impact of the global pandemic; our business and scientific strategies; risks and uncertainties associated with the commercialization, distribution and marketing of our products in the US and outside the US; risks that the FDA, EMA, the Medicines and Healthcare Products Regulatory Agency (MHRA) or other regulatory authorities may make adverse decisions regarding our products; the progress of our and our collaborators' product development programs, including clinical testing, and the timing of results thereof; our corporate collaborations and revenues that may be received from our collaborations and the timing of those potential payments; our expectations with respect to timing of recognizing product sales; our expectations with respect to regulatory submissions and approvals; our drug discovery technologies; our research and development expense; protection of our intellectual property and our intention to vigorously enforce our intellectual property rights; the availability and sufficiency of our cash and capital resources and the need for additional capital; our ability to successfully identify and acquire or in-license products or companies, and to successfully transition assets to operate acquisitions; our operations and legal risks; the effectiveness of our cybersecurity risk management process; and our acquisition of certain assets comprising rights to GAVRETO (pralsetinib) in the US. You should not place undue reliance on these forward-looking statements. Our actual results could differ materially from those anticipated in these forward-looking statements for many reasons, including as a result of the risks and uncertainties discussed under the heading "Risk Factors" in Item 1A of Part II of this Quarterly Report on Form 10-Q. Any forward-looking statement speaks only as of the date on which it is made, and we undertake no obligation to update any forward-looking statement to reflect events or circumstances after the date on which the statement is made or to reflect the occurrence of unanticipated events, except as required by applicable law. New factors emerge from time to time, and it is not possible for us to predict which factors will arise. In addition, we cannot assess the impact of each factor on our business or the extent to which any factor, or combination of factors, may cause actual results to differ materially from those contained in any forward-looking statements.

2224

[Table of Contents](#)

Overview

We are a biotechnology company dedicated to discovering, developing and providing novel therapies that significantly improve the lives of patients with hematologic disorders and cancer. We focus on products that address signaling pathways that are critical to disease mechanisms.

Our first product approved by the FDA is TAVALISSE (fostamatinib disodium hexahydrate) tablets, the only approved oral SYK inhibitor for the treatment of adult patients with chronic ITP who have had an insufficient response to a previous treatment. The product is also commercially available in Europe and the UK (as TAVLESSE), and in Canada, Israel and Japan (as TAVALISSE) for the treatment of chronic ITP in adult patients.

Our second FDA approved product is REZLIDHIA (olutasidenib) capsules for the treatment of adult patients with R/R AML with a susceptible IDH1 mutation as detected by an FDA-approved test. We began our commercialization of REZLIDHIA in December 2022. We in-licensed olutasidenib from Forma, (now Novo Nordisk), with exclusive, worldwide rights for its development, manufacturing and commercialization.

In February 2024, we entered into an Asset Purchase Agreement with Blueprint to purchase certain assets comprising the right to research, develop, manufacture and commercialize GAVRETO (pralsetinib) in the US. GAVRETO (pralsetinib) is a once daily, small molecule, oral, kinase inhibitor of wild-type RET and oncogenic RET fusions. GAVRETO is approved by the FDA for the treatment of adult patients with metastatic RET fusion-positive NSCLC as detected by an FDA-approved test. GAVRETO is also approved under accelerated approval based on overall response rate and duration response rate, for the treatment of adult and pediatric patients 12 years of age and older with advanced or metastatic RET fusion-positive thyroid cancer who require systemic therapy and who are radioactive iodine-refractory (if radioactive iodine is

appropriate). We intend to distribute and market GAVRETO for approved indications in RET fusion-positive NSCLC and advanced thyroid cancers, and we expect to complete the transition of the asset and start recognizing product sales in July of 2024.

We continue to advance the development of our **IRAK 1/IRAK1/4** inhibitor program, in an open-label, Phase 1b trial to determine the tolerability and preliminary efficacy of the drug in patients with lower-risk MDS who are refractory or resistant to prior therapies.

We have **strategic development collaborations** with MDACC to expand our evaluation of REZLIDHIA (olutasidenib) in AML and other hematologic cancers, and with CONNECT to conduct a Phase 2 clinical trial to evaluate REZLIDHIA (olutasidenib) in combination with temozolomide in patients with HGG harboring an IDH1 mutation.

We have a **RIPK1** inhibitor program in clinical development with our partner Lilly. We also have product candidates in clinical development with partners BerGenBio and Daiichi.

Business Updates

TAVALISSE IN ITP

For the **nine** **three** months ended **September 30, 2023** **March 31, 2024**, net product sales of TAVALISSE were **\$68.1 million** **\$21.1 million**, **a 26% increase** **decreased by \$1.2 million or 5%** compared to **\$22.3 million** net product sales in the same period in **2022**. The **increase** **decrease** was driven partly by a decrease in our TAVALISSE net product sales was primarily driven by number of bottles remaining in distributors channels, and partly due to the increase in quantities sold as a result of increased number of patients under therapy, revenue reserves driven by higher government and private payor rebates. These decreases were partially offset by the increase in price per bottle partly offset by the increase in revenue reserves mainly due to higher government rebates. Our of TAVALISSE. Typically, our first quarter net sales are typically impacted by the first quarter reimbursement issues such as the resetting of co-pays and the Medicare donut hole.

REZLIDHIA in R/R AML with mIDH1

For the **nine** **three** months ended **September 30, 2023** **March 31, 2024**, we recognized **\$6.7 million** of net product sales of REZLIDHIA. We began REZLIDHIA were **\$4.9 million**, increased by **\$3.4 million** compared to **\$1.5 million** net product sales in the **commercialization** same period in 2023. The increase was primarily due to increased quantities sold primarily driven by increased number of REZLIDHIA patients under therapy, partially offset by the increase in December 2022 following the FDA approval. On December 1, 2022, the FDA approved REZLIDHIA capsules for the treatment of adult patients with R/R AML with a susceptible IDH1 mutation as detected by an FDA-approved test. Our commercial efforts focus on targeting healthcare professionals (HCPs) who manage patients with R/R AML. For further discussions and other updates including recent publications on REZLIDHIA, refer revenue reserves primarily due to "Commercial Products – REZLIDHIA in R/R AML with mIDH1" section below higher government rebates.

[Table of Contents](#)

We in-licensed olutasidenib from Forma, (now Novo Nordisk), with exclusive, worldwide rights for development, manufacturing and commercialization of olutasidenib for any uses, including for the treatment of AML and other malignancies. In accordance with the terms of the license and transition services agreement, we paid an upfront fee of \$2.0 million, with the potential to pay up to \$67.5 million additional payments upon achievement of specified development and regulatory milestones and up to \$165.5 million additional payments upon achievement of certain commercial milestones. In addition, subject to the terms and conditions of the license and transition services agreement, Forma (now Novo Nordisk) would be entitled to tiered royalty payments on net sales of licensed products at percentages ranging from low-teens to mid-thirties, as well as certain portions of our sublicensing revenue, subject to certain standard reductions and offsets. During the year ended December 31, 2022, In 2022, certain milestones were met which entitled Forma (now Novo Nordisk) to receive a \$17.5 million milestone payments. No new milestone was met in 2023 and during the **nine** **three** months ended **September 30, 2023** **March 31, 2024**. For further discussions, see "Note 4 - Sponsored Research

GAVRETO(pralsetinib) in metastatic RET fusion-positive NSCLC and License Agreements and Government Contract" to our "Notes to Condensed Financial Statements" contained in Part I, Item 1 of this Quarterly Report on Form 10-Q. advanced thyroid

On February 22, 2024, we entered into an Asset Purchase Agreement with Blueprint to purchase certain assets comprising the right to research, develop, manufacture and commercialize GAVRETO (pralsetinib) in the US. Under the terms of the agreement, we agreed to pay Blueprint a purchase price of \$15.0 million, \$10.0 million of which is payable upon our first commercial sale of GAVRETO (pralsetinib) and an additional \$5.0 million of which is payable on the first anniversary of the closing date of the agreement, subject to certain conditions. Blueprint is also eligible to receive up to \$97.5 million in future commercial milestone payments and up to \$5.0 million in future regulatory milestone payments, in addition to tiered royalties ranging from 10% to 30%.

Simultaneously and in conjunction with entering into the Asset Purchase Agreement, we also entered into certain supporting agreements, including a customary transition agreement, pursuant to which, during the transition period, Blueprint will transition regulatory and distribution responsibility for GAVRETO (pralsetinib) to us. We also agreed to purchase certain drug product inventories from Blueprint amounting to approximately \$7.0 million under a Material Transfer Agreement. As of March 31, 2024, we received inventories amounting to approximately \$3.1 million, and the remaining inventories are expected to be delivered to us in the second quarter of 2024.

We believe GAVRETO will be highly synergistic with our current product portfolio, and we expect to leverage our existing commercial infrastructure to ensure current and newly prescribed GAVRETO patients have continued access to this important treatment option. We intend to distribute and market GAVRETO for approved indications in RET fusion-positive NSCLC and advanced thyroid cancers, and we expect to complete the transition of the asset and start recognizing product sales in July of 2024.

GAVRETO (pralsetinib) is a once daily, small molecule, oral, kinase inhibitor of wild-type RET and oncogenic RET fusions. Currently, GAVRETO (pralsetinib) is one of only two approved RET inhibitors on the market for patients. GAVRETO is approved by the FDA for the treatment of adult patients with metastatic RET fusion-positive NSCLC as detected by an FDA-approved test. GAVRETO is also approved for the treatment of adult and pediatric patients 12 years of age and older with advanced or metastatic RET fusion-positive thyroid cancer who require systemic therapy and who are radioactive iodine-refractory (if radioactive iodine is appropriate). This indication was approved by the FDA under accelerated approval based on overall response rate and duration of response. Continued approval for this indication may be contingent upon verification and description of clinical benefit in confirmatory trial. Discussions with the FDA regarding confirmatory requirements are ongoing.

GAVRETO has been co-marketed by Blueprint and Genentech, a member of Roche Group (Roche), to patients in the US since September 2020 pursuant to a collaboration agreement between Blueprint and Roche, which was terminated effective in February 2024.

The patent portfolio covering pralsetinib contains patents and patent applications directed to compositions of matter for pralsetinib, including solid forms, formulations, and methods of use and manufacture. Pralsetinib is covered as a composition of matter in a US issued patent that has an expiration date in November 2036 and subject to potential extensions. Patents that have been issued or are expected to be issued covering pralsetinib will have statutory expiration dates between 2036 and 2041. Patent term adjustments, patent term extensions, and supplementary protection certificates could result in later expiration dates. The FDA granted GAVRETO (pralsetinib) new chemical entity exclusivity until September 2025 and orphan drug exclusivity until September 2027 with respect to the approval for treatment of adult

23 26

[Table of Contents](#)

patients with metastatic RET fusion-positive NSCLC as detected by an FDA-approved test. The FDA also granted GAVRETO (pralsetinib) two orphan drug exclusivities until December 2027 with respect to FDA approval for the treatment of adult and pediatric patients 12 years of age and older with advanced or metastatic RET fusion-positive thyroid cancer who require systemic therapy and who are radioactive iodine-refractory (if radioactive iodine is appropriate), and for the treatment of adult and pediatric patients 12 years of age and older with advanced or metastatic RET-mutant medullary thyroid carcinoma who require systemic therapy.

RET is involved in the physiological development of some organ systems. RET is a receptor tyrosine kinase that activates multiple downstream pathways involved in cell proliferation and survival. RET can be activated by mutation or when a portion of the RET gene that encodes the kinase domain is joined to part of another gene creating a fusion gene that encodes an aberrantly activated RET fusion protein.

RET alterations, such as fusions or mutations, drive the growth of multiple tumor types. It is estimated that over 230,000 adult patients in the US will be diagnosed with lung cancer in 2024. NSCLC is the most common type of lung cancer in the US accounting for 80-85% of all lung cancer diagnoses. RET activating fusions are key disease drivers in NSCLC. RET fusions are implicated in approximately 1-2% of patients with NSCLC.

GAVRETO (pralsetinib) faces competition for RET fusion-positive NSCLC and advanced thyroid cancers from Lilly's selpercatinib. In addition, other commercially available therapies used to treat RET fusion-positive NSCLC include cabozantinib and platinum-based chemotherapy regimens with or without pembrolizumab, atezolizumab, nivolumab/ipilimumab, cemiplimab or tremelimumab-durvalumab. Pralsetinib may also face competition from other drug candidates in development for RET-altered cancers, as well as multi-kinase inhibitors with RET activity being evaluated in clinical trials.

R289, an Oral IRAK1/4 Inhibitor for Hematology-Oncology, Autoimmune, and Inflammatory Diseases

We continue to advance advanced the development of our IRAK 1/4 inhibitor program, completing the following further evaluation of a new pro-drug formulation of R835, R289, in single-ascending single and multiple ascending dose studies with positive safety results in 2021. In January 2022, we received clearance from the FDA on our clinical trial design to explore doses of R289 in lower-risk MDS. healthy subjects. The open-label, Phase 1b open-label, multicentric trial will determine evaluates the safety, tolerability and preliminary efficacy of R289 in patients with R/R lower-risk MDS who are refractory or resistant to prior therapies. In December 2022, we announced that we dosed the first patient in our Phase 1b trial of R289. The MDS. This Phase 1b trial of R289 is expected to enroll approximately 22 patients. 40 patients (up to 30 participants in the dose escalation phase, and up to 10 participants in the dose expansion phase). The primary objective of the trial is safety, with secondary and exploratory objectives to assess preliminary efficacy and characterize the pharmacokinetic and pharmacodynamic profile of R289. The safety and efficacy data from this Phase 1b trial along with the safety and pharmacokinetic/pharmacodynamic data from the completed first-in-human study in healthy volunteers, are intended to be used to determine inform the recommended Phase 2 dose for future clinical development of R289 targeting for further clinical evaluation in lower-risk MDS. To date, target enrollment in the second third cohort of the trial has been completed and we are currently enrolling patients in the third cohort. planning to include two additional cohorts with twice daily dosing regimens. Preliminary results data are expected by mid-year the end of 2024.

REZLIDHIA (olutasidenib) in AML, Other Hematologic Cancers and HGG

In December 2023, we entered into a Strategic Collaboration Agreement with MDACC, a comprehensive cancer research, treatment, and prevention center. The collaboration will expand our evaluation of REZLIDHIA (olutasidenib) in AML and other hematologic cancers with IDH1 mutations. Under the Strategic Collaboration Agreement, we will jointly lead the clinical development efforts with MDACC to evaluate the potential of olutasidenib to treat newly diagnosed and R/R patients with AML, higher-risk MDS, and advanced myeloproliferative neoplasms, in combination with other agents. The collaboration will also support the evaluation of olutasidenib as monotherapy in patients with IDH1 mutated clonal cytopenia of undetermined significance and lower-risk MDS, as well as maintenance therapy following hematopoietic stem cell transplant. Under the Strategic Collaboration Agreement, we will provide MDACC the study materials and \$15.0 million in time-based milestone payments as compensation for services to be provided for the studies, over the five-year collaboration term, unless terminated earlier as provided for in the agreement. Through March 31, 2024, we provided \$2.0 million funding to MDACC.

[Table of Contents](#)

In January 2024, we announced our collaboration with Collaborative Network for Neuro-Oncology Clinical Trials (CONNECT), an international collaborative network of pediatric cancer centers, to conduct a Phase 2 clinical trial to evaluate REZLIDHIA (olutasidenib) in combination with temozolamide in patients with HGG harboring an IDH1 mutation. Under the collaboration, CONNECT will include olutasidenib in CONNECT's TarGet-D, a molecularly guided Phase 2 umbrella clinical trial for HGG. Our sponsored arm, adolescents and young adult patients (<39 years old) with newly-diagnosed IDH1-mutation positive HGG will receive maintenance therapy with olutasidenib in combination with temozolamide for the first year after radiotherapy, followed by olutasidenib monotherapy for the second year. Under the collaboration, we will provide CONNECT with a funding up to \$3.0 million and study material over the four-year collaboration.

Global Strategic Partnership with Lilly

Lilly is continuing to advance R552, an investigational, potent and selective RIPK1 inhibitor. Lilly has initiated the Phase 2a trial studying R552 in adult patients with moderately to severely active rheumatoid arthritis. The trial plans to enroll 100 patients globally. RIPK1 is implicated in a broad range of key inflammatory cellular processes and plays a key role in tumor necrosis factor signaling, especially in the induction of pro-inflammatory necroptosis. The program also includes RIPK1 compounds that cross the blood-brain barrier (CNS-penetrants) to address neurodegenerative diseases such as Alzheimer's disease and amyotrophic lateral sclerosis.

Under the Lilly Agreement, we are responsible for 20% of the development costs for R552 in the US, Europe, and Japan, up to a specified cap. Lilly is responsible for funding the remainder of all development activities for R552 and other non-CNS disease development candidates. Under the Lilly Agreement, (prior to an amendment as discussed below), we have the right to opt-out of co-funding the R552 development activities in the US, Europe and Japan at two different specified times and as a result receive lesser royalties from sales. Prior to us providing our first opt-out notice as discussed below, under the Lilly Agreement, we were required to fund our share of the R552 development activities in the US, Europe, and Japan up to a maximum funding commitment of \$65.0 million through April 1, 2024. On September 28, 2023, we entered into an amendment to the Lilly Agreement which provides, among others, other things, that if we exercise our first opt-out right, we have the right to opt-in to co-funding of R552 development, upon us providing notice to Lilly within 30 days of certain events, as specified in the Lilly Agreement. Following the amendment to the Lilly Agreement, in September 2023, we provided the first opt-out notice to Lilly. We continue to fund our share of the R552 development activities up to \$22.6 million through April 1, 2024 as provided for in the amended Lilly Agreement. Through March 31, 2024, Lilly billed us \$20.3 million of the funding development costs incurred as of the fourth quarter of 2023. If we decide to exercise our opt-in right, we will be required to continue to share in global development costs, and if we later exercise our second opt-out right (no later than April 1, 2025), our share in global development costs will be up to a specified cap through December 31, 2025, as provided for in the Lilly Agreement. On September 29, 2023.

Patent Infringement Lawsuit

In June 2022, we provided received a notice letter regarding an Abbreviated New Drug Application (ANDA) submitted to the first opt-out notice FDA by Annora Pharma Private Limited (Annora) requesting approval to Lilly. We will continue to fund our share market a generic version of the R552 development activities up to \$22.6 million through April 1, 2024 as provided for TAVALISSE. In July 2022, we filed a lawsuit in the amended Lilly Agreement. Through September 30, 2023, Lilly billed us \$17.7 million of the funding development costs and the amounts were fully paid as of September 30, 2023.

Fostamatinib in Hospitalized COVID-19 patients

We previously announced in November 2022 the top-line results from the FOCUS Phase 3 clinical trial to evaluate the safety and efficacy of fostamatinib in hospitalized COVID-19 patients without respiratory failure that have certain high-risk prognostic factors did not meet statistical significance in the primary efficacy endpoint of the number of days on oxygen through Day 29. Upon further analysis, we discovered an error by the contract research organization (CRO) in the application of a statistical stratification factor. The contracted programming CRO misinterpreted receipt of prior COVID-19 treatment of interest 14 days before randomization (regardless of continuation post randomization), as those medications taken 14 days before the date of randomization and ended prior to the day of randomization. After correcting for this statistical error, the primary endpoint of the study was met; those who received fostamatinib had lower mean days on oxygen than those who received placebo (4.8 vs. 7.6 days, p=0.0136). Further, fostamatinib showed significance or trend towards significance in all secondary endpoints of reducing mortality and morbidity compared to placebo after correcting US District Court for the error. The results were recently presented at the IDWeek 2023 held District of New Jersey against Annora and its subsidiaries for infringement of certain of our US patents. Litigation continues, and no trial date is currently set. For a more detailed discussion of this litigation matter, see Part II, Item 1, "Legal Proceedings" of this Quarterly Report on October 11-15, 2023 in Boston, Massachusetts. During our continued analysis regarding fostamatinib in hospitalized COVID-19 Form 10-Q.

24 28

Table of Contents

patients, we provided the updated analysis to the FDA and our partner, the US Department of Defense. Given the end of the federal COVID-19 Public Health Emergency (PHE) in May 2023, and based on feedback from the FDA, US Department of Defense and other advisors regarding

the program's regulatory requirements, costs, timeline and potential for success, we decided not to submit an Emergency Use Authorization (EUA) or sNDA.

The Accelerating COVID-19 Therapeutic Inventions and Vaccines Phase 2/3 trial (ACTIV-4 Host Tissue Trial), conducted and sponsored by the National Institute of Health (NIH)/National Heart, Lung, and Blood Institute (NHLBI), is a randomized, placebo-controlled trial of therapies, including fostamatinib, targeting the host response to COVID-19 in hospitalized patients. The ACTIV-4 Host Tissue Trial evaluated fostamatinib in a targeted population of approximately 600 hospitalized patients with COVID-19, 300 fostamatinib versus 300 placebo. During the first quarter of 2023, an interim analysis of the trial was completed by the Data and Safety Monitoring Board (DSMB) with a recommendation for the trial to continue. In September 2023, the DSMB recommended that the fostamatinib study arm of the ACTIV-4 Host Tissue Trial platform cease enrollment. Based on the DSMB's review of a conditional power analysis, the DSMB determined that there was an extremely low likelihood of fostamatinib providing benefits related to the primary outcome (oxygen free days) or other secondary outcomes in patients hospitalized and on oxygen therapy for COVID-19. No safety concerns were identified. The NIH/NHLBI concurs with the DSMBs recommendation and has asked the trial investigators to cease enrollment, complete follow-up for participants already enrolled, and complete study closeout. The full study data will be analyzed and disseminated as previously planned.

Update on Current and Potential Future Impact of COVID-19 on our Business

The COVID-19 pandemic has adversely impacted our business and operations. Although the World Health Organization declared the end of COVID-19 PHE in May 2023, the degree to which another global pandemic may affect our business and operations and financial condition in the future will depend on developments that are highly uncertain and beyond our knowledge or control. As such, we cannot ascertain the full extent of the future impacts it may have on our business. See also "Part I, Item 1A, Risk Factors" of this Quarterly Report on Form 10-Q for additional information on risks and uncertainties related to the COVID-19 pandemic.

Our Product Portfolio

The following table summarizes our portfolio:



Graphic

25

[Table of Contents](#)

Commercial Products

TAVALISSE/Fostamatinib in ITP

Chronic ITP affects an estimated 81,300 adult patients in the US. In patients with ITP, the immune system attacks and destroys the body's own blood platelets, which play an active role in blood clotting and healing. ITP patients can suffer extraordinary bruising, bleeding and fatigue as a result of low platelet counts. Current therapies for ITP include steroids, blood platelet production boosters that imitate thrombopoietin (TPO) and splenectomy.

Taken in tablet form, fostamatinib blocks the activation of SYK inside immune cells. ITP is typically characterized by the body producing antibodies that attach to healthy platelets in the blood stream. Immune cells recognize these antibodies and affix to them, which activates the SYK enzyme inside the immune cell, and triggers the destruction of the antibody and the attached platelet. When SYK is inhibited by fostamatinib, it interrupts this immune cell function and allows the platelets to escape destruction. The results of our Phase 2 clinical trial, in which fostamatinib was orally administered to 16 adults with chronic ITP, published in *Blood*, showed that fostamatinib significantly increased the platelet counts of certain ITP patients, including those who had failed other currently available agents.

Our Fostamatinib for Immune Thrombocytopenia (FIT) Phase 3 clinical program had a total of 150 ITP patients which were randomized into two identical multi-center, double-blind, placebo-controlled clinical trials. The patients were diagnosed with persistent or chronic ITP, and had blood platelet counts consistently below 30,000 per microliter of blood. Two-thirds of the subjects received fostamatinib orally at 100 mg twice daily (bid) and the other third received placebo on the same schedule. Subjects were expected to remain on treatment for up to 24 weeks. At week four of treatment, subjects who failed to meet certain platelet counts and met certain tolerability thresholds could have their dosage of fostamatinib (or corresponding placebo) increased to 150 mg bid. The primary efficacy endpoint of this program was a stable platelet response by week 24 with platelet counts at or above 50,000 per microliter of blood for at least four of the final six qualifying blood draws. In August 2016, we announced the results of the first FIT study, reporting that fostamatinib met the study's primary efficacy endpoint. The study showed that 18% of patients receiving fostamatinib achieved a stable platelet response compared to none receiving a placebo control. In October 2016, we announced the results of the second FIT study, reporting that the response rate (16% in the treatment group, versus 4% in the placebo group) was consistent with the first study, although the difference was not statistically significant. In the ITP double-blind studies, the most commonly reported adverse reactions occurring in at least 5% of patients treated with TAVALISSE were diarrhea, hypertension, nausea, dizziness, increased alanine aminotransferase, increased aspartate

29

[Table of Contents](#)

aminotransferase, respiratory infection, rash, abdominal pain, fatigue, chest pain, and neutropenia. Serious adverse drug reactions occurring in at least 1% of patients treated with TAVALISSE in the ITP double-blind studies were febrile neutropenia, diarrhea, pneumonia, and hypertensive crisis. A post-hoc analysis from our Phase 3 clinical program in adult patients with chronic ITP, highlighting the potential benefit of using TAVALISSE in earlier lines of therapy, was published in the *British Journal of Haematology* in July 2020. In addition, a report describing the long-term safety and durable efficacy of TAVALISSE with up to **5** years of treatment was published in *Therapeutic Advances in Hematology* in 2021.

The FDA granted our request for orphan drug designation for fostamatinib for the treatment of ITP in August 2015. TAVALISSE was approved by the FDA in April 2018 for the treatment of ITP in adult patients who have had an insufficient response to a previous treatment, and successfully launched in the US in May 2018. The FDA granted orphan drug designation for fostamatinib for the treatment of ITP in August 2015.

In January 2020, the EC European Commission (EC) granted a centralized MA for fostamatinib (TAVLESSE) valid throughout the European Union (EU) and in the UK, after the departure of the UK from the EU, for the treatment of chronic ITP in adult patients who are refractory to other treatments. In December 2022, Japan's PMDA Pharmaceuticals and Medical Devices Agency (PMDA) approved the NDA for fostamatinib in chronic ITP.

26

[Table of Contents](#)

Competitive landscape for TAVALISSE

Our industry is intensely competitive and subject to rapid and significant technological change. TAVALISSE is competing with other existing therapies. In addition, a number of companies are pursuing the development of pharmaceuticals that target the same diseases and conditions that we are targeting. For example, there are existing therapies and drug candidates in development for the treatment of ITP that may be alternative therapies to TAVALISSE.

Currently, corticosteroids remain the most common first line therapy for ITP, occasionally in conjunction with intravenous immunoglobulin (IVIg) or anti-Rh(D) to help further augment platelet count recovery, particularly in emergency situations. However, it has been estimated that frontline agents lead to durable remissions in only a small percentage of newly diagnosed adults with ITP. Moreover, concerns with steroid-related side effects often restrict therapy to approximately four weeks. As such, many patients progress to persistent or chronic ITP, requiring other forms of therapeutic intervention. In long-term treatment of chronic ITP, patients are often cycled through several therapies over time in order to maintain a sufficient response to the disease.

Other approaches to treat ITP are varied in their mechanism of action, and there is no consensus about the sequence of their use. Options include splenectomy, thrombopoietin receptor agonists (**TPO-RAs**) (**TPO-Ras**) and various immunosuppressants (such as rituximab). The response rate criteria of the above-mentioned options vary, precluding a comparison of response rates for individual therapies.

Even with the above treatment options, a significant number of patients remain severely thrombocytopenic for long durations and are subject to risk of spontaneous or trauma-induced hemorrhage. The addition of fostamatinib to the currently available treatment options could be beneficial because it has a different mechanism of action than any of the therapies that are currently available. Fostamatinib is a potent and relatively selective SYK inhibitor, and its inhibition of Fc receptors and B-cell receptors of signaling pathways make it a potentially broad immunomodulatory agent.

Other products in the US that are approved by the FDA to increase platelet production through binding to TPO receptors on megakaryocyte precursors include PROMACTA® (Novartis International AG (Novartis)), Nplate® (Amgen, Inc.) and DOPTELET® (Swedish Orphan Biovitrum AB). In the longer term, we may eventually face competition from potential manufacturers of generic versions of our marketed products, including the proposed generic version of TAVALISSE that is the subject of an **Abbreviated New Drug Application (ANDA)** **ANDA** submitted to the FDA by Annora, **Pharma Private Limited (Annora)**, which, if approved and allowed to enter the market, it could result in significant decreases in the revenue derived from sale of TAVALISSE and thereby materially harm our business and financial condition.

Commercial activities, including sales and marketing

Our marketing and sales efforts are focused on hematologists and hematologist-oncologists in the US who manage chronic adult ITP patients. We have a fully integrated commercial team consisting of sales, marketing, market access, and commercial operations functions. Our sales team promotes our products in the US using customary

Table of Contents

pharmaceutical company practices, and we concentrate our efforts on hematologists and hematologist-oncologists. Our products are sold initially through third-party wholesale distribution and specialty pharmacy channels and group purchasing organizations before being ultimately prescribed to patients. To facilitate our commercial activities in the US, we also enter into arrangements with various third parties, including advertising agencies, market research firms and other sales-support-related services as needed. We believe that our commercial team and distribution practices are adequate to ensure that our marketing efforts reach relevant customers and deliver our products to patients in a timely and compliant fashion. Also, to help ensure that all eligible patients in the US have appropriate access to our products, we have established a reimbursement and patient support program called Rigel OneCare (ROC). Through ROC, we provide co-pay assistance to qualified, commercially insured patients to help minimize out-of-pocket costs and provide free product to uninsured or under-insured patients who meet certain established clinical and financial eligibility criteria. In addition, ROC is designed to provide reimbursement support, such as information related to prior authorizations, benefits investigations and appeals.

[Table of Contents](#)

We have entered into various license and commercial agreements to commercialize fostamatinib globally, but we retain the global rights to fostamatinib outside of the respective territories under such license and commercial agreements. Our collaborative partner Grifols has S.A. (Grifols) launched TAVLESSE in the UK and certain countries in Europe including Germany, France, Italy and Spain, and continues a phased rollout across the rest of Europe. Our collaborative partner Medison has also Pharma Trading AG (Medison Canada) and Medison Pharma Ltd. (Medison Israel, and together with Medison Canada, Medison) launched TAVALISSE in Canada and Israel. Further, our collaborative partner Kissei has also recently Pharmaceutical Co., Ltd. (Kissei) launched TAVALISSE in Japan.

Fostamatinib in Europe/Turkey Europe

We have a commercialization license agreement with Grifols entered in January 2019, for an exclusive rights to commercialize fostamatinib for human diseases, including chronic ITP and AIHA, and non-exclusive rights to develop, fostamatinib in their territory. Grifols territory includes Europe, the UK, Turkey, the Middle East, North Africa and Russia (including Commonwealth of Independent States).

We are responsible for performing and funding certain development activities for fostamatinib for ITP and AIHA and Grifols is responsible for all other development activities for fostamatinib in such territories. We remain responsible for the manufacturing and supply of fostamatinib for all development and commercialization activities under the agreement. Under the terms of the agreement, we received an upfront cash payment of \$30.0 million and will be eligible to receive regulatory and commercial milestones of up to \$297.5 million. In January 2020, the EC granted a MA for fostamatinib for the treatment of chronic ITP in adult patients who are refractory to other treatments. With this approval, we received a \$20.0 million non-refundable milestone payment, consisted of a \$17.5 million payment due upon MAA Market Authorization Application (MAA) approval by the EMA of fostamatinib for the first indication and a \$2.5 million creditable advance royalty payment due upon EMA approval of fostamatinib in the first indication. We are also entitled to receive stepped double-digit royalty payments based on tiered net sales which may reach 30% of net sales. sales of fostamatinib in the Grifols territory.

Fostamatinib in Japan/Asia

We have an exclusive license and supply agreement with Kissei entered in October 2018, to develop and commercialize fostamatinib in all current and potential indications in Kissei's territory, which includes Japan, China, Taiwan and the Republic of Korea. Kissei is a Japan-based pharmaceutical company addressing patients' unmet medical needs through its research, development and commercialization efforts, as well as through collaborations with partners.

Under the terms of the agreement, we received an upfront cash payment of \$33.0 million, with the potential for an additional \$147.0 million in development and commercial milestone payments, and will receive product transfer price payments in the mid to upper twenty percent range based on tiered net sales for the exclusive supply of fostamatinib. Kissei receives exclusive rights to fostamatinib in ITP and all future indications in Kissei's territory.

In September 2019, Kissei initiated a Phase 3 trial in Japan of fostamatinib in adult Japanese patients with chronic ITP. The efficacy and safety of orally administered fostamatinib was assessed by comparing it with placebo in a randomized, double-blind study. Japan has the third highest prevalence of chronic ITP in the world behind the US and Europe. In February 2020, Kissei was granted orphan drug designation from the Japanese Ministry of Health, Labor and Welfare for R788 (fostamatinib) in chronic ITP. In December 2021, Kissei reported positive top-line results for a Phase 3 clinical trial, meeting its primary endpoint. The Phase 3 clinical trial showed that patients receiving fostamatinib achieved a stable platelet response significantly higher than patients receiving a placebo control. Based on the positive

[Table of Contents](#)

Phase 3 results, in April 2022, Kissei submitted an NDA to Japan's PMDA for fostamatinib in chronic ITP. With this milestone event, we received \$5.0 million non-refundable and non-creditable payment from Kissei and recognized the amount as revenue in the second quarter of 2022. Kissei. In December 2022, Japan's PMDA approved TAVALISSE for the treatment of chronic ITP. With this milestone event, we were entitled to receive received \$20.0 million non-refundable and non-creditable payment from Kissei based on the terms of our collaboration agreement, and such amount was recognized as revenue in the fourth quarter of 2022. The amount was subsequently collected in January 2023. agreement. In April 2023, Kissei announced the commercial launch of launched TAVALISSE for chronic ITP in Japan.

[Table of Contents](#)*Fostamatinib in Canada/Israel*

We have two exclusive commercial and license agreements with Medison entered in October 2019, to commercialize fostamatinib in all potential indications in Canada and Israel. Under the terms of the agreements, we received an upfront payment of \$5.0 million with the potential for approximately \$35.0 million in regulatory and commercial milestones. In addition, we will receive royalty payments beginning at 30% of net sales. Under our agreement with Medison for the Canada territory, we have the option to buy back all rights to the product upon regulatory approval in Canada for the indication of AIHA. The buyback provision, if exercised, would require both parties to mutually agree on commercially reasonable terms for us to purchase back the rights, taking into account Medison's investment and the value of the rights, among others. Pursuant to this exclusive commercialization license agreement, in August 2020, we entered into a commercial supply agreement with Medison.

In November 2020, Health Canada approved the New Drug Submission for TAVALISSE for the treatment of thrombocytopenia in adult patients with chronic ITP who have had an insufficient response to other treatments. In August 2021, Medison Israel received the licenses for registrational approval from the Ministry of Health, which event entitled us to receive \$0.1 million of non-refundable milestone payment. In November 2022, Medison Israel made its first commercial sale of TAVALISSE and obtained its national reimbursement in February 2023.

Fostamatinib in Latin America

In May 2022, we entered into commercial license agreement with Knight Therapeutics International SA (Knight) for the commercialization of fostamatinib for approved indications in Latin America, consisting of Mexico, Central and South America, and the Caribbean. Pursuant to such commercial license agreement, we received a \$2.0 million one-time, non-refundable, and non-creditable upfront payment, with potential for up to an additional \$20.0 million in regulatory and sales-based commercial milestone payments, and will receive twenty- to mid-thirty percent, tiered, escalated net-sales based royalty payments. We are also responsible for the exclusive manufacture and supply of fostamatinib for all future development and commercialization activities under a Commercial and Supply Agreement. In August 2023, Knight submitted the MAA for regulatory approval in Mexico, Colombia and Colombia Brazil for fostamatinib for the treatment of adult patients with ITP who had insufficient response to a previous treatment.

REZLIDHIA in R/R AML with mIDH1

mIDH1 alterations are seen in AML, MDS, glioma, chondrosarcoma, and intrahepatic cholangiocarcinoma. It is estimated that there are approximately 1,000 adult patients, a well-identified patient population, with mIDH1 R/R AML, part of an AML market estimated to have an incidence of approximately 20,000 cases in the US and an estimated 120,000 cases globally. Despite having approved treatment options for R/R AML patients who are mIDH1 positive, an unmet need remains.

Oltasidenib, an oral, small molecule drug designed to selectively bind to and inhibit mIDH1, is a treatment option with durable remissions, reduced QTc potential, and a stable pharmacokinetics profile that enables a consistent drug exposure over time. This targeted agent has the potential to provide therapeutic benefit by reducing 2-hydroxyglutarate levels and restoring normal cellular differentiation. IDH1 is a natural enzyme that is part of the normal metabolism of all cells. When mutated, IDH1 activity can promote blood malignancies and solid tumors. Oltasidenib was designated by the FDA as an orphan drug for the treatment of AML, which provides orphan drug market exclusivity from the time of marketing approval on December 1, 2022.

[Table of Contents](#)

REZLIDHIA (olutasidenib) is designed to bind to and inhibit mIDH1 to reduce 2-hydroxyglutarate levels and restore normal cellular differentiation of myeloid cells. REZLIDHIA is a novel, non-intensive monotherapy treatment in the R/R AML setting demonstrating a CR+CRh rate of 35% in patients with over 90% of those responders in complete remission. **The safety of REZLIDHIA 150 mg administered twice daily was evaluated in 153 adults with relapsed or refractory AML with an IDH1 mutation.**

On December 1, 2022, the FDA has approved REZLIDHIA capsules for the treatment of adult patients with R/R AML with IDH1 mutation as detected by an **FDA approved** **FDA-approved** test. On December 22, 2022, we began the commercialization of REZLIDHIA and made it available to patients. The recommended dosage of REZLIDHIA is 150 mg taken orally twice daily until disease progression or unacceptable toxicity. The FDA approval was based on the NDA

[Table of Contents](#)

for olutasidenib for the treatment of m1DH1 R/R AML submitted by Forma, **(now Novo Nordisk)**, that had a PDUFA action date for the application of February 15, 2023. The NDA application was supported with a Phase 2 registrational trial for olutasidenib in mIDH1 R/R AML. Interim results from the Phase 2 registrational trial were reported at the American Society of Clinical Oncology (ASCO) annual meeting in June 2021. The interim results of this trial of 153 patients showed that olutasidenib demonstrated a favorable tolerability profile as a monotherapy in patients with R/R AML who have a susceptible mIDH1, and achieved a complete remission (CR) plus CR with partial hematologic recovery (CRh) rate of 33.3% (30% CR and 3% CRh), the primary efficacy endpoint. While a median duration of CR/CRh was not yet reached, a sensitivity analysis (with a hematopoietic stem cell transplant, as the end of a response) indicated the median duration of CR/CRh was 13.8 months. The overall response rate, comprised CR, CRh, **CRI**, **Cri**, partial response, and morphologic leukemia-free state (MLFS), was 46% and the median duration of overall response rate (ORR) was 11.7 months. The median overall survival was 10.5 months. For patients with CR/CRh, the median overall survival was not reached, but the estimated 18-month survival was 87%. The most frequently reported treatment emergent adverse events were nausea, constipation, increased white blood cell count, decreased **RBC** **red blood cell** count, pyrexia, febrile neutropenia, and fatigue.

In November 2022, we announced the presentation of five posters highlighting data from our commercial and clinical hematology-oncology portfolio at the 64th American Society of Hematology (ASH) Annual Meeting and Exposition which was held in December 2022. An updated interim analysis from the Phase 2 registrational trial of olutasidenib in patients with R/R AML demonstrated robust efficacy and safety results. The registrational cohort of the Phase 2 trial enrolled 153 patients with mIDH1 R/R AML who received olutasidenib monotherapy 150 mg twice daily. The efficacy evaluable population was 147 patients who received their first dose at least six months prior to the interim analysis cutoff date of June 18, 2021. The primary endpoint was a CR/CRh defined as less than 5% blasts in the bone marrow, no evidence of disease, and partial recovery of peripheral blood counts (platelets >50,000/microliter and absolute neutrophil count >500/microliter). The results from the updated interim analysis of patients with mIDH1 R/R AML demonstrated a 35% CR+CRh rate with a median duration of 25.9 months. The ORR a secondary end point, was 48%, and was defined as the rate of CR, CRh, CR with incomplete blood count recovery **(CRI)** **(Cri)**, partial remission (which required recovery of neutrophil and platelet counts consistent with a CR), or MLFS. Olutasidenib was effective in a broad range of patients including those with prior high-intensity chemotherapy and/or post-venetoclax. The abstract concluded that the observed activity is clinically meaningful and represents a therapeutic advance in the treatment of this patient population. In this pivotal cohort, olutasidenib was well tolerated with an adverse event profile largely characteristic of symptoms or conditions experienced by patients undergoing treatment for AML or of the underlying disease itself.

In November 2022, we **also** announced the publication of data in *The Lancet Haematology*, which summarizes the Phase 1 results of the Phase 1/2 trial of olutasidenib. The objectives of the first phase of the multi-center, open-label Phase 1/2 trial were to assess the safety, pharmacokinetic and pharmacodynamic profile, and clinical activity of olutasidenib, both as monotherapy and in combination with azacitidine, in patients with treatment-naïve or R/R AML or MDS harboring IDH1 mutations. The published data suggest that olutasidenib, with or without azacitidine, was well-tolerated and was associated with improvements in clinical efficacy endpoints in patients with mIDH1 AML. This trial showed that olutasidenib has the potential to provide an additional treatment option for mIDH1 AML.

In January 2023, we announced that REZLIDHIA has been added by the National Comprehensive Cancer Network (NCCN) to the latest NCCN Clinical Practice Guidelines in Oncology (NCCN Guidelines) for AML. REZLIDHIA is now included as a recommended targeted therapy for adult patients with R/R AML with IDH1 mutation.

In February 2023, we announced peer-reviewed publication data in *Blood Advances*, which summarize clinical results from the Phase 2 registration trial of REZLIDHIA in patients with mIDH1 R/R AML. The published data

33

[Table of Contents](#)

demonstrate that REZLIDHIA induced durable remissions and transfusion independence with a well-characterized safety profile. The observed efficacy is clinically meaningful and represents a therapeutic advance in this poor prognosis patient population with limited treatment options. REZLIDHIA demonstrated both a high rate of response and an extended median duration of complete response of 28.1 months, which is more than a year longer than what is reported with the **Standard** **standard** of Care (SoC). **care**. In June 2023, we announced the second REZLIDHIA publication in *Blood Advances*, a review article examining the preclinical and clinical development, and the positioning of olutasidenib in the mIDH1 AML treatment landscape. The review concluded that the approval of REZLIDHIA is a critical addition to the mIDH1 AML treatment landscape. Further, the available data support the use of REZLIDHIA as **monotherapy** **monotherapy** in R/R AML

30

[Table of Contents](#)

patients who have failed intensive **chemotherapy** **chemotherapy** or venetoclax plus hypomethylating agents (HMA) combination therapy.

In June 2023, we announced presentation of data from an analysis from the Phase 2 study of REZLIDHIA in 17 patients with mIDH1 AML who were previously treated with venetoclax. Data was featured in a poster presentation at the European Hematology Association 2023 Hybrid Congress. The data support **olutasidenib** **REZLIDHIA** induced durable remissions in patients with mIDH1 AML in this poor-prognosis patient population who were **relapsed** or **refractory** R/R to venetoclax-based treatment.

We plan in April 2024, we announced a peer-reviewed publication in *Leukemia & Lymphoma* on data from an analysis of the Phase 2 study evaluating REZLIDHIA in patients with mIDH1 AML who are R/R to pursue strategic actions to further develop olutasidenib for the treatment prior venetoclax-based regimens. The findings from these analyses suggest that REZLIDHIA alone in combination with azacitidine demonstrated potential efficacy in patients with AML following failure of other malignancies and expansion of commercialization, venetoclax combination therapy.

Competitive landscape for REZLIDHIA

There is currently one other product approved in the US for patients with IDH1 mutation. The FDA granted approval to TIBSOVO® (ivosidenib), an oral targeted IDH1 mutation inhibitor, (i) in July 2018, for adult patients with R/R AML with a susceptible IDH1 mutation, (ii) in May 2019, for newly diagnosed AML with a susceptible IDH1 mutation who are at least 75 years old or who have comorbidities that preclude use of intensive induction chemotherapy, (iii) in August 2021, for adult patients with previously treated, locally advanced or metastatic cholangiocarcinoma with an IDH1 mutation as detected by an FDA-approved test, and (iv) in May 2022, in combination with azacitidine (azacitidine for injection) for newly diagnosed AML with a susceptible IDH1 mutation, as detected by an FDA-approved test in adults 75 years or older, or who have comorbidities that preclude use of intensive induction chemotherapy, and (v) in October 2023, for adult patients with R/R MDS with a susceptible IDH1 mutation, as detected by an FDA-approved test. In addition, some clinicians may utilize non-targeted treatments for patients with mIDH1 R/R AML, including use of venetoclax combinations, hypomethylating agents, other chemotherapy regimens, or investigational agents that may be available to them.

Commercial activities, including sales and marketing

We believe REZLIDHIA is highly synergistic with our existing hematology-oncology focused commercial and medical affairs infrastructure. Our commercial efforts will focus effort focuses on targeting growing awareness of REZLIDHIA within key institutions, and among targeted HCPs who manage patients with R/R AML with mIDH1. We plan to enter collaborations with third parties to commercialize REZLIDHIA outside of US.

GAVRETO(pralsetinib) in metastatic RET fusion-positive NSCLC and advanced thyroid cancers

Please refer to related discussions above under "Business Updates", titled "GAVRETO(pralsetinib) in metastatic RET fusion-positive NSCLC and advanced thyroid cancers" in this Item 2, Management's Discussion and Analysis of this Quarterly Report on Form 10-Q.

34

[Table of Contents](#)

Clinical Stage Programs

R289, an Oral IRAK 1/4 Inhibitor for Hematology-Oncology, Autoimmune, and Inflammatory Diseases

During the second quarter of 2018, we selected R835, the active metabolite of R289, a proprietary molecule from our IRAK 1/IRAK1/4 inhibitor program, for human clinical trials. This investigational candidate is an orally administered, potent and selective inhibitor of IRAK1 and IRAK4 that blocks inflammatory cytokine production in response to toll-like receptor (TLR) and the interleukin-1 receptor (IL-1R) family signaling. TLRs and IL-1Rs play a critical role in the innate immune response and dysregulation of these pathways can lead to a variety of inflammatory conditions. R835 prevents cytokine release in response to TLR and IL-1R activation in vitro. R835 is active in multiple rodent models of inflammatory disease including psoriasis, arthritis, lupus, multiple sclerosis and gout. Preclinical studies show that R835 inhibits both the IRAK1 and IRAK4 signaling pathways, which play a key role in inflammation and immune responses to tissue damage. Dual inhibition of IRAK1 and IRAK4 allows for more complete suppression of pro-inflammatory cytokine release than inhibition of either one individually.

In October 2019, we announced results from a Phase 1 randomized, placebo-controlled, double-blind clinical trial of R835 in healthy subjects to assess evaluating the safety, tolerability, pharmacokinetics (PK) and pharmacodynamics. The Phase 1 trial was a randomized, placebo-controlled, double-blind trial pharmacodynamics of R835 in 91 healthy subjects, ages 18 to 55, adult subjects. The Phase 1 trial showed positive that R835 had a favorable safety, tolerability and PK data as well as profile and established proof-of-mechanism by demonstrating the inhibition of inflammatory cytokine production in response to a lipopolysaccharide (LPS) challenge.

31

Table of Contents

We continue to advance the development of our IRAK 1/4 inhibitor program, completing the following further evaluation of single and multiple ascending doses of R289, a new pro-drug formulation of R835 R289, in single-ascending and multiple ascending dose studies with positive safety results in 2021, healthy subjects. In January 2022, we received clearance from the FDA on our clinical to initiate a Phase 1 open-label, multicenter trial design to explore R289 in lower-risk MDS. The open-label, Phase 1b trial will determine evaluate the safety, tolerability and preliminary efficacy of R289 in patients with R/R lower-risk MDS who are refractory or resistant to prior therapies. MDS. In December 2022, we announced that we dosed the dosing of the first patient in our Phase 1b trial of R289. The patient. This Phase 1b trial of R289 is expected to enroll approximately 22 patients. 40 patients (up to 30 participants in the dose escalation phase, and up to 10 participants in the dose expansion phase). The primary objective of the trial is safety, with secondary and exploratory objectives to assess preliminary efficacy and characterize the pharmacokinetic and pharmacodynamic profile of R289. The safety and efficacy data from this Phase 1b trial along with the safety and pharmacokinetic/pharmacodynamic data from the completed first-in-human study in healthy volunteers, are intended to be used to determine inform the recommended Phase 2 dose for future clinical development of R289 targeting for further clinical evaluation in lower-risk MDS. To date, target enrollment in the second third cohort of the trial has been completed and and we are currently enrolling patients in the third cohort. planning to include two additional cohorts with twice daily dosing regimens. Preliminary results data are expected by mid-year the end of 2024.

Fostamatinib in Hospitalized COVID-19 Patients

COVID-19 is the infectious disease caused by Severe Acute Respiratory Syndrome Coronavirus-2 (SARS-CoV-2). SARS-CoV-2 primarily infects the upper and lower respiratory tract and can lead to acute respiratory distress syndrome (ARDS). Additionally, some patients develop other organ dysfunction including myocardial injury, acute kidney injury, shock resulting in endothelial dysfunction and subsequently micro and macrovascular thrombosis. Much of the underlying pathology of SARS-CoV-2 is thought to be secondary to We had a hyperinflammatory immune response associated with increased risk of thrombosis. SYK is involved in the intracellular signaling pathways of many different immune cells. Therefore, SYK inhibition may improve outcomes in patients with COVID-19 via inhibition of key Fc gamma receptor and c-type lectin receptor mediated drivers of pathology, such as inflammatory cytokine release by monocytes and macrophages, production of NETs by neutrophils, and platelet aggregation. Furthermore, SYK inhibition in neutrophils and platelets may lead to decreased thromboinflammation, alleviating organ dysfunction in critically ill patients with COVID-19.

Rigel-led Phase 3 Trial. In November 2020, we launched our FOCUS Phase 3 clinical trial to evaluate the safety and efficacy of fostamatinib in hospitalized COVID-19 patients without respiratory failure that have certain high-risk prognostic factors. In January 2021, we were awarded \$16.5 million from the US Department of Defense's The DOD's Joint Program Executive Office for Chemical, Biological, Radiological and Nuclear Defense (JPEO-CBRND) awarded us grants to support this Phase 3 clinical trial. This multi-center, double-blind, placebo-controlled, adaptive design study randomly assigns either fostamatinib plus SoC or matched placebo plus SoC (1:1) to targeted evaluable patients. Treatment is administered orally twice daily for 14 days with follow up to day 60. In December 2021, we expanded the inclusion criteria to include patients with more severe disease (NIAID Ordinal Scale 6) to more accurately reflect the clinically predominant patient population hospitalized with COVID-19 and help speed enrollment. In collaboration with the FDA and Department of Defense, we also updated the primary endpoint for the The trial from progression to severe disease within 29 days, to the number of days on oxygen through day 29. This endpoint allows for closer comparison of the results with earlier results from the NIH/NHLBI Phase 2 clinical trial with fostamatinib and various other NIH-sponsored trials, such as the ACTIV-4 Host Tissue Trial, which uses a similar outcome measure as a primary endpoint. In July 2022, we completed enrollment with 280 patients. The trial had originally targeted a total of 308 patients; however, we determined the trial would be sufficiently powered with 280 patients to potentially provide a clinically meaningful result and determine the efficacy and safety of fostamatinib in hospitalized COVID-19 patients. We previously announced in November 2022 the top-line results from the FOCUS Phase 3 clinical trial to evaluate the safety and efficacy of fostamatinib in hospitalized COVID-19 patients without respiratory failure that have certain high-risk prognostic factors did not meet statistical significance in the primary efficacy endpoint, of which is the number of days the patient on oxygen through Day 29. Upon further analysis, we discovered an error by the biostatistical CRO in the application of a statistical stratification factor. The contracted programming biostatistical CRO misinterpreted receipt of prior COVID-19 treatment of interest 14 days before randomization (regardless of continuation post randomization), as those medications taken 14 days before the date of randomization and ended prior to the day of randomization. After correcting for this statistical error, the primary endpoint of the study was met; those who received fostamatinib had lower mean days on oxygen than those who received placebo (4.8 vs. 7.6 days, p=0.0136). placebo. Further, fostamatinib showed significance or trend towards significance in all secondary endpoints of reducing mortality and morbidity compared to placebo after correcting for the error. The results were recently presented at the IDWeek 2023 held on October 11-15, 2023 in Boston, Massachusetts. During our

[Table of Contents](#)

continued analysis regarding fostamatinib in hospitalized COVID-19 patients, we provided the updated analysis to the FDA and our partner, the US Department of Defense. Given the end of the federal COVID-19 PHE in May 2023, and based on feedback from the FDA, US Department of Defense DOD and other advisors regarding the program's regulatory requirements, costs, timeline, and potential for success, we decided not to submit an EUA or sNDA.

NIH/NHLBI-sponsored Phase 2 Trial. In September 2020, we announced a Phase 2 clinical trial sponsored by the NIH/NHLBI to evaluate the safety of fostamatinib for the treatment of hospitalized COVID-19 patients. This multi-center, double-blind, placebo-controlled trial randomly assigned fostamatinib or matched placebo (1:1) to 59 evaluable patients. Treatment was administered orally twice daily for 14 days, and a follow-up period to day 60. The primary endpoint of this trial was cumulative incidence of Serious Adverse Events (SAEs) through day 29. The trial also included multiple secondary endpoints designed to assess the early efficacy and clinically relevant endpoints of disease course. The trial completed the enrollment in March 2021. In April 2021, we announced that the Phase 2 clinical trial met its primary endpoint of safety. Fostamatinib reduced the incidence of SAEs by half. By day 29, there were three SAEs in the fostamatinib plus SoC group of thirty patients compared to six SAEs in the placebo plus SoC group of twenty-nine patients ($p=0.23$). Of these, there was a reduction for the disease related SAE of hypoxia in the fostamatinib group compared to placebo (1 vs 3, respectively; $p=0.29$). The data from the NIH/NHLBI-Sponsored Phase 2 trial was published in *Clinical Infectious Diseases*, an official publication of the Infectious Disease Society of America in September 2021.

In May 2021, the NIH/NHLBI Phase 2 clinical data were submitted as part of a request for an EUA from the FDA for fostamatinib as a treatment for hospitalized patients with COVID-19. In August 2021, the FDA informed us that the clinical data submitted from the NIH/NHLBI-sponsored Phase 2 trial of fostamatinib to treat hospitalized patients suffering from COVID-19 was insufficient for an EUA.

ACTIV-4 Host Tissue Phase 2/3 Trial. In June 2021, we announced that fostamatinib had been selected for the NIH ACTIV-4 Accelerating COVID-19 Therapeutic Inventions and Vaccines Phase 2/3 trial (ACTIV-4 Host Tissue Trial in hospitalized patients with COVID-19 Trial), conducted and sponsored by the National Institute of Health (NIH)/National Heart, Lung, and Blood Institute (NHLBI). The ACTIV-4 Host Tissue Trial initiated and funded by NHLBI, is a randomized, placebo-controlled trial of therapies, including fostamatinib, targeting the host response to COVID-19 in hospitalized patients. The master protocol for this trial was designed to be flexible in the number of study arms, the use of a single placebo group, and the stopping and adding of new therapies. Eligible participants include patients hospitalized for COVID-19 with laboratory-confirmed SARS-CoV-2 infection and a new need for oxygen therapy. The primary outcome is oxygen-free days through day 28. Secondary outcomes include 28-day hospital mortality, use of mechanical ventilation, and severity of disease as measured by World Health Organization (WHO) scale scores. The ACTIV-4 Host Tissue Trial is evaluating fostamatinib in a targeted population of approximately 600 hospitalized patients with COVID-19, 300 fostamatinib versus 300 placebo. During the first quarter of 2023, an interim analysis of the trial was completed by the DSMB with a recommendation for the trial to continue. In September 2023, the DSMB recommended that the fostamatinib study arm of the ACTIV-4 Host Tissue Trial platform cease enrollment. Based on the DSMB's review of a conditional power analysis, the DSMB determined that there was an extremely low

[Table of Contents](#)

likelihood of fostamatinib providing benefits related to the primary outcome (oxygen free days) or other secondary outcomes in patients hospitalized and on oxygen therapy for COVID-19. No safety concerns were identified. The NIH/NHLBI concurs with the DSMB's recommendation and has asked the trial investigators to cease enrollment, complete follow-up for participants already enrolled, and complete study closeout. The full study data will be analyzed and disseminated as previously planned.

Imperial College of London Phase 2 Trial. In July 2020, we announced a Phase 2 clinical trial sponsored by Imperial College of London to evaluate the efficacy of fostamatinib for the treatment of COVID-19 pneumonia. This is a two-stage, open label, controlled clinical trial with patients randomized (1:1:1) to fostamatinib plus SoC, ruxolitinib plus SoC, or SoC alone. Treatment was administered twice daily for 14 days and patients receive a follow-up assessment at day 14 and day 28 after the first dose. The primary endpoint of this trial is progression from mild to severe COVID-19 pneumonia within 14 days in hospitalized patients (WHO COVID-19 Severity Scale 3-4). In April 2022, Imperial College of London completed a pre-planned interim analysis of the primary endpoint, patients progressing from mild or moderate (modified WHO COVID-19 scale 3-4) to severe disease (modified WHO COVID-19 scale ≥ 5) within 14 days, in the Phase 2 MATIS trial. The independent data monitoring committee determined that the fostamatinib plus SoC arm did not meet the prespecified criteria for continuation to the next stage of the trial. No safety concerns were identified. The trial remains blinded and Imperial College of London plans to share results with us and scientific community once the trial is complete.

33

[Table of Contents](#)

Other Publications. Researchers at MIT and Harvard led a screen to identify FDA-approved compounds that reduce MUC1 protein abundance. MUC1 is a biomarker used to predict the development of ALI and ARDS and correlates with poor clinical outcomes. In June 2020, the results were presented, and of the 3,713 compounds that were screened, fostamatinib was the only compound identified which both decreased expression of MUC1 and is FDA approved. Fostamatinib demonstrated preferential depletion of MUC1 from epithelial cells without affecting cell viability. The research was focused on drug repurposing for the much lower risk of toxicity and the ability of FDA-approved treatments to be delivered on a shortened timescale, which is critical for patients afflicted with lung disease resulting from COVID-19.

In addition, the in vitro studies led by the Amsterdam University Medical Center at the University of Amsterdam, showed that R406, the active metabolite of fostamatinib, blocked macrophage hyperinflammatory responses to a combination of immune complexes formed by anti-Spike IgG in serum from severe COVID-19 patients. Anti-Spike IgG levels are known to correlate with the severity of COVID-19. These results, presented in July 2020, suggest that by inhibiting anti-Spike IgG-mediated hyperinflammation, R406 could potentially play a role in the prevention of cytokine storms as well as pulmonary edema and thrombosis associated with severe COVID-19.

In December 2020, the Journal of Infectious Diseases published research from NIH which demonstrated that R406, the active metabolite of fostamatinib, was able to inhibit NETosis ex vivo in donor plasma from patients with COVID-19. NETosis is a unique type of cell death resulting in the release of NETs. NETs contribute to thromboinflammation and have been associated with mortality in COVID-19. These data provide insights for how fostamatinib may mitigate neutrophil-associated mechanisms contributing to COVID-19 immunopathogenesis.

Fostamatinib in wAIHA

AIHA is a rare, serious blood disorder where the immune system produces antibodies that result in the destruction of the body's own red blood cells. Symptoms can include fatigue, shortness of breath, rapid heartbeat, jaundice or enlarged spleen. While no medical treatments are currently approved for AIHA, physicians generally treat acute and chronic cases of the disorder with corticosteroids, other immunosuppressants, or splenectomy. Research has shown that inhibiting SYK with fostamatinib may reduce the destruction of red blood cells. AIHA affects an estimated 45,000 Americans, and approximately 36,000 of those patients have wAIHA, where no approved treatment options currently exist.

We completed our Phase 2 clinical trial, also known as the SOAR study, in patients with wAIHA. This trial was an open-label, multicenter, two-stage study that evaluated the efficacy and safety of fostamatinib in patients with wAIHA who had previously received treatment for the disorder but have relapsed. The primary efficacy endpoint of this study was to achieve increased hemoglobin levels by week 12 of greater than 10 g/dL, and greater than or equal to 2 g/dL higher than baseline. In November 2019, we announced updated data that in a Phase 2 open-label study of fostamatinib in patients with wAIHA, data showed that 44% (11/25) of evaluable patients met the primary efficacy endpoint of a hemoglobin level >10 g/dL with an increase of ≥ 2 g/dL from baseline by week 24. Including one late responder at week 30, the overall response rate was 48% (12/25). Adverse events were manageable and consistent with those previously reported with fostamatinib. In February 2022, the American Journal of Hematology published the data from our Phase 2 clinical trial of fostamatinib in adults with wAIHA who have failed at least one prior treatment. The published data demonstrate that fostamatinib rapidly and durably increased hemoglobin levels, with

clinically meaningful hemoglobin responses observed in nearly half of the patients, and a safety and tolerability profile consistent with the existing fostamatinib safety database of patients across multiple disease programs studied.

In January 2021, we announced that the FDA had granted Fast Track designation to fostamatinib for the treatment of wAIHA. The FDA granted orphan drug designation for fostamatinib for the treatment of wAIHA in January 2018.

In March 2019, we initiated our wAIHA pivotal Phase 3 clinical trial of fostamatinib, known as the FORWARD study. The clinical trial protocol calls for a placebo-controlled study of 90 patients with primary or secondary wAIHA who have failed at least one prior treatment. The primary endpoint is a durable hemoglobin response, defined as hemoglobin >10 g/dL and >2 g/dL increase from baseline and durability measure, with the response not being attributed to rescue therapy. In November 2020, we reached an agreement with the FDA on the durable response.

34

[Table of Contents](#)

measure for the primary efficacy endpoint of the trial as well as the inclusion of additional secondary endpoints. In November 2021, we completed the enrollment of this study. In April 2022, we completed the treatment period for the last patient under the trial. In June 2022, we announced top-line efficacy and safety data from the FORWARD study with 90 patients. Patients were randomized 1:1 to receive fostamatinib or matching placebo twice daily for 24 weeks. The primary efficacy endpoint of hemoglobin response was defined as achieving a hemoglobin ≥ 10 g/dL with an increase from baseline ≥ 2 g/dL on three consecutive available visits during the 24-week treatment period. The trial did not demonstrate statistical significance in the primary efficacy endpoint of durable hemoglobin response in the overall study population. The trial also included key secondary endpoints, including hemoglobin response on at least one visit, change in hemoglobin from baseline of ≥ 2 g/dL, use of permitted rescue therapy after week 4, change in hemoglobin from baseline to end of treatment and change from baseline to week 24 in Functional Assessment of Chronic Illness Therapy – Fatigue scale. Across the trial's overall patient population, fostamatinib was generally well-tolerated. The safety profile of the product was consistent with prior clinical experience and no new safety issues were discovered. The most common adverse events ($\geq 10\%$) with fostamatinib and placebo were diarrhea, hypertension, fatigue, pyrexia, nausea, and dyspnea. Treatment-related SAEs were 6.7% (3/45) for fostamatinib and 4.4% (2/45) for placebo. There were five deaths on the trial (2 with fostamatinib and 3 with placebo), all of which were determined to be unrelated to study drug. The safety results were consistent with the overall safety profile data collected to date, which includes more than 5,000 patients across multiple diseases. We conducted an in-depth analysis of these data to better understand differences in patient characteristics and outcomes and submitted these findings to the FDA. In October 2022, we announced that we received guidance from the FDA's review of these findings. Based on the result of the trial and the guidance from the FDA, we did not file an sNDA for this indication.

Of the 90 patients that completed the FORWARD study, 71 (79%) enrolled in the open-label extension study. We plan on closing this study by the end of the year.

Partnered Clinical Programs

R552 – Lilly

Lilly is continuing to advance R552 and has initiated the Phase 2a trial studying R552 in adult patients with moderately to severely active rheumatoid arthritis. The trial plans to enroll 100 patients globally. RIPK1 is implicated in a broad range of key inflammatory cellular processes and plays a key role in tumor necrosis factor signaling, especially in the induction of pro-inflammatory necrosis. The program also includes RIPK1 compounds that cross the blood-brain barrier (CNS-penetrants) to address neurodegenerative diseases such as Alzheimer's disease and amyotrophic lateral sclerosis.

BGB324 – BerGenBio

We have an exclusive, worldwide research, development and commercialization agreement with BerGenBio for our investigational AXL receptor tyrosine kinase inhibitor, BGB324/R428 (now referred to as bemcentinib). In October 2022, BerGenBio announced the initiation of a Phase 1b/2a trial evaluating bemcentinib in combination with the current SoC, checkpoint inhibitor pembrolizumab and doublet chemotherapy, for the treatment of first line non-small cell lung cancer (NSCLC) patients harboring serine/threonine kinase 11 mutations. (BGB324). In February 2023, BerGenBio also announced positive data from the Phase 2 trial of bemcentinib in combination with pembrolizumab in patients

with second-line NSCLC. The treatment with bemcentinib in combination with pembrolizumab demonstrated long survival benefit and sustained disease control, particularly in patients with AXL TPS > 5, substantiating the relevance of AXL as a target and bemcentinib's selective inhibition capabilities in NSCLC. In March 2023, BerGenBio announced its first patient dosed in a Phase 1B/2A trial evaluating bemcentinib in first-line NSCLC patients harboring STK11 mutations. The product is also being investigated in Phase 2 clinical trials in patients with AML and COVID-19. Bemcentinib is being studied in over 600 patients, demonstrating its safety as a monotherapy and in combination with chemotherapy and immune checkpoint inhibition. mutations.

DS-3032 – Daiichi

DS-3032 is an investigational oral selective inhibitor of the MDM2 protein investigated by Daiichi in three Phase 1 clinical trials for solid and hematological malignancies including AML, acute lymphocytic leukemia, chronic myeloid leukemia in blast phase, lymphoma and MDS. Preliminary safety and efficacy data from a Phase 1 trial of DS-3032 suggests that DS-3032 may be a promising treatment for hematological malignancies including R/R AML and high-risk MDS. In September 2020, worldwide rights to DS-3032 (milademetan) were out-licensed from Daiichi to Rain Oncology Inc., formerly Rain Therapeutics Inc. (Rain).

In July 2021, Rain announced January 2024, Phatos AI, Inc. (Pathos) completed the acquisition of Rain. Pathos indicated that it initiated a Phase 3 trial to evaluate the efficacy and safety of has continued interest in further developing milademetan (RAIN-32) for the treatment of cancer patients with unresectable or metastatic dedifferentiated liposarcoma, a rare cancer originating from fat cells located in the soft tissues of the body, and announced the completion of enrollment in August 2022. In May 2023, Rain announced that the trial did not meet using its primary endpoint of progression free survival by blinded independent central review compared to the standard of care. Based on the topline results, Rain does not expect

35

[Table of Contents](#)

to pursue further development of milademetan in dedifferentiated liposarcoma.

In late 2021, Rain commenced its second clinical trial of milademetan in patients with MDM2-amplified solid tumors. In November 2022, Rain provided an interim analysis of the trial which showed that the drug safety profile of milademetan is preliminarily consistent with its prior Phase 1 trial. In May 2023, Rain announced its plan to suspend the enrollment of its Phase 2 trial of milademetan in patients with MDM2-amplified solid tumors, and terminate its plans to initiate the Phase 1/2 study evaluating the safety and efficacy of milademetan in patients with CDKN2A loss/ TP53 wild type advanced solid tumors. propriety PathOS Platform.

Research, Preclinical and Clinical Development Programs

We have retained a selected team of experts in drug discovery and preclinical development to leverage our existing proprietary collection of inhibitors, small-molecule compound libraries and large database of associated phenotypic and biochemical assay results of therapeutic interest. We maintain leading expertise on specific areas of operation such as inhibition of SYK, IRAK 1/IRAK14, RIPK1 and mIDH1 kinases to assist clinical development and commercial affairs, as well as to expand and explore additional opportunities for such inhibitors in the clinical space. Our preclinical operations involve collaborations with clinical research organizations, leading investigators from universities and research organizations around the world, and strategic collaborations with other pharmaceutical companies.

We have assembled a team of experts in drug development to design and implement clinical trials and to analyze the data derived from these trials. The clinical development group possesses expertise in project management and regulatory affairs. We work with external clinical research organizations with expertise in managing clinical trials, drug formulation, and the manufacture of clinical trial supplies to support our drug development efforts.

36

[Table of Contents](#)

We also have strategic development collaborations with MDACC and CONNECT to conduct evaluation of REZLIDHIA (oluatasidenib) in AML, other hematologic cancers and glioma.

Commercialization and Sponsored Research and License Agreements

For a discussion of our Sponsored Research and License Agreements and Government Contract, see See "Note 4 - Sponsored Research, and License Agreements and Government Contract"Contracts" and "Note 5 – In-licensing and Acquisition" to our "Notes to Condensed Financial Statements" contained in Part I, Item 1 of this Quarterly Report on Form 10-Q for related discussions.

[Table of Contents](#)

Results of Operations

Revenues

	Nine Months Ended September						Three Months Ended March 31,		
	Three Months Ended September 30,		Aggregate Change	30,		Aggregate Change	Three Months Ended March 31,		Aggregate Change
	2023	2022		2023	2022		2024	2023	
			(in thousands)			(in thousands)			(in thousands)
Product sales, net	\$ 27,129	\$ 19,188	\$ 7,941	\$ 74,755	\$ 53,935	\$ 20,820	\$ 26,003	\$ 23,745	\$ 2,258
Contract revenues from collaborations	1,005	722	283	5,335	12,529	(7,194)	3,531	2,325	1,206
Government contract	—	2,500	(2,500)	1,000	2,500	(1,500)			
Total revenues	\$ 28,134	\$ 22,410	\$ 5,724	\$ 81,090	\$ 68,964	\$ 12,126	\$ 29,534	\$ 26,070	\$ 3,464

The following table summarizes the percentages of revenues from each of our customers who individually accounted for 10% or more (wherein * denotes less than 10%) of the total net product sales and revenues from collaborations:

	Nine Months Ended September				Three Months Ended March	
	Three Months Ended September 30,		30,		31,	31,
	2023	2022	2023	2022	2024	2023
McKesson Specialty Care Distribution Corporation	49%	44%	46%	38%	42%	45%
Cardinal Healthcare	28%	27%	27%	22%	23%	24%
ASD Healthcare and Oncology Supply	18%	25%	20%	21%		
Kissei	*	*	*	11%		
Cencora Inc. (formerly ASD Healthcare)				23%	22%	

Net Revenue from product sales pertained is related to sales our sale of our products in the US, net of chargebacks, discounts and fees, government and other rebates and returns. For the three and nine months ended September 30, 2023, net product sales increased by

41% and 39%, respectively, compared to the same periods in 2022. The increase was primarily driven by increased TAVALISSE net product sales and the current period net product sales from REZLIDHIA. TAVALISSE net product sales increased by \$5.3 million or 27% in the three months ended September 30, 2023, and \$14.1 million March 31, 2024 of \$21.1 million decreased by \$1.2 million or 5% compared to \$22.3 million in the nine same period in 2023. The decrease was driven partly by a decrease in number of bottles remaining in distributors channels, and partly due to the increase in revenue reserves driven by higher government and private payor rebates. These decreases were partially offset by the increase in price per bottle of TAVALISSE. REZLIDHIA net product sales in the three months ended September 30, 2023, March 31, 2024 of \$4.9 million increased by \$3.4 million compared to \$1.5 million in the same period in 2022. The increase was primarily as a result of increased quantities sold as a result of primarily driven by increased number of patients under therapy, and higher price per bottle, partly offset by the increase in revenue reserves mainly due to higher government rebates. We began our commercialization of REZLIDHIA in December 2022. In the three and nine months ended September 30, 2023, we recognized \$2.7 million and \$6.7 million, respectively, of net product sales from REZLIDHIA.

Contract revenues from collaborations in the three and nine months ended September 30, 2023 include royalty March 31, 2024 consisted of revenue from Kissei of \$2.3 million related to the delivery of drug supplies, revenue from Grifols of \$0.8 million and \$2.3 million, respectively, \$1.1 million related to earned royalty, and revenue from Medison of \$0.2 million for each of the respective periods, \$0.1 million related to the delivery of drug supplies and a milestone pursuant to earned royalty. In the commercial license agreement. In addition, the nine three months ended September 30, 2023 March 31, 2023, contract revenues from collaborations consisted of revenue from Grifols of \$2.8 million \$2.3 million related to the delivery of drug supplies. Contract revenues from collaborations in the three supplies and nine months ended September 30, 2022 include \$0.7 million earned royalty, and \$2.4 million, respectively, in revenue from Grifols related delivery of drug supplies, deferred revenue recognized associated with the remaining performance obligation to research and development services, and royalty revenue. In addition, the nine months ended September 30, 2022 also include \$7.6 million \$0.1 million of revenue from Kissei related to a milestone payment and delivery of drug supplies, \$2.0 million in revenue related to our license agreement with Knight, and \$0.5 million of revenue from Lilly Medison related to the delivery of CNS penetrant IP drug supplies.

Government contract revenue We expect that our future revenues to include product sales of our existing commercial products, TAVALISSE and REZLIDHIA, product sales from our upcoming commercialization later this year of GAVRETO, and product sales from new commercial products we may have in the respective periods presented above were related future. Our net product sales may be impacted by the demand from our customers, changes to government and private payor rebate programs, chargeback and discount programs, co-payment assistance programs, and any other rebate and discount programs we may enter in the income we recognized upon achievement future. In addition, our future revenues may include payments from our existing and new collaboration partners and government grants. As of certain milestones from the \$16.5 million government award granted to us, pursuant to the agreement we entered in January 2021 with the US Department of Defense to support our ongoing Phase 3 clinical trial to evaluate the safety and efficacy of fostamatinib in hospitalized COVID-19 patients. Through September 30, 2023 March 31, 2024, we received \$16.0 million had \$1.4 million of the awards deferred revenue relating to our collaboration agreement with Kissei which we recognized will recognize as revenue in the respective periods, with upon satisfaction of our remaining \$0.5 million available, subject to us meeting certain milestone and approval by the US Department of Defense as specified in the agreement performance obligations.

Table of Contents

Our potential future revenues may include product sales, payments from our collaboration partners and from new collaboration partners with whom we enter into agreements in the future, if any, and from existing government grants and any future grants we may be entitled to, if any, the timing and amount of which is unknown at this time. Our net product sales may be impacted by changes to the government program rebates and new private payor rebate contracts we entered or may enter in the future. As of September 30, 2023, we had deferred revenues of \$1.4 million, which we will recognize as revenue upon satisfaction of our remaining performance obligations under our collaboration agreement with Kissei.

Cost of Product Sales

			Nine Months Ended September						Three Months Ended March 31,			Aggregate		
Three Months Ended September 30,			30,			Aggregate			Three Months Ended March 31,			Aggregate		
2023		2022	Change	2023		2022	Change	2024		2023	Change	2024		2023

Cost of product sales	(in thousands)			(in thousands)			(in thousands)		
	\$ 1,268	\$ 250	\$ 1,018	\$ 3,320	\$ 1,407	\$ 1,913	\$ 2,025	\$ 977	\$ 1,048

The cost of product sales includes the cost of inventories sold to specialty distributors and to our collaborative partners. Inventories sold for the periods presented include inventory quantities acquired or produced prior to the FDA approval of the product, and do not reflect the full cost of the inventories sold, since such costs incurred prior to FDA approval were previously expensed and charged to research and development expense. In particular, we still utilize active pharmaceutical ingredient with zero cost for our TAVALISSE inventories, which we expect to make use of for the next 2 to 3 years. As such, we recognize lower cost of product sales in the periods where we sell inventory quantities acquired or produced prior to the FDA approval of the product. As we acquire or produce more FDA approved inventory quantities in the future, our inventory cost in the balance sheet and cost of product sales will reflect the full cost of acquiring or producing such products. Cost of product sales may also include reserves for potential excess, dated or obsolete inventories, estimated based upon assumptions about future demand and market conditions as well as product shelf life. Further, following the approval of REZLIDHIA, we recognize amortization expense of intangible assets acquired from capitalized intangible asset and in-licensing or acquisition of commercialized products as well as royalty expense on REZLIDHIA sales within cost of sales.

Cost The increase in cost of product sales in the three and nine months ended September 30, 2023 increased March 31, 2024 compared to the same periods period in 2022 2023 was primarily due to amortization increased royalty from sale of capitalized intangible asset recorded in the three REZLIDHIA and nine months ended September 30, 2023 of \$0.3 million and \$0.8 million, respectively, and royalty expense of \$0.4 million and \$1.0 million, respectively. In addition, during the three and nine months ended September 30, 2023, we recorded \$0.4 million of inventory reserves. No such expenses were incurred in the same periods in 2022. These increases were partly offset by the decrease in cost of product sales due to the timing of increased delivery of drug supplies pursuant to our supply agreements with our collaborative partners.

Research and Development Expense

Research and development expense	Nine Months Ended September								
	Three Months Ended September 30,			30,			Three Months Ended March 31,		
	2023	2022	Change	2023	2022	Change	2024	2023	Change
	(in thousands)			(in thousands)			(in thousands)		
Research and development expense	\$ 6,475	\$ 14,666	\$ (8,191)	\$ 21,336	\$ 44,907	\$ (23,571)	\$ 6,026	\$ 10,089	\$ (4,063)
Stock-based compensation expense included in research and development expense	\$ 347	\$ 588	\$ (241)	\$ 1,746	\$ 1,514	\$ 232	\$ 650	\$ 1,023	\$ (373)

Stock-based compensation expense in the nine three months ended September 30, 2023 March 31, 2024 above include an incremental charge of approximately \$0.5 million from stock option modifications recorded in the first quarter of 2023 related to the acceleration of vesting and extension of exercise period of vested stock option grants made to a former officer whose employment ended in March 2023.

The decrease in research and development expense in three months ended September 30, 2023 March 31, 2024 compared to the same period in 2022 2023 was partly due decreased in clinical trial related expenses of \$1.1 million due to timing of trial activities of our IRAK 1/4 inhibitor program, as well as decreased clinical trial activities of \$1.0 million due to timing of trial completion activities of our Phase 3 clinical trials of fostamatinib for the treatment of hospitalized high-risk patients with COVID-19 of \$2.3 million and for the treatment of wAIHA of \$1.7 million. In addition, research and development expense in the three months ended September 30, 2022 included a

[Table of Contents](#)

\$2.0 million upfront payment to Forma (now Novo Nordisk) that was recorded as acquired IPR&D, wAIHA. Further, personnel-related costs and stock-based compensation expense decreased by \$0.9 million, consultants and third party services decreased by \$0.8 million, \$1.0 million and other research and development expense including allocated facilities and laboratory costs decreased by \$1.6 million. These decreases were partially offset by the increase in our research and development expense of our IRAK 1/4 inhibitor program of \$1.1 million.

The decrease in research and development expense in the nine months ended September 30, 2023 compared to the same period in 2022 was partly due to timing of trial completion activities of our Phase 3 clinical trials of fostamatinib for the treatment of hospitalized high-risk patients with COVID-19 of \$6.1 million and for the treatment of wAIHA of \$4.8 million, and timing of activities related to our IRAK 1/4 inhibitor program of \$2.8 million. In addition, research and development expense in the nine months ended September 30, 2022 included \$2.0 million upfront payment to Forma (now Novo Nordisk) that was recorded as acquired IPR&D. Further, personnel-related costs decreased by \$2.0 million, consultants and third party services decreased by \$1.9 million, and other research and development expense including allocated facilities and laboratory costs decreased by \$4.0 million \$1.0 million.

Our research and development expenditures include costs related to preclinical and clinical trials, scientific personnel, supplies, equipment, consultants, sponsored research, stock-based compensation, and allocated facility costs, and upfront payment related to our in-licensed agreement with Forma (now Novo Nordisk) costs. We expect to continue to incur significant research and development expense as we continue our activities in our clinical studies including our IRAK 1/IRAK1/4 inhibitor program program; our collaborative partnerships with MDACC and CONNECT to evaluate REZLDHIA (olutasidenib) in AML, other hematologic cancers and glioma; and any other clinical programs we may pursue in the future. In July 2022, we completed the enrollment of the FOCUS Phase 3 clinical trial of fostamatinib for the treatment of hospitalized high-risk patients with COVID-19. We previously announced in November 2022 the top-line results did not meet statistical significance in the primary efficacy endpoint of the number of days on oxygen through Day 29. Upon further analysis, we discovered an error by the CRO in the application of a statistical stratification factor. After correcting for this statistical error, the primary endpoint of the study was met. Given the end of the federal COVID-19 PHE in May 2023, and based on feedback from the FDA, US Department of Defense and other advisors regarding the program's regulatory requirements, costs, timeline and potential for success, we decided not to submit an EUA or sNDA. Our Phase 3 clinical trial for hospitalized COVID-19 patients was partially funded by the award granted to us by the US Department of Defense as discussed above. Our Phase 3 wAIHA study completed enrollment in November 2021, and in June 2022, we announced that the top-line results did not demonstrate statistical significance in the primary efficacy endpoint. In October 2022, we announced that we received guidance from the FDA's further review of these findings. Based on the result of the trial and the guidance from FDA, we did not file an sNDA for this indication.

We do not track fully burdened research and development costs separately for each of our drug candidates. We review our research and development expense by focusing on three categories: research, development, and other. Our research team is focused on identifying and evaluating product candidates in our focused range of therapeutic indications

[Table of Contents](#)

that can be developed into small molecule therapeutics in our own proprietary programs or with potential collaborative partners. "Research" expenses relate primarily to personnel expenses, lab supplies, fees to third party research consultants and compounds. Our development group leads the implementation of our clinical and regulatory strategies and prioritizes disease indications in which our compounds may be studied in clinical trials. "Development" expenses relate primarily to clinical trials, personnel expenses, costs related to our regulatory filings, lab supplies and fees to third party research consultants. "Other" expenses primarily consist of allocated facilities costs and allocated stock-based compensation expense relating to personnel in research and development groups. "Other" expenses also include the upfront payment to Forma (now Novo Nordisk) and pre-regulatory approval milestone recorded as research and development expense in 2022.

In addition to reviewing the three categories of research and development expense described in the preceding paragraph, we principally consider qualitative factors in making decisions regarding our research and development programs, which include enrollment in clinical trials and the results thereof, the clinical and commercial potential for our drug candidates and competitive dynamics. We also make our

research and development decisions in the context of our overall business strategy, which includes the evaluation of potential collaborations for the development of our drug candidates.

39

[Table of Contents](#)

We do not have reliable estimates regarding the timing of our clinical trials. Preclinical testing and clinical development are long, expensive and uncertain processes, and we cannot reliably predict the timing of such clinical trial activities. In general, biopharmaceutical development involves a series of steps, beginning with identification of a potential target and including, among others, proof of concept in animals and Phase 1, 2 and 3 clinical trials in humans. Significant delays in clinical testing could materially impact our product development costs and timing of completion of the clinical trials. We do not know whether planned clinical trials will begin on time, will need to be halted or revamped or will be completed on schedule, or at all. Clinical trials can be delayed for a variety of reasons, including delays in obtaining regulatory approval to commence a trial, delays from scale up, delays in reaching agreement on acceptable clinical trial agreement terms with prospective clinical sites, delays in obtaining institutional review board approval to conduct a clinical trial at a prospective clinical site or delays in recruiting subjects to participate in a clinical trial.

We currently do not have reliable estimates of total costs for a particular drug candidate to reach the market. Our potential products are subject to a lengthy and uncertain regulatory process that may involve unanticipated additional clinical trials and may not result in receipt of the necessary regulatory approvals. Failure to receive the necessary regulatory approvals would prevent us from commercializing the product candidates affected. In addition, clinical trials of our potential products may fail to demonstrate safety and efficacy, which could prevent or significantly delay regulatory approval.

The following table presents our total research and development expense by category (in thousands).

Categories:	Nine Months Ended September								From January 1, 2007*					
	Three Months Ended September 30,		30,		From January 1, 2007* to September 30, 2023		Three Months Ended March 31,							
	2023	2022	2023	2022	to September 30, 2023	2024	2023	to March 31, 2024						
Research	\$ 375	\$ 545	\$ 1,332	\$ 2,460	\$ 268,615	\$ 455	\$ 503	\$ 269,511						
Development	5,616	10,304	17,379	35,432	560,199	4,803	7,940	567,278						
Other	484	3,817	2,625	7,015	276,781	768	1,646	278,018						
	\$ 6,475	\$ 14,666	\$ 21,336	\$ 44,907	\$ 1,105,595	\$ 6,026	\$ 10,089	\$ 1,114,807						

* We started tracking research and development expense by category on January 1, 2007.

"Other" expenses in the three and nine months ended September 30, 2023 March 31, 2024 and 2023 consisted of allocated facilities costs of \$0.1 million and \$0.9 million \$0.6 million, respectively, and allocated stock-based compensation expense of \$0.3 million \$0.7 million and \$1.7 million \$1.0 million, respectively. "Other" expenses for the three and nine months ended September 30, 2022 consisted of allocated facilities costs of \$1.2 million and \$3.5 million, respectively, allocated stock-based compensation expense of \$0.6 million and \$1.5 million, respectively, and acquired IPR&D of \$2.0 million related to our in-license agreement with Forma (now Novo Nordisk) for each of the respective periods.

The major portion of our total research and development expense in the three and nine months ended September 30, 2023 March 31, 2024 and 2023 was associated with our IRAK 1/4 inhibitor program. In the three and nine months ended September 30, 2022, the major portion of our total research and development expense was associated with our COVID-19, AIHA and IRAK 1/4 inhibitor program.

Selling, General and Administrative Expense

	Three Months Ended September 30,		Aggregate Change	Nine Months Ended September 30,		Aggregate Change
	2023	2022		2023	2022	
	(in thousands)	(in thousands)		(in thousands)	(in thousands)	

Selling, general and administrative expense	\$ 24,856	\$ 25,897	\$ (1,041)	\$ 78,891	\$ 80,279	\$ (1,388)
Stock-based compensation expense included in selling, general and administrative expense	\$ 1,596	\$ 2,119	\$ (523)	\$ 5,127	\$ 6,791	\$ (1,664)

40 39

[Table of Contents](#)

Selling, General and Administrative Expense

	Three Months Ended March 31,			Aggregate	
	2024		2023		
				(in thousands)	
Selling, general and administrative expense	\$ 28,449	\$ 27,729	\$ 720		
Stock-based compensation expense included in selling, general and administrative expense	\$ 4,484	\$ 1,735	\$ 2,749		

Stock-based compensation expense in the nine months ended September 30, 2022 above include an incremental charge of approximately \$0.8 million from stock option modifications recorded in the first quarter of 2022 related to the extension of the exercise period of the stock option grants made to our two former Board of Directors whose terms expired in May 2022.

The decrease increase in selling, general and administrative expense in the three months ended **September 30, 2023** March 31, 2024 compared to the same period in **2022** 2023 was mainly primarily due to lower the increase of \$2.7 million in stock-based compensation expense primarily from our performance-based stock awards. This was partially offset by the \$0.7 million decrease in consulting and other third-party services, decrease of \$0.5 million in commercial related expenses primarily driven by the timing of commercialization activities for our new products, and lower decrease of \$0.8 million in other various sales, general and administrative costs including allocated facilities of \$0.5 million.

The decrease in selling, general and administrative expense in the nine months ended September 30, 2023 compared to the same period in 2022 was mainly primarily due to lower stock-based compensation expense of \$1.7 million partly due to stock option modification as discussed above, and lower other various sales, general and administrative costs facilities cost.

of \$1.7 million. These decreases we partially offset by higher commercial-related expenses of \$1.0 million and higher travel-related expenses of \$1.0 million.

We expect to incur significant selling, general and administrative expenses, as we expect our commercial related expenses to increase as we continue to expand our commercial activities of our products existing products, TAVALISSE and REZLIDHIA, and our upcoming commercialization later this year of GAVRETO. We continue to deploy resources to enable our field-based employees to engage with healthcare providers. These engagements have enabled our field team to cover existing prescribers, as well as develop relationships with new prescribers to identify appropriate patients for our products.

Interest Income and Interest Expense

	Three Months Ended September 30,			September 30,			Three Months Ended March 31,			Aggregate		
	2023		2022	2023		2022	2024		2023	2023		Change
Interest income	\$ 672	\$ 192	\$ 480	\$ 1,594	\$ 255	\$ 1,339	\$ 593	\$ 393	\$ 200			
Interest expense	\$ (1,899)	\$ (826)	\$ (1,073)	\$ (4,965)	\$ (2,600)	\$ (2,365)	\$ (1,874)	\$ (1,204)	\$ (670)			

Interest income is related to our interest-bearing cash and investment balances. The increase interest income in the three and nine months ended **September 30, 2023, March 31, 2024** compared to the same periods period in **2022, 2023** was primarily driven by higher interest rates.

Interest expense comprised primarily of interest on the outstanding term loan with MidCap. The increase in interest expense in the three and nine months ended **September 30, 2023, March 31, 2024** compared to the same periods in **2022, 2023** was primarily due to higher interest expense on our term loan with Midcap of \$1.1 million and \$3.1 million, respectively, driven by higher outstanding term loan balance outstanding throughout the period, as well as higher interest rates. The increase interest expense the \$20.0 million (Tranche 5) was funded in the nine months ended **September 30, 2023** compared to the same period of 2022 was partly offset by the interest expense recognized in first quarter of 2022 related to the accretion of financing liability with Lilly of \$0.7 million, **March 2023**.

Critical Accounting Policies and Use of Estimates

Our discussion and analysis of our financial condition and results of operations is based upon our financial statements, which have been prepared in accordance with US GAAP. The preparation of these financial statements requires us to make estimates and assumptions that affect the amounts reported in the financial statements and accompanying notes. We base our estimates on historical experience and on various other assumptions that we believe to be reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions.

Our critical accounting estimates are described in "Item 7 – Management's Discussion and Analysis of Financial Condition and Results of Operations - Critical Accounting Estimates" in our Annual Report on Form 10-K. There had been no material changes to these accounting policies.

41 40

Table of Contents

Our critical accounting estimates and significant accounting policies are described in "Note 1 – Description of Business and Summary of Significant Accounting Policies" to our "Notes to Financial Statements" contained in "Part II, Item 8, Financial Statements and Supplementary Data" of our Annual Report on Form 10-K for the year ended **December 31, 2022, December 31, 2023**. There have been no material changes to these accounting policies, policies except for the accounting consideration related to the Asset Purchase Agreement with Blueprint as discussed in "Note 5 – In-licensing and Acquisition" to our "Notes to Condensed Financial Statements" contained in Part I, Item 1 of this Quarterly Report on Form 10-Q.

Recent Accounting Pronouncements

No new accounting guidance was adopted during the period. Recently See related discussions of recently issued accounting guidance is standards in "Note 1 – Organization and Summary of Significant Accounting Policies" to our "Notes to Condensed Financial Statements" contained in Part I, Item 1 of this Quarterly Report on Form 10-Q. We continue to evaluate accounting standards that were recently issued but not applicable or did not have, or is not expected to have, a material impact to us, yet adopted, as applicable.

Liquidity and Capital Resources

Liquidity

As of **September 30, 2023, March 31, 2024** and **December 31, 2022, December 31, 2023**, we had approximately **\$62.4 million, \$49.6 million and \$58.2 million, \$56.9 million**, respectively, in cash, cash equivalents and short-term investments. We continue to maintain investment portfolios primarily in money market funds, US treasury bills, government-sponsored enterprise securities, and corporate bonds and commercial paper. Cash in excess of immediate requirements is invested with regard to liquidity and capital preservation. We view our investments portfolio as available-for-sale and are available for use in current operations. Wherever possible, we seek to minimize the potential effects of concentration and degrees of risk. We continue to monitor the impact of the changes in the conditions of the credit and financial markets to our investment portfolio and assess if future changes in our investment strategy are necessary.

Following summarizes our cash flow activity for the periods presented:

	Nine Months Ended September 30,		Three Months Ended March	
	2023	2022	2024	2023
	(in thousands)			
Net cash provided by (used in):				
Operating activities	\$ 497	\$ (51,849)	\$ (5,013)	\$ (4,074)
Investing activities	2,577	54,179	313	777
Financing activities	17,881	8,646	(2,512)	19,123
Net increase in cash and cash equivalents	\$ 20,955	\$ 10,976	\$ (7,212)	\$ 15,826
Net (decrease) increase in cash and cash equivalents				

Net cash provided used in operating activities for the **nine** **three** months ended **September 30, 2023** was **March 31, 2024** and **2023** were primarily due to payments of our operating expenses, partially offset by the proceeds received from sales of our existing commercial products, (TAVALISSE and REZLIDHIA), cash receipt received from our collaboration partners, including partners. Net cash used in operating activities for the **three** months ended **March 31, 2023** include \$20.0 million regulatory milestone payment from Kissei received in January **2023**, and cash received from the awards granted by the US Department of Defense, partially offset by the payments of our operating expenses. Net cash used in operating activities for the **nine** months ended **September 30, 2022** was primarily due to payments for our operating expenses, partially offset by the proceeds from sales of our product (TAVALISSE), and cash received from collaboration partners. **2023**.

Net cash provided by investing activities for the **nine** **three** months ended **September 30, 2023** comprises **March 31, 2024** comprised net maturities of short-term investments of \$17.4 million \$0.5 million, partially offset by a payment for capitalized intangible asset related to the **Asset Purchase Agreement with Blueprint** of \$0.1 million. Net cash provided by investing activities for the **three** months ended **March 31, 2023** comprised maturities of short-term investments of \$15.7 million and proceeds from sale of property and equipment of \$0.1 million, partially offset by the payment of milestone obligations to Forma (now Novo Nordisk) recorded as intangible assets of \$15.0 million.

Net cash provided by investing used in operating activities for the **nine** **three** months ended **September 30, 2022** comprises net maturities **March 31, 2024** comprised cost share payments to a collaboration partner of short-term investments of \$54.0 million and proceeds from sale of property and equipment of \$0.5 million \$2.6 million, partially offset by purchases the net proceeds from issuance of property and equipment common stock upon exercise of \$0.4 million.

stock options. Net cash provided by financing activities for the **nine** **three** months ended **September 30, 2023** **March 31, 2023** was primarily due to the net cash proceeds from term loan financing (Tranche 5) of \$20.0 million and proceeds from exercise of stock options and participation in the Purchase Plan of \$0.6 million, partially offset by our cost share payments to Lilly of \$2.6 million. Net cash provided by financing activities for the **nine** months ended **September 30, 2022** was primarily due to the net cash proceeds from term loan financing (Tranche 3) of \$19.5 million and proceeds from exercise of stock options and participation in the Purchase Plan of \$1.5 million, partially offset by our payment of cost share to Lilly of \$12.4 million \$0.8 million.

4241

[Table of Contents](#)

We believe that our existing capital resources will be sufficient to support our current and projected funding requirements, including the continued commercialization of our products, through at least the next 12 months from the Form 10-Q filing date. We have based this estimate on assumptions that may prove to be wrong, and we could utilize our available capital resources sooner than we currently expect. Because of

the numerous risks and uncertainties associated with commercializing a product, the development of our product candidates and other research and development activities, we are unable to estimate with certainty our future product revenues, our revenues from our current and future collaborative partners, the amounts of increased capital outlays and operating expenditures associated with our current and anticipated clinical trials and other research and development activities.

Capital Resources

Since inception, we have financed our operations primarily through sales of equity securities, debt financing, from sales of our products, and contract payments under our collaboration agreements.

Under our existing collaboration agreements that we entered in the ordinary course of business, we received or may be entitled to receive upfront cash payments, payments contingent upon specified events achieved by such partners and royalties on any net sales of products sold by such partners under the agreements. As of **September 30, 2023** **March 31, 2024**, total future contingent payments to us under our existing agreements, excluding terminated agreements, could exceed \$1.3 billion if all potential product candidates achieved all of the payment triggering events under all of our current agreements. This estimated future contingent amount does not include any estimated royalties that could be due to us if the partners successfully commercialize any of the licensed products. Future events that may trigger payments to us under the agreements are based solely on our partners' future efforts and achievements of specified development, regulatory and/or commercial events. See further discussion in "Note 4 – Sponsored Research, and License Agreements and Government Contract"Contracts" to our "Notes to Condensed Financial Statements" contained in Part I, Item 1 of this Quarterly Report on Form 10-Q.

In January 2021, we were awarded \$16.5 million by the US Department of Defense to support our ongoing Phase 3 clinical trial to evaluate the safety and efficacy of fostamatinib in hospitalized COVID-19 patients. Under the agreement with the US Department of Defense, we are entitled to receive such award based on the agreed-upon payment schedule, subject to submission of proper documentation as evidence of completion of certain clinical trial events or milestones as specified in the agreement, and approval by the US Department of Defense that such events or milestones have been met. Through September 30, 2023, we received \$16.0 million of the awards which we recognized as revenue in the respective periods, with remaining \$0.5 million available, subject to us meeting certain milestone and approval by the US Department of Defense that such milestone has been met, as specified in the agreement.

In August 2020, we entered into an Open Market Sale Agreement with Jefferies LLC (Jefferies), as a sole agent, pursuant to which we may sell from time to time, through Jefferies, shares of our common stock in sales deemed to be "at-the-market offerings" as defined in Rule 415 under the Securities Act, subject to conditions specified in the Open Market Sale Agreement, including maintaining an effective registration statement covering the sale of shares under the Open Market Sale Agreement. We have a shelf registration statement filed with the SEC that was declared effective on May 3, 2022, which registered, among other securities, a base prospectus which covers the offering, issuance, and sale by us of up to \$250.0 million in the aggregate of the securities identified from time to time in one or more offerings, which include the \$100.0 million of shares of our common stock that may be offered, issued and sold under the Open Market Sale Agreement. As of **September 30, 2023** **March 31, 2024**, we have not sold any shares of common stock under such Open Market Sale Agreement.

We have a Credit Agreement with MidCap entered in September 2019, and subsequently amended in March 2021 (First Amendment), in February 2022 (Second Amendment) and in July 2022 (Third Amendment). The Credit Agreement that provides for \$60.0 million term loan credit facility, which was fully funded as of **September 30, 2023** **March 31, 2024**. To date, no remaining funds are available See also "Note 12 – Subsequent Events" to our "Notes to Condensed Financial Statements" contained in Part I, Item 1 of this Quarterly Report on Form 10-Q for draw under additional details of the term loan credit facility with Midcap, recent amendment to the Credit Agreement.

Our operations will require significant additional funding in the foreseeable future. Unless and until we can generate sufficient cash from our operating activities, we may choose to raise additional funds through public and/or private offerings of equity securities, debt financings, or from other sources. However, certain external factors such as global pandemics, the global tensions arising from the Russia-Ukraine war and Hamas-Israel war, political and economic legislations, and other factors may continue to rapidly evolve which could significantly disrupt the global financial

markets. Our ability to raise additional funds may be adversely impacted by potential worsening of global economic conditions and volatility in the credit and financial markets in the US and worldwide. We could experience an inability to access additional funds, which could in the future negatively affect our capacity for certain corporate development transactions or our ability to make important, opportunistic investments. To the extent that we raise additional funds through the sale of equity, our shareholders' ownership interest may experience substantial dilution. Our current credit facility with MidCap and any debt financing that we can obtain in the future may involve operating covenants that may restrict our business. To the extent that we raise additional funds through collaboration and licensing arrangements, we may be required to relinquish some of our rights to our technologies or product candidates or grant licenses on terms that are not favorable to us.

[Table of Contents](#)

Our future funding requirements will depend upon many factors, including, but not limited to:

- the ongoing costs to commercialize our products, or any other future product candidates, if any such candidate receives regulatory approval for commercial sale;
- our ability to generate expected revenue from our commercialization efforts;
- the progress and success of our clinical trials and preclinical activities (including studies and manufacture of materials) of our product candidates conducted by us;
- our ability to secure and maintain our patent protection and regulatory rights;
- our ability to meet operating covenants under our current and future credit facilities, if any;
- our ability to enter into partnering opportunities across our pipeline within and outside the US;
- the costs and timing of regulatory filings and approvals by us and our collaborators;
- the progress of research and development programs carried out by us and our collaborative partners;
- any changes in the breadth of our research and development programs;
- the ability to achieve the events identified in our collaborative agreements that may trigger payments to us from our collaboration partners;
- our ability to acquire or license other technologies or compounds that we may seek to pursue;
- our ability to manage our growth;
- competing technological and market developments;
- the costs and timing of obtaining, enforcing and defending our patent and other intellectual property rights, including regulatory rights such as regulatory data exclusivities; and
- expenses associated with any unforeseen litigation, including any arbitration and securities class action lawsuits.

Insufficient funds may require us to delay, scale back or eliminate some or all of our commercial efforts and/or research or development programs, to lose rights under existing licenses or to relinquish greater or all rights to product candidates at an earlier stage of development or on less favorable terms than we would otherwise choose or may adversely affect our ability to operate as a going concern.

Material Cash Requirements

We conduct our commercial activities and research and development programs internally and through third parties that include, among others, arrangements with vendors, consultants, contract research organizations (CROs) and universities. We have contractual arrangements with these parties, however our contracts with them are cancelable generally on reasonable notice within one year and our obligations under these contracts are primarily based on services performed. We do not have any purchase commitments under any collaboration arrangements.

We have agreements with certain clinical research organizations to conduct our clinical trials **including our recent strategic development collaborations with MDACC and CONNECT, as well as** with third parties relative to our commercialization of our products. The

timing of payments for any amounts owed under the respective agreements will depend on various factors including, but not limited to, patient enrollment and other progress

44

[Table of Contents](#)

of the clinical trial trials, and various activities related to commercial launch. commercialization. We expect that we will continue to enter into contracts in the normal course of business with various third parties who support our clinical trials, support our preclinical research studies, and provide other services related to our operating purposes as well as our commercialization of our products. We can terminate these agreements at any time, and if terminated, we would not be liable for the full amount of the respective agreements. Instead, we will be liable for services provided through the termination date plus certain cancellation charges, if any, as defined in each of the respective agreements. In addition, these agreements may, from time to time, be subjected to amendments as a result of any change orders executed by the parties.

43

[Table of Contents](#)

As discussed in detail in "Note 4 – Sponsored Research, and License Agreements and Government Contract" Contracts" of our "Notes to Condensed Financial Statements" contained in Part I, Item 1 of this Quarterly Report on Form 10-Q, pursuant to the amended Lilly Agreement, and us providing the first opt-out notice to Lilly on September 29, 2023, we are responsible for funding the development costs for R552 in the US, Europe, and Japan, up to \$22.6 million through April 1, 2024. Through September 30, 2023 March 31, 2024, Lilly billed us \$17.7 million \$20.3 million of the funding development costs incurred as of the fourth quarter of 2023, and the amounts were amount was fully paid as of September 30, 2023 March 31, 2024. The amended Lilly Agreement, however, provides us the right to opt-in to co-funding the R552 development, upon us providing notice to Lilly within 30 days of certain events, as specified in the Lilly Agreement. If we decide to exercise our opt-in right, we will be required to continue to share in global development costs, and if we later exercise our second opt-out right (no later than April 1, 2025), our share in global development costs will be up to a specified cap through December 31, 2025, as provided for in the Lilly Agreement.

As discussed in detail in "Note 5 – In-licensing and Acquisition" of our "Notes to Condensed Financial Statements" contained in Part I, Item 1 of this Quarterly Report on Form 10-Q, pursuant to an Asset Purchase Agreement with Blueprint entered in February 2024, we agreed to pay Blueprint a purchase price of \$15.0 million, \$10.0 million of which is payable upon our first commercial sale of GAVRETO (pralsetinib) and an additional \$5.0 million of which is payable on the first anniversary of the closing date of the agreement, subject to certain conditions. Blueprint is also eligible to receive up to \$97.5 million in future commercial milestone payments and up to \$5.0 million in future regulatory milestone payments, in addition to tiered royalties ranging from 10% to 30%. Simultaneously and in conjunction with entering into the Asset Purchase Agreement, we also entered into certain supporting agreements, including a customary transition agreement, pursuant to which, during the transition period, Blueprint will transition regulatory and distribution responsibility for GAVRETO (pralsetinib) to us. We also agreed to purchase certain drug product inventories from Blueprint amounting to approximately \$7.0 million under a Material Transfer Agreement. As of March 31, 2024, we received inventories amounting to approximately \$3.1 million, and the remaining inventories are expected to be delivered to us in the second quarter of 2024.

Additionally, as discussed in detail in "Note 45 – Sponsored Research In-licensing and License Agreements and Government Contract" Acquisition" of our "Notes to Condensed Financial Statements" contained in Part I, Item 1 of this Quarterly Report on Form 10-Q, pursuant to our license and transition services agreement, Forma (now Novo Nordisk) is entitled to potential development and regulatory milestone payments of up to \$67.5 million, commercial milestone payments of up to \$165.5 million, and tiered royalty payments. As of

December 31, 2022, in 2022, certain milestones were met which entitled Forma (now Novo Nordisk) to receive \$17.5 million milestone payments, of which, \$2.5 million was paid in the fourth quarter of 2022 and \$15.0 million was paid in the first quarter of 2023. No new milestone was met in 2023 and during the nine three months ended September 30, 2023 March 31, 2024.

As of September 30, 2023 March 31, 2024, we have a contractual commitment related to our leased facilities of \$1.2 million \$0.9 million, with approximately \$0.7 million payable within 12 months. See "Note 10 11 – Leases" to our "Notes to Condensed Financial Statements" contained in Part I, Item 1 of this Quarterly Report on Form 10-Q for further discussions of our leases.

As discussed above, we have a contractual commitment with respect to our credit facility with MidCap. Under the amended Credit Agreement, the term loans mature on September 1, 2026, MidCap, and the interest-only period is through October 1, 2024. The interest rate applicable to the term loans under the amended Credit Agreement is the sum as of one-month SOFR, plus an adjustment of 0.11448%, subject to 1.50% applicable floor, plus applicable margin of 5.65%. A final payment fee of 2.5% of principal is due at maturity date of the term loans. As of September 30, 2023 March 31, 2024, the outstanding principal amount of the loan was \$60.0 million. As discussed in detail in "Note 12 – Subsequent Events" to our "Notes to Condensed Financial Statements" contained in Part I, Item 1 of this Quarterly Report on Form 10-Q, in April 2024, we entered into Fourth Amendment to the Credit Agreement with MidCap, which among other things, (i) extended the maturity date for the term loans to September 1, 2027, (ii) extended the interest only period for the term loans to October 1, 2025, (iii) revised the interest rate payable on the term loans to SOFR plus an adjustment of 0.11448%, subject to 4.00% applicable floor, plus applicable margin of 6.50%, (iv) reset the prepayment fee applicable to the term loans, (v) increased the exit fee payable on the term loans to 4.25% and (vi) updated certain financial covenants in connection with the new maturity date. Following the Amended Credit Agreement which extended the interest only period for the term loans to October 1, 2025, no principal payments are due within 12 months. We are also obligated to pay annual administrative fees. As Further, as of September 30, 2023 March 31, 2024, future interest including final fee calculated using following the base interest rate as per Fourth Amendment to the Credit Agreement and the final fee payments associated with the credit facility amounted to \$10.5 million \$19.3 million, with of which, approximately \$4.4 million \$6.6 million is payable within 12 months.

We are also subject to claims related to the patent protection of certain of our technologies, as well as purported securities class action lawsuit, other litigations, and other contractual agreements. We are required to assess the likelihood of any adverse judgments or outcomes to these matters as well as potential ranges of probable losses. A determination of the amount of reserves required, if any, for these contingencies is made after careful analysis of each

44

[Table of Contents](#)

individual matter. We do not have other material contractual commitments with respect to matters discussed above.

45

[Table of Contents](#)

Item 3. Quantitative and Qualitative Disclosures About Market Risk

We are exposed to market risks in the ordinary course of our business. These risks primarily include interest rate sensitivities related to our short-term investments and outstanding loans, borrowings. There were no material changes to our quantitative and qualitative disclosures about market risk related to our investment activities risks during the nine three months ended September 30, 2023 March 31, 2024 as disclosed in "Item 7A. Quantitative and Qualitative Disclosures About Market Risks" of our Annual Report on Form 10-K for the year ended December 31, 2022 December 31, 2023.

Item 4. Controls and Procedures

Evaluation of Disclosure Controls and Procedures. Based on the evaluation of our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act), our chief executive officer (who serves as our principal executive officer) and our chief financial officer (who serves as our principal financial officer) have concluded that, as of the end of the period covered by this report, our disclosure controls and procedures were effective.

Changes in Internal Controls. There were no changes in our internal control over financial reporting that occurred during the quarter ended September 30, 2023 March 31, 2024 that have materially affected or are reasonably likely to materially affect our internal control over financial reporting.

Limitations on the Effectiveness of Controls. A control system, no matter how well conceived and operated, can provide only reasonable, not absolute, assurance that the objectives of the controls are met. Because of the inherent limitations in all control systems, no evaluation of controls can provide absolute assurance that all control issues, if any, within a company have been detected. Accordingly, our disclosure controls and procedures are designed to provide reasonable, not absolute, assurance that the objectives of our disclosure control system are met and, as set forth above, our chief executive officer and chief financial officer have concluded, based on their evaluation as of the end of the period covered by this report, that our disclosure controls and procedures were sufficiently effective to provide reasonable assurance that the objectives of our disclosure control system were met.

PART II. OTHER INFORMATION

Item 1. Legal Proceedings

From time to time, we may be a party or subject to legal proceedings and claims, either asserted or unasserted, which arise in the ordinary course of business. Some of these proceedings that we may be involved in the future, are claims that are subject to substantial uncertainties and unascertainable damages or other remedies.

Our threshold for disclosing material environmental legal proceedings involving a government authority where potential monetary sanctions are involved is \$1.0 million.

In June 2022, we received a notice letter regarding an ANDA submitted to the FDA by Annora Pharma Private Limited (Annora), requesting approval to market a generic version of TAVALISSE. In July 2022, The notice letter included a Paragraph IV certification with respect to our US Patent Nos. 7,449,458; 8,263,122; 8,652,492; 8,771,648 and 8,951,504, which are listed in the FDA's Approved Drug Products with Therapeutic Equivalence Evaluations (referred to as the "Orange Book"). The notice letter asserts that these patents will not be infringed by Annora's proposed product, are invalid and/or are unenforceable. Annora's notice letter does not provide a Paragraph IV certification against our other patents listed in the Orange Book. On July 25, 2022, we filed a lawsuit in the United States US District Court for the District of New Jersey against Annora and its subsidiaries affiliates, Hetero Labs Ltd., and Hetero USA, Inc., for infringement of certain of our US patents. In September 2022, patents identified in Annora's Paragraph IV certification. On September 21, 2022, Annora and its affiliates answered and counterclaimed for declaratory judgment of non-infringement and invalidity of certain the '458, '122, '492, '648, and '504 patents. We served an answer to Annora's counterclaims in October 2022. Annora served invalidity and non-infringement contentions in December 2022. We served an answer to Annora's invalidity and non-infringement contentions in March 2023. Litigation continues, with preparation for a Markman hearing expected early next year. and no trial date is currently set. We intend to vigorously enforce and defend our intellectual property related to TAVALISSE. For a more detailed discussion of this litigation matter, see Part I, Item 3, "Legal Proceedings" of our Annual Report on Form 10-K as of December 31, 2022.

46 45

[Table of Contents](#)

Item 1A. Risk Factors

In evaluating our business, you should carefully consider the following risks, as well as the other information contained in this Quarterly Report on Form 10-Q. These risk factors could cause our actual results to differ materially from those contained in forward-looking statements we have made in this Quarterly Report on Form 10-Q and those we may make from time to time. If any of the following risks actually occurs,

our business, financial condition and operating results could be harmed. The risks and uncertainties described below are not the only ones facing us. Additional risks and uncertainties not presently known to us, or that we currently see as immaterial, may also harm our business.

We have marked with an asterisk (*) those risk factors below that reflect a substantive change from the risk factors included in our Annual Report on Form 10-K for the year ended December 31, 2022 December 31, 2023 filed with the SEC on March 7, 2023 March 5, 2024, if any.

Risk Factor Summary

- Our prospects are highly dependent on our existing commercial products, TAVALISSE (fostamatinib disodium hexahydrate) and REZLIDHIA (olutasidenib), and our upcoming commercialization later this year of GAVRETO (pralsetinib) which we recently acquired from Blueprint. To the extent that the commercial success of our products in the US and respective territories outside of the US is diminished or halted, our business, financial condition and results of operations may be adversely affected, and the price of our common stock may decline.
- We may not be able to successfully develop or commercialize our product candidates if problems arise in the clinical testing and/or approval process. There is a high risk that drug discovery and development efforts might not generate successful product candidates. If the results of our clinical trials do not meet the primary efficacy endpoints, or if the top-line data from the results of our clinical trials may not ultimately meet the requirements for an NDA approval by the FDA and other regulatory authorities, the commercial prospects of our business may be harmed, and our ability to generate product revenues may be delayed or eliminated.
- Our strategy to expand our hematology and oncology pipeline on our own, or through acquisitions or in-licensing of early or late-stage products or companies, or through partnerships with pharmaceutical and biotechnology companies, as well as academic institutions and government organizations, may not be successful.
- Even if we, or any of our collaborative partners, are able to continue to commercialize our products or any product candidate that we, or they, develop, the product may become subject to unfavorable pricing regulations, unfavorable health technology assessments (HTA), third-party payor reimbursement practices or labeling restrictions, all of which may vary from country to country and any of which could harm our business.
- If we are unable to successfully market and distribute our products and retain experienced commercial personnel, our business will be substantially harmed.
- We are subject to stringent and evolving healthcare regulatory, privacy and information security laws, regulations, rules, policies and contractual obligations, and changes in such laws, regulations, rules, policies, contractual obligations and our actual or perceived failure to comply with such requirements could subject us to significant investigations, audits, fines, penalties, and claims, any of which may have a material adverse effect on our business, financial condition, results of operations or prospects.
- If manufacturers obtain approval for generic versions of our products, or of products with which we compete, our business may be harmed.
- Unforeseen safety issues could emerge with our products that could require us to change the prescribing information to add warnings, limit use of the product, and/or result in litigation. Any of these events could have a negative impact on our business.
- We rely and may continue to rely on third party third-party distribution facilities for the sale of our products and potential sale of any of our product candidates. If any or all of them become subject to adverse findings from inspections or face other difficulties to operate, then the distribution of our products may be interrupted or otherwise

[Table of Contents](#)

adversely affected.

- We lack the capability to manufacture compounds for clinical development and we intend to rely on third parties for commercial supply, manufacturing and distribution, if any, of our product candidates which receive regulatory approval and we may be unable to obtain required material or product in a timely manner, at an acceptable cost or at a quality level required to receive regulatory approval.

[Table of Contents](#)

- Any product for which we have obtained regulatory approval, or for which we obtain approval in the future, is subject to, or will be subject to, extensive ongoing regulatory requirements by the FDA, EMA, MHRA and other comparable regulatory authorities, and if we fail to comply with regulatory requirements or if we experience unanticipated problems with our products, we may be subject to penalties, we may be unable to generate revenue from the sale of such products, our potential for generating positive cash flow may be diminished, and the capital necessary to fund our operations will be increased. **Additionally, approval of a drug under the accelerated drug approval program may be withdrawn or the labeled indication of the drug changed if trials fail to verify clinical benefit or do not demonstrate sufficient clinical benefit to justify the risks associated with the drug.**
- If our corporate collaborations or license agreements are unsuccessful, or if we fail to form new corporate collaborations or license agreements, our research and development efforts could be delayed.
- Our success is dependent on **securing intellectual property rights** and data exclusivity and **similar other** regulatory rights (such as **orphan exclusivity, pediatric extensions and supplementary protection certificate**) held by us and third parties, and our interest in such rights is complex and uncertain.
- If a dispute arises regarding the infringement or misappropriation of the proprietary rights of others, such dispute could be costly and result in delays in our research and development activities, partnering and commercialization activities.
- If our competitors develop technologies that are more effective than ours, our commercial opportunity will be reduced or eliminated.
- If product liability lawsuits are successfully brought against us, we may incur substantial liabilities and may be required to limit commercialization of our products.

[Table of Contents](#)

Risks Related to Our Business and Our Industry

If the market opportunities for our products and product candidates are smaller than we believe they are, our revenues may be adversely affected, and our business may suffer.

Certain of the diseases that our products and our other product candidates being developed to address are in underserved and underdiagnosed populations. Our projections of both the number of people who have these diseases, as well as the subset of people with these diseases who will seek treatment utilizing our products or product candidates, may not be accurate. If our estimates of the prevalence or number of patients potentially on therapy prove to be inaccurate, the market opportunities for **fostamatinib** **our products** and our other product candidates may be smaller than what we believe they are, our prospects for generating expected revenue may be adversely affected and our business may suffer.

We may need to continue to increase the size of our organization and we may encounter difficulties with managing our growth, which could adversely affect our business and results of operations.

While we have substantially increased the size of our organization particularly in our sales force in the third quarter of 2021, we also implemented two separate reductions in workforce particularly in November our research and development group in 2021 and October 2022, and 2022. We may need to add additional qualified personnel and resources to support our commercial activities and expected growth. Our current infrastructure may be inadequate to support our development and commercialization efforts and expected growth. Future growth will impose significant added responsibilities on members of management, including the need to identify, recruit, maintain and integrate additional employees, and may take time away from running other aspects of our business, including commercialization of our products and development of our other product candidates.

[Table of Contents](#)

Our future financial performance and our ability to sustain successful commercialization of our products and our ability to commercialize other product candidates that may receive regulatory approval will depend, in part, on our ability to manage any future growth effectively. In particular, as we continue to commercialize our products, we will need to support the training and ongoing activities of our sales force and will likely need to continue to expand the size of our employee base for managerial, operational, financial and other resources. To that end, we must be able to successfully:

- manage our development efforts effectively;
- integrate additional management, administrative and manufacturing personnel;
- further develop our marketing and sales organization; and
- maintain sufficient administrative, accounting and management information systems and controls.

We may not be able to accomplish these tasks or successfully manage our operations and, accordingly, may not achieve our research, development, and commercialization goals. Our failure to accomplish any of these goals, including as a result of business or other interruptions resulting from if any, a potential future impacts of a pandemic or global economic slowdown, could adversely affect our business and operations.

Our strategy to expand our hematology and oncology pipeline on our own, or through acquisitions or in-licensing of early or late-stage products or companies, or through partnerships with pharmaceutical and biotechnology companies, as well as academic institutions and government organizations, may not be successful.

Our business is focused on the development and commercialization of novel therapies that significantly improve the lives of patients with hematologic disorders and cancer. In this regard, we continue to pursue internal drug discovery efforts or partnerships with pharmaceutical and biotech companies, as well as academic institutions and government organizations, with the goal of identifying new product candidates to advance into clinical trials. Our discovery efforts to identify new product candidates require substantial technical, financial and human resources. These discovery efforts may initially show promise in identifying potential product candidates, yet ultimately fail to yield product candidates for clinical development for a number of reasons. For example, potential product candidates may, on later stage clinical trial, be shown to have inadequate efficacy, harmful side effects, suboptimal pharmaceutical profiles or other characteristics suggesting that they are unlikely to be commercially viable products.

Apart from our discovery efforts, we continue to seek to broaden and diversify our product portfolio through acquisition or in-licensing of a product. This strategy is dependent on our ability to successfully identify and acquire or in-license relevant product candidates. In July 2022, we entered into a license and transition services agreement with Forma for an exclusive license to develop, manufacture and commercialize olutasidenib, a proprietary inhibitor of mIDH1, for any uses worldwide, including for the treatment of AML and other malignancies. On December 1, 2022, the FDA approved REZLIDHIA capsules for the treatment of adult patients with R/R AML with a susceptible IDH1 mutations as detected by an FDA-approved test. REZLIDHIA is our second commercial product and we believe is highly synergistic with our existing hematology-oncology focused commercial and medical affairs infrastructure. Further, in February 2024, we entered into an Asset Purchase Agreement with Blueprint to purchase certain assets comprising the right to research, develop, manufacture and commercialize GAVRETO (pralsetinib), Blueprint's proprietary RET inhibitor of tyrosine kinase for the treatment of metastatic RET fusion-positive NSCLC and advanced

thyroid cancer, in the US. Simultaneously and in connection with entering into the Asset Purchase Agreement, we also entered into certain supporting agreements with Blueprint, including a customary transition agreement, pursuant to which, during a transition period, Blueprint will transition regulatory and distribution responsibility for pralsetinib to us. We expect to complete the transition of the asset and start recognizing product sales in July of 2024. The in-licensing and acquisition of a product is a highly competitive area, and many other companies are pursuing the same or similar product candidates to those that we may consider attractive. In particular, larger companies with more well-established and diverse revenue streams may have a competitive advantage over us due to their size, financial resources and more extensive clinical development and commercialization capabilities. Furthermore, companies that perceive us to be a competitor may be unwilling to assign or license rights to us. The success of this strategy depends partly upon our ability to identify, select and acquire or in-license promising product candidates and technologies. The process of proposing, negotiating and implementing a license or acquisition of a product candidate is lengthy and complex, and we may be unable to in-license or acquire the rights to any such products, product candidates or technologies from third parties for

[Table of Contents](#)

several reasons. We may also be unable to in-license or acquire additional relevant product candidates on acceptable terms. Further, even if we identify acquisition or in-licensing targets, we may not be able to complete the transactions or we may determine after due diligence investigation not to pursue identified targets. Even if we succeed in our efforts to obtain rights to suitable product candidates, the success of our investments in these areas, our investment strategy will remain subject to the inherent risks associated with the development and commercialization of the product, and with the competitive business environment in which we operate

In addition, acquisitions and in-licensing may entail numerous operational, financial and legal risks, including:

- potential failure of the due diligence process to identify significant problems, liabilities or other shortcomings or challenges of an acquired or licensed product candidate or technology, including problems, liabilities or other shortcomings or challenges with respect to intellectual property, product quality, partner disputes or issues and other legal and financial contingencies and known and unknown liabilities;
- inability to integrate the target company or in-licensed asset successfully into our existing business, inability to maintain the key business relationships of the target;
- in an in-licensing or an asset acquisition of a product that is commercially available in the market, we may not be able to successfully transition the existing patients who are dependent to the acquired or in-licensed product, or successfully enter into a reimbursement coverage contracts that the existing patients were previously dependent into, or successfully enter into a contract with contract manufacturers to continue the production of the in-licensed or acquired product;
- assumption of unknown or contingent liabilities or incurrence of unanticipated expenses;
- exposure to known and unknown liabilities, including possible intellectual property infringement claims, violations of laws, tax liabilities and commercial disputes;
- incurrence of substantial debt, dilutive issuances of securities or depletion of cash to pay for acquisitions;
- incurrence of large one-time expenses and acquiring intangible assets that could result in significant future amortization expense and significant write-offs;
- higher than expected acquisition and integration costs; and
- inability to maintain uniform standards, controls, procedures and policies;

There is a high risk that drug discovery and development efforts might not generate successful product candidates.

At the present time, a significant portion of our operations are focused on various stages of drug identification and development. We currently have various product candidates in the clinical testing stage, stage and may further pursue to expand our clinical testing efforts. In our industry, it is statistically unlikely that the limited number of compounds that we have identified as potential product candidates will actually lead to successful product development efforts. We have invested a significant portion of our efforts and financial resources into the development of

fostamatinib clinical development. Our ability to generate product revenue, which will not occur until after regulatory approval, if ever, will depend on the successful development, regulatory approval and eventual commercialization of one of our product candidates.

Our compounds in clinical trials and our future leads for potential drug compounds are subject to the risks and failures inherent in the development of pharmaceutical products. These risks include, but are not limited to, the inherent difficulty in selecting the right drug and drug target and avoiding unwanted side effects, as well as unanticipated problems relating to product development, testing, enrollment, obtaining regulatory approvals, obtaining and maintaining reimbursement in national markets and positive recommendation from HTA bodies, maintaining regulatory compliance, manufacturing, competition and costs and expenses that may exceed current estimates. In future clinical

49

[Table of Contents](#)

trials, we or our partners may discover additional side effects and/or a higher frequency of side effects than those observed in previously completed clinical trials. The results of preliminary and mid-stage clinical trials do not necessarily predict clinical or commercial success, and larger later-stage clinical trials may fail to confirm the results observed in the previous clinical trials. Similarly, a clinical trial may show that a product candidate is safe and effective for certain patient populations in a particular indication, but other clinical trials may fail to confirm those results in a

49

[Table of Contents](#)

subset of that population or in a different patient population, which may limit the potential market for that product candidate. With respect to our own compounds in development, we have established anticipated timelines with respect to the initiation of clinical trials based on existing knowledge of the compounds. However, we cannot provide assurance that we will meet any of these timelines for clinical development. Additionally, the initial results of a completed earlier clinical trial of a product candidate do not necessarily predict final results and the results may not be repeated in later clinical trials.

Because of the uncertainty of whether the accumulated preclinical evidence (PK, pharmacodynamic, safety and/or other factors) or early clinical results will be observed in later clinical trials, we can make no assurances regarding the likely results from our future clinical trials or the impact of those results on our business. For example, we initiated our FORWARD study, a Phase 3 pivotal trial of fostamatinib in patients with wAIHA in March 2019, completed the enrollment in November 2021 and completed the treatment period for the last patient under the trial in April 2022. In June 2022, we announced top-line efficacy and safety data results of our FORWARD study, and the results of the trial did not demonstrate statistical significance in the primary efficacy endpoint of durable hemoglobin response in the overall study population. We conducted an in-depth analysis of these data to better understand differences in patient characteristics and outcomes and submitted these findings to the FDA. In October 2022, we announced that we received guidance from the FDA's review of these findings. Based on the result of the trial and the guidance from the FDA, we did not file an sNDA for this indication. Further, we may experience errors in the analysis of our clinical trial results. For example, we conducted our Phase 3 clinical trial to evaluate safety and efficacy of fostamatinib in hospitalized COVID-19 patients, which we launched in November 2020 and completed the enrollment on this trial in July 2022. We previously announced in November 2022 the top-line results did not meet statistical significance in the primary efficacy endpoint. Upon further analysis, we discovered an error by the **biostatistical** CRO in the application of a statistical stratification factor. After correcting for this statistical error, the primary endpoint of the study was met. However, given the end of the federal COVID-19 PHE in May 2023, and based on feedback from the FDA, **US Department of Defense DOD** and other advisors regarding the program's regulatory requirements, costs, timeline and potential for success, we decided not to submit an EUA or sNDA.

If the results of our clinical trials fail to meet the primary efficacy endpoints, or otherwise do not ultimately meet the requirements for an NDA approval by the FDA, the commercial prospects of our business may be harmed, our ability to generate product revenues may be delayed or eliminated or we may be forced to undertake other strategic alternatives that are in our shareholders' best interests, including cost reduction measures. If we are unable to obtain adequate financing or engage in a strategic transaction on commercially reasonable terms or at all, we may be required to implement further cost reduction strategies which could significantly impact activities related to our commercial efforts and/or research and development of our future product candidates, and could significantly harm our business, financial condition and results of operations. In addition, these cost reduction strategies could cause us to further curtail our operations or take other actions that would adversely impact our shareholders.

We are subject to federal and state healthcare fraud and abuse laws, false claims laws and other federal and state healthcare laws, and the failure to comply with such laws could result in substantial penalties. Our employees, independent contractors, consultants, principal investigators, CROs, commercial partners and vendors may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements.

Our business operations and current and future arrangements with investigators, healthcare professionals, consultants, third-party payors and customers, may expose us to broadly applicable federal, state and foreign fraud and abuse and other healthcare laws and regulations including anti-kickback and false claims laws, data privacy and security laws, and transparency reporting laws. These laws may constrain the business or financial arrangements and relationships through which we conduct our operations, including how we research, market, sell and distribute any product for which we have obtained regulatory approval, or for which we obtain regulatory approval in the future. In particular, the promotion, sales and marketing of healthcare items and services, as well as certain business arrangements in the healthcare industry, are subject to extensive laws and regulations intended to prevent fraud, misconduct, bribery kickbacks, self-dealing and other abusive or inappropriate practices. These laws and regulations may restrict or prohibit

50

[Table of Contents](#)

a wide range of pricing, discounting, marketing and promotion, including promoting off-label uses of our products, certain commission compensation, certain customer incentive programs, certain patient support offerings, and other business arrangements generally. Activities subject to these laws also involve the improper use or misrepresentation of information obtained in the course of patient recruitment for clinical trials, creating fraudulent data in our preclinical studies or clinical trials or illegal misappropriation of drug product, which could result in regulatory sanctions and cause

50

[Table of Contents](#)

serious harm to our reputation. See "Part I, Item 1, Business"Business – Government Regulation – Healthcare and Privacy Law and Regulation and Healthcare Reform" contained in Part I, Item 1 of our Annual Report on Form 10-K for the year ended December 31, 2022 December 31, 2023, for more information on the healthcare laws and regulations that may affect our ability to operate.

We are also exposed to the risk of fraud, misconduct or other illegal activity by our employees, independent contractors, consultants, principal investigators, CROs, commercial partners and vendors. Misconduct by these parties could include intentional, reckless and/or negligent conduct that fails to: comply with the laws of the FDA and other similar foreign regulatory bodies; provide true, complete and accurate information to the FDA and other similar foreign regulatory bodies; comply with manufacturing standards we have established; comply with federal and state data privacy, security, fraud and abuse and other healthcare laws and regulations in the US and similar foreign fraudulent misconduct laws; or report financial information or data accurately or to disclose unauthorized activities to us. It is not always possible to

identify and deter employee misconduct, and the precautions we take to detect and prevent inappropriate conduct may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws or regulations.

We are also subject to the risk that a person or government could allege such fraud or other misconduct, even if none occurred. Efforts to ensure that our business arrangements will comply with applicable healthcare laws and regulations will involve substantial costs. It is possible that governmental and enforcement authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law interpreting applicable fraud and abuse or other healthcare laws and regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, including the imposition of significant civil, criminal and administrative penalties, damages, disgorgement, monetary fines, imprisonment, additional reporting obligations and oversight if we become subject to a corporate integrity agreement or other agreement to resolve allegations of non-compliance with these laws, possible exclusion from participation in Medicare, Medicaid and other federal healthcare programs, contractual damages, reputational harm, diminished profits and future earnings, and curtailment or restructuring of our operations, any of which could adversely affect our ability to operate our business and our results of operations.

We are subject to stringent and evolving privacy and information security laws, regulations, rules, policies, and contractual obligations, and changes in such laws, regulations, rules, policies, contractual obligations and our actual or perceived failure to comply with such requirements could subject us to significant investigations, fines, penalties and claims, any of which may have a material adverse effect on our business, financial condition, results of operations or prospects.*

We are subject to, or affected by, various federal, state and foreign laws, rules, directives, and regulations, as well as regulatory guidance, policies and contractual obligations relating to privacy and information security, governing the acquisition, collection, access, use, disclosure, processing, modification, retention, storage, transfer, destruction, protection, and security (collectively, "processing") of personal information and other sensitive information about individuals. The global privacy and information security landscape is evolving rapidly, and implementation standards and enforcement practices are likely to continue to develop for the foreseeable future and may result in conflicting or inconsistent compliance obligations. Legislators and regulators are increasingly adopting or amending privacy and information security laws, rules, directives, and regulations that may create uncertainty in our business, affect our or our collaborators', service providers' and contractors' ability to operate in certain jurisdictions or to process personal information, transfer data internationally, necessitate the acceptance of more onerous obligations in our contracts, result in enforcement actions, litigation or other liability or impose additional costs on us. The cost of compliance with these laws, regulations and standards is high and is likely to increase in the future. Any failure or perceived failure by us or our collaborators, service providers and contractors to comply with federal, state or foreign laws or regulations, our internal policies and procedures or our contracts governing the processing of personal information could result in negative publicity, diversion of management time and effort and proceedings against us by governmental entities or others. In many jurisdictions, enforcement actions, litigation, and other consequences for noncompliance with privacy and

51

[Table of Contents](#)

information security laws and regulations are rising. Compliance with applicable privacy and information security laws and regulations, as well as regulatory guidance, policies and contractual obligations, is a rigorous and time-intensive process, and we may be required to put in place additional mechanisms to ensure compliance with the new privacy and information security requirements. If we fail to comply with any such obligations, we may face significant investigations, fines, penalties and claims that could materially and adversely affect our business, financial condition,

51

[Table of Contents](#)

results of operations, ability to process personal information and income from certain business initiatives.

In the US, these obligations include various federal, state, and local statutes, rules, and regulations relating to privacy and data security. The Federal Trade Commission (FTC) has authority under Section 5 of the FTC Act to regulate unfair or deceptive practices, and has used this authority to initiate enforcement actions against companies that implement inadequate controls around privacy and information security in violation of their externally facing policies. The FTC has recently brought several cases alleging violations of Section 5 of the FTC Act with respect to health information, and has proposed rulemaking on privacy and data security, including with respect to the Health Breach Notification Rule. The US federal government has also enacted statutes to address privacy and information security issues impacting particular industries or activities, including the following laws and regulations: the Electronic Communications Privacy Act, the Computer Fraud and Abuse Act, the Health Insurance Portability and Accountability Act, the Health Information Technology for Economic and Clinical Health Act, the Telephone Consumer Protection Act, the CAN-SPAM Act, and other laws and regulations. In addition, state legislatures have enacted statutes to address privacy and information security issues, including the California Consumer Privacy Act of 2018 (the CCPA), and similar state laws such as Virginia's Consumer Data Protection Act and the Colorado Privacy Act. For example, the CCPA, as amended by the California Privacy Rights Act (CPRA) in 2020, establishes a privacy framework applicable to for-profit entities that are doing business in California, including an expansive definition of personal information and data privacy rights for California residents, and authorizes potentially severe statutory damages and creates a private right of action for certain data security breaches. The CCPA also requires businesses subject to the law to provide new disclosures to California residents and to provide them with expanded rights with respect to their personal information, including the right to opt out of the sale of such information. Moreover, the CPRA, among other things, impose new requirements relating to data minimization and correction, and gives California residents additional rights over their personal information, including the right to opt-out of the use of their personal information in online behavioral advertising and to opt-out of certain types of consumer profiling. The CPRA also provides for penalties for CPRA violations concerning California residents under the age of 16, and establishes a new California Privacy Protection Agency to implement and enforce the law. Although there are limited exemptions for clinical trial and other research-related data under the CCPA, the CCPA and other similar laws could impact our business depending on how it will be interpreted by the new California Privacy Protection Agency. As we expand our operations, the CCPA may increase our compliance costs and potential liability. In addition, California voters approved the California Privacy Rights Act of 2020 (CPRA), which became effective on January 1, 2023. The CPRA, among other things, gives California residents the ability to limit the use of their sensitive information, opt out of certain types of profiling and automated processing activities, provides for penalties for CPRA violations concerning California residents under the age of 16, and establishes a new California Privacy Protection Agency to implement and enforce the law. Additionally, Colorado, Connecticut, Utah and Virginia passed comprehensive state privacy laws, which became effective on July 1, 2023, July 1, 2023, December 31, 2023, and January 1, 2023, respectively. Likewise, Utah passed a comprehensive privacy law, which will go into effect on December 31, 2023. Several states have also passed similar privacy laws that will become effective in 2024 or later, including Delaware, Indiana, Iowa, Montana, New Jersey, Oregon, Tennessee and Texas. Multiple other states and the federal government are considering enacting similar legislation. Other states have passed or amended existing state privacy laws to impose enhanced privacy and cybersecurity obligations for consumer health data, such as, the Washington My Health My Data Act and Nevada's Consumer Health Data Privacy Law. Many states also have in place data security laws requiring companies to maintain certain safeguards with respect to the processing of personal information, and all states require companies to notify individuals or government regulators in the event of a data breach impacting such information. New privacy laws add additional complexity, requirements, restrictions and potential legal risk. Accordingly, compliance programs may require additional investment in resources, and could impact availability of previously useful data.

Internationally, our operations abroad may also be subject to increased scrutiny or attention from foreign data protection authorities. For example, our clinical trial programs and research collaborations outside the US may implicate foreign data protection laws, including those in the European Economic Area, Switzerland, and/or the UK (collectively, Europe). Many jurisdictions have established or are in the process of establishing privacy and data security legal frameworks with which we, our collaborators, service providers, including our CROs, and contractors must comply. For example, European in the EU, the collection, use, disclosure, transfer and other processing of personal data protection laws, including, without limitation, the General Data Protection Regulation (the EU GDPR), impose strict requirements for processing personal information (i.e., data which identifies an individual or from which an individual is identifiable) is governed by the EU General Data Protection Regulation 2016/679 (the EU GDPR), including clinical trial data which came into direct effect in all EU Member States on and grant individuals various data protection rights (e.g., the right to erasure of personal information) from May 25, 2018. In turn, The UK has implemented the EU GDPR as the UK GDPR which sits alongside the UK Data Protection Act 2018 (the UK GDPR, together with the EU GDPR, the GDPR). The GDPR has direct effect where an entity is established in the European Economic Area (EEA) or the UK (as applicable) and similar laws increase our obligations with respect has extraterritorial effect, including where an entity established outside of the EEA or the UK processes personal data in relation to clinical trials conducted offering goods or services to individuals in Europe by expanding the definition of personal information to also include coded data and requiring (i) changes to informed consent practices and more detailed notices for clinical trial participants and EEA and/or the UK or monitoring their behavior.

[Table of Contents](#)

investigators; (ii) consideration of data protection as any new products or services are developed, The GDPR imposes obligations on controllers, including, to limit the amount of personal information processed; and (iii) implementation of appropriate technical and organizational measures to safeguard personal information and to report certain breaches of personal information to the relevant supervisory authority without undue delay (for the EU GDPR no later than 72 hours where feasible). In the event of non-compliance, the EU GDPR provides for robust regulatory enforcement and fines of up to €20 million or 4% of the annual global revenue, whichever is greater. among others:

- accountability and transparency requirements, requiring controllers to demonstrate and record compliance with the GDPR and to provide more detailed information to data subjects regarding processing of their personal data;
- requirements to process personal data lawfully including specific requirements for obtaining valid consent where consent is the lawful basis for processing;
- obligations to consider data protection when any new products or services are developed and designed (including e.g., to limit the amount of personal data processed);
- obligations to comply with data protection rights of data subjects including a right: (i) of access to, erasure of, or rectification of personal data, (ii) to restriction of processing or to withdraw consent to processing, and (iii) to object to processing or to ask for a copy of personal data to be provided to a third party; and
- an obligation to report personal data breaches to: (i) the data supervisory authority without undue delay (and no later than 72 hours after discovering the personal data breach, where feasible), unless the personal data breach is unlikely to result in a risk to the data subjects' rights and freedoms; and (ii) to affected data subjects, where the personal data breach is likely to result in a high risk to their rights and freedoms.

In addition, the EU GDPR confers a private right of action on data subjects and consumer associations to lodge complaints with supervisory authorities, seek judicial remedies and obtain compensation for damages resulting from violations of prohibits the EU GDPR.

European data protection laws, including the EU GDPR, generally also restrict the international transfer of personal information data from Europe the EEA to jurisdictions that the US and most other countries that are European Commission does not recognize as having "adequate" 'adequate' data protection laws unless a data transfer mechanism has been put in place or a derogation under the parties to the transfer have implemented specific safeguards to protect the transferred personal information. One of the primary safeguards allowing US companies to import personal information from Europe was certification to the EU-US Privacy Shield and Swiss-US Privacy Shield frameworks administered by the US Department of Commerce. However, EU GDPR can be relied on. In July 2020, the Court of Justice of the European Union EU (CJEU) issued a decision in July 2020 its Schrems II judgement limited how organizations could lawfully transfer personal data from the EEA to the US by invalidating the EU-US Privacy Shield framework as a data transfer mechanism (Schrems II) for purposes of international transfers and imposing further restrictions on the use of standard contractual clauses (SCCs). The SCCs were subsequently updated by the EC and these now place (EU SCCs), including a contractual obligation on the parties, requirement for companies to carry out a transfer privacy impact assessment which, (TIA). A TIA, among other things, assesses laws governing access to personal information data in the recipient country and considers whether supplementary measures that provide privacy protections additional to those provided under the EU SCCs will need to be implemented to ensure an essentially equivalent 'essentially equivalent' level of data protection to that afforded in Europe. Following that decision, the Swiss Federal Data Protection and Information Commissioner (FDPIC) took a similar view and considered that data transfers based on the Swiss-US Privacy Shield framework were no longer lawful. EEA.

On October 7, 2022, the US President Biden introduced an Executive Order to facilitate a new Trans-Atlantic Data Privacy Framework (DPF) and on July 10, 2023, the EC also European Commission adopted its Final Implementing Decision granting the US adequacy (Adequacy Decision) with respect to companies that subscribe for EU-US transfers of personal data for entities self-certified to the DPF. Entities relying on EU SCCs for transfers to the US are also able to rely on the analysis in the EC's Adequacy Decision as support for their transfer impact assessments required by Schrems II. Given TIA regarding the above, any transfers by us or equivalence of US national security safeguards and redress. This may have implications for our third-party vendors, collaborators or others of personal information from Europe to the US or elsewhere may not comply with European data protection laws, may increase our exposure to European data protection laws' heightened sanctions for cross-border data transfer restrictions may restrict our clinical trial activities in Europe flows and has and may limit our ability to collaborate with CROs, service providers, contractors and other companies subject to European data protection laws. Loss of our ability to

transfer personal information from Europe may also require us to increase our data processing capabilities in those jurisdictions at significant expense.

Following the UK's departure from the EU (Brexit), the EU GDPR's data protection obligations continue to apply to the UK in substantially unvaried form under the so-called "UK GDPR" (i.e., the EU GDPR as it continues to form part of law in the UK by virtue of section 3 of the European Union (Withdrawal) Act 2018, as amended (including by the various Data Protection, Privacy and Electronic Communications (Amendments etc.) (EU Exit) Regulations)). future result in increased compliance costs.

The UK GDPR exists alongside also imposes similar restrictions on transfers of personal data from the UK Data Protection Act 2018 that implements certain derogations in the UK GDPR into UK law. Under the UK GDPR, companies not established in the UK but that process personal information either in relation to the offering of goods or services to individuals in the UK, or to monitor their behavior will be subject to the UK GDPR, the requirements of which are (at this time) largely aligned with those under the EU GDPR, and as such, may lead to similar compliance and operational costs with potential fines of up to £17.5 million or 4% of global turnover. As a result, we are potentially exposed to two parallel data protection regimes, each of which authorizes fines and the potential for divergent enforcement actions. It should also be noted jurisdictions that the UK Government does not consider adequate, including the US. The UK Government has published its own form of the EU SCCs, known as the International Data Transfer Agreement (IDTA) and an International Data Transfer Addendum (UK Addendum) to the new EU SCCs. The UK Information Commissioner's Office (ICO) has also published its version of the transfer impact assessment TIA and revised guidance on international transfers, although entities may choose to adopt either the EU or UK style transfer impact assessment. In terms of international data transfers between TIA. Further, on September 21, 2023, the UK Secretary of State for Science, Innovation and US, on September 21, 2023 Technology established a UK-US data bridge (i.e., a UK adequacy decision) and adopted UK regulations were laid to implement the UK-US data bridge (UK Adequacy Regulations). The UK Adequacy Regulations have now been passed in the UK Parliament, to give effect to and personal data may be transferred from the UK Extension under the UK-US data bridge through the UK extension to the DPF, – otherwise known as from October 12, 2023, to organizations self-certified under the "UK-U.S. Data Bridge." DPF.

The regulations took effect on October 12, 2023 GDPR imposes fines for serious breaches of up to the higher of 4% of the organization's annual worldwide turnover or €20m (under the EU GDPR) or £17.5m (under the UK GDPR). The UK-US GDPR identifies a list of points to consider when determining the level of fines for data supervisory authorities to impose (including the nature, gravity and duration of the infringement). Data Bridge – subjects also have a right to compensation, as a result of an organization's breach of the GDPR which is the UK's equivalent to the EC's Adequacy Decision - will enable companies in the UK to lawfully transfer personal information to participating organizations in the US without the need to implement additional safeguards. has affected them, for financial or non-financial losses (e.g., distress).

[Table of Contents](#)

Privacy and data protection compliance has and may in the future require substantial amendments to our procedures and policies and the changes could adversely impact our business by increasing operational and compliance costs or impact business practices. Further, there is a risk that the amended policies and procedures will not be implemented correctly or that individuals within the business will not be fully compliant with the new procedures. If there are breaches of these measures, we could face significant litigation, government investigations, administrative and monetary sanctions as well as reputational damage which may have a material adverse effect on our operations, financial condition and prospects. There is a risk that we could be impacted by a cybersecurity incident that results in loss or unauthorized disclosure of personal data, potentially resulting in us facing harms similar to those described above.

Additionally, other countries outside of Europe have enacted or are considering enacting similar cross-border data transfer restrictions and laws requiring local data residency, with strict requirements and limitations for processing personal information, which could increase the cost and complexity of delivering our services and operating our business. For example, Brazil enacted the General Data Protection Law, New Zealand enacted the New Zealand Privacy Act, China released its Personal Information Protection Law, which went into effect November 1, 2021, and Canada introduced the Digital Charter Implementation Act. As with the EU GDPR, these laws are broad and may increase our compliance burdens, including by mandating potentially burdensome documentation requirements and granting certain rights to individuals to control how we collect, use, disclose, retain, and process personal information about them.

We publish privacy policies and other documentation regarding our collection, processing, use and disclosure of personal information and/or other confidential information. Although we endeavor to comply with our published policies and other documentation, we may at times fail to do so or may be perceived to have failed to do so. Moreover, despite our efforts, we may not be successful in achieving compliance if our employees, collaborators, contractors, service providers or vendors fail to act in accordance with our published policies and documentation. Such failures can subject us to potential foreign, local, state and federal action if they are found to be deceptive, unfair, or misrepresentative of our actual practices. Moreover, trial participants or research subjects about whom we or our partners obtain information, as well as the providers who share this information with us, may contractually limit our ability to use and disclose the information or exercise their right to do so under applicable privacy legislation. Claims that we have violated individuals' privacy rights or failed to comply with data protection laws or applicable privacy policies and documentation, even if we are not found liable, could be expensive and time-consuming to defend and could result in adverse publicity that could harm our business.

In addition to data privacy requirements, cybersecurity requirements are laid down in various laws in the EU and the UK, the key ones being: (i) the GDPR (as discussed in detail above), which requires controllers and processors to implement appropriate technical and organizational measures to safeguard personal data to a level of security appropriate to the data protection risk; and (ii) the UK Network and Information Systems Regulation 2018 (NIS Regulations), and the the EU Network and Information Systems Security 1 Directive (NISD1) as implemented into EU Member State law (and as updated by the EU Network and Information Systems Security 2 Directive (NISD2)).

The GDPR does not provide for a specific set of cybersecurity requirements or measures to be implemented, but rather requires a controller or processor to implement appropriate cyber and data security measures in accordance with the then-current risk, the state of the art, the costs of implementation and the nature, scope, context and purposes of the processing. The GDPR however does explicitly require that controllers notify personal data breaches, within the meaning of the GDPR, without undue delay and in any event within 72 hours after becoming aware of it, to the relevant data protection supervisory authority, unless the breach is unlikely to result in a risk to the rights and freedoms of individuals. In addition, controllers are required to notify the individuals concerned of any personal data breach, without undue delay, when the personal data breach is likely to result in a high risk to the rights and freedoms of individuals. Processors are required to notify the controller without undue delay after becoming aware of a personal data breach.

In the UK, the NIS Regulations apply to 'operators of essential services' (OES) and 'relevant digital service providers' (RDSP) and it was announced in January 2022, that the NIS Regulations will be updated to also cover 'managed service providers' (MSP) and potentially other digital service providers. The NIS Regulations require that appropriate and proportionate technical and organizational measures are implemented to manage the risk of network and information systems, and impose requirements related to incident handling and notification in relation to incidents with significant disruptive effect. Under the NIS Regulations, the UK's data protection supervisory authority, the Information Commissioner's Office, may issue fines of up to £17 million and take other action following non-compliance.

[Table of Contents](#)

In the EU, the NISD1 applies to 'operators of essential services' (OES) and 'digital service providers' (DSP) and an updated version of NISD1 has been adopted and entered into force on January 17, 2023, called NISD2. The NISD2 will take full effect following implementation into national EU Member State law (i.e., by October 17, 2024). Under the NISD1, OESs and DSPs are required to implement appropriate and proportionate technical and organizational measures to manage the risk of network and information systems, and adhere to incident handling and notification requirements regarding incidents with significant disruptive effect. Importantly, under the NISD2, more stringent cybersecurity and incident reporting requirements are imposed on 'essential' and 'important' entities, which include ICT managed service providers (MSP), cloud service providers as well as entities carrying out research and development activities of medicinal products, and certain specific medical device manufacturers. Our entities may be in scope of the NISD2 where they qualify as a MSP, cloud provider, R&D entity and/or medical device manufacturer within the meaning of NISD2 and offer those services in the EU.

The NISD2 empowers the EU Member States to define all rules regarding penalties applicable to infringements, provided that they are effective, proportionate, and dissuasive. NISD2 states that any maximum fine which national implementing law provides for should at least be set at €10 million or 2% of total worldwide turnover, whichever is higher, where essential entities are concerned. Other sanctions may include (i)

a temporary suspension to provide services in the EU (by suspending relevant authorizations/certifications); (ii) an order to make public certain elements of the infringement and/or inform customers; and (iii) injunctions to immediately cease infringing conduct. Importantly, NISD2 also provides that senior members of staff can be held personally liable, and face administrative fines or be temporarily suspended from exercising managerial functions at the legal representative or chief executive officer level.

In addition, the EU Critical Entities Resilience Directive (CER) entered into force on January 17, 2023 and will take full effect following implementation into national EU Member State law (i.e., by October 17, 2024 – coinciding with the NISD2). The CER is aimed at strengthening the resilience of 'critical infrastructure' against specific threats including cyber incidents, natural hazards, terrorist attacks, insider threats, and sabotage. The scope of CER includes entities designated as 'critical' under CER and includes (among other things) the health sector and the manufacturers of medical devices as 'essential services.' The CER imposes cybersecurity and resilience requirements in particular in relation to incidents with so-called 'significant disruptive effects' – which are incidents that are able to significantly impact the continuation of the critical infrastructure service offering in the EU. Requirements include to: (i) identify relevant risks that may significantly disrupt the provision of essential services (i.e., pursuant to a risk assessment); (ii) take appropriate and proportionate technical, security and organizational measures to ensure resilience (i.e., based on the outcome of the risk assessment); and (iii) notify disruptive incidents to the competent authorities within 24 hours after becoming aware of an incident. The CER is enforceable on a national EU Member State level by the competent authorities, and allows EU Member States to set penalties as long as they are effective, proportionate, and dissuasive. Our entities may be in scope of the CER where they qualify as critical entities within the meaning of CER.

In the EU, a number of new laws related to digital data and AI have also recently entered into force, are expected to enter into force in the foreseeable future, or have been proposed and are being considered. We are still assessing the scope of application, impact, and risk of these recent EU laws on our business, and will continue to assess this moving forward, including for example: (i) the EU's Data Act – expected to come into force in the first quarter to second quarter of 2024 – which seeks to, among other things regulate the use of, and access to, data generated through connected (or Internet-of-Things) devices and introduces a new means for public sector bodies to access, use and re-use private sector data; and (ii) the proposed European Health Data Space Regulation (EHDS) – expected to be agreed in the third quarter of 2024 – which seeks to, among other things, provide individuals with more control over their electronic health data (EHD), enable cross-border sharing of EHD between national EU healthcare systems and facilitate the sharing of EHD for secondary research purposes.

The EU has also developed a standalone law to govern the offering and use of AI systems in the EU (the AI Act) which reached political agreement on December 8, 2023 and is expected to be adopted and enter into force during the first quarter to second quarter of 2024. The AI Act imposes regulatory requirements onto AI system providers, importers, distributors, and users of AI systems, in accordance with the level of risk involved with the AI system ("unacceptable", "high", "limited", and "minimal" risk). Unacceptable-risk AI systems are banned from being offered and used in the EU, and high-risk AI systems (which include AI used as part of medical devices in certain instances) are subject to a set of regulatory requirements under the AI Act including to establish quality and post-marketing monitoring and risk assessment systems, requirements related to the training of AI systems and training data, and requirements

[Table of Contents](#)

related to human oversight. Limited-risk AI systems are subject mainly to transparency requirements only and minimal-risk AI systems are not subject to obligations under the AI Act. In the most recent iteration of the AI Act's text, general-purpose AI systems have also been made subject to a number of requirements – mostly akin to the requirements that apply to high-risk AI systems under the AI Act.

Currently, the AI Act is expected to enter into application (i.e., be enforceable) in a gradual manner – depending on the regulatory requirement in question, and ranging anywhere from 6 to 36 months following adoption and entry into force of the AI Act (i.e., between fourth quarter of 2024 to first quarter of 2027). Non-compliance with the AI Act may be subject to regulatory fines of up to 7% of annual worldwide turnover. In parallel, the EU has proposed revisions to the EU Product Liability Directive and has introduced a new EU AI Liability Directive to facilitate claims for damages brought by EU users of AI systems.

The UK has adopted a "soft law" approach to AI regulation meaning it has not adopted formal legislation to regulate AI but has adopted soft law guidelines in the form of a White Paper.

Further, many jurisdictions impose mandatory clinical trial information obligations on sponsors. In the EU, such obligations arise under the Transparency Regulation No 1049/ 2001, EMA Policy 0043, EMA Policy 0070 and the Clinical Trials Regulation No 536/2014, all of which impose on sponsors the obligation to make publicly available certain information stemming from clinical studies. In the EU, the transparency framework provides EU-based parties the right to submit an access to documents request to the EMA for information included in the MAA dossier for approved medicinal products. Only very limited information is exempted from disclosure, i.e., commercially confidential information (which is construed increasingly narrowly) and protected personal data. It is possible for competitors to access and use this data in their own research and development programs anywhere in the world, once this data is in the public domain.

On May 3, 2022, the EC published a proposal for a regulation on the European Health Data Space (EHDS), which aims to further enable exchange of electronic health data both for primary use (among national EU healthcare systems for patient care) and secondary use (among private companies and regulators to enable scientific research). Whilst the regulation is currently under discussions among the EU legislators, the text is expected to be finalized by the end of 2023 and for the EHDS to become applicable in 2025. This will impose new obligations, but also create opportunities, for entities engaged in health-related research to share and access health data on a scale much larger than what is foreseen under current applicable transparency provisions.

Our business could be materially and adversely affected by pandemics as a result of their potential impacts on our sales force and commercialization efforts, supply chain, regulatory, clinical development and corporate development activities and other business operations, in addition to the impact of a global economic slowdown.*

Pandemics may result in extended travel and other restrictions in order to reduce the spread of diseases. Government measures taken in response to pandemics could have a significant impact, both direct and indirect, on our business and commerce, as significant reductions in business related activities may occur, supply chains may be disrupted, and manufacturing and clinical development activities may be curtailed or suspended.

For example, due to the effects of the COVID-19 pandemic, we have observed reduced patient-doctor interactions and our representatives have had fewer visits with health care providers, which has negatively affected our product sales and may continue to negatively affect our product sales in the future. Physicians with practices severely impacted by the COVID-19 pandemic, and who currently prescribe our products, may eventually decide to close their independent practices and join a larger medical organization with a practice that does not prescribe our products. Additionally, commercial-related activities, such as our marketing programs, speaker bureaus, and market access initiatives were conducted virtually, delayed or cancelled as a result of the COVID-19 pandemic. We had to deploy

[Table of Contents](#)

resources to enable our field-based employees to continue to engage with health care providers in hybrid virtual and in-person interactions.

With respect to clinical development, in response to the COVID-19 pandemic, we had taken, and may continue to take, measures to implement remote and virtual approaches, including remote patient monitoring where possible and working with our investigators for appropriate care of these patients in a safe manner. Due to the effects of COVID-19 pandemic, we had experienced a number of our clinical trial investigators either paused, postponed or delayed new patient enrollment and restricted site visits of existing patients enrolled. In the event that a global pandemic occurs in the future, we may need to make decisions on a country-by-country basis to minimize risk to the patients and clinical trial sites. We may also rely heavily on our clinical trial investigators to inform us of the best course of action with respect to resuming enrollment/screening, considering the ability of sites to ensure patient safety or data integrity. We experienced slower than anticipated enrollment in some of our clinical trials due to adverse effects of COVID-19 pandemic, and in the future, we may experience adverse impact of a global pandemic on our clinical trials, including the timing thereof, or our ability to continue to treat patients enrolled in our trials, enroll and assess new patients, supply study drugs and obtain complete data points in accordance with study protocol.

Pandemics may cause significant disruption in the supply chain for our commercial products. We currently rely on third parties to, among other things, manufacture and ship our commercial product, raw materials and product supply for our clinical trials, perform quality testing and supply other goods and services to help manage our commercial activities, our clinical trials and our operations in the ordinary course of business. While we have engaged actively with various elements of our supply chain and distribution channel, including our customers, contract manufacturers, and logistics and transportation provider to meet demand for our products and to remain informed of any

challenges within our supply chain, we may face disruptions to our supply chain and operations, and associated delays in the manufacturing and supply of our products. Such supply disruptions would adversely impact our ability to generate sales of and revenues from our products and our business, financial condition, results of operations and growth prospects could be adversely affected.

Pandemics may affect our collaboration and licensing partners for the commercialization of fostamatinib globally, as well as our ability to advance our various clinical stage programs. We cannot predict the impact of such disruptions on our partners' ability to advance commercialization of fostamatinib in the market and the timing of enrollment and completion of various clinical trials being conducted by our collaboration partners.

Health regulatory agencies globally may experience prolonged disruptions in their operations as a result of pandemics. For example, in response to the COVID-19 pandemic, the FDA delayed inspections and evaluations of certain drug manufacturing facilities and clinical research sites. We cannot predict whether, and when, health regulatory agencies will decide to pause or resume inspections due to pandemics. Any de-prioritization of our clinical trials or delay in regulatory review resulting from such disruptions could materially affect the completion of our clinical trials.

In addition, as seen in the COVID-19 pandemic, pandemics could result in a significant disruption of global financial markets. We could experience an inability to access additional capital or an impact on liquidity, which could in the future negatively affect our capacity for certain corporate development transactions or our ability to make other important, opportunistic investments, or we may not be able to meet the requirements under our Credit Agreement with MidCap. While we expect pandemics to adversely affect our business, financial condition, results of operations and growth prospects in the future periods, the extent of the impact on our ability to generate sales of and revenues from our approved products, our ability to continue to secure new collaborations and support existing collaboration efforts with our partners, our clinical development and regulatory efforts, our corporate development objectives and the value of and market for our common stock, will depend on future circumstances that are highly uncertain and cannot be predicted with confidence at this time, such as the ultimate duration and severity of pandemics, travel restrictions, quarantines, social distancing and business closure requirements in the US and other countries, and the effectiveness of actions taken globally to contain and treat diseases. To the extent pandemics adversely affect our business and results of operations, it may also have the effect of heightening many of the other risks and uncertainties described elsewhere in this "Risk Factors" section.

[Table of Contents](#)

Enhanced governmental and public scrutiny over, or investigations or litigation involving, pharmaceutical manufacturer donations to patient assistance programs may require us to modify our programs and could negatively impact our business practices, harm our reputation, divert the attention of management and increase our expenses.

To help patients afford our products, we have a manufacturer-sponsored patient assistance program that helps financially needy patients in the US access our ~~therapies~~, ~~therapies~~. This type of program has become the subject of enforcement scrutiny in recent years. For example, some pharmaceutical manufacturers have been named in lawsuits challenging the legality of their patient assistance programs under a variety of federal and state laws. In addition, certain state and federal enforcement authorities have pursued investigations and settlements and members of Congress have initiated inquiries about manufacturer-sponsored patient support programs, including, for example, manufacturer-sponsored patient assistance programs, co-payment assistance programs, and manufacturer contributions to independent charitable patient assistance programs. Moreover, the ~~Department of Health and Human Services~~, ~~DHHS~~, Office of the Inspector General continues to publish advisory opinions and other agency guidance on the topic of patient assistance, which reflects the government's continued scrutiny of manufacturer sponsored or supported patient assistance programs. Numerous organizations, including pharmaceutical manufacturers, have been subject to ongoing litigation, enforcement activities and settlements related to their patient support programs and certain of these organizations have entered into, or have otherwise agreed to, significant civil settlements with applicable enforcement authorities. It is possible that future legislation may be proposed that would establish requirements or restrictions with respect to these programs and/or support that would affect pharmaceutical manufacturers.

Our patient assistance program could become the target of similar inquiries, litigation, enforcement, and/or legislative proposals. If we are deemed not to have complied with laws or regulations in the operation of, or our interactions with, these programs, we could be subject to damages, fines, penalties or other criminal, civil or administrative sanctions or enforcement actions. We cannot ensure that our compliance

controls, policies and procedures will be sufficient to protect against acts of our employees, business partners or vendors that may violate the laws or regulations of the jurisdictions in which we operate. A government investigation could negatively impact our business practices, harm our reputation, divert the attention of management and increase our expenses.

[Table of Contents](#)

If manufacturers obtain approval for generic versions of our products, or of products with which we compete, our business may be harmed.*

Under the FDCA, the FDA can approve an ANDA for a generic version of a branded drug without the ANDA applicant undertaking the clinical testing necessary to obtain approval to market a new drug. Generally, in place of such clinical studies, an ANDA applicant usually needs only to submit data demonstrating that its product has the same active ingredient(s), strength, dosage form and route of administration and that it is bioequivalent to the branded product. In September 2019, the FDA published product-specific bioequivalence guidance on fostamatinib disodium to let potential ANDA applicants understand the data FDA would expect to see for approval of a generic version of our products.

The FDCA requires that an applicant for approval of a generic form of a branded drug certify either that its generic product does not infringe any of the patents listed by the owner of the branded drug in the FDA's Approved Drug Products with Therapeutic Equivalence Evaluations (referred to as the "Orange Book") or that those patents are not enforceable. This process is known as a paragraph IV challenge. Upon notice of a paragraph IV challenge, a patent owner has 45 days to bring a patent infringement suit in federal district court against the company seeking ANDA approval of a product covered by one of the owner's patents. If this type of suit is commenced, the FDCA provides a 30-month stay on the FDA's approval of the competitor's application. If the litigation is resolved in favor of the ANDA applicant or the challenged patent expires during the 30-month stay period, the stay is lifted, and the FDA may thereafter approve the application based on the standards for approval of ANDAs. Once an ANDA is approved by the FDA, the generic manufacturer may market and sell the generic form of the branded drug in competition with the branded medicine.

The ANDA process can result in generic competition if the patents at issue are not upheld or if the generic competitor is found not to infringe the owner's patents. If this were to occur with respect to our products or products with which it competes, our business would be harmed. **We have a number of patents listed in the Orange Book, the last of which is expected to expire in July 2032.**

[Table of Contents](#)

In June 2022, we received a notice letter regarding an ANDA submitted to the FDA by Annora, requesting approval to market a generic version of TAVALISSE. The notice letter included a Paragraph IV certification with respect to our US Patent Nos. 7,449,458; 8,263,122; 8,652,492; 8,771,648 and 8,951,504, which are listed in the Orange Book. The notice letter asserts that these patents will not be infringed by Annora's proposed product, are invalid and/or are unenforceable. Annora's notice letter does not provide a Paragraph IV certification against our other patents listed in the Orange Book. On July 25, 2022, we filed a lawsuit in the US District Court for the District of New Jersey against Annora and its affiliates, Hetero Labs Ltd., and Hetero USA, Inc., for infringement of our US patents identified in Annora's Paragraph IV certification. On September 21, 2022, Annora and its affiliates answered and counterclaimed for declaratory judgment of non-infringement and invalidity of the '458, '122, '492, '648, and '504 patents. We filed an answer to Annora's counterclaims on October 12, 2022. Annora served invalidity and non-infringement contentions on December 31, 2022. We filed an answer to Annora's invalidity and non-infringement contentions in March 2023. Litigation continues, **with preparation for a Markman hearing expected early next year and no trial date is currently set.** We intend to vigorously enforce and defend our intellectual property related to TAVALISSE. We cannot be assured that such lawsuit will prevent the

introduction of a generic version of TAVALISSE for any particular length of time, or at all. If an ANDA from Annora or any other generic manufacturer is approved, and a generic version of TAVALISSE is introduced, whether following the expiration of our patents, the invalidation of our patents as a result of any litigation, or the determination that the proposed generic product does not infringe on our patents, our sales of TAVALISSE would be adversely affected. In addition, we cannot predict what additional ANDAs could be filed by Annora or other potential generic competitors requesting approval to market generic forms of **fostamatinib**,**our products**, which would require us to incur significant additional expense and result in distraction for our management team, and if approved, result in significant decreases in the revenue derived from sales of our marketed products and thereby materially harm our business and financial condition.

Unforeseen safety issues could emerge with our products that could require us to change the prescribing information to add warnings, limit use of the product, and/or result in litigation. Any of these events could have a negative impact on our business.

Discovery of unforeseen safety problems or increased focus on a known problem could impact our ability to commercialize our products and could result in restrictions on its permissible uses, including withdrawal of the medicine from the market.

[Table of Contents](#)

If we or others identify additional undesirable side effects caused by our products after approval:

- regulatory authorities may require the addition of labeling statements, specific warnings, contraindications, or field alerts to physicians and pharmacies;
- regulatory authorities may withdraw their approval of the product and require us to take our approved drugs off the market or suspend their commercialization until the identified issues have been satisfactorily addressed;
- we may be required to change the way the product is administered, conduct additional clinical trials, change the labeling of the product, or implement a Risk Evaluation and Mitigation Strategy (REMS);
- we may have additional limitations on how we promote our drugs;
- third-party payors may limit coverage or reimbursement for our products;
- sales of our products may decrease significantly;
- we may be subject to litigation or product liability claims; and
- our reputation may suffer.

Any of these events could prevent us from achieving or maintaining market acceptance of our products and could substantially increase our operating costs and expenses, which in turn could delay or prevent us from generating significant revenue from sale of our products.

[Table of Contents](#)

Side effects and toxicities associated with our products, as well as the warnings, precautions and requirements listed in the prescribing information for our products, could affect the willingness of physicians to prescribe, and patients to utilize, our products and thus harm commercial sales of our products. **The FDA approved label** For example, for REZLIDHIA, **the FDA-approved label** contains a boxed warning

describing the risk of differentiation syndrome, which can be fatal, in patients receiving REZLIDHIA, the drug. This and other restrictions could limit the commercial success of REZLIDHIA, the product.

If a safety issue emerges post-approval, we may become subject to costly product liability litigation by our customers, their patients or payors. Product liability claims could divert management's attention from our core business, be expensive to defend, and result in sizable damage awards against us that may not be covered by insurance. If we cannot successfully defend ourselves against claims that our products caused injuries, we will incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- decreased demand for any product candidates or products that we may develop;
- the inability to commercialize any products that we may develop;
- injury to our reputation and significant negative media attention;
- withdrawal of patients from clinical studies or cancellation of studies;
- significant costs to defend the related litigation;
- substantial monetary awards to patients; and
- loss of revenue.

We currently hold \$10.0 million in product liability insurance coverage, which may not be adequate to cover all liabilities that we may incur. Insurance coverage is increasingly expensive. We may not be able to obtain insurance coverage at a reasonable cost or in amounts adequate to satisfy any liability or associated costs that may arise in the future. These events could harm our business and results of operations and cause our stock price to decline.

[Table of Contents](#)

Our business could be materially and adversely affected by pandemics as a result of their potential impacts on our sales force and commercialization efforts, supply chain, regulatory, clinical development and corporate development activities and other business operations, in addition to the impact of a global economic slowdown.

Pandemics may result in extended travel and other restrictions in order to reduce the spread of diseases. Government measures taken in response to pandemics could have a significant impact, both direct and indirect, on our business and commerce, as significant reductions in business related activities may occur, supply chains may be disrupted, and manufacturing and clinical development activities may be curtailed or suspended.

For example, during the COVID-19 pandemic, we observed reduced patient-doctor interactions and our representatives had fewer visits with health care providers, which negatively affected our product sales. Physicians with practices severely impacted by the COVID-19 pandemic, or a pandemic occurring in the future, and who currently prescribe our products, may eventually decide to close their independent practices and join a larger medical organization with a practice that does not prescribe our products. Additionally, a pandemic, including COVID-19 or any resurgence thereof, may impact commercial-related activities, such as our marketing programs, speaker bureaus, and market access initiatives which may be required to be conducted virtually, delayed or cancelled, all of which occurred as a result of the COVID-19 pandemic. During the COVID-19 pandemic, we had to deploy resources to enable our field-based employees to continue to engage with health care providers in hybrid virtual and in-person interactions, which may be required in the event a pandemic occurs in the future.

With respect to clinical development, in response to the COVID-19 pandemic, we took measures to implement remote and virtual approaches, including remote patient monitoring where possible and working with our investigators for appropriate care of these patients in a safe manner. Due to the effects of COVID-19 pandemic, we experienced a number of our clinical trial investigators either paused, postponed or delayed new patient enrollment and restricted site visits of existing patients enrolled. In the event that a global pandemic, or a resurgence of the COVID-19 pandemic, occurs in the future, we may need to make decisions on a country-by-country basis to minimize risk to the patients and clinical trial sites. We may also rely heavily on our clinical trial investigators to inform us of the best course of action with respect to

resuming enrollment/screening, considering the ability of sites to ensure patient safety or data integrity. We experienced slower than anticipated enrollment in some of our clinical trials due to adverse effects of COVID-19 pandemic, and in the future, we may experience adverse impacts of a global pandemic on our clinical trials, including the timing thereof, or our ability to continue to treat patients enrolled in our trials, enroll and assess new patients, supply study drugs and obtain complete data points in accordance with study protocol.

Pandemics may cause significant disruption in the supply chain for our commercial products. We rely on third parties to, among other things, manufacture and ship our commercial product, raw materials and product supply for our clinical trials, perform quality testing and supply other goods and services to help manage our commercial activities, our clinical trials and our operations in the ordinary course of business. While we have engaged actively with various elements of our supply chain and distribution channel, including our customers, contract manufacturers, and logistics and transportation provider to meet demand for our products and to remain informed of any challenges within our supply chain, we may face disruptions to our supply chain and operations, and associated delays in the manufacturing and supply of our products. Such supply disruptions would adversely impact our ability to generate sales of and revenues from our products and our business, financial condition, results of operations and growth prospects could be adversely affected.

Pandemics may affect our collaboration and licensing partners for the commercialization of our products globally, as well as our ability to advance our various clinical stage programs. We cannot predict the impact of such disruptions on our partners' ability to advance commercialization of our products in the market and the timing of enrollment and completion of various clinical trials being conducted by our collaboration partners.

Health regulatory agencies globally may experience prolonged disruptions in their operations as a result of pandemics. For example, in response to the COVID-19 pandemic, the FDA delayed inspections and evaluations of certain drug manufacturing facilities and clinical research sites. We cannot predict whether, and when, health regulatory agencies will decide to pause or resume inspections due to pandemics. Any de-prioritization of our clinical trials or delay in regulatory review resulting from such disruptions could materially affect the completion of our clinical trials.

[Table of Contents](#)

In addition, as seen in the COVID-19 pandemic, pandemics could result in a significant disruption of global financial markets. We could experience an inability to access additional capital or an impact on liquidity, which could in the future negatively affect our capacity for certain corporate development transactions or our ability to make other important, opportunistic investments, or we may not be able to meet the requirements under our Credit and Security Agreement (Credit Agreement) with MidCap Financial Trust (MidCap). While we expect pandemics to adversely affect our business, financial condition, results of operations and growth prospects in the future periods, the extent of the impact on our ability to generate sales of and revenues from our approved products, our ability to continue to secure new collaborations and support existing collaboration efforts with our partners, our clinical development and regulatory efforts, our corporate development objectives and the value of and market for our common stock, will depend on future circumstances that are highly uncertain and cannot be predicted with confidence at this time, such as the ultimate duration and severity of pandemics, travel restrictions, quarantines, social distancing and business closure requirements in the US and other countries, and the effectiveness of actions taken globally to contain and treat diseases. To the extent pandemics adversely affect our business and results of operations, it may also have the effect of heightening many of the other risks and uncertainties described elsewhere in this "Risk Factors" section.

*If we fail to comply with our reporting and payment obligations under the Medicaid Drug Rebate Program or other governmental pricing programs in the US, we could be subject to additional rebate or discount requirements, fines, sanctions and exposure under other laws which could have an adverse effect on our business, results of operations and financial condition.**

We participate in the Medicaid Drug Rebate Program, as administered by the **Centers for Medicare and Medicaid Services (CMS)**, the 340B Drug Pricing Program, as administered by the **Health Resources and Services Administration**, and other federal and state government drug pricing programs in the US, and we may participate in additional government pricing programs in the future. These programs generally require us to pay rebates or otherwise provide discounts to government payors **and/or required covered entities** in connection with drugs that are dispensed to beneficiaries/recipients of these programs. In some cases, such as with the Medicaid Drug Rebate Program, the rebates are based on pricing metrics that we report on a monthly and quarterly basis to the government agencies that administer

the programs. Pricing requirements and rebate/discount calculations are complex, vary among products and programs, and are often subject to interpretation by governmental or regulatory agencies and the courts. The requirements of these programs, including, by way of example, their respective terms and scope, change frequently. Responding to current and future changes may increase our costs, and the complexity of compliance will be time consuming. Invoicing for rebates is provided in arrears, and there is frequently a time lag of up to several months between the sales to which rebate notices relate and our receipt of those notices, which further complicates our ability to accurately estimate and accrue for rebates related to the Medicaid program as implemented by individual states. Thus, there can be no assurance that we will be able to identify all factors that may cause our discount and rebate payment obligations to vary from period to period, and our actual results may differ significantly from our estimated allowances for discounts and rebates. Changes in estimates and assumptions may have an adverse effect on our business, results of operations and financial condition.

[Table of Contents](#)

In addition, the **Department of Health and Human Services, DHHS**, Office of Inspector General and other governmental enforcement and administrative bodies have increased their focus on pricing requirements for products, including, but not limited to the methodologies used by manufacturers to calculate average manufacturer price and best price for compliance with reporting requirements under the Medicaid Drug Rebate Program. We are liable for errors associated with our submission of pricing data and for any overcharging of government payors. Failure to make necessary disclosures and/or to identify overpayments could result in allegations against us under the federal False Claims Act and other laws and regulations. Any required refunds to the US government or response to a government investigation or enforcement action would be expensive and time consuming and could have an adverse effect on our business, results of operations and financial condition. In addition, in the event that CMS were to terminate our rebate agreement, no federal payments would be available under Medicaid for our covered outpatient drugs or under Medicare Part B for any of our products that may be reimbursed under Part B.

Finally, we may be affected by developments relating to the 340B **drug pricing program, Drug Pricing Program**. Recently, multiple manufacturers have implemented policies to reduce diversion and inappropriate claims for discounts and rebates by **in-house and** contract pharmacies affiliated with 340B-eligible entities. The **Department of Health and Human Services DHHS** has sent several of these **manufacturers' letters** claiming that the policies violate the 340B statute and referring the manufacturers for potential enforcement action. Manufacturers have challenged these letters in federal court, and the **U.S. US** Court of Appeals for the Third Circuit **recently has ruled**

[Table of Contents](#)

in favor of several **manufacturers; other challenges are still pending. Further, manufacturers.** Arkansas and Louisiana recently enacted laws requiring manufacturers to ship 340B drugs to certain contract pharmacies and imposing penalties on manufacturers that do not comply. Both laws have been challenged in federal court. **Other** In March 2024, the US Court of Appeals for the Eighth Circuit upheld the Arkansas law prohibiting drug makers for restricting 340B drug discounts for providers using contract pharmacies, potentially setting up other states **are considering to pass similar laws. legislation. Our ability to reduce diversion and inappropriate or duplicate claims from 340B-eligible entities and their affiliated pharmacies may be further hampered by the final rule on procedures for the 340B program's administrative dispute resolution process enacted by DHHS in April 2024.** It is unclear how **this the other** pending litigation, recent and proposed legislation, or future administrative action relating to the 340B program will impact our business.

Even for those product candidates that have or may receive regulatory approval, they may fail to achieve the degree of market acceptance by physicians, patients, **healthcare third-party payors and others in the medical community necessary for commercial success, in which case we may not generate significant revenues or become profitable.***

For our product candidates that have or may receive regulatory approval, they may nonetheless fail to gain sufficient market acceptance by physicians, hospital administrators, patients, third-party payors and others in the medical community. The degree of market acceptance of our product candidates, if approved for commercial sale, will depend on a number of factors, including the following:

- relative convenience and ease of administration;
- the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies;
- the willingness of physicians to change their current treatment practices;
- any additional support that may be required to administer the treatment to patients;
- the willingness of hospitals and hospital systems to include our product candidates as treatment options;
- demonstration of efficacy and safety in clinical trials;
- the prevalence and severity of any side effects;
- the ability to offer product candidates for sale at competitive prices;
- the price we charge for our product candidates;
- the strength of marketing and distribution support; and
- the availability of third-party coverage and adequate reimbursement and the willingness of patients to pay out-of-pocket in the absence of such coverage and adequate reimbursement.

59

[Table of Contents](#)

Efforts to educate the physicians, patients, **healthcare** third-party payors and others in the medical community on the benefits of our product candidates may require significant resources and may not be successful. If any of our product candidates are approved, but do not achieve an adequate level of acceptance, we may not generate significant product revenue and we may not become profitable on a sustained basis.

We will need additional capital in the future to sufficiently fund our operations and research.

We have consumed substantial amounts of capital to date as we continue our research and development activities, including preclinical studies and clinical trials and for the commercialization of our products. We may seek another collaborator or licensee in the future for further clinical development and commercialization of **fostamatinib**, our products, as well as our other clinical programs, which we may not be able to obtain on commercially reasonable terms or at all. We believe that our existing capital resources will be sufficient to support our current and projected funding requirements, including the continued commercialization of our products through at least the next 12 months. We have based this estimate on assumptions that may prove to be wrong, and we could utilize our available capital resources sooner than we currently expect. Because of the numerous risks and uncertainties associated with commercial launch, the development

61

[Table of Contents](#)

of our product candidates and other research and development activities, we are unable to estimate with certainty our future product revenues, our revenues from our current and future collaborative partners, the amounts of increased capital outlays and operating expenditures

associated with our current and anticipated clinical trials and other research and development activities.

We will continue to need additional capital and the amount of future capital needed will depend largely on the success of our commercialization of our products, and the success of our internally developed programs as they proceed in later and more expensive clinical trials, including any additional clinical trials that we may decide to conduct with respect to **fostamatinib**, **our products**. While we intend to opportunistically seek access to additional funds through public or private equity offerings or debt financings, we do not know whether additional financing will be available when needed, or that, if available, we will obtain financing on reasonable terms. Our ability to raise additional capital, including our ability to secure new collaborations and continue to support existing collaboration efforts with our partners, may also be adversely impacted by potential worsening global economic conditions and the recent disruptions to, and volatility in, the credit and financial markets in the US and worldwide resulting from the COVID-19 pandemic and the global tensions arising from the Russia-Ukraine war and the Hamas-Israel war. Unless and until we are able to generate a sufficient amount of product, royalty or milestone revenue, which may never occur, we expect to finance future cash needs through public and/or private offerings of equity securities, debt financings or collaboration and licensing arrangements, as well as through proceeds from the exercise of stock options and interest income earned on the investment of our cash balances and short-term investments. To the extent we raise additional capital by issuing equity securities in the future, our stockholders could at that time experience substantial dilution. In addition, we have a significant number of stock options outstanding. To the extent that outstanding stock options have been or may be exercised or other shares issued, our stockholders may experience further dilution. Further, we may choose to raise additional capital due to market conditions or strategic considerations even if we believe we have sufficient funds for our current or future operating plans. Our credit facility with MidCap includes certain covenants that may restrict our business, and any other debt financing that we are able to obtain in the future may involve operating covenants that restrict our business. To the extent that we raise additional funds through any new collaboration and licensing arrangements, we may be required to refund certain payments made to us, relinquish some rights to our technologies or product candidates or grant licenses on terms that are not favorable to us.

We have indebtedness in the form of a term loan pursuant to the Credit Agreement (as defined below) with MidCap, which could adversely affect our financial condition and our ability to respond to changes in our business. Further, if we are unable to satisfy certain conditions of the Credit Agreement, we will be unable to draw down the remainder of the facility.*

We entered into **the** **a** Credit Agreement with MidCap on September 27, 2019, amended on March 29, 2021, February 11, 2022, **July 27, 2022**, and **July 27, 2022** **April 11, 2024**. The Credit Agreement provides for a \$60.0 million term loan credit facility. As of **September 30, 2023** **March 31, 2024**, the outstanding principal balance of the loan was \$60.0 million, and no remaining funds were available under the term loan credit facility. Under the Credit Agreement, we are required to repay amounts due when there is an event of default for the term loans that results in the principal, premium, if any, and interest, if any, becoming due prior to the maturity date for the term loans. The Credit Agreement also contains a number of other affirmative and restrictive covenants. See "Note 9 10 – Debt" and "Note 12 – Subsequent Events" to our "Notes to Condensed Financial Statements" contained in Part I, Item 1 of this Quarterly Report on Form 10-Q for additional details of the Credit Agreement. These and other terms in the

60

[Table of Contents](#)

Credit Agreement have to be monitored closely for compliance and could restrict our ability to grow our business or enter into transactions that we believe would be beneficial to our business. Our business may not generate cash flow from operations in the future sufficient to service our debt and support our growth strategies. If we are unable to generate such cash flow, we may be required to adopt one or more alternatives, such as restructuring our debt or obtaining additional equity capital on terms that may be onerous or highly dilutive. Our ability to refinance our indebtedness will depend on the capital markets and our financial condition at such time. We may not be able to engage in any of these activities or engage in these activities on desirable terms, which could result in a default on our current debt obligations. In addition, we cannot be sure that additional financing will be available when required or, if available, will be on terms satisfactory to us. Further, even if we are able to obtain additional financing, we may be required to use such proceeds to repay a portion of our debt.

Our indebtedness may have other adverse effects, such as:

- our vulnerability to adverse general economic conditions and heightened competitive pressures;

62

[Table of Contents](#)

- dedication of a portion of our cash flow from operations to interest payments, limiting the availability of cash for other operational purposes;
- limited flexibility in planning for, or reacting to, changes in our business and industry; and
- our inability to obtain additional financing in the future.

Our Credit Agreement with MidCap contains a mandatory prepayment provision that gives MidCap and/or its agent the right to demand payment of the outstanding principal and additional interest and fees in the event of default. We may not have enough available cash or be able to obtain financing at the time we are required to repay the term loan with additional interest and fees prior to maturity.

We rely and may continue to rely on two distribution facilities for the sale of our products and potential sale of any of our product candidates.

Our distribution operations for the sale of our products are currently concentrated in two distribution centers owned by a third-party logistics provider. Additionally, our distribution operations, if and when we launch any of our product candidates in the future, may also be concentrated in such distribution centers owned by a third-party logistics provider. Any errors in inventory level management and unforeseen inventory shortage could adversely affect our business. In addition, any significant disruption in the operation of the facility due to natural disaster or severe weather, or events such as fire, accidents, power outages, system failures, or other unforeseen causes, could devalue or damage a significant portion of our inventories and could adversely affect our product distribution and sales until such time as we could secure an alternative facility. Further, climate change may increase both the frequency and severity of extreme weather conditions and natural disasters, which may affect our business operations. If we encounter difficulties with any of our distribution facilities, whether due to the potential future impacts of a global pandemic (including as a result of disruptions of global shipping and the transport of products) or otherwise, or other problems or disasters arise, we cannot ensure that critical systems and operations will be restored in a timely manner or at all, and this would have an adverse effect on our business. In addition, growth could require us to further expand our current facility, which could affect us adversely in ways that we cannot predict.

Forecasting potential sales for any of our product candidates will be difficult, and if our projections are inaccurate, our business may be harmed, and our stock price may be adversely affected.*

Our business planning requires us to forecast or make assumptions regarding product demand and revenues for any of our product candidates if they are approved despite numerous uncertainties. These uncertainties may be increased if we rely on our collaborators or other third parties to conduct commercial activities in certain geographies and provide us with accurate and timely information. Actual results may differ materially from projected results for various reasons, including the following, as well as risks identified in other risk factors:

- the efficacy and safety of any of our product candidates, including as relative to marketed products and product candidates in development by third parties;
- pricing (including discounting or other promotions), reimbursement, product returns or recalls,

[Table of Contents](#)

competition, labeling, adverse events and other items that impact commercialization;

- the rate of adoption in the particular market, including fluctuations in demand for various reasons;

- potential future impacts, if any, due to including a global pandemic;
- lack of patient and physician familiarity with the drug;
- lack of patient use and physician prescribing history;
- lack of commercialization experience with the drug;
- actual sales to patients may significantly differ from expectations based on sales to wholesalers; and
- uncertainty relating to when the drug may become commercially available to patients and rate of adoption

63

[Table of Contents](#)

in other territories.

We expect that our revenues from sales of any of our products will continue to be based in part on estimates, judgment and accounting policies. Any incorrect estimates or disagreements with regulators or others regarding such estimates or accounting policies may result in changes to our guidance, projections or previously reported results. We make estimates for provisions for sales discounts, returns and allowances. Our estimates are based on available customer and payer payor data received from the specialty pharmacies and distributors, as well as third party market research data. In part, our estimates are dependent on our distribution channel and payer payor mix. If actual results in the future vary from our estimates, we adjust these estimates, which would affect our net product revenue and earnings in the period such variances become known. Expected and actual product sales and quarterly and other results may greatly fluctuate, including in the near-term, and such fluctuations can adversely affect the price of our common stock, perceptions of our ability to forecast demand and revenues, and our ability to maintain and fund our operations.

We do not and will not have access to all information regarding fostamatinib and product candidates we licensed to Lilly, Kissei, Grifols, Medison and Knight.

We do not and will not have access to all information regarding fostamatinib and other product candidates, including potentially material information about commercialization plans, medical information strategies, clinical trial design and execution, safety reports from clinical trials, safety reports, regulatory affairs, process development, manufacturing and other areas known by Lilly, Kissei, Grifols, Medison and Knight. In addition, we have confidentiality obligations under our respective agreements with Lilly, Kissei, Grifols, Medison and Knight. Thus, our ability to keep our shareholders informed about the status of fostamatinib and other product candidates will be limited by the degree to which Lilly, Kissei, Grifols, Medison and/or Knight keep us informed and allows us to disclose such information to the public. If Lilly, Kissei, Grifols, Medison and/or Knight fail to keep us informed about commercialization efforts related to fostamatinib, or the status of the clinical development or regulatory approval pathway of other product candidates licensed to them, we may make operational and/or investment decisions that we would not have made had we been fully informed, which may adversely affect our business and operations.

Our future funding requirements will depend on many uncertain factors.*

Our future funding requirements will depend upon many factors, many of which are beyond our control, including, but not limited to:

- the costs to commercialize our products in the US, or any other future product candidates, if any such candidate receives regulatory approval for commercial sale;
- the progress and success of our clinical trials and preclinical activities (including studies and manufacture of materials) of our product candidates conducted by us;
- our ability to secure patent and regulatory protection;
- our ability to secure a favorable price or a positive HTA assessment;

62

[Table of Contents](#)

- potential future impacts, if any, of a global pandemic;
- the costs and timing of regulatory filings and approvals by us and our collaborators;
- the progress of research and development programs carried out by us and our collaborative partners;
- any changes in the breadth of our research and development programs;
- the ability to achieve the events identified in our collaborative agreements that may trigger payments to us from our collaboration partners;
- our ability to acquire or license other technologies or compounds that we may seek to pursue;
- our ability to manage our growth;

64

[Table of Contents](#)

- competing technological and market developments;
- the costs and timing of obtaining, enforcing and defending our patent and other intellectual property rights; and
- expenses associated with any unforeseen litigation, including any arbitration and securities class action lawsuits.

Insufficient funds may require us to delay, scale back or eliminate some or all of our commercial efforts and/or research and development programs, to reduce personnel and operating expenses, to lose rights under existing licenses or to relinquish greater or all rights to product candidates at an earlier stage of development or on less favorable terms than we would otherwise choose or may adversely affect our ability to operate as a going concern.

Our success as a company is uncertain due to our history of operating losses and the uncertainty of any future profitability.*

For the **nine** **three** months ended **September 30, 2023** **March 31, 2024**, we recognized loss from operations of **\$22.5 million** **\$7.0 million** primarily due to higher operating and non-operating expenses, partly offset by our net product sales and collaboration revenues. We have historically incurred losses from operations each year since we were incorporated in June 1996 other than in fiscal year 2010, due in large part to the significant research and development expenditures required to identify and validate new product candidates and pursue our development efforts, and the costs of our ongoing commercial efforts for our products. We expect to continue to incur losses from operations, at least in the next 12 months, and there can be no assurance that we will generate annual operating income in the foreseeable future. Currently, our potential sources of revenues are our sales of our products, upfront payments, research and development contingent payments and royalty payments pursuant to our collaboration arrangements, which may never materialize if our collaborators do not achieve certain events or generate net sales to which these contingent payments are dependent on. If our future drug candidates fail or do not gain regulatory approval, or if our drugs do not achieve sustainable market acceptance, we may not be profitable. As of **September 30, 2023** **March 31, 2024**, we had an accumulated deficit of approximately \$1.4 billion. The extent of our future losses or profitability, if any, is highly uncertain.

If our corporate collaborations or license agreements are unsuccessful, or if we fail to form new corporate collaborations or license agreements, our research and development efforts could be delayed.

Our strategy depends upon the formation and sustainability of multiple collaborative arrangements and license agreements with third parties now and in the future. We rely on these arrangements for not only financial resources, but also for expertise we need now and in the future relating to clinical trials, manufacturing, sales and marketing, and for licenses to technology rights. To date, we have entered into several such arrangements with corporate collaborators; however, we do not know if these collaborations or additional collaborations with third parties, if any, will dedicate sufficient resources or if any development or commercialization efforts by third parties will be successful. In addition, our

corporate collaborators may delay clinical trials, provide insufficient funding for a clinical trial program, stop a clinical trial or abandon a drug candidate or development program. Should a collaborative partner fail to develop or commercialize a compound or product to which it has rights from us for any reason, including corporate restructuring, such failure might delay our ongoing research and development efforts, because we might not receive any future

63

[Table of Contents](#)

payments, and we would not receive any royalties associated with such compound or product. We may seek another collaborator or licensee in the future for clinical development and commercialization of **fostamatinib**, **our products**, as well as our other clinical programs, which we may not be able to obtain on commercially reasonable terms or at all. If we are unable to form new collaborations or enter into new license agreements, our research and development efforts could be delayed. In addition, the continuation of some of our partnered drug discovery and development programs may be dependent on the periodic renewal of our corporate collaborations.

Each of our collaborations could be terminated by the other party at any time, and we may not be able to renew these collaborations on acceptable terms, if at all, or negotiate additional corporate collaborations on acceptable terms, if at all. If these collaborations terminate or are not renewed, any resultant loss of revenues from these collaborations or loss of the resources and expertise of our collaborative partners could adversely affect our business.

Conflicts also might arise with collaborative partners concerning proprietary rights to particular compounds. While our existing collaborative agreements typically provide that we retain milestone payments, royalty rights and/or

65

[Table of Contents](#)

revenue sharing with respect to drugs developed from certain compounds or derivative compounds, any such payments or royalty rights may be at reduced rates, and disputes may arise over the application of payment provisions or derivative payment provisions to such drugs, and we may not be successful in such disputes. For example, in September 2018, BerGenBio served us with a notice of arbitration seeking declaratory relief related to the interpretation of provisions under our June 2011 license agreement, particularly as they relate to the rights and obligations of the parties in the event of the license or sale of a product in the program by BerGenBio and/or the sale of BerGenBio to a third party. The arbitration panel dismissed four of the six declarations sought by BerGenBio, and we thereafter consented to one of the remaining declarations requested by BerGenBio. On February 27, 2019, the arbitration panel issued a determination granting the declaration sought by BerGenBio on the remaining issue, and held that in the event of a sale of shares by BerGenBio's shareholders where there is no monetary benefit to BerGenBio, we would not be entitled to a portion of the proceeds from such a sale. In this circumstance where the revenue share provision is not triggered, the milestone and royalty payment provisions remain in effect. While we do not believe that the determination will have an adverse effect on our operations, cash flows or financial condition, we can make no assurance regarding any such impact. Additionally, the management teams of our collaborators may change for various reasons including due to being acquired. Different management teams or an acquiring company of our collaborators may have different priorities which may have adverse results on the collaboration with us.

We are also a party to various license agreements that give us rights to use specified technologies in our research and development processes. The agreements pursuant to which we have in-licensed technology permit our licensors to terminate the agreements under certain circumstances. If we are not able to continue to license these and future technologies on commercially reasonable terms, our product development and research may be delayed or otherwise adversely affected.

If conflicts arise between our collaborators or advisors and us, any of them may act in their self-interest, which may be adverse to our stockholders' interests.

If conflicts arise between us and our corporate collaborators or scientific advisors, the other party may act in its self-interest and not in the interest of our stockholders. Some of our corporate collaborators are conducting multiple product development efforts within each disease area that is the subject of the collaboration with us or may be acquired or merged with a company having a competing program. In some of our collaborations, we have agreed not to conduct, independently or with any third party, any research that is competitive with the research conducted under our collaborations. Our collaborators, however, may develop, either alone or with others, products in related fields that are competitive with the products or potential products that are the subject of these collaborations. Competing products, either developed by our collaborators or to which our collaborators have rights, may result in their withdrawal of support for our product candidates.

If any of our corporate collaborators were to breach or terminate its agreement with us or otherwise fail to conduct the collaborative activities successfully and in a timely manner, the preclinical or clinical development or commercialization of the affected product candidates or research programs could be delayed or terminated. We generally do not control the amount and timing of resources that our corporate collaborators devote to our programs or potential products. We do not know whether current or future collaborative partners, if any, might pursue alternative technologies or develop alternative products either on their own or in collaboration with others, including our competitors, as a means for developing treatments for the diseases targeted by collaborative arrangements with us.

64

[Table of Contents](#)

Our success is dependent on intellectual property rights held by us and third parties, and our interest in such rights is complex and uncertain.

Our success will depend to a large part on our own, our licensees' and our licensors' ability to obtain and defend patents for each party's respective technologies and the compounds and other products, if any, resulting from the application of such technologies. For example, fostamatinib is covered as a composition of matter in a US issued patent that has expiration date of September 2031, olutasidenib is covered as a composition of matter in a US issued patent that has an expected expiration date of September 2031, December 2036, after taking into account patent term adjustment extension rules, and extension rules, pralsetinib is covered as a composition of matter in a US issued patent that has an expiration date in November 2036 and subject to extensions.

In the future, our patent position might be highly uncertain and involve complex legal and factual questions. questions, and the cost to defend may also be significant. For example, we may be involved in post-grant proceedings before the

66

[Table of Contents](#)

US Patent and Trademark Office. Post-grant proceedings are complex and expensive legal proceedings and there is no assurance we will be successful in any such proceedings. A post-grant proceeding could result in us losing our patent rights and/or our freedom to operate and/or require us to pay significant royalties. Additionally, third parties may challenge the validity, enforceability or scope of our issued patents, which may result in such patents being narrowed, invalidated or held unenforceable through interference, opposition or invalidity proceedings before the US Patent and Trademark Office or non-US patent offices. Any successful opposition to our patents could deprive us of exclusive rights necessary for the successful commercialization of fostamatinib our products or our other product candidates. Oppositions could also be filed to complementary patents, such as formulations, methods of manufacture and methods of use, that are intended to extend the patent life of the overall portfolio beyond the patent life covering the composition of matter. A successful opposition to any such complementary patent could impact our ability to extend the life of the overall portfolio beyond that of the related composition of matter patent.

An adverse outcome may allow third parties to use our intellectual property without a license and/or allow third parties to introduce generic and other competing products, any of which would negatively impact our business. For example, in June 2022, we received a notice

letter from Annora advising that it has filed an ANDA with the FDA for a generic version of TAVALISSE and asserting that certain patents related to TAVALISSE that are listed in the Orange Book will not be infringed by Annora's proposed product, are invalid and/or are unenforceable. In July 2022, we filed a lawsuit in the US District Court for the District of New Jersey against Annora and its subsidiaries for infringement of those US patents. In September 2022, Annora and its subsidiaries answered and counterclaimed for declaratory judgment of non-infringement and invalidity of those patents. We filed an answer to Annora's counterclaims on October 12, 2022. Annora served invalidity and non-infringement contentions on December 31, 2022. **We filed an answer to Annora's invalidity and non-infringement contentions in March 2023. Litigation continues, and no trial date is currently set.** We intend to vigorously enforce and defend our intellectual property rights related to TAVALISSE. Should Annora or any other third parties receive FDA approval of an ANDA for a generic version of fostamatinib or a 505(b)(2) NDA with respect to fostamatinib, and if our patents covering fostamatinib were held to be invalid (or if such competing generic versions of fostamatinib were found to not infringe our patents), then they could introduce generic versions of fostamatinib or other such 505(b)(2) products before our patents expire, and the resulting competition would negatively affect our business, financial condition and results of operations. Please also see the risk factor entitled, "If manufacturers obtain approval for generic versions of our products, or of products with which we compete, our business may be harmed." **In the future, there might be other claims that are subject to substantial uncertainties and unascertainable damages or other remedies, and the cost to defend may also be significant.**

Additional uncertainty may result because no consistent policy regarding the breadth of legal claims allowed in biotechnology patents has emerged to date. Accordingly, we cannot predict the breadth of claims allowed in our or other companies' patents.

Because the degree of future protection for our proprietary rights is uncertain, we cannot assure **you** that:

- we were the first to make the inventions covered by each of our pending patent applications;
- we were the first to file patent applications for these inventions;
- others will not independently develop similar or alternative technologies or duplicate any of our technologies;
- any of our pending patent applications will result in issued patents;
- any patents issued to us or our collaborators will provide a basis for commercially viable products or will provide us with any competitive advantages or will not be challenged by third parties;

65

[Table of Contents](#)

- we will develop additional proprietary technologies that are patentable;
- we will obtain a **supplemental** **supplementary** protection certificate that will extend the protection afforded by the patent to the product with a marketing authorization; or
- the patents of others will not have a negative effect on our ability to do business.

We rely on trade secrets to protect technology where we believe patent protection is not appropriate or

67

[Table of Contents](#)

obtainable; however, trade secrets are difficult to protect. While we require employees, collaborators and consultants to enter into confidentiality agreements, we may not be able to adequately protect our trade secrets or other proprietary information in the event of any

unauthorized use or disclosure or the lawful development by others of such information.

We are a party to certain in-license agreements that are important to our business, and we generally do not control the prosecution of in-licensed technology. Accordingly, we are unable to exercise the same degree of control over this intellectual property as we exercise over our internally developed technology. Moreover, some of our academic institution licensors, research collaborators and scientific advisors have rights to publish data and information in which we have rights. If we cannot maintain the confidentiality of our technology and other confidential information in connection with our collaborations, our ability to receive patent protection or protect our proprietary information may otherwise be impaired. In addition, some of the technology we have licensed relies on patented inventions developed using US government resources.

The US government retains certain rights, as defined by law, in such patents, and may choose to exercise such rights. Certain of our in-licenses may be terminated if we fail to meet specified obligations. If we fail to meet such obligations and any of our licensors exercise their termination rights, we could lose our rights under those agreements. If we lose any of our rights, it may adversely affect the way we conduct our business. In addition, because certain of our licenses are sublicenses, the actions of our licensors may affect our rights under those licenses.

If a dispute arises regarding the infringement or misappropriation of the proprietary rights of others, such dispute could be costly and result in delays in our research and development activities, partnering and commercialization activities.

Our success will depend, in part, on our ability to operate without infringing or misappropriating the proprietary rights of others. There are many issued patents and patent applications filed by third parties relating to products or processes that are similar or identical to our licensors or ours, and others may be filed in the future. There may also be copyrights or trademarks that third parties hold. There can be no assurance that our activities, or those of our licensors, will not violate intellectual property rights of others. We believe that there may be significant litigation in the industry regarding patent and other intellectual property rights, and we do not know if our collaborators or we would be successful in any such litigation. Any legal action against our collaborators or us claiming damages or seeking to enjoin commercial activities relating to the affected products, our methods or processes could:

- require our collaborators or us to obtain a license to continue to use, manufacture or market the affected products, methods or processes, which may not be available on commercially reasonable terms, if at all;
- prevent us from using the subject matter claimed in the patents held by others;
- subject us to potential liability for damages;
- consume a substantial portion of our managerial and financial resources; and
- result in litigation or administrative proceedings that may be costly, whether we win or lose.

Our effective tax rate may fluctuate, and we may incur obligations in tax jurisdictions in excess of accrued amounts.

We are subject to taxation in numerous US states and territories. As a result, our effective tax rate is derived from a combination of applicable tax rates in the various places that we operate. In preparing our financial statements, we estimate the amount of tax that will become payable in each of such places. Nevertheless, our effective tax rate may be different than experienced in the past due to numerous factors, including passage of the newly enacted federal income tax law, changes in the mix of our profitability from state to state, the results of examinations and audits of our tax

[Table of Contents](#)

filings, our inability to secure or sustain acceptable agreements with tax authorities, changes in accounting for income taxes and changes in tax laws. Any of these factors could cause us to experience an effective tax rate significantly different from previous periods or our current expectations and may result in tax obligations in excess of amounts accrued in our financial statements.

Our ability to use net operating losses (NOLs) and certain other tax attributes is uncertain and may be limited.

Our ability to use our federal and state NOLs to offset potential future taxable income and related income taxes

[Table of Contents](#)

that would otherwise be due is dependent upon our generation of future taxable income before the expiration dates of the NOLs, and we cannot predict with certainty when, or whether, we will generate sufficient taxable income to use all of our NOLs. Federal NOLs generated prior to 2018 will continue to be governed by the NOL carryforward rules as they existed prior to the adoption of the Tax Cuts and Jobs Act (Tax Act), which means that generally they will expire 20 years after they were generated if not used prior thereto. Many states have similar laws. Accordingly, our federal and state NOLs could expire unused and be unavailable to offset future income tax liabilities. Under the Tax Act as modified by the Coronavirus Aid, Relief, and Economic Security Act (CARES Act), federal NOLs incurred in tax years beginning after December 31, 2017 and before January 1, 2021 may be carried back to each of the five tax years preceding such loss, and NOLs arising in tax years beginning after December 31, 2020 may not be carried back. Moreover, federal NOLs generated in tax years ending after December 31, 2017 may be carried forward indefinitely, but the deductibility of such federal NOLs may be limited to 80% of current year taxable income for tax years beginning after January 1, 2021. Under A.B. 85, our California NOL carryforwards are suspended for tax years 2020, 2021, and 2022, but the period to use these carryovers was extended. Further, the Tax Act requires the taxpayers to capitalize Research and Experimental (R&E) expenditures under Section 174 of the Internal Revenue Code, as amended (Code), effective for taxable years beginning after December 31, 2021, which will reduce our NOLs beginning in 2022. R&E expenditures attributable to US-based research must be amortized over a period of 5 years and R&E expenditures attributable to research conducted outside of the US must be amortized over a period of 15 years.

In addition, utilization of NOLs to offset potential future taxable income and related income taxes that would otherwise be due is subject to annual limitations under the "ownership change" provisions of Sections 382 and 383 of the Code and similar state provisions, which may result in the expiration of NOLs before future utilization. In general, under the Code, if a corporation undergoes an "ownership change," generally defined as a greater than 50% change (by value) in its equity ownership over a three-year period, the corporation's ability to use its pre-change NOLs and other pre-change tax attributes (such as research and development credit carryforwards) to offset its post-change taxable income or taxes may be limited. Our equity offerings and other changes in our stock ownership, some of which are outside of our control, may have resulted or could in the future result in an ownership change. Although we have completed studies to provide reasonable assurance that an ownership change limitation would not apply, we cannot be certain that a taxing authority would reach the same conclusion. If, after a review or audit, an ownership change limitation were to apply, utilization of our domestic NOLs and tax credit carryforwards could be limited in future periods and a portion of the carryforwards could expire before being available to reduce future income tax liabilities. Moreover, our ability to utilize our NOLs is conditioned upon us achieving profitability and generating US federal taxable income.

Because we expect to be dependent upon collaborative and license agreements, we might not meet our strategic objectives.

Our ability to generate revenue in the near term depends on the timing of recognition of certain upfront payments, achievement of certain payment triggering events with our existing collaboration agreements and our ability to enter into additional collaborative agreements with third parties. Our ability to enter into new collaborations and the revenue, if any, that may be recognized under these collaborations is highly uncertain. If we are unable to enter into one or more new collaborations, our business prospects could be harmed, which could have an immediate adverse effect on our ability to continue to develop our compounds and on the trading price of our stock. Our ability to enter into a collaboration may be dependent on many factors, such as the results of our clinical trials, competitive factors and the fit of one of our programs with another company's risk tolerance, including toward regulatory issues, patent portfolio, clinical pipeline, the stage of the available data, particularly if it is early, overall corporate goals and financial position.

To date, a portion of our revenues have been related to the research or transition phase of each of our collaborative agreements. Such revenues are for specified periods, and the impact of such revenues on our results of

[Table of Contents](#)

operations is at least partially offset by corresponding research costs. Following the completion of the research or transition phase of each collaborative agreement, additional revenues may come only from payments triggered by milestones and/or the achievement of other contingent events, and royalties, which may not be paid, if at all, until certain conditions are met. This risk is heightened due to the fact that unsuccessful research efforts may preclude us from receiving any contingent payments under these agreements. Our receipt of revenues from collaborative arrangements is also significantly affected by the timing of efforts expended by us and our collaborators and the timing of lead compound identification. We have received payments from our current collaborations including Lilly, Grifols, Kissei, Medison, Knight, BerGenBio, and Daiichi. Under several agreements, future payments may not be earned until the

69

[Table of Contents](#)

collaborator has advanced product candidates into clinical testing, which may never occur or may not occur until sometime well into the future. If we are not able to generate revenue under our collaborations when and in accordance with our expectations or the expectations of industry analysts, this failure could harm our business and have an immediate adverse effect on the trading price of our common stock.

Our business requires us to generate meaningful revenue from royalties and licensing agreements. To date, we have not recognized material amount of revenue from royalties for the commercial sale of drugs, and we do not know when we will be able to generate such meaningful revenue in the future.

Securities class action lawsuits or other litigation could result in substantial damages and may divert management's time and attention from our business.

We have been subject to class action lawsuits in the past and we may be subject to lawsuits in the future, such as those that might occur if there was to be a change in our corporate strategy. These and other lawsuits are subject to inherent uncertainties, and the actual costs to be incurred relating to the lawsuit will depend upon many unknown factors. The outcome of litigation is necessarily uncertain, and we could be forced to expend significant resources in the defense of such suits, and we may not prevail. Monitoring and defending against legal actions is time-consuming for our management and detracts from our ability to fully focus our internal resources on our business activities. In addition, we may incur substantial legal fees and costs in connection with any such litigation. We have not established any reserves for any potential liability relating to any such potential lawsuits. It is possible that we could, in the future, incur judgments or enter into settlements of claims for monetary damages. A decision adverse to our interests on any such actions could result in the payment of substantial damages, or possibly fines, and could have an adverse effect on our cash flow, results of operations and financial position.

If our competitors develop technologies that are more effective than ours, our commercial opportunity will be reduced or eliminated.*

The biotechnology and pharmaceutical industries are intensely competitive and subject to rapid and significant technological change. Many of the drugs that we are attempting to discover will be competing with existing therapies. In addition, a number of companies are pursuing the development of pharmaceuticals that target the same diseases and conditions that we are targeting. For example, the commercialization of new pharmaceutical products is highly competitive, and we face substantial competition with respect to our products in which there are existing therapies and drug candidates in development for the treatment of hematologic disorders and cancer that may be alternative therapies to our products. Many of our competitors, including a number of large pharmaceutical companies that compete directly with us, have significantly greater financial resources and expertise commercializing approved products than we do. Also, many of our competitors are large pharmaceutical companies that will have a greater ability to reduce prices for their competing drugs in an effort to gain market share and undermine the value proposition that we might otherwise be able to offer to payers. We face, and will continue to face, intense competition from pharmaceutical and biotechnology companies, as well as from academic and research institutions and government agencies, both in the US and abroad. Some of these competitors are pursuing the development of pharmaceuticals that target the same diseases and conditions as our research programs. Our competitors including fully integrated pharmaceutical companies have extensive drug discovery efforts and are developing novel small-molecule pharmaceuticals. We also face significant competition from organizations that

are pursuing the same or similar technologies, including the discovery of targets that are useful in compound screening, as the technologies used by us in our drug discovery efforts.

Competition may also arise from:

- new or better methods of target identification or validation;

68

[Table of Contents](#)

- generic versions of our products or of products with which we compete;
- other drug development technologies and methods of preventing or reducing the incidence of disease;
- new small molecules; or
- other classes of therapeutic agents.

Our competitors or their collaborative partners may utilize discovery technologies and techniques or partner

70

[Table of Contents](#)

with collaborators in order to develop products more rapidly or successfully than we or our collaborators are able to do. Many of our competitors, particularly large pharmaceutical companies, have substantially greater financial, technical and human resources and larger research and development staffs than we do. In addition, academic institutions, government agencies and other public and private organizations conducting research may seek patent protection with respect to potentially competitive products or technologies and may establish exclusive collaborative or licensing relationships with our competitors.

We believe that our ability to compete is dependent, in part, upon our ability to create, maintain and license scientifically-advanced technology and upon our and our collaborators' ability to develop and commercialize pharmaceutical products based on this technology, as well as our ability to attract and retain qualified personnel, obtain patent protection or otherwise develop proprietary technology or processes, secure effective market access by ensuring competitive pricing and reimbursement in territories of interest, and secure sufficient capital resources for the expected substantial time period between technological conception and commercial sales of products based upon our technology. The failure by any of our collaborators or us in any of those areas may prevent the successful commercialization of our potential drug targets.

Many of our competitors, either alone or together with their collaborative partners, have significantly greater experience than we do in:

- identifying and validating targets;
- screening compounds against targets; and
- undertaking preclinical testing and clinical trials.

Accordingly, our competitors may succeed in obtaining patent protection, identifying or validating new targets or **discovering developing** new drug compounds before we do.

Our competitors might develop technologies and drugs that are more effective or less costly than any that are being developed by us or that would render our technology and product candidates obsolete and noncompetitive. In addition, our competitors may succeed in obtaining the approval of the FDA or other regulatory agencies for product candidates more rapidly. Companies that complete clinical trials, obtain required regulatory agency approvals and commence commercial sale of their drugs before us may achieve a significant competitive advantage, including certain patent and FDA marketing exclusivity rights that would delay or prevent our ability to market certain products. Any drugs resulting from our research and development efforts, or from our joint efforts with our existing or future collaborative partners, might not be able to compete successfully with competitors' existing or future products or obtain regulatory approval in the US or elsewhere.

We face and will continue to face intense competition from other companies for collaborative arrangements with pharmaceutical and biotechnology companies, for establishing relationships with academic and research institutions and for licenses to additional technologies. These competitors, either alone or with their collaborative partners, may succeed in developing technologies or products that are more effective than ours.

Our ability to compete successfully will depend, in part, on our ability to:

- identify and validate targets;
- discover candidate drug compounds that interact with the targets we identify in a safe and efficacious way;

69

[Table of Contents](#)

- attract and retain scientific and product development personnel;
- recruit subjects into our clinical trials;
- obtain and maintain required regulatory approvals;
- obtain patent or other proprietary protection for our new drug compounds and technologies;
- obtain access to manufacturing resources of the sufficient standard and scale;

71

[Table of Contents](#)

- enter commercialization agreements for our new drug compounds; and
- obtain and maintain appropriate reimbursement price and positive recommendations by HTA bodies.

Our stock price may be volatile, and our stockholders' investment in our common stock could decline in value.

The market prices for our common stock and the securities of other biotechnology companies have been highly volatile and may continue to be highly volatile in the future. The following factors, in addition to other risk factors described in this section, may have a significant impact on the market price of our common stock:

- the progress and success of our clinical trials and preclinical activities (including studies and manufacture of materials) of our product candidates conducted by us;
- our ability to continue to sell our products in the US;

- our ability to enter into partnering opportunities across our pipeline;
- the receipt or failure to receive the additional funding necessary to conduct our business;
- selling of our common stock by large stockholders;
- presentations of detailed clinical trial data at medical and scientific conferences and investor perception thereof;
- announcements of technological innovations or new commercial products by our competitors or us;
- the announcement of regulatory applications, such as Annora's ANDA, seeking approval of generic versions of our marketed products;
- developments concerning proprietary rights, including patents;
- developments concerning our collaborations;
- publicity regarding actual or potential medical results relating to products under development by our competitors or us;
- regulatory developments in the US and foreign countries;
- changes in the structure of healthcare payment systems;
- litigation or arbitration;
- economic and other external factors or other disaster or crisis; and
- period-to-period fluctuations in financial results.

70

[Table of Contents](#)

If we fail to continue to meet the listing standards of Nasdaq, our common stock may be delisted, which could have a material adverse effect on the liquidity of our common stock.

Our common stock is currently listed on the Nasdaq Global Market. The Nasdaq Stock Market LLC (Nasdaq) has requirements that a company must meet in order to remain listed on Nasdaq. In particular, Nasdaq rules require us to maintain a minimum bid price of \$1.00 per share of our common stock (the "Bid Price Requirement"). If the closing bid price of our common stock falls below \$1.00 per share for 30 consecutive trading days or we do not meet other listing requirements, we would fail to be in compliance with Nasdaq listing standards. There can be no assurance that we will continue to meet the Bid Price Requirement, or any other requirement in the future.

On November 22, 2022, we received a deficiency letter from the Listing Qualifications Department of the Nasdaq Stock Market LLC notifying us that, for the last 30 consecutive business days, the bid price for our common stock had closed below the Bid Price Requirement.

We were provided a period of 180 calendar days, or until May 22, 2023 (the "Compliance Date"), to regain compliance with the Bid Price Requirement. On January 5, 2023, we received notification from the Listing Qualifications Department of the Nasdaq Stock Market LLC

72

[Table of Contents](#)

that we ~~have had~~ regained compliance with the Bid Price Requirement because the closing bid price of our common stock closed at \$1.00 or more for over 10 consecutive business days from December 13, 2022 to January 4, 2023.

On November 27, 2023, we again received a deficiency letter from the Listing Qualifications Department of Nasdaq notifying us that, for the last 30 consecutive business days, the bid price for our common stock had closed below the Bid Price Requirement. On December 12, 2023, we received notification from the Listing Qualifications Department of Nasdaq that we had regained our compliance with the Bid Price Requirement because the closing bid price of our common stock closed at \$1.00 or more for over 10 consecutive business days from November 28, 2023 to December 11, 2023.

Although we have regained compliance, the Nasdaq ~~Stock Market LLC~~ may in the future initiate the delisting process with a notification letter if we were to again fall out of compliance. If we were to receive such a notification, we would be afforded a grace period of 180 calendar days to regain compliance with the ~~minimum bid price requirement~~, Bid Price Requirement. In order to regain compliance, shares of our common stock would need to maintain a minimum closing bid price of at least \$1.00 per share for a minimum of 10 consecutive trading days. ~~In addition, We would be required to notify Nasdaq of our intent to cure the minimum bid price deficiency, which may include, if necessary, seeking stockholder approval to implement a reverse stock split. Any reverse stock split may not be approved by our stockholders, or if approved the market price per share of our common stock after the reverse stock split may not remain unchanged or increase in proportion to the reduction in the number of common stock outstanding before the reverse stock split.~~

~~Additionally, we may be unable to meet other applicable Nasdaq listing requirements, including maintaining minimum levels of stockholders' equity or market values of our common stock in which case, our common stock could be delisted. If our common stock were to be delisted, the liquidity of our common stock would be adversely affected and the market price of our common stock could decrease.~~

*The withdrawal of the UK from the EU may adversely impact our ability to obtain regulatory approvals of our product candidates in the UK, result in restrictions or imposition of taxes and duties for importing our product candidates into the UK, and may require us to incur additional expenses in order to develop, manufacture and commercialize our product candidates in the UK.**

Following the result of a referendum in 2016, the UK left the EU on January 31, 2020, commonly referred to as Brexit. Pursuant to the formal withdrawal arrangements agreed between the UK and the EU, the UK was subject to a transition period until December 31, 2020, or the Transition Period, during which EU rules continued to apply. A trade and cooperation agreement (Trade Agreement) that outlines the future trading relationship between the UK and the EU was agreed to in December 2020 and has been approved by each EU member state and the UK.

Since a significant proportion of the regulatory framework in the UK applicable to our business and our product candidates is derived from EU directives and regulations, Brexit has had, and will continue to have, a material impact upon the regulatory regime with respect to the development, manufacture, importation, approval and commercialization of our product candidates in the UK or the EU. Great Britain (made up of England, Scotland, and Wales) is no longer covered by the EEA's procedures for the grant of marketing authorizations (Northern Ireland will be covered by such procedures). The UK Government and the EU recently adopted a new agreement, the "Windsor Framework" which will replace the Northern Ireland Protocol. According to the Windsor Framework, medicinal products intended for the UK market including Northern Ireland will be authorized by the MHRA, and will bear a "UK only" label. This means that Medicinal products placed on the market in Northern Ireland will no longer need to be compliant with EU law. These new measures will be implemented from January 1, 2025.

A separate marketing authorization will be required to market drugs in Great Britain. ~~The MHRA has launched the MHRA in the UK is sufficiently prepared to handle the increased volume of Innovative Licensing and Access Pathway, or ILAP, a new accelerated assessment procedure for marketing authorization applications that it is likely facilitating the interaction with pricing authorities and HTA bodies and aiming to receive, enable companies to enter the UK market faster. On January 1, 2024, the MHRA launched a new International Recognition Procedure for Great Britain (England, Scotland and Wales) marketing authorization applications whereby the MHRA will, when considering such applications, recognize the approval of medicines by trusted reference regulators in Australia, Canada, Switzerland, Singapore, Japan, United States and EU following its own abbreviated assessment. Any delay in obtaining, or an inability to obtain, any marketing approvals would delay or prevent us from commercializing our product candidates in the UK or the EU and restrict our ability to generate revenue and achieve and sustain~~

revenue and achieve and sustain profitability.

While the Trade Agreement provides for the tariff-free trade of medicinal products between the UK and the EU, there may be additional non-tariff costs to such trade which did not exist prior to the end of the Transition Period. Further, should the UK diverge from the EU from a regulatory perspective in relation to medicinal products, tariffs could be put into place in the future. We could therefore, both now and in the future, face significant additional expenses (when compared to the position prior to the end of the Transition Period) to operate our business, which could significantly and materially harm or delay our ability to generate revenues or achieve profitability of our business. Any further changes in international trade, tariff and import/export regulations as a result of Brexit or otherwise may impose unexpected duty costs or other non-tariff barriers on us. These developments, or the perception that any of them could occur, may significantly reduce global trade and, in particular, trade between the impacted nations and the UK. It is also possible that Brexit may negatively affect our ability to attract and retain employees, particularly those from the EU.

Orphan designation in Great Britain following Brexit is granted on an essentially identical basis as in the EU but is based on the prevalence of the condition in Great Britain. It is therefore possible that conditions that are currently designated as orphan conditions in Great Britain will no longer be, and conditions that are not currently designated as orphan conditions in the EU will be designated as such in Great Britain.

In April 2023, the European Commission adopted a wide ranging proposal for a new Directive and a new Regulation. If made into law, this proposal will revise and replace the existing general pharmaceutical legislation. This change will likely result in significant changes to the pharmaceutical industry. In particular, it is expected that the new Directive and Regulations will, if made into law, affect the duration of the period of regulatory protection afforded to medicinal products including regulatory data protection (also called "data exclusivity"), marketing exclusivity afforded to orphan medicinal products, as well as the conditions of eligibility to the orphan designation.

If product liability lawsuits are successfully brought against us, we may incur substantial liabilities and may be required to limit commercialization of our products.

The testing and marketing of medical products and the sale of any products for which we obtain marketing approval exposes us to the risk of product liability claims. Product liability claims might be brought against us by consumers, health care providers, pharmaceutical companies or others selling or otherwise coming into contact with our products. If we cannot successfully defend ourselves against product liability claims, we may incur substantial liabilities or be required to limit commercialization of our products. We carry product liability insurance that is limited in scope and amount and may not be adequate to fully protect us against product liability claims. If and when we obtain marketing approval for our product candidates, we intend to expand our insurance coverage to include the sale of commercial products; however, we may be unable to obtain product liability insurance on commercially reasonable terms or in adequate amounts. Our inability to obtain sufficient product liability insurance at an acceptable cost to protect against potential product liability claims could prevent or inhibit the commercialization of pharmaceutical products we develop, alone or with corporate collaborators. We, or our corporate collaborators, might not be able to obtain insurance at a reasonable cost, if at all. While under various circumstances we are entitled to be indemnified against losses by our corporate collaborators, indemnification may not be available or adequate should any claim arise.

We depend on various scientific consultants and advisors for the success and continuation of our research and development efforts.

We work extensively with various scientific consultants and advisors. The potential success of our drug discovery and development programs depends, in part, on continued collaborations with certain of these consultants and advisors. We, and various members of our management and research staff, rely on certain of these consultants and advisors for expertise in our research, regulatory and clinical efforts. Our scientific advisors are not our employees and may have commitments to, or consulting or advisory contracts with, other entities that may limit their availability to us. We do not know if we will be able to maintain such consulting agreements or that such scientific advisors will not enter into consulting arrangements with competing pharmaceutical or biotechnology companies, any of which may have a detrimental impact on our research objectives and could have an adverse effect on our business, financial condition and results of operations.

While we have a strong compliance process in place to ensure we are complying with all requirements of law, our consulting or advisory contracts with our scientific consultants and advisors may be scrutinized under the Anti-

Kickback Statute, the UK Bribery Act 2010, and other similar national and state-level legislation, which prohibit, among other things, companies from offering or paying anything of value as remuneration for ordering, purchasing, or recommending the ordering or purchasing of pharmaceutical and biological products that may be paid for, in whole or in part, by Medicare, Medicaid, or another federal healthcare program. Although there are several statutory exceptions and regulatory safe harbors that may protect these arrangements from prosecution or regulatory sanctions, our consulting and advising contracts may be subject to scrutiny if they do not fit squarely within an available exception or safe harbor.

If we use biological and hazardous materials in a manner that causes injury or violates laws, we may be liable for damages, penalties or fines.

Our research and development activities involve the controlled use of potentially harmful biological materials as well as hazardous materials, chemicals, animals, and various radioactive compounds. We cannot completely eliminate the risk of accidental contamination or injury from the use, storage, handling or disposal of these animals and materials. In the event of contamination or injury, we could be held liable for damages that result or for penalties or fines that may be imposed, and such liability could exceed our resources. We are also subject to federal, state and local laws and regulations governing the use, storage, handling and disposal of these materials and specified waste products. The cost of compliance with, or any potential violation of, these laws and regulations could be significant.

Our information technology systems, or those used by our CROs or other contractors or consultants, may fail or suffer other breakdowns, cyber-attacks, or information security breaches.*

We are **increasingly** dependent upon information technology systems, infrastructure, and data to operate our **business**, particularly **during the COVID-19 pandemic**. **business**. While we believe our **cybersecurity measures are adequate**, our **cybersecurity risk management, strategy and governance may be found to be inadequate that could harm our business**. We also rely on **third party** **third-party** vendors and their information technology systems. Despite the implementation of security measures, our recovery systems, security protocols, network protection mechanisms and other security measures and those of our CROs and other contractors and consultants are vulnerable to compromise from natural disasters; terrorism; war; telecommunication and electric failures; traditional computer hackers; malicious code (such as computer viruses or worms); employee error, theft or misuse; denial-of-service attacks; cyber-attacks by sophisticated nation-state and nation-state supported actors including ransomware; or other system disruptions. We receive, generate and store significant and increasing volumes of personal (including health), confidential and proprietary information. There can be no assurance that we, or our collaborators, CROs, third-party vendors, contractors and consultants will be successful in efforts to detect, prevent, protect against or fully recover systems or data from all breakdowns, service interruptions, attacks or breaches. Any breakdown, cyber-attack or information security breach could result in a disruption of our drug development programs or other aspects of our business. For example, the loss of clinical trial data from completed or ongoing clinical trials for a product candidate could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security breach were to result in a loss of or damage to our data or applications, or inappropriate disclosure of personal, confidential or proprietary information, we could incur liability, incur significant remediation or litigation costs, result in product development delays, disrupt key business operations, cause loss of revenue and divert attention of management and key information technology resources.

Hackers and data thieves are increasingly sophisticated and operate large-scale and complex automated attacks, including on companies within the healthcare industry. As the cyber-threat landscape evolves, these threats are likely growing in frequency, sophistication and intensity and are increasingly difficult to detect. The costs of maintaining or upgrading our cyber-security systems at the level necessary to keep up with our expanding operations and prevent against potential attacks are increasing. Cyber threats may be generic, or they may be targeted against our information systems. Our network and storage applications and those of our contract manufacturing organizations, collaborators, contractors, CROs or vendors may be subject to unauthorized access or processing by hackers or breached due to operator or other human error, theft, malfeasance or other system disruptions. We may be unable to anticipate or immediately detect information security incidents and the damage caused by such incidents. These data breaches and any unauthorized access, processing or disclosure of our information or intellectual property could compromise our intellectual property and expose our sensitive business information. Such attacks, such as in the case of a ransomware attack, also may interfere with our ability to continue to operate and may result in delays and shortcomings due to an attack that may encrypt our or our service providers' or partners' systems unusable. Additionally, because our services involve the processing of personal information and other sensitive information about individuals, we are subject to **various laws, regulations, industry standards, and contractual requirements related to such processing**. Any event that

[Table of Contents](#)

various laws, regulations, industry standards, and contractual requirements related to such processing. Any event that leads to unauthorized access, processing or disclosure of personal information, including personal information regarding our clinical trial participants or employees, could harm our reputation and business, compel us to comply with federal and/or state breach notification laws and foreign law equivalents, subject us to investigations and mandatory corrective action, and otherwise subject us to liability under laws, regulations or contracts that protect the privacy and security of personal information, which could disrupt our business, damage our reputation with our stakeholders, result in increased costs or loss of revenue, lead to negative publicity or result in significant financial exposure. The CCPA, in particular, includes a private right of action for California consumers whose personal information is impacted by a data security incident resulting from a company's failure to maintain reasonable security procedures, and hence may result in civil litigation in the event of a security breach impacting such information. In addition, legislators and regulators in the US have enacted and are proposing new and more robust privacy and cybersecurity laws and regulations in response to increasing broad-based cyberattacks, including the CCPA and New York SHIELD Act. Notably, on July 26, 2023, the SEC adopted a final rule on cybersecurity risk management, strategy, governance and incident disclosure (the "SEC Cyber Rule"). The SEC Cyber Rule requires public companies to make current disclosures about material cybersecurity incidents as well as annual disclosures of material information about their cybersecurity risk management, strategy and governance. The SEC Cyber Rule became effective on September 5, 2023. New data security laws add additional complexity, requirements, restrictions and potential legal risk, and compliance programs may require additional investment in resources, and could impact strategies and availability of previously useful data.

The costs to respond to a security breach and/or to mitigate any identified security vulnerabilities could be significant, our efforts to address these issues may not be successful, and these issues could result in interruptions, delays, negative publicity, loss of customer trust, and other harms to our business and competitive position. Remediation of any potential security breach may involve significant time, resources, and expenses. We could be required to fundamentally change our business activities and practices in response to a security breach and our systems or networks may be perceived as less desirable, which could negatively affect our business and damage our reputation.

A security breach may cause us to breach our contracts with third parties. Our agreements with relevant stakeholders such as collaborators may require us to use legally required, industry-standard or reasonable measures to safeguard personal information. A security breach could lead to claims by relevant stakeholders that we have failed to comply with such contractual obligations, or require us to cooperate with these stakeholders in their own compliance efforts related to the security breach. In addition, any non-compliance with our data privacy obligations in our contracts or our inability to flow down such obligations from relevant stakeholders to our vendors may cause us to breach our contracts. As a result, we could be subject to legal action or the relevant stakeholders could end their relationships with us. There can be no assurance that the limitations of liability in our contracts would be enforceable or adequate or would otherwise protect us from liabilities or damages.

We may not have adequate insurance coverage for security incidents or breaches. The successful assertion of one or more large claims against us that exceeds our available insurance coverage, or results in changes to our insurance policies (including premium increases or the imposition of large deductible or co-insurance requirements), could have an adverse effect on our business. In addition, we cannot be sure that our existing insurance coverage will continue to be available on acceptable terms or that our insurers will not deny coverage as to any future claim.

Future equity issuances or a sale of a substantial number of shares of our common stock may cause the price of our common stock to decline.

Because we will continue to need additional capital in the future to continue to expand our business and our research and development activities, among other things, we may conduct additional equity offerings. For example, on August 3, 2021, a new automatic shelf registration statement was filed by us as a well-known seasoned issuer (Wksi). The automatic shelf registration statement was filed to register, among other securities, the sale of up to a maximum aggregate offering price of \$100.0 million of shares of our common stock that may be issued and sold from time to time under our Open Market Sale Agreement with Jefferies LLC (Jefferies), and a base prospectus which covers the offering, issuance, and sale by us of the securities identified above from time to time in one or more offerings. On March 1, 2022, we filed a post-effective amendment to the automatic shelf registration statement immediately after filing our Annual Report Form 10-K for the year ended December 31, 2021 because we no longer qualified as a Wksi upon filing of such Annual Report. The post-effective amendment was declared effective on May 3, 2022. The post-effective

[Table of Contents](#)

amendment registers, among other securities, a base prospectus which covers the offering, issuance, and sale by us of up to \$250.0 million in

74

[Table of Contents](#)

the aggregate of the securities identified from time to time in one or more offerings, which include the \$100.0 million of shares of our common stock that may be offered, issued and sold under the Open Market Sale Agreement.

We may also in the future enter into underwriting or sales agreements with financial institutions for the offer and sale of any combination of common stock, preferred stock, debt securities and warrants in one or more offerings. If we or our stockholders sell, or if it is perceived that we or they will sell, substantial amounts of our common stock in the public market, the market price of our common stock could fall. A decline in the market price of our common stock could make it more difficult for us to sell equity or equity-related securities in the future at a time and price that we deem appropriate. In addition, future sales by us of our common stock may be dilutive to existing stockholders. Furthermore, if we obtain funds through a credit facility or through the issuance of debt or preferred securities, these securities would likely have rights senior to the rights of our common stockholders, which could impair the value of our common stock.

Risks Related to Clinical Development and Regulatory Approval

*Enacted or future legislation, and/or potentially unfavorable pricing regulations or other healthcare reform initiatives, may increase the difficulty and cost for us to obtain regulatory approval of our product candidates and/or commercialize **fostamatinib** our products or our product candidates, once approved, and affect the prices we may set or obtain.**

The regulations that govern, among other things, regulatory approvals, coverage, pricing and reimbursement for new drug products vary widely from country to country. In the US and some foreign jurisdictions, there have been a number of legislative and regulatory changes and proposed changes regarding the healthcare system that could prevent or delay regulatory approval of our product candidates, restrict or regulate post-approval activities and affect our ability to successfully sell **TAVALISSE**, **REZLIDHIA**, **our products**, or any product candidates for which we obtain regulatory approval in the future. In particular, in March 2010, the Affordable Care Act (ACA) was enacted, which substantially changed the way health care is financed by both governmental and private insurers, and continues to significantly impact the US pharmaceutical industry. On June 17, 2021, the US Supreme Court dismissed the most recent judicial challenge to the **ACA Affordable Care Act** brought by several states without specifically ruling on the constitutionality of the law. It is unclear how future actions before the Supreme Court, other such litigation, and the healthcare reform measures of the Biden administration will impact the Affordable Care Act and our business.

There have been, and likely will continue to be, legislative and regulatory proposals at the foreign, federal and state levels directed at broadening the availability of healthcare and containing or lowering the cost of healthcare. We cannot predict the initiatives that may be adopted in the future. The continuing efforts of the government, insurance companies, managed care organizations and other payors of healthcare services to contain or reduce the costs of healthcare and/or impose price controls may adversely affect, for example:

- the demand for **TAVALISSE**, **REZLIDHIA**, **our products**, or our product candidates, if we obtain regulatory approval;
- our ability to set a price that we believe is fair for our products;
- our ability to generate revenue and achieve or maintain profitability;
- the level of taxes that we are required to pay; and
- the availability of capital.

Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private payors, which may adversely affect our future profitability.

In the US, the EU and other potentially significant markets for our current and future products, government authorities and third-party payors are increasingly attempting to limit or regulate the price of medical products and services, particularly for new and innovative products and therapies, which has resulted in lower average selling prices. In the US, there have been several Congressional inquiries and federal legislation designed to, among other things, bring more transparency to drug pricing, review the relationship between pricing and manufacturer-sponsored patient assistance programs, and reform government program reimbursement methodologies for drugs.

[Table of Contents](#)

On March 11, 2021, President Biden signed the American Rescue Plan Act of 2021 into law, which, among other changes, eliminates the statutory Medicaid drug rebate cap, currently set at 100% of a drug's average manufacture price, for single source and innovator multiple source drugs, beginning January 1, 2024. The American Rescue Plan Act also temporarily increased premium tax credit assistance for individuals eligible for subsidies under the ACA Affordable Care Act for 2021 and 2022 and removed the 400% federal poverty level limit that otherwise applies for purposes of eligibility to receive premium tax credits. The Inflation Reduction Act of 2022 (IRA) extended this increased tax credit assistance and removal of the 400% federal poverty limit through 2025. Additionally, beginning in April 2013, the Budget Control Act of 2011 created an automatic reduction of Medicare payments to providers of up to 2%. As a result of the COVID-19 pandemic, this reduction was temporarily suspended from May 1, 2020 through March 31, 2022, with subsequent reductions to 1% from April 1, 2022 until June 30, 2022. The 2% reduction was then reinstated and has been in effect since June 30, 2022 July 1, 2022, and will remain in effect (with additional reductions of 2.25% in through the first half six months of 2030 and 3% in the second half of 2030 to offset the COVID-19 suspension) until 2031, fiscal year 2032 sequestration order, unless additional Congressional action is taken. Moreover, on June 16, 2022, the Federal Trade Commission issued a policy statement stating its intent to increase enforcement scrutiny of "exclusionary rebates" to PBMs and other intermediaries that "foreclose competition." On August 16, 2022, President Biden signed into law the IRA, which, among other reforms, allows Medicare to: beginning in 2026, establish a "maximum fair price" for a fixed number of pharmaceutical and biological products covered under Medicare Parts B and D following a price negotiation with CMS; beginning in 2023, penalize drug companies that raise prices for products covered under Medicare Parts B and D faster than inflation; and beginning in 2025, impose new discount obligations on pharmaceutical and biological manufacturers for products covered under Medicare Part D. CMS has recently taken steps to implement the IRA. First, on June 9, 2023, CMS released a list of 43 Medicare Part B products that had an adjusted coinsurance rate based on the inflationary rebate provisions of the IRA for the time period of July 1, 2023 to September 30, 2023. Additionally, on June 30, 2023, CMS issued guidance detailing the requirements and parameters of the first round of price negotiations for products subject to the "maximum fair price" provision. Most recently, on August 29, 2023, CMS released the initial list of ten drugs subject to price negotiations. This negotiation process will occur during 2023 and 2024 and result in maximum prices that will be effective beginning in 2026. None of our products were listed among the first ten products slated for the program as announced on August 29, 2023. On November 17, 2023, CMS released guidance outlining the methodology for identifying certain manufacturers eligible to participate in a phase-in period where discounts on applicable products will be lower than those required by the Medicare Part D Manufacturer Discount Program. Most recently, on December 14, 2023, CMS released a list of 48 Medicare Part B products that had adjusted coinsurance rates based on the inflationary rebate provisions of the IRA for the time period of January 1, 2024 to March 31, 2024 and also issued revised guidance for manufacturers in the Medicare Part B and D drug discount programs. While it remains to be seen how the drug pricing provisions imposed by the IRA will affect the broader pharmaceutical industry, several pharmaceutical manufacturers and other industry stakeholders have challenged the law, including through lawsuits brought against the U.S. Department of Health and Human Services, DHHS, the Secretary of the U.S. Department of Health and Human Services, DHHS, CMS, and the CMS Administrator challenging the constitutionality and administrative implementation of the IRA's drug price negotiation provisions.

The Biden administration has also taken executive action to address drug pricing and other healthcare policy changes. For example, on September 12, 2022, President Biden issued an Executive Order to promote biotechnology and biomanufacturing innovation. The Order noted several methods through which the Biden Administration would support the advancement of biotechnology and biomanufacturing in healthcare, and instructed the Department of Health and Human Services DHHS to submit, within 180 days of the Order, a report assessing how to use biotechnology and biomanufacturing to achieve medical breakthroughs, reduce the overall burden of disease, and improve health outcomes. On October 14, 2022, President Biden issued an Executive Order on Lowering Prescription Drug Costs for Americans which instructed the Secretary of the Department of Health and Human Services DHHS to consider whether to select for testing by the CMS Innovation Center new health care payment and delivery models that would lower drug costs and promote access to innovative drug therapies for beneficiaries enrolled in the Medicare and Medicaid programs. The Executive Order further directed the Secretary of the Department of

Health and Human Services to submit, within 90 days after the date of the Executive Order, a report regarding any models that may lead to lower cost-sharing for commonly used drugs and support value-based payment that promotes high-quality care. On February 14, 2023, the Department of Health and Human Services DHHS issued a report in response to the October 14, 2022, Executive Order, which, among other things, selects three potential drug affordability and accessibility models to be tested by the CMS Innovation Center. Specifically, the report addresses: (1) a model that would allow Part D Sponsors to establish a "high-value drug list" setting the maximum co-payment amount for certain common generic drugs at \$2; (2) a Medicaid-focused model that would establish a partnership between CMS, manufacturers, and state Medicaid agencies that would result in multi-state outcomes-based agreements for certain cell and gene therapy drugs; and (3) a model that would adjust Medicare Part B payment amounts for Accelerated Approval Program drugs to advance the developments of novel treatments.

78

[Table of Contents](#)

Other proposed administrative actions may affect our government pricing responsibilities. For example, CMS has issued proposals to amend the existing Medicaid Drug Rebate Program regulations. In addition, there are pending legal and legislative developments relating to the 340B drug pricing program, Drug Pricing Program, including ongoing litigation challenging

76

[Table of Contents](#)

federal enforcement actions against manufacturers and recently introduced and enacted state legislation. It remains to be seen how these drug pricing initiatives will affect the broader pharmaceutical industry.

At the state level, individual states are increasingly aggressive in passing legislation and implementing regulations designed to control pharmaceutical and biological product pricing. Specifically, several U.S. states and localities have enacted legislation requiring pharmaceutical companies to establish marketing compliance programs, file periodic reports, and/or make periodic public disclosures on sales, marketing, pricing, clinical trials, and other activities. Other state laws prohibit certain marketing-related activities including the provision of gifts, meals or other items to certain healthcare providers, and restrict the ability of manufacturers to offer co-pay support to patients for certain prescription drugs. Several state laws require disclosures related to state agencies and/or commercial purchasers with respect to certain price increases and new product launches that exceed a certain level thresholds as identified in the relevant statutes. Another emerging trend at the state level is the establishment of prescription drug affordability boards, some of which will prospectively permit certain states to establish upper payment limits for drugs that the state has determined to be "high-cost." Some of these laws and regulations contain ambiguous requirements that government officials have not yet clarified. Given the lack of clarity in the laws and their implementation, our reporting actions could be subject to the penalty provisions of the pertinent federal and state laws and regulations.

Furthermore, the increased emphasis on managed healthcare in the US and on country and regional pricing and reimbursement controls in the EU and the UK will put additional pressure on product pricing, reimbursement and usage, which may adversely affect our sales and results of operations. These pressures can arise from rules and practices of managed care groups, judicial decisions and governmental laws and regulations related to Medicare, Medicaid, and healthcare reform, pharmaceutical reimbursement policies and pricing in general. Legislative and regulatory proposals have been made to expand post-approval requirements and restrict sales and promotional activities for pharmaceutical products.

We cannot predict the likelihood, nature, or extent of health reform initiatives that may arise from future legislation or administrative action. However, we expect these initiatives to increase pressure on drug pricing. Further, certain broader legislation that is not targeted to the health care industry may nonetheless adversely affect our profitability. If we or any third parties we may engage are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we or such third parties are not able to maintain

regulatory compliance, our product candidates may lose any regulatory approval that may have been obtained and we may not achieve or sustain profitability.

See "Part I, Item 1 – Business "Business – Government Regulation – Healthcare Reform" contained in Part I, Item 1 of our Annual Report on Form 10-K for the year ended ~~December 31, 202~~ December 31, 2023, for additional information.²

Regulatory approval for any approved product is limited by the FDA, the EC and other regulators to those specific indications and conditions for which clinical safety and efficacy have been demonstrated, and we may incur significant liability if it is determined that we are promoting the "off-label" use of our products or any of our future product candidates if approved.

Any regulatory approval is limited to those specific diseases, indications and patient populations for which a product is deemed to be safe and effective by the FDA, the EC and other regulators. For example, the FDA-approved label for TAVALISSE is only approved for use in adults with ITP who have had an insufficient response to other treatments and for REZLIDHIA is only approved for use in adult patients with R/R AML with a susceptible IDH1 mutation as detected by an FDA-approved test. In addition to the FDA approval required for new formulations, any new indication for an approved product also requires FDA approval. If we are not able to obtain FDA approval for any desired future indications for our products and product candidates, our ability to effectively market and sell our products may be reduced and our business may be adversely affected.

While physicians may choose to prescribe drugs for uses that are not described in the product's labeling and for uses that differ from those tested in clinical studies and approved by the regulatory authorities, our ability to promote the

79

[Table of Contents](#)

products is limited to those indications and patient populations that are specifically approved by the FDA. These "off-label" uses are common across medical specialties and may constitute an appropriate treatment for some patients in varied circumstances. We have implemented compliance and monitoring policies and procedures, including a process for internal review of promotional materials, to deter the promotion of our products for off-label uses. We cannot guarantee

77

[Table of Contents](#)

that these compliance activities will prevent or timely detect off-label promotion by sales representatives or other personnel in their communications with health care professionals, patients and others, particularly if these activities are concealed from us. Regulatory authorities in the US generally do not regulate the behavior of physicians in their choice of treatments. Regulatory authorities do, however, restrict communications by pharmaceutical companies on the subject of off-label use. If our promotional activities fail to comply with the FDA's or other competent national authority's regulations or guidelines, we may be subject to warnings from, or enforcement action by, these regulatory authorities. In addition, our failure to follow FDA rules and guidelines relating to promotion and advertising may cause the FDA to issue warning letters or untitled letters, suspend or withdraw an approved product from the market, require a recall or institute fines, which could result in the disgorgement of money, operating restrictions, injunctions or civil or criminal enforcement, and other consequences, any of which could harm our business.

Notwithstanding the regulatory restrictions on off-label promotion, the FDA and other regulatory authorities allow companies to engage in truthful, non-misleading and non-promotional scientific exchange concerning their products. We engage in medical education activities and communicate with investigators and potential investigators regarding our clinical trials. If the FDA or other regulatory or enforcement authorities

determine that our communications regarding our marketed product are not in compliance with the relevant regulatory requirements and that we have improperly promoted off-label uses, or that our communications regarding our investigational products are not in compliance with the relevant regulatory requirements and that we have improperly engaged in pre-approval promotion, we may be subject to significant liability, including civil and administrative remedies as well as criminal sanctions.

Delays in clinical testing could result in increased costs to us.*

We may not be able to initiate or continue clinical studies or trials for our product candidates if we are unable to locate and enroll a sufficient number of eligible patients to participate in these clinical trials as required by the FDA or other regulatory authorities, whether due to the impacts of a global pandemic, ~~the~~ global tensions arising from the Russian-Ukrainian war and Hamas-Israel war or otherwise. Even if we are able to enroll a sufficient number of patients in our clinical trials, if the pace of enrollment is slower than we expect, the development costs for our product candidates may increase and the completion of our clinical trials may be delayed, or our clinical trials could become too expensive to complete. Significant delays in clinical testing could negatively impact our product development costs and timing. Our estimates regarding timing are based on a number of assumptions, including assumptions based on past experience with our other clinical programs. If we are unable to enroll the patients in these trials at the projected rate, the completion of the clinical program could be delayed and the costs of conducting the program could increase, either of which could harm our business.

Clinical trials can be delayed for a variety of reasons, including delays in obtaining regulatory approval to commence a study, delays from scaling up of a study, delays in reaching agreement on acceptable clinical trial agreement terms with prospective clinical sites, delays in obtaining institutional review board approval to conduct a study at a prospective clinical site or delays in recruiting subjects to participate in a study. In addition, we typically rely on third-party clinical investigators to conduct our clinical trials and other third-party organizations to oversee the operations of such trials and to perform data collection and analysis. The clinical investigators are not our employees, and we cannot control the amount or timing of resources that they devote to our programs. Failure of the third-party organizations to meet their obligations, whether due to the potential future impacts of a global pandemic, the global tensions arising from ~~the~~ Russian-Ukrainian war and Hamas-Israel war ~~or otherwise~~, could adversely affect clinical development of our products. As a result, we may face additional delaying factors outside our control if these parties do not perform their obligations in a timely fashion. For example, any number of those issues could arise with our clinical trials causing a delay. Delays of this sort could occur for the reasons identified above or other reasons. If we have delays in conducting the clinical trials or obtaining regulatory approvals, our product development costs will increase. For example, we may need to make additional payments to third-party investigators and organizations to retain their services or we may need to pay recruitment incentives. If the delays are significant, our financial results and the commercial prospects for our product candidates will be harmed, and our ability to become profitable will be delayed. Moreover, these third-party investigators and organizations may also have relationships with other commercial entities, some of

80

[Table of Contents](#)

which may compete with us. If these third-party investigators and organizations assist our competitors at our expense, it could harm our competitive position.

78

[Table of Contents](#)

Due to the effects of the COVID-19 pandemic, for several of our development programs, we ~~had~~ experienced disruption or delay in our ability to enroll and assess patients, maintain patient enrollment, supply study ~~drug~~, ~~drugs~~, report trial results, or interact with regulators, ethics committees or other important agencies due to limitations in employee resources or otherwise. In addition, in the event that a global pandemic

occurs in the future, some patients in our clinical trial may not be able or willing to comply with clinical trial protocols if quarantines impede patient movement or interrupt healthcare services. Similarly, our ability to recruit and retain patients and principal investigators and site staff may be adversely affected if a global pandemic continues and persists for an extended period of time, and we may experience significant disruptions to our clinical development timelines, which would adversely affect our business, financial condition, results of operations and growth prospects in the future.

We have conducted in the past and are currently conducting or may conduct in the future clinical trials in the US and outside the US including Ukraine, Russia and Israel. Recent actions taken by the Russian Federation in Ukraine and surrounding areas have destabilized the region and caused the adoption of comprehensive sanctions by, among others, the EU, the US and the UK, which restrict a wide range of trade and financial dealings with Russia and Russian persons, as well as certain regions in Ukraine. Also, the recent global tensions arising from the Hamas-Israel war may result in disruptions in the broader global economic environment. Further, some patients may not be able to comply with clinical trial protocols if the conflict impedes patient movement or interrupts healthcare services. In addition, clinical trial site initiation and patient enrollment may be delayed, and we may not be able to access sites for initiation and monitoring in regions affected by the Russian-Ukrainian conflict war or the Hamas-Israel war including due to the prioritization of hospital resources away from clinical trials or as a result of warfare, violence, government-imposed curfews, warfare, violence or events or other governmental actions or events that restrict movement. We could also experience disruptions in our supply chain or limits our ability to obtain sufficient materials for our drug products in certain regions.

Public perception of the risk-benefit balance for our COVID-19 product candidates may be affected by adverse events in clinical trials involving our product candidate or other COVID-19 treatments.

Negative perception of the efficacy, safety, or tolerability of any investigational medicines that we develop, or of other products similar to products we are developing, such as fostamatinib for the treatment of COVID-19, could adversely affect our ability to conduct our business, advance our investigational medicines, or obtain regulatory approvals.

Adverse events in clinical trials of our investigational medicines or in clinical trials of others developing similar products, including other COVID-19 treatments, could result in a decrease in the perceived benefit of one or more of our programs, increased regulatory scrutiny, decreased confidence by patients and clinical trial collaborators in our investigational medicines, and less demand for any product that we may develop. If and when they are used in clinical trials, our developmental candidates and investigational medicines could result in a greater quantity of reportable adverse events, including suspected unexpected serious adverse reactions, other reportable negative clinical outcomes, manufacturing reportable events or material clinical events that could lead to clinical delay or hold by the FDA or applicable regulatory authority or other clinical delays, any of which could negatively impact the perception of one or more of our programs, as well as our business as a whole. In addition, responses by US, state, or foreign governments to negative public perception may result in new legislation or regulations that could limit our ability to develop any investigational medicines or commercialize any approved products, obtain or maintain regulatory approval, or otherwise achieve profitability. More restrictive statutory regimes, government regulations, or negative public opinion would have an adverse effect on our business, financial condition, results of operations, and prospects and may delay or impair the development of our investigational medicines and commercialization of any approved products or demand for any products we may develop.

We lack the capability to manufacture compounds for clinical development, and we rely on and intend to continue relying on third parties for commercial supply, manufacturing and distribution if any of our product candidates which receive regulatory approval and we may be unable to obtain required material or product in a timely manner, at an acceptable cost or at a quality level required to receive regulatory approval.

We currently do not have the manufacturing capabilities or experience necessary to produce our products or any product candidates for clinical trials, including fostamatinib in our ongoing clinical trials for certain indications. We currently use one active pharmaceutical ingredient manufacturer and one

[Table of Contents](#)

unpartnered product candidates, we rely on third-party manufacturers for the active pharmaceutical ingredients, as well as various manufacturers to manufacture starting components, excipients and formulated drug products. Our ability to develop our product candidates, and our ability to commercially supply our products will depend, in part, on our ability to successfully obtain the active pharmaceutical ingredients and other substances and materials used in our product candidates from third parties and to have finished products manufactured by third parties in accordance with regulatory requirements and in sufficient quantities for preclinical and clinical testing and commercialization. If we fail to develop and maintain supply relationships with these third parties, we may be unable to continue to develop or commercialize our product candidates.

We rely and will continue to rely on certain third parties, including those located outside the US, as our limited source of the materials they supply or the finished products they manufacture. The drug substances and other materials used in our product candidates are currently available only from one or a limited number of suppliers or manufacturers and certain of our finished product candidates are manufactured by one or a limited number of contract manufacturers. Any of these existing suppliers or manufacturers may:

- fail to supply us with product on a timely basis or in the requested amount due to unexpected damage to or destruction of facilities or equipment or otherwise;
- fail to increase manufacturing capacity and produce drug product and components in larger quantities and at higher yields in a timely or cost-effective manner, or at all, to sufficiently meet our commercial needs;
- be unable to meet our production demands due to issues related to their reliance on sole-source suppliers and manufacturers;
- supply us with product that fails to meet regulatory requirements;
- become unavailable through business interruption or financial insolvency;
- lose regulatory status as an approved source;
- be unable or unwilling to renew current supply agreements when such agreements expire on a timely basis, on acceptable terms or at all; or
- discontinue production or manufacturing of necessary drug substances or products.

Our current and anticipated future dependence upon these third-party manufacturers may adversely affect our ability to develop and commercialize product candidates on a timely and competitive basis, which could have an adverse effect on sales, results of operations and financial condition. If we were required to transfer manufacturing processes to other third-party manufacturers and we were able to identify an alternative manufacturer, we would still need to satisfy various regulatory requirements. Satisfaction of these requirements could cause us to experience significant delays in receiving an adequate supply of our products and products in development and could be costly. Moreover, we may not be able to transfer processes that are proprietary to the manufacturer, if any. These manufacturers may not be able to produce material on a timely basis or manufacture material at the quality level or in the quantity required to meet our development timelines and applicable regulatory requirements and may also experience a shortage in qualified personnel. Our third-party manufacturers import certain materials from China to produce our products. The tensions between the US and China have led to a series of tariffs and sanctions being imposed by the US on imports from China mainland, as well as other business restrictions. Such tensions could adversely impact us and our third-party manufacturers. We may not be able to maintain or renew our existing third-party manufacturing arrangements, or enter into new arrangements, on acceptable terms, or at all. Our third-party manufacturers could terminate or decline to renew our manufacturing arrangements based on their own business priorities, at a time that is costly or inconvenient for us. If we are unable to contract for the production of materials in sufficient quantity and of sufficient quality on acceptable terms, our planned clinical trials may be significantly delayed. Manufacturing delays could postpone the filing of our investigational new drug (IND) applications and/or the initiation or completion of clinical trials that we have currently planned or may plan in the future.

[Table of Contents](#)

Drug manufacturers are subject to ongoing periodic unannounced inspection by the FDA, the Drug Enforcement Administration, the European Medicines Agency, EMA, national competent authorities in the EU and UK and

80

[Table of Contents](#)

other federal and state government and regulatory agencies to ensure strict compliance with cGMP and other government regulations and corresponding foreign standards. We do not have control over third-party manufacturers' compliance with these regulations and standards and they may not be able to comply. Switching manufacturers may be difficult because the number of potential manufacturers is limited. It may be difficult or impossible for us to find a replacement manufacturer quickly on acceptable terms, or at all. Additionally, if we are required to enter into new supply arrangements, we may not be able to obtain approval from the FDA of any alternate supplier in a timely manner, or at all, which could delay or prevent the clinical development and commercialization of any related product candidates. Failure of our third-party manufacturers or us to comply with applicable regulations, whether due to the impacts of a global pandemic or otherwise, could result in sanctions being imposed on us, including fines, civil penalties, delays in or failure to grant marketing approval of our product candidates, injunctions, delays, suspension or withdrawal of approvals, license revocation, seizures or recalls of products and compounds, operating restrictions and criminal prosecutions, warning or similar letters or civil, criminal or administrative sanctions against us, any of which could adversely affect our business.

Any product for which we have obtained regulatory approval, or for which we obtain approval in the future, is subject to, or will be subject to, extensive ongoing regulatory requirements by the FDA, EMA, MHRA and other comparable regulatory authorities, and if we fail to comply with regulatory requirements or if we experience unanticipated problems with our products, we may be subject to penalties, we may be unable to generate revenue from the sale of such products, our potential for generating positive cash flow will be diminished, and the capital necessary to fund our operations will be increased.

In April 2018, the FDA approved TAVALISSE for the treatment of adult patients with chronic ITP who have had insufficient response to previous treatment. In December 2022, the FDA approved REZLIDHIA for the treatment of adult patients with R/R AML with a susceptible IDH1 mutation as detected by an FDA-approved test. We commercialize TAVALISSE and REZLIDHIA our products in the US and we have entered into commercialization agreements with third parties to commercialize fostamatinib outside the US. Any product for which we have obtained regulatory approval, or for which we obtain regulatory approval in the future, along with the manufacturing processes and practices, post-approval clinical research, product labeling, advertising and promotional activities for such product, are subject to continual requirements of, and review by, the FDA, the EMA and other comparable international regulatory authorities. These requirements include submissions of safety and other post-marketing information and reports, registration and listing requirements, cGMP requirements relating to manufacturing, quality control, quality assurance and corresponding maintenance of records and documents, requirements regarding the distribution of samples to physicians, import and export requirements and recordkeeping. If we or our suppliers encounter manufacturing, quality or compliance difficulties with respect to our products or any of our product candidates, when and if approved, whether due to the impacts of a global pandemic (including as a result of disruptions of global shipping and the transport of products) or otherwise, we may be unable to obtain or maintain regulatory approval or meet commercial demand for such products, which could adversely affect our business, financial conditions, results of operations and growth prospects.

Promotional communications with respect to prescription drugs are subject to a variety of legal and regulatory restrictions and must be consistent with the information in the product's approved labeling. Thus, we will not be able to promote any products we develop for indications or uses for which they are not approved.

In addition, the FDA often requires post-marketing testing and surveillance to monitor the effects of products. The FDA, the EMA and other comparable international regulatory agencies may condition approval of our product candidates on the completion of such post-marketing clinical studies. These post-marketing studies may suggest that a product causes undesirable side effects or may present a risk to the patient. Additionally, the FDA may require REMS to help ensure that the benefits of the drug outweigh its risks. A REMS may be required to include

various elements, such as a medication guide or patient package insert, a communication plan to educate healthcare providers of the drug's risks, limitations on who may prescribe or dispense the drug, requirements that patients enroll in a registry or undergo certain health evaluations or other measures that the FDA deems necessary to ensure the safe use of the drug.

Discovery after approval of previously unknown problems with any of our products, manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may result in actions such as:

- restrictions on our ability to conduct clinical trials, including full or partial clinical holds on ongoing or planned trials;
- restrictions on product manufacturing processes;

81 83

[Table of Contents](#)

- restrictions on product manufacturing processes;
- restrictions on the marketing of a product;
- restrictions on product distribution;
- requirements to conduct post-marketing clinical trials;
- untitled or warning letters or other adverse publicity;
- withdrawal of products from the market;
- refusal to approve pending applications or supplements to approved applications that we submit;
- recall of products;
- refusal to permit the import or export of our products;
- product seizure;
- fines, restitution or disgorgement of profits or revenue;
- refusal to allow us to enter into supply contracts, including government contracts;
- injunctions; or
- imposition of civil or criminal penalties.

If such regulatory actions are taken, the value of our company and our operating results will be adversely affected. Additionally, if the FDA, the EMA or any other comparable international regulatory agency withdraws its approval of a product that is or may be approved, we will be unable to generate revenue from the sale of that product in the relevant jurisdiction, our potential for generating positive cash flow will be diminished and the capital necessary to fund our operations will be increased. Accordingly, we continue to expend significant time, money and effort in all areas of regulatory compliance, including manufacturing, production, product surveillance, post-marketing studies and quality control.

If any of our third-party contractors fail to perform their responsibilities to comply with FDA rules and regulations, the marketing and sales of our products could be delayed and we may be subject to enforcement action, which could decrease our revenues.

Conducting our business requires us to manage relationships with third-party contractors. As a result, our success depends partially on the success of these third parties in performing their responsibilities to comply with FDA rules and regulations. Although we pre-qualify our contractors and we believe that they are fully capable of performing their contractual obligations, we cannot directly control the adequacy and timeliness of the resources and expertise that they apply to these activities.

If any of our partners or contractors fail to perform their obligations in an adequate and timely manner, or fail to comply with the FDA's rules and regulations, then the marketing and sales of our products could be delayed. The FDA may also take enforcement actions against us

based on compliance issues identified with our contractors. If any of these events occur, we may incur significant liabilities, which could decrease our revenues. For example, sales and medical science liaison or MSL personnel, including contractors, must comply with FDA requirements for the advertisement and promotion of products.

82

[Table of Contents](#)

If we are unable to obtain regulatory approval to market products in the US and foreign jurisdictions, we will not be permitted to commercialize products we or our collaborative partners may develop.

We cannot predict whether regulatory clearance will be obtained for any product that we, or our collaborative partners, hope to develop. Satisfaction of regulatory requirements typically takes many years, is dependent upon the type, complexity and novelty of the product and requires the expenditure of substantial resources. Of particular significance to us are the requirements relating to research and development and testing.

84

[Table of Contents](#)

Before commencing clinical trials in humans in the US, we, or our collaborative partners, will need to submit and receive approval from the FDA of an IND application. Clinical trials are subject to oversight by institutional review boards and the FDA and:

- must be conducted in conformance with the FDA's good clinical practices and other applicable regulations;
- must meet requirements for institutional review board oversight;
- must meet requirements for informed consent;
- are subject to continuing FDA and regulatory oversight;
- may require large numbers of test subjects; and
- may be suspended by us, our collaborators or the FDA at any time if it is believed that the subjects participating in these trials are being exposed to unacceptable health risks or if the FDA finds deficiencies in the IND or the conduct of these trials.

While we have stated that we intend to file additional INDs for future product candidates, this is only a statement of intent, and we may not be able to do so because we may not be able to identify potential product candidates. In addition, the FDA may not approve any IND we or our collaborative partners may submit in a timely manner, or at all.

Before receiving FDA approval to market a product, we must demonstrate with substantial clinical evidence that the product is safe and effective in the patient population and the indication that will be treated. Data obtained from preclinical and clinical activities are susceptible to varying interpretations that could delay, limit or prevent regulatory approvals. In addition, delays or rejections may be encountered based upon additional government regulation from future legislation or administrative action or changes in FDA policy during the period of product development, clinical trials and FDA regulatory review. Failure to comply with applicable FDA or other applicable regulatory requirements may result in criminal prosecution, civil penalties, recall or seizure of products, total or partial suspension of production or injunction, adverse publicity, as well as other regulatory action against our potential products or us. Additionally, we have limited experience in conducting and managing the clinical trials necessary to obtain regulatory approval.

If regulatory approval of a product is granted, this approval will be limited to those indications or disease states and conditions for which the product is demonstrated through clinical trials to be safe and efficacious. We cannot assure you that any compound developed by us, alone or with others, will prove to be safe and efficacious in clinical trials and will meet all of the applicable regulatory requirements needed to receive marketing approval.

Outside the US, our ability, or that of our collaborative partners, to market a product is contingent upon receiving a marketing authorization from the appropriate regulatory authorities. This foreign regulatory approval process typically includes all of the risks and costs associated with FDA approval described above and may also include additional risks and costs, such as the risk that such foreign regulatory authorities, which often have different regulatory and clinical trial requirements, interpretations and guidance from the FDA, may require additional clinical trials or results for approval of a product candidate, any of which could result in delays, significant additional costs or failure to obtain such regulatory approval. There can be no assurance, however, that we or our collaborative partners will not have to provide additional information or analysis, or conduct additional clinical trials, before receiving approval to market product candidates.

We have an orphan drug designation from the FDA for fostamatinib for the treatment of ITP and wAIHA, and for olutasidenib for the treatment of AML, but we may not be able to obtain additional orphan drug designations in the future, or maintain the orphan drug designations or exclusivity for the approved drugs for the treatment of respective indications, or we may be unable to maintain the benefits associated with orphan drug designations, including the potential for market exclusivity.

We have an orphan drug designation in the US for fostamatinib for the treatment of ITP and wAIHA, and for olutasidenib for the treatment of AML. Also, pralsetinib has orphan drug designations in the US for the treatment of adult patients with metastatic RET fusion-positive NSCLC as detected by an FDA-approved test, for the treatment of

83 85

[Table of Contents](#)

We may be unable to expand our product pipeline, which could limit our growth adult and revenue potential.

Our business pediatric patients 12 years of age and older with advanced or metastatic RET fusion-positive thyroid cancer who require systemic therapy and who are radioactive iodine-refractory (if radioactive iodine is focused on the development appropriate), and commercialization of novel therapies that significantly improve the lives of patients with hematologic disorders and cancer. In this regard, we are pursuing internal drug discovery efforts with the goal of identifying new product candidates to advance into clinical trials. Internal discovery efforts to identify new product candidates require substantial technical, financial and human resources. These internal discovery efforts may initially show promise in identifying potential product candidates, yet ultimately fail to yield product candidates for clinical development for a number of reasons. For example, potential product candidates may, on later stage clinical trial, be shown to have inadequate efficacy, harmful side effects, suboptimal pharmaceutical profiles or other characteristics suggesting that they are unlikely to be commercially viable products.

Apart from our internal discovery efforts, our strategy to expand our development pipeline is also dependent on our ability to successfully identify and acquire or in-license relevant product candidates. In July 2022, we entered into a license and transition services agreement with Forma (now Novo Nordisk) for an exclusive license to develop, manufacture and commercialize olutasidenib, a proprietary inhibitor of mIDH1, for any uses worldwide, including for the treatment of AML and other malignancies. On December 1, 2022, the FDA approved REZLIDHIA capsules for the treatment of adult and pediatric patients 12 years of age and older with R/R AML with a susceptible IDH1 mutations as detected by and FDA-approved test. REZLIDHIA is our second commercial product and is highly synergistic with our existing hematology-oncology focused commercial and medical affairs infrastructure. The in-licensing and acquisition of product candidates is a highly competitive area, and many other companies are pursuing the same advanced or similar product candidates to those that we may consider attractive. In particular, larger companies with more well-established and diverse revenue streams may have a competitive advantage over us due to their size, financial resources and more extensive clinical development and commercialization capabilities. Furthermore, companies that perceive us to be a competitor may be unwilling to assign or license rights to us. We may also be unable to in-license or acquire additional relevant product candidates on acceptable terms that would allow us to realize an appropriate return on our investment. If we are unable to develop suitable product candidates through internal discovery efforts, whether due to the impacts of a global pandemic or otherwise, or if we are unable to successfully obtain rights to additional suitable product candidates, our business and prospects for growth could suffer. Even if we succeed in our efforts to obtain rights to suitable product candidates, the competitive business environment may result in higher acquisition or licensing costs, and our investment in these potential products will remain subject to the inherent risks associated with

the development and commercialization of new medicines. In certain circumstances, we may also be reliant on the licensor for the continued development of the in-licensed technology and their efforts to safeguard their underlying intellectual property.

With respect to acquisitions, we may not be able to integrate the target company successfully into our existing business, maintain the key business relationships of the target, or retain key personnel of an acquired business. Furthermore, we could assume unknown or contingent liabilities or incur unanticipated expenses. Any acquisitions or investments made by us also could result in our spending significant amounts, issuing dilutive securities, assuming or incurring significant debt obligations and contingent liabilities, incurring large one-time expenses and acquiring intangible assets that could result in significant future amortization expense and significant write-offs, any of which could harm our operating results.

We have obtained orphan drug designation from the FDA for fostamatinib for the treatment of ITP and wAIHA, but we may not be able to obtain or maintain orphan drug designation or exclusivity for fostamatinib for the treatment of ITP, wAIHA or our other product candidates, or we may be unable to maintain the benefits associated with orphan drug designation, including the potential for market exclusivity.

We have obtained orphan drug designation in the US for fostamatinib for the treatment of ITP and wAIHA, metastatic RET-mutant medullary thyroid carcinoma who require systemic therapy. We may seek orphan drug designation for other product candidates in the future. Under the Orphan Drug Act, the FDA may grant orphan drug designation to a drug or biologic intended to treat a rare disease or condition, which is defined as one occurring in a patient population of fewer than 200,000 in the US, or a patient population greater than 200,000 in the US where there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the US. In the US, orphan drug designation entitles a party to financial incentives such as opportunities for grant funding towards clinical trial costs, tax advantages and user-fee waivers. In addition, if a product that has orphan drug designation subsequently receives the first FDA approval for the disease for which it has such designation, the product is entitled to orphan drug exclusivity, which means that the FDA may not approve any other applications, including a full NDA, to

[Table of Contents](#)

market the same drug for the same indication for seven years, except in limited circumstances, such as a showing of clinical superiority to the product with orphan drug exclusivity or where the manufacturer is unable to assure sufficient product quantity. At this time, we do not have nor will we seek to apply for orphan drug designation in the EU or the UK in the foreseeable future.

We cannot assure you that any future application for orphan drug designation with respect to any other product candidate will be granted. If we are unable to obtain orphan drug designation with respect to other product candidates in the US, we will not be eligible to obtain the period of market exclusivity that could result from orphan drug designation or be afforded the financial incentives associated with orphan drug designation. Even though we have received orphan drug designation for fostamatinib for the treatment of ITP and wAIHA in the US, we may not be the first to obtain marketing approval for the orphan-designated indication due to the uncertainties associated with developing pharmaceutical products or we might not maintain our orphan drug designation. In addition, exclusive marketing rights in the US for fostamatinib for the treatment of ITP, wAIHA or any future product candidate may be limited if we seek approval for an indication broader than the orphan-designated indication or may be lost if the FDA later determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantities of the product to meet the needs of patients with the rare disease or condition. Further, even if we obtain orphan drug exclusivity for a product, that exclusivity may not effectively protect the product from competition because different drugs with different active moieties can be approved for the same condition. Even after an orphan product is approved, the FDA can subsequently approve the same drug with the same active moiety for the same condition if the FDA concludes that the later drug is safer, more effective, or makes a major contribution to patient care. Orphan drug designation neither shortens the development time or regulatory review time of a drug nor gives the drug any advantage in the regulatory review or approval process.

In addition, Congress is considering updates to the orphan drug provisions of the FDCA in response to a recent recent 11th Circuit decision. Any changes to the orphan drug provisions could change our opportunities for, or likelihood of success in obtaining, orphan drug exclusivity and would materially adversely affect our business, financial condition, results of operations, cash flows and prospects.

Risks Related to Commercialization

Our prospects are highly dependent on our commercial products. To the extent that the commercial success of our products in the US is diminished or is not commercially successful, our business, financial condition and results of operations may be adversely affected, and the price of our common stock may decline.

TAVALISSE is our first drug that has been approved for sale in the US and Europe for patients with chronic ITP. REZLIDHIA is our second drug product which was recently approved by the FDA for the treatment of adult patients with R/R AML with susceptible IDH1 mutations as detected by an FDA-approved test, and began its commercialization in December 2022. We are focusing a significant portion of our activities and resources on our products, and we believe our prospects are highly dependent on, and a significant portion of the value of our company relates to, our ability to sustain successful commercialization of our products in the US. We have also entered into exclusive commercialization agreements with third parties to commercialize fostamatinib outside the US, and we plan to further enter partnership with existing or other third parties to commercialize fostamatinib and olutasidenib our products outside the US in the future.

Sustained successful commercialization of our products is subject to many risks and uncertainties, including the impact of a global pandemic on the successful commercialization in the US, as well as the successful commercialization efforts for our products through our collaborative partners. There are numerous examples of unsuccessful product launches and failures to meet high expectations of market potential, including by pharmaceutical companies with more experience and resources than us.

As we continue to build out our commercial team, there 86

[Table of Contents](#)

There are many factors that could cause the commercialization of our products to be unsuccessful, including a number of factors that are outside our control. The commercial success of our products depends on the extent to which patients and physicians accept and adopt our products to treat the related diseases. We also do not know how physicians, patients and payors will respond to our future price increases of our products. Physicians may not prescribe our products and patients may be unwilling to use our products if coverage is not provided or reimbursement is inadequate to cover a significant portion of the cost. Our products compete, and may in the

85

[Table of Contents](#)

future compete, with currently existing therapies, including generic drugs, and products currently under development. Our competitors, particularly large pharmaceutical companies, may deploy more resources to market, sell and distribute their products. If our efforts are not appropriately resourced to adequately promote our products, the commercial potential of our sales may be diminished. Additionally, any negative development for fostamatinib our products in clinical development in additional indications such as in the clinical trials of fostamatinib in COVID-19 patients, may adversely impact the commercial results commercialization and potential of fostamatinib. Thus, significant uncertainty remains regarding the commercial potential of fostamatinib our products.

Market acceptance of fostamatinib our products will depend on a number of factors, including:

- the timing of market introduction of the product as well as competitive products;
- the clinical indications for which the product is approved;
- acceptance by physicians, the medical community and patients of the product as a safe and effective treatment;
- potential future impacts, if any, due to the effects of a global pandemic and the global tensions arising from the Russian-Ukrainian war and Hamas-Israel war;

- the ability to distinguish safety and efficacy from existing, less expensive generic alternative therapies, if any;
- the convenience of prescribing, administrating and initiating patients on the product and the length of time the patient is on the product;
- the potential and perceived value and advantages of the product over alternative treatments;
- the cost of treatment in relation to alternative treatments, including any similar generic treatments;
- pricing and the availability of coverage and adequate reimbursement by third-party payors and government authorities;
- a positive HTA concluding that the product is cost-effective and the HTA bodies issuing a positive recommendation for the use of the product as a first or second line of treatment for the granted therapeutic indication;
- the prevalence and severity of adverse side effects; and
- the effectiveness of sales and marketing efforts.

If we are unable to sustain anticipated level of sales growth from our products, or if we fail to achieve anticipated product royalties and collaboration milestones, we may need to reduce our operating expenses, access other sources of cash or otherwise modify our business plans, which could have a negative impact on our business, financial condition and results of operations. For example, during 2021, we experienced lower than anticipated sales of our products due to continuing impacts of physician and patient access issues created by the COVID-19 pandemic. From time to time, our net product sales are negatively impacted by the decrease in level of inventories remaining at our distribution channels.

We also may not be successful entering into arrangements with third parties to sell and market one or more of our product candidates or may be unable to do so on terms that are favorable to us. We likely will have little control over such third parties, including Kissei's development and commercialization of fostamatinib in all indications in Japan,

87

[Table of Contents](#)

China, Taiwan, and the Republic of Korea, Grifols' commercialization of fostamatinib in Europe and Turkey, Medison for future commercialization of fostamatinib in Canada and Israel, and Knight for commercialization of fostamatinib in Latin America. As a consequence of our license agreements with Kissei, Grifols, Medison and Knight, we rely heavily upon their regulatory, commercial, medical affairs, market access and other expertise and resources for commercialization of fostamatinib in their respective territories outside of the US. We cannot control the amount of

86

[Table of Contents](#)

resources that our partners dedicate to the commercialization of fostamatinib, and our ability to generate revenues from the commercialization of fostamatinib by our partners depends on their ability to achieve market acceptance of fostamatinib in its approved indications in their respective territories.

Furthermore, foreign sales of fostamatinib by our partners could be adversely affected by the imposition of governmental controls, political and economic instability, outbreaks of pandemic diseases, such as the COVID-19 pandemic, trade restrictions or barriers and changes in tariffs and escalating global trade and political tensions. For example, the COVID-19 pandemic has resulted in increased travel restrictions and extended shutdowns of certain businesses in the US and around the world. If our collaborators are unable to successfully complete clinical trials, delay

commercialization of fostamatinib or do not invest the resources necessary to successfully commercialize fostamatinib in international territories where it has been approved, this could reduce the amount of revenue we are due to receive under these license agreements, resulting in harm to our business and operations. If we do not establish and maintain sales and marketing capabilities successfully, either on our own or in collaboration with third parties, we will not be successful in commercializing our product candidates.

Even if we, or any of our collaborative partners, are able to continue to commercialize our products or any product candidate that we, or they, develop, the product may become subject to unfavorable pricing regulations, third-party payor reimbursement practices or labeling restrictions, all of which may vary from country to country and any of which could harm our business.

The commercial success of any product for which we have obtained regulatory approval, or for which we obtain regulatory approval in the future will depend substantially on the extent to which the costs of our product or product candidates are or will be paid by third-party payors, including government health care programs and private health insurers. There is a significant trend in the health care industry by public and private payors to contain or reduce their costs, including by taking the following steps, among others: decreasing the portion of costs payors will cover, ceasing to provide full payment for certain products depending on outcomes, and/or not covering certain products at all. If payors implement any of the foregoing with respect to our products, it would have an adverse impact on our revenue and results of operations. If coverage is not available, or reimbursement is limited, we, or any of our collaborative partners, may not be able to successfully commercialize our products or any of our product candidates in some jurisdictions. Even if coverage is provided, the approved reimbursement amount may not be at a rate that covers our costs, including research, development, manufacture, sale and distribution. In the US, no uniform policy of coverage and reimbursement for products exists among third-party payors; therefore, coverage and reimbursement levels for products can differ significantly from payor to payor. As a result, the coverage determination process is often a time consuming and costly process that may require us to provide scientific, clinical or other support for the use of our products to each payor separately, with no assurance that coverage and adequate reimbursement will be applied consistently or obtained in the first instance.

There is significant uncertainty related to third-party payor coverage and reimbursement of newly approved drugs. Marketing approvals, pricing and reimbursement for new drug products vary widely from country to country. Some countries require approval of the sale price of a drug before it can be marketed, which could delay market entry (or, if pricing is not approved, we may be unable to sell at all in a country where we have received regulatory approval for a product. In many countries, the pricing review period begins after marketing or product licensing approval is granted. In some countries, the proposed pricing for a drug must be approved before it may be lawfully **marketed**). In addition, authorities in some countries impose additional obligations, such as HTAs, which assess the performance of a drug in comparison with its cost. The outcome of HTA assessments is judged on a national basis and some payors may not reimburse the use of our products or may reduce the rate of reimbursement for our products and as a result, revenue from such products may decrease.

In some foreign markets, prescription pharmaceutical pricing remains subject to continuing governmental control even after initial approval is granted. As a result, we, or any of our collaborative partners, might obtain marketing approval for a product in a particular country, but then be subject to price regulations that delay commercial launch of the product, possibly for lengthy time periods, which may negatively impact the revenues we are able to generate from

88

[Table of Contents](#)

the sale of the product in that country. In particular, we cannot predict to what extent the effects of a global pandemic, depending on its scale and duration, may **continue to** disrupt global healthcare systems and access to our products or result in a widespread loss of individual health insurance coverage due to unemployment, a shift from commercial payor coverage to government payor coverage, or an increase in demand for patient assistance and/or free drug programs, any of which would adversely affect access to and demand for our products and our net sales. Adverse pricing limitations

87

[Table of Contents](#)

may also hinder our ability or the ability of any future collaborators to recoup our or their investment in one or more product candidates, even if our product candidates obtain marketing approval. Further, even if favorable coverage and reimbursement status is attained for one or more products for which we or our collaborative partners receive regulatory approval, less favorable coverage policies and reimbursement rates may be implemented in the future.

Patients who are provided medical treatment for their conditions generally rely on third-party payors to reimburse all or part of the costs associated with their treatment. Therefore, our ability, and the ability of any of our collaborative partners, to successfully commercialize our products or any of our product candidates will depend in part on the extent to which coverage and adequate reimbursement for these products and related treatments will be available from third-party payors.

Additionally, the labeling ultimately approved for any of our product candidates for which we have or may obtain regulatory approval may include restrictions on their uses and may be subject to ongoing FDA or international regulatory authority requirements governing the labeling, packaging, storage, distribution, safety surveillance, advertising, promotion, record-keeping and reporting of safety and other post-market information. If we or any of our collaborative partners do not timely obtain or comply with the labeling approval by the FDA or international regulatory authorities on any of our product candidates, it may delay or inhibit our ability to successfully commercialize our products and generate revenues.

If we are unable to successfully market and distribute our products and retain experienced commercial personnel, our business will be substantially harmed.*

We currently have limited experience in marketing and selling pharmaceutical products. As a result, we will be required to continuously expend significant time and resources to maintain a sales force that is credible and compliant with applicable laws in marketing our products. In addition, we must continually train our sales force to ensure that an appropriate and compliant message about our products is being delivered. If we are unable to effectively train our sales force and equip them with compliant and effective materials, including medical and sales literature to help them appropriately inform and educate health care providers regarding the potential benefits and proper administration of our products, our efforts to successfully commercialize our products could be put in jeopardy, which would negatively impact our ability to generate product revenues.

We have established our distribution, sales, marketing and market access capabilities, all of which will be necessary to successfully commercialize our products. As a result, we will be required to expend significant time and resources to market, sell, and distribute our products to hematologists and hematologist-oncologists. There is no guarantee that the marketing strategies we have developed, or the distribution, sales, marketing and market access capabilities that we have developed will be successful. Particularly, we are dependent on third-party logistics, specialty pharmacies and distribution partners in the distribution of our products. If they are unable to perform effectively or if they do not provide efficient distribution of the medicine to patients, our business may be harmed.

Maintaining our sales, marketing, market access and product distribution capabilities requires significant resources, and there are numerous risks involved with managing our commercial team, including our potential inability to successfully train, retain and incentivize adequate numbers of qualified and effective sales and marketing personnel. We are also competing for talent with numerous commercial and pre-commercial-stage oncology-focused biotechnology companies seeking to build out their commercial organizations, as well as other large pharmaceutical organizations that have extensive, well-funded and more experienced sales and marketing operations, and we may be unable to maintain or adequately scale our commercial organization as a result of such competition. If we cannot maintain effective sales, marketing, market access and product distribution capabilities, we may be unable to realize the commercial potential of our products. Also, to the extent that the commercial opportunities for our products grow over time, we may not properly judge the requisite size and experience of our current commercialization teams or the level of distribution necessary to market and sell our products, which could have an adverse impact on our business, financial condition and results of operations.

We may not be able to successfully develop or commercialize our product candidates if problems arise in the clinical testing and approval process.

The activities associated with the research, development and commercialization of **fostamatinib** our products and other product candidates in our pipeline must undergo extensive clinical trials, which can take many years and require substantial expenditures, subject to extensive regulation by the FDA and other regulatory agencies in the US and by comparable authorities in other countries. The process of obtaining regulatory approvals in the US and other foreign jurisdictions is expensive, and lengthy, if approval is obtained at all.

Our clinical trials may fail to produce results satisfactory to the FDA or regulatory authorities in other jurisdictions. The regulatory process also requires preclinical testing, and data obtained from preclinical and clinical activities are susceptible to varying interpretations. The FDA has substantial discretion in the approval process and may refuse to approve any NDA or sNDA and decide that our data is insufficient for approval and require additional preclinical, clinical or other studies. Varying interpretations of the data obtained from preclinical and clinical testing could delay, limit or prevent regulatory approval of **fostamatinib** our products for any individual, additional indications. For example, in June 2022, we announced that the top-line results from our Phase 3 trial in wAIHA did not demonstrate statistical significance in the primary efficacy endpoint of durable hemoglobin response in the overall study population. While we conducted an in-depth analysis of these data to better understand differences in patient characteristics and outcomes and submitted these findings to the FDA, in October 2022, we announced that we received guidance from the **FDA's** **FDA** of these findings. Based on the result of the trial and the guidance from the FDA, we did not file an sNDA for wAIHA.

Due to the effects of a global pandemic, it It is also possible that we could experience delays in the timing of our interactions with regulatory authorities due to absenteeism by governmental employees or the diversion of regulatory authority efforts and attention to approval of other therapeutics, or other **activities related to COVID-19 or other** public health emergencies **including a global pandemic**, which could delay or limit our ability to make planned regulatory submissions or develop and commercialize our product candidates on anticipated timelines.

In addition, delays or rejections may be encountered based upon changes in regulatory policy for product approval during the period of product development and regulatory agency review, which may cause delays in the approval or rejection of an application for **fostamatinib** our products or for our other product candidates.

Commercialization of our product candidates depends upon successful completion of extensive preclinical studies and clinical trials to demonstrate their safety and efficacy for humans. Preclinical testing and clinical development are long, expensive and uncertain processes.

In connection with clinical trials of our product candidates, we may face the following risks among others:

- the product candidate may not prove to be effective;
- the product candidate may cause harmful side effects;
- the clinical results may not replicate the results of earlier, smaller trials;
- we or third parties with whom we collaborate, may be significantly impacted by force majeure events;
- we, or the FDA or similar foreign regulatory authorities, may delay, terminate or suspend the trials;
- our results may not be statistically significant;
- patient recruitment and enrollment may be slower than expected;
- patients may drop out of the trials or otherwise not enroll; and
- regulatory and clinical trial requirements, interpretations or guidance may change.

We do not know whether we will be permitted to undertake clinical trials of potential products beyond the trials already concluded and the trials currently in process. It will take us or our collaborative partners several years to complete any such testing, and failure can occur at any stage of testing. Interim results of trials do not necessarily predict

final results, and acceptable results in early trials may not be repeated in later trials. A number of companies in the pharmaceutical industry, including biotechnology companies, have suffered significant setbacks in advanced clinical trials, even after achieving promising results in earlier trials.

Further, evolving FDA standards may cause additional setbacks. In 2023, FDA published guidance documents and a final rule which all concern clinical trial requirements. In June 2023, FDA published a draft guidance, E6(R3) Good Clinical Practice, which seeks to unify standards for clinical trial data for the International Council for Harmonisation of Technical Requirements of Pharmaceuticals for Human Use member countries and regions. In August 2023, FDA published a guidance document, Informed Consent, Guidance for IRBs, Clinical Investigators, and Sponsors, which supersedes past guidance and finalizes draft guidance on informed consent. Further, in December 2023, FDA published a final rule, Institutional Review Board Waiver or Alteration of Informed Consent for Minimal Risk Clinical Investigations, which allows exceptions from informed consent requirements when a clinical investigation poses no more than minimal risk to the human subject and includes appropriate safeguards to protect the rights, safety, and welfare of human subjects.

Alterations to clinical trial requirements may affect recruitment and retention of patients and may hinder or delay a clinical trial. Further, changes to data requirements may cause FDA or comparable foreign regulatory authorities to disagree with data from preclinical studies or clinical trials, and may require further studies. Changes to trial requirements or trial data may increase costs and delay product development.

General Risk Factors

Global economic conditions could adversely impact our business.*

Deterioration in the macroeconomic economy or financial services industry could lead to losses or defaults by our customers or suppliers, which in turn, could have a material adverse effect on our current and/or projected business operations and results of operations and financial condition. The global credit financial markets and financial markets economy are currently, and have from time to time experienced extreme volatility and disruptions, including severely diminished liquidity and credit availability, rising interest and inflation rates, declines in consumer confidence, declines in economic growth, increases in unemployment rates and uncertainty about economic stability. More

Any significant deterioration in the US economy would likely affect the operation of our business and ability to raise capital. In addition, US debt ceiling and budget deficit concerns have increased the possibility of additional credit-rating downgrades and economic slowdowns, or a recession in the US. Although US lawmakers passed legislation to raise the federal debt ceiling on multiple occasions, ratings agencies have lowered or threatened to lower the long-term sovereign credit rating on the US. The impact of this or any further downgrades to the US government's sovereign credit rating or its perceived creditworthiness could adversely affect the US and global financial markets and economic conditions.

The global financial markets and economy may also be adversely affected by the current or anticipated impact of military conflict, including the ongoing Russian-Ukrainian war, and the Hamas-Israel war, terrorism or other geopolitical events. Sanctions imposed by the US and other countries in response to such conflicts, including the Russian-Ukrainian war and the Hamas-Israel war, may also adversely impact the financial markets and the global economy, and any economic countermeasures by the affected countries or others could exacerbate market and economic instability.

The US government has indicated its intent to alter its approach to international trade policy and in some cases to renegotiate, or potentially terminate, certain existing bilateral or multi-lateral trade agreements and treaties with foreign countries. In addition, the US government has initiated or is considering imposing tariffs on certain foreign goods. Related to this action, certain foreign governments, including China, have instituted or are considering imposing tariffs on certain US goods. It remains unclear what the US Administration or foreign governments will or will not do with respect to tariffs or other international trade agreements and policies. A trade war or other governmental action related to tariffs or international trade agreements or policies has the potential to disrupt our research activities, affect our suppliers and/or the US or global economy or certain sectors thereof and, thus, could adversely impact our businesses.

Bank failures or other events affecting financial institutions could adversely impact our liquidity and other business.

Financial institutions have recently experienced, and may experience in the future, industry instability and failures which have led to disruptions in access to bank deposits or lending commitments. In 2023, the closures of Silicon Valley Bank (SVB) and Signature Bank and their placement into receivership with the Federal Deposit Insurance Corporation (FDIC), as well as the FDIC's seizure and sale of First Republic Bank, created bank-specific and broader financial institution liquidity risk and concerns. On March 12, 2023, federal regulators announced that the FDIC would complete its resolution of SVB in a manner that fully protects all depositors. On March 27, 2023, First Citizens Bank (FCB) announced that it has entered into an agreement with FDIC to purchase all of the asset and liabilities of SVB. Customers of SVB automatically become customers of FCB following the acquisition.

We maintain a depository relationship with SVB/FCB. The amount of our cash held on deposit with SVB/FCB was not material with respect to our total cash, cash equivalents and short-term investments. All of our cash deposits with SVB/FCB are accessible to us, and we do not anticipate any losses with respect to such funds. Since the March 2023 financial institution failure, there has been a heightened risk and greater focus on the potential failures of other banks in the future. If these banks fail in the future, we may not be able to immediately (or ever) recover our cash in excess of the FDIC insured limits which would adversely impact our operating liquidity and could negatively impact our operations, results of operations and financial performance. Although we believe our exposure is limited, if in the future any of the financial institutions that we maintain depository or lending relationships were to be placed into receivership, we may be unable to access such funds to meet our working capital requirements. In addition, if any of our customers, suppliers or other parties with whom we conduct business are unable to access funds, such parties' ability to pay their obligations to us or to enter into new commercial arrangements requiring additional payments to us could be adversely affected. Although we assess our banking and customer relationships as we believe necessary or appropriate, our access to funding sources and other credit arrangements in amounts adequate to finance or capitalize our current and projected future business operations could be significantly impacted by factors that affect us, the financial institutions with which we have credit agreement or arrangements directly, or the financial services industry or economy in general.

In addition, any further deterioration in the US economy would likely affect the operation of our business Shareholder activism and ability to raise capital. In addition, US debt ceiling and budget deficit concerns have increased the possibility of additional credit-rating downgrades and economic slowdowns, or a recession in the US. Although US lawmakers passed legislation to raise the federal debt ceiling on multiple occasions, ratings agencies have lowered or threatened to lower the long-term sovereign credit rating on the US. The impact of this or any further downgrades to the US government's sovereign credit rating or its perceived creditworthiness could adversely affect the US and global financial markets and economic conditions.

The financial markets and the global economy may also be adversely affected by the current or anticipated impact of military conflict, including the ongoing conflict between Russia and Ukraine and the Hamas and Israel, terrorism or other geopolitical events. Sanctions imposed by the US and other countries in response to such conflicts, including the one in Ukraine, may also adversely impact the financial markets and the global economy, and any economic countermeasures by the affected countries or others could exacerbate market and economic instability.

The US government has indicated its intent to alter its approach to international trade policy and in some cases to renegotiate, or potentially terminate, certain existing bilateral or multi-lateral trade agreements and treaties with foreign countries. In addition, the US government has initiated or is considering imposing tariffs on certain foreign goods. Related to this action, certain foreign governments, including China, have instituted or are considering imposing tariffs on certain US goods. It remains unclear what the US Administration or foreign governments will or will not do with respect to tariffs or other international trade agreements and policies. A trade war or other governmental action related to tariffs or international trade agreements or policies has the potential to disrupt our research activities, affect our

Publicly traded companies have increasingly become subject to campaigns by our stakeholders, including investors, and more recently regulatory organizations advocating corporate actions such as actions related to environment, social and governance Environmental Social Governance (ESG) matters, impacts of climate change, financial restructuring, increased borrowing, dividends, share repurchases and even sales of assets or the entire company. Responding to proxy contests and other actions by such activist investors or others in the future could be costly and time-consuming, disrupt our operations and divert the attention of our Board of Directors and senior management from the pursuit of our business strategies, which could adversely affect our results of operations and financial condition.

There's a growing emphasis from select investors, regulators, and other stakeholders on corporate responsibility, particularly regarding ESG factors. Some investors and advocacy groups utilize these factors to shape investment strategies, potentially opting out of investing in our company if they perceive our corporate responsibility policies as insufficient. Third-party providers offering corporate responsibility ratings and reports have surged to meet rising investor demand, with numerous organizations evaluating companies on ESG matters, and these evaluations receive widespread attention. A low ESG or sustainability rating from such providers could lead certain investors to overlook our common stock in favor of competitors. Institutional investors, in particular, use these ratings to compare companies, and any perceived lag in our ESG efforts might prompt voting decisions or other actions to hold our board accountable. Furthermore, evolving assessment criteria for corporate responsibility practices may raise expectations, compelling us to undertake costly initiatives to meet new standards. Failure to meet these evolving criteria could reinforce the perception of inadequate corporate responsibility policies. Non-compliance could also lead to reputational damage if our procedures or standards fall short of stakeholder expectations.

Securities-related class action lawsuits and/or derivative lawsuits have often been brought against companies, including biotechnology and biopharmaceutical companies, that experience volatility in the market price of their securities. It is possible that such lawsuit will be filed, or allegations from stockholders with this matter. Such lawsuits and any other related lawsuits are subject to inherent uncertainties, and the actual defense and disposition costs will depend upon many unknown factors. The outcome of such lawsuits is necessarily uncertain. We could be forced to expend significant resources in the defense of the pending lawsuits and any additional lawsuits, and we may not prevail.

[Table of Contents](#)

Anti-takeover provisions in our charter documents and under Delaware law may make an acquisition of us, which may be beneficial to our stockholders, more difficult.

Provisions of our amended and restated certificate of incorporation and bylaws, as well as provisions of Delaware law, could make it more difficult for a third party to acquire us, even if doing so would benefit our stockholders. These provisions:

- establish that members of the board of directors may be removed only for cause upon the affirmative vote of stockholders owning a majority of our capital stock;
- authorize the issuance of "blank check" preferred stock that could be issued by our board of directors to increase the number of outstanding shares and thwart a takeover attempt;
- limit who may call a special meeting of stockholders;
- prohibit stockholder action by written consent, thereby requiring all stockholder actions to be taken at a meeting of our stockholders;
- establish advance notice requirements for nominations for election to the board of directors or for proposing matters that can be acted upon at stockholder meetings;
- provide for a board of directors with staggered terms; and
- provide that the authorized number of directors may be changed only by a resolution of our board of directors.

In addition, Section 203 of the Delaware General Corporation Law (DGCL), which imposes certain restrictions relating to transactions with major stockholders, may discourage, delay or prevent a third party from acquiring us.

Our bylaws designate a state or federal court located within the State of Delaware as the sole and exclusive forum for substantially all disputes between us and our stockholders, which could limit our stockholders' ability to obtain a favorable judicial forum for disputes with us or our current or former directors, officers, stockholders, or other employees.

Our bylaws provide that, unless we consent in writing to the selection of an alternative forum, the Court of Chancery of the State of Delaware shall be the sole and exclusive forum for (i) any derivative action or proceeding brought on behalf of us under Delaware law, (ii) any action asserting a claim of breach of a fiduciary duty **owed** by any current or former director, officer, or other employee of **Rigel** **ours** that is **owed** to us or our stockholders, (iii) any action asserting a claim against us or any of our directors, officers, or other employees arising pursuant to any provision of the DGCL or our amended and restated certificate of incorporation and bylaws (as either may be amended from time to time), (iv) any action asserting a claim against us governed by the internal affairs doctrine, or (v) any other action asserting an "internal corporate claim," as defined under Section 115 of the DGCL. The forgoing provisions do not apply to any claims arising under the Securities Act and, unless we consent in writing to the selection of an alternative forum, the federal district courts of the United States will be the sole and exclusive forum for resolving any action asserting a claim arising under the Securities Act.

91

[Table of Contents](#)

These choice of forum provisions may limit a stockholder's ability to bring a claim in a judicial forum that it finds favorable for disputes with us or any of our current or former directors, officers, or other employees, which may discourage lawsuits with respect to such claims. There is uncertainty as to whether a court would enforce such provisions, and the enforceability of similar choice of forum provisions in other companies' charter documents has been challenged in legal proceedings. It is possible that a court could find these types of provisions to be inapplicable or unenforceable, and if a court were to find the choice of forum provision to be inapplicable or unenforceable in an action, we may incur additional costs associated with resolving such action in other jurisdictions, which could harm our business, results of operations, and financial condition.

93

[Table of Contents](#)

Increasing use of social media could give rise to liability and may harm our business.

We and our employees are increasingly utilizing social media tools and our website as a means of communication. Despite our efforts to monitor evolving social media communication guidelines and comply with applicable laws, regulations and national and EU codes of conduct, there is risk that the unauthorized use of social media by us or our employees to communicate about our products or business, sharing of publications in unintended audiences in other jurisdictions, or any inadvertent promotional activity or disclosure of material, nonpublic information through these means, may cause us to be found in violation of applicable laws and regulations, which may give rise to liability and result in harm to our business. In addition, there is also risk of inappropriate disclosure of sensitive information, which could result in significant legal and financial exposure and reputational damages that could potentially have an adverse impact on our business, financial condition and results of operations. Furthermore, negative posts or comments about us or our products on social media could seriously damage our reputation, brand image and goodwill.

Our research and development efforts will be seriously jeopardized if we are unable to attract and retain key employees and relationships.

Our success depends We are highly dependent on the continued contributions commercial, research and development, clinical, business development, financial and legal expertise of our executive officers, as well as the other principal management members of our management. We expect to continue hiring and **scientific** retaining qualified personnel which is critical to our success. Replacing key employees and executive officers may be difficult and may take an extended period of time because of the limited number of individuals in our industry with the breadth of skills and experience required to successfully develop, gain regulatory approval of and commercialize drugs. Competition to hire from this limited pool is intense, and we may be unable to hire, train, retain or motivate these key personnel on our ability to develop acceptable terms given the competition among numerous pharmaceutical and maintain important relationships with leading academic institutions, scientists and biotechnology companies in the face of intense competition for such similar personnel. In particular, our research programs depend on our ability to attract and retain highly skilled chemists, other scientists, and development, regulatory and clinical personnel. If we lose the services of any of our key personnel, our research and development efforts could be seriously and adversely affected. Our employees can terminate their employment with us at any time.

Our facilities are located near known earthquake fault zones, and the occurrence of an earthquake or other catastrophic disaster could cause damage to our facilities and equipment, which could require us to cease or curtail operations.

Our facilities are located in the San Francisco Bay Area near known earthquake fault zones and are vulnerable to significant damage from earthquakes. We are also vulnerable to damage from other types of disasters, including fires, floods, power loss, communications failures and similar events. If any disaster were to occur, our ability to operate our business at our facilities would be seriously, or potentially completely, impaired, and our research could be lost or destroyed. In addition, the unique nature of our research activities and of much of our equipment could make it difficult for us to recover from a disaster. The insurance we maintain may not be adequate to cover our losses resulting from disasters or other business interruptions.

Item 2. Unregistered Sales of Equity Securities and Use of Proceeds

None.

Item 3. Defaults Upon Senior Securities

None.

Item 4. Mine Safety Disclosures

Not applicable.

92 94

[Table of Contents](#)

Item 5. Other Information

Securities Trading Plans of Directors and Executive Officers

During the three months ended **September 30, 2023** **March 31, 2024**, none of our directors or executive officers adopted or terminated any contract, instruction or written plan for the purchase or sale of our securities that was intended to satisfy the affirmative defense conditions of Rule 10b5-1(c) or any "non-Rule 10b5-1 trading arrangement" as defined in Item 408 of Regulation S-K under the Securities Exchange Act of 1934, as amended.

95

[Table of Contents](#)

Item 6. Exhibits

The exhibits listed on the accompanying index to exhibits are filed or incorporated by reference (as stated therein) as part of this Quarterly Report on Form 10-Q.

Exhibit Number	Description of Document
3.1	Amended and Restated Certificate of Incorporation. (1)
3.2	Amended and Restated Bylaws. (2)
3.3	Certificate of Amendment to the Amended and Restated Certificate of Incorporation. (3)
4.1	Form of warrant to purchase shares of common stock. (4)
4.2	Specimen Common Stock Certificate. (5)
10.1#^	Executive Severance Plan, as amended. Asset Purchase Agreement with Blueprint Medicines Corporation, dated February 22, 2024.
10.2#^	Amendment 1 No. 2 to the License and Collaboration Agreement with Eli Lilly and Company. Company, dated March 11, 2024.
10.3#^	Fourth Amendment to the Credit and Security Agreement with MidCap Financial Trust, dated April 11, 2024.
10.4#	Rigel Pharmaceuticals, Inc. Inducement Plan, as amended.
31.1#	Certification required by Rule 13a-14(a) or Rule 15d-14(a) of the Exchange Act.
31.2#	Certification required by Rule 13a-14(a) or Rule 15d-14(a) of the Exchange Act.
32.1*#	Certification required by Rule 13a-14(b) or Rule 15d-14(b) of the Exchange Act and Section 1350 of Chapter 63 of Title 18 of the United States Code (18 U.S.C. 1350).
101.INS	Inline XBRL Instance Document – the instance document does not appear in the Interactive Data File because its XBRL tags are embedded within the Inline XBRL document.
101.SCH	Inline XBRL Taxonomy Extension Schema Document.
101.CAL	Inline XBRL Taxonomy Extension Calculation Linkbase Document.
101.LAB	Inline XBRL Taxonomy Extension Labels Linkbase Document.
101.PRE	Inline XBRL Taxonomy Extension Presentation Linkbase Document.
101.DEF	Inline XBRL Taxonomy Extension Definition Linkbase Document.
104	Cover Page Interactive Data File (formatted as Inline XBRL and contained in Exhibit 101)

Filed herewith

^ Certain marked information has been omitted redacted from this exhibit pursuant to Item 601(b)(1)(iv) of Regulation S-K because it is both not material and is the type that the registrant treats as private and confidential. An unredacted copy of this exhibit will be furnished supplementally to the SEC upon request.

* The certifications attached as Exhibit 32.1 accompany this Quarterly Report on Form 10-Q pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, and shall not be deemed "filed" by the registrant for purposes of Section 18 of the Exchange Act.

- (1) Filed as an exhibit to Rigel's Current Report on Form 8-K dated June 24, 2003, and incorporated herein by reference.
- (2) Filed as an exhibit to Rigel's Current Report on Form 8-K dated November 3, 2022, and incorporated herein by reference.

93

[Table of Contents](#)

- (3) Filed as an exhibit to Rigel's Current Report on Form 8-K dated May 18, 2018, and incorporated herein by reference.
- (4) Filed as an exhibit to Rigel's Registration Statement on Form S-1 (No. 333-45864), filed on September 15, 2000, as amended, and incorporated herein by reference.
- (5) Filed as an exhibit to Rigel's Current Report on Form 10-K dated June 24, 2003, and incorporated herein by reference.

94 96

[Table of Contents](#)

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, as amended, the registrant has duly caused this report to be signed on its behalf by the undersigned, thereunto duly authorized.

RIGEL PHARMACEUTICALS, INC.

By: /s/ RAUL R. RODRIGUEZ
Raul R. Rodriguez
Chief Executive Officer
(Principal Executive Officer)
Date: November 7, 2023

By: /s/ DEAN L. SCHORNO
Dean L. Schorno
Chief Financial Officer
(Principal Financial Officer)
Date: November 7, 2023

Exhibit 10.1**RIGEL PHARMACEUTICALS, INC.****AMENDED AND RESTATED EXECUTIVE SEVERANCE PLAN****Section 1. Introduction.**

The Rigel Pharmaceuticals, Inc. Executive Severance Plan (the "Plan") is established effective January 24, 2018, and amended and restated effective August 10, 2023. The purpose of the Plan is to provide for the payment of severance benefits to certain eligible executives of Rigel Pharmaceuticals, Inc. (the "Company") who meet the eligibility criteria set forth in Section 2(a) below. This Plan document also is the Summary Plan Description for the Plan.

Section 2. Eligibility For Benefits.

(a) General Rules. Subject to the requirements of the Plan, the Company will grant the severance benefits described in Section 3 to Eligible Employees.

(1) Definition of "Eligible Employee." For purposes of this Plan, an Eligible Employee is an employee of the Company serving as (a) an Executive Officer (as defined in 3b-7 of the General Rules and Regulations promulgated under the Securities Exchange Act of 1934, as amended, and qualifying for treatment as an officer under Section 16 of the Security Exchange Act of 1934, as amended), or (b) a member of the executive committee leading the roles in any one of human resources, research, medical, legal, corporate development, or commercial (whether or not an Executive Officer), at the time he or she suffers a "Qualifying Termination" (as defined below). The Plan Administrator shall make the determination of whether an employee is an Eligible Employee, and such determination shall be binding and conclusive on all persons. Temporary employees and independent contractors are not eligible for severance benefits under the Plan.

(2) Obligations of Eligible Employees. In order to receive any benefits under the Plan:

(i) the Eligible Employee must remain on the job and satisfactorily provide services to the Company until his or her date of termination;

(ii) the Eligible Employee must execute and return to the Company a general waiver and release in substantially the form attached hereto as Exhibit A, Exhibit B or Exhibit C, as applicable, within the time frame set forth therein (the "Release") and such release must become effective in accordance with its terms but not later than the 60th day following the termination of employment (with the Company having the authority, in its discretion, to modify the form of the required release to comply with applicable law and to determine the form of the required release, which may be incorporated into a termination agreement or other agreement with the Eligible Employee) and notwithstanding the payment schedules set forth in Appendix A and Appendix B, no benefits will be paid prior to the effective date of the Release (the "Release Effective Date") but rather on the first regular payroll pay day following the Release Effective Date, the Company will pay the Eligible Employee the benefits the Eligible Employee would otherwise have received on or prior to the Release Effective Date but for the delay in payment related to the effectiveness of the Release, with the balance of the benefits being paid as originally scheduled; and

(iii) the Eligible Employee must remain in compliance with his or her continuing obligations to the Company, including obligations under his or her Employee Proprietary Information and Inventions Assignment Agreement (such form,

or any similar form, the "Proprietary Agreement").

(b) Exceptions to Benefit Entitlement. An employee who otherwise is an Eligible Employee will not receive benefits under the Plan (or will receive reduced benefits under the Plan) in the following

circumstances, as determined by the Company in its sole discretion:

(1) The employee is covered by any other severance or separation pay plan, policy or practice of the Company or has executed an individually negotiated employment contract or agreement with the Company relating to severance benefits, in either case with respect to severance benefits payable upon an event that constitutes a Qualifying Termination (used herein as defined herein), and such agreement, plan, policy or practice is in effect on his or her termination date. In such case, the employee's severance benefit upon a Qualifying Termination, if any, shall be governed by the terms of such other agreement, plan, policy or practice and shall be governed by this Plan only to the extent that (i) the employee elects to waive and release all claims and rights the employee has to severance pay or benefits upon a Qualifying Termination under such other agreement, plan, policy, or practice or (ii) the reduction pursuant to Section 3(c) below does not entirely eliminate benefits under this Plan.

(2) The employee's employment terminates other than as a result of a Qualifying Termination (including a termination for Cause prior to the effective date of a previously scheduled Qualifying Termination, a termination as a result of death or disability, or the employee voluntarily terminates employment with the Company other than as a Resignation for Good Reason). Voluntary terminations include, but are not limited to, resignation, retirement, failure to return from leave of absence on the scheduled date and/or termination in order to accept employment with another entity (including but not limited to any entity that is wholly or partly owned (directly or indirectly) by the Company or an affiliate of the Company.)

(3) The employee has not signed an enforceable Proprietary Agreement covering the employee's period of employment with the Company (and with any predecessor) and does not confirm in writing that he or she is and shall remain subject to the terms of that Proprietary Agreement.

(4) Following notice of a Qualifying Termination, the employee's behavior rises to level of Cause for termination.

(c) An involuntary termination without "Cause" means an involuntary termination of an employee's employment by the Company other than as a result of death or disability and other than for one of the following reasons:

(1) an intentional action or intentional failure to act by the employee that was performed in bad faith and to the material detriment of the business of the Company or an Employer;

(2) an employee's intentional refusal or intentional failure to act in accordance with any lawful and reasonable order of his or her superiors that has not been cured within ten (10) days after written notice from the Company, or that has caused irreparable damage incapable of cure;

(3) an employee's habitual or gross neglect of the duties of employment that has not been cured within ten (10) days after written notice from the Company, or that has caused irreparable damage incapable of cure;

(4) an employee's indictment, charge, or conviction of a felony or any crime involving moral turpitude, or participation in any act of theft or dishonesty, in each case, that has had or could reasonably be expected to have a material detrimental effect on the business of the Company; or

(5) an employee's violation of any material provision of the Proprietary Agreement or violation of any material provision of any other written Company policy or procedure.

(d) A "Change of Control" means the consummation, in a single transaction or in a series of related transactions, of any one or more of the following events:

(1) a sale, lease or other disposition of all or substantially all of the assets of the Company, other than a sale, lease or other disposition of all or substantially all of the assets of the Company to an

entity, more than fifty percent (50%) of the combined voting power of the voting securities of which are owned by stockholders of the Company in substantially the same proportions as their ownership of the outstanding voting securities of the Company immediately prior to such sale, lease or other disposition;

(2) a merger, consolidation or similar transaction involving (directly or indirectly) the Company and, immediately after the consummation of such merger, consolidation or similar transaction, the stockholders of the Company immediately prior thereto do not own, directly or indirectly, either (A) outstanding voting securities representing more than fifty percent (50%) of the combined outstanding voting power of the surviving entity in such merger, consolidation or similar transaction or (B) more than fifty percent (50%) of the combined outstanding voting power of the parent of the surviving entity in such merger, consolidation or similar transaction, in each case in substantially the same proportions as their ownership of the outstanding voting securities of the Company immediately prior to such transaction; or

(3) any "Exchange Act Person" becomes the owner, directly or indirectly, of securities of the Company representing more than fifty percent (50%) of the combined voting power of the Company's then outstanding securities other than by virtue of a merger, consolidation or similar transaction.

(e) A "COC Qualifying Termination" means an involuntary termination without Cause or a Resignation for Good Reason and in either case provided such termination is a separation from service" (as such term is defined in Section 1.409A-1(h) of the Treasury Regulations) and such termination occurs on or within eighteen (18) months following the effective date of the Change of Control.

(f) An "Exchange Act Person" means any natural person, entity or "group" (within the meaning of Section 13(d) or 14(d) of the Securities Exchange Act of 1934, as amended), except that "Exchange Act Person" shall not include (1) the Company or any subsidiary of the Company, (2) any employee benefit plan of the Company or any subsidiary of the Company or any trustee or other fiduciary holding securities under an employee benefit plan of the Company or any subsidiary of the Company, (3) an underwriter temporarily holding securities pursuant to an offering of such securities, (4) an entity owned, directly or indirectly, by the stockholders of the Company in substantially the same proportions as their ownership of stock of the Company; or (5) any natural person, entity or "group" (within the meaning of Section 13(d) or 14(d) of the Securities Exchange Act of 1934, as amended) that, as of the effective date of this Plan, is the owner, directly or indirectly, of securities of the Company representing more than fifty percent (50%) of the combined voting power of the Company's then outstanding securities.

(g) A "Non-COC Qualifying Termination" means an involuntary termination without Cause or a Resignation for Good Reason and in either case provided such termination is a separation from service" (as such term is defined in Section 1.409A-1(h) of the Treasury Regulations) and such termination occurs before, or more than eighteen (18) months following the effective date of the Change of Control.

(h) A "Qualifying Termination" means either a COC Qualifying Termination, or Non-COC Qualifying Termination.

(i) A "Resignation for Good Reason" means the Eligible Employee has resigned from all positions he or she then holds with the Company (or any successor thereto), and:

(1) one of the following actions has been taken:

(i) there is a material diminution of Eligible Employee's authority, including but not limited to decision-making authority, duties, or responsibilities;

(ii) there is a material reduction in the Eligible Employee's annual base compensation (including the base salary and target bonus opportunity), where material is considered greater than 5%;

(iii) the Eligible Employee is required to relocate his or her primary work

location to a facility or location that would increase the Eligible Employee's one way commute distance by more than twenty (20) miles from the Eligible Employee's primary work location as of immediately prior to such change;

(iv) A material diminution in the authority, duties, or responsibilities of the supervisor to whom the Eligible Employee is required to report, including a requirement that the Eligible Employee report to a corporate officer or employee instead of reporting directly to the board of directors of a corporation (or similar governing body with respect to an entity other than a corporation);

(v) A material diminution in the budget over which the Eligible Employee retains authority;

(vi) the Eligible Employee is required, as a condition to continued service, to enter into any agreement with the Company or a successor thereto regarding confidentiality, non-competition, non-solicitation or other similar restrictive covenant that is materially more restrictive than under the Proprietary Agreement;

(vii) the Company materially breaches its obligations under this Plan or any then-effective written employment agreement with the Eligible Employee; or

(viii) any acquirer, successor or assign of the Company fails to assume and perform, in all material respects, the obligations of the Company hereunder; and

(2) the Eligible Employee provides written notice to the Company's General Counsel within the 60-day period immediately following the occurrence of any such action specified in clause (1) above; and

(3) such action is not remedied by the Company within thirty (30) days following the Company's receipt of such written notice; and

(4) the Eligible Employee's resignation is effective no later than sixty (60) days after the expiration of such thirty (30) day cure period.

Section 3. Amount Of Benefit.

(a) Severance Benefits. Subject to the terms and conditions of the Plan, the severance benefits that shall be provided to Eligible Employees under the Plan are set forth in Appendix A ("COC Qualifying Termination"), and Appendix B ("Non-COC Qualifying Termination").

(b) Additional Benefits. Notwithstanding the foregoing, the Company may, in its sole discretion, authorize benefits in an amount in addition to those benefits set forth in Section 3(a) to an Eligible Employee. The provision of any such benefits to an Eligible Employee shall in no way obligate the Company to provide such benefits to any other Eligible Employee or to any other employee, even if similarly situated. Receipt of benefits under this Plan pursuant to such exceptions may be subject to a covenant of confidentiality and non-disclosure.

(c) Certain Reductions. Except with respect to any bonus amount that may be payable to the Eligible Employee upon such Eligible Employee's Qualifying Termination pursuant to the terms of the Company's bonus policy, the Company shall reduce an Eligible Employee's severance benefits under this Plan, in whole or in part, by any other severance benefits, pay in lieu of notice, or other similar benefits payable to the Eligible Employee by the Company in connection with the Eligible Employee's Qualifying Termination, including but not limited to any payments or benefits that are due pursuant to (i) any other severance plan, policy or practice, or any individually negotiated employment contract or agreement with the Company relating to severance benefits, in each case, as is in effect on the Eligible Employee's termination date, (ii) any applicable legal requirement, including, without limitation, the Worker Adjustment and Retraining Notification Act (the "WARN Act"), or (iii) any Company policy or practice providing for the Eligible Employee to remain on the payroll without being in active service for a limited period of time after being given notice of the termination of the Eligible Employee's employment. The benefits provided under this Plan are intended to satisfy, to the greatest extent possible, any and all statutory obligations that may arise out of an Eligible Employee's termination of employment, and the Plan Administrator shall so construe and implement the terms of the Plan. In the Company's sole discretion, such

reductions may be applied on a retroactive basis, with severance benefits previously paid being recharacterized as payments pursuant to the Company's statutory obligation.

(d) Parachute Payments. If any payment or benefit an Eligible Employee would receive pursuant to a Change of Control from the Company or otherwise ("Payment") would (i) constitute a "parachute payment"

within the meaning of Section 280G of the Internal Revenue Code of 1986, as amended (the "Code"), and (ii) but for this sentence, be subject to the excise tax imposed by Section 4999 of the Code (the "Excise Tax"), then such Payment shall be equal to the Reduced Amount. The "Reduced Amount" shall be either (x) the largest portion of the Payment that would result in no portion of the Payment being subject to the Excise Tax or (y) the largest portion, up to and including the total, of the Payment, whichever amount, after taking into account all applicable federal, state and local employment taxes, income taxes, and the Excise Tax (all computed at the highest applicable marginal rate), results in the Eligible Employee's receipt, on an after-tax basis, of the greater economic benefit notwithstanding that all or some portion of the Payment may be subject to the Excise Tax. If a reduction in payments or benefits constituting "parachute payments" is necessary so that the Payment equals the Reduced Amount, the reduction shall occur in the manner that results in the greatest economic benefit for the Eligible Employee. If more than one method of reduction will result in the same economic benefit, the items so reduced will be reduced pro rata.

In the event it is subsequently determined by the Internal Revenue Service that some portion of the Reduced Amount as determined pursuant to clause (x) in the preceding paragraph is subject to the Excise Tax, the Eligible Employee agrees to promptly return to the Company a sufficient amount of the Payment so that no portion of the Reduced Amount is subject to the Excise Tax. For the avoidance of doubt, if the Reduced Amount is determined pursuant to clause (y) in the preceding paragraph, the Eligible Employee will have no obligation to return any portion of the Payment pursuant to the preceding sentence.

Unless the Eligible Employee and the Company agree on an alternative accounting firm or law firm, the accounting firm engaged by the Company for general tax compliance purposes as of the day prior to the effective date of the Change of Control shall perform the foregoing calculations. If the accounting firm so engaged by the Company is serving as accountant or auditor for the individual, entity or group effecting the Change of Control, the Company shall appoint a nationally recognized accounting or law firm to make the determinations required hereunder. The Company shall bear all expenses with respect to the determinations by such accounting or law firm required to be made hereunder.

The Company shall use commercially reasonable efforts to cause the accounting or law firm engaged to make the determinations hereunder to provide its calculations, together with detailed supporting documentation, to the Eligible Employee and the Company within fifteen (15) calendar days after the date on which the Eligible Employee's right to a Payment is triggered (if requested at that time by the Eligible Employee or the Company) or such other time as requested by the Eligible Employee or the Company.

(e) Code Section 409A. If the Company (or, if applicable, the successor entity thereto) determines that the payments and benefits provided under the Plan (the "Plan Payments") constitute "deferred compensation" under Code Section 409A (together, with any state law of similar effect, "Section 409A") and an Eligible Employee is a "specified employee" of the Company or any successor entity thereto, as such term is defined in Section 409A(a)(2)(B)(i) (a "Specified Employee"), then, solely to the extent necessary to avoid the incurrence of the adverse personal tax consequences under Section 409A, the timing of the Plan Payments shall be delayed as follows: on the earliest to occur of (1) the date that is six months and one day after a "separation from service" (as such term is defined in Section 1.409A-1(h) of the Treasury Regulations), and (2) the date of the Eligible Employee's death (such earliest date, the "Delayed Initial Payment Date"), and the Company (or the successor entity thereto, as applicable) shall then (i) pay to the Eligible Employee a lump sum amount equal to the sum of the Plan Payments that the Eligible Employee would otherwise have received through the Delayed Initial Payment Date if the commencement of the payment of the Plan Payments had not been delayed pursuant to this Section 3(e) and

(ii) commence paying the balance of the Plan Payments in accordance with the applicable payment schedules set forth in Appendix A and Appendix B. Prior to the imposition of any delay on the Plan Payments as set forth above, it is intended that (A) each installment of the Plan Payments provided in Appendix A and Appendix B be regarded as a separate "payment" for purposes of Treasury Regulations Section 1.409A-2(b)(2)(i), (B) all Plan Payments provided in Appendix A and Appendix B satisfy, to the greatest extent possible, the exemptions from the application of Section 409A provided under Treasury Regulations Sections 1.409A-1(b)(4) and 1.409A-1(b)(9)(iii), and (C) the Plan Payments consisting of COBRA premiums also satisfy, to the greatest extent possible, the

exemption from the application of Section 409A provided under Treasury Regulations Section 1.409A-1(b)(9)(v). In addition to the above, to the extent required to comply with Section 409A and the applicable regulations and guidance issued thereunder, if the applicable permitted period for Executive to execute (and not revoke) the applicable Release spans two calendar years, payment of the applicable severance payments shall not commence until the beginning of the second calendar year.

Section 4. Company Property.

(a) Return of Company Property. An Eligible Employee will not be entitled to any severance benefits under the Plan unless and until the Eligible Employee returns all Company Property. For this purpose, "Company Property" means all paper and electronic company documents (and all copies thereof) created and/or received by the Eligible Employee during his or her period of employment with the Company and other Company Property which the Eligible Employee had in his or her possession or control at any time, including, but not limited to, Company and/or Employer files, notes, drawings records, plans, forecasts, reports, studies, analyses, proposals, agreements, financial information, research and development information, sales and marketing information, operational and personnel information, specifications, code, software, databases, computer-recorded information, tangible property and equipment (including, but not limited to, leased vehicles, computers, computer equipment, software programs, facsimile machines, mobile telephones, servers), credit and calling cards, entry cards, identification badges and keys; and any materials of any kind which contain or embody any proprietary or confidential information of the Company and/or an Employer (and all reproductions thereof in whole or in part). As a condition to receiving benefits under the Plan, Eligible Employees must not make or retain copies, reproductions or summaries of any such Company Property. However, an Eligible Employee is not required to return his or her personal copies of documents evidencing the Eligible Employee's hire, termination, compensation, benefits and equity awards and any other documentation received as a shareholder of the Company.

(b) Transition of Work. An Eligible Employee will not be entitled to any severance benefits under the Plan unless and until the Eligible Employee (1) has satisfactorily transitioned his or her work and information concerning his or her work to the Company to the extent reasonably requested in writing by the Company and (2) has provided the Company with all logins, passwords, passcodes and similar information created by the Eligible Employee for documents, email and electronic files that the Eligible Employee created or used on Company systems.

Section 5. Time Of Payment and Form Of Benefit.

Except as otherwise provided in Section 3, all severance benefits under the Plan shall be paid at the time and in the form provided in Appendix A and Appendix B following the Eligible Employee's satisfaction of all of the requirements under the Plan. All payments under the Plan will be subject to applicable withholding for federal, state and local taxes. If an Eligible Employee is indebted to the Company at his or her termination date, the Company reserves the right to offset any severance benefits under the Plan by the amount of such indebtedness. Additionally, if an Eligible Employee is subject to withholding for taxes related to any non-Plan benefits, the Company may offset any severance benefits under the Plan by the amount of such withholding taxes. However, payments under the Plan will not be subject to any other deductions such as, but not limited to, 401(k) plan contributions and/or 401(k) loan repayments or other employee benefit and benefit plan contributions.

Section 6. Right To Interpret Plan; Amendment and Termination.

(a) Exclusive Discretion. The Plan Administrator is the Company. As Plan Administrator, the Company is the named fiduciary charged with the responsibility for administering the Plan. The Plan Administrator shall have the exclusive discretion and authority to establish rules, forms, and procedures for the administration of the Plan and to construe and interpret the Plan and to decide any and all questions of fact, interpretation, definition, computation or administration arising in connection with the operation of the Plan, including, but not limited to, the eligibility to participate in the Plan and amount of benefits paid under the Plan. The Plan Administrator may delegate any or all of its administrative duties to an officer of the Company and any such delegation shall convey with it the full discretionary authority of the Plan Administrator to carry out the delegated duties. The Company

the Plan Administrator shall indemnify and hold harmless any person to whom it delegated its responsibilities; *provided, however,* such person does not act with gross negligence or willful misconduct. The rules, interpretations, computations and other actions of the Plan Administrator or its delegate shall be binding and conclusive on all persons.

(b) Termination; Amendment. The Company reserves the right to amend or terminate this Plan (including the exhibits and appendices hereto) and the benefits provided hereunder at any time prior to a Change of Control of the Company; *provided, however,* that no such amendment or termination shall affect the right to any unpaid benefit of any Eligible Employee whose Qualifying Termination date has occurred prior to amendment of the Plan.

(c) Any purported amendment or termination of this Plan (and the exhibits and appendices hereto) upon or following a Change of Control of the Company will not be effective as to any Eligible Employee who has not consented, in writing, to such amendment or termination. Any action amending or terminating the Plan shall be in writing and executed by a duly authorized executive officer of the Company.

Section 7. No Implied Employment Contract.

The Plan shall not be deemed (i) to give any employee or other person any right to be retained in the employ of the Company or (ii) to interfere with the right of the Company to discharge any employee or other person at any time, with or without cause, which right is hereby reserved.

Section 8. Legal Construction.

This Plan is intended to be governed by and shall be construed in accordance with the Employee Retirement Income Security Act of 1974 ("ERISA") and, to the extent not preempted by ERISA, the laws of the State of California (without regard to principles of conflict of laws).

Section 9. Claims, Inquiries And Appeals.

(a) Applications for Benefits and Inquiries. Any application for benefits, inquiries about the Plan or inquiries about present or future rights under the Plan must be submitted to the Plan Administrator in writing by an applicant (or his or her authorized representative). The Plan Administrator is:

Rigel Pharmaceuticals, Inc. Attn: General Counsel
611 Gateway Boulevard, Suite 900 South San Francisco, CA
94080

(b) Denial of Claims. In the event that any application for benefits is denied in whole or in part, the Plan Administrator must provide the applicant with written or electronic notice of the denial of the application, and of the applicant's right to review the denial. Any electronic notice will comply with the regulations of the U.S. Department of Labor. The notice of denial will be set forth in a manner designed to be understood by the applicant and will include the following:

- (1) the specific reason or reasons for the denial;
- (2) references to the specific Plan provisions upon which the denial is based;
- (3) a description of any additional information or material that the Plan Administrator needs to complete the review and an explanation of why such information or material is necessary; and
- (4) an explanation of the Plan's review procedures and the time limits applicable to such procedures, including a statement of the applicant's right to bring a civil action under Section 502(a) of ERISA following a denial on review of the claim, as described in Section 10(d) below.

This notice of denial will be given to the applicant within ninety (90) days after the Plan Administrator receives the application, unless special circumstances require an extension of time, in which case, the Plan Administrator has up to an additional ninety (90) days for processing the application. If an extension of time for processing is required, written notice of the extension will be furnished to the applicant before the end of the initial ninety (90) day period.

This notice of extension will describe the special circumstances necessitating the additional time and the date by which the Plan Administrator is to render its decision on the application.

(c) Request for a Review. Any person (or that person's authorized representative) for whom an application for benefits is denied, in whole or in part, may appeal the denial by submitting a request for a review to the Plan Administrator within sixty (60) days after the application is denied. A request for a review shall be in writing and shall be addressed to:

Rigel Pharmaceuticals, Inc. Attn: General Counsel
611 Gateway Boulevard, Suite 900 South San Francisco, CA
94080

A request for review must set forth all of the grounds on which it is based, all facts in support of the request and any other matters that the applicant feels are pertinent. The applicant (or his or her representative) shall have the opportunity to submit (or the Plan Administrator may require the applicant to submit) written comments, documents, records, and other information relating to his or her claim. The applicant (or his or her representative) shall be provided, upon request and free of charge, reasonable access to, and copies of,

all documents, records and other information relevant to his or her claim. The review shall take into account all comments, documents, records and other information submitted by the applicant (or his or her representative) relating to the claim, without regard to whether such information was submitted or considered in the initial benefit determination.

(d) Decision on Review. The Plan Administrator will act on each request for review within sixty (60) days after receipt of the request, unless special circumstances require an extension of time (not to exceed an additional sixty (60) days), for processing the request for a review. If an extension for review is required, written notice of the extension will be furnished to the applicant within the initial sixty (60) day period. This notice of extension will describe the special circumstances necessitating the additional time and the date by which the Plan Administrator is to render its decision on the review. The Plan Administrator will give prompt, written or electronic notice of its decision to the applicant. Any electronic notice will comply with the regulations of the U.S. Department of Labor. In the event that the Plan Administrator confirms the denial of the application for benefits in whole or in part, the notice will set forth, in a manner calculated to be understood by the applicant, the following:

- (1) the specific reason or reasons for the denial;
- (2) references to the specific Plan provisions upon which the denial is based;
- (3) a statement that the applicant is entitled to receive, upon request and free of charge, reasonable access to, and copies of, all documents, records and other information relevant to his or her claim; and
- (4) a statement of the applicant's right to bring a civil action under Section 502(a) of ERISA.

(e) Rules and Procedures. The Plan Administrator will establish rules and procedures, consistent with the Plan and with ERISA, as necessary and appropriate in carrying out its responsibilities in reviewing benefit claims. The Plan Administrator may require an applicant who wishes to submit additional information in connection with an appeal from the denial of benefits to do so at the applicant's own expense.

(f) Exhaustion of Remedies. No legal action for benefits under the Plan may be brought until the applicant (i) has submitted a written application for benefits in accordance with the procedures described by Section

10(a) above, (ii) has been notified by the Plan Administrator that the application is denied, (iii) has filed a written request for a review of the application in accordance with the appeal procedure described in Section 10(c) above, and (iv) has been notified that the Plan Administrator has denied the appeal. Notwithstanding the foregoing, if the Plan Administrator does not respond to an applicant's claim or appeal within the relevant time limits specified in this Section 10, the applicant may bring legal action for benefits under the Plan pursuant to Section 502(a) of ERISA.

Section 10. Basis Of Payments To And From Plan.

The Plan shall be unfunded, and all benefits under the Plan shall be paid only from the general assets of the Company. An Eligible Employee's right to receive payments under the Plan is no greater than that of the Company's unsecured general creditors. Therefore, if the Company were to become insolvent, the Eligible Employee might not receive benefits under the Plan.

Section 11. Other Plan Information.

(a) Employer and Plan Identification Numbers. The Employer Identification Number assigned to the Company (which is the "Plan Sponsor" as that term is used in ERISA) by the Internal Revenue Service is 94-3248524. The Plan Number assigned to the Plan by the Plan Sponsor pursuant to the instructions of the Internal Revenue Service is 510.

(b) Ending Date for Plan's Fiscal Year and Type of Plan. The date of the end of the fiscal year for the purpose of maintaining the Plan's records is December 31. The Plan is a welfare benefit plan.

(c) Agent for the Service of Legal Process. The agent for the service of legal process with respect to the Plan is:

Rigel Pharmaceuticals, Inc. Attn: General Counsel
611 Gateway Boulevard, Suite 900 South San Francisco, CA
94080

(d) **Plan Sponsor and Administrator.** The Plan Sponsor and the "Plan Administrator" of the Plan is:

Rigel Pharmaceuticals, Inc. Attn: General Counsel
611 Gateway Boulevard, Suite 900 South San Francisco, CA
94080

The Plan Sponsor's and Plan Administrator's telephone number is (650) 624-1100 and facsimile number is (650) 624-1101.

Section 12. Statement Of ERISA Rights.

Participants in this Plan are entitled to certain rights and protections under ERISA. If you are an Eligible Employee, you are considered a participant in the Plan and, under ERISA, you are entitled to:

(a) Receive Information About Your Plan and Benefits

(1) Examine, without charge, at the Plan Administrator's office and at other specified locations, such as worksites, all documents governing the Plan and a copy of the latest annual report (Form 5500 Series), if applicable, filed by the Plan with the U.S. Department of Labor and available at the Public Disclosure Room of the Employee Benefits Security Administration;

(2) Obtain, upon written request to the Plan Administrator, copies of documents governing the operation of the Plan and copies of the latest annual report (Form 5500 Series), if applicable, and an updated (as necessary) Summary Plan Description. The Administrator may make a reasonable charge for the copies; and

(3) Receive a summary of the Plan's annual financial report, if applicable. The Plan Administrator is required by law to furnish each participant with a copy of this summary annual report.

(b) Prudent Actions by Plan Fiduciaries. In addition to creating rights for Plan participants, ERISA imposes duties upon the people who are responsible for the operation of the employee benefit plan. The people who operate the Plan, called "fiduciaries" of the Plan, have a duty to do so prudently and in the interest of you and other Plan participants and beneficiaries. No one, including your employer, your union or any other person, may fire you or otherwise discriminate against you in any way to prevent you from obtaining a Plan benefit or exercising your rights under ERISA.

(c) Enforce Your Rights. If your claim for a Plan benefit is denied or ignored, in whole or in part, you have a right to know why this was done, to obtain copies of documents relating to the decision without charge, and to appeal any denial, all within certain time schedules as set forth in detail in Section 10 herein.

Under ERISA, there are steps you can take to enforce the above rights. For instance, if you request a copy of Plan documents or the latest annual report from the Plan, if applicable, and do not receive them within 30 days, you may file suit in a Federal court and you are not required to follow the claims procedure set forth in Section 10 herein. In such a case, the court may require the Plan Administrator to provide the materials and pay you up to \$110 a day until you receive the materials, unless the materials were not sent because of reasons beyond the control of the Plan Administrator.

If you have completed the claims and appeals procedure described in Section 10 and have a claim for benefits which is denied or ignored, in whole or in part, you may file suit in a state or Federal court.

If you are discriminated against for asserting your rights, you may seek assistance from the U.S. Department of Labor, or you may file suit in a Federal court. The court will decide who should pay court costs and legal fees. If you are successful, the court may order the person you have sued to pay these costs and

fees. If you lose, the court may order you to pay these costs and fees, for example, if it finds your claim is frivolous.

(d) Assistance with Your Questions. If you have any questions about the Plan, you should contact the Plan Administrator. If you have any questions about this statement or about your rights under ERISA, or if you need assistance in obtaining documents from the Plan Administrator, you should contact the nearest office of the Employee Benefits Security Administration, U.S. Department of Labor, listed in your telephone directory or the Division of Technical Assistance and Inquiries, Employee Benefits Security Administration, U.S. Department of Labor, 200 Constitution Avenue N.W., Washington, D.C. 20210. You may also obtain certain

publications about your rights and responsibilities under ERISA by calling the publications hotline of the Employee Benefits Security Administration or accessing its website at <http://www.dol.gov/ebsa/>.

Section 13. GENERAL PROVISIONS.

(a) Notices. Any notice, demand or request required or permitted to be given by either the Company or an Eligible Employee pursuant to the terms of this Plan shall be in writing and shall be deemed given when delivered personally or deposited in the U.S. mail, with postage prepaid, and addressed to the parties, in the case of the Company, at the address set forth in Section 12(d) and, in the case of an Eligible Employee, at the address as set forth in the Company's employment file maintained for the Eligible Employee as previously furnished by the Eligible Employee or such other address as a party may request by notifying the other in writing.

(b) Transfer and Assignment. The rights and obligations of an Eligible Employee under this Plan may not be transferred or assigned without the prior written consent of the Company. This Plan shall be binding upon any person who is a successor by merger, acquisition, consolidation or otherwise to the business formerly carried on by the Company without regard to whether or not such person or entity actively assumes the

obligations hereunder. Following a Change of Control, any references to the "Company" in this Plan shall be deemed to be references also to any successor to the company.

(c) Waiver. Any party's failure to enforce any provision or provisions of this Plan shall not in any way be construed as a waiver of any such provision or provisions, nor prevent any party from thereafter enforcing each and every other provision of this Plan. The rights granted the parties herein are cumulative and shall not constitute a waiver of any party's right to assert all other legal remedies available to it under the circumstances.

(d) Severability. Should any provision of this Plan be declared or determined to be invalid, illegal or unenforceable, the validity, legality and enforceability of the remaining provisions shall not in any way be affected or impaired.

(e) Section Headings. Section headings in this Plan are included for convenience of reference only and shall not be considered part of this Plan for any other purpose.

Section 14. Execution

To record the adoption of the Plan as set forth herein, effective as of January 24, 2018, as most recently amended and restated on August 10, 2023, Rigel Pharmaceuticals, Inc. has caused its duly authorized officer to execute the same this 10th day of August, 2023.

Rigel Pharmaceuticals, Inc.

By: /s/Raymond Furey

Title: EVP, General Counsel, Corporate Secretary

Exhibit A

RELEASE AGREEMENT

I understand and agree completely to the terms set forth in the Rigel Pharmaceuticals, Inc. Executive Severance Plan (the "Plan").

I understand that this Release, together with the Plan, constitutes the complete, final and exclusive embodiment of the entire agreement between the Company, affiliates of the Company and me with regard to the subject matter hereof. I am not relying on any

promise or representation by the Company or the Employers that is not expressly stated therein. Certain capitalized terms used in this Release are defined in the Plan.

I hereby confirm my obligations under my Proprietary Agreement with the Company and/or the Employer.

Except as otherwise set forth in this Release, I hereby generally and completely release the Company, the Employers, and their current and former directors, officers, employees, stockholders, shareholders, partners, agents, attorneys, predecessors, successors, parent and subsidiary entities, insurers, affiliates, and assigns (collectively, the "**Released Parties**") from any and all claims, liabilities and obligations, both known and unknown, that arise out of or are in any way related to events, acts, conduct, or omissions occurring prior to my signing this Agreement (collectively, the "**Released Claims**"). The Released Claims include, but are not limited to: (1) all claims arising out of or in any way related to my employment with the Company, the Employers or their affiliates, or the termination of that employment; (2) all claims related to my compensation or benefits, including salary, bonuses, commissions, vacation pay, expense reimbursements, severance pay, fringe benefits, stock, stock options, or any other ownership interests in the Company, the Employers, or their affiliates; (3) all claims for breach of contract, wrongful termination, and breach of the implied covenant of good faith and fair dealing; (4) all tort claims, including claims for fraud, defamation, emotional distress, and discharge in violation of public policy; and (5) all federal, state, and local statutory claims, including claims for discrimination, harassment, retaliation, attorneys' fees, or other claims arising under the federal Civil Rights Act of 1964 (as amended), the federal Americans with Disabilities Act of 1990, the federal Age Discrimination in Employment Act of 1967 (as amended) ("**ADEA**"), the federal Employee Retirement Income Security Act of 1974 (as amended), and the California Fair Employment and Housing Act (as amended). Notwithstanding the foregoing, the following are not included in the Released Claims (the "**Excluded Claims**"):

(1) any rights or claims for indemnification I may have pursuant to any written indemnification agreement with the Company to which I am a party, the charter, bylaws, or operating agreements of the Company, or under applicable law; or (2) any rights which are not waivable as a matter of law. In addition, I understand that nothing in this Release limits my ability to file a charge or complaint with the Equal Employment Opportunity Commission, the Department of Labor, the National Labor Relations Board, the Occupational Safety and Health Administration, the Securities and Exchange Commission or any other federal, state or local governmental agency or commission ("**Government Agencies**"). I further understand that this Release does not limit my ability to communicate with any Government Agencies or otherwise participate in any investigation or proceeding that may be conducted by any Government Agency, including providing documents or other information, without notice to the Company and/or the Employer. While this Release does not limit my right to receive an award for information provided to the Securities and Exchange Commission, I understand and agree that, to maximum extent permitted by law, I am otherwise waiving any and all rights I may have to individual relief based on any claims that I have released and any rights I have waived by signing this Release. I hereby represent and warrant that, other than the Excluded Claims, I am not aware of any claims I have or might have against any of the Released Parties that are not included in the Released Claims.

I acknowledge that I am knowingly and voluntarily waiving and releasing any rights I may have under the ADEA. I also acknowledge that the consideration given for the Released Claims is in addition to anything of value to which I was already entitled. I further acknowledge that I have been advised by this writing, as required by the ADEA, that: (a) the Released Claims do not apply to any rights or claims that arise after the date I sign this Release;

(b) I should consult with an attorney prior to signing this Release (although I may choose voluntarily not to do so); (c) I have twenty-one (21) days to consider this Release (although I may choose to voluntarily to sign it sooner); (d) I have seven (7) days following the date I sign this Release to revoke the Release by providing written notice to an

officer of the Company; and (e) the Release will not be effective until the date upon which the revocation period has expired unexercised, which will be the eighth day after I sign this Release ("**Effective Date**").

I acknowledge that I have read and understand Section 1542 of the California Civil Code which reads as follows: "**A general release does not extend to claims which the creditor does not know or suspect to exist in his or her favor at the time of executing the release, which if known by him or her must have materially affected his or her settlement with the debtor.**" I hereby expressly waive and relinquish all rights and benefits under that section and any law of any jurisdiction of similar effect with respect to my release of any claims hereunder.

I hereby represent that I have been paid all compensation owed and for all hours worked, I have received all the leave and leave benefits and protections for which I am eligible, and I have not suffered any on-the-job injury for which I have not already filed a workers' compensation claim.

I hereby agree not to disparage the Company, the Employers, or their officers, directors, employees, shareholders or agents, in any manner likely to be harmful to them or their business, business reputation, or personal reputation; provided, however, that I will respond accurately and fully to any question, inquiry or request for information when required by legal process or in connection with a government investigation. In addition, I understand that nothing in this Release is intended to prohibit or restrain me in any manner from making disclosures that are protected under the whistleblower provisions of federal or state law or regulation.

I acknowledge that to become effective, I must sign and return this Release to the Company so that it is received not later than twenty-one (21) days following the date it is provided to me, and I must not revoke it thereafter.

Employee

Name: Date:

Exhibit B
RELEASE AGREEMENT

I understand and agree completely to the terms set forth in the Rigel Pharmaceuticals, Inc. Executive Severance Plan (the "Plan").

I understand that this Release, together with the Plan, constitutes the complete, final and exclusive embodiment of the entire agreement between the Company, affiliates of the Company and me with regard to the subject matter hereof. I am not relying on any promise or representation by the Company or the Employers that is not expressly stated therein. Certain capitalized terms used in this Release are defined in the Plan.

I hereby confirm my obligations under my Proprietary Agreement with the Company and/or the Employer.

Except as otherwise set forth in this Release, I hereby generally and completely release the Company, the Employers, and their current and former directors, officers, employees, stockholders, shareholders, partners, agents, attorneys, predecessors, successors, parent and subsidiary entities, insurers, affiliates, and assigns (collectively, the "**Released Parties**") from any and all claims, liabilities and obligations, both known and unknown, that arise out of or are in any way related to events, acts, conduct, or omissions occurring prior to my signing this Agreement (collectively, the "**Released Claims**"). The Released Claims include, but are not limited to: (1) all claims arising out of or in any way related to my employment with the Company, the Employers or their affiliates, or the termination of that employment; (2) all claims related to my compensation or benefits, including salary, bonuses, commissions, vacation pay, expense reimbursements, severance pay, fringe benefits, stock, stock options, or any other ownership interests in the Company, the Employers, or their affiliates; (3) all claims for breach of contract, wrongful termination, and breach of the implied covenant of good faith and fair dealing; (4) all tort claims, including claims for fraud, defamation, emotional distress, and discharge in violation of public policy; and (5) all federal, state, and local statutory claims, including claims for discrimination, harassment, retaliation, attorneys' fees, or other claims arising under the federal Civil Rights Act of 1964 (as amended), the federal Americans with Disabilities Act of 1990, the federal Age Discrimination in Employment Act of 1967 (as amended) ("**ADEA**"), the federal Employee Retirement Income Security Act of 1974 (as amended), and the California Fair Employment and Housing Act (as amended). Notwithstanding the foregoing, the following are not included in the Released Claims (the "**Excluded Claims**"): (1) any rights or claims for indemnification I may have pursuant to any written indemnification agreement with the Company to which I am a party, the charter, bylaws, or operating agreements of the Company, or under applicable law; or (2) any rights which are not waivable as a matter of law. In addition, I understand that nothing in this Release limits my ability to file a charge or complaint with the Equal Employment Opportunity Commission, the Department of Labor, the National Labor Relations Board, the Occupational Safety and Health Administration, the Securities and Exchange Commission or any other federal, state or local governmental agency or commission ("**Government Agencies**"). I further understand that this Release does not limit my ability to communicate with any Government Agencies or otherwise participate in any investigation or proceeding that may be conducted by any Government Agency, including providing documents or other information, without notice to the Company and/or the Employer. While this Release does not limit my right to receive an award for information provided to the Securities and Exchange Commission, I understand and agree that, to maximum extent permitted by law, I am otherwise waiving any and all rights I may have to individual relief based on any claims that I have released and any rights I have waived by signing this Release. I hereby represent and warrant that, other than the

Excluded Claims, I am not aware of any claims I have or might have against any of the Released Parties that are not included in the Released Claims.

I acknowledge that I am knowingly and voluntarily waiving and releasing any rights I may have under the ADEA. I also acknowledge that the consideration given for the Released Claims is in addition to anything of value to which I was already entitled. I further acknowledge that I have been advised by this writing, as required by the ADEA, that:

(a) the Released Claims do not apply to any rights or claims that arise after the date I sign this Release; (b) I should consult with an attorney prior to signing this Release (although I may choose voluntarily not to do so); (c) I have forty- five (45) days to consider this Release (although I may choose to voluntarily to sign it sooner); (d) I have seven

(7) days following the date I sign this Release to revoke the Release by providing written notice to an officer of the Company; and (e) the Release will not be effective until the date upon which the revocation period has expired unexercised, which will be the eighth day after I sign this Release ("**Effective Date**").

I have received with this Release all of the information required by the ADEA (under 29 U.S.C. § 626(f)(1)(H)), including without limitation a detailed list of the job titles and ages of all employees who were terminated in this group termination and the ages of all employees of the Company in the same job classification or organizational unit who were not terminated, along with information on the eligibility factors used to select employees for the group termination and any time limits applicable to this group termination program.

I acknowledge that I have read and understand Section 1542 of the California Civil Code which reads as follows: "**A general release does not extend to claims which the creditor does not know or suspect to exist in his or her favor at the time of executing the release, which if known by him or her must have materially affected his or her settlement with the debtor.**" I hereby expressly waive and relinquish all rights and benefits under that section and any law of any jurisdiction of similar effect with respect to my release of any claims hereunder.

I hereby represent that I have been paid all compensation owed and for all hours worked, I have received all the leave and leave benefits and protections for which I am eligible, and I have not suffered any on-the-job injury for which I have not already filed a workers' compensation claim.

I hereby agree not to disparage the Company, the Employers, or their officers, directors, employees, shareholders or agents, in any manner likely to be harmful to them or their business, business reputation, or personal reputation; *provided, however*, that I will respond accurately and fully to any question, inquiry or request for information when required by legal process or in connection with a government investigation. In addition, I understand that nothing in this Release is intended to prohibit or restrain me in any manner from making disclosures that are protected under the whistleblower provisions of federal or state law or regulation.

I acknowledge that to become effective, I must sign and return this Release to the Company so that it is received not later than forty-five (45) days following the date it is provided to me, and I must not revoke it thereafter.

Employee

Name: Date:

Exhibit C

RELEASE AGREEMENT

I understand and agree completely to the terms set forth in the Rigel Pharmaceuticals, Inc. Executive Severance Plan (the "Plan").

I understand that this Release, together with the Plan, constitutes the complete, final and exclusive embodiment of the entire agreement between the Company, affiliates of the Company and me with regard to the subject matter hereof. I am not relying on any

promise or representation by the Company or the Employers that is not expressly stated therein. Certain capitalized terms used in this Release are defined in the Plan.

I hereby confirm my obligations under my Proprietary Agreement with the Company and/or the Employer.

Except as otherwise set forth in this Release, I hereby generally and completely release the Company, the Employers, and their current and former directors, officers, employees, stockholders, shareholders, partners, agents, attorneys, predecessors, successors, parent and subsidiary entities, insurers, affiliates, and assigns (collectively, the "**Released Parties**") from any and all claims, liabilities and obligations, both known and unknown, that arise out of or are in any way related to events, acts, conduct, or omissions occurring prior to my signing this Agreement (collectively, the "**Released Claims**"). The Released Claims include, but are not limited to: (1) all claims arising out of or in any way related to my employment with the Company, the Employers or their affiliates, or the termination of that employment; (2) all claims related to my compensation or benefits, including salary, bonuses, commissions, vacation pay, expense reimbursements, severance pay, fringe benefits, stock, stock options, or any other ownership interests in the Company, the Employers, or their affiliates; (3) all claims for breach of contract, wrongful termination, and breach of the implied covenant of good faith and fair dealing; (4) all tort claims, including claims for fraud, defamation, emotional distress, and discharge in violation of public policy; and (5) all federal, state, and local statutory claims, including claims for discrimination, harassment, retaliation, attorneys' fees, or other claims arising under the federal Civil Rights Act of 1964 (as amended), the federal Americans with Disabilities Act of 1990, and the California Fair Employment and Housing Act (as amended). Notwithstanding the foregoing, the following are not included in the Released Claims (the "**Excluded Claims**"): (1) any rights or claims for indemnification I may have pursuant to any written indemnification agreement with the Company to which I am a party, the charter, bylaws, or operating agreements of the Company, or under applicable law; or (2) any rights which are not waivable as a matter of law. In addition, I understand that nothing in this Release limits my ability to file a charge or complaint with the Equal Employment Opportunity Commission, the Department of Labor, the National Labor Relations Board, the Occupational Safety and Health Administration, the Securities and Exchange Commission or any other federal, state or local governmental agency or commission ("**Government Agencies**"). I further understand that this Release does not limit my ability to communicate with any Government Agencies or otherwise participate in any investigation or proceeding that may be conducted by any Government Agency, including providing documents or other information, without notice to the Company and/or the Employer. While this Release does not limit my right to receive an award for information provided to the Securities and Exchange Commission, I understand and agree that, to maximum extent permitted by law, I am otherwise waiving any and all rights I may have to individual relief based on any claims that I have released and any rights I have waived by signing this Release.. I hereby represent and warrant that, other than the Excluded Claims, I am not aware of any claims I have or might have against any of the Released Parties that are not included in the Released Claims.

I acknowledge that I have read and understand Section 1542 of the California Civil Code which reads as follows: "**A general release does not extend to claims which the creditor does not know or suspect to exist in his favor at the time of executing the release, which if known by him or her must have materially affected his or her settlement with the debtor.**" I hereby expressly waive and relinquish all rights and benefits under that section and any law of any jurisdiction of similar effect with respect to my release of any claims hereunder.

I hereby represent that I have been paid all compensation owed and for all hours worked, I have received all the leave and leave benefits and protections for which I am eligible, and I have not suffered any on-the-job injury for which I have not already filed a workers' compensation claim.

I hereby agree not to disparage the Company, the Employers, or their officers, directors, employees, shareholders or agents, in any manner likely to be harmful to them or their business, business reputation, or personal reputation; provided, however, that I will respond accurately and fully to any question, inquiry or request for information when required by legal process or in connection with a government investigation. In addition, I understand that nothing in this Release is intended to prohibit or restrain me in any manner from making disclosures that are protected under the whistleblower provisions of federal or state law or regulation.

I acknowledge that to become effective, I must sign and return this Release to the Company so that it is received not later than fourteen (14) days following the date it is provided to me.

Employee

Name: Date:

Appendix A

Rigel Pharmaceuticals, Inc. Executive Severance Plan (COC Qualifying Termination)

Severance benefits provided to Eligible Employees under the Rigel Pharmaceuticals, Inc. Executive Severance Plan (the "Plan") are as follows:

1. **Severance Benefits.** Subject to the exceptions set forth in Section 2 of the Plan, each Eligible Employee who suffers a COC Qualifying Termination and who meets all of the requirements set forth in the Plan, including, without limitation, executing and not revoking a general waiver and release in substantially the form attached to the Plan as Exhibit A, Exhibit B or Exhibit C, as applicable, within the applicable time period set forth therein, shall receive severance benefits as set forth in this Appendix A.

- (a) **Cash Severance.** The Company shall make a lump sum payment of "**Cash Severance**" to the Eligible Employee in an amount determined as follows in the table below:

Title at Termination	Amount
CEO, President or EVP	2.5 x (Base Salary + Eligible Bonus)
SVP or VP	2.0 x (Base Salary + Eligible Bonus)

Subject to Sections 2(a)(2)(ii) and 3(e) of the Plan, the Cash Severance will be paid in a lump sum on the first regular payroll date following the Release Effective Date, but in no event later than March 15 of the year following the year in which the COC Qualifying Termination occurs.

- (b) **COBRA Premium Benefit.** If the Eligible Employee was enrolled in a group health plan (i.e., medical, dental, or vision plan) sponsored by the Company or an affiliate of the Company immediately prior to the COC Qualifying Termination, the Eligible Employee may be eligible to continue coverage under such group health plan (or to convert to an individual policy) at the time of the Eligible Employee's termination of employment under the Consolidated Omnibus Budget Reconciliation Act of 1985 (together with any state law of similar effect, "**COBRA**"). The Company will notify the Eligible Employee of any such right to continue such coverage at the time of termination pursuant to COBRA. No provision of this Plan will affect the continuation coverage rules under COBRA, except that the Company's payment, if any, of applicable insurance premiums, or waiver of any cost of coverage under any self-funded group health plan, will be credited as payment by the Eligible Employee for purposes of the Eligible Employee's payment required under COBRA. Therefore, the period during which an Eligible Employee may elect to continue the Company's or its affiliate's group health plan coverage at his or her own expense under COBRA, the length of time during which COBRA coverage will be made available to the Eligible Employee, and all other rights and obligations of the Eligible Employee under COBRA (except the obligation to pay insurance premiums that the Company pays, if any, or, with respect to a self-funded plan, any obligation to pay the cost of coverage to the Company that the Company waives, if any) will be applied in the same manner that such rules would apply in the absence of this Plan.

Provided that the Eligible Employee and/or his or her eligible dependents elect continued medical insurance coverage in accordance with the applicable provisions of COBRA, the Company will continue to pay the employer portion of applicable premium payments for Eligible Employee's and his or her eligible dependents' continued COBRA coverage under such plan (as in effect or amended from time to time) at the same rates and levels in effect immediately prior to Eligible Employee's termination of employment (the "**COBRA Subsidy**"), until the earlier of (1) the expiration of eighteen (18) months following the termination date or (2) the date Eligible Employee is covered or becomes eligible for any other health care plan coverage from a new employer that does not impose an applicable preexisting condition exclusion (the "**COBRA Subsidy Period**"). Eligible Employee shall promptly inform the Company in writing when he or she is covered or becomes eligible for

any such other health care coverage. The COBRA Subsidy for a given month shall be an amount equal to the employer share of the premium that the Company otherwise would pay for such group health care plan coverage if Eligible Employee were an active employee as of such month. Eligible Employee shall be responsible for paying the full

unsubsidized costs (without any COBRA Subsidy) of the applicable COBRA premium for any and all COBRA coverage after the COBRA Subsidy Period. In the event such COBRA Subsidy would result in adverse tax consequences, as determined by the Company, the Company shall reimburse Eligible Employee for the cost of the COBRA Subsidy rather than pay the COBRA Subsidy directly to the applicable plan administrator. If the Company reimburses Eligible Employee, the Company shall notify Eligible Employee in writing and Eligible Employee shall be responsible for timely paying the full costs of the applicable COBRA premium and submitting a request for reimbursement to the Company within thirty (30) days of such premium payment. The Company will then reimburse Eligible Employee on a monthly basis, within thirty (30) days after its receipt of sufficient documentation of payment by Eligible Employee of the applicable COBRA premium.

For purposes of this Section 1(b), any applicable insurance premiums that are paid by the Company shall not include any amounts payable by the Eligible Employee under an Internal Revenue Code Section 125 health care reimbursement plan, which amounts, if any, are the sole responsibility of the Eligible Employee.

- (c) **Accelerated Vesting.** The vesting and exercisability of all then-outstanding compensatory equity awards held by the Eligible Employee shall be accelerated such that the awards are fully vested and exercisable as of the date of the COC Qualifying Termination.
- (d) **Extended Period to Exercise Post Termination.** If the Eligible Employee has signed an agreement to extend the period to exercise post termination within thirty (30) days after becoming eligible to participate in the Plan, the Company will amend such Eligible Employee's then-outstanding stock options to extend the post-termination exercise period of such options that is applicable upon a COC Qualifying Termination until the earlier of (i) the original end of the term of each such option (generally 10 years from the date of grant) or (ii) the one (1) year anniversary of the date of the COC Qualifying Termination.

2. Definitions: The following definitions shall apply for purposes of this AppendixA:

- (a) **"Base Salary"** shall mean the greater of the Eligible Employee's base salary in effect immediately prior to (i) the Change of Control or (ii) the date of the COC Qualifying Termination. Base Salary does not include variable forms of compensation such as bonuses, incentive compensation, commissions, expenses or expense allowances and Base Salary shall not include any impact of a reduction in Base Salary that gives rises to Good Reason.

 Graphic
(b)

"Eligible Bonus" shall mean the product of (i) the average percentage of the target annual incentive bonus earned by the Eligible Employee for performance during the two fiscal years immediately prior to the fiscal year in which the Qualifying Termination occurs and (ii) the target annual incentive bonus, expressed in dollars, which the Eligible Employee is eligible to earn in the fiscal year in which (A) the Change of Control occurs or (B) the Qualifying Termination occurs, whichever of (A) or (B) is greater, provided that the target incentives shall not include any impact of a reduction in target annual incentive that gives rises to Good Reason, provided further that if the Eligible Employee has been employed for less than two years or has not participated in the Company's annual incentive bonus program for each of the prior two years, the performance achievement for purposes of clause (i) shall be 100% for the years in which the Eligible Employee was not employed or did not participate in the annual incentive bonus program.

The foregoing severance benefits are subject to all of the terms and conditions of the Plan, including reduction against any other severance owed to the Eligible Employee.

Rigel Pharmaceuticals, Inc.

By:/s/Raymond Furey

Title: EVP, General Counsel, Corporate Secretary

Appendix B

Rigel Pharmaceuticals, Inc. Executive Severance Plan (Non-COC Qualifying Termination)

Severance benefits provided to Eligible Employees under the Rigel Pharmaceuticals, Inc. Executive Severance Plan (the "Plan") are as follows:

1. **Severance Benefits.** Subject to the exceptions set forth in Section 2 of the Plan, each Eligible Employee who suffers a Non-COC Qualifying Termination and who meets all of the requirements set forth in the Plan, including, without limitation, executing and letting become effective a general waiver and release in substantially the form attached to the Plan as Exhibit A, Exhibit B or Exhibit C, as applicable, within the applicable time period set forth therein, shall receive severance benefits as set forth in this Appendix B.

(a) **Cash Severance.** The Company shall provide for continuing Base Salary payments ("Cash Severance") to the Eligible Employee in an amount equal to the Base Salary that would have been paid during the period (the "Severance Period") in the table below had the Eligible Employee continued the service through such Severance Period:

Title at Termination	Severance Period
CEO	18 months
EVP	12 months
SVP	12 months
VP	9 months

Subject to Sections 2(a)(2)(ii) and 3(e) of the Plan, the Cash Severance will be paid in regular installments on each regularly scheduled payroll date until the earlier to occur of the Severance Period following the Non-COC Qualifying Termination or the March 14 of the calendar year following the Non-COC Qualifying Termination.

(b) **COBRA Premium Benefit.** If the Eligible Employee was enrolled in a group health plan (i.e., medical, dental, or vision plan) sponsored by the Company or an affiliate of the Company immediately prior to the Non-COC Qualifying Termination, the Eligible Employee may be eligible to continue coverage under such group health plan (or to convert to an individual policy) at the time of the Eligible Employee's termination of employment under COBRA. The Company will notify the Eligible Employee of any such right to continue such coverage at the time of termination pursuant to COBRA. No provision of this Plan will affect the continuation coverage rules under COBRA, except that the Company's payment, if any, of applicable insurance premiums, or waiver of any cost of coverage under any self-funded group health plan, will be credited as payment by the Eligible Employee for purposes of the Eligible Employee's payment required under COBRA. Therefore, the period during which an Eligible Employee may elect to continue the Company's or its affiliate's group health plan coverage at his or her own expense under COBRA, the length of time during which COBRA coverage will be made available to the Eligible Employee, and all other rights and obligations of the Eligible Employee under COBRA (except the obligation to pay insurance premiums that the Company pays, if any, or, with respect to a self-funded plan, any obligation to pay the cost of coverage to the Company that the Company waives, if any) will be applied in the same manner that such rules would apply in the absence of this Plan.

Provided that the Eligible Employee and/or his or her eligible dependents elect continued medical insurance coverage in accordance with the applicable provisions of COBRA, the Company will continue to pay the employer portion of applicable premium payments for Eligible Employee's and his or her eligible dependents' continued COBRA coverage under such plan (as in effect or amended from time to time) at the same rates and levels in effect immediately prior to Eligible Employee's termination of employment (the "COBRA Subsidy"), until the earlier of (1) the

Severance Period in the table above or (2) the date Eligible Employee is covered or becomes eligible for any other health care plan coverage from a new employer that does not impose an applicable preexisting condition exclusion (the "COBRA Subsidy Period"). Eligible Employee shall promptly inform the Company in writing when he or she is covered or becomes eligible for any such other health care coverage. The COBRA Subsidy for a given month shall be an amount equal to the employer share of the premium that the Company otherwise would pay for such group health care plan coverage if Eligible Employee were an active employee as of such month. Eligible Employee shall be responsible for

paying the full unsubsidized costs (without any COBRA Subsidy) of the applicable COBRA premium for any and all COBRA coverage after the COBRA Subsidy Period. In the event such COBRA Subsidy would result in adverse tax consequences, as determined by the Company, the Company shall reimburse Eligible Employee for the cost of the COBRA Subsidy rather than pay the COBRA Subsidy directly to the applicable plan administrator. If the Company reimburses Eligible Employee, the Company shall notify Eligible Employee in writing and Eligible Employee shall be responsible for timely paying the full costs of the applicable COBRA premium and submitting a request for reimbursement to the Company within thirty (30) days of such premium payment. The Company will then reimburse Eligible Employee on a monthly basis, within thirty (30) days after its receipt of sufficient documentation of payment by Eligible Employee of the applicable COBRA premium.

For purposes of this Section 1(b), any applicable insurance premiums that are paid by the Company shall not include any amounts payable by the Eligible Employee under an Internal Revenue Code Section 125 health care reimbursement plan, which amounts, if any, are the sole responsibility of the Eligible Employee.

(c) **Accelerated Vesting.** The vesting and exercisability of any then-outstanding and unvested time- based compensatory equity awards held by the Eligible Employee that would have vested during the period in the table below (the “**Vesting Amount**”) had the Eligible Employee continued employment with the Company through the end of such period shall become vested and exercisable as of the date of the Non-COC Qualifying Termination:

Title at Termination	Vesting Amount
CEO	18 months
EVP	12 months
SVP	12 months
VP	9 months

Further, the vesting and exercisability of any then-outstanding and unvested performance-based compensatory equity awards held by the Eligible Employee that would have vested during the Period to Exercise in the table below had the Eligible Employee continued employment with the Company through the end of such period shall become vested and exercisable as of the date, if any, that the performance goal relating to such performance-based vesting awards is achieved.

(d) **Extended Period to Exercise Post Termination.** If the Eligible Employee has signed an agreement to extend the period to exercise post termination within thirty (30) days after becoming eligible to participate in the Plan, the post-termination exercise period of such Eligible Employee's then-outstanding stock options will be automatically extended until the earlier of (i) the original end of the term of each such option (generally 10 years from the date of grant) or (ii) the Period to Exercise in the table below:

Title at Termination	Period to Exercise
CEO	30 months
EVP	24 months
SVP	24 months
VP	21 months

2. Definition: The following definition shall apply for purposes of this Appendix:

(a) **“Base Salary”** shall mean the Eligible Employee's base salary in effect immediately prior to the date of the Non-COC Qualifying Termination. Base Salary does not include variable forms of compensation such as bonuses, incentive compensation, commissions, expenses or expense allowances and Base Salary shall not include any impact of a reduction in Base Salary that gives rise to Good Reason.

The foregoing severance benefits are subject to all of the terms and conditions of the Plan, including reduction against any other severance owed to the Eligible Employee.

By:/s/Raymond Furey

Title: EV.P, General Counsel, Corporate Secretary

[CERTAIN CONFIDENTIAL INFORMATION CONTAINED IN THIS DOCUMENT, MARKED BY [**] = CERTAIN MARKED INFORMATION, HAS BEEN OMITTED FROM THIS EXHIBIT BECAUSE IT IS BOTH (I) NOT MATERIAL AND (II) IS THE TYPE THAT THE REGISTRANT TREATS AS PRIVATE OR CONFIDENTIAL.

ASSET PURCHASE AGREEMENT

between

BLUEPRINT MEDICINES CORPORATION

and

RIGEL PHARMACEUTICALS, INC.

DATED AS OF FEBRUARY 22, 2024

TABLE OF CONTENTS

Page

ARTICLE I DEFINITIONS AND TERMS1

Section 1.1	Definitions1
Section 1.2	Other Definitional Provisions16

ARTICLE II PURCHASE AND SALE; LICENSES16

Section 2.1	Purchase and Sale of Assets16
Section 2.2	Consents17
Section 2.3	Excluded Assets18
Section 2.4	Assumption of Liabilities20
Section 2.5	Retained Liabilities20

Section 2.6 License Grants; Right of Reference21

ARTICLE III CONSIDERATION25

Section 3.1 Purchase Price25

Section 3.2 Additional Consideration25

ARTICLE IV CLOSING30

Section 4.1 Closing30

Section 4.2 Additional Transfer Documents32

ARTICLE V REPRESENTATIONS AND WARRANTIES OF SELLER32

Section 5.1 Organization; Qualification 32

Section 5.2 Title to Assets, Sufficiency32

Section 5.3 Authority; Binding Effect33

Section 5.4 No Conflicts33

Section 5.5 Governmental Authorizations33

Section 5.6 Real Property33

Section 5.7 No Litigation33

Section 5.8 Compliance with Laws34

Section 5.9 Product Registrations; Regulatory Compliance34

Section 5.10 Intellectual Property; Performance of Transition35

Section 5.11 Taxes36

Section 5.12 Assumed Contracts36

Section 5.13 Anti-Corruption36

Section 5.14 Brokers37

Section 5.15 No Other Representations or Warranties37

ARTICLE VI REPRESENTATIONS AND WARRANTIES OF PURCHASER38

Section 6.1 Organization and Qualification38

Section 6.2 Corporate Authorization38

Section 6.3 Binding Effect 38

Section 6.4 No Conflict38

Section 6.5 Governmental Authorization38

Section 6.6 Third Party Approvals 38

Section 6.7 Financial Capability39

Section 6.8 Litigation39

Section 6.9 No Debarment 39

Section 6.10 Anti-Corruption39

Section 6.11 Brokers40

Section 6.12 Solvency40

i

Section 6.13 No Other Representation and/or Warranty40

ARTICLE VII COVENANTS40

Section 7.1 Information and Documents40

Section 7.2 Conduct41

Section 7.3 Insurance41

Section 7.4 Trade Notifications41

Section 7.5 Included Accounts Receivable; Excluded Accounts Receivable41

Section 7.6	Payments under Assumed Contracts	42
Section 7.7	Transition Services	43
Section 7.8	Confidentiality	43
Section 7.9	Wrongfully Transferred or Retained Assets and Liabilities	43
Section 7.10	[***]	43
Section 7.11	Further Actions	44

ARTICLE VIII CONDITIONS TO CLOSING44

Section 8.1	Conditions to the Obligations of Purchaser	44
Section 8.2	Conditions to the Obligations of Seller	45

ARTICLE IX [RESERVED]45

ARTICLE X SURVIVAL OF REPRESENTATIONS AND WARRANTIES;

INDEMNIFICATION 45

Section 10.1	Survival	45
Section 10.2	Indemnification Survival	45
Section 10.3	Indemnification	45
Section 10.4	Notice; Defense of Claims	48
Section 10.5	Remedies Exclusive	49

ARTICLE XI MISCELLANEOUS50

Section 11.1	Notices	50
Section 11.2	Amendment; Waiver	51
Section 11.3	Assignment	51
Section 11.4	Entire Agreement	51
Section 11.5	Parties in Interest	51
Section 11.6	Public Disclosure	51
Section 11.7	Expenses, Taxes, and Fees	51
Section 11.8	Schedules	52
Section 11.9	Governing Law; Jurisdiction	52

Section 11.10	WAIVER OF JURY TRIAL	53
Section 11.11	Counterparts	53
Section 11.12	Headings	53
Section 11.13	Severability	53
Section 11.14	Specific Performance	54
Section 11.15	Non-Recourse	54

ATTACHMENTS

EXHIBIT A	Form of Bill of Sale and Assignment and Assumption Agreement
EXHIBIT B	Form of Intellectual Property Assignment Agreement
EXHIBIT C	Disclosure Schedule
EXHIBIT D	Form of Transition Agreement
EXHIBIT E	Form of Material Transfer Agreement

This Asset Purchase Agreement is made and entered into as of the 22nd day of February, 2024, between Blueprint Medicines Corporation, a Delaware corporation ("Seller"), and Rigel Pharmaceuticals, Inc., a Delaware corporation ("Purchaser"). Seller and Purchaser are individually referred to as a "Party" and collectively as the "Parties".

WITNESSETH:

WHEREAS, Seller desires to sell, transfer, assign, convey and deliver to Purchaser, and Purchaser desires to purchase, acquire and assume from Seller, all of Seller's right, title and interest to the product known as GAVRETO® (pralsetinib) in the United States and other Purchased Assets and Assumed Liabilities, in each case, on the terms and subject to the conditions set forth in this Agreement (the "Transaction"); and

WHEREAS, Seller and Purchaser desire to make certain representations, warranties, covenants, and agreements in connection with the Transaction and prescribe various conditions to the Transaction.

NOW, THEREFORE, in consideration of the foregoing and the representations, warranties, covenants and agreements contained herein, the Parties hereby agree as follows:

ARTICLE I

DEFINITIONS AND TERMS

Section 1.1 **Definitions.** As used in this Agreement, the following terms shall have the meanings set forth or as referenced below:

"2024 CStone Cooperation Agreement" shall have the meaning set forth in [Section 7.6\(f\)](#).

"AAA" shall have the meaning set forth in [Section 7.6\(e\)](#).

"Accounting Standards" means generally accepted accounting principles as applicable in the U.S. as generally and consistently applied throughout Purchaser's organization.

"Actual Fraud" means a claim for common law fraud with a specific intent to deceive based on a representation contained in this Agreement; provided that, at the time such representation was made, (i) such representation was inaccurate and (ii) the Party making such representation had actual knowledge of the inaccuracy of such representation.

"Additional Transfer Document" shall have the meaning set forth in [Section 4.2\(a\)](#).

"Affiliate" means, with respect to any Person, any other Person that controls, is controlled by or is under common control with such Person (and for this purpose, the term control means the power to direct, or cause the direction of, the management and policies of a Person (directly or indirectly), whether through ownership of voting securities, by contract or otherwise (and the terms controlling and controlled have meanings correlative to the foregoing)).

"Agreement" means this Asset Purchase Agreement, as the same may be amended or supplemented from time to time in accordance with the terms hereof, including the Exhibits and Schedules hereto.

"Ancillary Agreements" means, collectively, the Bill of Sale and Assignment and Assumption Agreement, the Intellectual Property Assignment Agreement, the Transition Agreement, the Material Transfer Agreement, and the 2024 CStone Cooperation Agreement.

[***]

"Anti-Corruption Laws" shall have the meaning set forth in [Section 5.13\(a\)](#).

"Assumed Contracts" shall have the meaning set forth in [Section 2.1\(a\)](#).

"Assumed Liabilities" shall have the meaning set forth in [Section 2.4](#).

"Bankruptcy Code" shall have the meaning set forth in Section 2.6(g).

"Bill of Sale and Assignment and Assumption Agreement" shall have the meaning set forth in Section 4.1(b)(i).

[***]

[***]

"Business" means the Exploitation of the Product in the Territory [***] as of the Closing Date.

"Business Day" means any day other than a Saturday, a Sunday or a day on which banks in New York, New York are authorized or obligated by law or executive order to close.

"Calendar Quarter" means each of the three (3) month periods ending on March 31, June 30, September 30, and December 31 of any Calendar Year.

"Calendar Year" means each twelve (12) month period commencing on January 1 and ending on December 31, provided that (a) the first calendar year shall begin on the Closing Date and end on December 31 and (b) the last calendar year shall begin on January 1 and end on the effective date of expiration or termination.

"Change of Control" shall mean (i) any merger, reorganization, consolidation or combination in which the subject entity is not the surviving corporation, or (ii) any "person" (within the meaning of Sections 13(d) and 14(d)(2) of the Securities Exchange Act of 1934), excluding the subject entity and its Affiliates, is or becomes the beneficial owner, directly or indirectly, of securities of the subject entity representing 50% or more of either (a) the then-outstanding shares of common stock of the subject entity or its parent corporation, or (b) the combined voting power of the subject entity's then outstanding voting securities; or (iii) if individuals who as of the effective date constitute the board of directors of the subject entity or its parent corporation (the "Incumbent Board") cease for any reason to constitute at least a majority of such Incumbent Board; provided, however, that any individual becoming a director subsequent to the effective date whose election, or nomination for election by the subject entity's shareholders, was approved by a vote of at least a majority of the directors then comprising the Incumbent Board shall be considered as though such individual were a member of the Incumbent Board, but excluding, for this purpose, any such individual whose initial assumption of office occurs as a result of an actual or threatened election contest with respect to the election or removal of directors or other actual or threatened solicitation of proxies or consents by or on behalf of a person other than the Incumbent Board; or (iv) approval by the shareholders of the subject entity of a complete liquidation or the complete dissolution of the subject entity.

"Claims Threshold" shall have the meaning set forth in Section 10.3(c)(i).

"Closing" means the closing of the transactions contemplated by this Agreement pursuant to Article IV of this Agreement.

"Closing Date" shall have the meaning set forth in Section 4.1(a).

"CMC" means the Chemistry, Manufacturing and Controls portion of a Regulatory Filing or in any supporting development reports thereto, in each case, with respect to the Product.

"CMS" means the Centers for Medicare and Medicaid Services or any successor agency.

2

"Combination Product" means: [***] Drug delivery vehicles, adjuvants and excipients will not be deemed to be "active ingredients," except in the case where such delivery vehicle, adjuvant or excipient is recognized by the FDA as an active ingredient in accordance with 21 C.F.R. 210.3(b)(7).

"Compound" means (a) Seller's proprietary RET inhibitor known as pralsetinib (also known as BLU-667), or (b) any salt, metabolite, prodrug (including ester prodrug) that convert to the compound described in clause (a), free base, hydrate, solvate, polymorph, stereoisomer or enantiomer of pralsetinib.

"Confidentiality Agreement" means the Confidentiality Agreement between Seller and Purchaser, dated as of [***], as amended or supplemented from time to time.

"Contract" means any written contract, agreement, lease, instrument, note, bond, loan, indenture, license or sublicense, or other legally binding written commitment or arrangement.

"Control" or **"Controlled"** means, with respect to any Intellectual Property, Regulatory Filing, Regulatory Approval, or other property right, the legal authority or right (whether by ownership, license (other than a license granted pursuant to this Agreement) or otherwise) of a Person or its Affiliate, to grant the right to access or use, or to grant a license or a sublicense to or under such Intellectual Property, Regulatory Filing, Regulatory Approval, or other property right (in whole or in part), without breaching the terms of any agreement or other arrangement between such Person (or any of its Affiliates) and a Third Party or creating a payment obligation upon such Person.

"Controlling Party" shall have the meaning set forth in Section 10.4.

"Cover," "Covering" or "Covered" means: (a) with respect to a patent, that, in the absence of a license granted to a Person under an issued Valid Claim included in such patent, the practice by such Person of the subject matter at issue would infringe such Valid Claim, or (b) with respect to an application for patent, that, in the absence of a license granted to a Person under a pending Valid Claim included in such application, the practice by such Person of the subject matter at issue would infringe such Valid Claim if such patent application were to issue as a patent.

"CStone" means CStone Pharmaceuticals.

"CStone Agreements" means the CStone License Agreement, that certain Pharmacovigilance Agreement by and among Seller, CStone and Roche dated as of [***].

"CStone License Agreement" means that certain License and Collaboration Agreement between Seller and CStone dated as of June 1, 2018.

"CStone Global Development Plan" means the global development plan agreed by Seller and CStone pursuant to the CStone License Agreement, as may be amended from time to time.

"CStone Territory" means the People's Republic of China, Hong Kong Special Administrative Region, Macau Special Administrative Region and Taiwan.

"Delayed Purchase Price" means Five Million Dollars (\$5,000,000), less any set-off permitted by Article X.

"Development Milestone Event" shall have the meaning set forth in Section 3.2(a).

"Development Milestone Payment" shall have the meaning set forth in Section 3.2(a).

"Disclosure Schedule" shall have the meaning set forth in Article V.

"Divestiture" (and other correlative terms) means any transaction in which any Licensed Product and the associated intellectual property assets related to the foregoing are divested or transferred by any means, including by way of merger, consolidation, asset acquisition or sale, exercised option, purchase, sale, assignment or other similar transfer.

"DMF" means any drug master file with the FDA, and any equivalent filing in other countries or regulatory jurisdictions.

"Excluded Accounts Receivable" shall have the meaning set forth in Section 2.3(e).

"Excluded Assets" shall have the meaning set forth in Section 2.3.

"Excluded Contracts" means all contracts that are not Assumed Contracts.

[***]

"Excluded Intellectual Property" shall have the meaning set forth in Section 2.3(g).

"Exploit" or "Exploiting" means to research, develop, manufacture, import, export, sell, offer for sale or commercialize, or have others do the same. When used as a noun, "Exploitation" means any activities involved in Exploiting.

"FDA" means the United States Food and Drug Administration or any successor agency.

"Federal Healthcare Program" has the meaning set forth in 42 U.S.C. § 1320a-7b(f) and any implementing regulations thereto and, without limiting the foregoing shall include any plan or program that provides health care benefits, whether directly, through insurance, or otherwise, that is funded directly, in whole or in part, by the government of the United States of America (other than the Federal Employees Health Benefits Program), including, without limitation, Medicare, Medicaid, the Children's Health Insurance Program, TRICARE, 340B Federal Drug Discount Program, and Veterans Health Administration programs (described in Title XVIII of the Social Security Act ("SSA"), Title XIX of the SSA, Title XXI of the SSA, Title 10, Chapter 55 of the U.S.C., 42 U.S.C. § 256b and 38 U.S.C. § 8126, respectively), or any state health care program (as defined in Section 1128(h) of the SSA).

"FFDCA" shall have the meaning set forth in Section 5.9(f).

"Field" means all uses in humans and animals.

"First Commercial Sale" means, with respect to each Licensed Product, the first sale of the Product recorded in accordance with the Accounting Standards by or on behalf of a Selling Party for distribution, use or consumption after the Closing Date. [***]

"Fundamental Claim Expiration Date" shall mean [***] after the expiration of the statute of limitations applicable to the subject matter thereof.

"Fundamental Obligations" shall have the meaning set forth in Section 10.1.

"General Indemnity Cap" means an amount equal to [***].

"General Indemnity Expiration Date" shall have the meaning set forth in Section 10.1.

[***]

[***]

"Governmental Authority" means any supranational, national, federal, state or local judicial, legislative, executive, enforcement, administrative or regulatory authority.

"Governmental Authorizations" means all licenses, permits, certificates and other authorizations and approvals under the applicable Laws of any Governmental Authority.

"Governmental Order" means any order, writ, judgment, injunction, decree, stipulation, determination or award entered by or with any Governmental Authority.

"Healthcare Regulatory Law" means Laws relating to healthcare regulatory matters, including, but not limited to: (a) 42 U.S.C. §§ 1320a-7, 7a, and 7b, which are commonly referred to as the "Federal Fraud Statutes"; Title XVIII of the Social Security Act (42 U.S.C. § 1395 et seq.), the "Medicare Laws"; Title XIX of the Social Security Act (42 U.S.C. § 1396 et seq.), the "Medicaid Laws"; Title XXI of the Social Security Act (42 U.S.C. § 1397 et seq.), the "Children's Health Insurance Program (CHIP) Laws"; 10 U.S.C. § 1071 et seq., the "TRICARE Laws"; 38 U.S.C. Chapter 17, the "Veterans Health Administration Laws"; 42 U.S.C. § 201 et seq., the "Public Health Service Act"; the Patient Protection and Affordable Care Act (Pub. L. No. 111-148) as amended by the Health Care and Education Reconciliation Act of 2010 (Pub. L. No. 111-152); 42 U.S.C. § 1395nn, which is commonly referred to as the "Stark Statute"; 31 U.S.C. §§ 3729-3733, which is commonly referred to as the "Federal False Claims Act"; 42 U.S.C. §§ 286, 287 and 1001, which are commonly referred to as the "Federal Criminal False Claims Statutes"; 18 U.S.C. § 1035, which is commonly referred to as the "False Statements Relating to Health Care Matters Law"; 31 U.S.C. § 3801 et seq., which is commonly referred to as "Federal Program Fraud Civil Remedies Act"; 42 U.S.C.

§§ 1320d through 1320d-8 and 42 C.F.R. §§ 160, 162 and 164, which are commonly referred to as the "Health Insurance Portability and Accountability Act of 1996"; the FFDCA; and any implementing regulations and comparable international, state, or local Laws for any of the foregoing; (b) any Laws governing any Federal Healthcare Program or otherwise governing or regulating the provision of, or payment for, healthcare items and services; (c) any federal, state or local applicable Law that regulates the clinical development, manufacturing, approval, promotion or distribution of products; and (d) any state Law regulating the interactions with health care professionals and reporting thereof.

"Included Accounts Receivable" shall have the meaning set forth in Section 2.1(h).

"IND" means an application submitted to the FDA for authorization to commence clinical studies, including (a) an Investigational New Drug Application as defined in 21 C.F.R. Part 312 or any successor application or procedure submitted to the FDA and (b) all supplements, amendments, variations, extensions and renewals thereof that may be submitted with respect to the foregoing.

"Indemnified Parties" shall have the meaning set forth in Section 10.3(b).

"Indemnifying Party" shall have the meaning set forth in Section 10.4.

"Independent Arbitrator" shall have the meaning set forth in Section 7.6(e).

"Indication" means [***] that the Product [***] in the indication section of the label approved by the FDA relevant to usage of the Product [***].

"Insolvent Party" shall have the meaning set forth in Section 2.6(g).

"Intellectual Property" means all patents, trademarks, service marks, logos, trade names, internet domain names, database rights, rights in designs, rights in inventions, trade secrets; rights in information, know-how, trade dress, product livery, source identifiers, all copyrightable works of authorship (whether published or unpublished) and all technology, specifications, information, records, techniques, processes, procedures, documentation, data, databases and other proprietary information that is identified or identifiable in a tangible form, in each case whether registered or unregistered, and all registrations and applications therefor, and all rights or forms of protection having equivalent or similar effect anywhere in the world.

"Intellectual Property Assignment Agreement" shall have the meaning set forth in Section 4.1(b)(iv).

"Knowledge of Seller" means [***].

"Laws" means any federal, state, provincial, regional, territorial, foreign or local law, common law, statute, ordinance, rule, regulation, code, Governmental Order, or requirement issued, enacted, promulgated, implemented or

5

otherwise put into effect by or under the authority of any Governmental Authority, consent order, supervisory requirements, directives, circulars, opinions, interpretive letters, guidelines and policies, in each case with the force of law, including Healthcare Regulatory Laws.

"Legal Proceedings" means any litigation complaint (including a qui tam complaint), audit, judicial, administrative or arbitral action, suit, investigation, inquiry, claims (including counterclaims), litigation, civil investigative demand, criminal information, subpoena, search warrant, or proceeding (public or private) by or before a Governmental Authority.

"Liability" and "Liabilities" means any and all Losses, debts, damages, adverse claims, liabilities, commitments and obligations of any nature or kind, whether accrued or unaccrued, fixed, known or unknown, express or implied, absolute or contingent, accrued or unaccrued, disputed, liquidated, executory, matured or unmatured or determined or determinable.

"License" means a grant of rights from Purchaser to an Affiliate or Third Party under any of the Purchased Assets or the rights licensed to Purchaser by Seller under Section 2.6 of the Agreement with respect to the Exploitation of the Product in the Territory, including, for the avoidance of doubt, Third Party distributors.

"Licensed Product" means the pharmaceutical preparation containing Compound as an active ingredient, and any form, formulation, presentation and line extension thereof, including the pralsetinib product known as GAVRETO® (pralsetinib), and including any Combination Product.

"Licensee" means any Affiliate or permitted Third Party granted a License.

"Liens" means any lien, security interest, pledge, mortgage, charge, restriction on transfer conditional sale or other title retention agreement, or other or encumbrance.

[***]

"Loss Tax Benefit" means the cash Tax savings or benefits actually received by such Purchaser Indemnified Party in the taxable period in which the applicable indemnifiable Losses are incurred or the [***] following such taxable period that are attributable to any deduction, loss, credit, refund or other reduction in Tax resulting from or arising out of a Loss, in each case computed at the highest marginal tax rates applicable to the Purchaser Indemnified Party.

"Losses" of a Person means any and all losses, damages, awards, judgments, costs and expenses (including reasonable attorneys' fees and expenses) actually suffered or incurred by such Person. Notwithstanding anything herein to the contrary, no Indemnified Party shall have the right to be indemnified for any Losses to the extent they are in the nature of consequential damages, incidental or indirect damages, diminution in value damages, lost profits, punitive, special or exemplary damages, and in particular, without limitation, no "multiple of profits" or "multiple of cash flow" or similar valuation methodology shall be used in calculating the amount of any Losses, except to the extent such damages are awarded to a Third Party in connection with a Third Party indemnification claim.

[***]

"Manufacture" or **"Manufacturing"** means to engage in activities related to and perform the production, manufacture, synthesis, processing, filling, finishing, packaging, labeling, shipping and holding of product or any intermediate thereof, including process development, process qualification and validation, scale-up, commercial manufacture and analytic development, product characterization, stability testing, quality assurance and quality control, including all development activities enabling Regulatory Approval of Manufacturing. When used as a noun, "Manufacturing" means any of the foregoing activities. "Manufacturing" refers to both nonclinical and clinical Manufacturing for research and development, and Manufacturing for commercialization or future business and/or regulatory requirements.

"Material Adverse Effect" means a change, effect, event or occurrence that has or would be reasonably expected to have a material adverse effect on the Purchased Assets, taken as a whole, or on the ability of Seller to perform its obligations under, or consummate, the transactions contemplated by this Agreement; provided, however, that none of the following changes, events, effects, occurrences, developments, state of circumstances, facts, or conditions shall be deemed, either alone or in combination, to constitute a Material Adverse Effect, or be taken into account in determining whether there has or will be a Material Adverse Effect: (a) changes or effects in business, economic, tax, regulatory, legal or political conditions or financial markets generally within or outside the United States, provided that such matters will only be excluded so long as such matters do not have a disproportionate adverse effect on the Purchased Assets relative to other comparable assets in the pharmaceutical industry; (b) changes in United States generally accepted accounting principles; (c) changes or effects generally affecting the pharmaceutical and/or biotechnology industry; (d) changes or effects that arise out of or are attributable to the commencement, occurrence, continuation or intensification or reduction or cessation of any war (whether or not declared), sabotage, armed hostilities or acts of terrorism; (e) the occurrence of any act of God or other calamity or force majeure events (whether or not declared as such), including any pandemic (including the COVID-19 pandemic, and any future resurgence, or evolutions or mutations, of COVID-19 or related disease outbreaks, epidemics or pandemics), natural disaster, fire, flood, hurricane, tornado, or other weather event; (f) changes or effects that relate to any failure by Seller to meet internal projections or forecasts for any period (including with respect to the Purchased Assets or the Product); (g) any matter disclosed in, or reasonably determinable from the Disclosure Schedule to this Agreement; (h) any action taken by Seller or its Affiliates as contemplated by this Agreement (including without limitation the Ancillary Agreements) or with Purchaser's consent; (i) changes or effects that arise out of or are attributable to the negotiation, execution, public announcement or performance of this Agreement; or (j) any existing event or occurrence or circumstance of which Purchaser has actual knowledge as of the date hereof.

"Material Transfer Agreement" shall have the meaning set forth in Section 4.1(b)(vi).

"Milestone Event" shall have the meaning set forth in Section 3.2(a)(ii).

"Milestone Payments" shall have the meaning set forth in Section 3.2(a)(ii).

"NDA" means a new drug application submitted to the FDA pursuant to Section 505(b) of the Federal Food, Drug and Cosmetic Act, 21 U.S.C. § 355(b).

"Net Sales" means, with respect to each Payment Product, [***].

"Non-Assignable Right" shall have the meaning set forth in Section 2.2(a).

"Non-controlling Party" shall have the meaning set forth in Section 10.4.

"Party" and "Parties" shall have the meaning set forth in the preamble.

"Patent Coordination Team" shall have the meaning set forth in Section 2.6(c).

"Payment Product" means any Licensed Product or any pharmaceutical product containing a RET inhibitor compound that is Covered by a Transferred Patent.

"Permitted Encumbrances" means (i) Liens approved in writing by Purchaser prior to Closing; (ii) statutory Liens arising out of operation of Law with respect to a Liability incurred in the ordinary course of business and which is not delinquent; (iii) Liens for Taxes not yet subject to penalties for nonpayment or that are being contested in good faith by appropriate proceedings; (iv) mechanics', materialmen's, carriers', workmens', warehousemen's, repairmen's, landlords' or other like Liens and security obligations that are not delinquent; (v) Liens arising under original purchase price conditional sales contracts with third parties entered into in the ordinary course of business; (vi) restrictions under leases, subleases, licenses or occupancy agreements that are Purchased Assets; (vii) Liens pursuant to any Assumed Contract; and (viii) all matters of record or otherwise disclosed in any title policy and/or survey provided by Seller to Purchaser or otherwise obtained by Purchaser or that an inspection or survey would disclose.

"Person" means an individual, a limited liability company, joint venture, a corporation, a partnership, an association, a trust, governmental entity, a division or operating group of any of the foregoing or other entity or organization.

"Personal Information" means, with respect to any Person, any information that allows for identification of such Person and that is defined as "personal data," "personally identifiable information," "personal information" and is regulated by applicable Privacy Laws.

"Post-Closing Tax Period" means any Tax period (or portion thereof) beginning after the Closing Date.

"Pre-Closing Tax Period" means any Tax period (or portion thereof) ending on or before the Closing Date.

"PRC" shall have the meaning set forth in Section 7.6(g).

"Privacy Laws" means all applicable Laws that regulate privacy and security of Personal Information.

"Product" means the pharmaceutical product known as GAVRETO® (pralsetinib).

"Product Data" means all of the following, to the extent included in one or more Product Registrations and exclusively related to the Territory: (a) clinical data, results (including all tables, listing and graphs) and reports, case reports, and other written materials or correspondence filed with or received from a Governmental Authority, (b) records and data necessary to manufacture, formulate, test, package and store the Compound including (i) Compound and raw material specifications, (ii) standard operating procedures and master batch records and related manufacturing, engineering or other manuals as applicable, (iii) process validation reports and process development reports, (iv) technical storage conditions and stability assay procedures and other assay procedures, (v) quality control and release testing procedures and records, (vi) batch documentation including copies of executed batch records and disposition packages for

the inventory set forth in the Material Transfer Agreement, in any form whatsoever, including relevant portions of laboratory notebooks or electronic files, and (vii) scientific reports owned by Seller related to the Compound.

"Product Registrations" shall have the meaning set forth in Section 5.9(a).

"Public Official" means (i) any official or employee of any Governmental Authority, including officials and employees who are appointed, elected or hired at any level within the legislative, administrative, executive, judicial or regulatory bodies of a national, regional or local government (e.g., military personnel, police, judges, inspectors, licensing officers, customs agents); (ii) any director, officer or employee at any level of any company, legal entity or other instrumentality owned or controlled by a Governmental Authority; (iii) any director, officer or employee at any level of public international organizations (e.g., the United Nations, the World Bank, the International Monetary Fund); (iv) any third party acting in an official capacity for a Governmental Authority or enterprise owned or controlled by a Governmental Authority (e.g., a third party acting under a delegation of authority to conduct government functions); (v) any political party, any official of a political party or any candidate for political office; or (vi) any member of a royal or ruling family.

"Purchase Price" means Fifteen Million Dollars (\$15,000,000).

"Purchased Assets" shall have the meaning set forth in Section 2.1, it being understood that the Purchased Assets do not include the Excluded Assets.

"Purchaser" shall have the meaning set forth in the preamble.

"Purchaser Fundamental Representation" means the representations and warranties of Purchaser set forth in [***].

"Purchaser Indemnified Party" shall have the meaning set forth in Section 10.3(a).

"Registered Intellectual Property" means all issued patents, registered trademarks and registered domain names and all applications for the foregoing included in the Transferred Intellectual Property.

"Regulatory Approval" means the approval, license, registration or authorization of the applicable Regulatory Authority necessary for the marketing and sale of the Product in a country or jurisdiction. Regulatory Approvals include approvals by Regulatory Authorities of NDAs.

"Regulatory Authority" means any multinational, federal, national, state, provincial or local regulatory agency, department, bureau or other governmental entity with authority over the clinical development, manufacture, marketing or sale of the Product in a country or region, including the FDA in the United States.

"Regulatory Data" means any and all research data, pharmacology data, CMC data, safety data, nonclinical data, clinical data and all other documentation submitted, or required to be submitted, to Regulatory Authorities in association with Regulatory Filings and Regulatory Approvals for the Product (including any applicable DMFs or similar documentation).

"Regulatory Filing" means any documentation comprising or relating to or supporting any submission or application with any Regulatory Authority in the Territory with respect to the Product or its use or potential use in humans, including any documents submitted to any Regulatory Authority, including INDs and NDAs, and copies of all correspondence with any Regulatory Authority with respect to the Product (including minutes of any meetings, telephone conferences or discussions with any Regulatory Authority).

"Representatives" means, in respect of a Party, any Affiliates and/or any directors, officers, employees, agents, contractors and/or advisors (including financial advisors, counsels, consultants and accountants) of such Party or any of its Affiliates.

"Retained Liabilities" shall have the meaning set forth in Section 2.5.

"Roche" means, collectively, F. Hoffmann-La Roche Ltd and Genentech, Inc. ("Genentech").

"Roche Collaboration Agreement" means the Collaboration Agreement by and between Seller and Roche dated as of July 13, 2020.

[***]

"ROW Territory" means worldwide, excluding the Territory and the CStone Territory.

"Royalty Payments" shall have the meaning set forth in Section 3.2(d)(i).

"Royalty Reduction Trigger" shall have the meaning set forth in Section 3.2(d)(iv)(3).

"Royalty Report" shall have the meaning set forth in Section 3.2(d)(iii).

"Royalty Term" shall have the meaning set forth in Section 3.2(d)(ii).

"Sales Milestone Event" shall have the meaning set forth in Section 3.2(a)(ii).

"Seller" shall have the meaning set forth in the preamble.

"Seller Financing Agreement" means that certain Financing Agreement, dated as of June 30, 2022, by and among Seller, certain subsidiaries of Seller as guarantors, the lenders from time to time party thereto and Tao Talents, LLC, as administrative agent.

"Seller Fundamental Representation" means the representations and warranties of Seller set forth in [***].

9

"Seller Indemnified Party" shall have the meaning set forth in Section 10.3(b).

"Seller Intellectual Property" means any Excluded Intellectual Property that [***]. The patents within the Seller Intellectual Property existing as of the Closing Date are set forth in Section 1.1 of the Disclosure Schedule. Notwithstanding anything to the contrary herein, the Seller Intellectual Property shall exclude [***].

"Solvent", when used with respect to any Person, means that, as of any date of determination, (a) the amount of the "fair saleable value" of the assets of such Person on a going concern basis will, as of such date, exceed (i) the value of all "liabilities of such Person, including contingent and other liabilities" as of such date, as such quoted terms are generally determined in accordance with applicable United States federal laws governing determinations of the insolvency of debtors and (ii) the amount that will be required to pay the probable liabilities of such Person on its existing debts (including contingent liabilities) as such debts become absolute and matured, (b) such Person will not have, as of such date, an unreasonably small amount of capital for the operation of the businesses in which it is engaged or proposed to be engaged following such date and (c) such Person will be able to pay its liabilities, including contingent and other liabilities, as they mature. For purposes of this definition, each of the phrases "not have an unreasonably small amount of capital for the operation of the businesses in which it is engaged or proposed to be engaged" and "able to pay its liabilities, including contingent and other liabilities, as they mature" means that such Person will be able to generate enough cash from operations, asset dispositions or refinancing, or a combination thereof, to meet its obligations as they become due.

"Subsidiary" means an entity as to which Seller or Purchaser or any other relevant entity, as the case may be, owns directly or indirectly fifty percent (50%) or more of the voting power or other similar interests. Any Person which comes within this definition as of the date of this Agreement but thereafter fails to meet such definition shall from and after such time not be deemed to be a Subsidiary of Seller or Purchaser or any other relevant entity, as the case may be. Similarly, any Person which does not come within such definition as of the date of this Agreement but which thereafter meets such definition shall from and after such time be deemed to be a Subsidiary of Seller or Purchaser or any other relevant entity, as the case may be.

"Tax" or "Taxes" means all taxes, including income, excise, property, ad valorem, sales or use, value added, profits, license, withholding, payroll, employment, net worth, capital gains, transfer, stamp, social security, environmental, occupation and franchise taxes imposed by any Taxing Authority, together with any interest, penalties and additions to tax attributable thereto.

"Tax Return" or "Tax Returns" means any return, report, declaration, information return, statement or other document filed or required to be filed with any Taxing Authority, in connection with the determination, assessment or collection of any Tax.

"Taxing Authority" means any Governmental Authority exercising any authority to impose, regulate or administer the imposition of Taxes.

"Territory" means the United States.

"Third Party" means any Person who is not a Party. "Third Party" shall not include any Affiliate of a Party, except where the context otherwise requires.

"Transaction" shall have the meaning set forth in the Recitals.

"Transfer of Booking of Sales" means the point in time at which the First Commercial Sale occurs.

"Transfer Taxes" means any sales, use, excise, transfer, value added, conveyance, documentary transfer, stamp, recording, registration or other similar Tax (including any notarial fee) imposed in connection with, or otherwise relating to, the transactions contemplated by this Agreement or the recording of any sale, transfer or assignment of property (or any interest therein) effected pursuant to this Agreement.

"Transferred Books and Records" means, subject to Section 2.3, all current and historical books, and records owned by Seller or its Affiliates, in whatever form kept, including electronic form, exclusively related to the Product in the Territory or primarily related to the Product in the Territory and reasonably necessary for the development, commercialization, Manufacturing, packaging, distributing, marketing and selling of the Product in the Territory, including but not limited to all technological, scientific, chemical, biological, pharmacological, toxicological and regulatory material and information primarily related to the Product in the Territory or otherwise reasonably necessary for the development, commercialization, Manufacturing, packaging, distributing, marketing and selling of the Product, including records related to past price increases (exclusively with respect to the Product in the Territory) and records that are exclusively related to the Product in the Territory and are necessary or useful for Purchaser in its compliance with the Inflation Reduction Act; provided, however, that the Transferred Books and Records shall not include (a) invoices and government claims, (b) books or records that are subject to restrictions on transfer pursuant to applicable Law regarding personally identifiable information or subject to privacy policies regarding personally identifiable information, (c) Tax Returns of Seller or its Affiliates (or their respective beneficial owners) or any Tax-related documentation or records, other than any Tax documentation or records exclusively related to the Business, (d) personnel files for employees of Seller or its Affiliates or (e) any records relating to the negotiation and consummation of the transactions contemplated by this Agreement or any of the Ancillary Agreements, including (i) communications with Third Parties and analyses relating to such transactions and (ii) communications with legal counsel representing Seller or any of its Affiliates or their respective licensees. For clarity "Transferred Books and Records" shall not include "Product Data" or "Regulatory Data".

"Transferred Domain Names" means the internet domain names that are listed on Section 5.10(a) of the Disclosure Schedule.

"Transferred Intellectual Property" means, collectively, (a) the Transferred Domain Names, the Transferred Know-How, Transferred Marks, the Transferred Patents, and Transferred Regulatory Data, and (b) all copyrights in the Territory owned by Seller that are solely related to the Product, RET inhibitor compounds claimed or covered by the Transferred Patents.

"Transferred Know-How" means all information, inventions (whether patentable or not), know-how and data owned by Seller and/or its Affiliates and relating exclusively to the Product in the Territory, including, scientific and regulatory know-how, instructions, trade secrets, processes, formulae, product specifications, finished goods analytical test methods, stability data, Manufacturing and quality control data for finished products, but excluding any information, know-how, data and/or other above-referred assets which relate to Excluded Assets.

"Transferred Marks" means the marks that are listed on Section 5.10(a) of the Disclosure Schedule.

"Transferred Patents" means the patent registrations and applications listed on Section 5.10(a) of the Disclosure Schedule.

"Transferred Regulatory Data" means all Regulatory Data owned by Seller and/or its Affiliates for the Product in the Territory.

"Transferred Regulatory Filings and Approvals" means the Regulatory Filings and the Regulatory Approvals listed on Section 2.1(f) of the Disclosure Schedule.

"Transition Agreement" shall have the meaning set forth in Section 4.1(b)(v).

"Upfront Purchase Price" means Ten Million Dollars (\$10,000,000).

"Valid Claim" means, with respect to a particular country, a claim of (a) an issued and unexpired patent (or a supplementary protection certificate or foreign equivalent thereof) that has not (i) irretrievably lapsed or been abandoned, permanently revoked, dedicated to the public or disclaimed or (ii) been held invalid, unenforceable or not patentable by a court, governmental agency, national or regional patent office or other appropriate body that has competent jurisdiction, which holding, finding or decision is final and unappealable or unappealed within the time allowed for appeal or (b) a pending patent application, which claim has not been abandoned or finally disallowed

without the possibility of appeal; provided that, if a pending patent application has been pending for longer than [***] from the date of filing of the initial priority application, then such corresponding claim in such pending patent application will not be deemed to be a Valid Claim; provided that, if a claim ceases to be a Valid Claim by reason of the foregoing subclause (b), then such claim will again be deemed a Valid Claim in the event such claim subsequently issues prior to the end of the Royalty Term in such country.

Section 1.2 Other Definitional Provisions.

- (a) The words "hereof", "herein", "hereto" and "hereunder" and words of similar import, when used in this Agreement, shall refer to this Agreement as a whole and not to any particular provision of this Agreement.
- (b) The terms defined in the singular shall have a comparable meaning when used in the plural, and vice versa.
- (c) The terms "dollars" and "\$" shall mean United States of America dollars.
- (d) The term "including" shall mean "including, without limitation."
- (e) When a reference is made in this Agreement to an Article, a Section, an Exhibit or Schedule, such reference shall be to an Article of, a Section of, or an Exhibit or Schedule to, this Agreement unless otherwise indicated.

ARTICLE II

PURCHASE AND SALE; LICENSES

Section 2.1 Purchase and Sale of Assets. Upon the terms and subject to the conditions set forth herein and subject to Section 2.2, at the Closing, Seller shall sell, convey, assign and transfer to Purchaser, and Purchaser shall purchase, acquire and accept from Seller, free and clear of all Liens, other than Permitted Encumbrances, all of Seller's right, title and interest in those assets described in the following clauses (a) through (j) (the "Purchased Assets":)

- (a) all contracts and licenses set forth on Section 2.1(a) of the Disclosure Schedule (collectively, the "Assumed Contracts"), and any causes of action, lawsuits, judgments, claims or demands with respect to such Assumed Contracts;
- (b) the Transferred Intellectual Property and the Transferred Regulatory Data, but, for clarity, excluding the Excluded Intellectual Property;
- (c) the Product Data;
- (d) the Product Registrations set forth on Section 2.1(d) of the Disclosure Schedule;
- (e) the Transferred Books and Records including, but not limited to, those set forth on Section 2.1(e) of the Disclosure Schedule;

(f) all governmental licenses, registrations, listings, permits, consents or other governmental authorizations of Seller or its Affiliates that are primarily related to the Product in the Territory, including the Transferred Regulatory Filings and Approvals, except those governmental licenses, registrations, listings, permits, consents or other governmental authorizations set forth on Section 2.1(f) of the Disclosure Schedule because the transfer thereof would violate or would not be permitted or effective under applicable Law or the terms of such license or such license is otherwise not transferable;

(g) all rights of Seller under any confidentiality, non-competition, nondisclosure, assignment of invention or proprietary rights or similar agreements, in each case, solely to the extent exclusively related to the Purchased Assets or the Product in the Territory;

(h) any accounts receivable of Seller or any of its Affiliates, and other rights to receive payment directly allocable to the sale of the Product in the Territory on or after the Closing Date ("Included Accounts Receivable");

(i) all rights of Seller under or pursuant to all warranties, representations and guarantees made by suppliers, manufacturers and contractors, in each case, solely to the extent relating to Product sold, or services provided, to Seller related to the Territory or to the extent affecting any Purchased Asset; and

(j) [***].

Notwithstanding anything to the contrary in this Agreement, the Purchased Assets shall not include any Excluded Assets.

Section 2.2 Consents.

(a) Notwithstanding any other provision of this Agreement and other than with respect to the Seller Financing Agreement and the consent of any Third Party required for the development, commercialization, Manufacturing, packaging, distributing, marketing and selling of the Product, this Agreement does not constitute an agreement to sell, convey, assign, assume, transfer or deliver any interest in any Purchased Asset if an attempted direct or indirect assignment thereof, or agreement to sell, convey, assign, assume, transfer or deliver, without the consent of any Third Party, would constitute a breach or other contravention of the rights of such Third Party under an Assumed Contract (each such Purchased Asset, a "Non-Assignable Right"). If any direct or indirect transfer or assignment or agreement to do so by Seller to, or any direct or indirect assumption by Purchaser of, any interest in, or liability, obligation or commitment under, any Purchased Asset requires the consent of a Third Party, then such transfer, assignment or assumption or agreement shall be made subject to such consent being obtained.

(b) If any such consent referred to in Section 2.2(a) is not obtained prior to the Closing Date (excluding with respect to any Governmental Authority or any Governmental Authorization), the Closing shall nonetheless take place, and notwithstanding anything to the contrary in this Agreement or any Ancillary Agreement, (a) this Agreement and the related instruments of transfer shall not constitute an assignment or transfer of the applicable Non-Assignable Right until and unless such consent is obtained (at which point such Non-Assignable Right will be deemed to have been assigned or transferred under this Agreement on such date), and, if requested by Purchaser, Seller shall use commercially reasonable efforts to obtain such consent, and in any case as soon as possible after the Closing Date; and (b) upon delivery of Purchaser's written election to Seller, (i) such Non-Assignable Right shall be considered an Excluded Asset and Purchaser shall have no Liability whatsoever with respect to any such Non-Assignable Right or any Liability with respect thereto (and any consent to transfer or assignment obtained thereafter shall have no effect) or (ii) Seller and Purchaser shall cooperate to obtain for Purchaser substantially all of the practical benefit and burden of such Non-Assignable Right, including by (A) entering into appropriate and reasonable alternative arrangements on terms mutually agreeable to Purchaser and Seller, (B) subject to the consent and control of Purchaser, enforcement, at the cost and for the account of Purchaser, of any and all rights of Seller against the other party thereto arising out of the breach or cancellation thereof by such other party or otherwise and (C) continuing to comply with, and perform, any contractual obligations associated with such Non-Assignable Right. To the extent that Purchaser is provided the benefits and burdens of any Purchased Asset or right, benefit or obligation thereunder or resulting therefrom referred to herein (whether from Seller or otherwise) as if the appropriate consent had been obtained, at Purchaser's option, Purchaser shall either (x) discharge and perform the Liabilities of Seller thereunder (other than any Liability arising in connection with a breach or violation by Seller of such Purchased Asset or any other applicable Contract, Governmental Authorization, Regulatory Approval or Law) or in connection therewith, as applicable, to the same

extent as if the appropriate consent had been obtained or (y) such Purchased Asset shall not be considered to have been sold, conveyed, assigned, assumed, transferred or delivered pursuant to this Agreement.

(c) Notwithstanding anything to the contrary set forth in this Agreement, Purchaser agrees that no representation, warranty or covenant of Seller contained herein shall be breached or deemed breached, and no

condition to Purchaser's obligations to close the transactions contemplated by this Agreement shall be deemed not satisfied, as a result of (i) the failure to obtain any such consent; or (ii) any Legal Proceeding commenced or threatened by or on behalf of any Person arising out of or relating to the failure to obtain any consent or any such default, acceleration or termination.

Section 2.3 Excluded Assets. Nothing herein contained shall be deemed to sell, transfer, assign or convey the Excluded Assets to Purchaser, and Seller or its Affiliates shall retain all right, title and interest to, in and under the Excluded Assets. "Excluded Assets" means all assets, properties, interests and rights of Seller and its Affiliates other than the Purchased Assets, including each of the following assets:

- (a) all right, title or interest to the Product outside of the Territory;
- (b) any starting material, active pharmaceutical ingredient, intermediates, drug substance, or bristock or labeled drug product (with it being understood that Purchaser will purchase the inventory described in the Material Transfer Agreement directly from Seller on the terms set forth in the Material Transfer Agreement);
- (c) the Excluded Contracts;
- (d) all cash, cash equivalents, securities or negotiable instruments, bank deposits or similar cash items of Seller and its Affiliates;
- (e) any accounts receivable of Seller or any of its Affiliates, and other rights to receive payment related to the sale of the Product (i) in the Territory prior to the Closing Date or (ii) anywhere in the world outside of the Territory at any time (collectively, the "Excluded Accounts Receivable");
- (f) all books and records relating to (i) the Product and the Purchased Assets that are required to be maintained by Seller itself or its Affiliate under applicable Laws or (ii) the Product anywhere in the world outside of the Territory, in each case other than the Transferred Books and Records; provided that Seller shall deliver to Purchaser copies of any such other books and records relating to the Product in the Territory and the Purchased Assets upon the reasonable request of Purchaser to the extent permitted by applicable Laws and consistent with Section 7.11(b);
- (g) all Intellectual Property of Seller and its Affiliates that is not Transferred Intellectual Property (collectively, the "Excluded Intellectual Property");
- (h) any documents or other materials related to the Product containing trademarks, service marks, logos or tradenames owned or controlled by Roche or CStone;
- (i) all insurance policies or rights to proceeds thereof relating to the Purchased Assets or the Product;
- (j) any rights, claims or causes of action of Seller or any of its Affiliates against Third Parties in connection with the Purchased Assets or the Product arising out of (i) events in the Territory occurring on or prior to the Closing Date or (ii) events solely outside of the Territory;
- (k) all Tax Returns and financial statements of Seller and its Affiliates and all books and records (including working papers) related thereto;
- (l) all refunds for Taxes or other Tax assets (i) of Seller and its Affiliates, or
 - (i) relating to the Purchased Assets or the development, commercialization, Manufacturing, packaging, distributing, marketing and selling of the Product with respect to a Pre-Closing Tax Period;

(m) all of Seller's or any of its Affiliates' causes of action, claims, credits, demands or rights of set-off against Third Parties, to the extent related exclusively to any Excluded Asset;

(n) all rights that accrue to Seller and its Affiliates under this Agreement and the Ancillary Agreements;

(o) any real or personal property other than personal property expressly referred to in Section 2.1;

(p) all Product Registrations except for those set forth on Section 2.1(d) of the Disclosure Schedule, and except for the marketing applications covering the Purchased Assets and governmental licenses, permits or other governmental authorizations of Seller or its Affiliates that are exclusively related to the development, commercialization, Manufacturing, packaging, distributing, marketing and selling of the Product in the Territory; and

(q) those assets listed on Section 2.3(g) of the Disclosure Schedule;

Section 2.4 Assumption of Liabilities. Upon the terms and subject to the conditions of this Agreement, at the Closing, Purchaser shall assume, and agrees to pay, perform, satisfy and discharge when due, the following Liabilities (the "Assumed Liabilities"):

(a) any Liability arising from and after the Closing Date arising out of or otherwise in any way relating to the ownership, possession, use or operation of any of the Purchased Assets, or the development, commercialization, Manufacturing, packaging, distributing, marketing and selling of the Product in the Territory, in each case by Purchaser, any of its Affiliates or their respective Licensees or subcontractors [***];

(b) except as otherwise provided in this Agreement, all Liabilities in respect of any Legal Proceeding (whether class, individual or otherwise in nature, in law or in equity) that is commenced on or after the Closing Date, to the extent (i) first arising or accruing after the Closing Date and (ii) [***];

(c) all Liabilities for Taxes relating to the Purchased Assets or the development, commercialization, Manufacturing, packaging, distributing, marketing and selling of the Product in the Territory that are attributable to a Post-Closing Tax Period and [***] of all Transfer Taxes;

(d) except as otherwise provided in this Agreement, all Liabilities arising out of, relating to, resulting from, or in connection with the performance by Purchaser Group of the Assumed Contracts, in each case arising in respect of periods or occurrences subsequent to the Closing Date; and

(e) any and all other Liabilities arising after the Closing Date relating to the Purchased Assets and the Product in the Territory that are not Retained Liabilities, including to any Governmental Authority and fees arising from or related to any Transferred Intellectual Property.

Section 2.5 Retained Liabilities. Notwithstanding any provision in this Agreement, Seller shall retain and be responsible for, and Purchaser shall not assume or be liable for, the following liabilities (the "Retained Liabilities"):

(a) all Liabilities in respect of any Legal Proceeding (whether class, individual or otherwise in nature, in law or in equity), arising out of or to the extent relating to or otherwise in any way relating to the ownership, possession, use or operation of any of the Purchased Assets or the development, commercialization, Manufacturing, packaging, distributing, marketing and selling of the Product [***] prior to the Closing Date solely to the extent not arising out of or accruing as a result of any facts or occurrences arising after the Closing as a result of Purchaser's activities Exploiting the Licensed Product, including any failure to perform or other breach, default or violation by Purchaser or any of its Affiliates after the Closing;

(b) all Liabilities for accounts payable, accrued expenses and similar items to the extent that they arise or are incurred prior to the Closing Date (even if such Liabilities are invoiced after the Closing);

- (c) all Liabilities of Seller to the extent related to the Excluded Assets;
- (d) all Liabilities for Taxes relating to the Purchased Assets for a Pre-Closing Tax Period;
- (e) [***]; and
- (f) [***].

Section 2.6 License Grants; Right of Reference.

(a) Licenses and Sublicenses to Purchaser Under Excluded Intellectual Property.

(i) License Grant. Effective as of the Closing, Seller hereby grants Purchaser (A) an exclusive, royalty-free, fully paid-up, perpetual and irrevocable [***], transferable, sublicensable (through multiple tiers) license under the Seller Intellectual Property (other than trademarks, service marks, logos or tradenames) Controlled by Seller to Exploit the Licensed Products in the Field in the Territory, subject to the remainder of this Section 2.6(a), and (B) a non-exclusive, royalty-free, fully paid-up, perpetual, irrevocable (except in the event of Purchaser's material breach of this Agreement), transferable, sublicensable (through multiple tiers) license under the Seller Intellectual Property (other than trademarks, service marks, logos or tradenames) Controlled by Seller to Manufacture or have Manufactured the Licensed Products in the ROW Territory for (x) Exploitation in the Territory and [***].

(ii) Sublicense under Roche Collaboration Agreement. Seller hereby grants to Purchaser an exclusive, royalty-free, fully paid-up, perpetual and irrevocable [***], transferable sublicense under the Roche Technology (as defined in the Roche Collaboration Agreement) to Exploit (as defined in the Roche Collaboration Agreement) the Lead Product (as defined in the Roche Collaboration Agreement) in the Territory (for clarity, as defined in this Agreement). For clarity, no licenses are granted with respect to a Roche Other Component (as defined in the Roche Collaboration Agreement).

(iii) Retained Rights of Seller. Seller hereby retains the right to, under the Seller Intellectual Property and the Roche Technology (as defined in the Roche Collaboration Agreement), itself or through its Affiliates, (sub)licensees or subcontractors, (A) Manufacture Licensed Products anywhere in the world for (i) Exploitation by Purchaser, its Affiliates and their respective Licensees or subcontractors in the Territory, and (ii) Exploitation by Seller, its Affiliates, and their respective (sub)licensees anywhere in the world outside of the Territory, or the Territory to perform activities under the Transition Agreement [***], (B) [***], and (C) perform its and Roche's obligations under this Agreement, the Transition Agreement, [***], and the CStone Agreements, either itself or through its Affiliates, (sub)licensees (including without limitation, Roche) or subcontractors, including for purposes of performing activities under the Ancillary Agreements or a global safety data exchange agreement related to Licensed Products.

(iv) Combination Products. Notwithstanding anything to the contrary in this Agreement, for purposes of the license grant under this Section 2.6(a), with respect to any Licensed Product that is a Combination Product, such license will only include a license with respect to the Compound component of such Combination Product.

(v) Seller Intellectual Property. [***].

(b) Licenses to Seller Under Transferred Intellectual Property. Effective as of the Closing, Purchaser hereby grants to Seller:

(i) A non-exclusive, royalty-free, fully paid-up, transferable, perpetual, irrevocable [***], sublicensable (through multiple tiers) license under the Transferred Intellectual Property to, either itself or through its Affiliates, (sub)licensees (including without limitation, Roche) or subcontractors, (A) perform its and Roche's obligations under this Agreement, the Transition Agreement,

[***], and the CStone Agreements, including for purposes of performing activities under the Ancillary Agreements or a global safety data exchange agreement related to Licensed Products, and [***];

(ii) An exclusive, royalty-free, fully paid-up, perpetual and irrevocable [***], transferrable, sublicensable (through multiple tiers) license under the Transferred Intellectual Property to Exploit Licensed Products in the Field anywhere in the world outside the Territory; and

(iii) a non-exclusive, royalty-free, fully paid-up, perpetual and irrevocable [***], transferrable, sublicensable (through multiple tiers) license under the Transferred Intellectual Property to Manufacture, either itself or through its Affiliates, (sub)licensees (including without limitation, Roche) or subcontractors, Licensed Products anywhere in the world for:

(1) Exploitation by Purchaser, its Affiliates and their respective Licensees or subcontractors in the Territory, and

(2) Exploitation by Seller, its Affiliates, and their respective (sub)licensees anywhere in the world outside of the Territory, or in the Territory to perform activities under the Transition Agreement [***].

(iv) Right of Reference to Seller. In connection with the licenses granted by Purchaser to Seller pursuant to Section 2.6(a)(i), effective as of the Closing, Purchaser hereby grants Seller, its Affiliates and their respective (sub)licensees or subcontractors a perpetual, irrevocable, sublicensable (through multiple tiers) and transferrable right of reference to any Regulatory Filings and Approvals for the Product in the Territory and all data and other know-how included or referenced therein in support of any such Transferred Regulatory Filings and Approvals, specifically including Transferred Regulatory Data and patient registries (and any data and other know-how therein) for the Product in the Territory, which Regulatory Filings, Regulatory Approvals, data and other information is Controlled by Purchaser or any of its Affiliates, solely for the purpose of Seller, its Affiliates, and their respective (sub)licensees or subcontractors (i) Manufacturing the Licensed Product anywhere in the world for (A) for Exploitation by Purchaser, its Affiliates and their respective (sub)licensees or subcontractors in the Territory and (B) for Exploitation by Seller, its Affiliates and their respective (sub)licensees or subcontractors anywhere in the world outside of the Territory (including, without limitation, on behalf of CStone and Roche and their respective Third Party contract research organizations, contract manufacturing organizations, distributors and other sublicensees and subcontractors), (ii) Exploiting the Products anywhere in the world outside of the Territory or in the Territory to perform activities under the Transition Agreement [***], (iii) [***], and (iv) performing its and Roche's obligations under this Agreement, the Transition Agreement, [***] and the CStone Agreements, including for purposes of performing activities under the Ancillary Agreements or a global safety data exchange agreement related to Licensed Products. Purchaser shall provide to Seller access to any reasonably required Purchaser know-how to facilitate Seller's use of the Regulatory Filings and Regulatory Approvals as provided in this Section 2.6(b)(iv). Purchaser shall duly execute and deliver, or cause to be duly executed and delivered, such instruments and shall do and cause to be done such reasonable acts and things, as may be necessary under, or as the Seller may reasonably request, to effectuate the rights of reference contemplated in this Section 2.6(b)(iv).

(c) Patent Coordination Team. The Parties shall form a patent coordination team (the "Patent Coordination Team") within [***] after the Closing Date. The Patent Coordination Team shall meet as it deems necessary but no less than [***]. Purchaser will organize such meetings. Through the Patent Coordination Team, (i) each Party shall regularly provide the other Party with copies of all material matters relating to the preparation, filing, prosecution and maintenance of patents Covering the Product [***] (ii) consult with each other on patent strategy for (A) filing, prosecuting, maintaining, and enforcing patents that Cover the Product (including providing all draft submissions, applications, and correspondence with any applicable patent office in sufficient time to allow for review and comment, and providing a list of filing deadlines at least [***] prior to any filing deadline) and (B) defending against patent challenges specifically related to the Product, (iii) review Seller, Purchaser, and CStone publications related to the Product at least [***] prior to the date of submission for publication or of public disclosure, and (iv) consider [***] and incorporate where appropriate the other Party's comments related thereto, provided that the publishing Party shall retain the sole authority to submit the proposed publication. [***] Purchaser shall, at Seller's

Party shall bear its own costs and expenses it may incur in connection with its review and consultation concerning any such patents.

(d) Abandonment. If Seller intends to abandon patent applications for any patent within the Seller Intellectual Property outside of the Territory [***].

(e) Negative Covenant. Each Party covenants that it shall not use or practice any of the other Party's Intellectual Property rights licensed to it under this Article II in a manner that would constitute infringement or misappropriation of such Intellectual Property rights except for the purposes expressly permitted in the applicable license grant. [***].

(f) No Implied Licenses. Except as explicitly set forth in this Agreement, neither Party grants to the other Party any license, express or implied, under its Intellectual Property right.

(g) Bankruptcy Code § 365(n) Election. All rights and licenses now or hereafter granted under or pursuant to this Agreement, are rights to "Intellectual Property" (as defined in Section 101(35A) of Title 11 of the United States Code (such Title 11, the "Bankruptcy Code")). Each Party, as licensee of such rights under this Agreement, will retain and may fully exercise all of its rights and elections under the United States Bankruptcy Code. In the event of the commencement of a bankruptcy proceeding by or against a Party under the Bankruptcy Code (the "Insolvent Party"), the other Party will be entitled to a complete duplicate of (or complete access to, as appropriate) any Intellectual Property licensed to it under this Agreement and all embodiments of such Intellectual Property (including all information related to such Intellectual Property and rights of reference with respect to Regulatory Filings and Regulatory Approvals), and same, if not already in its possession, will be promptly delivered to it (a) upon any such commencement of a bankruptcy proceeding upon its written request therefore, unless the Insolvent Party continues to perform all of its obligations under this Agreement, or (b) if not delivered or granted under clause (a) above, upon rejection of this Agreement by or on behalf of the Insolvent Party upon written request therefore by the other Party. The Insolvent Party (in any capacity, including debtor-in-possession) and its successors and assigns (including any trustee) agrees not to interfere with the exercise by other Party or its Affiliates of its rights and licenses to such Intellectual Property and such embodiments of Intellectual Property in accordance with this Agreement, and agrees to assist the other Party and its Affiliates in obtaining such Intellectual Property and such embodiments of Intellectual Property in the possession or control of Third Parties as reasonably necessary or desirable for the other Party to exercise such rights and licenses in accordance with this Agreement. The Parties hereto acknowledge and agree that all payments by Purchaser to Seller under this Agreement, other than royalty payments pursuant to Section 3.2(b), do not constitute royalties within the meaning of Bankruptcy Code § 365(n) or relate to licenses of Intellectual Property under this Agreement. The foregoing provisions are without prejudice to any rights the Parties may have arising under the Bankruptcy Code or other applicable Laws.

(h) Covenant.

(i) Seller shall not, and shall cause its Affiliates and (sub)licensees not to, Exploit (itself or through any Third Party) or enable any Third Party to Exploit, [***]. Notwithstanding the foregoing, in the case of a Change of Control of Seller, this Section 2.6(g) shall not to apply to the acquirer or successor entity in such Change of Control.

(ii) [***].

18

ARTICLE III

CONSIDERATION

Section 3.1 Purchase Price.

(a) Upon the terms and subject to the conditions of this Agreement, in consideration of the sale, transfer and assignment of the Purchased Assets, and the assumption and satisfaction of the Assumed Liabilities, the Purchaser shall pay the Purchase Price. Purchaser shall deliver or cause to be delivered the Purchase Price to Seller as follows:

(i) The Upfront Purchase Price shall be due and payable by Purchaser to Seller on [***] after the Transfer of Booking of Sales; and

(ii) The Delayed Purchase Price shall be due and payable by Purchaser to Seller on the first (1st) anniversary of the Closing Date [***].

(b) Payment of the Upfront Purchase Price shall be made by wire transfer of immediately available funds to such bank account of Seller as shall have been notified in writing to Purchaser by Seller no less than [***] in advance of the anticipated date of the Transfer of Booking of Sales. Payment of the Delayed Purchase Price shall be made by wire transfer of immediately available funds to such bank account of Seller as shall have been notified in writing to Purchaser by Seller no less than [***] in advance of the date specified by Section 3.1(a)(ii).

(c) The Parties agree that the Purchase Price shall be allocated, in accordance with Section 1060 of the U.S. Internal Revenue Code of 1986, as amended (the "Code") and the Treasury Regulations promulgated thereunder, among the Purchased Assets and Assumed Liabilities in a manner consistent with [***] of the Purchase Price being allocated to Class VI assets. Both Parties shall report the transaction consistent with such allocation and shall not take a position contrary thereto for income Tax purposes unless required by applicable Law.

Section 3.2 Additional Consideration. As additional consideration for the sale, transfer and assignment of the Purchased Assets, and the assumption and satisfaction of the Assumed Liabilities, Purchaser shall make Milestone Payments and payments for royalties on Net Sales of the Licensed Product on the terms and subject to the conditions set forth in this Section 3.2.

(a) Milestone Payments.

(i) Development Milestone. If, at any time after the Closing Date until the [***] anniversary thereof, a Development Milestone Event is achieved, then Purchaser will pay Seller the corresponding payment described in the table below (each, a "Development Milestone Payment") within [***] following achievement of such Development Milestone Event to occur. "Development Milestone Event" shall mean each of the events described in the table below.

Development Milestone Event		Development Milestone Payment
1	[***]	[***]
2	[***]	[***]

(ii) [***] Sales Milestones. At any time after the Closing until the [***] anniversary of the Closing Date, Purchaser shall pay to Seller the following irrevocable, nonrefundable, non-creditable, one-time milestone payments (each, a "Sales Milestone Payment" and together with the Development Milestone Payments, the "Milestone Payments") for each Calendar Year during the Royalty Term in which the aggregate Net Sales of all Payment Products by Purchaser, its Affiliates and Licensees in the Territory achieves the sales threshold set forth in the table and subsequent paragraph below corresponding to such Sales Milestone Payment (each, a "Sales Milestone Event" and together with Development Milestone Events, the "Milestone Events"):

19

Upon the first achievement of Calendar Year cumulative annual Net Sales of all Payment Products in the Territory		Milestone Payment
1	[***]	[***]
2	[***]	[***]
3	[***]	[***]
4	[***]	[***]
5	[***]	[***]

(iii) Within [***] after Purchaser becomes aware that a Milestone Event was achieved, Purchaser will notify Seller thereof in writing, including identifying the event and the date of its achievement. Purchaser shall make the corresponding Milestone Payment to Seller within [***] following the date of the achievement of the applicable Milestone Event. Each Milestone Payment will be payable only one time and only upon the first achievement of the applicable Milestone Event for Net Sales of the Payment Products in the Territory, and no amounts would be due for repeated achievements. For clarity, more

than one (1) Milestone Event may occur in a single Calendar Year. [***] The Milestone Events are intended to be sequential, such that satisfaction of any later stage Milestone Event by the Payment Products shall be deemed to have satisfied all earlier stage Milestone Events for Payment Products (to the extent not previously satisfied).

(b) Divestitures. If at any time after the Closing until the payment in full of all Milestone Payments, (i) Purchaser undergoes a Change of Control transaction, or (ii) Purchaser Divests to a Third Party or any Affiliate any Licensed Product and its associated Intellectual Property, the definitive agreement for such Purchaser Change of Control or Divestiture shall provide for the acquirer or successor entity in such Purchaser Change of Control or Divestiture to assume the obligations of Purchaser set forth in Section 2.6(h) and this ARTICLE III.

(c) Flash Reports. Within [***] after the end of each Calendar Quarter, beginning with the Calendar Quarter in which the First Commercial Sale occurs, Purchaser shall provide Seller with a flash report providing Purchaser's good faith estimate of the Royalty Payment due to Seller in respect such Calendar Quarter (expressed in U.S. dollars) and which, if any, Milestone Event occurred during such Calendar Quarter.

(d) Royalty Payments and Royalty Reports.

(i) Royalty Payments. During the applicable Royalty Term, Purchaser will pay to Seller royalties ("Royalty Payments") based on the aggregated Net Sales of all Payment Products (whether by Purchaser, its Affiliates or Licensees) at the rates set forth in the table below:

Calendar Year Cumulative Annual Net Sales of all Payment Products in the Territory	Royalty (% of Net Sales)
[***]	[***]
[***]	[***]
[***]	[***]
[***]	[***]
[***]	[***]

(ii) Royalty Term. On a Payment Product-by-Payment Product basis, Royalty Payments will be due under this Section 3.2(b) during the period commencing on the First Commercial Sale and ending [***] (such period for such Payment Product, the "Royalty Term").

20

(iii) Royalty Payments; Royalty Reports. Commencing with the Calendar Quarter in which the First Commercial Sale occurs, Purchaser (a) will pay to Seller the Royalty Payments within [***] after the end of each Calendar Quarter, and (b) will provide to Seller concurrently with such Royalty Payment a written report setting forth, on a Payment Product-by-Payment Product basis, (i) the amount of Net Sales during such Calendar Quarter; (ii) the applicable royalty rates after applying any permitted deductions pursuant to Section 3.2(d)(i); and (iii) a calculation of the royalties due to Seller for such Calendar Quarter (the "Royalty Report").

(iv) Royalty Payment Reductions.

(1) [***]. Subject to Section 3.2(d)(iv)(4), Purchaser will be entitled to deduct from Royalty Payments otherwise payable to Seller on Net Sales of Payment Products by an amount equal to [***] of any [***] actually paid by Purchaser or any of its Affiliates or Licensees [***] applicable to such Payment Product in the Territory, up to a

maximum reduction under this Section 3.2(d)(iv)(1) of [***] of the Royalty Payments otherwise owed to Seller hereunder for the applicable Calendar Quarter for such Payment [***].

(2) **No Exclusivity.** Subject to Section 3.2(d)(iv)(4), if at any time during the Royalty Term a Payment Product is sold in the Territory by Purchaser or an Affiliate or Licensee and such Payment Product at the time of such sale:

(A) is not Covered by a Valid Claim within the Transferred Patents, but is within any applicable regulatory exclusivity period, then the applicable royalty in effect with respect to such sale of the Payment Product as specified in Section 3.2(d)(i) will be reduced by [***]; or

(B) is not Covered by a Valid Claim within the Transferred Patents and is not within any applicable regulatory exclusivity period, then, the applicable royalty in effect with respect to such sale of the Payment Product as specified in Section 3.2(d)(i) will be reduced by [***].

(3) [***]. Subject to Section 3.2(d)(iv)(4), on a Payment Product-by-Payment Product basis, if during any Calendar Quarter, there is [***].

(4) **Maximum Aggregate Reduction.** On a Payment Product-by-Payment Product basis, the maximum aggregate of all reductions under Section 3.2(d)(iv)(1)-(3) will reduce the amount of royalties owed to Seller with respect to such Payment Product hereunder in any given [***] by no more than [***] from the amounts otherwise due to Seller hereunder in such [***].

(e) **Milestone and Royalty Obligations.** Purchaser shall use (and shall cause its Affiliates and Licensees to use) Commercially Reasonable Efforts to achieve the Milestone Events and commercialize at least [***]. Purchaser shall (and shall cause its Affiliates and Licensees to) not take any action with the intent of not achieving any Milestone Event. For the purposes of this Section 3.2(e), "Commercially Reasonable Efforts" means, with respect to the performance of an obligation under this Agreement, [***].

(f) **Currency of Payments; Payments.** All amounts payable and calculations under this Agreement will be in dollars. As applicable, Net Sales and any royalty reductions will be translated into dollars using the average of the applicable daily exchange rates published in *The Wall Street Journal* (or any other qualified source that is acceptable to both Parties) for [***]. All payments due to Purchaser under this Agreement will be paid in Dollars by bank wire transfer of immediately available funds.

(g) **Interest on Overdue Payments.** Interest will be payable on any payments that are not paid on or before [***] after the date such payments are due under this Agreement at a rate per annum equal to the lesser of (a) [***] the prime rate as reported in *The Wall Street Journal*, Eastern Edition on the first day of each in which

such payments are overdue or (b) the highest rate allowed by applicable Law, as applicable and commencing on the date such payments are due and ending when paid.

(h) **Reporting Obligations.** Until the earlier of (i) the [***] anniversary of the Closing Date or (ii) such time as all of the applicable Milestone Payments and royalties have been paid, Purchaser shall provide to Seller [***] written report [***] with respect to (A) commercialization of the Licensed Products and (B) progress towards and achievement of the Milestone Events during the [***]. Purchaser shall keep data and records in accordance with its customary internal practices concerning the activity and progress related to the Licensed Products.

(i) **Records and Audit.** For as long as Milestone Events or royalty payments are outstanding, Purchaser shall keep, and shall cause its Affiliates and its and their Licensees that sell the Licensed Products to keep, records that are necessary to ascertain the payments due hereunder. Such records shall be kept for such period of time required by applicable Laws, but no [***] following the end of the Calendar Quarter to which they pertain. For as long as Milestone Events or royalty payments are outstanding, Seller shall not more than [***] have the right to have an external independent registered public accounting firm of Purchaser's choosing inspect Purchaser's records for the purpose of determining the accuracy of Milestone Payments or royalty payments for a period covering not [***] following the Calendar Quarter to which they pertain. [***]. Such auditors shall keep confidential any information obtained during such inspection

and shall report to Seller and Purchaser only the amounts of payments due and payable. Such audits may be exercised during normal business hours upon reasonable prior written notice to Purchaser. Seller shall bear the full cost of such audit unless such audit discloses Purchaser's failure to make a Milestone Payment or an underpayment of greater than [***] of royalty payments otherwise due under this Agreement, in which case, Purchaser shall bear the cost of such audit and shall remit to Seller, in accordance with this Agreement, the outstanding payment within [***] of the date the auditors' written report is received. Any underpayment by Purchaser revealed by an audit shall be paid to Seller, within [***] of the date the auditors' written report is delivered.

(j) **Tax Treatment.** For U.S. federal and applicable state and local income tax purposes, the Parties intend that the Milestone Payments and Royalty Payments shall be treated as deferred contingent purchase price eligible for installment sale treatment under Section 453 of the Code (subject to imputation of interest under Section 483 or Section 1274 of the Code, as applicable).

ARTICLE IV

CLOSING

Section 4.1 **Closing.** The Closing shall take place remotely [***] on the date of this Agreement. The date on which the Closing occurs is called the "Closing Date." Unless the Parties hereto agree otherwise, the Closing shall be deemed to occur and be effective as of the time first set forth in this section. With respect to the wire transfer specified in Section 4.1(c), Seller shall provide Purchaser wire transfer instructions in writing for such account(s) at least [***] prior to the Closing Date. All matters at the Closing will be considered to take place simultaneously, and no delivery of any documents required to be completed at or in connection with the Closing will be deemed completed until all transactions and deliveries of documents required by this Agreement to be completed at or in connection with the Closing are completed. All documents delivered electronically in connection with the closing shall be deemed to be originals.

(a) Upon the occurrence of the Closing, Seller hereby assigns to Purchaser all of Seller's right title and interest in, to and under the Purchased Assets.

(b) At or prior to the Closing, Seller shall deliver or cause to be delivered to Purchaser the following instruments and documents, in each case in a form reasonably acceptable to Purchaser:

(i) counterpart of the bill of sale and assumption agreement substantially in the form attached hereto as Exhibit A executed by Seller (the "Bill of Sale and Assignment and Assumption Agreement"), executed by Seller;

(ii) evidence, reasonably satisfactory to Purchaser, that the Seller has obtained all consents, approvals, filings, and releases required under the Seller Financing Agreement in order

22

to (A) sell, transfer, assign, convey, and deliver to Purchaser the Purchased Assets free and clear of all Liens, (B) execute, delivery and perform the obligations under the Ancillary Agreements, and (C) consummate the transactions contemplated by this agreement;

(iii) a duly executed IRS Form W-9 from Seller;

(iv) counterpart of the intellectual property assignment agreement, substantially in the form attached hereto as Exhibit B (the "Intellectual Property Assignment Agreement"), executed by Seller;

(v) counterpart of the transition agreement in the form attached hereto as Exhibit D (the "Transition Agreement") executed by Seller;

(vi) counterpart of the material transfer agreement in the form attached hereto as Exhibit E (the "Material Transfer Agreement") executed by Seller; and

(vii) a certificate, executed by an executive officer on behalf of Seller, that the Roche Collaboration Agreement has been terminated [***].

(c) At or prior to the Closing, Purchaser shall deliver to Seller, the following:

- (i) certificates of the Secretary or an Assistant Secretary of Purchaser and each Purchaser entity as to the resolutions adopted by the boards of directors of Purchaser or each Purchaser entity relating to the transactions contemplated hereby;
- (ii) any federal, state, local or foreign Tax forms, certificates, instruments or other documents requested by Seller or otherwise required to be provided by Purchaser in connection with the consummation of the transactions contemplated by this Agreement;
- (iii) counterpart of the Bill of Sale and Assignment and Assumption Agreement, executed by Purchaser;
- (iv) counterpart of the Material Transfer Agreement, executed by Purchaser;
- (v) counterpart of the Intellectual Property Assignment Agreement executed by Purchaser; and
- (vi) counterpart of the Transition Agreement executed by Purchaser.

Section 4.2 Additional Transfer Documents.

- (a) In the event that, in addition to this Agreement or the Ancillary Agreements, other agreements, transfers, conveyances and other documents are required by Law in any applicable jurisdiction to effect the transfer of the Purchased Assets and the Assumed Liabilities (each, an "Additional Transfer Document"), Seller shall (and shall cause its Affiliates to) execute, and Purchaser shall (and shall cause its Affiliates to) execute such Additional Transfer Documents at Closing or as soon as practicable thereafter.
- (b) To the extent that the provisions of an Additional Transfer Document are inconsistent with or additional to the provisions of this Agreement, the provisions of this Agreement shall prevail; and so far as permissible by Law, Seller and Purchaser shall procure that the provisions of the relevant Additional Transfer Document are adjusted, to the extent necessary to give effect to the provisions of this Agreement.
- (c) Seller and Purchaser shall not (and shall procure that none of their Affiliates shall), bring any claim against the other Party or any of its Affiliates in respect of or based upon the Additional Transfer Documents. All such claims shall be brought and be subject to the provisions, rights and limitations as set out in this Agreement

23

and no Person shall be entitled to recover damages or obtain payment, reimbursement, restitution or indemnity under or pursuant to the terms of any of the Additional Transfer Documents.

ARTICLE V

REPRESENTATIONS AND WARRANTIES OF SELLER

Except as set forth in the disclosure schedule attached hereto as Exhibit C (the "Disclosure Schedule"), Seller hereby represents and warrants to Purchaser the following, in each case as of the date hereof:

Section 5.1 Organization; Qualification. Seller is a corporation duly organized, validly existing and in good standing under the Laws of Delaware. Seller is duly licensed or qualified to do business and is in good standing in each jurisdiction in which the ownership of the Purchased Assets or the operation of its business as currently conducted makes such licensing or qualification necessary, except as would not, individually or in the aggregate, reasonably be expected to have a Material Adverse Effect.

Section 5.2 Title to Assets, Sufficiency. Seller owns, and has good, valid and marketable title to, the Purchased Assets, free and clear of any Liens (other than Permitted Encumbrances). Upon the sale, conveyance, transfer, assignment and delivery of the Purchased Assets in accordance with this Agreement, Purchaser will own, and acquire good, valid and marketable title to, the Purchased Assets, free and clear of any Liens (other than Permitted Encumbrances). Except as expressly set forth on Section 5.2 of the Disclosure Schedule, the Purchased Assets, when taken together with the rights and services conveyed and provided under the Ancillary Agreements, are sufficient for the development, commercialization, Manufacturing, packaging, distributing, marketing and selling of the

Product in the Territory [**], Seller and their respective Affiliates and their respective licensees immediately prior to the date of this Agreement.

Section 5.3 Authority; Binding Effect.

(a) Seller has all requisite corporate power and authority to execute and deliver this Agreement and each Ancillary Agreement and to perform its obligations hereunder and under each of the Ancillary Agreements to which it will be a party, and to consummate the transactions contemplated by this Agreement. The execution and delivery by Seller of this Agreement and each of the Ancillary Agreements to which it will be a party, and the performance by it of its obligations hereunder and thereunder, have been duly and validly authorized by all requisite corporate action.

(b) This Agreement and each Ancillary Agreement has been duly executed and delivered by Seller and, assuming the due authorization, execution and delivery by Purchaser, constitutes a legal, valid and binding obligation of Seller, in each case enforceable against Seller in accordance with its terms, except as enforcement may be limited by bankruptcy, insolvency, reorganization, fraudulent conveyance, moratorium or similar laws affecting creditors' rights generally or by general principles of equity (regardless of whether enforcement is sought in a proceeding in equity or Law).

Section 5.4 No Conflicts. Neither execution, delivery and performance of this Agreement by Seller or of any of the Ancillary Agreements, nor the consummation by Seller of the transactions contemplated hereby or thereby (a) conflict with or violate any provision of the organizational documents of Seller; (b) create any Lien (other than a Permitted Encumbrance) upon any of the Purchased Assets; (c) conflict with, or result in the breach of, constitute a default under, result in the termination, cancellation or acceleration (whether after the giving of notice or the lapse of time or both) of any right or obligation of Seller under, or to a loss of any benefit to which Seller is entitled under, any Contract to which Seller is a party or to which its assets are subject; and (d) assuming compliance with the matters set forth in Sections 5.5 and 6.5, violate or result in a breach of or constitute a default under any Law or other restriction of any Governmental Authority to which Seller is subject; except, with respect to clauses (b) through (d), for any violations, breaches, conflicts, defaults, terminations, cancellations or accelerations as would not, individually or in the aggregate, have a Material Adverse Effect or prevent or materially delay or impair the ability of the Seller to consummate the transactions contemplated by this Agreement.

Section 5.5 Governmental Authorizations. The execution and delivery of this Agreement by Seller does not require any consent or approval of any Governmental Authority, except for (a) the consents or approvals set forth on Section 5.5 of the Disclosure Schedule, and (b) such consents or approvals for which a failure to obtain would not have a Material Adverse Effect or prevent or materially delay or impair the ability of the Seller to consummate the transactions contemplated by this Agreement.

Section 5.6 Real Property. The Purchased Assets do not include any owned or leased real property.

Section 5.7 No Litigation. [**] there is not, and has never been, a Legal Proceeding initiated or, to the Knowledge of Seller, threatened, before any Governmental Authority or arbitral body relating to the Purchased Assets, the Assumed Liabilities or the Product. There are no outstanding orders of any Governmental Authority or arbitral body affecting the Purchased Assets, the Assumed Liabilities or the Product, including any Legal Proceedings involving Healthcare Regulatory Laws. [***].

Section 5.8 Compliance with Laws.

(a) Except with respect to Product Registrations (which are the subject of Section 5.9) and Taxes (which are the subject of Section 5.11):

(i) Seller is in material compliance with all Laws applicable to the Purchased Assets, including all Healthcare Regulatory Laws; and

(ii) Seller possesses all Governmental Authorizations necessary for the development and commercialization of the Product as it is currently conducted.

(b) Seller has not received any written notice from any Governmental Authority alleging any failure of the development, commercialization, Manufacturing, packaging, distributing, marketing and selling of the Product to comply with any

applicable Law. Seller is in compliance in all material respects with all applicable Privacy Laws with respect to the Business and the Product. For the [***], there has been no unauthorized access to any Personal Information maintained by Seller related to the Business or the Product. Seller has not received written notice of any claim of any alleged violation of any Privacy Law with respect to the Business or the Product.

Section 5.9 Product Registrations; Regulatory Compliance.

(a) Section 5.9(a) of the Disclosure Schedule sets forth, as of the date hereof, a list of all registrations, marketing authorizations and licenses held by Seller or any of its Affiliates that are necessary for the development, Manufacture or commercialization of the Product (the "Product Registrations") in the Territory.

(b) Neither Seller nor any of its officers, directors, or employees has made a fraudulent statement or knowingly made an untrue statement of material fact to the FDA or any other Governmental Authority with respect to any Product or Product Registrations in the Territory, or failed to disclose a material fact required to be disclosed to any Governmental Authority, or committed an act, made a statement or failed to make a statement that, at the time such disclosure was made, would reasonably be expected to provide a basis for any investigation by, and no such investigation has been instituted or threatened by the FDA or any other Governmental Authority with respect to the Product or any Product Registrations in the Territory.

(c) Neither Seller nor its officers, directors, employees, agents, or independent contractors that have performed or will perform activities in connection with the development, manufacturing or commercialization of the Product in the Territory has ever been, or is currently: (i) debarred under 21 U.S.C. § 335a or its equivalents; (ii) listed in the FDA's Clinical Investigators – Disqualification Proceedings Database, including for restrictions; or (iii) convicted of a criminal offense that falls within the scope of 21 U.S.C. § 335a or its equivalents.

(d) Since [***], neither Seller nor its officers, directors, and employees have been convicted of or charged or threatened in writing with prosecution or, to the Knowledge of Seller, have been under investigation,

25

by a Governmental Authority for any violation of a Healthcare Regulatory Law, including any Law applicable to a Federal Healthcare Program.

(e) Seller and its officers, directors, and employees have not been excluded, suspended, or debarred from participation, or are otherwise ineligible to participate in any Federal Health Care Program, any federal, state, or local government procurement or non-procurement program, or any other federal or state government program or activity under applicable Healthcare Regulatory Laws. Seller and its officers, directors, and employees have not been found to have committed any violation of the Law that is reasonably expected to serve as the basis for any such exclusion, suspension, debarment, or other ineligibility.

(f) Since [***], Seller has conducted, [***], the development, commercialization, Manufacturing, packaging, distributing, marketing and selling of the Product in the Territory in compliance in all material respects with the Federal Food, Drug, and Cosmetic Act, 21 U.S.C. § 301 et seq., and applicable regulations promulgated thereunder by the FDA (collectively, "FFDCA"), and such applicable Laws governing the development, Manufacture and commercialization of the Product. All studies, tests and preclinical and clinical trials of the Product conducted by Seller [***] are being (or if completed, have been) conducted in material compliance with applicable Laws and experimental protocols, procedures and controls pursuant to accepted professional scientific standards. Seller has not received any written notices from the FDA or other Governmental Authority alleging material noncompliance with applicable Laws or Product Registrations for the Product in the Territory.

(g) Notwithstanding any other provision of this Agreement, this Section 5.9 sets forth the sole and exclusive representations and warranties of Seller with respect to Product Registrations in the Territory and the regulatory matters described in this Section 5.9.

Section 5.10 Intellectual Property; Performance of Transition.

(a) Section 5.10(a) of the Disclosure Schedule sets forth a true, complete and correct list, as of the date of this Agreement, of the Transferred Patents, Transferred Marks and Transferred Domain Names Controlled by Seller and its Affiliates and that

are used in, held for use in or specifically related to the Business, including all patents made, conceived of or reduced to practice under the Roche Collaboration Agreement required to be assigned by Roche to Seller in the Territory under its terms.

(b) To the Knowledge of Seller, each item of Registered Intellectual Property is valid (or in the case of applications, applied for) and subsisting, all registration, maintenance and renewal fees due as of the Closing Date in connection with such Registered Intellectual Property have been paid, and all documents, recordations, and certificates in connection with such Registered Intellectual Property currently required to be filed have been filed with the relevant patent, copyright, trademark, or other governmental entities in the Territory for the purposes of prosecuting and maintaining such Registered Intellectual Property and recording the Seller's ownership interests therein.

(c) Except as set forth in Section 5.10(c) of the Disclosure Schedule, the Seller has (free and clear of any and all Liens) full title and ownership of all Transferred Intellectual Property.

(d) Section 5.10(d) of the Disclosure Schedule sets forth a true, complete and correct list, as of the date of this Agreement, of Assumed Contracts pursuant to which the Seller has (i) licensed or otherwise received rights under or in respect to any Intellectual Property rights owned by a Third Party that are necessary for the Exploitation of any Product in the Territory, or (ii) licensed or otherwise granted any rights to a Third Party under or with respect to any Transferred Intellectual Property.

(e) [***] (i) Seller has not received written notice alleging that the Exploitation of the Product infringes or misappropriates the Intellectual Property of any Person; (ii) to the Knowledge of Seller, there is no action or proceeding pending, nor any notice of any objection or claim asserted in writing by any Person, with respect to ownership of any Transferred Intellectual Property; (iii) all items of Transferred Intellectual Property set forth on Section 5.10(a) of the Disclosure Schedule are free and clear of any Liens; (iv) to the Knowledge of Seller, the Exploitation of the Product in the Territory in the manner conducted by the Seller, [***] Affiliates as of the date hereof

does not infringe any issued patent of any Third Party; (v) to the Knowledge of Seller, no Person has infringed any patent within the Transferred Intellectual Property.

(f) Notwithstanding any other provision of this Agreement, this Section 5.10 sets forth the sole and exclusive representations and warranties of Seller with respect to Intellectual Property.

(g) As of the date hereof, the Roche Collaboration Agreement has been terminated and is no longer in force and effect other than the provisions that survive pursuant to Section 13.7 thereof [***].

(h) [***]

Section 5.11 Taxes. Seller and its Affiliates (i) have filed (or caused to be filed) with the appropriate Taxing Authorities all material Tax Returns relating to the Purchased Assets required to be filed, (ii) have paid (or caused to be paid) all material Taxes relating to the Purchased Assets whether or not shown as due on such Tax Returns and (iii) all such Tax Returns are true and complete in all material respects.

Section 5.12 Assumed Contracts. Each of the Assumed Contracts is in full force and effect and constitutes a legal, valid and binding agreement of Seller, and to the Knowledge of Seller, each other party thereto, enforceable in accordance with its terms, subject to bankruptcy, insolvency, fraudulent transfer, reorganization, moratorium or similar Laws of general application affecting or relating to the enforcement of creditors' rights generally, and subject to general principles of equity. Neither Seller, nor, to the Knowledge of Seller, any other party thereto is (with or without notice or lapse of time, or both) in material breach or default in the performance, observance or fulfillment of any Assumed Contract, nor does there exist any condition which upon the passage of time or the giving of notice or both, would reasonably be expected to cause such material violation of or material default under or permit the termination or modification of, or acceleration of any obligation under, any Assumed Contract. Seller has not given or received written or, to the Knowledge of Seller, oral notice to or from any Person relating to any such actual or alleged, breach or default. Seller has not received any written or, to the Knowledge of Seller, oral notice from a Third Party stating that such Third Party intends to terminate or materially reduce its relationship with Seller under any Assumed Contract and Seller has not waived any right under the Assumed Contracts.

Section 5.13 Anti-Corruption. To the Knowledge of Seller, within the [**], neither Seller nor any of its Affiliates, nor any of its or their directors, officers, employees, distributors, agents, representatives, sales intermediaries, or other Third Parties acting on behalf of Seller or any of its Affiliates (in each case, in their capacity as such), with respect to the Purchased Assets:

- (a) has taken any action in violation of any applicable anti-corruption laws including the provisions of the United States Foreign Corrupt Practices Act of 1977, as amended (collectively, the "Anti-Corruption Laws"); or
- (b) has, in violation of Anti-Corruption Laws, corruptly offered, paid, given, promised to pay or give, or authorized the payment or gift of anything of value, directly or indirectly, to any Public Official, for the purposes of:
 - (i) influencing any act or decision of any Public Official in his or her official capacity;
 - (ii) inducing such Public Official to do or omit to do any act in violation of his or her lawful duty;
 - (iii) securing any improper advantage; or
 - (iv) inducing such Public Official to use his or her influence with a government, governmental entity, or commercial enterprise owned or controlled by any government (including state-owned or controlled veterinary, laboratory or medical facilities) in obtaining or retaining any business whatsoever.

27

Section 5.14 Brokers. Except for [**], the fees and expenses of which shall be paid by Seller, no broker, finder or investment banker is entitled to any brokerage, finder's or other fee or commission in connection with the transactions contemplated by this Agreement based upon arrangements made by or on behalf of Seller.

Section 5.15 No Other Representations or Warranties.

- (a) EXCEPT FOR THE REPRESENTATIONS AND WARRANTIES EXPRESSLY CONTAINED IN THIS ARTICLE V (AS MODIFIED BY THE DISCLOSURE SCHEDULE), NEITHER SELLER NOR ANY OTHER PERSON MAKES ANY OTHER EXPRESS OR IMPLIED REPRESENTATION OR WARRANTY WITH RESPECT TO SELLER, THE PURCHASED ASSETS, OR THE TRANSACTIONS CONTEMPLATED BY THIS AGREEMENT, THE ASSUMED LIABILITIES AND ANY OTHER RIGHTS OR OBLIGATIONS TO BE TRANSFERRED HEREUNDER OR PURSUANT HERETO, AND SELLER DISCLAIMS ANY OTHER REPRESENTATIONS OR WARRANTIES, WHETHER MADE BY SELLER OR ANY OF ITS AFFILIATES, OFFICERS, DIRECTORS, EMPLOYEES, AGENTS OR REPRESENTATIVES. EXCEPT FOR THE REPRESENTATIONS AND WARRANTIES EXPRESSLY CONTAINED IN THIS ARTICLE V (AS MODIFIED BY THE DISCLOSURE SCHEDULE) OR IN THE ANCILLARY AGREEMENTS, SELLER HEREBY DISCLAIMS ALL LIABILITY AND RESPONSIBILITY FOR ANY REPRESENTATION, WARRANTY, PROJECTION, FORECAST, STATEMENT, OR INFORMATION MADE, COMMUNICATED, OR FURNISHED (ORALLY OR IN WRITING) TO PURCHASER OR ITS AFFILIATES OR REPRESENTATIVES (INCLUDING ANY OPINION, INFORMATION, PROJECTION, OR ADVICE THAT MAY HAVE BEEN OR MAY BE PROVIDED TO PURCHASER BY ANY DIRECTOR, OFFICER, EMPLOYEE, AGENT, CONSULTANT, OR REPRESENTATIVE OF SELLER OR ANY OF ITS AFFILIATES). SELLER MAKES NO REPRESENTATIONS OR WARRANTIES TO PURCHASER REGARDING THE PROBABLE SUCCESS OR PROFITABILITY OF THE PURCHASED ASSETS OR THE PRODUCT IN THE TERRITORY.

ARTICLE VI

REPRESENTATIONS AND WARRANTIES OF PURCHASER

Except as set forth on each Disclosure Schedule attached hereto that relates to such Section of this Agreement or in another Disclosure Schedule to the extent that it is reasonably apparent on the face of such disclosure that such disclosure is applicable to such Section of this Agreement, Purchaser hereby represents and warrants to Seller as follows:

Section 6.1 Organization and Qualification. Purchaser is a corporation duly organized, validly existing and in good standing under the Laws of Delaware and has full corporate power and authority to conduct its business as it is presently being conducted and to own and lease its properties and assets.

Section 6.2 Corporate Authorization. Purchaser has all requisite corporate power and authority to execute and deliver this Agreement and each Ancillary Agreement to which it will be a party, and to perform its obligations hereunder and thereunder. The execution, delivery and performance by Purchaser of this Agreement and each such Ancillary Agreement, and the performance by Purchaser of its obligations hereunder and thereunder, have been duly authorized by all requisite corporate action on the part of Purchaser.

Section 6.3 Binding Effect. This Agreement and each Ancillary Agreement has been duly executed and delivered by Purchaser and, assuming the due authorization, execution and delivery by Seller, constitutes a valid and binding obligation of Purchaser, in each case, enforceable against Purchaser in accordance with its terms, except as enforcement may be limited by bankruptcy, insolvency, reorganization, fraudulent conveyance, moratorium or similar laws affecting creditors' rights generally or by general principles of equity (regardless of whether enforcement is sought in a proceeding in equity or law).

Section 6.4 No Conflict. The execution, delivery and performance by Purchaser of this Agreement, and the consummation of the transactions contemplated hereby, do not and will not (a) violate any provision of the certificate of incorporation, bylaws or other organizational documents of Purchaser; (b) result in a breach of, or default under, or right to accelerate with respect to, any term or provision of any contract, commitment or other obligation to

which Purchaser and any of its Affiliates is a party or is subject; or (c) assuming compliance with the matters set forth in Sections 5.5 and 6.5, violate or result in a breach of or constitute a default under any Law or other restriction of any Governmental Authority to which Purchaser is subject except, with respect to clauses (b) through (c), for any violations, breaches, conflicts, defaults, terminations, cancellations or accelerations as would not, individually or in the aggregate, reasonably be expected to prevent or materially delay the consummation of the transactions contemplated hereby.

Section 6.5 Governmental Authorization. The execution and delivery of this Agreement by Purchaser do not and will not require any material consent or approval of any Governmental Authority, except for (a) the consents or approvals set forth on Section 6.5 of the Disclosure Schedule and (b) where the failure to obtain such consent or approval would not, individually or in the aggregate, reasonably be expected to prevent or materially delay the consummation of the transactions contemplated hereby.

Section 6.6 Third Party Approvals. The execution, delivery and performance by Purchaser of this Agreement and the transactions contemplated hereby do not require any consents, waivers, authorizations or approvals of, or filings with, any third Persons which have not been obtained by Purchaser (other than as contemplated by Section 6.5).

Section 6.7 Financial Capability. Purchaser (a) has sufficient cash (without giving effect to any unfunded financing regardless of whether any such financing is committed) available to pay in cash the Purchase Price and any expenses incurred by Purchaser in connection with the transactions contemplated by this Agreement, (b) has the resources and capabilities (financial or otherwise) to perform its obligations hereunder, and (c) has not incurred any obligation, commitment, restriction or Liability of any kind, which would materially impair or adversely affect such resources and capabilities.

Section 6.8 Litigation. There is no material action, order, writ, injunction, judgment or decree outstanding, or suit, litigation, proceeding, labor dispute (other than routine grievance procedures or routine, uncontested claims for benefits under any benefit plans for any officers, employees or agents of Purchaser), arbitration, investigation or reported claim, pending or, threatened, before any court, governmental entity or arbitrator, which seeks to delay or prevent the consummation of the transactions contemplated by this Agreement or would, if successful, materially and adversely affect the ability of Purchaser to consummate the transactions contemplated by this Agreement.

Section 6.9 No Debarment. Neither Purchaser nor any of its or its Affiliates' employees, agents or independent contractors that will perform activities in connection with the development, manufacturing or commercialization of the Product in the Territory has ever been, or is currently: (i) debarred under 21 U.S.C. § 335a or its equivalents; (ii) excluded, debarred, suspended, or otherwise ineligible to participate in federal health care programs or in federal procurement or non-procurement programs; (iii) listed in the FDA's Clinical Investigators – Disqualification Proceedings Database, including for restrictions; or (iv) convicted of a criminal offense that falls within the scope of 42 U.S.C. § 1320a-7(a) or its equivalents, but has not yet been excluded, debarred, suspended, or otherwise declared ineligible. Purchaser further covenants that if it becomes aware that it or any of its or its Affiliates' employees, agents or independent contractors

perform activities in connection with the development, Manufacturing or commercialization of the Product in the Territory is the subject of any investigation or proceeding that could lead to that Purchaser becoming a debarred entity or individual, an excluded entity or individual or a convicted entity or individual, Purchaser shall immediately notify Seller.

Section 6.10 Anti-Corruption. To its knowledge, within the [***], neither Purchaser nor any of its Affiliates, nor any of its or their directors, officers, employees, distributors, agents, representatives, sales intermediaries, or other Third Parties acting on behalf of Purchaser or any of its Affiliates (in each case, in their capacity as such):

- (a) has taken any action in violation of any Anti-Corruption Laws; or
- (b) has, in violation of Anti-Corruption Laws, corruptly offered, paid, given, promised to pay or give, or authorized the payment or gift of anything of value, directly or indirectly, to any Public Official, for the purposes of:

29

(i) influencing any act or decision of any Public Official in his or her official capacity;

(ii) inducing such Public Official to do or omit to do any act in violation of his or her lawful duty;

(iii) securing any improper advantage; or

(iv) inducing such Public Official to use his or her influence with a government, governmental entity, or commercial enterprise owned or controlled by any government (including state-owned or controlled veterinary, laboratory or medical facilities) in obtaining or retaining any business whatsoever.

Section 6.11 Brokers. No broker, finder or investment banker is entitled to any brokerage, finder's or other fee or commission in connection with the transactions contemplated by this Agreement based upon arrangements made by or on behalf of Purchaser.

Section 6.12 Solvency. Immediately after the Closing, and after giving effect to the transactions contemplated by this Agreement, Purchaser will be Solvent.

Section 6.13 No Other Representation and/or Warranty. PURCHASER ACKNOWLEDGES AND AGREES THAT IT (I) HAS MADE ITS OWN INQUIRY AND INVESTIGATION INTO, AND, BASED THEREON, HAS FORMED AN INDEPENDENT JUDGMENT CONCERNING SELLER, THE PURCHASED ASSETS, THE PRODUCT, THE TRANSACTIONS CONTEMPLATED BY THIS AGREEMENT, THE ASSUMED LIABILITIES AND ANY OTHER ASSETS, RIGHTS OR OBLIGATIONS TO BE TRANSFERRED HEREUNDER OR PURSUANT HERETO, AND (II) HAS BEEN FURNISHED WITH, OR GIVEN ADEQUATE ACCESS TO, SUCH INFORMATION ABOUT SELLER, THE PURCHASED ASSETS, THE PRODUCT IN THE TERRITORY, THE ASSUMED LIABILITIES AND ANY OTHER RIGHTS OR OBLIGATIONS TO BE TRANSFERRED HEREUNDER OR PURSUANT HERETO, AS IT HAS REQUESTED. EXCEPT FOR THE SPECIFIC REPRESENTATIONS AND WARRANTIES EXPRESSLY MADE BY SELLER IN ARTICLE V OF THIS AGREEMENT, (I) PURCHASER ACKNOWLEDGES AND AGREES THAT (A) SELLER IS NOT MAKING AND HAS NOT MADE ANY REPRESENTATION OR WARRANTY, EXPRESSED OR IMPLIED, IN RESPECT OF THE PURCHASED ASSETS, THE ASSUMED LIABILITIES OR THE BUSINESS, INCLUDING WITH RESPECT TO THE PROSPECTS OF THE PURCHASED ASSETS OR THE PRODUCT OR THE BUSINESS, OR THE EFFECTIVENESS OR THE SUCCESS OF ANY OPERATIONS OR THE BUSINESS, AND (B) NO OFFICER, AGENT, REPRESENTATIVE OR EMPLOYEE OF SELLER OR ANY OF SELLER'S AFFILIATES HAS ANY AUTHORITY, EXPRESS OR IMPLIED, TO MAKE ANY REPRESENTATIONS, WARRANTIES OR AGREEMENTS NOT SPECIFICALLY SET FORTH IN THIS AGREEMENT AND SUBJECT TO THE REMEDIES HEREIN PROVIDED; AND (II) PURCHASER SPECIFICALLY DISCLAIMS THAT IT IS RELYING UPON OR HAS RELIED UPON ANY SUCH OTHER REPRESENTATIONS OR WARRANTIES THAT MAY HAVE BEEN MADE BY ANY PERSON, AND ACKNOWLEDGES AND AGREES THAT SELLER HAS SPECIFICALLY DISCLAIMED AND DOES HEREBY SPECIFICALLY DISCLAIM ANY SUCH OTHER REPRESENTATION OR WARRANTY MADE BY ANY PERSON.

ARTICLE VII

COVENANTS

Section 7.1 Information and Documents. Seller, its Affiliates and their respective licensees shall have the right to retain copies of all books and records related to the development, commercialization, Manufacturing, packaging, distributing, marketing and selling of the Product during the period ending on the Closing Date. Purchaser agrees that it shall preserve and keep, or cause to be preserved and kept, all original books and records in respect of the development, commercialization, Manufacturing, packaging, distributing, marketing and selling of the Product during the period ending on the Closing Date in the possession of Purchaser or its Affiliates for the longer of (i) any applicable statute of limitations and (ii) a period of [**] from the Closing Date. During such [**] or longer period, Representatives of Seller and its Affiliates shall, upon reasonable notice and for any reasonable business purpose

(including any Tax related purpose), have access during normal business hours to examine, inspect and copy such books and records. During such [**] or longer period, Purchaser shall provide, or cause to be provided to, Seller or its Affiliates access to such original books and records related to the development, commercialization, manufacturing, packaging, distributing, marketing and selling of the Product as Seller or its Affiliates shall reasonably request in connection with any Legal Proceeding to which Seller or any of its Affiliates are parties or in connection with the requirements of any Law applicable to Seller or any of its Affiliates. Seller or its Affiliates, as applicable, shall return such original books and records to Purchaser or such Affiliate as soon as such books and records are no longer needed in connection with the circumstances described in the immediately preceding sentence. After such [**] or longer period, before Purchaser or any Affiliate shall dispose of any of such books and records, Purchaser shall give at least [**] prior written notice of such intention to dispose to Seller, and Seller or any of its Affiliates shall be given an opportunity, at their cost and expense, to remove and retain all or any part of such books and records as it may elect. If so requested by Purchaser, Seller or its Affiliate, as applicable, shall enter into a customary joint defense agreement with Purchaser or such Affiliate with respect to any information to be provided to such Seller or its Affiliate pursuant to this Section 7.1.

Section 7.2 Conduct. For a period of [**] after the date hereof, Purchaser, its Affiliates and its Representatives will not, directly or indirectly, solicit for employment or hire, or cause to be solicited or hired, any employee of Seller or its Affiliates with whom Purchaser, its Affiliates or its Representatives first came in contact in connection with the transactions contemplated hereby; provided that this Agreement shall not prohibit any advertisement or general solicitation (or hiring as a result thereof) that is not specifically targeted at such persons.

Section 7.3 Insurance. As of the Closing Date, the coverage under all insurance policies related to the Purchased Assets shall continue in force only for the benefit of Seller and its Affiliates and not for the benefit of Purchaser or any of its Affiliates. Purchaser agrees to arrange for its own insurance policies with respect to the Purchased Assets covering all periods and agrees not to seek, through any means, to benefit from any of Seller's or its Affiliates' insurance policies that may provide coverage for claims relating in any way to the Purchased Assets prior to the Closing (except to the extent provided for in ARTICLE X). Seller shall be entitled to make arrangements with its insurers to reflect this Section 7.3.

Section 7.4 Trade Notifications. After the Closing Date, Seller and Purchaser shall agree on the method and content of the notifications to customers of the sale of the Purchased Assets to Purchaser. Seller and Purchaser agree that said notifications are to provide sufficient advance notice of the sale and the plans associated therewith.

Section 7.5 Included Accounts Receivable; Excluded Accounts Receivable.

(a) If Seller receives payment of any Included Accounts Receivable on or following the Closing Date, then Seller shall pay to Purchaser, such amounts within [**] of receipt.

(b) If at any time after the Closing Date, Purchaser or any of its Affiliates receives payment of any Excluded Accounts Receivable, then Purchaser shall pay (or shall cause such Affiliate to pay) to Seller (or to such Affiliate of Seller as Seller may have designated in writing to Purchaser), as soon as practicable the amount recovered.

(c) After the Closing Date, Seller shall be entitled to collect the Excluded Accounts Receivable and to initiate any Legal Proceedings or any other action with a view to collecting the Excluded Accounts Receivable. Purchaser shall not waive or release any of the Excluded Accounts Receivable without the prior written consent of Seller or otherwise interfere with the collection of the Excluded Accounts Receivable.

Section 7.6 Payments under Assumed Contracts.

(a) If and to the extent that Seller (or any of its Affiliates) has, prior to the Closing Date, received any deposit or payment in advance in respect of obligations to be satisfied after the Closing under any Assumed Contracts or other agreement assumed by Purchaser or its Affiliates, Seller (or its Affiliate) shall reimburse to Purchaser, within [***] from the Closing Date, an amount corresponding to the amount of such deposit or payment in advance net of any Taxes or other expenses payable with respect thereto.

31

(b) If and to the extent that Seller (or any of its Affiliates) has, prior to the Closing Date, made any deposit or payment in respect of supplies of goods or services not received prior to Closing under any Assumed Contracts, Purchaser shall reimburse to Seller (or its Affiliate), within [***] from the Closing Date, the amount thereof.

(c) Purchaser shall use commercially reasonable efforts to ensure that Seller and each of its Affiliates are released in full on Closing or, if not practicable, as soon as reasonably practicable following Closing but with the effect as of Closing, from the guarantees, indemnities, counter-indemnities and letters of comfort of any nature given by it to a Third Party relating to any Assumed Contract. Pending release of any guarantee, indemnity, counter-indemnity or letter of comfort referred to in this Section 7.6(c), Purchaser shall indemnify Seller and each of its Affiliates against any and all Losses arising from events, developments and circumstances after Closing under that guarantee, indemnity, counter-indemnity or letter of comfort, as the case may be.

(d) If and to the extent that Seller (or any Affiliate thereof) has, prior to the Closing Date, received any good or service under any Assumed Contract, the payment for which becomes due and payable and is paid by Purchaser (or any of its Affiliates) after the Closing Date, upon request and the presentation of reasonable supporting documentation of such payment by Purchaser (or any of its Affiliates), Seller shall reimburse Purchaser or its Affiliates, as applicable, for the amount of such payment within [***] from the date of such request.

(e) In the event of a disagreement between the Parties as to the determination of amounts due pursuant to this Section 7.6, and if the Parties are unable to resolve such disagreement within [***] after notification of such disagreement has been given by either Party, either Party may submit all remaining matters in dispute to an internationally reputed accounting firm (other than the independent auditors of Seller or Purchaser) as Seller and Purchaser may agree. In the event that the Parties are unable to agree upon such accounting firm within [***], either Party may request the designation of such other accounting firm by the American Arbitration Association (the "AAA"), with each Party hereto having the right to be heard (any firm appointed or designated pursuant to this Section 7.6(e), the "Independent Arbiter"). The AAA shall not, however, resolve any dispute between the Parties under this Agreement, which shall be exclusively resolved in accordance with Section 11.9. The request to the AAA shall include a copy of this Agreement.

(f) The Independent Arbiter shall, acting as experts and not as arbitrators, make a final determination as to all remaining matters in dispute with respect to the amounts due pursuant to this Section 7.6, which determination shall be conclusive and binding on Purchaser and Seller in the absence of manifest error. The cost of retaining the Independent Arbiter with respect to such determination shall be borne by [***]. Purchaser and Seller agree to cooperate with each other in order to resolve any and all matters in dispute as soon as possible and all commercially reasonable efforts to cause the Independent Arbiter to determine disputed matters within [***] following its appointment.

(g) 2024 CStone Cooperation Agreement. As promptly as practicable, but not later than [***] following the Closing Date, Seller and Purchaser shall negotiate in good faith with each other and with CStone and enter into an agreement to reflect the Parties' respective rights and obligations in regard to the CStone Territory (the "2024 CStone Cooperation Agreement"). [***]

Section 7.7 Transition Services. Seller shall comply with its material obligations under [***].

Section 7.8 Confidentiality. Purchaser and Seller acknowledge that the information provided to it in connection with this Agreement and the transactions contemplated hereby is subject to the terms of the Confidentiality Agreement, the terms of which are incorporated herein by reference. Effective upon, and only upon, the Closing Date, the Confidentiality Agreement shall terminate with respect to information relating solely to the development and commercialization of the Product or assets otherwise included in the Purchased Assets. Upon and after the Closing, Seller will, and will cause its Affiliates and its and their Representatives to, keep confidential, not disclose to any Person and not use any non-public, confidential or proprietary information in its possession, under its

Control or to which it has access relating to the development, Manufacturing or commercialization of the Product. The obligations of Seller under this Section 7.7 shall not apply to information to the extent such information (i) becomes generally available to the public without breach of Seller's obligations under this Section 7.7 or (ii) is required to be disclosed by Law; provided, however, that in the case of the foregoing clause (ii), to the extent not prohibited by Law, Seller shall notify Purchaser as early in advance of such disclosure as is practicable to allow Purchaser to take appropriate

measures (and Seller shall reasonably cooperate, at the expense of Purchaser, in the taking of such measures) to preserve the confidentiality of such information.

Section 7.9 Wrongfully Transferred or Retained Assets and Liabilities. In the event any of the Parties discovers after the Closing that it, or one or more of its Affiliates, is the owner of, receives or otherwise comes to possess any asset (including the receipt of payments made pursuant to Assumed Contracts and proceeds from accounts receivable) or is liable for any Liability that is allocated to any Person other than in accordance with this Agreement or any Ancillary Agreement (except as the Parties may otherwise agree), such Party shall, or shall cause its Affiliates to, use all commercially reasonable efforts to convey such asset or Liability, at no cost, to the Party so entitled thereto in accordance with this Agreement (and the relevant Party will cause such entitled Party to accept such asset or assume such Liability).

Section 7.10 [***]

Section 7.11 Further Actions.

(a) Each of the Parties shall execute, deliver and file such instruments of transfer or assignment, files, books and records and shall take such other actions as may be required or reasonably requested by the other Party to carry out the intent of this Agreement and to consummate the transactions contemplated hereby, at the requesting Party's cost and expense.

(b) After the Closing, upon reasonable advance written notice, Purchaser and Seller shall furnish or cause to be furnished to each other, as promptly as reasonably practicable, such information and assistance (to the extent within the reasonable control of such Party) relating to the Purchased Assets (including access to books and records) as is reasonably requested for the filing of all Tax Returns or the satisfaction of contractual or legal obligations to Third Parties, in each case at the requesting Party's cost and expense, provided that no such access shall unreasonably interfere with either Party's Affiliates' and their respective licensees' operation of business; and provided further that such Party may restrict the foregoing access to the extent that (A) in the reasonable judgment of such Party, any applicable Law requires such Party to restrict or prohibit access to any information, (B) in the reasonable judgment of such Party, the information is subject to confidentiality obligations owed by such Party to a Third Party, (C) such disclosure would result in disclosure of any proprietary information or trade secrets of such Party or a Third Party that are not included in the Purchased Assets, or (D) disclosure of any such information or document could result in the loss or waiver of the attorney-client or other applicable privilege.

After the Closing, Purchaser and Seller shall negotiate in good faith with each other and with Roche and CStone a safety data exchange agreement related to the Product as soon as practicable, and in any event within [***] after the Closing.

ARTICLE VIII

CONDITIONS TO CLOSING

Section 8.1 Conditions to the Obligations of Purchaser. The obligation of Purchaser to consummate the transactions contemplated by this Agreement shall be subject to the satisfaction of the following conditions precedent (any or all of which may be waived by Purchaser in whole or in part to the extent permitted by Law):

(a) There shall be no Governmental Order in existence that prohibits or materially restrains the consummation of the transactions contemplated by this Agreement or the Ancillary Agreements, there shall be no proceeding pending by any Governmental Authority seeking such a Governmental Order and there shall be no Legal Proceeding commenced against Purchaser or Seller or which would prohibit the consummation of the transactions contemplated by this Agreement; and

(b) Seller or its Affiliates shall have made or caused to be made delivery to Purchaser of the items required by [Section 4.1\(b\)](#).

Section 8.2 Conditions to the Obligations of Seller. The obligation of Seller to consummate the transactions contemplated by this Agreement shall be subject to the satisfaction of the following conditions precedent (any or all of which may be waived by Seller in whole or in part to the extent permitted by Law):

(a) There shall be no Governmental Order in existence that prohibits or materially restrains the consummation of the transactions contemplated by this Agreement or the Ancillary Agreements, there shall be no proceeding pending by any Governmental Authority seeking such a Governmental Order and there shall be no Legal Proceeding commenced against Purchaser or Seller or which would prohibit the consummation of the transactions contemplated by this Agreement; and

(b) Purchaser and its Affiliates shall have made or caused to be made delivery to Seller of the items required by [Section 4.1\(c\)](#).

ARTICLE IX

[RESERVED]

ARTICLE X

SURVIVAL OF REPRESENTATIONS AND WARRANTIES; INDEMNIFICATION

Section 10.1 Survival. Subject to the limitations and other provisions of this Agreement, the representations and warranties of the Parties hereto, and the right of a party hereto to bring an indemnifiable claim under this [Article X](#) in respect of any breach thereof, shall survive the Closing and shall remain in full force and effect until the date that is [**] following the Closing (the "General Indemnity Expiration Date"); provided that (i) the right of Purchaser to bring a claim in respect of the representations of Seller in [Section 5.10](#) shall survive the Closing and shall remain in full force and effect until the date that is [**] following the Closing and (ii) the right of Purchaser and Seller to bring a claim in respect of the Seller Fundamental Representations and the Purchaser Fundamental Representations, respectively, will survive until the Fundamental Claim Expiration Date, taking into account any extensions or waivers thereof. Except to the extent expressly provided herein, no claim for breach of representation or warranty may be brought by any party after such applicable survival period set forth in the preceding sentence. The covenants, agreements and obligations set forth in [Section 3.2 \("Fundamental Obligations"\)](#), and the right of the Seller Indemnified Parties to bring an indemnifiable claim under this [Article X](#) in respect of any breach thereof shall survive the Closing and shall remain in full force and effect the date that such covenants, agreements and obligations are fully performed. The Parties acknowledge that the time periods set forth in this [Article X](#) for the assertion of claims under this Agreement are the result of arm's length negotiation among the parties and that they intend for the time periods to be enforced as agreed by the Parties.

Section 10.2 Indemnification Survival. Notwithstanding anything contained in this Agreement to the contrary, any claim for indemnification pursuant to [Section 10.3\(a\)\(ii\)](#) or [Section 10.3\(b\)\(ii\)](#) will not be subject to any survival limitation and may be made at any time.

Section 10.3 Indemnification.

(a) Subject to the other terms and conditions of this Agreement, from and after the Closing, Purchaser and its Affiliates (each, a "Purchaser Indemnified Party" and collectively the "Purchaser Indemnified Parties") shall be held harmless and indemnified by the Seller for any Losses (subject to the limitations set forth in this [Article X](#)) incurred by Purchaser in respect of:

- (i) any breach of any representation or warranty of the Seller contained in [Article V](#);
- (ii) any Retained Liability;
- (iii) any breach of any covenant or agreement of the Seller contained herein;

or

(iv) [***].

(b) Subject to the other terms and conditions of this Agreement, from and after the Closing, Seller and its Affiliates (each, a "Seller Indemnified Party", and collectively the "Seller Indemnified Parties", and, together with the Purchaser Indemnified Parties, the "Indemnified Parties") shall be held harmless and indemnified by Purchaser for any Losses (subject to the limitations set forth in this Article X) incurred as a result of:

- (i) any breach of any representation or warranty of the Purchaser contained in Article VI;
- (ii) any Assumed Liability; or
- (iii) any breach of any covenant or agreement of the Purchaser contained herein.

(c) The Indemnified Parties' indemnification rights pursuant to Section 10.3(a) and Section 10.3(b) shall be limited as follows:

(i) The Purchaser Indemnified Parties, on the one hand, or the Seller Indemnified Parties, on the other hand, shall not be entitled to any recovery resulting from Section 10.3(a)(i) and Section 10.3(b)(i), respectively, until such time as the total amount of all Losses that have been incurred by one or more Indemnified Parties with respect to such matters [***] (the "Claims Threshold"), and in such event, the Purchaser Indemnified Parties or the Seller Indemnified Parties, as the case may be, shall, subject to the limitations set forth in the remaining subsections of this Article X, be entitled to be indemnified against and compensated and reimbursed to the extent all Losses from the first dollar thereof; provided that the limitations set forth in this Section 10.3(c)(i) shall not apply to any indemnification claims relating to (A) any breach of any representation or warranty that involves Actual Fraud or (B) any breach of a Seller Fundamental Representation or Purchaser Fundamental Representation, as applicable.

(ii) The Purchaser Indemnified Parties shall not be able to seek or entitled to indemnification under Section 10.3(a)(i) for any dollar amount of Losses (individually or in the aggregate) in excess of an amount equal to the General Indemnity Cap; provided that solely in the case of any Losses resulting from a breach of any Seller Fundamental Representation, the maximum amount that the Purchaser Indemnified Parties may recover from the Seller shall be limited to the amount of the Purchase Price previously paid to Seller; [***]. Notwithstanding anything contained herein to the contrary, nothing herein shall limit the recovery amount against the Seller, or remedies available to a Purchaser Indemnified Party, in respect of indemnification claims relating to (A) Actual Fraud by the Seller; (B) any covenant of Seller contained in this Agreement; or (C) the Retained Liabilities.

(iii) The Seller Indemnified Parties shall not be able to seek or entitled to indemnification under Section 10.3(b)(i) for any dollar amount of Losses (individually or in the aggregate) in excess of an amount equal to the General Indemnity Cap; [***]. Notwithstanding anything contained herein to the contrary, nothing herein shall limit the recovery amount against the Purchaser, or remedies available to a Seller Indemnified Party, in respect of indemnification claims relating to (A) Actual Fraud by the Purchaser; (B) any covenant of Purchaser contained in this Agreement; or (C) the Assumed Liabilities.

(d) For the purposes of determining whether a representation, warranty, or covenant is inaccurate or misrepresented or has been breached, failed, or non-fulfilled, as applicable, and for calculating the amount of any Losses related thereto, the representations, warranties, and covenants will be read without regard to any materiality qualifiers contained therein.

(e) The amount of any Losses subject to indemnification under this Section 10.3 shall be calculated net of (i) any Loss Tax Benefit, (ii) any insurance proceeds received or receivable by the Indemnified Party on account of such Losses and/or (iii) any indemnification paid or payable by any third party as follows:

(i) The Purchaser Indemnified Party or Seller Indemnified Party, as the case may be, shall use commercially reasonable efforts to seek recovery under all insurance policies covering any Loss by exhausting any available remedies against insurers to the same extent as they would if such Loss were not subject to indemnification hereunder. In the event that an insurance or other recovery is made by any Purchaser Indemnified Party or Seller Indemnified Party with respect to any Loss for which any such Person has been indemnified hereunder, then a refund equal to the aggregate amount of the recovery shall be promptly delivered by Purchaser (or its agent) to Seller, or Seller (or its agent) to Purchaser, as the case may be.

(ii) The Indemnifying Party shall be subrogated to all rights of the Indemnified Party in respect of any Loss borne by the Indemnifying Party. The Indemnified Party shall, and shall cause its Affiliates to, use commercially reasonable efforts to bring indemnity claims against any Third Party who has an indemnification obligation to either of them with respect to any Loss and to diligently pursue such claims.

(f) Purchaser and each other Purchaser Indemnified Party, and Seller and each other Seller Indemnified Party, shall take all reasonable steps to mitigate Losses for which indemnification may be claimed by them under this Agreement promptly upon and after becoming aware of any event that could reasonably be expected to give rise to any such Losses.

(g) Any Loss for which any Purchaser Indemnified Party or Seller Indemnified Party is entitled to indemnification under this Section 10.3 shall be determined without duplication of recovery by reason of the state of facts giving rise to such Loss constituting a breach of more than one representation, warranty or covenant.

(h) All claims for indemnification by a Purchaser Indemnified Party under this Agreement must be made on or before the General Indemnity Expiration Date, or, solely in the case of any claims for indemnification for Losses resulting from a breach of any Seller Fundamental Representation, on or before the Fundamental Claim Expiration Date. No indemnification shall be payable to a Purchaser Indemnified Party with respect to claims asserted by such Purchaser Indemnified Party after the General Indemnity Expiration Date, or, solely in the case of any claims for indemnification for Losses resulting from a breach of any Seller Fundamental Representation, on or after the applicable Fundamental Claim Expiration Date, regardless of when the claim accrued or the circumstances that resulted in the claim being asserted after the General Indemnity Expiration Date or the applicable Fundamental Claim Expiration Date, as the case may be. In the event a claim has been properly made on or prior to the General Indemnity Expiration Date or the applicable Fundamental Claim Expiration Date, as applicable, and such claim is unresolved as of the General Indemnity Expiration Date or the applicable Fundamental Claim Expiration Date, as applicable, then the right to indemnification with respect to such claim shall remain in effect until such matter shall have been finally determined by a Governmental Authority or arbitral body, as applicable.

(i) All claims for indemnification by a Seller Indemnified Party under this Agreement must be made on or before the General Indemnity Expiration Date, or, solely in the case of any claims for indemnification for Losses resulting from a breach of any Purchaser Fundamental Representation or a Fundamental Obligation, on or before the applicable Fundamental Claim Expiration Date. No indemnification shall be payable to a Seller Indemnified Party with respect to claims asserted by such Seller Indemnified Party after the General Indemnity Expiration Date, or, solely in the case of any claims for indemnification for Losses resulting from a breach of any Purchaser Fundamental Representation or a Fundamental Obligation, on or after the applicable Fundamental Claim Expiration Date, regardless of when the claim accrued or the circumstances that resulted in the claim being asserted after the General Indemnity Expiration Date or the applicable Fundamental Claim Expiration Date, as the case may be. In the event a claim has been properly made on or prior to the General Indemnity Expiration Date or the applicable Fundamental Claim Expiration Date, as applicable, and such claim is unresolved as of the General Indemnity Expiration Date or the applicable Fundamental Claim Expiration Date, as applicable, then the right to indemnification with respect to such claim shall remain in effect until such matter shall have been finally determined by a Governmental Authority or arbitral body, as applicable.

(i) The Seller Indemnified Parties shall be third party beneficiaries for purposes of this Section 10.3 and shall have the right to enforce the provisions hereof.

(k) The Purchaser Indemnified Parties shall be third party beneficiaries of this Agreement solely with respect to and for purposes of this Section 10.3 and shall have the right to enforce the provisions hereof.

Section 10.4 Notice; Defense of Claims. Any Indemnified Party may make claims for indemnification hereunder by giving prompt written notice thereof to the Seller, in the case of claims made by a Purchaser Indemnified Party, or to Purchaser, in the case of claims made by a Seller Indemnified Party, prior to the General Indemnity Expiration Date. If indemnification is sought for a claim by or in respect of any third party, the Indemnified Party shall also give the Seller or Purchaser, as the applicable Indemnifying Party, written notice of such claim as to which such Indemnified Party may request indemnification hereunder or as to which the Claims Threshold may be applied as soon as is practicable and in any event within [***] of the time that such Indemnified Party learns of such claim; provided, however, that the failure to do so shall not relieve the party with the indemnification obligation hereunder (each, an "Indemnifying Party" and collectively, the "Indemnifying Parties") from any liability except to the extent that it is materially prejudiced by the failure or delay in giving such notice. Such notice shall state all of the information then available regarding the amount and nature of such claim and shall specify the representation, warranty or covenant in this Agreement under which the liability or obligation is asserted. In the case of any third party claim, the Seller or Purchaser, whichever is the Indemnifying Party, shall have the right to direct, through counsel of its own choosing, the defense or settlement of any such claim at its own expense (subject to the limitations set forth in this Article X, including those in Section 10.3(b)). If the Seller or Purchaser, as applicable, elects to assume the defense of any such claim, the Seller or Purchaser, as applicable, shall consult with the Indemnified Party for the purpose of allowing the Indemnified Party to participate in such defense. If the Seller or Purchaser, as applicable, elects not to defend or if, after commencing or undertaking any such defense, the Seller or Purchaser, as applicable, fails to diligently prosecute or withdraws from such defense, the Indemnified Party shall have the right to undertake the defense. If Seller or Purchaser, as applicable, does not so assume control of such defense, the Indemnified Party shall be the Controlling Party. The party not controlling such defense (the "Non-controlling Party" and the party controlling such defense, the "Controlling Party") may participate therein at its own expense, which expense shall not be recoverable as part of any indemnification claim. The Non-controlling Party shall provide, and shall cause its Affiliates to provide, as applicable, the Controlling Party and its counsel with access to its records and personnel relating to any such claim as reasonably necessary during normal business hours and shall otherwise cooperate with the Controlling Party in the defense or settlement thereof. If the Controlling Party elects to direct the defense of any such claim, the Non-controlling Party shall not pay, or permit to be paid, any part of any claim or demand arising from such asserted liability unless Controlling Party consents in writing to such payment. If the Controlling Party assumes the defense of any such claim and proposes to settle such claim prior to a final judgment thereon or to forego any appeal with respect thereto, then the Controlling Party shall give the Non-controlling Party prompt written notice thereof, and the Non-controlling Party shall have the right to participate in and approve (such approval not to be unreasonably withheld, conditioned or delayed) the settlement or assume or reassume the defense of such claim or proceeding.

Section 10.5 Remedies Exclusive.

(a) Purchaser hereby acknowledges and agrees that, prior to the Closing, Purchaser shall have no right or remedy to take any action in respect of, and Seller shall have not any liability to Purchaser in respect of, any breach by the Seller or any representations or warranties contained herein or any failure to comply with any of the covenants, agreements or conditions contained herein.

(b) From and after the Closing, the rights of Purchaser Indemnified Parties and Seller Indemnified Parties to indemnification relating to this Agreement, the Ancillary Agreements entered into in connection herewith and the transactions contemplated hereby and thereby shall be strictly limited to those contained in this Article X, and such indemnification rights shall be the sole and exclusive remedies of the Purchaser Indemnified Parties and Seller Indemnified Parties subsequent to the Closing Date with respect to any matter in any way relating to this Agreement or its subject matter or arising in connection herewith; provided that this Section 10.5 shall not be deemed a waiver by any Party hereto of its right to seek specific performance or injunctive relief in the case of a failure by a Party hereto to comply with the covenants made by such other Party hereto. To the maximum extent permitted by law, the Purchaser Indemnified Parties and Seller Indemnified Parties hereby waive all other rights and remedies with respect to any matter in any way relating to this Agreement or arising in connection herewith, whether under any

laws at common law, in equity or otherwise. Notwithstanding anything to the contrary herein, the existence of this Article X and of the rights and restrictions set forth herein do not limit any legal remedy against the parties hereto to claims based on Actual Fraud.

ARTICLE XI

MISCELLANEOUS

Section 11.1 Notices. All notices and other communications required or permitted to be given or made pursuant to this Agreement shall be made in a writing signed by the sender and shall be deemed duly given (a) on the date delivered, if personally delivered, or sent by email and receipt is confirmed by email or telephone, or (b) on the Business Day after being sent by Federal Express or another recognized overnight mail service that utilizes a written form of receipt for next day or next Business Day delivery (with a courtesy copy sent by email to the addresses specified below, which will not constitute notice), in each case addressed to the applicable Party at the address set forth below; provided that a Party may change its address for receiving notice by the proper giving of notice hereunder:

To Seller:

Blueprint Medicines Corporation
45 Sidney Street
Cambridge, MA, USA 02139
Attention:Chief Executive Officer
Email:[***]

with a copy (which shall not constitute notice) to:

Blueprint Medicines Corporation
45 Sidney Street
Cambridge, MA, USA 02139
Attention:Chief Legal Officer
Email:[***]

and

Goodwin Procter LLP
100 Northern Avenue
Boston, MA, USA 02210
Attention:Kingsley Taft; Danielle Lauzon
Email:[***]

to Purchaser:

Rigel Pharmaceuticals, Inc.
611 Gateway Blvd.
Suite 900
South San Francisco, CA USA 94080
Attention:General Counsel
Email:[***]

with a copy (which shall not constitute notice) to:

Sidley Austin LLP
555 California Street

Suite 2000
San Francisco, CA USA 94014
Attention: Carlton Fleming
Stephen Abreu
Email: [***]
[***]

Section 11.2 Amendment; Waiver. Any provision of this Agreement may be amended or waived if, and only if, such amendment or waiver is in writing and signed, in the case of an amendment, by Purchaser and Seller, or in the case of a waiver, by the Party against whom the waiver is to be effective. No failure or delay by any Party in exercising any right, power or privilege hereunder shall operate as a waiver thereof nor shall any single or partial exercise thereof preclude any other or further exercise thereof or the exercise of any other right, power or privilege.

Section 11.3 Assignment. No Party to this Agreement may assign any of its rights or obligations under this Agreement including by sale of stock, operation of Law in connection with a merger or sale of substantially all the assets of Purchaser or Seller without the prior written consent of the other Party hereto; provided, however, that nothing in the foregoing shall prohibit Seller or Purchaser from making any assignment to any of its Affiliates; and provided further that, notwithstanding anything to the contrary contained in this Agreement, Purchaser may collaterally assign this Agreement and/or any of its rights hereunder for collateral security purposes to any lender providing financing to Purchaser.

Section 11.4 Entire Agreement. This Agreement (including all Schedules and Exhibits hereto), together with the Ancillary Agreements, contains the entire agreement between the Parties hereto with respect to the subject matter hereof and supersedes all prior agreements and understandings, oral or written, with respect to such matters, except for (i) the Confidentiality Agreement which will remain in full force and effect for the term provided for therein and (ii) any written agreement of the Parties that expressly provides that it is not superseded by this Agreement or any Ancillary Agreement.

Section 11.5 Parties in Interest. This Agreement shall inure to the benefit of and be binding upon the Parties hereto and their respective successors and permitted assigns. Nothing in this Agreement, express or implied, is intended to confer upon any Person other than Purchaser, Seller, or their successors or permitted assigns, any rights or remedies under or by reason of this Agreement, provided that the provisions of this Section 11.15 shall inure to the benefit of the Persons referenced therein.

Section 11.6 Public Disclosure. Except (a) for a press release previously approved in form and substance by Seller and Purchaser or any other public announcement using substantially the same text as such press release and (b) any disclosure required by applicable Law, by the rules and regulations of any securities exchange or market on which any security of such party hereto may be listed or traded or by any Governmental Authority of competent jurisdiction, neither Purchaser nor Seller shall, and each party hereto shall cause its Affiliates not to, without the prior written consent of the other party hereto (which consent shall not be unreasonably withheld, delayed or conditioned), issue any press release or make any other public disclosure with respect to this Agreement or any of the other Ancillary Agreements or any of the transactions contemplated hereby or thereby.

Section 11.7 Expenses, Taxes, and Fees.

(a) Except as otherwise expressly provided in this Agreement all costs and expenses incurred in connection with this Agreement and the transactions contemplated hereby shall be borne by the Party incurring such expenses. Notwithstanding the foregoing, [**] of all Transfer Taxes shall be paid by Purchaser with the remaining [**] paid by Seller, and Purchaser shall prepare and timely file all Tax Returns required to be filed with respect thereto; provided that the Parties shall cooperate, including to provide any certificates or forms as may be necessary, to establish any available exemption from (or otherwise reduce) any such Transfer Taxes.

(b) Purchaser shall be responsible for paying, and shall bear the expense of, all fees and/or payments pursuant to section 9008 of the Patient Protection and Affordable Care Act, Public Law 111-148, also known as the Branded Prescription Drug Fee, in connection with all sales of the Product after Closing.

(c) Purchaser and each of its Affiliates and agents will be entitled to deduct and withhold from the payment of any amounts (or any portion thereof) payable under this Agreement such amounts as are required to be deducted and withheld with respect to the making of such payment under the Code or any other Tax law. Purchaser will use commercially reasonable efforts to notify Seller at least [***] prior to Closing of any such withholding it believes is required. To the extent that amounts are so withheld and remitted to the applicable Taxing Authority, such withheld amounts will be treated for all purposes of this Agreement as having been paid to the applicable payee to whom such amounts would otherwise have been paid. The Parties will reasonably cooperate to minimize any withholding.

Section 11.8 Schedules. The disclosure of any matter in any Disclosure Schedule to this Agreement shall be deemed to be a disclosure for all purposes of this Agreement, but shall expressly not be deemed to constitute an admission by Seller or Purchaser, or to otherwise imply, that any such matter is material for the purposes of this Agreement.

Section 11.9 Governing Law; Jurisdiction.

(a) This Agreement and its negotiation, execution, performance or non-performance, interpretation, termination, construction and all Legal Proceedings (whether in contract, in tort, at law, or otherwise) that may be based upon, arise out of, or relate to this Agreement, or the transactions contemplated hereby (including any claim or cause of action based upon, arising out of or related to any representation or warranty made in connection with this Agreement or as an inducement to enter this Agreement), shall be exclusively governed by, and construed in accordance with, the laws of the State of New York regardless of Laws that might otherwise govern under any applicable conflict of laws principles.

(b) Any Legal Proceeding (whether in contract, in tort, at law, or otherwise) based upon, arising out of, or related to this Agreement and its negotiation, execution, performance, nonperformance, interpretation, termination, construction or the transactions contemplated hereby shall be heard and determined in the United States District Court for the Southern District of New York or any New York State court sitting in New York City and the Parties hereto hereby irrevocably submit to the exclusive jurisdiction and venue of such courts in any such Legal Proceeding and irrevocably and unconditionally waive the defense of an inconvenient forum, or lack of jurisdiction to the maintenance of any such Legal Proceeding. The consents to jurisdiction and venue set forth herein shall not constitute general consents to service of process in the State of New York and shall have no effect for any purpose except as provided in this Section 11.9 and shall not be deemed to confer rights on any Person other than the Parties hereto. Each Party hereto agrees that the service of process upon such Party in any Legal Proceeding arising out of or relating to this Agreement shall be effective if notice is given by overnight courier at the address set forth in Section 11.1. Each of the Parties also agrees that any final, non-appealable judgment against a Party in connection with any Legal Proceeding arising out of or relating to this Agreement shall be conclusive and binding on such Party and that such award or judgment may be enforced in any court of competent jurisdiction, either within or outside of the United States. A certified or exemplified copy of such award or judgment shall be conclusive evidence of the fact and amount of such award or judgment.

Section 11.10 WAIVER OF JURY TRIAL. TO THE FULLEST EXTENT PERMITTED BY LAW, THE PARTIES HERETO HEREBY WAIVE THEIR RESPECTIVE RIGHTS TO A JURY TRIAL OF ANY LEGAL PROCEEDING (WHETHER IN CONTRACT, IN TORT, AT LAW, OR OTHERWISE) BASED UPON, ARISING OUT OF, OR RELATED TO THIS AGREEMENT OR ANY OF THE TRANSACTIONS CONTEMPLATED BY THIS AGREEMENT. THE SCOPE OF THIS WAIVER IS INTENDED TO BE ALL-ENCOMPASSING OF ANY AND ALL DISPUTES THAT MAY BE FILED IN ANY COURT AND THAT RELATE TO THE SUBJECT MATTER OF THIS AGREEMENT, INCLUDING, WITHOUT LIMITATION, CONTRACT CLAIMS, TORT CLAIMS, BREACH OF DUTY CLAIMS, AND ALL OTHER COMMON LAW AND STATUTORY CLAIMS. THE PARTIES HERETO ACKNOWLEDGE THAT THIS WAIVER IS A MATERIAL INDUCEMENT TO ENTER INTO A BUSINESS RELATIONSHIP AND THAT EACH HAS ALREADY RELIED ON THE WAIVER IN ENTERING INTO THIS AGREEMENT. THE PARTIES HERETO FURTHER WARRANT AND REPRESENT

THAT EACH HAS REVIEWED THIS WAIVER WITH ITS OR HIS, AS THE CASE MAY BE, LEGAL COUNSEL, AND THAT EACH KNOWINGLY AND VOLUNTARILY WAIVES ITS JURY TRIAL RIGHTS FOLLOWING CONSULTATION WITH LEGAL COUNSEL. THIS WAIVER IS IRREVOCABLE, MEANING THAT IT MAY NOT BE MODIFIED EITHER ORALLY OR IN WRITING, AND THE WAIVER SHALL APPLY TO ANY SUBSEQUENT AMENDMENTS, RENEWALS, SUPPLEMENTS OR MODIFICATIONS TO THIS AGREEMENT OR TO ANY OTHER DOCUMENTS OR AGREEMENTS RELATING TO THE TRANSACTIONS CONTEMPLATED HEREBY. IN THE EVENT OF LITIGATION, THIS AGREEMENT MAY BE FILED AS A WRITTEN CONSENT TO A TRIAL BY THE COURT.

Section 11.11 Counterparts. This Agreement may be signed in any number of counterparts, each of which shall be an original, with the same effect as if the signatures thereto and hereto were upon the same instrument. This Agreement shall become effective when each party hereto shall have received a counterpart hereof signed by the other party hereto. Any counterpart may be executed by facsimile or portable document format (PDF) sent by electronic mail or any electronic signature complying with the U.S. Federal ESIGN Act of 2000 will be deemed to be original signatures, will be valid and binding upon the parties, and, upon delivery, will constitute due execution of this Agreement.

Section 11.12 Headings. The heading references herein and the table of contents hereto are for convenience purposes only, do not constitute a part of this Agreement and shall not be deemed to limit or affect any of the provisions hereof.

Section 11.13 Severability. The provisions of this Agreement shall be deemed severable and the invalidity or unenforceability of any provision shall not affect the validity or enforceability of the other provisions hereof. If any term or other provision of this Agreement, or the application thereof to any person or entity or any circumstance, is invalid, illegal or unenforceable, (a) a suitable and equitable provision shall be substituted therefor in order to carry out, so far as may be valid and enforceable, the intent and purpose of such invalid or unenforceable provision and (b) the remainder of this Agreement and the application of such provision to other Persons, entities or circumstances shall not be affected by such invalidity, illegality or unenforceability, nor shall such invalidity, illegality or unenforceability affect the validity or enforceability of such provision, or the application thereof, in any other jurisdiction.

Section 11.14 Specific Performance. The Parties hereto agree that irreparable damage would occur in the event that any of the provisions of this Agreement were not performed by the Parties hereto in accordance with their specific terms or were otherwise breached. It is accordingly agreed that Purchaser, on the one hand, and Seller, on the other hand, shall be entitled to an injunction or injunctions to prevent breaches or threatened breaches of this Agreement and to enforce specifically the terms and provisions hereof in any court of competent jurisdiction and that this shall include the right of Seller to cause Purchaser, on the one hand, and the right of Purchaser to cause Seller, on the other hand, to fully perform the terms of this Agreement to the fullest extent permissible pursuant to this Agreement and applicable Laws and to thereafter cause this Agreement and the transactions contemplated hereby to be consummated on the terms and subject to the conditions thereto set forth in this Agreement. Such remedies shall, however, be cumulative and not exclusive and shall be in addition to any other remedies which any Party may have under this Agreement or otherwise. Each of the Parties hereto hereby waives (i) any defenses in any action for specific performance, including the defense that a remedy at law would be adequate and (ii) any requirement under any Law to post a bond or other security as a prerequisite to obtaining equitable relief.

Section 11.15 Non-Recourse.

- (a) Except as expressly provided for herein, any claim or cause of action based upon, arising out of, or related to this Agreement may only be brought against the entities that are expressly named as Parties hereto.
- (b) Except as expressly set forth in ARTICLE X, this Agreement is for the sole benefit of the parties and nothing in this Agreement, express or implied, is intended to or will confer upon any other Person any legal or equitable right, benefit, or remedy of any nature whatsoever under or by reason of this Agreement.

IN WITNESS WHEREOF, the Parties have executed or caused this Agreement to be executed by their respective duly authorized representatives as of the date first written above.

BLUEPRINT MEDICINES CORPORATION

By: /s/ Kate Haviland
Name: Kate Haviland
Title: Chief Executive Officer

RIGEL PHARMACEUTICALS, INC.

By: /s/ Raul R. Rodriguez
Name: Raul R. Rodriguez
Title: President and Chief Executive

[Signature Page to Asset Purchase Agreement]

EXHIBIT A

FORM OF BILL OF SALE AND ASSIGNMENT AND ASSUMPTION AGREEMENT

This Bill of Sale and Assignment and Assumption Agreement (this "Bill of Sale") is made as of [•] (the "Effective Date"), by and between Blueprint Medicines Corporation, a Delaware corporation ("Seller"), and Rigel Pharmaceuticals, Inc., a Delaware corporation ("Purchaser").

WHEREAS, Seller and Purchaser entered into that certain Asset Purchase Agreement dated as of the date hereof, as may be amended from time to time (the "Asset Purchase Agreement"), pursuant to which, among other things, Seller has agreed to grant, sell, transfer, assign, convey and deliver to Purchaser, and Purchaser has agreed to purchase, acquire and assume from Seller, all of Seller's right, title and interest to the product known as GAVRETO® (pralsetinib) in the United States and other Purchased Assets and Assumed Liabilities, in each case, on the terms and subject to the conditions set forth in the Asset Purchase Agreement.

NOW, THEREFORE, pursuant and subject to terms of the Asset Purchase Agreement and in consideration of the mutual covenants set forth herein and in the Asset Purchase Agreement, and other good and valuable consideration as set forth in the Asset Purchase Agreement, the receipt and sufficiency of which is hereby acknowledged, Seller and Purchaser agree as follows:

1. Definitions. Capitalized terms used but not otherwise defined herein shall have the meaning set forth in the Asset Purchase Agreement. Whenever used in this Agreement, the terms "include," "includes" and "including" mean "include, without limitation," "includes, without limitation" and "including, without limitation," respectively.

2. Sale and Assignment. Seller hereby grants, sells, transfers, assigns, conveys, and delivers to Purchaser, and Purchaser hereby purchases, acquires and accepts such sale, transfer, assignment, conveyance, and delivery of all of Seller's right, title and interest in and to the Purchased Assets effective as of the date hereof, other than the Transferred Patents and Transferred Marks assigned to Purchaser pursuant to that certain Intellectual Property Assignment Agreement, dated as of the date hereof, in the manner and subject to the terms and conditions set forth in the Asset Purchase Agreement, free and clear of any Liens other than Permitted Encumbrances.

3. Purchase and Assumption. Seller hereby sells, transfers, assigns, conveys, and delivers to Purchaser, and Purchaser hereby purchases, acquires and accepts such sale, transfer, assignment, conveyance, and delivery of all of Seller's right, rights, benefits, titles, interests in, obligations and liabilities to and under the Assumed Liabilities, in the manner and subject to the terms and conditions set forth in the Asset Purchase Agreement. Purchaser hereby assumes and agrees to satisfy and discharge, the Assumed Liabilities.

4. No Assumption of Retained Liabilities. Other than the Assumed Liabilities, Purchaser expressly does not, and shall not, assume or agree to assume, pay, satisfy, discharge, perform or be responsible for in any manner and shall not, by virtue of the execution

and delivery of this Bill of Sale, be deemed to have assumed or to have agreed to pay, satisfy, discharge or perform or be responsible for in any manner, any Retained Liabilities of any nature whatsoever.

5. **Further Assurances.** Seller shall, at any time and from time to time after the Closing Date, upon the request of Purchaser, do, perform, execute, acknowledge, deliver or file, or cause to be done, performed, executed, acknowledged, delivered or filed, all such further acts, deeds, transfers, conveyances, agreements, certificates, instruments, documents, filings, assignments or assurances as may be reasonably required for the better transferring, conveying, assigning and assuring to Purchaser, or for the aiding and assisting in the filing, registering, documenting, memorializing or reducing to possession by Purchaser of, any of the Purchased Assets and Assumed Liabilities.

6. **General.**

(a) **Governing Law.** This Bill of Sale and its negotiation, execution, performance or non-performance, interpretation, termination, construction and all Legal Proceedings (whether in contract, in tort, at law, or otherwise) that may be based upon, arise out of, or relate to this Bill of Sale, or the transactions contemplated

hereby, shall be exclusively governed by, and construed in accordance with, the laws of the State of New York regardless of Laws that might otherwise govern under any applicable conflict of laws principles.

(b) **Jurisdiction.** Any Legal Proceeding (whether in contract, in tort, at law, or otherwise) based upon, arising out of, or related to this Bill of Sale and its negotiation, execution, performance, non-performance, interpretation, termination, construction or the transactions contemplated hereby shall be heard and determined in the United States District Court for the Southern District of New York or any New York State court sitting in New York City and the Parties hereto hereby irrevocably submit to the exclusive jurisdiction and venue of such courts in any such Legal Proceeding and irrevocably and unconditionally waive the defense of an inconvenient forum to the maintenance of any such Legal Proceeding. The consents to jurisdiction and venue set forth herein shall not constitute general consents to service of process in the State of New York and shall have no effect for any purpose except as provided in this Section 6(b) and shall not be deemed to confer rights on any Person other than the Parties hereto. Each Party hereto agrees that the service of process upon such Party in any Legal Proceeding arising out of or relating to this Bill of Sale shall be effective if notice is given in accordance with the terms of Section 11.1 of the Asset Purchase Agreement. Each of the Parties also agrees that any final, non-appealable judgment against a Party in connection with any Legal Proceeding arising out of or relating to this Bill of Sale shall be conclusive and binding on such Party and that such award or judgment may be enforced in any court of competent jurisdiction, either within or outside of the United States. A certified or exemplified copy of such award or judgment shall be conclusive evidence of the fact and amount of such award or judgment.

(c) **WAIVER OF JURY TRIAL.** TO THE FULLEST EXTENT PERMITTED BY LAW, THE PARTIES HERETO HEREBY WAIVE THEIR RESPECTIVE RIGHTS TO A JURY TRIAL OF ANY LEGAL PROCEEDING (WHETHER IN CONTRACT, IN TORT, AT LAW, OR OTHERWISE) BASED UPON, ARISING OUT OF, OR RELATED TO THIS BILL OF SALE OR ANY OF THE TRANSACTIONS CONTEMPLATED BY THIS BILL OF SALE. THE SCOPE OF THIS WAIVER IS INTENDED TO BE ALL-ENCOMPASSING OF ANY AND ALL DISPUTES THAT MAY BE FILED IN ANY COURT AND THAT RELATE TO THE SUBJECT MATTER OF THIS BILL OF SALE, INCLUDING, WITHOUT LIMITATION, CONTRACT CLAIMS, TORT CLAIMS, BREACH OF DUTY CLAIMS, AND ALL OTHER COMMON LAW AND STATUTORY CLAIMS. THE PARTIES HERETO ACKNOWLEDGE THAT THIS WAIVER IS A MATERIAL INDUCEMENT TO ENTER INTO A BUSINESS RELATIONSHIP AND THAT EACH HAS ALREADY RELIED ON THE WAIVER IN ENTERING INTO THIS BILL OF SALE. THE PARTIES HERETO FURTHER WARRANT AND REPRESENT THAT EACH HAS REVIEWED THIS WAIVER WITH ITS OR HIS, AS THE CASE MAY BE, LEGAL COUNSEL, AND THAT EACH KNOWINGLY AND VOLUNTARILY WAIVES ITS JURY TRIAL RIGHTS FOLLOWING CONSULTATION WITH LEGAL COUNSEL. THIS WAIVER IS IRREVOCABLE, MEANING THAT IT MAY NOT BE MODIFIED EITHER ORALLY OR IN WRITING, AND THE WAIVER SHALL APPLY TO ANY SUBSEQUENT AMENDMENTS, RENEWALS, SUPPLEMENTS OR MODIFICATIONS TO THIS BILL OF SALE OR TO ANY OTHER DOCUMENTS OR AGREEMENTS RELATING TO THE TRANSACTIONS CONTEMPLATED HEREBY. IN THE EVENT OF LITIGATION, THIS BILL OF SALE MAY BE FILED AS A WRITTEN CONSENT TO A TRIAL BY THE COURT.

(d) **Counterparts.** This Bill of Sale may be signed in any number of counterparts, each of which shall be an original, with the same effect as if the signatures thereto and hereto were upon the same instrument. This Bill of Sale shall become effective when each party hereto shall have received a counterpart hereof signed by the other party hereto. Any counterpart may be executed by facsimile or portable document format (PDF) sent by electronic mail or any electronic signature complying with the U.S. Federal ESIGN

Act of 2000 will be deemed to be original signatures, will be valid and binding upon the parties, and, upon delivery, will constitute due execution of this Bill of Sale.

(e) **Entire Agreement.** This Bill of Sale, the Asset Purchase Agreement, and the agreements and documents contemplated thereunder, including the Ancillary Agreements, contains the entire agreement between the Parties hereto with respect to the subject matter hereof and thereof and supersedes all prior agreements and understandings, oral or written, with respect to such matters, except for (i) the Confidentiality Agreement which will remain in full force and effect for the term provided for therein and (ii) any written agreement of the Parties that expressly provides that it is not superseded by this Bill of Sale, the Asset Purchase Agreement or any Ancillary Agreement. This Bill of Sale may not be amended, supplemented or otherwise modified except by an instrument in writing signed by authorized representatives of both Seller and Purchaser.

(f) **Binding Agreement.** This Bill of Sale is being delivered pursuant to the Asset Purchase Agreement and shall be construed consistently with the Asset Purchase Agreement. This Bill of Sale shall be binding upon and shall inure to the benefit of the Parties hereto and their respective successors and permitted assigns. Nothing in this Bill of Sale, express or implied, is intended to or shall be construed to supersede, modify, replace, amend, change, rescind, waive, exceed, enlarge, expand or limit in any way the terms of the Asset Purchase Agreement. To the extent that any provision of this Bill of Sale conflicts or is inconsistent with the terms of the Asset Purchase Agreement, the Asset Purchase Agreement shall govern. This Bill of Sale is only intended to effect the transfer of the Purchased Assets and the assignment and assumption of the Assumed Liabilities pursuant to the Asset Purchase Agreement and shall be governed entirely in accordance with the terms and conditions of the Asset Purchase Agreement.

EXHIBIT B

FORM OF INTELLECTUAL PROPERTY ASSIGNMENT AGREEMENT

This Intellectual Property Assignment Agreement ("IP Assignment Agreement") is made as of [•] (the "Effective Date"), by and between Blueprint Medicines Corporation, a Delaware corporation ("Assignor"), and Rigel Pharmaceuticals, Inc., a Delaware corporation ("Assignee").

WHEREAS, Assignor and Assignee entered into that certain Asset Purchase Agreement dated [•], as may be amended from time to time (the "Asset Purchase Agreement"); and

WHEREAS, Assignee is desirous of acquiring, in connection with the transactions contemplated by the Asset Purchase Agreement, the entire right, title and interest in and to the assets set forth in Section 2 of this IP Assignment Agreement.

NOW, THEREFORE, subject to the terms and conditions set forth in the Asset Purchase Agreement and in consideration of the premises and other good and valuable consideration as set forth in the Asset Purchase Agreement, the receipt and sufficiency of which are hereby acknowledged, Assignor and Assignee hereby agree as follows:

1. **Definitions.** Capitalized terms used but not otherwise defined herein shall have the meaning set forth in the Asset Purchase Agreement. Whenever used in this IP Assignment Agreement, the terms "include," "includes" and "including" mean "include, without limitation," "includes, without limitation" and "including, without limitation," respectively.

2. **Assignment.** Assignor hereby sells, conveys, assigns, transfers, delivers and sets over unto Assignee, its successors, legal representatives and assigns, Assignor's entire right, title and interest in and to all Transferred Patents and Transferred Marks, each as listed on Exhibit A attached hereto, and

(a) with respect to the Transferred Patents, (i) patents which may be granted from divisions, reissues, substitutions, continuations, continuations-in-part, reexaminations, and extensions thereof, in each case in the Territory and claiming priority to the underlying said Transferred Patents, (ii) all damages, claims, and payments for infringement of the foregoing in the Territory occurring [***], (iii) all rights to sue for [***] infringement of the foregoing in the Territory (including the right to settle such suits), and (iv) the right to assign the rights conveyed herein, the same to be held and enjoyed by Assignee for its own use and benefit, and for the benefit of its successors, assigns, and legal representatives, and

(b) with respect to the Transferred Marks, (i) the goodwill of the business symbolized thereby in the Territory, (ii) all renewals and extensions of any application, registration and filing that is a Transferred Mark, (iii) damages, claims, and payments for [**] infringements of the foregoing in the Territory occurring [**], (iv) all rights to sue for [**] infringements of the foregoing in the Territory (including the right to settle any such suit), and (v) the right to assign the rights conveyed herein, the same to be held and enjoyed by Assignee for its own use and benefit, and for the benefit of its successors, assigns, and legal representatives, in each case effective as of the Effective Date.

3. Authorization. Assignor hereby authorizes and requests the United States Patent and Trademark Office to respectively issue the same to the Assignee and to respectively record the Assignee as owner of the Transferred Patents and the Transferred Marks, as assignee of the entire right, title and interest in, to and under the same, for the sole use and enjoyment of the Assignee and its successors, legal representatives and assigns.

4. Further Assurances. Assignor shall provide Assignee with all such assistance that Assignee may reasonably request for the full utilization of the rights granted in Section 2 above, including making or executing (or causing Assignor's current or former employees or contractors to make or execute), as applicable, all filings, applications and any further assignments or other documents or instruments, signing all lawful papers, and making all rightful oaths necessary or desirable to carry out the purposes or intent of this IP Assignment Agreement and to aid the Assignee and its successors, legal representatives and assigns to obtain and enforce proper protection for the Transferred Patents and Transferred Marks in the United States and to record the Assignee as owner of the Transferred Patents and Transferred Marks. Assignor's reasonable costs and expenses incurred in connection with such assistance to Assignee shall be borne by Assignee.

5. General.

(a) Governing Law. This IP Assignment Agreement and its negotiation, execution, performance or non-performance, interpretation, termination, construction and all Legal Proceedings (whether in contract, in tort, at law, or otherwise) that may be based upon, arise out of, or relate to this IP Assignment Agreement, or the transactions contemplated hereby, shall be exclusively governed by, and construed in accordance with, the Laws of the State of New York regardless of Laws that might otherwise govern under any applicable conflict of laws principles.

(b) Jurisdiction. Any Legal Proceeding (whether in contract, in tort, at law, or otherwise) based upon, arising out of, or related to this IP Assignment Agreement and its negotiation, execution, performance, non-performance, interpretation, termination, construction or the transactions contemplated hereby shall be heard and determined in the United States District Court for the Southern District of New York or any New York State court sitting in New York City and the Parties hereto hereby irrevocably submit to the exclusive jurisdiction and venue of such courts in any such Legal Proceeding and irrevocably and unconditionally waive the defense of an inconvenient forum to the maintenance of any such Legal Proceeding. The consents to jurisdiction and venue set forth herein shall not constitute general consents to service of process in the State of New York and shall have no effect for any purpose except as provided in this Section 5(b) and shall not be deemed to confer rights on any Person other than the Parties hereto. Each Party hereto agrees that the service of process upon such Party in any Legal Proceeding arising out of or relating to this IP Assignment Agreement shall be effective if notice is given in accordance with the terms of Section 11.1 of the Asset Purchase Agreement. Each of the Parties also agrees that any final, non-appealable judgment against a Party in connection with any Legal Proceeding arising out of or relating to this IP Assignment Agreement shall be conclusive and binding on such Party and that such award or judgment may be enforced in any court of competent jurisdiction, either within or outside of the United States. A certified or exemplified copy of such award or judgment shall be conclusive evidence of the fact and amount of such award or judgment.

(c) WAIVER OF JURY TRIAL. TO THE FULLEST EXTENT PERMITTED BY LAW, THE PARTIES HERETO HEREBY WAIVE THEIR RESPECTIVE RIGHTS TO A JURY TRIAL OF ANY LEGAL PROCEEDING (WHETHER IN CONTRACT, IN TORT, AT LAW, OR OTHERWISE) BASED UPON, ARISING OUT OF, OR RELATED TO THIS IP ASSIGNMENT AGREEMENT OR ANY OF THE TRANSACTIONS CONTEMPLATED BY THIS IP ASSIGNMENT AGREEMENT. THE SCOPE OF THIS WAIVER IS INTENDED TO BE ALL-ENCOMPASSING OF ANY AND ALL DISPUTES THAT MAY BE FILED IN ANY COURT AND THAT RELATE TO THE SUBJECT MATTER OF THIS IP ASSIGNMENT AGREEMENT, INCLUDING, WITHOUT LIMITATION, CONTRACT CLAIMS, TORT CLAIMS, BREACH OF DUTY CLAIMS, AND ALL OTHER COMMON LAW AND STATUTORY CLAIMS. THE PARTIES HERETO ACKNOWLEDGE THAT THIS WAIVER IS A MATERIAL INDUCEMENT TO ENTER INTO A BUSINESS RELATIONSHIP AND THAT

EACH HAS ALREADY RELIED ON THE WAIVER IN ENTERING INTO THIS IP ASSIGNMENT AGREEMENT. THE PARTIES HERETO FURTHER WARRANT AND REPRESENT THAT EACH HAS REVIEWED THIS WAIVER WITH ITS OR HIS, AS THE CASE MAY BE, LEGAL COUNSEL, AND THAT EACH KNOWINGLY AND VOLUNTARILY WAIVES ITS JURY TRIAL RIGHTS FOLLOWING CONSULTATION WITH LEGAL COUNSEL. THIS WAIVER IS IRREVOCABLE, MEANING THAT IT MAY

NOT BE MODIFIED EITHER ORALLY OR IN WRITING, AND THE WAIVER SHALL APPLY TO ANY SUBSEQUENT AMENDMENTS, RENEWALS, SUPPLEMENTS OR MODIFICATIONS TO THIS IP ASSIGNMENT AGREEMENT OR TO ANY OTHER DOCUMENTS OR AGREEMENTS RELATING TO THE TRANSACTIONS CONTEMPLATED HEREBY. IN THE EVENT OF LITIGATION, THIS IP ASSIGNMENT AGREEMENT MAY BE FILED AS A WRITTEN CONSENT TO A TRIAL BY THE COURT.

(d) Counterparts. This IP Assignment Agreement may be signed in any number of counterparts, each of which shall be an original, with the same effect as if the signatures thereto and hereto were upon the same instrument. This IP Assignment Agreement shall become effective when each party hereto shall have received a counterpart hereof signed by the other party hereto. Any counterpart may be executed by facsimile or portable document format (PDF) sent by electronic mail or any electronic signature complying with the U.S. Federal ESIGN Act of 2000 will be deemed to be original signatures, will be valid and binding upon the parties, and, upon delivery, will constitute due execution of this IP Assignment Agreement.

(e) Entire Agreement. This IP Assignment Agreement, the Asset Purchase Agreement, and the agreements and documents contemplated thereunder, including the Ancillary Agreements, contains the entire agreement between the Parties hereto with respect to the subject matter hereof and thereof and supersedes all prior agreements and understandings, oral or written, with respect to such matters, except for (i) the Confidentiality Agreement which will remain in full force and effect for the term provided for therein and (ii) any written agreement of the Parties that expressly provides that it is not superseded by this IP Assignment Agreement, the Asset Purchase Agreement or any Ancillary Agreement. This IP Assignment Agreement may not be amended, supplemented or otherwise modified except by an instrument in writing signed by authorized representatives of both Assignor and Assignee.

(f) Binding Agreement. This IP Assignment Agreement shall be binding upon and shall inure to the benefit of the Parties hereto and their respective successors and permitted assigns. Nothing in this IP Assignment Agreement, express or implied, is intended to or shall be construed to supersede, modify, replace, amend, change, rescind, waive, exceed, enlarge, expand or limit in any way the terms of the Asset Purchase Agreement. To the extent that any provision of this IP Assignment Agreement conflicts or is inconsistent with the terms of the Asset Purchase Agreement, the Asset Purchase Agreement shall govern. This IP Assignment Agreement is only intended to effect the transfer of the Transferred Patents and Transferred Marks pursuant to the Asset Purchase Agreement and shall be governed entirely in accordance with the terms and conditions of the Asset Purchase Agreement.

[Signature page follows.]

Schedule A

Intellectual Property (Transferred Patents, Transferred Marks and Transferred Domain Names)

[***]

EXHIBIT C

DISCLOSURE SCHEDULE

[***]

EXHIBIT D

FORM OF TRANSITION AGREEMENT

TRANSITION AGREEMENT

This Transition Agreement (this "Agreement") is made and entered into as of the [*] day of [*] (the "Effective Date"), between Blueprint Medicines Corporation, a Delaware corporation ("Seller"), and Rigel Pharmaceuticals, Inc., a Delaware corporation ("Purchaser"). Seller and Purchaser are individually referred to as a "Party" and collectively as the "Parties." Capitalized terms used and not otherwise defined in this Agreement shall have the respective meanings set forth in the Asset Purchase Agreement (as defined below).

WHEREAS, Seller previously entered into a Collaboration Agreement dated as of July 13, 2020 (the "Roche Collaboration Agreement") by and among Seller, F. Hoffmann-La Roche Ltd ("Roche Basel"), and Genentech, Inc. ("Genentech" and together with Roche Basel, "Roche") for the development and commercialization of GAVRETO® (pralsetinib) globally (excluding the People's Republic of China, Hong Kong Special Administrative Region, Macau Special Administrative Region and Taiwan); and

WHEREAS, pursuant to the Roche Collaboration Agreement, as of the Effective Date (i) Genentech holds the NDA for the Product and is booking sales of the Product in the United States, and (ii) Roche holds the global safety database for the Product and manufactures the Product globally; and

WHEREAS, the Roche Collaboration Agreement was terminated effective as of [*]; and

WHEREAS, in connection with the termination of the Roche Collaboration Agreement, Seller and Roche have [***]; and

WHEREAS, Seller and Purchaser entered into an Asset Purchase Agreement dated as of the date hereof (the "Asset Purchase Agreement") pursuant to which Purchaser has purchased and assumed all of Seller's right, title and interest to the Product in the United States on the terms and conditions set forth therein; and

WHEREAS, pursuant to Section 4.1(b)(v) of the Asset Purchase Agreement, the Parties desire to agree upon certain terms and conditions to enable an orderly transition of certain assets and responsibilities related to the Product, including without limitation an orderly transition of the booking of sales of the Product in the United States from Genentech to Purchaser;

NOW THEREFORE, Seller and Purchaser hereby agree as follows:

1. General.

(a) Transition Plan; Coordination of Activities. In order to ensure an orderly transition of the Product from Roche and Seller to Purchaser in the United States, the Parties have agreed to the plan set forth in Schedule 1 hereto (the "Transition Plan"). To the extent the Transition Plan does not include material tasks that are reasonably necessary to ensure an orderly transition of the Product in the

United States, Purchaser shall have the right within [***] after the Effective Date to notify Seller of such material tasks and the Parties shall work in good faith to add such item(s) to the Transition Plan. Each Party will reasonably cooperate with the other Party to the extent required for effective conduct of the Transition Plan. Each of the Parties have designated a primary contact in the Transition Plan (a "Transition Coordinator") who will (i) review, coordinate and integrate the activities of the Parties under the relevant portions of the Transition Plan, (ii) facilitate communications between the Parties with respect to such deliverables and activities, (iii) coordinate resolution of any issues that may arise during the performance of the Transition Plan and (iv) perform such other functions as the Parties may mutually agree.

(b) **Standard of Performance; Compliance with Applicable Laws.** Each Party shall perform its obligations and responsibilities under the Transition Plan in accordance with (i) the timelines set forth in the Transition Plan or this Agreement (or, if none are specified, then promptly), (ii) the prevailing industry standards for comparable services and (iii) any additional requirements set forth in the Transition Plan. In addition, each Party shall perform such activities in accordance with this Agreement and all applicable Laws.

2. **Regulatory.**

(a) **NDA.** Seller will cause the NDA for the Product to be assigned by Genentech to Seller promptly after the Closing Date (taking into consideration the ongoing discussions with the FDA related to post-marketing commitments for the Product) and will [***] cause such transfer to occur in sufficient time for Seller to transfer the NDA to Purchaser by the date set forth below in this Section 2(a). [***]. Promptly after the NDA for the Product is assigned by Genentech to Seller and subject to this Section 2(a), each of the Parties shall take all steps necessary to cause the NDA to be assigned by Seller to Purchaser by no later than [***], including without limitation submitting to the FDA a letter or other documentation notifying the FDA of the transfer of the NDA from Seller to Purchaser, provided that both Parties acknowledge and agree that should the Transfer of Booking of Sales be delayed beyond [***] as a result of Seller not performing its obligations under this Agreement, [***], then Seller will continue to hold the NDA until the occurrence of Transfer of Booking of Sales. Each Party shall provide the other Party with copies of such letters and documentation submitted to the FDA. Any regulatory decisions related to the NDA following Genentech's assignment of the NDA to Seller will be made at Purchaser's reasonable discretion, including while Seller is temporarily holding the NDA until transfer of the NDA to Purchaser, and Seller shall follow and implement Purchaser's [***] instructions during such intervening time with respect to the maintenance and transfer of the NDA, provided that such instructions (i) comply with applicable Law, (ii) do not require Seller to incur material additional costs or breach any of its then-existing contractual obligations, or (iii) would not reasonably be expected to cause a delay in the assignment of the NDA by Seller to Purchaser by the date set forth above unless otherwise required to comply with applicable Law.

(b) **Right of Reference; Regulatory Support.** Effective immediately after the NDA for the Product is assigned by Genentech to Seller, Seller shall (i) grant Purchaser a right of reference under the NDA until assigned by Seller to Purchaser and (ii) provide Purchaser with reasonable regulatory support as set forth in the Transition Plan until [***] after the NDA is assigned by Seller to Purchaser.

(c) **Copies of other Regulatory Documents.** Seller will transfer Orphan Drug Designations to Purchaser and provide Purchaser with electronic copies of Investigational New Drug applications, FDA correspondence, IBs, PBRERs, and DSURs upon the completion and availability of each document, each as defined in the Transition Plan.

3. **Manufacturing.**

(a) **Purchaser Agreements with CMOs for the Product.** Purchaser will [***] establish its own contractual relationships with the contract manufacturing organizations listed in the Transition Plan by the dates set forth in the Transition Plan in order to promptly establish Purchaser's own supply of the Product for sale in the Territory.

(b) **Inventory Purchased from Seller.** Pursuant to that certain Material Transfer Agreement entered into by the Parties contemporaneously with this Agreement ("Rigel-Blueprint MTA"), Purchaser will acquire certain batches of Product from Seller. Purchaser will [***] cause such drug product to be packaged, labeled and released in a timely manner to enable Purchaser to achieve Transfer of Booking of Sales in accordance with the timing set forth in Section 4(a) below. In addition, at any time within [***] after the Effective Date, if mutually agreed, the Parties shall discuss with Roche the potential sale by Roche to Seller, Purchaser or any Third Party designated by Seller or Purchaser of the remaining inventory of the Product that Roche might have at a given time in such quantities as may be agreed upon at such time, at a price equal to [***].

4. Transition of Booking of Sales in the Territory.

- (a) Timing. The Parties desire to achieve Transfer of Booking of Sales on [***].
- (b) [***].
- (c) [***].
- (d) [***].

2

5. Pharmacovigilance.

- (a) US Safety Data Exchange Agreement. Within [***] after the Closing Date, and in any event no later than the date of transfer of the NDA from Seller to Purchaser, the Parties will negotiate [***] a safety data exchange agreement describing the pharmacovigilance roles and responsibilities of the Parties related to the Product in the Territory (the "US SDEA").
- (b) Global Safety Database; Pharmacovigilance Support. It is anticipated that Roche will transfer the global safety database for the Product to Seller's vendor, [***], by no later than [***] pursuant to [***]. Seller will provide Purchaser with the pharmacovigilance support [***] until the earlier of (a) such date as the Parties determine [***] that the migration of the global safety database for the Product into Purchaser's safety database has been completed or (b) [***]. Until such time, Seller will continue to hold the global safety database and, working in concert with Roche and CStone, will work with CStone and Roche, as appropriate, to fulfill regulatory reporting responsibilities worldwide pursuant to the Global SDEA (as amended) between Roche, CStone, and Seller.
- (c) Future Agreements. Prior to the transfer of the global safety database to Purchaser, following the completion of the transfer of the global safety database from Roche to Seller by [***], (i) Seller, Roche, CStone, and Purchaser shall execute, as required and applicable, a further amendment to the CStone Agreements to include Purchaser as a party to such applicable agreement, (ii) Roche and Purchaser shall enter into any privacy related agreements, including a data processing agreement and other standard contractual clauses between controllers and processors, as may be required under the applicable Laws.
- (d) Continued Global Coverage. After Purchaser assumes responsibility for the global safety database for the Product, it will revise the Global SDEA as appropriate to reflect Purchaser's ownership of the global safety database for the Product and to enable continued coverage thereunder for CStone and Roche to fulfil regulatory reporting obligations worldwide. The Parties acknowledge and agree that Purchaser will have no operational responsibility for (i) submitting safety reports and aggregate reports to Regulatory Authorities outside of the Territory related to the Exploitation of the Product outside of the Territory or (ii) directly interacting with such Regulatory Authorities in connection with such safety reports. As between the Parties, all such operational obligations for safety reporting to Regulatory Authorities outside the Territory will be the responsibility of Seller.

6. . [***]

7. Clinical Studies.

- (a) Purchaser hereby acknowledges that on or prior to the Closing Date, [***].
- (b) Clinical Trial Master File Information if needed for Regulatory Authorities. In the event that, in connection with an inspection or otherwise, a Regulatory Authority requires Purchaser to (i) provide such Regulatory Authority with access to or (ii) deliver information or documents that are included in the clinical study Trial Master Files, in each case, for the Product that have not been delivered by Roche [***] or that are not otherwise already in Purchaser's possession, Purchaser will [***] the requisite information or documents in the manner and within the timeframes required by such Regulatory Authority.

8. Transfer of Materials.

- (a) [***]. In accordance with the Asset Purchase Agreement, [***] the Disclosure Schedule will be delivered by Seller (either itself or via Genentech) to Purchaser by no later than [***] following the Effective Date. Notwithstanding the foregoing, the Parties will work

together [***] to determine a plan for the migration of the U.S. Product website that is currently managed through Genentech by no later than [***].

(b) **Other Materials.** In addition, Seller shall either deliver to Purchaser or its designee the other materials listed in the Transition Plan in accordance with the delivery instructions set forth therein.

9. **Transferred Know-How Support.** Seller will provide reasonable consultation and assistance for [***] following the Effective Date with respect to the disclosure and provision of relevant Transferred Know-How

3

[***]. After [***] period, Purchaser will reimburse Seller for its reasonable support at [***] rate to be agreed to [***] at the time of the request.

10. **Intellectual Property.** Purchaser will assume responsibility for the prosecution and maintenance of the Transferred Intellectual Property as of the Closing Date. Seller will deliver to Purchaser copies of the data underlying patents and patent applications for the Product listed in the Transition Plan by [***]. The Parties shall cooperate with each other in connection with transitioning prosecution and maintenance of the Transferred Intellectual Property from Seller to Purchaser, and any filings with respect to the FDA's Orange Book applicable to Transferred Intellectual Property until the NDA is assigned by Seller to Purchaser, *provided* that Purchaser shall have the final decision making authority with respect to which Transferred Patents are to be listed in such Orange Book. Once the NDA has been assigned from Seller to Purchaser, Purchaser will assume all responsibility for any future filings with the applicable Regulatory Authority.

11. **Publications.** Notwithstanding anything to the contrary in the Asset Purchase Agreement, Seller or Roche may publish or present, the academic, scientific or medical abstracts, articles, papers, presentations or other type of public disclosures listed in the Transition Plan; *provided that* [***].

12. **Term and Termination.**

(a) **Term.** The term of this Agreement shall commence on the date first written above and shall continue, unless earlier terminated as permitted herein, until completion of all activities set forth in the Transition Plan unless the Parties agree to extend the term of this Agreement beyond the date of such completion or as necessary to comply with Applicable Law (the "Term"); *provided*, however, that the Parties shall use commercially reasonable efforts to complete the activities set forth in the Transition Plan by [***].

(b) **Effects of Termination and Expiration.** Termination or expiration of this Agreement for any reason shall be without prejudice to any rights that shall have accrued to the benefit of a Party prior to such termination or expiration. Notwithstanding anything to the contrary, the following provisions shall survive and apply after the expiration or termination of this Agreement: 5(c), 11(b), 12, 13, 14, and 15.

13. **Confidentiality.** Each of the Parties acknowledge that the information provided to it in connection with this Agreement and the transactions contemplated hereby is subject to Section 7.8 of the Asset Purchase Agreement.

14. **Indemnification.**

(a) **Indemnification by Seller.** Seller shall defend, indemnify, and hold Purchaser and its Affiliates, and each of their respective officers, directors, employees and agents (individually and collectively, the "Purchaser Indemnitees"), harmless from and against any and all damages or other amounts payable to a Third Party, as well as any reasonable attorneys' fees and costs of litigation (collectively, "Damages"), incurred by the Purchaser Indemnitees, to the extent resulting from claims, suits, proceedings or causes of action brought by or on behalf of such Third Party ("Third Party Claims") against the Purchaser Indemnitees that arise from or are based on: (i) a breach of any of Seller's representations, warranties, covenants or obligations under this Agreement; (ii) fraud, wilful misconduct, or grossly negligent acts of Seller or its Affiliates in the performance of its obligations under this Agreement; (iii) any violation of applicable Law by Seller or its Affiliates in the performance of its obligations under this Agreement; or (iv) [***], excluding, in each case ((i) through (iv)), any Damages for which Purchaser has an obligation to indemnify any Seller Indemnitee pursuant to Section 14(b).

(b) Indemnification by Purchaser. Purchaser will indemnify and hold harmless Seller and its Affiliates, and each of their respective directors, officers, employees and agents (individually and collectively, the "Seller Indemnitee(s)") from and against any and all Damages incurred by the Seller Indemnitees to the extent resulting from Third Party Claims against the Seller Indemnitees that arise from or are based on: (i) Purchaser's breach of any of its representations, warranties, covenants, or obligations under Agreement; (ii) fraud, willful misconduct, or grossly negligent acts of Purchaser or its Affiliates in the performance of its obligations under this Agreement; or (iii) any violation of applicable Law by Purchaser or its Affiliates in the performance of its obligations under this Agreement,

4

excluding, in each case (i) through (iii)), any Damages for which Seller has an obligation to indemnify any Purchaser Indemnitee pursuant to Section 14(a).

(c) Indemnification Procedures. The Party claiming indemnity under this Section 1 (the "Indemnified Party") shall give written notice to the Party from whom indemnity is being sought (the "Indemnifying Party") promptly after learning of the claim, suit, proceeding or cause of action for which indemnity is being sought ("Action"). The Indemnifying Party's obligation to defend, indemnify, and hold harmless pursuant to Section 14(a) or (b), as applicable, shall be reduced to the extent the Indemnified Party's delay in providing notification pursuant to the previous sentence results in material prejudice to the Indemnifying Party. At its option, the Indemnifying Party may assume the defense of any Action for which indemnity is being sought by giving written notice to the Indemnified Party within [**] after receipt of the notice of the Action. The assumption of defense of the Action shall not be construed as an acknowledgment that the Indemnifying Party is liable to indemnify any Indemnified Party in respect of the Action, nor shall it constitute waiver by the Indemnifying Party of any defenses it may assert against the Indemnified Party's claim for indemnification. The Indemnified Party shall provide the Indemnifying Party with reasonable assistance, at the Indemnifying Party's expense, in connection with the defense. The Indemnified Party may participate in and monitor such defense with counsel of its own choosing at its sole expense; *provided, however,* the Indemnifying Party shall have the right to assume and conduct the defense of the Action with counsel of its choice. The Indemnifying Party shall not admit liability or settle any Action without the prior written consent of the Indemnified Party, not to be unreasonably withheld, conditioned or delayed. The Indemnified Party shall not settle any such Action without the prior written consent of the Indemnifying Party, which consent shall not be unreasonably withheld, conditioned or delayed. If the Indemnifying Party does not assume and conduct the defense of the Action as provided above, (i) the Indemnified Party may defend against, and consent to the entry of any judgment or enter into any settlement with respect to the Action in any manner the Indemnified Party may deem reasonably appropriate (and the Indemnified Party need not consult with, or obtain any consent from, the Indemnifying Party in connection therewith), and (ii) the Indemnified Party reserves any right it may have under this Section 14 to obtain indemnification from the Indemnified Party.

15. Limitation of Liability. NEITHER PARTY SHALL BE LIABLE TO THE OTHER PARTY FOR ANY SPECIAL, CONSEQUENTIAL, INCIDENTAL, PUNITIVE, DIMINUTION IN VALUE, LOST PROFITS [**], EXEMPLARY OR INDIRECT DAMAGES ARISING FROM OR RELATING TO ANY BREACH OF THIS AGREEMENT OR ANY TORT CLAIMS ARISING HEREUNDER, REGARDLESS OF ANY NOTICE OF THE POSSIBILITY OF SUCH DAMAGES. NOTWITHSTANDING THE FOREGOING, NOTHING IN THIS SECTION 15 IS INTENDED TO OR SHALL LIMIT OR RESTRICT (A) THE INDEMNIFICATION RIGHTS OR OBLIGATIONS UNDER SECTION 14, (B) DAMAGES AVAILABLE FOR A PARTY'S BREACH OF ITS CONFIDENTIALITY OBLIGATIONS UNDER SECTION 13, OR (C) DAMAGES AVAILABLE IN THE CASE OF A PARTY'S FRAUD, GROSS NEGLIGENCE OR INTENTIONAL OR WILLFUL MISCONDUCT.

16. Miscellaneous.

(a) Notices. Except as provided in Section 4(b), all notices and other communications required or permitted to be given or made pursuant to this Agreement shall be delivered in accordance with Section 11.1 of the Asset Purchase Agreement.

(b) Amendment; Waiver. Any provision of this Agreement may be amended or waived if, and only if, such amendment or waiver is in writing and signed, in the case of an amendment, by Purchaser and Seller, or in the case of a waiver, by the Party against whom the waiver is to be effective. No failure or delay by any Party in exercising any right, power or privilege hereunder shall operate as a waiver thereof nor shall any single or partial exercise thereof preclude any other or further exercise thereof or the exercise of any other right, power or privilege.

(c) Assignment. No Party to this Agreement may assign any of its rights or obligations under this Agreement without the prior written consent of the other Party, provided however, that either Party may, without consent of the other Party, assign this Agreement

pursuant to a sale of stock, operation of Law in connection with a merger or sale of substantially all the assets of such Party; provided, further, that nothing in the foregoing shall prohibit either Party from making any assignment to any of its Affiliates without Purchaser's consent.

(d) **Force Majeure.** Neither Party shall be held liable to the other Party nor be deemed to have defaulted under or breached this Agreement for failure or delay in performing any obligation under this Agreement to the extent that such failure or delay is caused by or results from causes beyond the reasonable control of the affected Party that are not reasonably foreseeable or avoidable, potentially including embargoes, war, acts of war (whether war be declared or not), insurrections, riots, civil commotions, strikes, lockouts or other labor disturbances, fire, earthquakes, floods, pandemics or other acts of God (provided that such failure or delay could not have been prevented by the exercise of skill, diligence, and prudence that would be reasonably and ordinarily expected from a skilled and experienced person engaged in the same type of undertaking under the same or similar circumstances) (each a "**Force Majeure Event**"). The affected Party shall notify the other Party of such Force Majeure Event as soon as reasonably practical and shall promptly undertake all reasonable efforts necessary to cure such Force Majeure Event and resume performance of its obligations hereunder.

(e) **Schedules.** The Schedules to this Agreement shall form an integral part of this Agreement.

(f) **Headings.** The heading references in the Agreement and in the Schedules hereto are for convenience purposes only, do not constitute a part of this Agreement and shall not be deemed to limit or affect any of the provisions of this Agreement or the Schedules hereto.

(g) **Severability.** The provisions of this Agreement shall be deemed severable and the invalidity or unenforceability of any provision shall not affect the validity or enforceability of the other provisions hereof. If any term or other provision of this Agreement, or the application thereof to any Person or entity or any circumstance, is invalid, illegal or unenforceable, (i) a suitable and equitable provision shall be substituted therefor in order to carry out, so far as may be valid and enforceable, the intent and purpose of such invalid or unenforceable provision and (ii) the remainder of this Agreement and the application of such provision to other Persons, entities or circumstances shall not be affected by such invalidity, illegality or unenforceability, nor shall such invalidity, illegality or unenforceability affect the validity or enforceability of such provision, or the application thereof, in any other jurisdiction.

(h) **No Strict Construction.** This Agreement and the Schedules have been prepared jointly and will not be strictly construed against either Party.

(i) **Counterparts.** This Agreement may be signed in any number of counterparts, each of which shall be an original, with the same effect as if the signatures thereto and hereto were upon the same instrument. This Agreement shall become effective when each Party hereto shall have received a counterpart hereof signed by the other Party hereto. Any counterpart may be executed by facsimile or portable document format (PDF) sent by electronic mail or any electronic signature complying with the U.S. Federal ESIGN Act of 2000 will be deemed to be original signatures, will be valid and binding upon the Parties, and, upon delivery, will constitute due execution of this Agreement.

(j) **Governing Law.** This Agreement shall be exclusively governed by, and construed in accordance with, the laws of the State of New York regardless of Laws that might otherwise govern under any applicable conflict of laws principles.

[Signature page follows]

Schedule 1

[***]

EXHIBIT E

FORM OF MATERIAL TRANSFER AGREEMENT

MATERIAL TRANSFER AGREEMENT

between

Blueprint Medicines Corporation
45 Sidney Street
Cambridge
MA 02139
(hereinafter called "BPM")

and

Rigel Pharmaceuticals, Inc.
611 Gateway Blvd.
Suite 900
South San Francisco, CA USA 94080
(hereinafter called "Rigel")

[***]

Appendix A: Transition price

[***]

Appendix B: Quality Agreement pertaining to Lots in Appendix A only

[***]

Appendix C: Product Specification for US Markets

[***]

Appendix D: List of Contact Persons

Contact Persons (including, but not limited to Notices of Amendment, Assignment, Termination, Deviation Reports, Complaints, Recall, Resolution of Quality Issues)

[***]

Appendix E: Required Documents for Product

Documents required for release of Product

[***]

Exhibit 10.2

CERTAIN CONFIDENTIAL INFORMATION CONTAINED IN THIS DOCUMENT, MARKED BY [**], HAS BEEN OMITTED BECAUSE IT IS BOTH
(I) NOT MATERIAL AND (II) IS THE TYPE THAT THE REGISTRANT TREATS AS PRIVATE OR CONFIDENTIAL.

AMENDMENT NO. 12 TO THE LICENSE AND COLLABORATION AGREEMENT

This Amendment No. 12 to the License and Collaboration Agreement by and between RIGEL PHARMACEUTICALS, INC. ("Rigel"), and ELI LILLY AND COMPANY ("Lilly"), is made and entered into as of the last signature to this Amendment No. 12 (the "Amendment No. 12 Effective Date"). Rigel and Lilly are each sometimes referred to individually as a "Party" and collectively as the "Parties."

WHEREAS, Rigel and Lilly entered into the License and Collaboration Agreement effective February 18, 2021 and as amended on September 28, 2023 (the "Agreement"), and

WHEREAS, Rigel and Lilly desire to amend certain of the Development provisions in Article 3 of the Agreement.

Now THEREFORE, in consideration of the premises and of the covenants contained herein and in the Agreement, the Parties hereto mutually agree to the following:

A) **Subsection 1 (Stage 1) of Section iv (Opt-Outs) of Section a (R552 Development Cost Sharing) of Section 3.8 (Expenses) under Article 3 (Development), Section 3.8(a) 3.4(b)(iv)(1)**, of the Agreement is hereby deleted and replaced in its entirety with the following:

(iv) Opt-Outs.

(1) Stage 1. Upon written notice provided by in the case of Rigel to Rxxx Continuation or Lilly at any time prior to Rxxx Continuation, not later than [***] (such event, "Stage 1", Rigel (in the case of Rigel Rxxx Continuation) or Lilly (in the case of Lilly Rxxx Continuation) shall provide the other Party, through the JSC, with a data package containing the results generated in connection with such period Rigel Rxxx Continuation or Lilly Rxxx Continuation, as the case may be (the "Stage 1 Opt-Out Period Rxxx Continuation Data Package" and such notice, a "Stage 1 Opt-Out Notice"). Rigel may terminate its obligation to share any R552 Development Costs incurred after the earlier of (i) the date when the Parties have collectively incurred Three Hundred and Twenty Five Million Dollars (\$325,000,000) of shared R552 Development Costs and (ii) [*] (such earlier date, the "Stage 1 Transition Date"). If Rigel timely provides a Stage 1 Opt-Out Notice, then Rigel shall continue to share any R552 Development Costs incurred prior to [Within [***], but only until Rigel's cumulative responsibility for R552 Development Costs is Twenty-Two Million Six Hundred Thousand (\$22,600,000), and thereafter, Lilly shall bear one hundred percent (100%) days after receipt of the costs and expenses incurred in the conduct of Development activities under this Agreement with respect to R552 and R552 Product. In addition, if the Parties have collectively incurred Three Hundred and Twenty Five Million Dollars (\$325,000,000) of R552 Development Costs prior to the expiration of the Stage 1 Opt-Out Period, then all R552 Development Costs that exceed such amount ("Stage 1 Excess R552 Development Costs") shall be borne by Lilly until the expiration of the Stage 1 Opt-Out Period and shared in accordance with Section 3.8(a)(i) (Allocation) following such expiration, unless Rigel provides a Stage 1 Opt-Out Notice, in which case Rigel shall have no obligation to share such Stage 1 Excess R552 Development Costs.

Notwithstanding the foregoing, should Rigel issue a Stage 1 Opt-Out Notice, Rigel shall have the right to opt back in to sharing R552 Development Costs by providing notice to Lilly [*] Rxxx Continuation Data Package (such period, the "Stage 1 Opt-In Period" and such notice, a "Stage Secondary 1 Rxxx Opt-In Election Notice Period"). [*] [*], Lilly may elect Rxxx Acceptance by giving Rigel an Rxxx Acceptance Notice. If, as of Rxxx Acceptance, Rxxx has not been designated pursuant to Section 3.4(a) (CNS Penetrant Lead Identification) or the JSC thereafter decides to designate an alternative RIP1 Inhibitor as Rxxx, then the JSC shall provide to Rigel the total R552 Development Costs incurred since the time designate a RIP1 Inhibitor as Rxxx for purposes of the Stage 1 Transition Date. If Rigel timely provides a Stage 1 Opt-In Notice, then all rights and obligations under this Agreement related to sharing of R552 Development Costs (including, but not limited, applicable increased royalty rates) shall apply and Rigel shall pay Rigel's share of any R552 Development Costs incurred after the Stage 1

1

Transition Date until the Stage 2 Opt-Out Period, at which time the provisions of Section 3.8(a)(iv)(2), Stage 2, shall be controlling as it relates to R552 Development Cost sharing.

B) **Under Section c (Conduct of Phase 2 Clinical Trials) of Section 3.3 (Non-CNS Penetrant Development Activities) of Article 3 (Development), Section 3.3(c), 3.4(c)(i)** of the Agreement Rigel hereby waives Lilly's obligations related to initiation of a psoriasis Phase 2 Clinical Trial and such Section 3.3(c) is hereby deleted and replaced in its entirety with the following:

(c) Conduct of Phase 2 Clinical Trials. Neither Party Following a Lilly Rxxx Continuation, during the period beginning on such Lilly Rxxx Continuation until [***], Lilly shall be required use Commercially Reasonable Efforts to Commence or continue a Clinical Trial in conduct the event that: (i) any representative of activities allocated to Lilly under the FDA or another Regulatory Authority in the United States, Japan or the European Union indicates in writing that the IND application or any equivalent non-US filing for the applicable Compound or Product is either terminated, placed on inactive status, or is otherwise no longer in force and valid due to factors other than the action or inaction of the responsible Party, (ii) [*] that there is a material risk that the continuation of such clinical trial would pose adverse risks to the health or safety of the trial subjects, (iii) the FDA or another Regulatory Authority in the United States, Japan or the European Union orders the responsible Party to suspend or delay such Clinical Trial in accordance with 21 CFR § 312.42 or any similar industry standard, Applicable Laws or guidance of any Regulatory Authority in the United States, Japan or the European Union, (iv)

any supply chain disruption or material shortage occurs that affects a raw material or other necessary resource required for the conduct of such Clinical Trial, or (v) CNS Penetrant Development Plan with respect to [*] as the responsible Party, [*] fails to perform any Development of its obligations under CNS Penetrants up through completion of the [*] first GLP toxicology study for Rxxx.

- C) All other terms and conditions of the Agreement shall continue in full force and effect.
- D) All capitalized terms used, but not defined, herein shall have the respective meaning set forth in the Agreement.
- E) This Amendment No. 1 may be executed in counterparts, each of which shall be deemed an original, but all of which will constitute one and the same instrument.

21

IN WITNESS WHEREOF, the Parties have executed this Amendment No. 12 as of the Amendment No. 12 Effective Date.

RIGEL PHARMACEUTICALS, INC.

ELI LILLY AND COMPANY

By: /s/ Raymon J. Furey Raul Rodriguez
Name: Raymond J. Furey Raul Rodriguez
Title: Executive Vice President General Counsel and Corporate
Secretary & CEO

By: /s/ Ajay Nirula
Name: Ajay Nirula
Title: Senior Vice President

Date: September 27, 2023 March 7, 2024

Date: September 28, 2023 March 11, 2024

2

Exhibit 10.3

CERTAIN CONFIDENTIAL INFORMATION CONTAINED IN THIS DOCUMENT, MARKED BY [], HAS BEEN OMITTED BECAUSE IT IS BOTH (I) NOT MATERIAL AND (II) IS THE TYPE THAT THE REGISTRANT TREATS AS PRIVATE OR CONFIDENTIAL.**

AMENDMENT NO. 4 TO CREDIT AND SECURITY AGREEMENT

This AMENDMENT NO. 4 TO CREDIT AND SECURITY AGREEMENT (this "Agreement") is made as of this 11th day of April, 2024, by and among **RIGEL PHARMACEUTICALS, INC.**, a Delaware corporation ("Rigel"), as a Borrower, **MIDCAP FINANCIAL TRUST**, as Agent (in such capacity, together with its successors and assigns, "Agent") and the financial institutions or other entities from time to time parties to the Credit Agreement referenced below, each as a Lender.

RECITALS

A. Agent, Lenders and Borrower have entered into that certain Credit and Security Agreement, dated as of September 27, 2019 (as amended by that certain Amendment No. 1 to Credit and Security, dated as of April 2, 2021, as amended by that certain Amendment No. 2 to Credit and Security, dated as of February 11, 2022, as amended by that certain Amendment No. 3 to Credit and Security Agreement, dated as of July 27, 2022, as supplemented by that certain Limited Consent to Credit and Security Agreement, dated February 21, 2024, and as further amended, supplemented or otherwise modified from time to time prior to the date hereof, the "Existing Credit Agreement" and, as the same is amended hereby and as it may be further amended, modified, supplemented and restated from time to time, the "Credit Agreement"), pursuant to which the Lenders have agreed to make certain advances of money and to extend certain financial accommodations to Borrower in the amounts and manner set forth in the Credit Agreement.

B. In accordance with Section 11 of the Credit Agreement, Borrower has notified Agent and Lenders of a change to its mailing address for the delivery of notices, consents, requests, approvals, demands, or other communication under the Financing Documents.

C. Borrower has requested, and Agent and Lenders have agreed, on and subject to the terms and conditions set forth in this Agreement and the other Financing Documents, to among other things (a) amend the Applicable Floor for each Credit Facility, (b) amend the Applicable Margin for each Credit Facility, (c) amend certain fees, (d) extend the Maturity Date and the Initial Amortization Start Date, and (e) amend certain other provisions of the Existing Credit Agreement related to the foregoing.

AGREEMENT

NOW, THEREFORE, in consideration of the foregoing, the terms and conditions set forth in this Agreement, and other good and valuable consideration, the receipt and sufficiency of which are hereby acknowledged, Agent, Lenders and Borrower hereby agree as follows:

1. **Recitals.** This Agreement shall constitute a Financing Document and the Recitals and each reference to the Credit Agreement, unless otherwise expressly noted, will be deemed to reference the Credit Agreement as amended hereby. Capitalized terms used but not otherwise defined herein shall have the meanings ascribed to them in the Credit Agreement (including those capitalized terms used in the Recitals hereto).

2. **Amendments to Existing Credit Agreement.** Subject to the terms and conditions of this Agreement, including, without limitation, the conditions to effectiveness set forth in Section 4 below, the Existing Credit Agreement is hereby amended as follows:

(a) The definition of "Maturity Date" in Section 15 of the Existing Credit Agreement is hereby amended and restated in its entirety as follows:

"Maturity Date" means September 1, 2027."

(b) Section 11 of the Existing Credit Agreement is hereby amended to replace the mailing and electronic mail addresses for the Borrower in their entirety with the following mailing and electronic mail address:

If to Borrower:

Rigel Pharmaceuticals, Inc.
611 Gateway Blvd., Suite 900
South San Francisco, CA 94080
Attn: General Counsel or Legal Department
Email: contracts@rigel.com

(c) The Credit Facility #1 Schedule attached to the Existing Credit Agreement is hereby deleted and replaced in its entirety with Schedule 1 to this Agreement.

(d) The Credit Facility #2 Schedule attached to the Existing Credit Agreement is hereby deleted and replaced in its entirety with Schedule 2 to this Agreement.

(e) The Credit Facility #3 Schedule attached to the Existing Credit Agreement is hereby deleted and replaced in its entirety with Schedule 3 to this Agreement.

(f) The Credit Facility #4 Schedule attached to the Existing Credit Agreement is hereby deleted and replaced in its entirety with Schedule 4 to this Agreement.

(g) The Credit Facility #5 Schedule attached to the Existing Credit Agreement is hereby deleted and replaced in its entirety with Schedule 5 to this Agreement.

(h) The Amortization Schedule attached to the Existing Credit Agreement is hereby deleted and replaced in its entirety with Schedule 6 to this Agreement.

(i) The Minimum TAVALISSE Net Revenue Schedule attached to the Existing Credit Agreement is hereby deleted and replaced in its entirety with Schedule 7 to this Agreement.

3. **Representations and Warranties; Reaffirmation of Security Interest.** Borrower hereby (a) confirms that all of the representations and warranties set forth in the Credit Agreement are true and correct in all material respects (without duplication of any materiality qualifier in the text of such representation or warranty) with respect to Borrower as of the date hereof except to the extent that any such representation or warranty relates to a specific date in which case such representation or warranty shall be true and correct as of such earlier date, and (b) covenants to perform its respective obligations under the Credit Agreement. Each Borrower confirms and agrees that all security interests and Liens granted to Agent continue in full force and effect, and all Collateral remains free and clear of any Liens, other than Permitted Liens. Nothing herein is intended to impair or limit the validity, priority or extent of Agent's security interests in and Liens on the Collateral. Borrower acknowledges and agrees that the Credit Agreement, the other Financing Documents and this Agreement constitute the legal, valid and binding obligation of Borrower, and are enforceable against Borrower in accordance with its terms, except as the enforceability thereof may be limited by bankruptcy, insolvency or other similar laws relating to the enforcement of creditors' rights generally and by general equitable principles.

4. **Conditions to Effectiveness.** This Agreement shall become effective as of the date on which each of the following conditions has been satisfied, as determined by Agent in its sole discretion:

(a) Agent shall have received (including by way of facsimile or other electronic transmission) a duly authorized, executed and delivered counterpart of the signature page to this Amendment from Borrower, Agent and the Lenders;

2

(b) Agent shall have received (including by way of facsimile or other electronic transmission) a duly authorized, executed and delivered counterpart of the signature page to the Second Amended and Restated Fee Letter from Borrower and Agent;

(c) all representations and warranties of Borrower contained herein shall be true and correct in all material respects (without duplication of any materiality qualifier in the text of such representation or warranty) as of the date hereof except to the extent that any such representation or warranty relates to a specific date in which case such representation or warranty shall be true and correct as of such earlier date (and such parties' delivery of their respective signatures hereto shall be deemed to be its certification thereof);

(d) prior to and after giving effect to the agreements set forth herein, no Default or Event of Default shall exist under any of the Financing Documents; and

(e) Agent shall have received such other documents, certificates, and information as Agent may reasonably request in connection with this Agreement.

5. **Release.** In consideration of the agreements of Agent and Lenders contained herein and for other good and valuable consideration, the receipt and sufficiency of which is hereby acknowledged, Borrower, voluntarily, knowingly, unconditionally and irrevocably, with specific and express intent, for and on behalf of itself and all of its respective parents, subsidiaries, affiliates, members, managers, predecessors, successors, and assigns, and each of its respective current and former directors, officers, shareholders, agents, and employees, and each of its respective predecessors, successors, heirs, and assigns (individually and collectively, the "Releasing Parties") does hereby fully and completely release, acquit and forever discharge each of Agent, Lenders, and each their respective parents, subsidiaries, affiliates, members, managers, shareholders, directors, officers and employees, and each of their respective

predecessors, successors, heirs, and assigns (individually and collectively, the "Released Parties"), of and from any and all actions, causes of action, suits, debts, disputes, damages, claims, obligations, liabilities, costs, expenses and demands of any kind whatsoever, at law or in equity, whether matured or unmatured, liquidated or unliquidated, vested or contingent, choate or inchoate, known or unknown that the Releasing Parties (or any of them) has against the Released Parties or any of them (whether directly or indirectly), based in whole or in part on facts, whether or not now known, existing on or before the date hereof, that relate to, arise out of or otherwise are in connection with: (i) any or all of the Financing Documents or transactions contemplated thereby or any actions or omissions in connection therewith or (ii) any aspect of the dealings or relationships between or among any Borrower, on the one hand, and any or all of the Released Parties, on the other hand, relating to any or all of the documents, transactions, actions or omissions referenced in clause (i) hereof, in each case, based in whole or in part on facts, whether or not now known, existing before the date hereof. Borrower acknowledges that the foregoing release is a material inducement to Agent's and each Lender's decision to enter into this Agreement and agree to the modifications contemplated hereunder, and has been relied upon by Agent and Lenders in connection therewith.

6. **No Waiver or Novation.** The execution, delivery and effectiveness of this Agreement shall not, except as expressly provided in this Agreement, operate as a waiver of any right, power or remedy of Agent, nor constitute a waiver of any provision of the Credit Agreement, the Financing Documents or any other documents, instruments and agreements executed or delivered in connection with any of the foregoing. Nothing herein is intended or shall be construed as a waiver of any existing Defaults or Events of Default under the Credit Agreement or the other Financing Documents or any of Agent's rights and remedies in respect of such Defaults or Events of Default. This Agreement (together with any other document executed in connection herewith) is not intended to be, nor shall it be construed as, a novation of the Credit Agreement.

7. **Affirmation.** Except as specifically amended pursuant to the terms hereof, Borrower hereby acknowledges and agrees that the Credit Agreement and all other Financing Documents (and all covenants, terms, conditions and agreements therein) shall remain in full force and effect, and are hereby ratified and confirmed in all respects by Borrower. Borrower covenants and agrees to comply with all of the terms, covenants and conditions of the Credit Agreement and the Financing Documents, notwithstanding any prior course of conduct, waivers, releases or other actions or inactions on Agent's or any Lender's part which might otherwise constitute or be construed as a waiver of or amendment to such terms, covenants and conditions.

8. **Miscellaneous.**

(a) **Reference to the Effect on the Credit Agreement.** Upon the effectiveness of this Agreement, each reference in the Credit Agreement to "this Agreement," "hereunder," "hereof," "herein," or words of similar import shall mean and be a reference to the Credit Agreement, as amended by this Agreement. Except as specifically amended above, the Credit Agreement, and all other Financing Documents (and all covenants, terms, conditions and agreements therein), shall remain in full force and effect, and are hereby ratified and confirmed in all respects by Borrower.

(b) **GOVERNING LAW.** THIS AGREEMENT AND ALL DISPUTES AND OTHER MATTERS RELATING HERETO OR THERETO OR ARISING THEREFROM (WHETHER SOUNDING IN CONTRACT LAW, TORT LAW OR OTHERWISE), SHALL BE GOVERNED BY, AND SHALL BE CONSTRUED AND ENFORCED IN ACCORDANCE WITH, THE LAWS OF THE STATE OF NEW YORK, WITHOUT REGARD TO CONFLICTS OF LAWS PRINCIPLES (OTHER THAN SECTION 5-1401 OF THE GENERAL OBLIGATIONS LAW).

(c) **WAIVER OF JURY TRIAL.** BORROWER, AGENT AND THE LENDERS PARTY HERETO HEREBY IRREVOCABLY WAIVES ANY AND ALL RIGHT TO TRIAL BY JURY IN ANY LEGAL ACTION OR PROCEEDING ARISING OUT OF OR RELATING TO THIS AGREEMENT OR THE TRANSACTIONS CONTEMPLATED HEREBY AND AGREES THAT ANY SUCH ACTION OR PROCEEDING SHALL BE TRIED BEFORE A COURT AND NOT BEFORE A JURY. BORROWER, AGENT AND EACH LENDER ACKNOWLEDGES THAT THIS WAIVER IS A MATERIAL INDUCEMENT TO ENTER INTO A BUSINESS RELATIONSHIP, THAT EACH HAS RELIED ON THE WAIVER IN ENTERING INTO THIS AGREEMENT, AND THAT EACH WILL CONTINUE TO RELY ON THIS WAIVER IN THEIR RELATED FUTURE DEALINGS. BORROWER, AGENT AND EACH LENDER WARRANTS AND REPRESENTS THAT IT HAS HAD THE OPPORTUNITY OF REVIEWING THIS JURY WAIVER WITH LEGAL COUNSEL, AND THAT IT KNOWINGLY AND VOLUNTARILY WAIVES ITS JURY TRIAL RIGHTS.

(d) Incorporation of Credit Agreement Provisions. The provisions contained in Article 12 (Choice of law; venue and jury trial waiver; California waivers) and Section 13.2 (Indemnification) of the Credit Agreement are incorporated herein by reference to the same extent as if reproduced herein in their entirety.

(e) Headings. Section headings in this Agreement are included for convenience of reference only and shall not constitute a part of this Agreement for any other purpose.

(f) Counterparts. This Agreement may be signed in any number of counterparts, each of which shall be deemed an original and all of which when taken together shall constitute one and the same instrument. The words "execution," "signed," "signature," and words of like import in this Amendment shall be deemed to include electronic signatures or electronic records, each of which shall be of the same legal effect, validity or enforceability as a manually executed signature or the use of a paper-based recordkeeping system, as the case may be, to the extent and as provided for in any applicable law, including the Federal Electronic Signatures in Global and National Commerce Act, the New York State Electronic Signatures and Records Act, or any other similar state laws based on the Uniform Electronic Transactions Act. Delivery of an executed counterpart of this Agreement by facsimile or by electronic mail delivery of an electronic version (e.g., .pdf or .tif file) of an executed signature page shall be effective as delivery of an original executed counterpart hereof and shall bind the parties hereto.

(g) Entire Agreement. This Agreement constitutes the entire agreement and understanding among the parties hereto and supersedes any and all prior agreements and understandings, oral or written, relating to the subject matter hereof.

(h) Severability. In case any provision of or obligation under this Agreement shall be invalid, illegal or unenforceable in any applicable jurisdiction, the validity, legality and enforceability of the remaining provisions or obligations, or of such provision or obligation in any other jurisdiction, shall not in any way be affected or impaired thereby.

4

(i) Successors/Assigns. This Agreement shall bind, and the rights hereunder shall inure to, the respective successors and assigns of the parties hereto, subject to the provisions of the Credit Agreement and the other Financing Documents.

[SIGNATURES APPEAR ON FOLLOWING PAGES]

5

IN WITNESS WHEREOF, intending to be legally bound, the undersigned have executed this Agreement as of the day and year first hereinabove set forth.

AGENT:

MIDCAP FINANCIAL TRUST,

By: Apollo Capital Management, L.P.,
its investment manager

By: Apollo Capital Management GP, LLC,
its general partner

By: /s/ Maurice Amsellem
Name: Maurice Amsellem
Title: Authorized Signatory

LENDERS:MIDCAP FUNDING XIII TRUST

By: Apollo Capital Management, L.P.,
its investment manager

By: Apollo Capital Management GP, LLC,
its general partner

By: /s/ Maurice Amsellem
Name: Maurice Amsellem
Title: Authorized Signatory

LENDERS:ELM 2020-3 TRUST

By: MidCap Financial Services Capital Management, LLC, as Servicer

By: /s/ John O'Dea
Name: John O'Dea
Title: Authorized Signatory

ELM 2020-4 TRUST

By: MidCap Financial Services Capital Management, LLC, as Servicer

By: /s/ John O'Dea
Name: John O'Dea
Title: Authorized Signatory

LENDERS:

MIDCAP FINANCIAL INVESTMENT CORPORATION

(formerly known as Apollo Investment Corporation)

By: /s/ Kristin Hester
Name: Kristin Hester
Title: Chief Legal Officer

BORROWER: **RIGEL PHARMACEUTICALS, INC.**

By: /s/ Dean Schorno
Name: Dean Schorno
Title: Chief Financial Officer

Schedule 1

Credit Facility #1:

Credit Facility and Type: Term, Tranche 1

Lenders for and their respective Applicable Commitments to this Credit Facility:

Lender	Applicable Commitment
Midcap Financial Trust	Seven Million Dollars (\$7,000,000)
Apollo Investment Corporation	Three Million Dollars (\$3,000,000)
Total:	Ten Million Dollars (\$10,000,000)

The following defined terms apply to this Credit Facility:

Applicable Interest Period: means the one-month period starting on the first (1st) day of each month and ending on the last day of such month; *provided, however,* that the first (1st) Applicable Interest Period for each Credit Extension under this Credit Facility shall commence on the date that the applicable Credit Extension is made and end on the last day of such month.

Applicable Floor: means four percent (4.00%) per annum.

Applicable Margin: a rate of interest equal to six and one half percent (6.50%) per annum.

Applicable Prepayment Fee: means the following amount, calculated as of the date (the "Accrual Date") that the Applicable Prepayment Fee becomes payable in the case of prepayments required under the Financing Documents or the date any voluntary prepayment is made: (a) for an Accrual Date on or after the Closing Date through and including September 30, 2025, one and one half percent (1.5%) multiplied by the amount of the outstanding principal of the Credit Extension prepaid or required to be prepaid (whichever is greater) and (b) for an Accrual Date after September 30, 2025 through and including the date immediately preceding the Maturity Date,

one percent (1.0%) multiplied by the amount of the outstanding principal of the Credit Extension prepaid or required to be prepaid (whichever is greater).

Commitment Commencement Date: Closing Date.

Commitment Termination Date: the close of the Business Day following the Closing Date.

Minimum Credit Extension Amount: \$10,000,000.00

Schedule 2

Credit Facility #2:

Credit Facility and Type: Term, Tranche 2

Lenders for and their respective Applicable Commitments to this Credit Facility:

Lender	Applicable Commitment
MidCap Financial Trust	Seven Million Dollars (\$7,000,000)
Apollo Investment Corporation	Three Million Dollars (\$3,000,000)
Total:	Ten Million Dollars (\$10,000,000)

The following defined terms apply to this Credit Facility:

Applicable Funding Conditions: N/A.

Applicable Interest Period: means the one-month period starting on the first (1st) day of each month and ending on the last day of such month; *provided, however,* that the first (1st) Applicable Interest Period for each Credit Extension under this Credit Facility shall commence on the date that the applicable Credit Extension is made and end on the last day of such month.

Applicable Floor: means four percent (4.00%) per annum.

Applicable Margin: a rate of interest equal to six and one half percent (6.50%) per annum.

Applicable Prepayment Fee: means the following amount, calculated as of the date (the "Accrual Date") that the Applicable Prepayment Fee becomes payable in the case of prepayments required under the Financing Documents or the date any voluntary prepayment is made: (a) for an Accrual Date on or after the Closing Date through and including September 30, 2025, one and one half percent (1.5%) multiplied by the amount of the outstanding principal of the Credit Extension prepaid or required to be prepaid (whichever is greater) and (b) for an Accrual Date after September 30, 2025 through and including the date immediately preceding the Maturity Date, one percent (1.0%) multiplied by the amount of the outstanding principal of the Credit Extension prepaid or required to be prepaid (whichever is greater).

Commitment Commencement Date: Closing Date.

Commitment Termination Date: the earliest to occur of (a) December 31, 2020, (b) the date on which any Credit Extensions are made by the Lenders in respect of Credit Facility #3 or Credit Facility #4, and (c) the delivery of a written notice by Agent to Borrower terminating the Applicable Commitments following an Event of Default that has not been waived or cured at the time such notice is delivered.

Minimum Credit Extension Amount: \$10,000,000.00

Schedule 3

Credit Facility #3:

Credit Facility and Type: Term, Tranche 3

Lenders for and their respective Applicable Commitments to this Credit Facility:

Lender	Applicable Commitment
MidCap Financial Trust	Seven Million Dollars (\$7,000,000)
Apollo Investment Corporation	Three Million Dollars (\$3,000,000)
Total:	Ten Million Dollars (\$10,000,000)

The following defined terms apply to this Credit Facility:

Applicable Funding Conditions: N/A.

Applicable Interest Period: means the one-month period starting on the first (1st) day of each month and ending on the last day of such month; *provided, however,* that the first (1st) Applicable Interest Period for each Credit Extension under this Credit Facility shall commence on the date that the applicable Credit Extension is made and end on the last day of such month.

Applicable Floor: means four percent (4.00%) per annum.

Applicable Margin: a rate of interest equal to six and one half percent (6.50%) per annum.

Applicable Prepayment Fee: means the following amount, calculated as of the date (the "Accrual Date") that the Applicable Prepayment Fee becomes payable in the case of prepayments required under the Financing Documents or the date any voluntary prepayment is made: (a) for an Accrual Date on or after the Closing Date through and including September 30, 2025, one and one half percent (1.5%) multiplied by the amount of the outstanding principal of the Credit Extension prepaid or required to be prepaid (whichever is greater) and (b) for an Accrual Date after September 30, 2025 through and including the date immediately preceding the Maturity Date, one percent (1.0%) multiplied by the amount of the outstanding principal of the Credit Extension prepaid or required to be prepaid (whichever is greater).

Commitment Commencement Date: the Second Amendment Effective Date.

Commitment Termination Date: the close of business on the Business Day following the Second Amendment Effective Date.

Minimum Credit Extension Amount: \$10,000,000.00

Schedule 4

Credit Facility #4:

Credit Facility and Type: Term, Tranche 4

Lenders for and their respective Applicable Commitments to this Credit Facility:

Lender	Applicable Commitment
MidCap Financial Trust	Seven Million Dollars (\$7,000,000)
Apollo Investment Corporation	Three Million Dollars (\$3,000,000)
Total:	Ten Million Dollars (\$10,000,000)

The following defined terms apply to this Credit Facility:

Applicable Funding Conditions: means:

(a) if the date on which the Credit Extensions under this Credit Facility #4 are to be made is prior to August 31, 2022, then the Applicable Funding Conditions are: N/A; or

(b) if the date on which the Credit Extensions under this Credit Facility #4 are to be made is on or after August 31, 2022, then the Applicable Funding Conditions are the following:

(i) [***]; and

(ii) Borrower is in compliance with all terms of the Financing Documents, including compliance with the financial covenant set forth in Sections 9.1 and 9.2, as of the most recent Testing Date occurring prior to the date on which the Credit Extensions under this Credit Facility #4 are to be made.

Applicable Interest Period: means the one-month period starting on the first (1st) day of each month and ending on the last day of such month; *provided, however, that the first (1st) Applicable Interest Period for each Credit Extension under this Credit Facility shall commence on the date that the applicable Credit Extension is made and end on the last day of such month.*

Applicable Floor: means four percent (4.00%) per annum.

Applicable Margin: a rate of interest equal to six and one half percent (6.50%) per annum.

Applicable Prepayment Fee: means the following amount, calculated as of the date (the "Accrual Date") that the Applicable Prepayment Fee becomes payable in the case of prepayments required under the Financing Documents or the date any voluntary prepayment is made: (a) for an Accrual Date on or after the Closing Date through and including September 30, 2025, one and one half percent (1.5%) multiplied by the amount of the outstanding principal of the Credit Extension prepaid or required to be prepaid (whichever is greater) and (b) for an Accrual Date after September 30, 2025 through and including the date immediately preceding the Maturity Date, one percent (1.0%) multiplied by the amount of the outstanding principal of the Credit Extension prepaid or required to be prepaid (whichever is greater).

Commitment Commencement Date: the Second Amendment Effective Date.

Commitment Termination Date: the earliest to occur of (a) March 31, 2023, (b) the date on which any Credit Extensions are made by the Lenders in respect of Credit Facility #5, and (c) the delivery of a written notice by Agent to Borrower terminating the Applicable Commitments following an Event of Default that has not been waived or cured at the time such notice is delivered.

Minimum Credit Extension Amount: \$10,000,000.00

Schedule 5

Credit Facility #5:

Credit Facility and Type: Term, Tranche 5

Lenders for and their respective Applicable Commitments to this Credit Facility:

Lender	Applicable Commitment
MidCap Financial Trust	Fourteen Million Dollars (\$14,000,000)
Apollo Investment Corporation	Six Million Dollars (\$6,000,000)
Total:	Twenty Million Dollars (\$20,000,000)

The following defined terms apply to this Credit Facility:

Applicable Funding Conditions: means the following:

(a) [**]; and

(c) Borrower is in compliance with all terms of the Financing Documents, including compliance with the financial covenant set forth in Sections 9.1 and 9.2, as of the most recent Testing Date occurring prior to the date on which the Credit Extensions under this Credit Facility #5 are to be made.

Applicable Interest Period: means the one-month period starting on the first (1st) day of each month and ending on the last day of such month; *provided, however,* that the first (1st) Applicable Interest Period for each Credit Extension under this Credit Facility shall commence on the date that the applicable Credit Extension is made and end on the last day of such month.

Applicable Floor: means four percent (4.00%) per annum.

Applicable Margin: a rate of interest equal to six and one half percent (6.50%) per annum.

Applicable Prepayment Fee: means the following amount, calculated as of the date (the "Accrual Date") that the Applicable Prepayment Fee becomes payable in the case of prepayments required under the Financing Documents or the date any voluntary prepayment is made: (a) for an Accrual Date on or after the Closing Date through and including September 30, 2025, one and one half percent (1.5%) multiplied by the amount of the outstanding principal of the Credit Extension prepaid or required to be prepaid (whichever is greater) and (b) for an Accrual Date after September 30, 2025 through and including the date immediately preceding the Maturity Date, one percent (1.0%) multiplied by the amount of the outstanding principal of the Credit Extension prepaid or required to be prepaid (whichever is greater).

Commitment Commencement Date: The satisfaction of the Applicable Funding Conditions for this Credit Facility.

Commitment Termination Date: the earliest to occur of (a) March 31, 2023, and (b) the delivery of a written notice by Agent to Borrower terminating the Applicable Commitments following an Event of Default that has not been waived or cured at the time such notice is delivered.

Minimum Credit Extension Amount: \$20,000,000.00

Schedule 6

AMORTIZATION SCHEDULE (FOR EACH CREDIT FACILITY)

Credit Facility #1

Commencing on October 1, 2025 (the "Initial Amortization Start Date") and continuing on the first day of each calendar month thereafter, an amount equal to the aggregate principal amount advanced under Credit Facility #1 divided by twenty-four (24).

Credit Facility #2

Commencing on the Initial Amortization Start Date and continuing on the first day of each calendar month thereafter, an amount equal to the aggregate principal amount advanced under Credit Facility #2 divided by twenty-four (24).

Credit Facility #3

Commencing on the Initial Amortization Start Date and continuing on the first day of each calendar month thereafter, an amount equal to the aggregate principal amount advanced under Credit Facility #3 divided by twenty-four (24).

Credit Facility #4:

Commencing on the Initial Amortization Start Date and continuing on the first day of each calendar month thereafter, an amount equal to the aggregate principal amount advanced under Credit Facility #4 divided by twenty-four (24).

Credit Facility #5:

Commencing on the Initial Amortization Start Date and continuing on the first day of each calendar month thereafter, an amount equal to the aggregate principal amount advanced under Credit Facility #5 divided by twenty-four (24).

Notwithstanding anything to the contrary contained in the foregoing, the entire remaining outstanding principal balance under all Credit Extensions shall mature and be due and payable upon the Maturity Date.

Schedule 7

MINIMUM TAVALISSE NET REVENUE SCHEDULE

[***]

Exhibit 10.4

RIGEL PHARMACEUTICALS, INC.

INDUCEMENT PLAN

ADOPTED BY THE COMPENSATION COMMITTEE: OCTOBER 10, 2016

AMENDED BY THE COMPENSATION COMMITTEE: JANUARY 3, 2017

AMENDED BY THE COMPENSATION COMMITTEE: AUGUST 16, 2017

AMENDED BY THE COMPENSATION COMMITTEE: NOVEMBER 7, 2017

AMENDED BY THE COMPENSATION COMMITTEE: DECEMBER 23, 2017

AMENDED BY THE COMPENSATION COMMITTEE: JANUARY 24, 2018

AMENDED BY THE COMPENSATION COMMITTEE: AUGUST 19, 2020

AMENDED BY THE COMPENSATION COMMITTEE: SEPTEMBER 30, 2021

AMENDED BY THE COMPENSATION COMMITTEE: JANUARY 4, 2022

AMENDED BY THE COMPENSATION COMMITTEE: APRIL 4, 2022

AMENDED BY THE COMPENSATION COMMITTEE: DECEMBER 8, 2022

AMENDED BY THE COMPENSATION COMMITTEE: APRIL 4, 2023

AMENDED BY THE COMPENSATION COMMITTEE: JUNE 29, 2023

AMENDED BY THE COMPENSATION COMMITTEE: OCTOBER 3, 2023

AMENDED BY THE COMPENSATION COMMITTEE: DECEMBER 26, 2023

1. GENERAL.

(a) Eligible Stock Award Recipients. The only persons eligible to receive grants of Stock Awards under this Plan are individuals who satisfy the standards for inducement grants under NASDAQ Marketplace Rule 5635(c)(4) and the related guidance under NASDAQ IM 5635-1. A person who previously served as an Employee or Director will not be eligible to receive Stock Awards under the Plan, other than following a *bona fide* period of non-employment. Persons eligible to receive grants of Stock Awards under this Plan are referred to in this Plan as "*Eligible Employees*". These Stock Awards must be approved by either a majority of the Company's "*Independent Directors*" (as such term is defined in NASDAQ Listing Rule 5605(a)(2)) or the Company's compensation committee, provided such committee is comprised solely of Independent Directors (the "*Independent Compensation Committee*") in order to comply with the exemption from the stockholder approval requirement for "inducement grants" provided under Rule 5635(c)(4) of the NASDAQ Listing Rules. NASDAQ Marketplace Rule 5635(c)(4) and the related guidance under NASDAQ IM 5635-1 are referred to in this Plan as the "*Inducement Award Rules*".

(b) Available Awards. The Plan provides for the grant of Options and Restricted Stock Unit Awards. All Options will be Nonstatutory Stock Options. Awards intended to qualify as stockholder-approved performance based compensation for purposes of Section 162(m) of the Code may not be granted under this Plan.

(c) Purpose. This Plan, through the granting of Stock Awards, is intended to provide (i) an inducement material for certain individuals to enter into employment with the Company within the meaning of Rule 5635(c)(4) of the NASDAQ Listing Rules, (ii) incentives for such persons to exert maximum efforts for the success of the Company

and any Affiliate and (iii) a means by which Eligible Employees may be given an opportunity to benefit from increases in value of the Common Stock through the granting of Stock Awards.

2. ADMINISTRATION.

(a) Administration by Board. The Board will administer the Plan, provided, however, that Stock Awards may only be granted by either (i) a majority of the Company's Independent Directors or (ii) the Independent Compensation Committee. Subject to those constraints and the other constraints of the Inducement Award Rules, the Board may delegate some of its powers of administration of the Plan to a Committee, as provided in Section 2(c).

(b) Powers of Board. The Board will have the power, subject to, and within the limitations of, the express provisions of the Plan and the Inducement Award Rules:

(i) To determine: (A) who will be granted Stock Awards; (B) when and how each Stock Award will be granted; (C) what type of Stock Award will be granted; (D) the provisions of each Stock Award (which need not be identical), including when a person will be permitted to exercise or otherwise receive cash or Common Stock under the Stock Award; (E) the number of shares of Common Stock subject to, or the cash value of, a Stock Award; and (F) the Fair Market Value applicable to a Stock Award; provided, however, that Stock Awards may only be granted by either (i) a majority of the Company's Independent Directors or (ii) the Independent Compensation Committee.

(ii) To construe and interpret the Plan and Stock Awards granted under it, and to establish, amend and revoke rules and regulations for administration of the Plan and Stock Awards. The Board, in the exercise of these powers, may correct any defect, omission or inconsistency in the Plan or in any Stock Award Agreement, in a manner and to the extent it will deem necessary or expedient to make the Plan or Stock Award fully effective.

(iii) To settle all controversies regarding the Plan and Stock Awards granted under it.

(iv) To accelerate, in whole or in part, the time at which a Stock Award may be exercised or vest (or at which cash or shares of Common Stock may be issued).

(v) To suspend or terminate the Plan at any time. Except as otherwise provided in the Plan or a Stock Award Agreement, suspension or termination of the Plan will not materially impair a Participant's rights under his or her then-outstanding Stock Award without his or her written consent except as provided in subsection (viii) below.

(vi) To amend the Plan in any respect the Board deems necessary or advisable, including, without limitation, adopting amendments relating to nonqualified deferred compensation under Section 409A of the Code and/or making the Plan or Stock Awards granted under the Plan exempt from or compliant with the requirements for nonqualified deferred compensation under Section 409A of the

Code, subject to the limitations, if any, of applicable law. If required by applicable law or listing requirements, and except as provided in Section 9(a) relating to Capitalization Adjustments, the Company will seek stockholder approval of any amendment of the Plan that (A) materially increases the number of shares of Common Stock available for issuance under the Plan, (B) materially expands the class of individuals eligible to receive Stock Awards under the Plan, (C) materially increases the benefits accruing to Participants under the Plan, (D) materially reduces the price at which shares of Common Stock may be issued or purchased under the Plan, (E) materially extends the term of the Plan, or (F) materially expands the types of Stock Awards available for issuance under the Plan. Except as otherwise provided in the Plan (including subsection (viii) below) or a Stock Award Agreement, no amendment of the Plan will materially impair a Participant's rights under an outstanding Stock Award without the Participant's written consent.

(vii) To submit any amendment to the Plan for stockholder approval, including, but not limited to, amendments to the Plan intended to satisfy the requirements of Rule 16b-3 of Exchange Act or any successor rule.

(viii) To approve forms of Stock Award Agreements for use under the Plan and to amend the terms of any one or more outstanding Stock Awards, including, but not limited to, amendments to provide terms more

favorable to the Participant than previously provided in the Stock Award Agreement, subject to any specified limits in the Plan that are not subject to Board discretion. A Participant's rights under any Stock Award will not be impaired by any such amendment unless the Company requests the consent of the affected Participant, and the Participant consents in writing. However, a Participant's rights will not be deemed to have been impaired by any such amendment if the Board, in its sole discretion, determines that the amendment, taken as a whole, does not materially impair the Participant's rights. In addition, subject to the limitations of applicable law, if any, the Board may amend the terms of any one or more Stock Awards without the affected Participant's consent (A) to clarify the manner of exemption from, or to bring the Stock Award into compliance with, Section 409A of the Code, or (B) to comply with other applicable laws or listing requirements.

(ix) Generally, to exercise such powers and to perform such acts as the Board deems necessary or expedient to promote the best interests of the Company and that are not in conflict with the provisions of the Plan and/or Stock Award Agreements.

(x) To adopt such procedures and sub-plans as are necessary or appropriate (A) to permit participation in the Plan by individuals who are foreign nationals or employed outside the United States or (B) allow Stock Awards to qualify for special tax treatment in a foreign jurisdiction; *provided* that Board approval will not be necessary for immaterial modifications to the Plan or any Stock Award Agreement that are required for compliance with the laws of the relevant foreign jurisdiction.

(c) Delegation to Committee.

(i) **General.** The Board may delegate some or all of the administration of the Plan to a Committee or Committees. If administration of the Plan is delegated to a Committee, the Committee will have, in connection with the administration of the Plan, the powers theretofore possessed by the Board that have been delegated to the Committee, including the power to delegate to a subcommittee of the Committee any of the administrative powers the Committee is authorized to exercise (and references in this Plan to the Board will thereafter be to the Committee or subcommittee). Any delegation of administrative powers will be reflected in resolutions, not inconsistent with the provisions of the Plan, adopted from time to time by the Board or Committee (as applicable). The Committee may, at any time, abolish the subcommittee and/or revest in the Committee any powers delegated to the subcommittee. The Board may retain the authority to concurrently administer the Plan with the Committee and may, at any time, revest in the Board some or all of the powers previously delegated.

(ii) **Rule 16b-3 Compliance.** The Committee may consist solely of two or more Non-Employee Directors, in accordance with Rule 16b-3 of the Exchange Act.

(d) **Effect of Board's Decision.** All determinations, interpretations and constructions made by the Board in good faith will not be subject to review by any person and will be final, binding and conclusive on all persons.

(e) **Cancellation and Re-Grant of Stock Awards.** Neither the Board nor any Committee will have the authority to: (i) reduce the exercise, purchase or strike price of any outstanding Option, or (ii) cancel any outstanding Option that has an exercise price or strike price greater than the current Fair Market Value of the Common Stock in exchange for cash or other Stock Awards under the Plan, unless the stockholders of the Company have approved such an action within twelve (12) months prior to such an event.

3. SHARES SUBJECT TO THE PLAN.

(a) Share Reserve.

(i) Subject to Section 9(a) relating to Capitalization Adjustments, the aggregate number of shares of Common Stock that may be issued pursuant to Stock Awards will not exceed 6,230,025 shares (the "Share Reserve").

Company Guide Section 711 or other applicable rule, and such issuance will not reduce the number of shares available for issuance under the Plan.

(b) Reversion of Shares to the Share Reserve. If a Stock Award or any portion of a Stock Award (i) expires or otherwise terminates without all of the shares covered by the Stock Award having been issued or (ii) is settled in cash (i.e., the Participant receives cash rather than stock), such expiration, termination or settlement will nevertheless reduce (or otherwise offset) the number of shares of Common Stock that are available for issuance under the Plan. If any shares of Common Stock issued under a Stock Award are forfeited back to or repurchased by the Company because of the failure to meet a contingency or condition required to vest such shares in the Participant, then the shares that are forfeited or repurchased will not revert to and again become available for issuance under the Plan. Any shares reacquired by the Company in satisfaction of tax withholding obligations on a Stock Award or as consideration for the exercise or purchase price of a Stock Award will not again become available for issuance under the Plan.

(c) Source of Shares. The stock issuable under the Plan will be shares of authorized but unissued or reacquired Common Stock, including shares repurchased by the Company on the open market or otherwise.

4. ELIGIBILITY.

(a) Eligibility for Specific Stock Awards. Stock Awards may only be granted to persons who are Eligible Employees described in Section 1(a) of the Plan, where the Stock Award is an inducement material to the individual's entering into employment with the Company or an Affiliate within the meaning of Rule 5635(c)(4) of the NASDAQ Listing Rules, *provided however*, that Stock Awards may not be granted to Eligible Employees who are providing Continuous Service only to any "parent" of the Company, as such term is defined in Rule 405 of the Securities Act, unless (i) the stock underlying such Stock Awards is treated as "service recipient stock" under Section 409A of the Code (for example, because the Stock Awards are granted pursuant to a corporate transaction such as a spin off transaction), or (ii) the Company, in consultation with its legal counsel, has determined that such Stock Awards are otherwise exempt from or comply with the distribution requirements of Section 409A of the Code.

(b) Approval Requirements. All Stock Awards must be granted either by a majority of the Company's independent directors or the Independent Compensation Committee.

5. PROVISIONS RELATING TO OPTIONS.

Each Option will be in such form and will contain such terms and conditions as the Board deems appropriate. All Options will be Nonstatutory Stock Options. The provisions of separate Options need not be identical; *provided, however*, that each Option Agreement will conform to (through incorporation of provisions hereof by reference in the applicable Option Agreement or otherwise) the substance of each of the following provisions:

(a) Term. No Option will be exercisable after the expiration of 10 years from the date of its grant or such shorter period specified in the Option Agreement.

(b) Exercise Price. The exercise or strike price of each Option will be not less than 100% of the Fair Market Value of the Common Stock subject to the Option on the date the Option is granted. Notwithstanding the foregoing, an Option may be granted with an exercise price lower than 100% of the Fair Market Value of the Common Stock subject to the Option if such Option is granted pursuant to an assumption of or substitution for another option or stock appreciation right pursuant to a Corporate Transaction and in a manner consistent with the provisions of Section 409A of the Code.

(c) Purchase Price for Options. The purchase price of Common Stock acquired pursuant to the exercise of an Option may be paid, to the extent permitted by applicable law and as determined by the Board in its sole discretion, by any combination of the methods of payment set forth below. The Board will have the authority to grant Options that do not permit all of the following methods of payment (or otherwise restrict the ability to use certain

methods) and to grant Options that require the consent of the Company to use a particular method of payment. The permitted methods of payment are as follows:

- (i) by cash, check, bank draft or money order payable to the Company;
- (ii) pursuant to a program developed under Regulation T as promulgated by the Federal Reserve Board that, prior to the issuance of the stock subject to the Option, results in either the receipt of cash (or check) by the Company or the receipt of irrevocable instructions to pay the aggregate exercise price to the Company from the sales proceeds;
- (iii) by delivery to the Company (either by actual delivery or attestation) of shares of Common Stock;
- (iv) by a "net exercise" arrangement pursuant to which the Company will reduce the number of shares of Common Stock issuable upon exercise by the largest whole number of shares with a Fair Market Value that does not exceed the aggregate exercise price; *provided, however,* that the Company will accept a cash or other payment from the Participant to the extent of any remaining balance of the aggregate exercise price not satisfied by such reduction in the number of whole shares to be issued. Shares of Common Stock will no longer be subject to an Option and will not be exercisable thereafter to the extent that (A) shares issuable upon exercise are used to pay the exercise price pursuant to the "net exercise," (B) shares are delivered to the Participant as a result of such exercise, and (C) shares are withheld to satisfy tax withholding obligations; or
- (v) in any other form of legal consideration that may be acceptable to the Board and specified in the applicable Option Agreement.

(d) **Transferability of Options.** The Board may, in its sole discretion, impose such limitations on the transferability of Options as the Board will determine. In the absence of such a determination by the Board to the contrary, the following restrictions on the transferability of Options will apply:

- (i) **Restrictions on Transfer.** An Option will not be transferable except by will or by the laws of descent and distribution (or pursuant to subsections (ii) and (iii) below), and will be exercisable during the lifetime of the Participant only by the Participant. The Board may permit transfer of the Option in a manner that is not prohibited by applicable tax and securities laws. Except as explicitly provided in the Plan, an Option may not be transferred for consideration.
- (ii) **Domestic Relations Orders.** Subject to the approval of the Board or a duly authorized Officer, an Option may be transferred pursuant to the terms of a domestic relations order, official marital settlement agreement or other divorce or separation instrument.
- (iii) **Beneficiary Designation.** Subject to the approval of the Board or a duly authorized Officer, a Participant may, by delivering written notice to the Company, in a form approved by the Company (or the designated broker), designate a third party who, on the death of the Participant, will thereafter be entitled to exercise the Option and receive the Common Stock or other consideration resulting from such exercise. In the absence of such a designation, the executor or administrator of the Participant's estate will be entitled to exercise the Option and receive the Common Stock or other consideration resulting from such exercise. However, the Company may prohibit designation of a beneficiary at any time, including due to any conclusion by the Company that such designation would be inconsistent with the provisions of applicable laws.
- (e) **Vesting Generally.** The total number of shares of Common Stock subject to an Option may vest and therefore become exercisable in periodic installments that may or may not be equal. The Option may be subject to such other terms and conditions on the time or times when it may or may not be exercised (which may be based on the satisfaction of performance goals or other criteria) as the Board may deem appropriate. The vesting provisions of individual Options may vary. The provisions of this Section 5(e) are subject to any Option provisions governing the minimum number of shares of Common Stock as to which an Option may be exercised.

(f) **Termination of Continuous Service.** Except as otherwise provided in the applicable Option Agreement or other agreement between the Participant and the Company, if a Participant's Continuous Service terminates (other than for Cause and other than upon the Participant's death or Disability), the Participant may exercise his or her Option (to the extent that the Participant was entitled to exercise such Option as of the date of termination of Continuous Service) within the period of time ending on the earlier of (i) the date which occurs 3 months following the termination of the Participant's Continuous Service, and (ii) the expiration of the term of the Option as set forth in the Option Agreement. If, after termination of Continuous Service, the Participant does not exercise his or her Option within the applicable time frame, the Option will terminate.

(g) **Extension of Termination Date.** Except as otherwise provided in the applicable Stock Award Agreement, if the exercise of an Option following the termination of the Participant's Continuous Service (other than for Cause and other than upon the Participant's death or Disability) would be prohibited at any time solely because the issuance of shares of Common Stock would violate the registration requirements under the Securities Act, then the Option will terminate on the earlier of (i) the expiration of a total period of time (that need not be consecutive) equal to the applicable post-termination exercise period after the termination of the Participant's Continuous Service during which the exercise of the Option would not be in violation of such registration requirements, and (ii) the expiration of the term of the Option as set forth in the applicable Option Agreement. In addition, unless otherwise provided in a Participant's Option Agreement, if the sale of any Common Stock received upon exercise of an Option following the termination of the Participant's Continuous Service (other than for Cause) would violate the Company's insider trading policy, then the Option will terminate on the earlier of (i) the expiration of a

period of days or months (that need not be consecutive) equal to the applicable post-termination exercise period after the termination of the Participant's Continuous Service during which the sale of the Common Stock received upon exercise of the Option would not be in violation of the Company's insider trading policy, or (ii) the expiration of the term of the Option as set forth in the applicable Option Agreement.

(h) Disability of Participant. Except as otherwise provided in the applicable Option Agreement or other agreement between the Participant and the Company, if a Participant's Continuous Service terminates as a result of the Participant's Disability, the Participant may exercise his or her Option (to the extent that the Participant was entitled to exercise such Option as of the date of termination of Continuous Service), but only within such period of time ending on the earlier of (i) the date 12 months following such termination of Continuous Service and (ii) the expiration of the term of the Option as set forth in the Option Agreement. If, after termination of Continuous Service, the Participant does not exercise his or her Option within the applicable time frame, the Option will terminate.

(i) Death of Participant. Except as otherwise provided in the applicable Option Agreement or other agreement between the Participant and the Company, if (i) a Participant's Continuous Service terminates as a result of the Participant's death, or (ii) the Participant dies within the period (if any) specified in the Option Agreement for exercisability after the termination of the Participant's Continuous Service for a reason other than death, then the Option may be exercised (to the extent the Participant was entitled to exercise such Option as of the date of death) by the Participant's estate, by a person who acquired the right to exercise the Option by bequest or inheritance or by a person designated to exercise the Option upon the Participant's death, but only within the period ending on the earlier of (i) the date 18 months following the date of death, and (ii) the expiration of the term of such Option as set forth in the Option Agreement. If, after the Participant's death, the Option is not exercised within the applicable time frame, the Option will terminate.

(j) Termination for Cause. Except as explicitly provided otherwise in a Participant's Stock Award Agreement or other individual written agreement between the Company or any Affiliate and the Participant, if a Participant's Continuous Service is terminated for Cause, the Option will terminate upon the date on which the event giving rise to the termination for Cause first occurred, and the Participant will be prohibited from exercising his or her Option from and after the date on which the event giving rise to the termination for Cause first occurred (or, if required by applicable law, the date of termination of Continuous Service). If a Participant's Continuous Service is suspended pending an investigation of the existence of Cause, all of the Participant's rights under the Option will also be suspended during the investigation period, except to the extent prohibited by applicable law.

(k) Non-Exempt Employees. If an Option is granted to an Employee who is a non-exempt employee for purposes of the Fair Labor Standards Act of 1938, as amended, the Option will not be first exercisable for any shares of Common Stock until at least 6 months following the date of grant of the Option (although the Option may

vest prior to such date). Consistent with the provisions of the Worker Economic Opportunity Act, (i) if such non-exempt Employee dies or suffers a Disability, (ii) upon a Corporate Transaction in which such Option is not assumed, continued, or substituted, or (iii) upon the non-exempt Employee's retirement (as such term may be defined in the non-exempt Employee's Option Agreement in another agreement between the non-exempt Employee and the Company, or, if no such definition, in accordance with the Company's then current employment policies and guidelines), the vested portion of any Options may be exercised earlier than 6 months following the date of grant. The foregoing provision is intended to operate so that any income derived by a non-exempt employee in connection with the exercise or vesting of an Option will be exempt from his or her regular rate of pay. To the extent permitted and/or required for compliance with the Worker Economic Opportunity Act to ensure that any income derived by a non-exempt Employee in connection with the exercise, vesting or issuance of any shares under any other Option will be exempt from such Employee's regular rate of pay, the provisions of this paragraph will apply to all Options and are hereby incorporated by reference into such Option Agreements.

6. PROVISIONS RELATING TO RESTRICTED STOCK UNIT AWARDS.

Each Restricted Stock Unit Award Agreement will be in such form and will contain such terms and conditions as the Board deems appropriate. The terms and conditions of Restricted Stock Unit Award Agreements may change from time to time, and the terms and conditions of separate Restricted Stock Unit Award Agreements need not be identical. Each Restricted Stock Unit Award Agreement will conform to (through incorporation of the provisions hereof by reference in the Agreement or otherwise) the substance of each of the following provisions:

(a) Consideration. At the time of grant of a Restricted Stock Unit Award, the Board will determine the consideration, if any, to be paid by the Participant upon delivery of each share of Common Stock subject to the Restricted Stock Unit Award. The consideration to be paid (if any) by the Participant for each share of Common Stock subject to a Restricted Stock Unit Award may be paid in any form of legal consideration that may be acceptable to the Board, in its sole discretion, and permissible under applicable law.

(b) Vesting. At the time of the grant of a Restricted Stock Unit Award, the Board may impose such restrictions on or conditions to the vesting of the Restricted Stock Unit Award as it, in its sole discretion, deems appropriate.

(c) Payment. A Restricted Stock Unit Award may be settled by the delivery of shares of Common Stock, their cash equivalent, any combination thereof or in any other form of consideration, as determined by the Board and contained in the Restricted Stock Unit Award Agreement.

(d) Additional Restrictions. At the time of the grant of a Restricted Stock Unit Award, the Board, as it deems appropriate, may impose such restrictions or conditions that delay the delivery of the shares of Common Stock (or their cash equivalent) subject to a Restricted Stock Unit Award to a time after the vesting of such Restricted Stock Unit Award.

(e) Dividend Equivalents. Dividend equivalents may be credited in respect of shares of Common Stock covered by a Restricted Stock Unit Award, as determined by the Board and contained in the Restricted Stock Unit Award Agreement. At the sole discretion of the Board, such dividend equivalents may be converted into additional shares of Common Stock covered by the Restricted Stock Unit Award in such manner as determined by the Board. Any additional shares covered by the Restricted Stock Unit Award credited by reason of such dividend equivalents will be subject to all of the same terms and conditions of the underlying Restricted Stock Unit Award Agreement to which they relate.

(f) Termination of Participant's Continuous Service. Except as otherwise provided in the applicable Restricted Stock Unit Award Agreement, such portion of the Restricted Stock Unit Award that has not vested will be forfeited upon the Participant's termination of Continuous Service.

7. COVENANTS OF THE COMPANY.

(a) Availability of Shares. The Company will keep available at all times the number of shares of Common Stock reasonably required to satisfy then-outstanding Stock Awards.

(b) Securities Law Compliance. The Company will seek to obtain from each regulatory commission or agency having jurisdiction over the Plan such authority as may be required to grant Stock Awards and to issue and sell shares of Common Stock upon exercise of the Stock Awards; *provided, however,* that this undertaking will not require the Company to register under the Securities Act the Plan, any Stock Award or any Common Stock issued or issuable pursuant to any such Stock Award. If, after reasonable efforts and at a reasonable cost, the Company is unable to obtain from any such regulatory commission or agency the authority that counsel for the Company deems necessary for the lawful issuance and sale of Common Stock under the Plan, the Company will be relieved from any liability for failure to issue and sell Common Stock upon exercise of such Stock Awards unless and until such authority is obtained. A Participant will not be eligible for the grant of a Stock Award or the subsequent issuance of cash or Common Stock pursuant to the Stock Award if such grant or issuance would be in violation of any applicable securities law.

(c) No Obligation to Notify or Minimize Taxes. The Company will have no duty or obligation to any Participant to advise such holder as to the time or manner of exercising such Stock Award. Furthermore, the Company will have no duty or obligation to warn or otherwise advise such holder of a pending termination or expiration of a Stock Award or a possible period in which the Stock Award may not be exercised. The Company has no duty or obligation to minimize the tax consequences of a Stock Award to the holder of such Stock Award.

8. MISCELLANEOUS.

(a) Use of Proceeds from Sales of Common Stock. Proceeds from the sale of shares of Common Stock pursuant to Stock Awards will constitute general funds of the Company.

(b) Corporate Action Constituting Grant of Stock Awards. Corporate action constituting a grant by the Company of a Stock Award to any Participant will be deemed completed as of the date of such corporate action, unless otherwise determined by the Board, regardless of when the instrument, certificate, or letter evidencing the Stock Award is communicated to, or actually received or accepted by, the Participant. In the event that the corporate records (e.g., Board consents, resolutions or minutes) documenting the corporate action constituting the grant contain terms (e.g., exercise price, vesting schedule or number of shares) that are inconsistent with those in the Stock Award Agreement as a result of a clerical error in the papering of the Stock Award Agreement, the corporate records will control and the Participant will have no legally binding right to the incorrect term in the Stock Award Agreement.

(c) Stockholder Rights. No Participant will be deemed to be the holder of, or to have any of the rights of a holder with respect to, any shares of Common Stock subject to a Stock Award unless and until (i) such Participant has satisfied all requirements for exercise of, or the issuance of shares of Common Stock under, the Stock Award pursuant to its terms, and (ii) the issuance of the Common Stock subject to such Stock Award has been entered into the books and records of the Company.

(d) No Employment or Other Service Rights. Nothing in the Plan, any Stock Award Agreement or any other instrument executed thereunder or in connection with any Stock Award granted pursuant thereto will confer upon any Participant any right to continue to serve the Company or an Affiliate in the capacity in effect at the time the Stock Award was granted or will affect the right of the Company or an Affiliate to terminate (i) the employment of an Employee with or without notice and with or without cause, including, but not limited to, Cause, (ii) the service of a Consultant pursuant to the terms of such Consultant's agreement with the Company or an Affiliate,

or (iii) the service of a Director pursuant to the bylaws of the Company or an Affiliate, and any applicable provisions of the corporate law of the state in which the Company or the Affiliate is incorporated, as the case may be.

(e) Change in Time Commitment. In the event a Participant's regular level of time commitment in the performance of his or her services for the Company and any Affiliates is reduced (for example, and without limitation, if the Participant is an Employee of the Company and the Employee has a change in status from a full-time Employee to a part-time Employee or takes an extended leave of absence) after the date of grant of any Stock Award

to the Participant, the Board has the right in its sole discretion to (i) make a corresponding reduction in the number of shares or cash amount subject to any portion of such Stock Award that is scheduled to vest or become payable after the date of such change in time commitment, and (ii) in lieu of or in combination with such a reduction, extend the vesting or payment schedule applicable to such Stock Award. In the event of any such reduction, the Participant will have no right with respect to any portion of the Stock Award that is so reduced or extended.

(f) Investment Assurances. The Company may require a Participant, as a condition of exercising or acquiring Common Stock under any Stock Award, (i) to give written assurances satisfactory to the Company as to the Participant's knowledge and experience in financial and business matters and/or to employ a purchaser representative reasonably satisfactory to the Company who is knowledgeable and experienced in financial and business matters and that he or she is capable of evaluating, alone or together with the purchaser representative, the merits and risks of exercising the Stock Award, and (ii) to give written assurances satisfactory to the Company stating that the Participant is acquiring Common Stock subject to the Stock Award for the Participant's own account and not with any present intention of selling or otherwise distributing the Common Stock. The foregoing requirements, and any assurances given pursuant to such requirements, will be inoperative if (i) the issuance of the shares upon the exercise of a Stock Award or acquisition of Common Stock under the Stock Award has been registered under a then currently effective registration statement under the Securities Act, or (ii) as to any particular requirement, a determination is made by counsel for the Company that such requirement need not be met in the circumstances under the then applicable securities laws. The Company may, upon advice of counsel to the Company, place legends on stock certificates issued under the Plan as such counsel deems necessary or appropriate in order to comply with applicable securities laws, including, but not limited to, legends restricting the transfer of the Common Stock.

(g) Withholding Obligations. Unless prohibited by the terms of a Stock Award Agreement, the Company may, in its sole discretion, satisfy any U.S. federal, state, local, foreign or other tax withholding obligation relating to a Stock Award by any of the following means or by a combination of such means: (i) causing the Participant to tender a cash payment; (ii) withholding shares of Common Stock from the shares of Common Stock issued or otherwise issuable to the Participant in connection with the Stock Award; *provided, however,* that no shares of Common Stock are withheld with a value exceeding the minimum amount of tax required to be withheld by law (or such other amount as may be necessary to avoid classification of the Stock Award as a liability for financial accounting purposes); (iii) withholding cash from a Stock Award settled in cash; (iv) withholding payment from any amounts otherwise payable to the Participant, including proceeds from the sale of shares of Common Stock issued pursuant to a Stock Award; or (v) by such other method as may be set forth in the Stock Award Agreement.

(h) Electronic Delivery. Any reference herein to a "written" agreement or document will include any agreement or document delivered electronically, filed publicly at www.sec.gov (or any successor website thereto), or posted on the Company's intranet (or other shared electronic medium controlled by the Company to which the Participant has access).

(i) Deferrals. To the extent permitted by applicable law, the Board, in its sole discretion, may determine that the delivery of Common Stock or the payment of cash, upon the exercise, vesting or settlement of all or a portion of any Stock Award may be deferred and may establish programs and procedures for deferral elections to be made by Participants. Deferrals by Participants will be made in accordance with Section 409A of the Code (to the extent applicable to a Participant). Consistent with Section 409A of the Code, the Board may provide for distributions while a Participant is still an employee or otherwise providing services to the Company. The Board is authorized to make deferrals of Stock Awards and determine when, and in what annual percentages, Participants may receive payments, including lump sum payments, following the Participant's termination of Continuous Service, and implement such other terms and conditions consistent with the provisions of the Plan and in accordance with applicable law.

(j) Compliance with Section 409A. Unless otherwise expressly provided for in a Stock Award Agreement and the Plan will be interpreted to the greatest extent possible in a manner that makes the Plan and the Stock Awards granted hereunder exempt from Section 409A of the Code, and, to the extent not so exempt, in compliance with Section 409A of the Code. If the Board determines that any Stock Award granted hereunder is not exempt from and is therefore subject to Section 409A of the Code, the Stock Award Agreement evidencing such Stock Award will incorporate the terms and conditions necessary to avoid the consequences specified in Section 409A(a)(1) of the Code, and to the extent a Stock Award Agreement is silent on terms necessary for compliance, such terms are

hereby incorporated by reference into the Stock Award Agreement. Notwithstanding anything to the contrary in this Plan (and unless the Stock Award Agreement specifically provides otherwise), if the shares of Common Stock are publicly traded, and if a Participant holding a Stock Award that constitutes "deferred compensation" under Section 409A of the Code is a "specified employee" for purposes of Section 409A of the Code, no distribution or payment of any amount that is due because of a "separation from service" (as defined in Section 409A of the Code) will be issued or paid before the date that is six (6) months following the date of such Participant's "separation from service" or, if earlier, the date of the Participant's death, unless such distribution or payment can be made in a manner that complies with Section 409A of the Code, and any amounts so deferred will be paid in a lump sum on the day after such six (6) month period elapses, with the balance paid thereafter on the original schedule.

(k) Clawback/Recovery. All Stock Awards granted under the Plan will be subject to recoupment in accordance with any clawback policy that the Company is required to adopt pursuant to the listing standards of any national securities exchange or association on which the Company's securities are listed or as is otherwise required by the Dodd-Frank Wall Street Reform and Consumer Protection Act or other applicable law. In addition, the Board may impose such other clawback, recovery or recoupment provisions in a Stock Award Agreement as the Board determines necessary or appropriate, including, but not limited to, a reacquisition right in respect of previously acquired shares of Common Stock or other cash or property upon the occurrence of an event constituting Cause. No recovery of compensation under such a clawback policy will be an event giving rise to a right to resign for "good reason" or "constructive termination" (or similar term) under any agreement with the Company or an Affiliate.

9. ADJUSTMENTS UPON CHANGES IN COMMON STOCK; OTHER CORPORATE EVENTS.

(a) Capitalization Adjustments. In the event of a Capitalization Adjustment, the Board will appropriately and proportionately adjust: (i) the class(es) and maximum number of securities subject to the Plan pursuant to Section 3(a) and (ii) the class(es) and number of securities and price per share of stock subject to outstanding Stock Awards. The Board will make such adjustments, and its determination will be final, binding and conclusive.

(b) Dissolution or Liquidation. In the event of a dissolution or liquidation of the Company, then all outstanding Stock Awards shall terminate immediately prior to such event.

(c) Corporate Transaction. In the event of (i) a sale, lease or other disposition of all or substantially all of the securities or assets of the Company, (ii) a merger or consolidation in which the Company is not the surviving corporation or (iii) a reverse merger in which the Company is the surviving corporation but the shares of Common Stock outstanding immediately preceding the merger are converted by virtue of the merger into other property, whether in the form of securities, cash or otherwise (a "Corporate Transaction"), then any surviving corporation or acquiring corporation may assume any Stock Awards outstanding under the Plan or may substitute similar stock awards (including an award to acquire the same consideration paid to the stockholders in such Corporate Transaction) for those outstanding under the Plan. In the event any surviving corporation or acquiring corporation does not assume such Stock Awards or substitute similar stock awards for those outstanding under the Plan, then with respect to Stock Awards held by Participants whose Continuous Service has not terminated, the vesting of such Stock Awards (and, if applicable, the time during which such Stock Awards may be exercised) shall be accelerated in full, and the Stock Awards shall terminate if not exercised (if applicable) at or prior to such event. With respect to any other Stock Awards outstanding under the Plan, such Stock Awards shall terminate if not exercised (if applicable) prior to such event.

10. TERMINATION OR SUSPENSION OF THE PLAN.

The Board may suspend or terminate the Plan at any time. No Stock Awards may be granted under the Plan while the Plan is suspended or after it is terminated.

11. EFFECTIVE DATE OF PLAN; TIMING OF FIRST GRANT OR EXERCISE.

The Plan will come into existence on the Effective Date. No Stock Award may be granted prior to the Effective Date.

10

12. CHOICE OF LAW.

The laws of the State of Delaware will govern all questions concerning the construction, validity and interpretation of this Plan, without regard to that state's conflict of laws rules.

13. DEFINITIONS. As used in the Plan, the following definitions will apply to the capitalized terms indicated below:

(a) "Affiliate" means, at the time of determination, any "parent" or "subsidiary" of the Company, as such terms are defined in Rule 405 of the Securities Act. The Board will have the authority to determine the time or times at which "parent" or "subsidiary" status is determined within the foregoing definition.

- (b) **"Board"** means the Board of Directors of the Company.
- (c) **"Capitalization Adjustment"** means any change that is made in, or other events that occur with respect to, the Common Stock subject to the Plan or subject to any Stock Award after the Effective Date without the receipt of consideration by the Company through merger, consolidation, reorganization, recapitalization, reincorporation, stock dividend, dividend in property other than cash, large nonrecurring cash dividend, stock split, reverse stock split, liquidating dividend, combination of shares, exchange of shares, change in corporate structure or other similar equity restructuring transaction, as that term is used in Financial Accounting Standards Board Accounting Standards Codification Topic 718 (or any successor thereto). Notwithstanding the foregoing, the conversion of any convertible securities of the Company will not be treated as a Capitalization Adjustment.
- (d) **"Cause"** will have the meaning ascribed to such term in any written agreement between the Participant and the Company or any Affiliate defining such term and, in the absence of such agreement, such term means, with respect to a Participant, the occurrence of any of the following events: (i) such Participant's conviction of any felony or any crime involving moral turpitude or dishonesty, (ii) such Participant's participation in a fraud or act of dishonesty against the Company, (iii) such Participant's conduct that, based upon a good faith and reasonable factual investigation and determination by the Board, demonstrates the Participant's gross unfitness to serve, or (iv) such Participant's intentional, material violation of any contract between the Company and the Participant or any statutory duty that the Participant has to the Company that the Participant does not correct within 30 days after written notice to the Participant thereof. The determination as to whether a Participant is being terminated for Cause will be made in good faith by the Company and will be final and binding on the Participant. Any determination by the Company that the Continuous Service of a Participant was terminated with or without Cause for the purposes of outstanding Stock Awards held by such Participant will have no effect upon any determination of the rights or obligations of the Company, any Affiliate or such Participant for any other purpose.
- (e) **"Code"** means the U.S. Internal Revenue Code of 1986, as amended, including any applicable regulations and guidance thereunder.
- (f) **"Committee"** means a committee of one (1) or more Independent Directors to whom authority has been delegated by the Board in accordance with Section 2(c).
- (g) **"Common Stock"** means the common stock of the Company.
- (h) **"Company"** means Rigel Pharmaceuticals, Inc., a Delaware corporation.
- (i) **"Consultant"** means any person, including an advisor, who is (i) engaged by the Company or an Affiliate to render consulting or advisory services and is compensated for such services, or (ii) serving as a member of the board of directors of an Affiliate and is compensated for such services. However, service solely as a Director, or payment of a fee for such service, will not cause a Director to be considered a "Consultant" for purposes of the Plan. Notwithstanding the foregoing, a person is treated as a Consultant

11

under this Plan only if a Form S-8 Registration Statement under the Securities Act is available to register either the offer or the sale of the Company's securities to such person.

- (j) **"Continuous Service"** means that the Participant's service with the Company or an Affiliate, whether as an Employee, Director or Consultant, is not interrupted or terminated. A change in the capacity in which the Participant renders service to the Company or an Affiliate as an Employee, Consultant or Director or a change in the Entity for which the Participant renders such service, provided that there is no interruption or termination of the Participant's service with the Company or an Affiliate, will not terminate a Participant's Continuous Service. For example, a change in status from an Employee of the Company to a Consultant of an Affiliate or to a Director will not constitute an interruption of Continuous Service. If the Entity for which a Participant is rendering services ceases to qualify as an Affiliate, as determined by the Board in its sole discretion, such Participant's Continuous Service will be considered to have terminated on the date such Entity ceases to qualify as an Affiliate. To the extent permitted by law, the Board or the chief executive officer of the Company, in that party's sole discretion, may determine whether Continuous Service will be considered interrupted in the case of (i) any leave of absence approved by the Board or chief executive officer, including sick leave, military leave or any other personal leave, or (ii) transfers between the Company, an Affiliate, or their successors. In addition, if required for exemption from or compliance with Section 409A of the Code, the determination of whether there has been a termination of Continuous Service will be made, and such term will be construed, in a manner that is consistent with the definition of "separation from service" as defined under Treasury Regulation Section 1.409A-1(h). A leave of absence will be treated as Continuous Service for purposes of vesting in a Stock Award only to such extent as may be provided in the Company's leave of absence policy, in the written terms of any leave of absence agreement or policy applicable to the Participant, or as otherwise required by law.

- (k) "Director" means a member of the Board. Directors are not eligible to receive Stock Awards under the Plan with respect to their service in such capacity.
- (l) "Disability" means the permanent and total disability of a person within the meaning of Section 22(e)(3) of the Code.
- (m) "Effective Date" means October 10, 2016.
- (n) "Employee" means any person employed by the Company or an Affiliate. However, service solely as a Director, or payment of a fee for such services, will not cause a Director to be considered an "Employee" for purposes of the Plan.
- (o) "Entity" means a corporation, partnership, limited liability company or other entity.
- (p) "Exchange Act" means the Securities Exchange Act of 1934, as amended, and the rules and regulations promulgated thereunder.
- (q) "Fair Market Value" means, as of any date, the value of the Common Stock determined as follows:
 - (i) If the Common Stock is listed on any established stock exchange or traded on any established market, the Fair Market Value of a share of Common Stock will be, unless otherwise determined by the Board, the closing sales price for such stock as quoted on such exchange or market (or the exchange or market with the greatest volume of trading in the Common Stock) on the last market trading day prior to the date of determination, as reported in a source the Board deems reliable.
 - (ii) Unless otherwise provided by the Board, if there is no closing sales price for the Common Stock on the date of determination, then the Fair Market Value will be the closing selling price on the last preceding date for which such quotation exists.

12

- (iii) In the absence of such markets for the Common Stock, the Fair Market Value will be determined by the Board in good faith and in a manner that complies with Section 409A of the Code.
- (r) "Independent Director" has the meaning set forth in Section 1(a) above.
- (s) "Non-Employee Director" means a Director who either (i) is not a current employee or officer of the Company or an Affiliate, does not receive compensation, either directly or indirectly, from the Company or an Affiliate for services rendered as a consultant or in any capacity other than as a Director (except for an amount as to which disclosure would not be required under Item 404(a) of Regulation S-K promulgated pursuant to the Securities Act ("Regulation S-K")), does not possess an interest in any other transaction for which disclosure would be required under Item 404(a) of Regulation S-K, and is not engaged in a business relationship for which disclosure would be required pursuant to Item 404(b) of Regulation S-K; or (ii) is otherwise considered a "non-employee director" for purposes of Rule 16b-3 of the Exchange Act.
- (t) "Nonstatutory Stock Option" means any option granted pursuant to Section 4(b) of the Plan that does not qualify as an "incentive stock option" within the meaning of Section 422 of the Code.
- (u) "Officer" means a person who is an officer of the Company within the meaning of Section 16 of the Exchange Act.
- (v) "Option" means a Nonstatutory Stock Option to purchase shares of Common Stock granted pursuant to the Plan.
- (w) "Option Agreement" means a written agreement between the Company and an Optionholder evidencing the terms and conditions of an Option grant. Each Option Agreement will be subject to the terms and conditions of the Plan.
- (x) "Optionholder" means a person to whom an Option is granted pursuant to the Plan or, if applicable, such other person who holds an outstanding Option.
- (y) "Participant" means a person to whom a Stock Award is granted pursuant to the Plan or, if applicable, such other person who holds an outstanding Stock Award.
- (z) "Plan" means thisRigel Pharmaceuticals, Inc. Inducement Plan, as it may be amended.
- (aa) "Restricted Stock Unit Award" means a right to receive shares of Common Stock which is granted pursuant to the terms and conditions of Section 6(b).
- (bb) "Restricted Stock Unit Award Agreement" means a written agreement between the Company and a holder of a Restricted Stock Unit Award evidencing the terms and conditions of a Restricted Stock Unit Award grant. Each Restricted Stock Unit Award Agreement will be subject to the terms and conditions of the Plan.

(cc) "Rule 16b-3" means Rule 16b-3 promulgated under the Exchange Act or any successor to Rule 16b-3, as in effect from time to time.

(dd) "Securities Act" means the Securities Act of 1933, as amended.

(ee) "Stock Award" means any right to receive Common Stock granted under the Plan, including an Option or a Restricted Stock Unit Award.

(ff) "Stock Award Agreement" means a written agreement between the Company and a Participant evidencing the terms and conditions of a Stock Award grant. Each Stock Award Agreement will be subject to the terms and conditions of the Plan.

CERTIFICATION

I, Raul R. Rodriguez, certify that:

1. I have reviewed this quarterly report on Form 10-Q of Rigel Pharmaceuticals, Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
 - a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
 - b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
 - c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
 - d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
 - a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
 - b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: **November 7, 2023** **May 7, 2024**

/s/ RAUL R. RODRIGUEZ

Raul R. Rodriguez
Chief Executive Officer

Exhibit 31.2

CERTIFICATION

I, Dean L. Schorno, certify that:

1. I have reviewed this quarterly report on Form 10-Q of Rigel Pharmaceuticals, Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
 - a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
 - b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
 - c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
 - d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
 - a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
 - b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: **November 7, 2023** **May 7, 2024**

/s/ DEAN L. SCHORNO

Dean L. Schorno

Chief Financial Officer

Exhibit 32.1

CERTIFICATIONS OF CHIEF EXECUTIVE OFFICER AND CHIEF FINANCIAL OFFICER
PURSUANT TO
18 U.S.C. SECTION 1350,
AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

Pursuant to the requirement set forth in Rule 13a-14(b) of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), and Section 1350 of Chapter 63 of Title 18 of the United States Code (18 U.S.C. §1350), Raul R. Rodriguez, Chief Executive Officer of Rigel Pharmaceuticals, Inc. (the "Company"), and Dean L. Schorno, Chief Financial Officer of the Company, each hereby certifies that, to the best of his knowledge:

1. The Company's Quarterly Report on Form 10-Q for the period ended **September 30, 2023** **March 31, 2024**, to which this Certification is attached as Exhibit 32.1 (the "Periodic Report"), fully complies with the requirements of Section 13(a) or Section 15(d) of the Exchange Act, and
2. The information contained in the Periodic Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

In Witness Whereof, the undersigned have set their hands hereto as of **November 7, 2023** **May 7, 2024**.

/s/ RAUL R. RODRIGUEZ

Raul R. Rodriguez
Chief Executive Officer

/s/ DEAN L. SCHORNO

Dean L. Schorno
Chief Financial Officer

This certification accompanies the Form 10-Q to which it relates, is not deemed filed with the Securities and Exchange Commission and is not to be incorporated by reference into any filing of Rigel Pharmaceuticals, Inc. under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended (whether made before or after the date of the Form 10-Q), irrespective of any general incorporation language contained in such filing.

DISCLAIMER

THE INFORMATION CONTAINED IN THE REFINITIV CORPORATE DISCLOSURES DELTA REPORT™ IS A COMPARISON OF TWO FINANCIALS PERIODIC REPORTS. THERE MAY BE MATERIAL ERRORS, OMISSIONS, OR INACCURACIES IN THE REPORT INCLUDING THE TEXT AND THE COMPARISON DATA AND TABLES. IN NO WAY DOES REFINITIV OR THE APPLICABLE COMPANY ASSUME ANY RESPONSIBILITY FOR ANY INVESTMENT OR OTHER DECISIONS MADE BASED UPON THE INFORMATION PROVIDED IN THIS REPORT. USERS ARE ADVISED TO REVIEW THE APPLICABLE COMPANY'S ACTUAL SEC FILINGS BEFORE MAKING ANY INVESTMENT OR OTHER DECISIONS.

©2024, Refinitiv. All rights reserved. Patents Pending.