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 June 30, 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: 001-38210

Krystal Biotech, Inc.

(Exact name of registrant as specified in its charter)

Delaware	**82-1080209**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification Number)**

2100 Wharton Street, Suite 701
Pittsburgh, Pennsylvania 15203
(Address of principal executive offices and zip code)

(412) 586-5830
(Registrant's telephone number, including area code)

N/A
(Former name, former address and former fiscal year, if changed since last report)

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

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock	KRYS	NASDAQ Global Select Market

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, 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 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 July 29, 2024, there were 28,729,950 shares of the registrant's common stock issued and outstanding.

Krystal Biotech, Inc.
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PART I. FINANCIAL INFORMATION

ITEM 1. FINANCIAL STATEMENTS

Krystal Biotech, Inc.
Condensed Consolidated Balance Sheets
(unaudited)

(in thousands, except par value)	June 30, 2024	December 31, 2023
Assets		
Current assets		
Cash and cash equivalents	$ 345,786	$ 358,328
Short-term investments	213,826	173,850
Accounts receivable, net	103,236	42,040
Inventory	12,179	6,985
Prepaid expenses and other current assets	7,745	6,706
Total current assets	682,772	587,909
Property and equipment, net	158,808	161,202
Long-term investments	69,292	61,954
Right-of-use assets	6,660	7,027
Other non-current assets	126	263
Total assets	$ 917,658	$ 818,355
Liabilities and Stockholders' Equity		
Current liabilities		
Accounts payable	$ 5,425	$ 4,132
Current portion of lease liability	1,361	1,474
Accrued rebates	21,733	5,977
Accrued expenses and other current liabilities	43,332	21,511
Total current liabilities	71,851	33,094
Lease liability	6,326	6,620
Other long-term liabilities	588	—
Total liabilities	78,765	39,714
Commitments and contingencies (see note 7)		
Stockholders' equity		
Common stock; $0.00001 par value; 80,000 shares authorized as of June 30, 2024 and December 31, 2023; 28,709 and 28,237 shares issued and outstanding as of June 30, 2024 and December 31, 2023, respectively.	—	—
Additional paid-in capital	1,092,854	1,047,830
Accumulated other comprehensive (loss) gain	(634)	638
Accumulated deficit	(253,327)	(269,827)
Total stockholders' equity	838,893	778,641
Total liabilities and stockholders' equity	$ 917,658	$ 818,355

The accompanying notes are an integral part of these condensed consolidated financial statements.

Krystal Biotech, Inc.
Condensed Consolidated Statements of Operations and Comprehensive Income (Loss)
(unaudited)

(in thousands, except per share data)	Three Months Ended June 30,		Six Months Ended June 30,	
	2024	**2023**	**2024**	**2023**
Product revenue, net	$ 70,284	$ —	$ 115,535	$ —
Expenses				
Cost of goods sold	6,009	—	8,428	—
Research and development	15,583	12,144	26,539	24,432
Selling, general and administrative	27,626	25,904	53,685	49,939
Litigation settlement	12,500	—	25,000	12,500
Total operating expenses	61,718	38,048	113,652	86,871
Income (loss) from operations	8,566	(38,048)	1,883	(86,871)
Other income				
Interest and other income, net	7,479	4,838	15,095	8,364
Income (loss) before income taxes	16,045	(33,210)	16,978	(78,507)
Income tax expense	(477)	—	(477)	—
Net income (loss)	15,568	(33,210)	16,501	(78,507)
Unrealized (loss) gain on available-for-sale securities and other	(335)	(82)	(1,272)	492
Comprehensive income (loss)	$ 15,233	$ (33,292)	$ 15,229	$ (78,015)
Net income (loss) per common share:				
Basic	$ 0.54	$ (1.25)	$ 0.58	$ (3.00)
Diluted	$ 0.53	$ (1.25)	$ 0.56	$ (3.00)
Weighted-average common shares outstanding:				
Basic	28,598	26,657	28,446	26,187
Diluted	29,637	26,657	29,504	26,187

The accompanying notes are an integral part of these condensed consolidated financial statements.

Krystal Biotech, Inc.
Condensed Consolidated Statements of Stockholders' Equity
(unaudited)

(in thousands)	Common Stock Shares	Common Stock Amount	Additional Paid-in Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders' Equity
Balances as of January 1, 2024	28,237	$ —	$ 1,047,830	$ 638	$ (269,827)	$ 778,641
Issuance of common stock upon exercise of stock options	260	—	15,969	—	—	15,969
Vesting of restricted stock units, net of shares withheld for taxes	39	—	(4,181)	—	—	(4,181)
Shares of restricted stock awards surrendered for taxes	(8)	—	(1,205)	—	—	(1,205)
Stock-based compensation	—	—	10,023	—	—	10,023
Unrealized (loss) on investments and other[1]	—	—	—	(937)	—	(937)
Net income	—	—	—	—	932	932
Balances at March 31, 2024	28,528	$ —	$ 1,068,436	$ (299)	$ (268,895)	$ 799,242
Issuance of common stock upon exercise of stock options	181	—	10,637	—	—	10,637
Stock-based compensation	—	—	13,781	—	—	13,781
Unrealized (loss) on investments and other[2]	—	—	—	(335)	—	(335)
Net income	—	—	—	—	15,568	15,568
Balances at June 30, 2024	28,709	$ —	$ 1,092,854	$ (634)	$ (253,327)	$ 838,893

(in thousands)	Common Stock Shares	Common Stock Amount	Additional Paid-in Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders' Equity
Balances as of January 1, 2023	25,764	$ —	$ 803,718	$ (728)	$ (280,759)	$ 522,231
Issuance of common stock upon exercise of stock options	42	—	2,208	—	—	2,208
Shares of restricted stock awards surrendered for taxes	(10)	—	(749)	—	—	(749)
Stock-based compensation	—	—	10,599	—	—	10,599
Unrealized gain on investments and other[1]	—	—	—	574	—	574
Net loss	—	—	—	—	(45,297)	(45,297)
Balances at March 31, 2023	25,796	$ —	$ 815,776	$ (154)	$ (326,056)	$ 489,566
Issuance of common stock in private placement offering, net of offering costs	1,730	—	159,951	—	—	159,951
Issuance of common stock upon exercise of stock options	449	—	25,446	—	—	25,446
Stock-based compensation	—	—	11,443	—	—	11,443
Unrealized (loss) on investments and other[2]	—	—	—	(82)	—	(82)
Net loss	—	—	—	—	(33,210)	(33,210)
Balances at June 30, 2023	27,975	$ —	$ 1,012,616	$ (236)	$ (359,266)	$ 653,114

(1) Includes foreign currency translation loss of $62 thousand and $35 thousand for the three months ended March 31, 2024 and 2023, respectively.

(2) Includes foreign currency translation loss of $83 thousand and gain of $57 thousand for the three months ended June 30, 2024 and 2023, respectively.

The accompanying notes are an integral part of these condensed consolidated financial statements.

Krystal Biotech, Inc.
Condensed Consolidated Statements of Cash Flows
(unaudited)

(in thousands)	Six Months Ended June 30, 2024	2023
Operating Activities		
Net income (loss)	$ 16,501	$ (78,507)
Adjustments to reconcile net income (loss) to net cash provided by (used in) operating activities		
Depreciation	3,262	2,348
Accretion on marketable securities	(1,104)	(1,046)
Amortization of operating lease right-of-use assets	368	441
Stock-based compensation expense, net	22,455	21,768
Realized gain on investments	(2,859)	(2,390)
Other, net	89	50
Changes in operating assets and liabilities		
Accounts receivable, net	(61,196)	—
Inventory	(1,708)	(906)
Prepaid expenses and other current assets	(2,532)	(649)
Other non-current assets	29	110
Lease liability	(406)	(380)
Other long-term liabilities	588	—
Accounts payable	1,511	481
Accrued expenses and other current liabilities	(4,034)	(1,666)
Accrued rebates	15,756	—
Accrued litigation settlement	25,000	—
Net cash provided by (used in) operating activities	11,720	(60,346)
Investing Activities		
Purchases of property and equipment	(2,391)	(8,171)
Purchases of investments	(201,736)	(319,969)
Maturities of investments	158,794	315,746
Net cash (used in) investing activities	(45,333)	(12,394)
Financing Activities		
Proceeds from issuance of common stock, net of offering costs	—	159,838
Proceeds from exercise of stock options	26,607	27,654
Taxes paid for employee tax withholding related to restricted stock units	(4,181)	—
Taxes paid related to settlement of restricted stock awards	(1,205)	(749)
Net cash provided by financing activities	21,221	186,743
Effect of exchange rate changes on cash and cash equivalents	(150)	(28)
Net (decrease) increase in cash and cash equivalents	(12,542)	113,975
Cash and cash equivalents at beginning of period	358,328	161,900
Cash and cash equivalents at end of period	$ 345,786	$ 275,875
Supplemental Disclosures of Non-Cash Investing Activities		
Unpaid purchases of property and equipment included in accounts payable and accrued expenses	$ 8,568	$ 10,998
Supplemental Cash Flow Information		
Income taxes paid	$ 2,002	$ —

The accompanying notes are an integral part of these condensed consolidated financial statements.

Krystal Biotech, Inc.
Notes to Condensed Consolidated Financial Statements
(unaudited)

1. Organization

Krystal Biotech, Inc. (the "Company," or "we" or other similar pronouns) commenced operations in April 2016. In March 2017, we converted from a California limited liability company to a Delaware C-corporation, and changed our name from Krystal Biotech LLC to Krystal Biotech, Inc. In June 2018, the Company incorporated a wholly-owned subsidiary in Australia for the purpose of undertaking preclinical and clinical studies in Australia. In April 2019, we incorporated Jeune Aesthetics, Inc. ("Jeune Aesthetics"), in Delaware, a wholly-owned subsidiary, for the purpose of undertaking preclinical and clinical studies for aesthetic skin conditions. In January 2022, August 2022, December 2022, August 2023 and March 2024, we incorporated wholly-owned subsidiaries in Switzerland, Netherlands, France, Germany and Japan, respectively, for the purpose of establishing initial operations in Europe and Japan for the commercialization of our product pipeline.

We are a fully integrated, commercial-stage biotechnology company focused on the discovery, development, manufacturing and commercialization of genetic medicines to treat diseases with high unmet medical needs. Using our patented gene therapy technology platform that is based on engineered herpes simplex virus-1 ("HSV-1"), we create vectors that efficiently deliver therapeutic transgenes to cells of interest in multiple organ systems. The cell's own machinery then transcribes and translates the transgene to treat the disease. Our vectors are amenable to formulation for non-invasive or minimally invasive routes of administration at a healthcare professional's office or in the patient's home by a healthcare professional. Our innovative technology platform is supported by two in-house, commercial scale Current Good Manufacturing Practice ("CGMP") manufacturing facilities.

Liquidity

As of June 30, 2024, the Company had an accumulated deficit of $ 253.3 million. Our operating profitability is dependent upon the continued successful commercialization of VYJUVEK, as well as successful development, approval, and commercialization of our other product candidates. Management intends to fund future operations through its on hand cash, cash equivalents and investments and revenue generated from the sale of VYJUVEK, and may also seek additional capital through the sale of equity, arrangements with strategic partners, debt financings or other sources. There can be no assurance that additional funding will be available on terms acceptable to the Company, if at all.

The Company is subject to risks common to companies in the biotechnology industry, including but not limited to the failure of product candidates in clinical and preclinical studies, the development of competing product candidates or other technological innovations by competitors, dependence on key personnel, protection of proprietary technology, compliance with government regulations and the ability to commercialize product candidates. The Company expects to incur significant costs to further its pipeline and to expand its commercialization capabilities in advance of the potential global regulatory approvals of VYJUVEK®, the Company's U.S. Food and Drug Administration (the "FDA") approved redosable gene therapy, for treating patients, six months of age or older, suffering from dystrophic epidermolysis bullosa, a rare and severe monogenic disease that affects the skin and mucosal tissues. The Company believes that its cash, cash equivalents and short-term investments of approximately $559.6 million as of June 30, 2024 will be sufficient to allow the Company to fund its planned operations for at least the next 12 months from the date of this Quarterly Report on Form 10-Q.

2. Summary of Significant Accounting Policies

Basis of Presentation

The accompanying interim condensed consolidated financial statements have been prepared in conformity with generally accepted accounting principles in the United States of America ("GAAP"). In the opinion of management, all adjustments, which consist of all normal recurring adjustments necessary for a fair presentation of the Company's financial position and results of operations for the interim periods presented, are reflected in the interim condensed consolidated financial statements. All intercompany balances and transactions have been eliminated in consolidation.

Certain prior period amounts have been reclassified to conform to the current period presentation. The reclassified amounts have no impact on the Company's previously reported financial position or results of operations.

The results of operations for the interim periods are not necessarily indicative of the results of operations to be expected for the full year. These unaudited interim condensed consolidated financial statements should be read in conjunction with the Company's audited consolidated financial statements and the notes thereto included in the Company's Annual Report on Form 10-K for the fiscal year ended December 31, 2023 ("2023 10-K"), as filed with the U.S. Securities and Exchange Commission ("SEC") on February 26, 2024.

Use of Estimates

The preparation of condensed consolidated financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts in the condensed consolidated financial statements and accompanying notes. Actual results could materially differ from those estimates. Management considers many factors in developing the estimates and assumptions that are used in the preparation of these financial statements. Management must apply significant judgment in this process. In addition, other factors may affect estimates, including expected business and operational changes, sensitivity and volatility associated with the assumptions used in developing estimates, and whether historical trends are expected to be representative of future trends. The estimation process often may yield a range of potentially reasonable estimates of the ultimate future outcomes and management must select an amount that falls within that range of reasonable estimates. If actual results in the future vary from the Company's estimates, the Company will adjust these estimates in the period these variances become known. Estimates are used in the following areas, among others: variable consideration associated with revenue recognition, stock-based compensation expense, accrued expenses, the fair value of financial instruments and the valuation allowance included in the deferred income tax calculation.

Concentration of Credit Risk and Off-Balance Sheet Risk

Financial instruments that subject the Company to credit risk primarily consist of cash and cash equivalents, short-term investments, long-term investments, and accounts receivable, net. The Company maintains its cash and cash equivalent balances with high-quality financial institutions and, consequently, the Company believes that such funds are subject to minimal credit risk. The Company is exposed to credit risk in the event of default by the financial institutions to the extent amounts recorded on the condensed consolidated balance sheets are in excess of insured limits. The Company has not experienced any credit losses in such accounts and does not believe it is exposed to any significant credit risk on these funds.

The Company's accounts receivable as of June 30, 2024 are primarily from one counterparty that distributes VYJUVEK in the U.S. on behalf of the Company. As of June 30, 2024, the credit profile for this counterparty was deemed to be in good standing and, as such, an allowance for credit losses was not recorded. For accounts receivable arising from named patient sales, the Company evaluates the creditworthiness of each counterparty on a regular basis. As of June 30, 2024, no allowance for credit losses was deemed necessary as a result of these counterparties.

For the six months ended June 30, 2024, the Company's counterparty distributed VYJUVEK within the U.S. to primarily one customer on behalf of the Company. No product revenue was recorded for the six months ended June 30, 2023.

The Company has no financial instruments with off-balance sheet risk of loss.

Summary of Significant Accounting Policies

See Note 2 to our consolidated financial statements included in our 2023 10-K. There were no material changes to the Company's significant accounting policies during the six months ended June 30, 2024.

Recent Accounting Pronouncements

There were no accounting pronouncements issued or adopted during the six months ended June 30, 2024 that are expected to have a material impact on the Company's condensed consolidated financial statements.

In December 2023, the Financial Accounting Standards Board ("FASB") issued Accounting Standards Update ("ASU") 2023-09 "Income Taxes (Topic 740): Improvements to Income Tax Disclosures." The purpose of this guidance is to enhance the transparency and usefulness of income tax disclosures and provide comprehensive income tax information, particularly in relation to rate reconciliation and income taxes paid in the U.S. and foreign jurisdictions. This new standard will be effective for fiscal years starting after December 15, 2024, with the option to apply it retrospectively. Early adoption is also allowed. Currently, the Company is assessing the potential impact of this guidance on its consolidated financial statement disclosures.

3. Revenue Recognition

Following FDA approval on May 19, 2023, the Company began commercial marketing and sales of VYJUVEK throughout the United States and began recognizing revenue in the third quarter of 2023.

The following table summarizes changes in allowances and discounts for the six months ended June 30, 2024:

(in thousands)	Rebates	Prompt Pay	Other Accruals	Total
Balance as of December 31, 2023	$ 5,977	$ 858	$ 279	$ 7,114
Provisions	18,132	4,008	521	22,661
Payments/Credits	(1,788)	(1,656)	(165)	(3,609)
Balance as of June 30, 2024	$ 22,321	$ 3,210	$ 635	$ 26,166

Rebates are included in accrued rebates and other long-term liabilities on the condensed consolidated balance sheets. Other long-term liabilities includes $588 thousand of long-term accrued rebates. Other accruals are included in accrued expenses and other current liabilities on the condensed consolidated balance sheets. Prompt pay is recorded as an allowance against accounts receivable, net on the condensed consolidated balance sheets. Provisions for rebates, prompt pay and other accruals are recorded as a reduction to product revenue, net on the condensed consolidated statements of operations and comprehensive income (loss).

4. Net Income (Loss) Per Share Attributable to Common Stockholders

Basic net income (loss) per share attributable to common stockholders is calculated by dividing net income (loss) attributable to common stockholders by the weighted-average shares outstanding during the period, without consideration for common stock equivalents. Diluted net income (loss) per share attributable to common stockholders is computed by dividing the net income (loss) by the weighted-average number of shares of common stock and common stock equivalents outstanding for the period. Common stock equivalents consist of common stock issuable upon (1) exercise of stock options and (2) vesting of restricted stock awards, restricted stock units and performance-based restricted stock units (collectively, "restricted stock").

For the three months ended June 30, 2024 and 2023, respectively, there were (1) 215 thousand and 3.2 million common stock equivalents outstanding in the form of stock options and (2) 2 thousand and 44 thousand in unvested restricted stock, that have each been excluded from the calculation of diluted net income (loss) per common share as their effect would be anti-dilutive.

For the six months ended June 30, 2024 and 2023, respectively, there were (1) 169 thousand and 3.2 million common stock equivalents outstanding in the form of stock options and (2) 1 thousand and 44 thousand in unvested restricted stock, that have each been excluded from the calculation of diluted net income (loss) per common share as their effect would be anti-dilutive.

	Three Months Ended June 30,		Six Months Ended June 30,	
(in thousands, except per share data)	**2024**	**2023**	**2024**	**2023**
Numerator:				
Net income (loss)	$ 15,568	$ (33,210)	$ 16,501	$ (78,507)
Denominator:				
Weighted-average basic common shares	28,598	26,657	28,446	26,187
Dilutive effect of stock options and unvested restricted stock	1,039	—	1,058	—
Weighted-average diluted common shares	29,637	26,657	29,504	26,187
Net income (loss) per common share—basic	$ 0.54	$ (1.25)	$ 0.58	$ (3.00)
Net income (loss) per common share—diluted	$ 0.53	$ (1.25)	$ 0.56	$ (3.00)

5. Fair Value Instruments

The following tables show the Company's cash, cash equivalents and available-for-sale securities by significant investment category as of June 30, 2024 and December 31, 2023:

	June 30, 2024						
(in thousands)	Amortized Cost	Gross Unrealized Gains	Gross Unrealized (Losses)	Aggregate Fair Value	Cash and Cash Equivalents	Short-term Marketable Securities[1]	Long-term Marketable Securities[2]
Level 1:							
Cash and cash equivalents	$ 345,786	$ —	$ —	$ 345,786	$ 345,786	$ —	$ —
Subtotal	345,786	—	—	345,786	345,786	—	—
Level 2:							
Commercial paper	37,193	3	(13)	37,183	—	37,183	—
Corporate bonds	96,553	38	(131)	96,460	—	62,778	33,682
U.S. government agency securities	149,722	28	(275)	149,475	—	113,865	35,610
Subtotal	283,468	69	(419)	283,118	—	213,826	69,292
Total	$ 629,254	$ 69	$ (419)	$ 628,904	$ 345,786	$ 213,826	$ 69,292

(1) The Company's short-term marketable securities mature in one year or less.

(2) The Company's long-term marketable securities mature between one and two years.

	December 31, 2023						
(in thousands)	Amortized Cost	Gross Unrealized Gains	Gross Unrealized (Losses)	Aggregate Fair Value	Cash and Cash Equivalents	Short-term Marketable Securities[1]	Long-term Marketable Securities[2]
Level 1:							
Cash and cash equivalents	$ 358,328	$ —	$ —	$ 358,328	$ 358,328	$ —	$ —
Subtotal	358,328	—	—	358,328	358,328	—	—
Level 2:							
Commercial paper	17,124	5	(1)	17,128	—	17,128	—
Corporate bonds	111,824	407	(27)	112,204	—	70,996	41,208
U.S. government agency securities	106,079	423	(30)	106,472	—	85,726	20,746
Subtotal	235,027	835	(58)	235,804	—	173,850	61,954
Total	$ 593,355	$ 835	$ (58)	$ 594,132	$ 358,328	$ 173,850	$ 61,954

(1) The Company's short-term marketable securities mature in one year or less.

(2) The Company's long-term marketable securities mature between one and two years.

6. Balance Sheet Components

Inventory

Inventory consisted of the following:

(in thousands)	June 30, 2024	December 31, 2023
Raw materials	$ 5,507	$ 3,154
Work-in-process	5,035	3,204
Finished goods	1,637	627
Inventory	$ 12,179	$ 6,985

Property and Equipment, Net

Property and equipment, net consisted of the following:

(in thousands)	June 30, 2024	December 31, 2023
Building and building improvements	$ 111,407	$ 111,180
Leasehold improvements	25,637	25,068
Manufacturing equipment	26,530	24,905
Construction in progress	6,020	7,291
Laboratory equipment	3,066	2,339
Computer equipment and software	1,932	1,614
Furniture and fixtures	1,645	1,632
Total property and equipment	176,237	174,029
Accumulated depreciation	(17,429)	(12,827)
Property and equipment, net	$ 158,808	$ 161,202

Depreciation expense was $1.8 million and $1.2 million for the three months ended June 30, 2024 and 2023, respectively, and $ 3.3 million and $2.3 million for the six months ended June 30, 2024 and 2023, respectively. Depreciation expense capitalized into inventory was $559 thousand and $72 thousand for the three months ended June 30, 2024 and 2023, respectively, and $1.4 million and $72 thousand for the six months ended June 30, 2024 and 2023, respectively.

In March 2023, the Company received the permanent occupancy permit for its second commercial scale CGMP facility, ASTRA, which allowed the Company to begin utilizing certain portions of the building. As a result, and as qualification of assets occurred through 2023 and the first half of 2024, the majority of assets relating to ASTRA were reclassified from construction in progress to leasehold improvements, manufacturing equipment, buildings and building improvements, furniture and fixtures, or computer equipment and software as it was determined that assets were ready for their intended use. As certain pieces of equipment are not yet qualified, the Company will continue to hold the remaining assets within construction in progress until qualification has been completed and the assets are ready for their intended use. Estimated remaining payments related to ASTRA were $8.0 million as of June 30, 2024 and are recorded in accounts payable and accrued expenses and other current liabilities on the condensed consolidated balance sheets.

Accrued Expenses and Other Current Liabilities

Accrued expenses and other current liabilities consisted of the following as of June 30, 2024 and December 31, 2023:

(in thousands)	June 30, 2024	December 31, 2023
Accrued litigation settlement	$ 25,000	$ —
Accrued payroll and benefits	5,321	8,778
Accrued construction-in-progress	5,148	5,182
Other current liabilities	2,500	1,876
Accrued professional fees	2,096	1,810
Accrued preclinical and clinical expenses	1,512	1,248
Accrued inventory	1,046	334
Accrued taxes	709	2,283
Total	$ 43,332	$ 21,511

7. Commitments and Contingencies

Agreements with Contract Manufacturing Organizations and Contract Research Organizations

The Company enters into various agreements in the normal course of business with Contract Research Organizations (“CROs”), Contract Manufacturing Organizations (“CMOs”) and other third parties for preclinical research studies, clinical trials and testing and manufacturing services. The agreements with CMOs primarily relate to the manufacturing of our sterile gel that is mixed with in-house produced vectors as part of the final drug product for VYJUVEK. Agreements with third parties may also include research and development consulting activities, clinical-trial agreements, testing of our clinical-stage, pre-commercial and commercial stage products and/or storage, packaging and labeling. The Company is obligated to make milestone payments under certain of these contracts. The Company may also be responsible for the payment of a monthly

service fee for project management services for the duration of any agreements. The estimated remaining commitments as of June 30, 2024 under these agreements was approximately $1.0 million. The Company has incurred research and development expenses under these agreements of $ 1.0 million and $2.5 million for the three and six months ended June 30, 2024 and $1.1 million and $3.1 million for the three and six months ended June 30, 2023.

Legal Proceedings

In May 2020, a complaint was filed against the Company in the United States District Court for the Western District of Pennsylvania by PeriphaGen, Inc. ("PeriphaGen") alleging breach of contract and misappropriation of trade secrets. On April 27, 2022, the Company and PeriphaGen entered into a final settlement agreement, and the Company paid PeriphaGen an upfront payment of $25.0 million on April 28, 2022 for: (i) the release of all claims in the litigation with PeriphaGen; (ii) the acquisition of certain PeriphaGen assets and (iii) the grant of a license by PeriphaGen for dermatological applications. In accordance with the settlement agreement, on June 15, 2023, the Company paid PeriphaGen an additional $12.5 million following the FDA's approval of VYJUVEK. The settlement agreement requires the Company to pay three additional $12.5 million contingent milestone payments upon reaching $100.0 million in cumulative sales, $200.0 million in cumulative sales and $300.0 million in cumulative sales.

On May 29, 2024, the parties entered into an amendment to the final settlement agreement ("Amendment") to clarify the definition of cumulative sales and modify the timing of the $12.5 million contingent milestone payment triggered by reaching $ 100.0 million in cumulative sales. As defined in the settlement agreement and clarified in the Amendment, cumulative sales means the total cumulative revenue from sales of the Company's products by the Company and its affiliates and licensees. The amendment modified the timing of the $12.5 million contingent milestone payment triggered by reaching $ 100.0 million in cumulative sales, such that $6.25 million is payable following the Company's filing of a Quarterly Report on Form 10-Q that reports $ 100.0 million in cumulative sales, and the remaining $6.25 million is payable within 120 days following the end of the fiscal year in which the initial $ 6.25 million is paid. There were no other revisions to the settlement agreement, and the contingent payments triggered upon reaching $200.0 million in cumulative sales and $300.0 million in cumulative sales continue to remain payable following the filing(s) by the Company of an Annual Report(s) on Form 10-K reporting $200.0 million in cumulative sales and $300.0 million in cumulative sales. If all milestones are achieved, the total consideration for settling the dispute, acquiring certain assets, and granting of a license from PeriphaGen will be $75.0 million, of which $37.5 million has been paid.

The Company recorded litigation settlement expense of $ 12.5 million and $25.0 million for the three and six months ended June 30, 2024, respectively, and zero and $12.5 million for the three and six months ended June 30, 2023, respectively, on the condensed consolidated statements of operations and comprehensive income (loss) in accordance with the settlement agreement and the Amendment. During the three months ended June 30, 2024, the Company reached cumulative sales of $100.0 million. Accordingly, following the filing of this Quarterly Report on Form 10-Q, the Company will make a $6.25 million milestone payment, which was fully accrued for in the first quarter of 2024. Also during the three months ended June 30, 2024, in accordance with ASC 450, "Contingencies", the Company determined that reaching $200.0 million in cumulative sales was probable, and recorded litigation settlement expense of $12.5 million relating to the milestone payment, which becomes payable following the filing of the Annual Report on Form 10-K that reports $200.0 million in cumulative sales. The Company previously recorded litigation settlement expense of $ 12.5 million for the six months ended June 30, 2023 following FDA approval of B-VEC. As of June 30, 2024, the Company has not recorded an accrual for the remaining contingent milestone payment of $12.5 million related to $300.0 million in cumulative sales.

8. Leases

As of June 30, 2024, future minimum commitments under the Company's operating leases with lease terms in excess of 12 months were as follows:

(in thousands)	Operating Leases
2024 (remaining six months)	$ 772
2025	1,277
2026	1,277
2027	1,300
2028	1,325
Thereafter	9,437
Future minimum operating lease payments	15,388
Less: Interest	(7,701)
Present value of lease liability	$ 7,687

As of June 30, 2024 and December 31, 2023, the Company's weighted-average remaining lease term for operating leases was 12.2 years and 12.3 years, respectively, and the Company's weighted-average discount rate for operating leases was 9.5% as of June 30, 2024 and December 31, 2023.

The components of the Company's lease expense are as follows:

	Three Months Ended June 30,		Six Months Ended June 30,	
(in thousands)	2024	2023	2024	2023
Lease cost:				
Operating lease expense	$ 346	$ 440	$ 645	$ 902
Variable lease expense	51	29	91	88
Total lease expense	$ 397	$ 469	$ 736	$ 990

9. Capitalization

ATM Program

On May 8, 2023, the Company entered into a sales agreement with Cowen and Company, LLC ("Cowen") with respect to an at-the-market equity offering program ("ATM Program"), under which the Company may issue and sell from time to time through Cowen, acting as agent and/or principal, shares of its common stock, par value $0.00001 per share ("Common Stock"), having an aggregate offering price up to $150.0 million ("Placement Shares").

The Placement Shares will be offered and sold pursuant to the Company's effective shelf registration statement on Form S-3 filed with the SEC on April 6, 2023, and a prospectus supplement relating to the Placement Shares that was filed with the SEC on May 8, 2023. During the six months ended June 30, 2024 and 2023, no shares of Common Stock were issued pursuant to the ATM Program, resulting in $ 150.0 million being available for issuance under the ATM Program.

2023 Private Placement Offering

On May 22, 2023 and May 23, 2023, the Company sold 1,720,100 and 9,629 shares of Common Stock, respectively, in a private placement to certain institutional investors at a price of $92.50 per share for aggregate net proceeds of $160.0 million. In addition, the Company entered into a Registration Rights Agreement with the investors ("Registration Rights Agreement") that required the Company to file a registration statement with the SEC within 60 days of the date of the Registration Rights Agreement registering the resale of the shares of Common Stock issued in the private placement. On July 18, 2023, the Company filed the resale registration statement on Form S-3ASR with the SEC, which became effective upon filing.

10. Stock-Based Compensation

In 2017, the Company adopted the 2017 IPO Stock Plan ("Plan"), which governs the issuance of equity awards to employees, certain non-employee consultants, and directors. Initially, the Company reserved 900 thousand shares for issuance under the Plan with an initial sublimit for incentive stock options of 900 thousand shares. On an annual basis, the amount of shares available for issuance under the Plan increases by an amount equal to four percent of the total outstanding shares as of the last day of the preceding calendar year. The sublimit of incentive stock options is not subject to the increase. The Company has historically granted stock options and restricted stock awards ("RSAs") to its employees. In February 2023, the Company

began issuing restricted stock units ("RSUs") and performance-based restricted stock units ("PSUs" and with RSUs commonly referred to collectively as "restricted stock units") to certain employees.

Shares remaining available for grant under the Plan were 2.4 million as of June 30, 2024.

Stock Options

The following table summarizes the Company's stock option activity for the six months ended June 30, 2024:

	Stock Options Outstanding	Weighted-Average Exercise Price	Weighted-Average Remaining Contractual Life *(in years)*	Aggregate Intrinsic Value[(1)] *(in thousands)*
Outstanding as of December 31, 2023	2,606,592	$ 66.39	7.9	$ 150,405
Granted	227,182	$ 160.67		
Exercised	(441,027)	$ 60.33		
Cancelled or forfeited	(321,134)	$ 68.85		
Outstanding as of June 30, 2024	2,071,613	$ 77.65	7.7	$ 219,580
Exercisable as of June 30, 2024	818,577	$ 62.85	6.9	$ 98,872

(1) Aggregate intrinsic value represents the difference between the closing stock price of our Common Stock on December 31, 2023 and June 30, 2024, respectively, and the exercise price of outstanding in-the-money options.

The following table summarizes the Company's stock option activity for the six months ended June 30, 2023:

	Stock Options Outstanding	Weighted-Average Exercise Price	Weighted-Average Remaining Contractual Life *(in years)*	Aggregate Intrinsic Value[(1)] *(in thousands)*
Outstanding as of December 31, 2022	3,582,181	$ 61.15	8.7	$ 64,880
Granted	389,280	$ 88.74		
Exercised	(490,995)	$ 56.41		
Cancelled or forfeited	(250,800)	$ 63.14		
Outstanding as of June 30, 2023	3,229,666	$ 65.04	8.4	$ 169,121
Exercisable as of June 30, 2023	778,737	$ 55.83	7.5	$ 47,952

(1) Aggregate intrinsic value represents the difference between the closing stock price of our Common Stock on December 31, 2022 and June 30, 2023, respectively, and the exercise price of outstanding in-the-money options.

The total intrinsic value (the amount by which the fair market value exceeds the exercise price) of stock options exercised was $ 19.8 million and $26.7 million during the three months ended June 30, 2024 and 2023, respectively, and $44.3 million and $27.9 million during the six months ended June 30, 2024 and 2023, respectively.

The weighted-average grant-date fair value per share of options granted to employees, non-employees, and directors was $ 112.05 and $72.95 during the three months ended June 30, 2024 and 2023, respectively, and $109.18 and $61.06 during the six months ended June 30, 2024 and 2023, respectively.

There was $63.6 million of unrecognized stock-based compensation expense related to employees', non-employees', and directors' options that is expected to be recognized over a weighted-average period of 2.5 years as of June 30, 2024.

Restricted Stock Awards

The following table summarizes the Company's RSA activity:

	Six Months Ended June 30,			
	2024		2023	
	Number of Shares	Weighted-Average Grant Date Fair Value	Number of Shares	Weighted-Average Grant Date Fair Value
Non-vested RSAs, beginning of period	44,400	$ 78.89	66,600	$ 78.89
Granted	—		—	
Vested	(14,523)	$ 78.89	(12,649)	$ 78.89
Surrendered for taxes	(7,677)	$ 78.89	(9,551)	$ 78.89
Non-vested RSAs, end of period	22,200	$ 78.89	44,400	$ 78.89

There was $1.2 million of unrecognized stock-based compensation expense related to employees' RSAs that is expected to be recognized over a weighted-average period of 8 months as of June 30, 2024.

Restricted Stock Units

The following table summarizes the Company's RSU activity:

	Six Months Ended June 30,			
	2024		2023	
	Number of Shares	Weighted-Average Grant Date Fair Value	Number of Shares	Weighted-Average Grant Date Fair Value
Non-vested RSUs, beginning of period	160,900	$ 81.91	—	
Granted	224,890	$ 159.59	186,900	$ 81.91
Vested	(40,075)	$ 81.91	—	
Forfeited	(29,314)	$ 103.31	(14,200)	$ 81.91
Non-vested RSUs, end of period	316,401	$ 135.14	172,700	$ 81.91

There was $39.0 million of unrecognized stock-based compensation expense related to employees' RSU awards that is expected to be recognized over a weighted-average period of 3.5 years as of June 30, 2024.

Performance-Based Restricted Stock Units

The following table summarizes the Company's PSU activity:

	Six Months Ended June 30,			
	2024		2023	
	Number of Shares	Weighted-Average Grant Date Fair Value	Number of Shares	Weighted-Average Grant Date Fair Value
Non-vested PSUs, beginning of period	50,000	$ 81.91	—	
Granted	112,500	$ 159.47	60,000	$ 81.91
Vested	(25,000)	$ 81.91	—	
Non-vested PSUs, end of period	137,500	$ 145.37	60,000	$ 81.91

PSUs vest ratably over two years based upon continued service through the vesting date and the achievement of specific regulatory and commercial performance criteria as determined by the Compensation Committee of the Company's Board of Directors. The performance criteria are to be completed by the end of the year in which the PSU awards were granted. As of the June 30, 2024, the Company estimated that 100% of the newly granted PSUs will be eligible to vest.

There was $16.3 million of unrecognized stock-based compensation expense related to employees' PSU awards that is expected to be recognized over a weighted-average period of 1.6 years as of June 30, 2024.

Stock-Based Compensation Expense, Net

The Company recorded stock-based compensation expense, net related to its stock options, RSAs, RSUs and PSUs in the condensed consolidated statements of operations and comprehensive income (loss) for the three and six months ended June 30, 2024 and 2023 as follows:

	Three Months Ended June 30,		Six Months Ended June 30,	
(in thousands)	**2024**	**2023**	**2024**	**2023**
Research and development	$ 2,772	$ 2,863	$ 4,640	$ 5,359
Selling, general and administrative	10,384	8,469	17,815	16,409
Total stock-based compensation	$ 13,156	$ 11,332	$ 22,455	$ 21,768

After the FDA approval of VYJUVEK on May 19, 2023, the Company began capitalizing stock-based compensation associated with the allocation of labor costs related to work performed to manufacture VYJUVEK. The Company capitalized stock-based compensation of $625 thousand and $113 thousand for the three months ended June 30, 2024 and 2023, respectively and $1.3 million and $112 thousand for the six months ended June 30, 2024 and 2023, respectively, into inventory.

Historically, the Company also capitalized the portion of stock-based compensation related to work performed on the construction of our manufacturing facilities. The Company capitalized stock-based compensation of zero for each the three months ended June 30, 2024 and 2023, respectively, and zero and $162 thousand for the six months ended June 30, 2024 and 2023, respectively, into property and equipment, net.

11. Income Taxes

The Company recorded an income tax provision of $477 thousand for the three and six months ended June 30, 2024. The tax provision for interim periods is calculated using an estimate of the annual effective tax rate, adjusted for discrete items. If there are any changes to the estimated annual tax rate, the Company will make a cumulative adjustment to the income tax provision in the period the change becomes known. The Company did not record an income tax provision for the three and six months ended June 30, 2023 as it generated sufficient tax losses, after consideration of discrete items, during each of the periods. The Company expects to maintain a full valuation allowance against its net deferred tax assets for the year.

12. Subsequent Events

The Company evaluates events or transactions that occur after the balance sheet date, but prior to the issuance of the financial statements, to identify matters that require recognition or disclosure. The Company concluded that no subsequent events have occurred, that would require recognition or disclosure in the condensed consolidated financial statements.

ITEM 2. MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

The following discussion and analysis of our financial condition and results of operations should be read together with the unaudited condensed consolidated financial statements and related notes included in Item 1 of Part I of this Quarterly Report on Form 10-Q and with the audited financial statements and the related notes included in our Annual Report on Form 10-K for the fiscal year ended December 31, 2023 ("2023 10-K"), as filed with the SEC on February 26, 2024.

SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q contains "forward-looking statements" within the meaning of Section 27A of the Securities Act of 1933, as amended, or the Securities Act, and Section 21E of the Securities Exchange Act of 1934, as amended, or the Exchange Act. Forward-looking statements include all statements that are not historical facts and can be identified by terms such as "anticipate," "believe," "continue," "could," "estimate," "expect," "intend," "may," "plan," "potential," "predict," "project," "seek," "should," "target," "will," "would," or similar expressions and the negatives of those terms. These statements relate to future events or to our future operating or financial performance and involve known and unknown risks, uncertainties and other factors that may cause our actual results, performance or achievements to be materially different from any future results, performances or achievements expressed or implied by the forward-looking statements. Some of such factors include, but are not limited to:

- the commercial success of VYJUVEK® (beremagene geperpavec-svdt), our U.S. Food and Drug Administration ("FDA") approved product for treating patients, six months of age or older, suffering from dystrophic epidermolysis bullosa ("DEB");
- the initiation, timing, cost, progress and results of our research and development activities, preclinical studies and clinical trials for our product candidates;
- the timing, scope or results of regulatory filings and approvals, including timing of final FDA and other regulatory approval of our product candidates;
- our ability to achieve certain accelerated or orphan drug designations from the FDA or other regulators;
- changes in our estimates regarding the potential market opportunity for VYJUVEK and our product candidates;
- increases in costs associated with our research and development programs for our product candidates;
- increases in our selling, general and administrative expenses;
- risks related to our ability to successfully develop and commercialize our product candidates;
- our ability to identify new product candidates;
- our ability to identify, recruit and retain key personnel;
- risks related to our marketing and manufacturing capabilities and strategy;
- our business model and strategic plans for our business, product candidates and technology;
- the rate and degree of market acceptance and clinical utility of our product candidates and gene therapy, in general;
- our competitive position and the success of competing therapies;
- our intellectual property position and our ability to protect and enforce our intellectual property;
- our ability to establish and maintain collaborations;
- our financial performance and our estimates regarding expenses, future revenue, capital requirements and needs for additional financing, as well as our ability to raise capital;
- our ability to successfully avoid or resolve any litigation, intellectual property or other claims, that may be brought against us;
- global economic conditions, including the recent rise in inflation and interest rates; and

- the impact of changes in laws and regulations.

Forward-looking statements are subject to a number of risks, uncertainties and assumptions, including those described in Item 1A of Part II of this Quarterly Report on Form 10-Q and other filings we make with the SEC from time to time. Moreover, we operate in a very competitive and rapidly changing environment, and new risks emerge from time to time. It is not possible for our management to predict all risks, nor can we assess the impact of all factors 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 we may make. In light of these risks, uncertainties and assumptions, the forward-looking events and circumstances discussed in this Quarterly Report on Form 10-Q may not occur and actual results could differ materially and adversely from those anticipated or implied in the forward-looking statements. Given these uncertainties, you should not place undue reliance on these forward-looking statements. You should read this Quarterly Report completely and with the understanding that our actual future results may be materially different from what we expect.

Forward-looking statements represent our management's beliefs and assumptions only as of the date of this Quarterly Report. Except as required by law, we assume no obligation to update these forward-looking statements publicly as a result of subsequent events, developments or otherwise, or to update the reasons actual results could differ materially from those anticipated in these forward-looking statements, even if new information becomes available in the future.

Throughout this Form 10-Q, unless the context requires otherwise, all references to "Krystal," "the Company," "we," "our," "us" or similar terms refer to Krystal Biotech, Inc., together with its consolidated subsidiaries. Web links throughout this document are provided for convenience only and are not intended to be active hyperlinks to the referenced websites. No content on the referenced websites shall be deemed incorporated by reference into this Quarterly Report on Form 10-Q.

Overview

We are a fully integrated, commercial-stage biotechnology company focused on the discovery, development, manufacturing and commercialization of genetic medicines to treat diseases with high unmet medical needs. Using our patented gene therapy technology platform that is based on engineered herpes simplex virus-1 ("HSV-1"), we create vectors that efficiently deliver therapeutic transgenes to cells of interest in multiple organ systems. The cell's own machinery then transcribes and translates the transgene to treat the disease. Our vectors are amenable to formulation for non-invasive or minimally invasive routes of administration at a healthcare professional's office or in the patient's home by a healthcare professional. Our innovative technology platform is supported by two in-house, commercial scale Current Good Manufacturing Practice ("CGMP") manufacturing facilities.

Our FDA Approved Commercial Product

VYJUVEK (beremagene geperpavec-svdt, or B-VEC; referred to as B-VEC outside the U.S.)

On May 19, 2023, the FDA approved VYJUVEK, the first ever redosable gene therapy, for treating patients, six months of age or older, suffering from DEB, a rare and severe monogenic disease that affects the skin and mucosal tissues and is caused by one or more mutations in a gene called *COL7A1*. VYJUVEK is a redosable topical gel containing our novel vector designed to deliver two copies of the *COL7A1* transgene to a patient's skin cells to produce the COL7 protein. VYJUVEK is the first and only corrective medicine approved by the FDA for the treatment of DEB, both recessive and dominant, that can be administered by a healthcare professional ("HCP") in either a clinical setting or in the home. We possess exclusive rights to develop, manufacture, and commercialize VYJUVEK and all our pipeline candidates throughout the world.

We launched VYJUVEK in the United States in the second quarter of 2023. Net VYJUVEK product revenue was $70.3 million for the three months ended June 30, 2024, and $166.2 million in cumulative net product revenue since launch in August 2023.

Gross margin for the three months ended June 30, 2024 was 91%. We define gross margin as product revenue, net less cost of goods sold expressed as a percentage of product revenue, net.

We have made steady progress securing access and reimbursement for VYJUVEK since launch and as of July 2024, positive access determinations have been achieved for 97% of lives covered under commercial and Medicaid plans.

As of July 2024, we have secured over 400 reimbursement approvals for VYJUVEK in the U.S.

We seek to make the experience of starting and continuing on VYJUVEK treatment seamless for the patient. Since launch, infrastructure has been in place for patients to be treated in their homes by an HCP, reducing the need for regular visits to a clinic or hospital. Krystal Connect™, our U.S. in-house patient services call center, has been active since FDA approval and assists patients, care givers and HCPs interested in accessing VYJUVEK. We also continue to offer no-cost genetic testing through our DecodeDEB program. Since launch and through the second quarter of 2024, patient compliance with once weekly treatment while on VYJUVEK remains high at 90%.

Preparations and infrastructure buildout are underway in Europe and Japan to support our planned direct commercial launch in these regions. In June 2024, the President and General Manager of Krystal Biotech Japan G.K. joined the Company in preparation for a Japanese launch.

In October 2023, we submitted a Marketing Authorization Application ("MAA") to the European Medicines Agency ("EMA") for B-VEC for the treatment of DEB. In November 2023, we were notified that the MAA had been validated and was now under Committee for Medicinal Products for Human Use review. In February 2024, the EMA completed inspection of our manufacturing facility as part of the MAA review process and, in May 2024, good manufacturing practices certification was granted by the EMA. We expect an EMA decision on our MAA in the second half of 2024.

In July 2023, the Pharmaceuticals and Medical Devices Agency ("PMDA") in Japan officially accepted the open label extension ("OLE") study of B-VEC. The efficacy portion of the Japan OLE study was completed in April 2024 and results closely mirrored those of our Phase 3 study in the U.S. A total of 5 patients were enrolled, with one patient discontinuing after 8 weeks due to scheduling challenges. B-VEC was well tolerated in the Japanese study population, with a safety profile consistent with previous studies, and all four patients that completed the study achieved the primary endpoint of complete wound closure at 6 months. We anticipate filing our Japan New Drug Application with Japan's PMDA in the second half of 2024, enabling a potential authorization by PMDA in 2025.

Pipeline Highlights and Recent Developments

Respiratory

KB407 is an inhaled (nebulized) formulation of our novel vector designed to deliver two copies of the full-length cystic fibrosis transmembrane conductance regulator ("CFTR") transgene for the treatment of cystic fibrosis ("CF"), a serious rare lung disease caused by missing or mutated CFTR protein. In July 2023, we announced that we had dosed the first patient in CORAL-1, a Phase 1 multi-center, dose-escalation study evaluating KB407, delivered via a nebulizer, in patients with CF, regardless of their underlying genotype. In May 2024, we cleared the safety evaluation window for the second cohort of CORAL-1, and we expect to initiate the third and final cohort in the second half of 2024. Details of the Phase 1 study can be found at www.clinicaltrials.gov under NCT identifier NCT05504837.

We presented preclinical data at the American Thoracic Society 2024 International Conference held in May 2024 demonstrating KB407 transduction of fully differentiated, patient airway epithelial cell-derived apical out airway organoids leading to production of full-length and fully glycosylated CFTR.

KB408 is an inhaled (nebulized) formulation of our novel vector designed to deliver two copies of the *SERPINA1* transgene, that encodes for normal human alpha-1 antitrypsin protein, for the treatment of alpha-1 antitrypsin deficiency ("AATD"), a serious rare lung disease. In February 2024, we dosed the first patient in SERPENTINE-1, a Phase 1, open-label, single dose escalation study evaluating KB408, delivered via a nebulizer, in adult patients with AATD with a Pi*ZZ or a Pi*ZNull genotype. In May 2024, we cleared the safety evaluation window for the first cohort of SERPENTINE-1 and enrollment in the second cohort is ongoing. We are working closely with the Alpha-1 Foundation and their Therapeutic Development Network on the SERPENTINE-1 study and intend to announce interim data from the study in the fourth quarter of 2024. Details of the Phase 1 study can be found at www.clinicaltrials.gov under NCT identifier: NCT06049082. An overview of KB408 IND-enabling studies conducted to support the initiation of SERPENTINE-1 was presented at the American Thoracic Society 2024 International Conference held in May 2024.

Ophthalmology

In April 2023, we announced clinical data on the compassionate use of B-VEC, administered as an eyedrop, to treat a patient suffering from ocular complications of DEB. Data was first presented at the Association for Research in Vision and Ophthalmology ("ARVO") 2023 Annual Meeting and subsequently published in the New England Journal of Medicine in February 2024. Regular application of B-VEC to the eye was well tolerated and associated with full corneal healing at 3 months and visual acuity improvement from hand motion to 20/25 by 8 months.

Based on this early clinical evidence of safety and potential benefit under compassionate use, we started discussions with the FDA in the first quarter of 2024 on a potential clinical development path for ophthalmic B-VEC, and in February 2024, we aligned with the FDA on our proposed single arm, open label study in approximately 10 to 15 patients to enable approval of B-VEC eyedrops to treat ocular complications which are thought to affect over 25% of DEB patients. We plan to initiate the registrational study in the fourth quarter of 2024.

In August 2024, we initiated a natural history study to prospectively collect data on the frequency and severity of corneal abrasions in patients with DEB and serve as a run-in period for patients who may be eligible to participate in the registrational study.

We are actively evaluating multiple, preclinical-stage genetic medicine candidates for the treatment of front and back of the eye diseases and presented preclinical data highlighting the potential of Krystal's HSV-1-based gene delivery platform for back of the eye gene delivery at the ARVO 2024 Annual Meeting that was held in May 2024.

Oncology

KB707 is a redosable, immunotherapy designed to deliver genes encoding both human interleukin-2 ("IL-2") and interleukin-12 ("IL-12") to the tumor microenvironment and promote systemic immune-mediated tumor clearance. Two formulations of KB707 are in development, a solution formulation for transcutaneous injection and an inhaled (nebulized) formulation for lung delivery. Both formulations of KB707 have been granted Fast Track Designation by the FDA and in May 2024, intratumoral KB707 was also granted Rare Pediatric Disease Designation by the FDA for the treatment of osteosarcoma.

In October 2023, we dosed the first patient in OPAL-1, an open-label, multi-center, monotherapy, dose escalation and expansion Phase 1 study, evaluating intratumoral KB707 in patients with locally advanced or metastatic solid tumors, who relapsed or are refractory to standard of care, with at least one measurable and injectable tumor accessible by transcutaneous route of administration. In May 2024, we cleared the safety evaluation window for our third and final dose escalation cohort of the OPAL-1 study. Enrollment in the dose expansion cohort is ongoing. Details of the Phase 1 study can be found at www.clinicaltrials.gov under NCT identifier NCT05970497. Based on the current pace of enrollment, we expect to report interim data in the fourth quarter of 2024.

In April 2024, we dosed the first patient in KYANITE-1, an open-label, monotherapy, dose escalation and expansion Phase 1 study, evaluating inhaled KB707 in patients with locally advanced or metastatic solid tumors of the lung. The safety evaluation window for the first dose escalation cohort of the KYANITE-1 study was cleared in June 2024 and enrollment of the second cohort is ongoing. Details of the Phase 1 study can be found at www.clinicaltrials.gov under NCT identifier NCT06228326.

We presented preclinical efficacy data generated in syngeneic mouse models using murine equivalents to KB707 at the American Association for Cancer Research Annual Meeting that was held in April 2024. Study results demonstrated that IL-12 and IL-2, delivered intratumorally using Krystal's HSV-1-based gene delivery platform, enhanced local and systemic T-cell effector responses consistent with previously reported anti-tumor activity.

Dermatology

KB105 is a topical gel containing our novel vector designed to deliver two copies of the *TGM1* transgene encoding the human enzyme transglutaminase-1 ("TGM1") for the treatment of lamellar icthyosis, a serious rare skin disorder most often caused by missing or mutated TGM1 protein. KB104 is a topical gel formulation of our novel vector designed to deliver two copies of the *SPINK5* transgene for the treatment of Netherton Syndrome, a debilitating autosomal recessive skin disorder caused by missing or mutated SPINK5 protein. We expect to resume enrollment in the Phase 2 portion of JADE-1, a randomized, placebo-controlled Phase 1/2 study evaluating KB105 for the treatment of lamellar icthyosis in the first half of 2025, and plan to file an investigational new drug ("IND") application and initiate a clinical trial of KB104 to treat patients with Netherton Syndrome following initiation of the KB105 Phase 2 study. Details of the JADE-1 Phase 1/2 study can be found at www.clinicaltrials.gov under NCT identifier NCT04047732.

Aesthetics

In addition to focusing on genetic medicines to treat patients with diseases with high unmet medical needs, we are leveraging the ability of our platform to deliver proteins of interest to cells in the skin in the context of aesthetic medicine via our wholly-owned subsidiary, Jeune Aesthetics, Inc. ("Jeune"). KB301 is a solution formulation of our novel vector for intradermal injection designed to deliver two copies of the *COL3A1* transgene to address signs of aging or damaged skin caused by declining levels of, or damaged proteins within the extracellular matrix, including type III collagen. In April 2023, Jeune initiated and treated the first subject in the Phase 1 PEARL-1 Cohort 3 clinical study. The PEARL-1 Cohort 3 study is an open label study to evaluate different doses of KB301 for the improvement of lateral canthal lines at rest in up to 20 subjects. In January 2024, Jeune initiated the PEARL-1 Cohort 4 clinical study, an open label study to evaluate KB301 for the improvement of dynamic wrinkles of the décolleté in up to 20 subjects. Cohorts 3 and 4 were fully enrolled in April 2024 and Jeune plans to announce results for both cohorts in the third quarter of 2024. Following completion of both cohorts, Jeune plans to initiate a Phase 2 study of KB301. Details of the Phase 1 study can be found at www.clinicaltrials.gov under NCT identifier NCT04540900. Jeune has several other aesthetic medicine product candidates in various stages of preclinical development.

Financial Overview

Product Revenue, Net

After FDA approval of VYJUVEK in May 2023, we began commercial marketing and sales throughout the United States and began recognizing revenue during the third quarter of 2023. Our future revenue will fluctuate from quarter to quarter for many reasons, including the uncertain timing and amount of any such sales.

We have contracted to sell VYJUVEK to a limited number of specialty pharmacy providers (“SPs”) that mix the medication and administer it to patients in the patient’s home by a healthcare professional and through a specialty distributor (“SD”) to hospitals and outpatient clinics where patients are administered the medication at a healthcare professional’s office. The transaction price that we recognize as revenue for VYJUVEK sales includes an estimate of variable consideration, which includes discounts, returns, copay assistance and rebates that are offered within contracts. Refer to Note 3 of the notes to condensed consolidated financial statements included in this Form 10-Q and Note 2 of our consolidated financial statements in our 2023 10-K for additional information.

Cost of Goods Sold

Cost of goods sold includes direct and indirect costs related to the manufacturing of VYJUVEK. These costs consist of manufacturing costs, personnel costs including stock-based compensation, facility costs, and other indirect overhead costs. Cost of goods sold may also include period costs related to certain manufacturing services and inventory adjustment charges.

Prior to receiving FDA approval in May 2023, costs associated with the manufacturing of VYJUVEK were expensed as research and development expenses.

Research and Development Expenses

Research and development expenses consist primarily of costs incurred to advance our preclinical and clinical candidates, which include:

- expenses incurred under agreements with contract manufacturing organizations, contract research organizations, consultants and other vendors that conduct our preclinical activities;
- costs of acquiring, developing and manufacturing clinical trial materials and lab supplies;
- facility costs, depreciation and other expenses, which include direct expenses for rent and maintenance of facilities and other supplies; and
- payroll related expenses, including stock-based compensation expense.

We expense internal research and development costs to operations as incurred. We expense third-party costs for research and development activities, such as the manufacturing of preclinical and clinical materials, based on an evaluation of the progress to completion of specific tasks such as manufacturing of drug substance, fill/finish and stability testing, which is provided to us by our vendors.

We expect our research and development expenses will increase as we continue the manufacturing of preclinical and clinical materials and manage the clinical trials of, and seek regulatory approval for, our product candidates and as we expand our product portfolio. In the near term, we expect that our research and development expenses will increase as we continue our Phase 1 trials for KB407, KB408, intratumoral KB707, and inhaled KB707, resume dosing with KB105 in our Phase 1/2 clinical trial, complete the Phase 1, Cohorts 3 and 4 study and initiate a Phase 2 trial for KB301, begin our open label study evaluating ophthalmic B-VEC, and incur preclinical expenses for our other product candidates. Due to the numerous risks and uncertainties associated with product development, we cannot determine with certainty the duration, costs and timing of clinical trials, and, as a result, the actual costs to complete clinical trials may exceed the expected costs.

Selling, General and Administrative Expenses

Selling, general and administrative expenses consist principally of salaries and other related costs, including stock-based compensation for personnel in our executive, finance, legal, commercial, business development, information technology and other general and administrative functions. Selling, general and administrative expenses also include professional fees associated with corporate and intellectual property-related legal expenses, consulting and accounting services, insurance, facility-related costs and expenses associated with obtaining and maintaining patents. Other selling, general and administrative costs include travel expenses, patient access program costs, management service fees, marketing expenses, and selling expenses which include transportation, shipping and handling fees.

We anticipate that our selling, general and administrative expenses will increase in the future relating to our commercialization efforts and to support the development of our product candidates. These increases will likely include increased costs for insurance, costs related to the hiring of additional personnel and payments to outside consultants, lawyers and accountants, among other expenses. Additionally, we anticipate that we will continue to increase our salary and personnel costs and other expenses to support B-VEC commercialization globally.

ASTRA Capital Expenditures

In March 2021, we closed on the purchase of the building that was constructed to house our second commercial scale CGMP facility, ASTRA. In March 2023, we received the permanent occupancy permit for ASTRA which allowed the Company to begin utilizing certain parts of the building for research and development operations once qualification was

completed and a portion of the assets were placed into service throughout 2023. We incurred significant capital expenditures related to the construction of ASTRA in 2023 and expect to continue to incur capital expenditures related to ASTRA throughout the operational life of the facility.

Interest and Other Income, Net

Interest and other income, net consists primarily of income earned from our cash, cash equivalents and investments.

Critical Accounting Policies, Significant Judgments and Estimates

There have been no significant changes during the six months ended June 30, 2024 to our critical accounting policies, significant judgments and estimates as disclosed in our management's discussion and analysis of financial condition and results of operations included in our 2023 10-K.

Results of Operations

Our management's discussion and analysis of our financial position and results of operations is based on our financial statements, which have been prepared in accordance with U.S. generally accepted accounting principles, or GAAP. The preparation of financial statements in conformity with GAAP requires us to make estimates and assumptions that affect the amounts reported in the financial statements and accompanying notes.

Three Months Ended June 30, 2024 and 2023

	Three Months Ended June 30,		
	2024	2023	Change
(in thousands)	(unaudited)		
Product revenue, net	$ 70,284	$ —	$ 70,284
Expenses			
Cost of goods sold	6,009	—	6,009
Research and development	15,583	12,144	3,439
Selling, general and administrative	27,626	25,904	1,722
Litigation settlement	12,500	—	12,500
Total operating expenses	61,718	38,048	23,670
Income (loss) from operations	8,566	(38,048)	46,614
Other income			
Interest and other income, net	7,479	4,838	2,641
Income (loss) before income taxes	16,045	(33,210)	49,255
Income tax expense	(477)	—	(477)
Net income (loss)	$ 15,568	$ (33,210)	$ 48,778

Product Revenue, Net

Product revenue, net was $70.3 million for the three months ended June 30, 2024, as compared to zero for the three months ended June 30, 2023 due to sales of VYJUVEK after FDA approval was obtained on May 19, 2023. As VYJUVEK initial product sales revenue did not begin until the third quarter of 2023, there was no comparative period revenue.

Cost of Goods Sold

Cost of goods sold was $6.0 million for the three months ended June 30, 2024, as compared to zero for the three months ended June 30, 2023 due to sales of VYJUVEK after FDA approval was obtained on May 19, 2023. Prior to receiving FDA approval for VYJUVEK in May 2023, costs associated with the manufacturing of VYJUVEK were expensed as research and development expense.

Research and Development Expenses

Research and development expenses increased $3.4 million in the three months ended June 30, 2024 compared to the three months ended June 30, 2023. The increase was primarily driven by the following:

- an increase in manufacturing expenses of $2.0 million related to our product candidates, including on-going manufacturing efficiency and process optimization costs for which such processes have not yet been approved by the FDA,

- an increase of $820 thousand in clinical development costs,
- an increase in depreciation of $621 thousand due to the Company's second CGMP facility being placed into service in 2023 partially offset by the capitalization of depreciation associated with commercial batches of VYJUVEK after FDA approval in May 2023 and
- an increase in other research and development expenses of $678 thousand primarily relating to licensing and regulatory costs.

The increases were partially offset by:

- a decrease of $407 thousand due to the capitalization of allocated overhead costs for commercial batches of VYJUVEK after FDA approval in May 2023 and
- a net decrease in direct manufacturing expenses of $269 thousand primarily due to the capitalization of costs to manufacture VYJUVEK into inventory following FDA approval.

Research and development expenses consist primarily of costs relating to our preclinical development, the development of our product candidates and our clinical trial programs. Direct research and development expenses associated with our product candidates or development programs consist of compensation related expenses for our internal resources conducting research and development activities, fees paid to external consultants, contract research organizations, or for costs to support our clinical trials. Indirect research and development expenses that are allocated to our product candidates or programs consist of lab supplies and software fees. A significant portion of our research and development expenses are not allocated to individual product candidates and preclinical programs, as certain expenses benefit multiple product candidates and preclinical programs. For example, we do not allocate costs associated with stock-based compensation, manufacturing of preclinical or clinical development products or costs relating to facilities and equipment to individual product candidates and preclinical programs.

The following table summarizes our research and development expenses by product candidate or program, and for unallocated expenses, by type, for the three months ended June 30, 2024 and 2023.

	Three Months Ended June 30,		
	2024	2023	Change
(in thousands)	(unaudited)		
B-VEC	$ 3,594	$ 2,130	$ 1,464
KB407	713	442	271
KB408	249	309	(60)
KB301	223	77	146
KB707	1,770	943	827
Ophthalmology programs	612	—	612
Other dermatology programs	21	16	5
Other aesthetics programs	823	1	822
Other research programs	393	131	262
Other development programs	193	194	(1)
Stock-based compensation	2,772	2,863	(91)
Other unallocated manufacturing expenses[(1)]	2,288	3,536	(1,248)
Other unallocated expenses[(2)]	1,932	1,502	430
Research and development expense	$ 15,583	$ 12,144	$ 3,439

(1) Unallocated manufacturing expenses consist of shared pre-commercial manufacturing costs, primarily relating to raw materials, contract manufacturing, contract testing, process development, quality control and quality assurance activities and other manufacturing costs which support the development of multiple product candidates in our preclinical and clinical development programs.

(2) Other unallocated expenses include rental, storage, depreciation, and other facility related costs that we do not allocate to our individual product candidates.

The primary changes in our research and development expenses by product candidate or program in the three months ended June 30, 2024 compared to the three months ended June 30, 2023 are as follows:

- a net increase in B-VEC costs of $1.5 million largely due to the following:

- on-going manufacturing efficiency and process optimization costs for which such processes have not yet been approved by the FDA,
- an increase in overseas preclinical and clinical trial costs and
- overseas licensing and regulatory costs;
- partially offset by costs being to research and development expense prior to receiving FDA approval in May 2023 that are now included as part of the cost of inventory,

- an increase in KB707 costs of $827 thousand following the expansion of our research and development pipeline to oncology consisting of an increase in payroll related costs to support our research and an increase in contract research expenses in preparation for the Phase 1 clinical trial of inhaled KB707 that has now commenced,
- an increase in other aesthetics programs of $822 thousand,
- an increase in ophthalmology programs of $612 thousand, inclusive of $214 thousand for ophthalmic B-VEC and
- an increase in other unallocated expenses of $430 thousand, which largely relates to depreciation due to the Company's second CGMP facility being placed into service in 2023 partially offset by the capitalization of depreciation associated with commercial batches of VYJUVEK after its approval in May 2023.

The increases were partially offset by:

- a decrease in other unallocated manufacturing expenses of $1.2 million primarily due to costs related to the manufacturing of VYJUVEK being recorded as inventory and cost of goods sold following FDA approval.

Selling, General and Administrative Expenses

Selling, general and administrative expenses increased $1.7 million in the three months ended June 30, 2024 as compared to the three months ended June 30, 2023. The increase was primarily driven by the following:

- an increase in stock-based compensation of $1.9 million,
- an increase related to professional services incurred to support our commercial growth of $938 thousand and
- an increase in selling expenses related to the launch of VYJUVEK of $909 thousand, which includes $184 thousand related to our patient access program.

The increases were partially offset by:

- a decrease in marketing costs of $1.5 million due to the timing of marketing activities ahead of the VYJUVEK launch and
- a decrease in information technology infrastructure costs of $355 thousand.

Litigation Settlement

Litigation settlement for the three months ended June 30, 2024 and 2023 was $12.5 million and zero, respectively, and consisted of amounts related to the settlement of litigation with PeriphaGen. See “Legal Proceedings” in Note 7 of the notes to condensed consolidated financial statements included in this Form 10-Q for more information.

Interest and Other Income, Net

Interest and other income, net for the three months ended June 30, 2024 and 2023 was $7.5 million and $4.8 million, respectively, and consisted of interest and dividend income earned from our cash, cash equivalents and investments. The increase in interest and dividend income is the result of increased investment activity and more favorable interest rates as compared to the prior period and an increase in our balance of cash, cash equivalents and investments.

Income Tax Expense

Income tax expense for the three months ended June 30, 2024 and 2023 was $477 thousand and zero, respectively.

Six Months Ended June 30, 2024 and 2023

	Six Months Ended June 30,		
	2024	2023	Change
(in thousands)	(unaudited)		
Product revenue, net	$ 115,535	$ —	$ 115,535
Expenses			
Cost of goods sold	8,428	—	8,428
Research and development	26,539	24,432	2,107
Selling, general and administrative	53,685	49,939	3,746
Litigation settlement	25,000	12,500	12,500
Total operating expenses	113,652	86,871	26,781
Income (loss) from operations	1,883	(86,871)	88,754
Other income			
Interest and other income, net	15,095	8,364	6,731
Income (loss) before income taxes	16,978	(78,507)	95,485
Income tax expense	(477)	—	(477)
Net income (loss)	$ 16,501	$ (78,507)	$ 95,008

Products Revenue, net

Product revenue, net was $115.5 million for the six months ended June 30, 2024 as compared to zero for the six months ended June 30, 2023 due to initial sales of VYJUVEK after FDA approval was obtained on May 19, 2023. As VYJUVEK was approved by the FDA in May 2023, there were no comparative period revenue.

Cost of Goods Sold

Cost of goods sold was $8.4 million for the six months ended June 30, 2024 as compared to zero for the six months ended June 30, 2023 due to initial sales of VYJUVEK after FDA approval was obtained on May 19, 2023. Prior to receiving FDA approval for VYJUVEK in May 2023, costs associated with the manufacturing of VYJUVEK were expensed as research and development expense.

Research and Development Expenses

Research and development expenses increased $2.1 million in the six months ended June 30, 2024 compared to the six months ended June 30, 2023. The increase was primarily driven by the following:

- an increase in manufacturing expenses of $1.8 million related to our product candidates,
- an increase in clinical development costs of $983 thousand,
- an increase in depreciation of $893 thousand due to the Company's second CGMP facility being placed into service in 2023 partially offset by the capitalization of depreciation associated with commercial batches of VYJUVEK after FDA approval in May 2023 and
- an increase in other research and development expenses of $1.0 million primarily relating to licensing and regulatory costs.

The increases were partially offset by:

- a decrease of $1.3 million due to the capitalization of allocated overhead costs for commercial batches of VYJUVEK after FDA approval in May 2023 partially offset by increased payroll related expenses, including stock-based compensation, primarily driven by an increase in headcount to support overall growth and
- a net decrease in direct manufacturing expenses of $1.2 million due to the capitalization of costs to manufacture VYJUVEK into inventory following FDA approval.

The following table summarizes our research and development expenses by product candidate or program, and for unallocated expenses, by type, for the six months ended June 30, 2024 and 2023:

	Six Months Ended June 30,		
	2024	2023	Change
(in thousands)	(unaudited)		
B-VEC	$ 5,815	$ 4,520	$ 1,295
KB407	1,451	819	632
KB408	495	419	76
KB301	383	329	54
KB707	3,156	1,408	1,748
Ophthalmology programs	712	—	712
Other dermatology programs	35	257	(222)
Other aesthetics programs	958	14	944
Other research programs	643	258	385
Other development programs	425	529	(104)
Stock-based compensation	4,640	5,359	(719)
Other unallocated manufacturing expenses[(1)]	4,419	7,456	(3,037)
Other unallocated expenses[(2)]	3,407	3,064	343
Research and development expense	$ 26,539	$ 24,432	$ 2,107

(1) Unallocated manufacturing expenses consist of shared pre-commercial manufacturing costs, primarily relating to raw materials, contract manufacturing, contract testing, process development, quality control and quality assurance activities and other manufacturing costs which support the development of multiple product candidates in our preclinical and clinical development programs.

(2) Other unallocated expenses include rental, storage, depreciation, and other facility related costs that we do not allocate to our individual product candidates.

The primary changes in our research and development expenses by product candidate or program in the six months ended June 30, 2024 compared to the six months ended June 30, 2023 are as follows:

- an increase in KB707 costs of $1.7 million following the expansion of our research and development pipeline to oncology consisting of increase in payroll related costs to support our research and an increase in contract research expenses in preparation for the Phase 1 clinical trial of inhaled KB707that has now commenced,
- a net increase in B-VEC costs of $1.3 million largely due to:
  - on-going manufacturing efficiency and process optimization costs for which such processes have not yet been approved by the FDA,
  - an increase in overseas preclinical and clinical trial costs and
  - overseas licensing and regulatory costs partially offset by costs being expensed to research and development expense prior to receiving FDA approval in May 2023 that are now included as part of the cost of inventory,
- an increase in other aesthetics programs of $944 thousand,
- an increase in ophthalmology programs of $712 thousand, inclusive of $214 thousand for ophthalmic B-VEC,
- an increase in KB407 costs of $632 thousand and
- an increase in other unallocated expenses of $343 thousand, which largely relates to depreciation due to the Company's second CGMP facility being placed into service in 2023 partially offset by the capitalization of depreciation associated with commercial batches of VYJUVEK after its approval in May 2023.

The increases were partially offset by:

- a decrease in other unallocated manufacturing expenses of $3.0 million primarily due to the costs related to the manufacturing of VYJUVEK following FDA approval being recorded as inventory and cost of goods sold and
- a decrease in stock-based compensation of $719 thousand due to the allocation of labor costs related to work performed to manufacture VYJUVEK to inventory following FDA approval.

Selling, General and Administrative Expenses

Selling, general and administrative expenses increased $3.7 million in the six months ended June 30, 2024 as compared to the six months ended June 30, 2023. The increase was primarily driven by the following:

- an increase in selling expenses related to the launch of VYJUVEK of $3.0 million, which includes $1.7 million related to our patient access program,
- an increase related to professional services incurred to support our commercial growth of $1.7 million,
- an increase in stock-based compensation of $1.4 million and
- an increase in payroll related expenses of $1.0 million, which was primarily driven by an increase in headcount to support overall growth.

The increases were partially offset by:

- a decrease in marketing costs of $2.5 million due to the timing of marketing activities ahead of the VYJUVEK launch and
- a decrease in information technology infrastructure costs of $436 thousand.

Litigation Settlement

Litigation settlement for the six months ended June 30, 2024 and 2023 was $25.0 million and $12.5 million, respectively, and consisted of amounts related to the settlement of litigation with PeriphaGen. See “Legal Proceedings” in Note 7 of the notes to condensed consolidated financial statements included in this Form 10-Q for more information.

Interest and Other Income, Net

Interest and other income, net for the six months ended June 30, 2024 and 2023 was $15.1 million and $8.4 million, respectively, and consisted of interest and dividend income earned from our cash, cash equivalents and investments. The increase in interest and dividend income is the result of increased investment activity and more favorable interest rates as compared to the prior period and an increase in our balance of cash, cash equivalents and investments.

Income Tax Expense

Income tax expense for the six months ended June 30, 2024 and 2023 was $477 thousand and zero, respectively.

Liquidity and Capital Resources

Overview

As of June 30, 2024, our cash, cash equivalents and short-term investments balance was approximately $559.6 million. As of June 30, 2024, we had an accumulated deficit of $253.3 million. We believe that our cash, cash equivalents and short-term investments as of June 30, 2024 will be sufficient to allow us to fund our operations for at least 12 months from the filing date of this Quarterly Report on Form 10-Q.

Our ability to continue to achieve operating profitability is dependent upon the continued successful commercialization of VYJUVEK and the successful development, approval, manufacturing, and commercialization of product candidates. Furthermore, we expect to incur increasing costs associated with satisfying regulatory and quality standards, maintaining and initiating product clinical trials, and furthering our efforts to discover, develop, manufacture, and commercialize current and future product candidates. We intend to fund future operations through on hand cash and cash equivalents, revenue generated from the sale of VYJUVEK, the sale of equity, debt financings, and we may also seek additional capital through arrangements with strategic partners or other sources.

Costs related to clinical trials can be unpredictable and, therefore, there can be no guarantee that we will have sufficient capital to fund the continued or planned pre-clinical and clinical studies for our product candidates, or our operations. Further, we expect future revenue to fluctuate from quarter to quarter for many reasons, including the uncertain timing and amount of any product sales. While we are in the process of building out our internal vector manufacturing capacity, some of our manufacturing activities will be contracted out to third parties. Additionally, we currently utilize third-party contract research organizations to carry out some of our clinical development activities. As we seek to obtain regulatory approval for our product candidates, we expect to continue to incur significant manufacturing and commercialization expenses as we prepare for product sales, marketing, commercial manufacturing, packaging, labeling and distribution. Furthermore, pursuant to our settlement agreement with PeriphaGen, we will be required to pay three $12.5 million contingent milestone payments upon reporting $100.0 million in cumulative sales, $200.0 million in cumulative sales and $300.0 million in cumulative sales. Our funds may not be sufficient to enable us to conduct pivotal clinical trials for, seek marketing approval for or commercially launch our product candidates. Accordingly, to obtain marketing approval for and to commercialize these or any other product

candidates, we may be required to obtain further funding through public or private equity offerings, debt financings, collaboration and licensing arrangements or other sources. Adequate additional financing may not be available to us on acceptable terms, if at all. Our failure to raise capital when needed could have a negative effect on our financial condition and our ability to pursue our business strategy.

Operating Capital Requirements

Our primary uses of capital are, and we expect will continue to be for the near future, compensation and related expenses, manufacturing costs for preclinical and clinical materials, regulatory expenses, third-party clinical trial research and development services, laboratory and related supplies, selling expenses, costs to manufacture our commercial product, legal expenses, payments of settlement amounts to PeriphaGen and general overhead costs. In order to complete the process of obtaining regulatory approval for any of our product candidates and to build the sales, manufacturing, marketing and distribution infrastructure that we believe will be necessary to commercialize our product candidates, if approved, we may require substantial additional funding.

We have based our projections of operating capital requirements on assumptions that may prove to be incorrect, and we may use all of our available capital resources sooner than we expect. Because of the numerous risks and uncertainties associated with research, development, manufacturing and commercialization of genetic medicines, we are unable to estimate the exact amount of our operating capital requirements. Our future funding requirements will depend on many factors, including, but not limited to:

- the costs needed to commercialize and market our lead product, VYJUVEK;
- the progress, timing and costs of clinical trials of our current product candidates;
- the progress, timing and costs of manufacturing VYJUVEK and revenue received from commercial sale of VYJUVEK;
- the continued development and the filing of IND applications for current and future product candidates;
- the initiation, scope, progress, timing, costs and results of drug discovery, laboratory testing, manufacturing, preclinical studies and clinical trials for any product candidates that we may pursue in the future, if any;
- the costs of maintaining our own commercial-scale CGMP manufacturing facilities;
- the outcome, timing and costs of seeking regulatory approvals;
- the costs associated with the manufacturing process development and evaluation of third-party manufacturers;
- the extent to which the costs of VYJUVEK and our product candidates, if approved, will be paid by health maintenance, managed care, pharmacy benefit and similar healthcare management organizations, or will be reimbursed by government authorities, private health coverage insurers and other third-party payors;
- the costs of commercialization activities for our current and future product candidates if we receive marketing approval for such product candidates, including the costs and timing of establishing product sales, medical affairs, marketing, distribution and manufacturing capabilities;
- subject to receipt of marketing approval, if any, revenue received from commercial sale of our current and future product candidates;
- the terms and timing of any future collaborations, licensing, consulting or other arrangements that we may establish;
- the amount and timing of any payments we may be required to make, or that we may receive, in connection with the licensing, filing, prosecution, maintenance, defense and enforcement of any patents or other intellectual property rights, including milestone and royalty payments and patent prosecution fees that we are obligated to pay pursuant to our license agreements;
- our current license agreements remaining in effect and our achievement of milestones under those agreements;
- our ability to establish and maintain collaborations and licenses on favorable terms, if at all; and
- the extent to which we acquire or in-license other product candidates and technologies.

We may need to obtain substantial additional funding in order to receive regulatory approval and to commercialize our product candidates. To the extent that we raise additional capital through the sale of common stock, convertible securities or other equity securities, the ownership interests of our existing stockholders may be materially diluted and the terms of these securities could include liquidation or other preferences that could adversely affect the rights of our existing stockholders. In addition, debt financing, if available, would result in increased fixed payment obligations and may involve agreements that

include restrictive covenants that limit our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends, that could adversely affect our ability to conduct our business. If we are unable to raise capital when needed or on attractive terms, we could be forced to significantly delay, scale back or discontinue the development or commercialization of our product candidates, seek collaborators at an earlier stage than otherwise would be desirable or on terms that are less favorable than might otherwise be available, and relinquish or license, potentially on unfavorable terms, our rights to our product candidates that we otherwise would seek to develop or commercialize ourselves.

Sources and Uses of Cash

The following table summarizes our sources and uses of cash for the six months ended June 30, 2024 and 2023:

	Six Months Ended June 30,	
	2024	**2023**
(in thousands)	**(unaudited)**	
Net cash provided by (used in) operating activities	$ 11,720	$ (60,346)
Net cash (used in) investing activities	(45,333)	(12,394)
Net cash provided by financing activities	21,221	186,743
Effect of exchange rate changes on cash and cash equivalents	(150)	(28)
Net (decrease) increase in cash	$ (12,542)	$ 113,975

Operating Activities

Net cash provided by operating activities for the six months ended June 30, 2024 was $11.7 million and consisted primarily of net income of $16.5 million adjusted for $22.2 million of non-cash items and a $27.0 million decrease in working capital. Non-cash adjustments included depreciation of $3.3 million, amortization of operating lease right-of-use assets of $368 thousand, stock-based compensation expense of $22.5 million and other adjustments of $89 thousand, offset by realized gain on investments of $2.9 million and accretion on marketable securities of $1.1 million.

Net cash used in operating activities for the six months ended June 30, 2023 was $60.3 million and consisted primarily of net loss of $78.5 million adjusted for $21.2 million of non-cash items and a $3.0 million decrease in working capital. Non-cash adjustments included depreciation of $2.3 million, amortization of operating lease right-of-use assets of $441 thousand, stock-based compensation expense of $21.8 million and other adjustments of $50 thousand, offset by realized gain on investments of $2.4 million and accretion on marketable securities of $1.0 million.

Investing Activities

Net cash used in investing activities for the six months ended June 30, 2024 was $45.3 million and consisted of $201.7 million in purchases of short-term and long-term investments and $2.4 million in purchases of property and equipment, partially offset by $158.8 million received from the maturities and early calls of short- and long-term investments.

Net cash used in investing activities for the six months ended June 30, 2023 was $12.4 million and consisted of $320.0 million in purchases of short-term and long-term investments and $8.2 million in purchases of property and equipment, partially offset by $315.7 million received from the maturities of short-term investments.

Financing Activities

Net cash provided by financing activities for the six months ended June 30, 2024 was $21.2 million and consisted of proceeds of $26.6 million from exercises of stock options, partially offset by $4.2 million used for employee tax withholding payments related to vested restricted stock units and $1.2 million used for employee tax withholding payments for settlement of vested restricted stock awards.

Net cash provided by financing activities for the six months ended June 30, 2023 was $186.7 million and consisted of proceeds of $159.8 million from issuances of common stock, proceeds of $27.7 million from exercises of stock options, partially offset by $749 thousand used for employee tax withholding payments for settlement of vested restricted stock awards.

ITEM 3. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK

We had cash, cash equivalents and short-term investments of $559.6 million as of June 30, 2024, which consisted primarily of money market funds, commercial paper, corporate bonds and U.S. government agency securities. The investments in these financial instruments are made in accordance with an investment policy which specifies the categories, allocations and ratings of securities we may consider for investment. The primary objective of our investment activities is to preserve principal while at the same time maximizing the income we receive without significantly increasing risk. Some of the financial instruments in which we invest could be subject to market risk. This means that a change in prevailing interest rates may cause the value of the instruments to fluctuate. For example, if we purchase a security that was issued with a fixed interest rate and the

prevailing interest rate later rises, the value of that security will probably decline. To minimize this risk, we intend to maintain a portfolio which may include cash, cash equivalents and short-term investment securities available-for-sale in a variety of securities which may include money market funds, government and non-government debt securities and commercial paper, all with various maturity dates. Based on our current investment portfolio, we do not believe that our results of operations or our financial position would be materially affected by an immediate change of 10% in interest rates.

We also have established operations in Europe, Australia and Japan and hold cash in Swiss Francs, Euros, Australian Dollars and Japanese Yen. We are subject to foreign exchange rate risk arising from transactions conducted in the aforementioned foreign currencies, however, our foreign operations are not currently material to our business. We do not believe that our results of operations or our financial position would be materially affected by an immediate change of 10% in foreign currency exchange rates.

We do not hold or issue derivatives, derivative commodity instruments or other financial instruments for speculative trading purposes. Further, we do not believe our cash, cash equivalents and short-term investments have significant risk of default or illiquidity. While we believe our cash, cash equivalents and short-term investments do not contain excessive risk, we cannot provide absolute assurance that any investments we make in the future will not be subject to adverse changes in market value. Our cash, cash equivalents and short-term investments are recorded at fair value.

ITEM 4. CONTROLS AND PROCEDURES

Evaluation of Disclosure Controls and Procedures

Our Chief Executive Officer and our Chief Accounting Officer, with the participation of other members of the Company's management, have evaluated the effectiveness of the Company's "disclosure controls and procedures" (as such term is defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended (the "Exchange Act")) as of the end of the period covered by this quarterly report, and our Chief Executive Officer and our Chief Accounting Officer have concluded that our disclosure controls and procedures are effective based on their evaluation of these controls and procedures as required by paragraph (b) of Exchange Act Rules 13a-15 or 15d-15.

Changes in Internal Control over Financial Reporting

There was no change in our internal control over financial reporting that occurred during the three months ended June 30, 2024 that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

PART II. OTHER INFORMATION

ITEM 1. LEGAL PROCEEDINGS

The information set forth under the heading “Legal Proceedings” in Note 7 of the notes to condensed consolidated financial statements included in Item 1 of Part I of this Form 10-Q is incorporated by reference in response to this item.

ITEM 1A. RISK FACTORS

Our business involves significant risks, some of which are described below. You should carefully consider the risks and uncertainties described below, together with all of the other information contained in this Quarterly Report on Form 10-Q, including “Management’s Discussion and Analysis of Financial Condition and Results of Operations” and the condensed consolidated financial statements and the related notes included in Item 1 of Part I of this Form 10-Q. If any of the following risks actually occur, it could harm our business, prospects, operating results and financial condition and future prospects. In such event, the market price of our common stock could decline, and you could lose all or part of your investment. Additional risks and uncertainties not presently known to us or that we currently deem immaterial may also impair our business operations.

Risks Related to Our Business and Industry

We are substantially dependent on the commercial success of VYJUVEK

To date, we have invested substantial efforts and financial resources in the research and development of VYJUVEK and our product candidates. Our near-term prospects, including our ability to develop our product candidates and generate revenue, and our future growth is substantially dependent on the commercial success of VYJUVEK.

Although we received approval from the FDA for VYJUVEK for the treatment of DEB on May 19, 2023, we can provide no assurances that we will obtain regulatory approval in any other jurisdiction, which would have an adverse impact on our results of operations. In addition, the successful commercialization of VYJUVEK will depend on a number of factors, including the risks identified in these “Risk Factors.” One or more of these factors, many of which are beyond our control, could cause significant delays or an inability to successfully commercialize VYJUVEK.

We may not be successful in our efforts to identify, develop and commercialize additional product candidates, which may impair our ability to expand our business and achieve our strategic objectives, and we may fail to capitalize on programs or product candidates that may be a greater commercial opportunity or for which there is a greater likelihood of success.

Although a substantial amount of our efforts focus on the commercialization of VYJUVEK and the development and potential approval of our current product candidates, a key component of our strategy is to identify, develop and potentially commercialize a portfolio of genetic medicines. Research programs to identify new product candidates require substantial technical, financial, and human resources and may not be successful in identifying potential product candidates. Even if we identify product candidates that initially show promise, we may fail to successfully develop and commercialize such product candidates for many reasons, including the following:

- competitors may develop alternatives that render our product candidates obsolete;
- product candidates we develop may be covered by third parties’ patents or other exclusive rights;
- a product candidate may, on further study, be shown to have harmful side effects or other characteristics that indicate it is unlikely to be effective or otherwise does not meet applicable regulatory criteria;
- a product candidate may not be capable of being produced in commercial quantities at an acceptable cost, or at all; and
- a product candidate may not be accepted as safe and effective by patients, the medical community, or third-party payors.

If we are unsuccessful in identifying and developing additional product candidates, our potential for growth may be impaired.

Additionally, because we have limited resources, we may forego or delay pursuit of opportunities with certain programs or product candidates or for indications that later prove to have commercial potential. Our resource allocation decisions may cause us to fail to timely capitalize on viable commercial products or profitable market opportunities. Our spending on current and future research and development programs may not yield any commercially viable products. If we do not accurately evaluate the commercial potential for a particular product candidate, we may relinquish valuable rights to that product candidate through strategic collaboration, licensing, or other arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate. Alternatively, we may allocate internal resources to a product candidate in a therapeutic area in which it would have been more advantageous to enter into a partnering arrangement.

If any of these events occur, we may be forced to abandon our development efforts with respect to a particular product candidate or fail to develop a potentially successful product candidate, which could have a material adverse effect on our business, financial condition, results of operations, and prospects.

VYJUVEK and, if approved, our investigational product candidates regulated as biologics may face competition from biosimilars approved through an abbreviated regulatory pathway.

The Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010 (collectively, the "ACA") includes a subtitle called the Biologics Price Competition and Innovation Act of 2009 ("BPCIA"), which created an abbreviated approval pathway for biological products that are biosimilar to or interchangeable with an FDA-licensed reference biological product. Under the BPCIA, an application for a biosimilar product may not be submitted to the FDA until four years following the date that the reference product was first licensed by the FDA. In addition, the approval of a biosimilar product may not be made effective by the FDA until 12 years from the date on which the reference product was first licensed. During this 12-year period of exclusivity, another company may still market a competing version of the reference product if the FDA approves a Biologics License Application, or BLA, for the competing product containing the sponsor's own preclinical data and data from adequate and well-controlled clinical trials to demonstrate the safety, purity, and potency of the other company's product. In addition, a competitor may choose to challenge our patent rights relating to the reference product by initiating litigation during the 12-year period of exclusivity. After the FDA approves the BLA for the competing product, the competitor may also bring a declaratory judgment action of non-infringement, invalidity, and/or unenforceability of our patent rights. The law is complex and is still being interpreted and implemented by the FDA. As a result, its ultimate impact, implementation, and meaning are subject to uncertainty.

We believe that any of our product candidates approved as a biological product under a BLA should qualify for the 12-year period of exclusivity. However, there is a risk that this exclusivity could be shortened due to congressional action or otherwise, or that the FDA will not consider our investigational medicines to be reference products for competing products, potentially creating the opportunity for generic competition sooner than anticipated. Other aspects of the BPCIA, some of which may impact the BPCIA exclusivity provisions, have also been the subject of recent litigation. Moreover, the extent to which a biosimilar, once licensed, will be substituted for any one of our approved products in a way that is similar to traditional generic substitution for non-biological products is not yet clear, and will depend on a number of marketplace and regulatory factors that are still developing.

If competitors are able to obtain marketing approval for biosimilars referencing any of our approved products, our approved products may become subject to competition from such biosimilars, which would impair our ability to successfully commercialize and generate revenue from sales of such products.

We face significant competition in an environment of rapid technological change and the possibility that our competitors may achieve regulatory approval before us or develop therapies that are more advanced or effective than ours, which may adversely affect our financial condition and our ability to successfully commercialize and market our product candidates.

We are aware of several companies and institutions that have developed, or are currently developing, alternative autologous or palliative gene therapy or other approaches for our targeted indications, including DEB, cystic fibrosis, solid tumors and aesthetic skin conditions. Many of our potential competitors, alone or with their strategic partners, have substantially greater financial, technical, and other resources, such as larger research and development, clinical, marketing, and manufacturing organizations. Mergers and acquisitions in the biotechnology and pharmaceutical industries may result in even more resources being concentrated among a smaller number of competitors. Our commercial opportunities could be reduced or eliminated if competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient or are less expensive than VYJUVEK or any product candidate that we may develop. Competitors also may obtain FDA or other regulatory approval for their products more rapidly or earlier than we may obtain approval for our product candidates, which could result in our competitors establishing a strong market position before we are able to enter the market. Additionally, technologies developed by our competitors may render VYJUVEK or any of our product candidates uneconomical or obsolete, and we may not be successful in marketing VYJUVEK or any of our product candidates that obtain regulatory approval against competitors.

Even if we commercialize a product candidate faster than our competitors, we could also face competition from lower cost biosimilars.

In addition, as a result of the expiration or successful challenge of our patent rights, we could face litigation with respect to the validity and/or scope of patents relating to our competitors' products. The availability of our competitors' products could limit the demand, and the price we are able to charge, for VYJUVEK or any product candidate that we may develop and commercialize.

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

We face an inherent risk of product liability lawsuits related to the sale of VYJUVEK, use of VYJUVEK and our product candidates, and testing of our product candidates. Product liability claims may be brought against us by participants enrolled in our clinical trials, patients, health care providers or others using, or administering VYJUVEK and our product candidates. If we cannot successfully defend ourselves against any such claims, we may incur substantial liabilities. Regardless of their merit or eventual outcome, liability claims may result in:

- decreased demand for VYJUVEK;
- injury to our reputation;
- withdrawal of clinical trial participants;
- termination of clinical trial sites or entire trial programs;
- increased regulatory scrutiny;
- significant litigation costs;
- substantial monetary awards to or costly settlement with claimants;
- product recalls for any approved products or a change in the indications for which they may be used;
- loss of revenue;
- diversion of management and scientific resources from our business operations; and
- the inability to successfully commercialize VYJUVEK or our product candidates, if approved.

With respect to VYJUVEK and any of our product candidates that are approved for commercial sale in the future, we are, and will be, highly dependent upon physician and patient perceptions of us and the safety and quality of our products. We could be adversely affected if we are subject to negative publicity. We could also be adversely affected if any of our products or any similar products distributed by other companies prove to be, or are asserted to be, harmful to patients. Because of our dependence upon consumer perceptions, any adverse publicity could have a material adverse impact on our financial condition or results of operations.

Our product liability insurance coverage may not be adequate to cover all liabilities that we may incur. We may need to increase our insurance coverage now that VYJUVEK has been approved by the FDA and when we begin the commercialization of our product candidates, if approved. Insurance coverage is becoming increasingly expensive. As a result, we may be unable to maintain or obtain sufficient insurance at a reasonable cost to protect us against losses that could have a material adverse effect on our business. A successful product liability claim, or series of claims brought against us, particularly if judgments exceed any insurance coverage we may have, could decrease our cash resources and adversely affect our business, financial condition, and results of operations.

Negative public opinion and increased regulatory scrutiny of gene therapy may damage public perception of the safety of our gene therapy product candidates and adversely affect our ability to conduct our business or obtain regulatory approvals for our product candidates.

Gene therapy remains a novel technology. Ethical, social, and legal concerns about gene therapy could result in additional regulations restricting or prohibiting VYJUVEK or our product candidates. Public perception may be influenced by claims that gene therapy is unsafe, and gene therapy may not gain the acceptance of the public or the medical community. In particular, our success depends upon physicians who specialize in the treatment of DEB or genetic diseases targeted by our product candidates prescribing VYJUVEK or treatments that involve the use of our product candidates that may be in lieu of, or in addition to, existing treatments with which they are familiar and for which greater clinical data may be available. More restrictive 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 and commercialization of VYJUVEK or our product candidates or demand for VYJUVEK or any product candidates we may develop. For example, earlier gene therapy trials led to several well- publicized adverse events, including cases of leukemia and death seen in trials using other vectors. Serious adverse events in our clinical trials, or other clinical trials involving gene therapy products or our competitors' products, even if not ultimately attributable to the relevant product candidates, and the resulting publicity, could result in increased government regulation, unfavorable public perception, potential regulatory delays in the testing or approval of our product candidates, stricter labeling requirements for those product candidates that are approved and a decrease in demand for any such product candidates.

Our business operations may subject us to disputes, claims and lawsuits, which may be costly and time-consuming and could materially and adversely impact our financial position and results of operations.

From time to time, we may become involved in disputes, claims and lawsuits relating to our business operations. For example, we may, from time to time, face or initiate claims related to intellectual property matters, employment matters, or commercial matters. Any dispute, claim or lawsuit may divert management's attention away from our business, we may incur significant expenses in addressing or defending any dispute, claim or lawsuit, and we may be required to pay damage awards or settlements or become subject to equitable remedies that could adversely affect our operations and financial results. Litigation related to these disputes may be costly and time-consuming and could materially and adversely impact our financial position and results of operations if resolved against us. In addition, the uncertainty associated with litigation could lead to increased volatility in our stock price.

The increasing use of social media platforms presents new risks and challenges.

Social media is increasingly being used by us, our employees, or others to communicate about our business, VYJUVEK, our clinical development programs, DEB, and the diseases our product candidates are being developed to treat. We use appropriate social media in connection with our commercialization efforts of VYJUVEK and intend to use it in connection with our commercialization efforts of our product candidates, if approved. Social media practices in the biotechnology and biopharmaceutical industries continue to evolve, and regulations and regulatory guidance relating to such use are evolving and not always clear. This evolution creates uncertainty and risk of noncompliance with regulations applicable to our business, resulting in potential regulatory actions against us, along with the potential for litigation and heightened scrutiny by the FDA, the Securities and Exchange Commission, or the SEC, and other regulators. For example, patients may use social media channels to comment on their experience in an ongoing clinical trial of our product candidates, or to report an alleged adverse event. If such disclosures occur, there is a risk that clinical trial enrollment may be adversely impacted, that we may fail to monitor and comply with applicable adverse event reporting obligations, or that we may not be able to defend our business or the public's legitimate interests in the face of the political and market pressures generated by social media due to restrictions on what we may say about our product candidates. There is also a risk of inappropriate disclosure of sensitive information, loss of trade secrets or other intellectual property, public exposure of personal information of our employees, patients who use VYJUVEK, clinical trial patients, and others, or negative or inaccurate posts or comments about us on any social networking website. In addition, we may encounter attacks on social media regarding our company, management, VYJUVEK, or our product candidates that seriously damage our reputation, brand image, and goodwill. If any of these events were to occur or we otherwise fail to comply with applicable regulations, we could incur liability, face regulatory actions, or incur other harm to our business that could have a material adverse effect on our business, prospects, operating results, and financial condition and could adversely affect the price of our common stock.

We have experienced significant growth in the number of employees and infrastructure and may experience difficulties in managing this growth. If we are unable to manage expected growth in the scale and complexity of our operations, our performance may suffer.

We have experienced a period of significant expansion in personnel and of our facilities, infrastructure and overhead as we developed our own manufacturing facilities, built our sales, marketing and distribution infrastructure that we believe is necessary to commercialize VYJUVEK, and increased our research and development efforts. The commercialization of VYJUVEK and our ongoing development of other product candidates will continue to impose significant capital requirements, as well as added responsibilities on members of management, including the need to identify, recruit, maintain and integrate new personnel. Our future performance and our ability to compete effectively will depend, in part, on our ability to manage our growth effectively. If we are successful in executing our business strategy, we will need to expand our managerial, operational, financial, and other systems and resources to manage our operations, continue our research and development activities and build a commercial infrastructure to support commercialization of any of our product candidates that are approved for sale, and, depending on demand, may need to scale up the manufacturing process for any approved product, which is subject to risks and uncertainties. Future growth would impose significant added responsibilities on members of management. Our management, finance, development personnel, systems, and facilities currently in place may not be adequate to support this expected future growth. Our need to effectively manage our operations, growth and product candidates requires that we continue to develop more robust business processes and improve our systems and procedures in each of these areas and to attract and retain enough numbers of talented employees. We may be unable to successfully implement these tasks on a larger scale and, accordingly, may not achieve our research, development, and growth goals.

Our future success depends on our ability to retain key employees and scientific advisors and to attract, retain and motivate qualified personnel.

We are highly dependent on members of our management team, the loss of whose services may adversely impact the achievement of our objectives. Our employees and scientific advisors are at-will employees and consultants, and the loss of one or more of them might impede the achievement of our research, development, and commercialization objectives.

Recruiting and retaining other qualified employees and scientific advisors for our business, including scientific and technical personnel, also will be critical to our success. Competition for skilled personnel, including in gene therapy research and vector manufacturing, is intense and the turnover rate can be high. We may not be able to attract and retain personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies and academic institutions for individuals with similar skill sets. In addition, failure to succeed in preclinical or clinical trials or applications for marketing approval may make it more challenging to recruit and retain qualified personnel. The inability to recruit, or loss of services of certain executives, key employees, or advisors, may impede the progress of our research, development and commercialization objectives and have a material adverse effect on our business, financial condition, results of operations and prospects.

Our employees, principal investigators and advisors may engage in misconduct or other improper activities, including non-compliance with regulatory standards and requirements.

We are exposed to the risk of fraud or other misconduct by our employees, principal investigators, and advisors. Misconduct by these parties could include intentional failures to comply with FDA regulations or the regulations applicable in the European Union, or EU, and other jurisdictions, provide accurate information to the FDA, the European Medicines Agency ("EMA") and other regulatory authorities, comply with healthcare fraud and abuse laws and regulations in the United States and abroad, report financial information or data accurately or disclose unauthorized activities. Sales, marketing, and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, misconduct, kickbacks, self-dealing and other abusive practices. These laws and regulations restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. It is not always possible to identify and deter employee misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from government investigations or other actions or lawsuits stemming from a failure to comply with these laws or 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, financial condition, results of operations and prospects, including the imposition of significant fines, criminal penalties, or other sanctions.

In addition, principal investigators for our clinical trials may serve as scientific advisors or consultants to us from time to time and receive compensation in connection with such services. Under certain circumstances, we may be required to report some of these relationships to the FDA. The FDA may conclude that a financial relationship between us and a principal investigator has created a conflict of interest or otherwise affected interpretation of the clinical trial. The FDA may therefore question the integrity of the data generated at the applicable clinical trial site and the utility of the clinical trial itself may be jeopardized. This could result in a delay in approval, or rejection, of our marketing applications by the FDA and may ultimately lead to the denial of marketing approval of our current and future product candidates.

Healthcare legislative reform measures may have a material adverse effect on our business and results of operations.

In the United States and some foreign jurisdictions, there have been, and continue to be, several legislative and regulatory changes and proposed changes regarding the healthcare system that could prevent or delay marketing approval of our product candidates, restrict or regulate post-approval activities, and affect our ability to profitably sell VYJUVEK and any product candidates for which we obtain marketing approval.

In the United States, there have been and continue to be a number of legislative efforts to contain healthcare costs. Any legislative changes that result in price controls, reduce access to and reimbursement for care or add additional regulations may have an adverse effect on our financial condition and results of operations. Any changes that reduce, or impede the ability to obtain, reimbursement for VYJUVEK or our product candidates that we intend to commercialize in the United States could adversely affect successful commercialization of VYJUVEK and our plans to introduce our product candidates in the United States. For example, the Bipartisan Budget Act of 2018, among other things, amended the ACA, effective January 1, 2019, to increase from 50 percent to 70 percent the point-of-sale discount that is owed by pharmaceutical manufacturers who participate in Medicare Part D and to close the coverage gap in most Medicare drug plans, commonly referred to as the "donut hole."

Other legislative changes have been proposed and adopted in the United States since the ACA was enacted. For example, in August 2011, the Budget Control Act of 2011, among other things, created measures for spending reductions by Congress. A Joint Select Committee on Deficit Reduction, tasked with recommending a targeted deficit reduction of at least $1.2 trillion for the years 2012 through 2021, was unable to reach required goals, thereby triggering the legislation's automatic reduction to several government programs. This includes aggregate reductions of Medicare payments to providers of up to 2% per fiscal year, which went into effect in April 2013 and will remain in effect through 2027 unless additional Congressional action is taken. In January 2013, the American Taxpayer Relief Act of 2012, among other things, further reduced Medicare payments to certain providers, and increased the time for Medicare contractors to recoup Medicare overpayments to providers from three to five years. In August 2022, the Inflation Reduction Act of 2022 ("IRA") was signed into law. The IRA includes several provisions to lower prescription drug costs for people with Medicare and reduce drug spending by the federal

government. In relevant part, the IRA allows Medicare to negotiate prices for certain prescription drugs, requires drug manufacturers to pay a rebate to the federal government if prices for single-source drugs and biologicals covered under Medicare Part B and nearly all covered drugs under Part D increase faster than the rate of inflation, caps out of pocket spending for Medicare Part D enrollees, and makes other benefit design changes to Medicare Part D intended to lower drug costs for enrollees and Medicare. Implementation of these changes began in 2023, and will continue to be implemented over the next several years. Multiple pharmaceutical manufacturers have challenged the law in court, largely on constitutional grounds. These suits will continue through 2024 and the ultimate effects of such legal challenges are unclear. At this time, we continue to evaluate the effect of the IRA on our business operations and financial condition and results as the full impact of the IRA remains uncertain.

Further, there has been heightened governmental scrutiny in recent years over the manner in which manufacturers set prices for their marketed products and the cost of prescription drugs to consumers and government healthcare programs, which have resulted in several recent Congressional inquiries and proposed and enacted bills designed to, among other things, reduce the cost of prescription drugs, bring more transparency to product pricing, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for products. In addition, the United States government, state legislatures, and foreign governments have shown significant interest in implementing cost containment programs, including price-controls, restrictions on reimbursement and requirements for substitution of generic products for branded prescription drugs to limit the growth of government paid health care costs. For example, the United States government has passed legislation requiring pharmaceutical manufacturers to provide rebates and discounts to certain entities and governmental payors to participate in federal healthcare programs. Individual states in the United States have also been increasingly passing legislation and implementing regulations designed to control pharmaceutical product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing.

Additional changes may affect our business, including those governing enrollment in federal healthcare programs, reimbursement changes, fraud and abuse enforcement, and expansion of new programs, such as Medicare payment for performance initiatives. In October 2022, President Biden signed Executive Order 14087 on “Lowering Prescription Drug Costs for Americans.” The Executive Order specifically requests that the Center for Medicare and Medicaid Innovation consider “models that may lead to lower cost sharing for commonly used drugs and support value-based payment that supports high-quality care.” The outcomes of the findings made under the Executive Order could lead to further drug pricing initiatives that could affect reimbursement for our product and product candidates.

These initiatives, as well as other healthcare reform measures that may be adopted in the future, may result in more rigorous coverage criteria and in additional downward pressure on the price that we receive for any approved product. Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private payors. The implementation of cost containment measures or other healthcare reforms could result in reduced demand for our product and product candidates or additional pricing pressures and may adversely impact our ability to generate sufficient revenue, attain consistent profitability, or commercialize our product candidates, if approved.

We are subject, directly or indirectly, to federal and state healthcare fraud and abuse laws, false claims laws and health information privacy and security laws. If we are unable to comply, or have not fully complied, with such laws, we could face substantial penalties.

With the FDA approval of VYJUVEK, our operations are directly, or indirectly through our prescribers, customers, and purchasers, subject to various federal and state fraud and abuse laws and regulations, including, without limitation, the federal Anti-Kickback Statute, federal civil and criminal false claims laws and the Physician Payments Sunshine Act and regulations. These laws impact, among other things, our sales, marketing, access assistance, sponsored genetic patient testing, and educational programs. In addition, we are subject to patient privacy laws by both the federal government and the states in which we conduct our business as well as other jurisdictions. The laws that affect our operations include, but are not limited to:

- the federal Anti-Kickback Statute, which prohibits, among other things, persons or entities from knowingly and willfully soliciting, receiving, offering or paying any remuneration (including any kickback, bribe or rebate), directly or indirectly, overtly or covertly, in cash or in kind, in return for the purchase, recommendation, leasing or furnishing of an item or service reimbursable under a federal healthcare program, such as the Medicare and Medicaid programs. This statute has been interpreted to apply to arrangements between pharmaceutical manufacturers on the one hand, and prescribers, purchasers, and formulary managers on the other. The ACA amended the intent requirement of the federal Anti-Kickback Statute to clarify that a person or entity does not have to have actual knowledge of this statute or specific intent to violate it;
- federal civil and criminal false claims laws and civil monetary penalty laws which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, claims for payment or approval

from Medicare, Medicaid, or other government payors that are false or fraudulent. The ACA provides that a claim for items or services resulting from an Anti-Kickback Statute violation is a false claim under the federal False Claims Act ("FCA"). Cases against pharmaceutical manufacturers support the view that certain marketing practices, including off-label promotion, may implicate the FCA;

- the federal Health Care Fraud statute imposes criminal and civil liability for executing a scheme to defraud any healthcare benefit program or making false statements relating to healthcare matters;
- the Health Insurance Portability and Accountability Act of 1996 ("HIPAA"), as amended by the Health Information Technology for Economic and Clinical Health Act ("HITECH"), and its implementing regulations, and as amended again by the final HIPAA omnibus rule (the "Omnibus Rule" and together with HIPAA and HITECH, the HIPAA Rules), which impose certain requirements relating to the privacy, security and transmission of individually identifiable health information by certain entities subject to the HIPAA Rules, such as health plans, health care clearinghouses and health care providers that engage in certain covered transactions;
- federal transparency laws, including the federal Physician Payment Sunshine Act, that require certain manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program, with specific exceptions, to report annually to the Centers for Medicare and Medicaid Services ("CMS") information related to: (i) payments or other "transfers of value" made to physicians and teaching hospitals, and (ii) ownership and investment interests held by physicians and their immediate family members;
- state and foreign law equivalents of each of the above federal laws, state laws that require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures and state laws governing the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and may not have the same effect, thus complicating compliance efforts in certain circumstances, such as specific disease states; and
- state and foreign laws that govern the privacy and security of health information in some circumstances, many of which differ from each other in significant ways and often are not preempted by the HIPAA Rules, thus complicating compliance efforts.

Because of the breadth of these laws and the narrowness of the statutory exceptions and safe harbors available, it is possible that some of our business activities could be subject to challenge under one or more of such laws. If our operations are found to be in violation of any of the laws described above or any other government regulations that apply to us, we may be subject to penalties, including civil and criminal penalties, damages, fines, exclusion from participation in government health care programs, such as Medicare and Medicaid, imprisonment and the curtailment or restructuring of our operations, any of which could adversely affect our ability to operate our business and our results of operations.

Often, to avoid the threat of treble damages and penalties under the FCA, health care providers will resolve allegations in a settlement without admitting liability. Any such settlement could materially affect our business, financial operations, and reputation.

Efforts to ensure that our business arrangements with third parties comply with applicable healthcare laws and regulations involve substantial costs. It is possible that governmental authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations.

The risk of our being found in violation of these laws is increased by the fact that many of them have not been fully interpreted by the regulatory authorities or the courts, and their provisions are open to a variety of interpretations. Any action against us for violation of these laws, even if we successfully defend against it, could cause us to incur significant legal expenses and divert our management's attention from the operation of our business. The shifting compliance environment and the need to build and maintain a robust and expandable systems to comply with multiple jurisdictions with different compliance and/or reporting requirements increases the possibility that we may run afoul of one or more of the requirements.

If we fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs that could have a material adverse effect on the success of our business.

We are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the generation, handling, use, storage, treatment, manufacture, transportation, and disposal of, and exposure to, hazardous materials and wastes, as well as laws and regulations relating to occupational health and safety. Our operations involve the use of hazardous and flammable materials, including chemicals and biologic materials. Our operations also produce hazardous waste products. We generally contract with third parties for the disposal of these materials and wastes. We cannot eliminate the risk of contamination or injury from these materials. In the event of contamination or injury resulting

from our use of hazardous materials, we could be held liable for any resulting damages, and any liability could exceed our resources. Moreover, certain environmental laws may impose liability without regard to fault or legality of the action at the time of its occurrence. We also could incur significant costs associated with civil or criminal fines and penalties. We do not carry specific biological or hazardous waste insurance coverage. Accordingly, in the event of contamination or injury, we could be held liable for damages or be penalized with fines in an amount that could have a material adverse effect on our business, financial condition, results of operations, and prospects, and our clinical trials or regulatory approvals could be suspended.

Although we maintain workers' compensation insurance for certain costs and expenses we may incur due to injuries to our employees resulting from the use of hazardous materials or other work-related injuries, this insurance may not provide adequate coverage against potential liabilities. We also may incur substantial costs to comply with current or future environmental, health and safety laws and regulations, which have tended to become more stringent over time. These current or future laws and regulations may impair our research, development, or production efforts. Failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions or liabilities, which could materially adversely affect our business, financial condition, results of operations and prospects.

We are subject to stringent and evolving U.S. and foreign laws, regulations and other obligations related to privacy and data security. Our actual or perceived failure to comply with such obligations could lead to regulatory inquiries or actions, litigation, fines and penalties, disruptions to our business operations, reputational harm, loss of revenue, and other adverse business consequences.

Privacy and data security have become significant areas of legal and regulatory focus in the United States, European Union and in many other jurisdictions where we conduct or may conduct our operations. In our ordinary course of business, we collect, receive, store, process, generate, use, transfer, disclose, make accessible, protect, secure, dispose of, transmit, and share (collectively, "process") personal information and other sensitive information, including, but not limited to, health information, individuals' financial information, as well as proprietary and confidential business data, including trade secrets, intellectual property, and sensitive third-party data (collectively, "sensitive data"). Our data processing activities may subject us to numerous privacy and data security obligations, including, but not limited to, domestic and international laws, regulations, guidance, industry standards, external and internal privacy and security policies, and contractual requirements.

In the United States, federal, state, and local governments have enacted numerous data privacy and security laws, including data breach notification laws, personal information privacy laws, consumer protection laws, and other similar laws. Notably, HIPAA, as amended by HITECH, imposes requirements on certain entities regarding the privacy, security, and transmission of individually identifiable health information and the California Consumer Privacy Act of 2018 ("CCPA") requires businesses to provide specific disclosures in their privacy notices and honor California residents' privacy rights. The CCPA provides for civil penalties of up to $7,500 per violation and allows private litigants affected by certain data breaches to recover significant statutory damages. Although the CCPA does not apply to certain data that we process in the context of clinical trials, efforts to comply with the CCPA may increase our annual compliance costs and subject us to potential liability with respect to other personal information we may maintain about California residents. In addition, the California Privacy Rights Act of 2020 ("CPRA"), which came into effect on January 1, 2023, expanded the CCPA's requirements, extending it to cover personal information of business representatives and employees and the CPRA established a new regulatory agency to implement and enforce the law. Other states, such as Virginia, Nevada, and Colorado, have also passed comprehensive privacy laws, and similar laws are being considered in several other states, as well as at the federal and local levels. While these states' laws, like the CCPA, also exempt some data processed in the context of clinical trials, these developments further complicate our compliance efforts and increase both legal risk and compliance costs for us and the third parties upon whom we rely.

Outside of the United States, there are an increasing number of laws, regulations, and industry standards regarding privacy and data security. For example, the EU General Data Protection Regulation ("GDPR") and UK GDPR impose strict requirements for processing personal information, and companies that violate the GDPR may face temporary or permanent bans on certain data processing activities and they may be subject to other penalties such as fines of up to 20 million Euros under the EU GDPR / 17.5 million pounds sterling under the UK GDPR or 4% of annual global revenue, whichever is greater; or private litigation related to processing of personal information brought by classes of data subjects or consumer protection organizations authorized to represent data subjects' interests.

In some circumstances, we may be unable to transfer personal information between certain jurisdictions due to data localization requirements or other limitations on cross-border data flows. Europe and other jurisdictions have enacted laws requiring data to be localized or limiting the transfer of personal information to other countries. In particular, the European Economic Area ("EEA") and the UK have significantly restricted the transfer of personal information to the United States and other countries whose privacy laws they consider inadequate. Although there are various mechanisms that may be used to transfer personal information from the EEA and UK to the United States in compliance with the law, such as the EEA and UK's standard contractual clauses, these mechanisms are subject to legal challenges, and we may be unable to rely on these measures to lawfully transfer personal information to the United States in all cases. If there is no lawful manner for us to transfer personal information from the EEA, the UK, or other jurisdictions to the United States, or if the requirements for a legally compliant

transfer are too onerous, we could face significant adverse consequences, including increased exposure to regulatory actions, substantial fines and penalties, the inability to transfer data and work with partners, vendors and other third parties, and injunctions against our processing or transferring of personal information necessary to operate our business. Some European regulators have ordered certain companies to suspend or permanently cease certain transfers of personal information to recipients outside Europe for allegedly violating the EU GDPR's cross-border data transfer limitations. Additionally, companies that transfer personal information to recipients outside of the EEA and/or UK to other jurisdictions, particularly to the United States, are subject to increased scrutiny from regulators, individual litigants, and activist groups.

In addition to any applicable privacy and data security laws and regulations, we may be subject to industry standards adopted by industry groups or bound by other contractual obligations related to privacy and data security. We may publish privacy policies, marketing materials, and other statements, such as compliance with certain certifications or self-regulatory principles, regarding data privacy and security. If these policies, materials, or statements are found to be deficient, lacking in transparency, deceptive, unfair, or misrepresentative of our practices, we may be subject to regulatory inquiries, regulatory enforcement actions and other adverse consequences.

Our obligations related to privacy and data security are quickly changing, becoming increasingly stringent, and creating regulatory uncertainty. Additionally, these obligations may be subject to differing applications and interpretations, which may be inconsistent between jurisdictions. Preparing for and complying with these obligations requires us to devote significant resources and may necessitate changes to our information technologies, systems, and practices and to those of any third parties that process personal information or other sensitive data on our behalf.

We may at times fail (or be perceived to have failed) in our efforts to comply with our privacy and data security obligations. Moreover, despite our efforts, our personnel or third parties on whom we rely on may fail to comply with such obligations, which could negatively impact our business operations. If we or the third parties that process personal information or other sensitive data our behalf fail, or are perceived to have failed, to address or comply with applicable privacy and data security obligations, we could face significant consequences, including but not limited to: government enforcement actions (e.g., investigations, fines, penalties, audits, and inspections); litigation (including class-action claims); additional reporting requirements and/or oversight; bans on processing personal information; and orders to destroy or not use personal information. Any of these events could have a material adverse effect on our reputation, business, or financial condition, including but not limited to loss of customers; significant reputational harm; an inability to process personal information or to operate in certain jurisdictions; limited ability to commercialize VYJUVEK or develop and commercialize our product candidates; expenditures of time and resources to defend ourselves against claims or inquiries; adverse publicity; or substantial changes to our business model or operations.

Unfavorable global economic conditions could adversely affect our business, financial condition or results of operations.

Our results of operations could be adversely affected by general conditions in the global economy and in the global financial markets, including high inflation and interest rates and concerns of a recession in the United States or other major markets due to a number of factors. For example, inflation and rising interest rates have caused volatility and disruptions in the capital and credit markets, and it is unclear how long such volatility will continue. In addition, Russia's invasion of Ukraine and/or the Israel-Hamas conflict may lead to a prolonged, adverse impact on global economic, sociopolitical and market conditions. A severe or prolonged economic downturn could result in a variety of risks to our business, including our ability to raise additional capital when needed or on acceptable terms, if at all. A weak or declining economy, sanctions, trade restrictions and other global conditions could also strain our suppliers, possibly resulting in supply delays or disruptions. Any of the foregoing could harm our business and we cannot anticipate all the ways in which the current economic climate and financial market conditions could adversely impact our business, financial condition, results of operations, and prospects.

Our internal computer systems, or those of any third-party with whom we do business may fail or suffer a cyber-security incident, such as a data breach or computer virus, which could harm our business by damaging our reputation, exposing us to liability, adversely impacting our revenue, or materially disrupting our operations, including production of VYJUVEK or our product development programs.

We rely on our information technology systems and infrastructure to manage our business. In addition, we receive, process, store, and transmit, often electronically, confidential data of others, including the participants in our clinical trials. Unauthorized access to our (or any third party with whom we do business, such as suppliers, distributors, manufacturers, or vendors) computer systems or stored data could result in the theft or improper disclosure of personal or confidential information or other sensitive data, the deletion or modification of records, or could cause interruptions in our operations. Cybersecurity threats include, but are not limited to, ransomware attacks, phishing attempts, and the exploitation of software vulnerabilities to gain access to our information technology environment, and cyber-security risks increase when we transmit information from one location to another, including transmissions over the Internet or other electronic networks. Despite our robust security measures and our commitment to implementing and continually improving our cybersecurity posture to mitigate the risk of a cybersecurity incident, we cannot guarantee that such incidents will not occur to us or any third-party with whom we do

business. For example, our specialty pharmacy provider was affected by a cybersecurity incident that delayed reimbursement approvals and had a negative impact on our product revenue, net for the first quarter of 2024. Any cybersecurity incident, even if promptly addressed, may harm our reputation, damage our brand, and erode trust. Our facilities and systems, and those of any third-party with whom we do business, may also be vulnerable to acts of vandalism, software viruses, misplaced or lost data, programming and/or human errors, or other similar events which may disrupt our operations or expose personal and confidential information.

Moreover, in the event of a cybersecurity incident, we may face investigations, legal actions, including class action litigation, regulatory inquiries, and regulatory enforcement actions. We may also be subject to fines, consent orders, or mandated corrective actions that could have a material adverse impact on our operations and financial position. Furthermore, cybersecurity incidents and their legal consequences may impact investor confidence, potentially leading to a decrease in our stock price or limitations on our access to capital markets. If such an event were to occur and cause material interruptions in our operations, it could result in a material disruption of our development programs and our business operations, whether due to a loss of our trade secrets or other proprietary information. For example, the loss of clinical trial data from completed or future clinical trials 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 confidential or proprietary information, we could incur liability, our competitive position could be harmed, and the further development and commercialization of our product candidates could be delayed.

Certain data breaches must be reported to affected individuals and various government and/or regulatory agencies, and in some cases to the media, under provisions of HIPAA, as amended by HITECH, other U.S. federal and state law, and requirements of non-U.S. jurisdictions, including the EU GDPR and relevant member state law in the European Union and other foreign laws, and financial penalties may also apply. Our insurance policies may not be adequate to compensate us for the potential losses arising from breaches, failures or disruptions of our infrastructure, catastrophic events and disasters or otherwise. In addition, such insurance may not be available to us in the future on economically reasonable terms, or at all. Further, our insurance may not cover all claims made against us and defending a suit, regardless of its merit, could be costly and divert management's attention. Any security breach involving the misappropriation, loss or other unauthorized disclosure or use of confidential information of others, whether by us or a third-party, could: (i) subject us to civil and criminal penalties; (ii) have a negative impact on our reputation; or (iii) expose us to liability to third parties or government authorities.

Our business continuity and disaster recovery plans may not adequately protect us from a serious disaster.

Natural disasters could severely disrupt our operations or the operations of third-party suppliers or service providers and have a material adverse effect on our business, financial condition, results of operations and prospects. The severity and frequency of weather-related natural disasters have been amplified, and are expected to continue to be amplified by, global climate change. Such natural disasters may cause damage to and/or disrupt our operations, which may result in a material adverse effect on our VYJUVEK sales, our other product candidates, business, and results of operations. Moreover, climate change may also result in various chronic physical changes, such as changes in temperature or precipitation patterns or sea-level rise, that could have an adverse impact on our operations. Our suppliers, vendors and business partners also face similar risks, and any disruption to their operations could have an adverse effect on our supply chain and manufacturing operations. If a natural disaster, power outage or other event occurred that prevented us from using all or a significant portion of our headquarters, that damaged critical infrastructure, such as our manufacturing facilities, or that otherwise disrupted operations, it may be difficult or, in certain cases, impossible for us to continue our business for a substantial period of time. The disaster recovery and business continuity plans that we have in place currently are limited and may not prove adequate in the event of a serious disaster or similar event. A significant portion of our current supply of drug product for VYJUVEK and our product candidates is located at our manufacturing facilities in Pittsburgh, Pennsylvania. We may incur substantial expenses as a result of the limited nature of our disaster recovery and business continuity plans, which could have a material adverse effect on our business, financial condition, results of operations and prospects.

Increased attention to, and evolving expectations for, environmental, social, and governance ("ESG") initiatives could increase our costs, harm our reputation, or otherwise adversely impact our business.

Companies across industries are facing increasing scrutiny from a variety of stakeholders related to their ESG and sustainability practices. Expectations regarding voluntary ESG initiatives and disclosures may result in increased costs (including but not limited to increased costs related to compliance, stakeholder engagement, contracting and insurance), enhanced compliance or disclosure obligations, or other adverse impacts to our business, financial condition, or results of operations.

While we may at times engage in voluntary initiatives (such as voluntary disclosures, certifications, or goals, among others) to improve the ESG profile of our company and/or VYJUVEK and our product candidates, such initiatives may be costly and may not have the desired effect. Moreover, we may not be able to successfully complete such initiatives due to factors that are within or outside of our control. Even if this is not the case, our actions may subsequently be determined to be

insufficient by various stakeholders, and we may be subject to investor or regulator engagement on our ESG efforts, even if such initiatives are currently voluntary.

Certain market participants, including major institutional investors and capital providers, use third-party benchmarks and scores to assess companies' ESG profiles in making investment or voting decisions. Unfavorable ESG ratings could lead to increased negative investor sentiment towards us or our industry, which could negatively impact our share price as well as our access to and cost of capital. Furthermore, certain investors and others have been engaged in "anti-ESG" campaigns, and, to the extent we take actions that are seen as positive to some investors other investors may take issue with such actions. To the extent ESG matters negatively impact our reputation, it may also impede our ability to compete as effectively to attract and retain employees or customers, which may adversely impact our operations.

In addition, we expect there will likely be increasing levels of regulation, disclosure-related and otherwise, with respect to ESG matters. For example, the SEC recently adopted new rules that require companies to provide significantly expanded climate-related disclosures in their periodic reporting. The new climate disclosure rules were the subject of multiple legal challenges, and the SEC voluntarily stayed the climate disclosure rules pending the completion of judicial review. Therefore, it is unknown whether the new rules will go into effect and if they do, whether there will be significant changes. If the new rules go into effect and are not substantially different than the rules adopted by the SEC, we will may be required to incur significant additional costs to comply, including the implementation of significant additional internal controls processes and procedures regarding matters that have not been subject to such controls in the past, and impose increased oversight obligations on our management and board of directors. These and other changes in stakeholder expectations will likely lead to increased costs as well as scrutiny that could heighten all of the risks identified in this risk factor. Additionally, our customers and suppliers may be subject to similar expectations, which may augment or create additional risks, including risks that may not be known to us.

Our international operations may expose us to business, regulatory, political, operational, financial, pricing and reimbursement and economic risks associated with doing business outside of the United States.

We currently have operations and employees located outside the United States and our business strategy incorporates potential additional international expansion to target patient populations outside the United States. Doing business internationally involves a number of risks, including, but not limited to:

- multiple, conflicting, and changing laws and regulations such as privacy regulations, tax laws, export and import restrictions, employment laws, regulatory requirements, and other governmental approvals, permits, and licenses;
- failure by us to obtain and maintain regulatory approvals for the use of our product candidates in various countries;
- additional potentially relevant third-party patent rights;
- complexities and difficulties in obtaining protection and enforcing our intellectual property;
- difficulties in staffing and managing foreign operations;
- complexities associated with managing multiple payor reimbursement regimes, government payors, or patient self-pay systems;
- limits in our ability to penetrate international markets;
- financial risks, such as longer payment cycles, difficulty collecting accounts receivable, the impact of local and regional financial crises on demand and payment for our products, and exposure to foreign currency exchange rate fluctuations;
- natural disasters, political and economic instability, including wars, terrorism, and political unrest, outbreak of disease, boycotts, curtailment of trade, and other business restrictions;
- certain expenses including, among others, expenses for travel, translation, and insurance; and
- regulatory and compliance risks that relate to maintaining accurate information and control over sales and activities that may fall within the purview of the U.S. Foreign Corrupt Practices Act, its books and records provisions, or its anti-bribery provisions.

Any of these factors could significantly harm our potential international expansion and operations and, consequently, our results of operations.

We are subject to U.S. and certain foreign export and import controls, anti-corruption laws and anti-money laundering laws and regulations. Compliance with these legal standards could impair our ability to compete in domestic and international markets. We can face criminal liability and other serious consequences for violations, which can harm our business.

We are subject to export control and import laws and regulations, including the U.S. Export Administration Regulations, U.S. Customs regulations, and various economic and trade sanctions regulations administered by the U.S. Treasury Department's Office of Foreign Assets Controls, and anti-corruption and anti-money laundering laws and regulations, including the U.S. Foreign Corrupt Practices Act of 1977, as amended, the U.S. domestic bribery statute contained in 18 U.S.C. § 201, the U.S. Travel Act, the USA PATRIOT Act, and other state and national anti-bribery and anti-money laundering laws in the countries in which we conduct activities. Anti-corruption laws are interpreted broadly and prohibit companies and their employees, agents, contractors and other collaborators and partners from authorizing, promising, offering, providing, soliciting, or receiving, directly or indirectly, improper payments or anything else of value to recipients in the public or private sector. We may engage third parties to sell VYJUVEK or our product candidates, if approved, abroad and/or to obtain necessary marketing authorizations, permits, licenses, patent registrations and other regulatory approvals. We may have direct or indirect interactions with officials and employees of government agencies or government-affiliated hospitals, universities, and other organizations. We can be held liable for the corrupt or other illegal activities of our employees, agents, contractors and other collaborators and partners, even if we do not explicitly authorize or have actual knowledge of such activities. Any violations of the laws and regulations described above may result in substantial civil and criminal fines and penalties, imprisonment, the loss of export or import privileges, debarment, tax reassessments, breach of contract and fraud litigation, reputational harm, and other adverse consequences.

Furthermore, U.S. export control laws and economic sanctions prohibit the provision of certain products and services to countries, governments, and persons targeted by U.S. sanctions.

The effect of pandemics, epidemics, outbreaks of infectious diseases, or similar public health crises on our operations and the operations of our customers, suppliers, third-party partners, and regulators could have an adverse impact our business.

Pandemics, epidemics, outbreaks of infectious diseases, or similar public health crises could adversely disrupt or impact our operations or those of our customers, suppliers, third-party partners, and regulators. In response to a pandemic or public health crisis, authorities may impose, and businesses and individuals may implement, numerous measures to try to contain the pandemic or public health crisis or treat its impact, such as travel bans and restrictions, quarantines, shelter-in-place/stay-at-home and social distancing orders, shutdowns, and vaccine requirements. In the event that such measures or similar measures or restrictions are implemented as a result of a pandemic or public health crisis, our employees conducting research and development or manufacturing activities may not be able to access our laboratory or manufacturing spaces, and our core activities may be significantly limited or curtailed, possibly for an extended period of time. In addition, the operations of our customers, suppliers, third-party partners, and regulators could be significantly limited or curtailed. Timely initiation and completion of clinical trials are essential to our business and clinical trials are dependent upon the availability of clinical trial sites, researchers and investigators, regulatory agency personnel, and materials, any of which may be adversely affected by public health crises, such as pandemics. The extent to which a health crisis may impact our business, results of operations and future growth prospects will depend on a variety of factors and future developments, which are highly uncertain and cannot be predicted with confidence, including the duration, scope, and severity of the public health crisis. A future public health crisis may have a material adverse effect on our business and results of operations.

Inadequate funding for the FDA and other government agencies, including from government shut downs, or other disruptions to these agencies' operations, could hinder their ability to hire and retain key leadership and other personnel, prevent new products and services from being developed or commercialized in a timely manner or otherwise prevent those agencies from performing normal business functions on which the operation of our business may rely, which could negatively impact our business.

The ability of the FDA to review and approve new products can be affected by a variety of factors, including government budget and funding levels, ability to hire and retain key personnel and accept the payment of user fees, and statutory, regulatory and policy changes. Disruptions at the FDA and other agencies may also slow the time necessary for new product candidates to be reviewed and/or approved by necessary government agencies, which could adversely affect our business. For example, when the U.S. government has shut down in the past, certain regulatory agencies, such as the FDA and the United States Securities and Exchange Commission have had to furlough critical employees and stop critical activities. If a prolonged government shutdown occurs, it could significantly impact the ability of the FDA to review and process our regulatory submissions in a timely manner, which could have a material adverse effect on our business. Further, future government shutdowns could impact our ability to access the public markets and obtain necessary capital. In addition, government funding of government agencies on which our operations may rely is subject to the political process, which is inherently fluid and unpredictable.

Risks Related to the Development, Regulatory Review and Approval of Our Product Candidates

If we are unable to advance our product candidates through clinical trials, obtain regulatory approval and ultimately commercialize our product candidates, or if we experience significant delays in doing so, our business will be materially harmed.

The development and commercialization of our product candidates are subject to many uncertainties, including the following:

- successful enrollment and completion of clinical trials;
- positive results from our current and planned clinical trials;
- receipt of regulatory approvals from applicable regulatory authorities;
- successful development of our internal manufacturing processes on an ongoing basis, including any required or desired changes to our manufacturing processes, and maintenance of our existing arrangements with third-party suppliers or manufacturers for clinical supply;
- commercial launch of our product candidates, if and when approved, whether alone or in collaboration with others; and
- acceptance of our product candidates, if and when approved, by patients, the medical community and third-party payors.

If we fail in one or more of these factors in a timely manner or at all, we could experience significant delays or an inability to successfully commercialize our product candidates, which would materially harm our business. If we do not receive regulatory approvals for our product candidates or changes to our manufacturing processes, our business, financial condition, results of operations and prospects could be materially and adversely affected.

Our gene therapy platform is based on a novel technology, which makes it difficult to predict the time and cost of obtaining regulatory approvals for our product candidates.

The clinical trial requirements of the FDA, EMA and other regulatory authorities and the criteria these regulators use to determine the safety and efficacy of a product candidate vary substantially according to the type, complexity, novelty and intended use and market of such product candidates. The regulatory approval process for novel product candidates such as ours, including approvals of or changes to manufacturing processes, can be more expensive and take longer than for other, better known or more extensively studied product candidates. It is difficult to determine how long it will take or how much it will cost to obtain regulatory approvals for our product candidates in the United States, the European Union, or elsewhere, or how long it will take to commercialize our product candidates. Approvals by the European Commission may not be indicative of what the FDA may require for approval and approval by the FDA may not be indicative of what the European Commission would require for approval.

Regulatory requirements and policy governing gene and cell therapy products have changed frequently and may continue to change in the future. In 2016, the FDA established the Office of Tissues and Advanced Therapies (“OTAT”) within its Center for Biologics Evaluation and Research to consolidate the review of gene therapy and related products, and has established the Cellular, Tissue and Gene Therapies Advisory Committee, among others, to advise this review. In September 2022, the FDA announced retitling of OTAT to the Office of Therapeutic Products (“OTP”) and elevation of OTP to a “Super Office” to meet its growing cell and gene therapy workload. If we engage a National Institutes of Health funded institution to conduct a clinical trial, that institution’s Institutional Biosafety Committee as well as its Institutional Review Board (“IRB”), would need to review the proposed clinical trial to assess the safety of the trial. Similarly, the EMA may issue new guidelines concerning the development and marketing authorization for gene therapy medicinal products and require that we comply with these new guidelines.

These regulatory review committees and advisory groups and the new guidelines they promulgate may lengthen the regulatory review process, require us to perform additional studies, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of our product candidates or lead to significant post-approval limitations or restrictions. These additional processes may result in a review and approval process that are longer than we otherwise would have expected. Delay or failure to obtain, or unexpected costs in obtaining, the regulatory approval necessary to bring a potential product to market could decrease our ability to generate sufficient product revenue, and our business, financial condition, results of operations and prospects would be materially and adversely affected.

Our product or product candidates may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval, limit the commercial potential, or result in significant negative consequences before or following any potential marketing approval.

There have been several significant adverse side effects in gene therapy trials using other vectors in the past. Gene therapy is still a relatively new approach to disease treatment and additional adverse side effects could develop. There also is the potential risk of delayed adverse events following exposure to gene therapy products due to persistent biologic activity of the genetic material or other components of products used to carry the genetic material.

In addition to side effects caused by our product candidates, the administration process or related procedures also can cause adverse side effects. If any such adverse events occur, our clinical trials could be suspended or terminated. If we are unable to demonstrate that such adverse events were caused by the administration process or related procedures and not by our product candidates, the FDA, the European Commission, the EMA, or other regulatory authorities could order us to cease further development of, or deny approval of, our product candidates for any or all targeted indications. Even if we can demonstrate that any serious adverse events are not product-related, such occurrences could affect patient recruitment or the ability of enrolled patients to complete the trial. Moreover, if we elect, or are required, to delay, suspend or terminate any clinical trial of our product candidates, the commercial prospects of such product candidates may be harmed and our ability to generate product revenue from the product candidates may be delayed or eliminated. Any of these occurrences may harm our ability to develop product candidates, and may harm our business, financial condition, and prospects significantly.

Additionally, if a product candidate receives marketing approval, the FDA could require us to adopt a post-approval safety monitoring program to ensure that the benefits outweigh its risks, which may include, among other things, a medication guide outlining the risks of the product for distribution to patients and a communication plan to health care practitioners. Furthermore, if we or others later identify undesirable side effects caused by VYJUVEK or our product candidates, several potentially significant negative consequences could result, including:

- regulatory authorities may suspend or withdraw approvals of VYJUVEK or our product candidates that may be approved;
- regulatory authorities may require additional warnings on the label;
- we may be required to change the way VYJUVEK or a product candidate is administered or conduct additional clinical trials;
- we could be sued and held liable for harm caused to patients; and
- our reputation may suffer.

Any of these events could prevent us from achieving or maintaining market acceptance of VYJUVEK or our product candidates and could significantly harm our business, financial condition, results of operations and prospects.

We may encounter substantial delays in our clinical trials, or we may fail to demonstrate safety and efficacy to the satisfaction of applicable regulatory authorities.

Before obtaining marketing approval from regulatory authorities for the sale of our product candidates, we must conduct extensive clinical trials to demonstrate the safety and efficacy of the product candidate for its intended indications. Obtaining marketing approval is an extensive, lengthy, expensive, and inherently uncertain process, and regulatory authorities may delay, limit, or deny approval of our product candidates for many reasons. We cannot guarantee that any clinical trials will be conducted as planned or completed on schedule, if at all. A failure of one or more clinical trials can occur at any stage of testing. Events that may prevent successful or timely completion of clinical trials include:

- delays in reaching a consensus with regulatory authorities on trial design;
- delays in opening sites and recruiting a sufficient number and diversity of suitable study subjects to participate in our clinical trials;
- imposition of a clinical hold by regulatory authorities as a result of a serious adverse event or concerns with a class of product candidates, or after an inspection of our clinical trial operations or trial sites;
- delays in having study subjects complete participation in a trial or return for post-treatment follow-up;
- occurrence of serious adverse events associated with the product candidate that are viewed to outweigh its potential benefits; or
- changes in regulatory requirements and guidance that require amending or submitting new clinical protocols.

The results of nonclinical and preclinical studies and early clinical trials may not be predictive of the results of later-stage clinical trials, and interim results of a clinical trial do not necessarily predict final results. In some instances, there can be

significant variability in safety or efficacy results between different clinical trials of the same product candidate due to numerous factors, including changes in trial procedures set forth in protocols, differences in the size and type of the study subject populations, changes in and adherence to the dosing regimen and other clinical trial protocols and the rate of dropout among clinical trial participants. In addition, preclinical and clinical data are often susceptible to various interpretations and analyses, and many companies that have believed their product candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain marketing approval. Product candidates in later stages of clinical trials may fail to show the desired safety and efficacy profile despite having progressed through nonclinical studies and initial clinical trials.

If we make manufacturing processes or formulation changes to our product or product candidates, we may need to conduct additional studies to bridge our modified product or product candidate to earlier versions and obtain regulatory approvals. Clinical trial delays could also shorten any periods during which we may have the exclusive right to commercialize our approved products or allow our competitors to bring products to market before we do, which could limit our potential revenue or impair our ability to successfully commercialize our approved products and may harm our business, financial condition, results of operations and prospects. Any delays, setbacks or failures in our clinical trials could materially and adversely affect our business, financial condition, results of operations and prospects.

Additionally, if the results of our clinical trials are inconclusive or if there are safety concerns or serious adverse events associated with our product candidates, we may:

- be delayed in obtaining marketing approval, if at all, or be required to conduct additional confirmatory safety and/or efficacy studies;
- obtain approval for indications or patient populations that are not as broad as intended or desired;
- obtain approval with labeling that includes significant use or distribution restrictions or safety warnings;
- obtain approval without labeling claims that are necessary or desirable for the successful commercialization of our product candidates;
- be subject to additional and costly post-marketing testing requirements or clinical trials;
- be required to perform additional clinical trials to support approval;
- have regulatory authorities withdraw, or suspend, their approval of the product or impose restrictions on its distribution;
- be subject to the addition of labeling statements, such as warnings, precautions, or contraindications;
- be sued; or
- experience damage to our reputation.

Our product development costs will also increase if we experience delays in testing or obtaining marketing approvals. We do not know whether any of our preclinical studies or clinical trials will begin as planned, need to be restructured or be completed on schedule, if at all.

Further, we, the FDA or an IRB, may suspend our clinical trials at any time if it appears that we or our collaborators are failing to conduct a trial in accordance with regulatory requirements, including the FDA's Current Good Clinical Practice, or CGCP, regulations, that we are exposing participants to unacceptable health risks, or if the FDA finds deficiencies in our IND, applications or the conduct of these trials. Therefore, we cannot predict with any certainty the schedule for commencement and completion of future clinical trials. If we experience delays in the commencement or completion of our clinical trials, or if we terminate a clinical trial prior to completion, the commercial prospects of our product candidates could be negatively impacted, and our ability to generate revenue from our product candidates may be eliminated or delayed.

We rely on third parties to conduct certain aspects of our preclinical studies and clinical trials. If these third parties do not successfully carry out their contractual duties, meet expected deadlines or comply with regulatory requirements, we may not be able to obtain regulatory approval for, or commercialize, our product candidates.

We depend upon third parties to conduct certain aspects of our preclinical studies and depend on third parties, including independent principal investigators, to conduct our clinical trials under agreements with universities, medical institutions, and others. We negotiate budgets and contracts with such third parties, which may result in delays to our development timelines and increased costs.

We rely on third parties over the course of our clinical trials, and, as a result, may have limited control over the clinical principal investigators and limited visibility into their day-to-day activities, including with respect to their compliance with the approved clinical protocol. Nevertheless, we are responsible for ensuring that each of our clinical trials is conducted in accordance with the applicable protocol, legal and regulatory requirements and scientific standards, and our reliance on third

parties does not relieve us of our regulatory responsibilities. We and these third parties are required to comply with CGCP requirements, which are regulations and guidelines enforced by the FDA and comparable foreign regulatory authorities for product candidates in clinical development. Regulatory authorities enforce these CGCP requirements through periodic inspections of clinical trial sponsors, clinical investigators, and clinical trial sites. If we or any of these third parties fail to comply with applicable CGCP requirements, the clinical data generated in our clinical trials may be deemed unreliable and the FDA or comparable foreign regulatory authorities may require us to suspend or terminate these clinical trials or perform additional preclinical studies or clinical trials before approving our marketing applications. We cannot be certain that, upon inspection, such regulatory authorities will determine that any of our clinical trials comply with CGCP requirements. In addition, our later-stage clinical trials must be conducted with product produced under CGMP requirements and may require a large number of study subjects.

Our failure or any failure by these third parties to comply with these regulations may require us to repeat clinical trials, which would delay the regulatory approval process. Moreover, our business may be adversely affected if any of these third parties violates federal or state fraud and abuse or false claims laws and regulations or healthcare privacy and security laws. Any third parties conducting aspects of our preclinical studies, or our clinical trials will not be our employees and, except for remedies that may be available to us under our agreements with such third parties, we cannot control whether or not they devote sufficient time and resources to our preclinical studies and clinical programs. These third parties may also have relationships with other commercial entities, including our competitors, for whom they may also be conducting clinical trials or other product development activities, which could affect their performance on our behalf. If these third parties do not successfully carry out their contractual duties or obligations or meet expected deadlines, if they need to be replaced or if the quality or accuracy of the preclinical or clinical data they obtain is compromised due to the failure to adhere to our protocols or regulatory requirements or for other reasons, our development timelines, including clinical development timelines, may be extended, delayed or terminated, and we may not be able to complete development of, obtain regulatory approval of, or successfully commercialize our product candidates. As a result, our financial results and the commercial prospects for our product candidates would be harmed, our costs could increase and our ability to generate revenue could be delayed or precluded entirely. Though we carefully manage our relationships with principal investigators and other third parties, there can be no assurance that we will not encounter challenges or delays or that these delays or challenges will not have a material adverse impact on our business, financial condition, and prospects.

Interim, "top-line," and preliminary data from our clinical trials that we announce or publish from time to time may change as more data becomes available or as additional analyses are conducted, and as the data are subject to audit and verification procedures that could result in material changes in the final data.

From time to time, we may publish interim, "top-line" or preliminary data from our clinical trials. Interim data from clinical trials that we may complete are subject to the risk that one or more of the clinical outcomes may materially change as study subject enrollment continues and more data becomes available. Preliminary or "top-line" data also remain subject to audit and verification procedures that may result in the final data being materially different from the preliminary data we previously published. As a result, interim and preliminary data should be viewed with caution until the final data are available. Material adverse changes between preliminary, "top-line" or interim data and final data could significantly harm our business, financial condition, results of operations and prospects.

Even if we obtain and maintain approval for our product candidates from the FDA, we may never obtain approval for them outside of the United States, which would limit our market opportunities and adversely affect our business.

Approval of a product candidate in the United States by the FDA does not ensure approval of such product candidate by regulatory authorities in other countries or jurisdictions, and approval by one foreign regulatory authority does not ensure approval by regulatory authorities in other foreign countries or by the FDA. Sales of VYJUVEK or our product candidates, if approved, outside of the United States will be subject to foreign regulatory requirements governing clinical trials and marketing approval. Even if the FDA grants marketing approval for a product candidate, comparable regulatory authorities of foreign countries also must approve the manufacturing and marketing of the product candidate in those countries and the process for obtaining such approval may be lengthy and expensive. Approval procedures vary among jurisdictions and can involve requirements and administrative review periods different from, and more onerous than, those in the United States, including additional preclinical studies or clinical trials. In many countries outside the United States, a product candidate must be approved for reimbursement before it can be approved for sale in that country. In some cases, the price that we intend to charge for our product candidates, if approved, is also subject to approval. Obtaining a Marketing Authorization Application ("MAA") from the European Commission following the opinion of the EMA is a lengthy and expensive process. Even if a product candidate is approved, the FDA or the European Commission, as the case may be, may limit the indications for which the product may be marketed, require extensive warnings on the product labeling or require expensive and time-consuming additional clinical trials or reporting as conditions of approval. Regulatory authorities in countries outside of the United States and the European Union also have requirements for approval of product candidates with which we must comply prior to marketing in those countries. Obtaining foreign regulatory approvals and compliance with foreign regulatory requirements

could result in significant delays, difficulties, and costs for us and could delay or prevent the introduction of our product candidates in certain countries.

Further, clinical trials conducted in one country may not be accepted by regulatory authorities in other countries. Also, regulatory approval for any of our product candidates may be withdrawn. If we fail to comply with the regulatory requirements, our target market will be reduced and our ability to realize the full market potential of our product candidates will be harmed and our business, financial condition, results of operations and prospects will be adversely affected.

VYJUVEK and our product candidates that receive marketing approvals remain subject to regulatory oversight even after regulatory approval. We will continue to incur costs related to regulatory compliance and are subject to risks related to non-compliance with or changes to applicable laws and regulations, which could cause VYJUVEK or any of our product candidates that obtain regulatory approval to lose that approval.

VYJUVEK, our first FDA-approved product, and any other product candidates that obtain regulatory approval in the future, will remain subject to ongoing regulatory requirements for manufacturing, labeling, packaging, storage, advertising, promotion, sampling, record-keeping and submission of safety and other post-market information. Any regulatory approvals that we receive for our product candidates may also be subject to a post-approval safety monitoring program, limitations on the approved indicated uses for which the product may be marketed or to the conditions of approval, or contain requirements for potentially costly post-marketing testing, including Phase 4 clinical trials, and surveillance to monitor the quality, safety, and efficacy of the product. For example, the holder of an approved BLA is obligated to monitor and report adverse events and any failure of a product to meet the specifications in the BLA. The holder of an approved BLA also must submit new or supplemental applications and obtain FDA approval for certain changes to the approved product, product labeling or manufacturing process. For example, if demand for an approved product increases more than we previously estimate, we may need or desire to scale up an existing FDA-approved manufacturing process and the scaled-up manufacturing process would be subject to FDA review and approval. Advertising and promotional materials must comply with FDA rules and are subject to FDA review, in addition to other potentially applicable federal and state laws.

In addition, product manufacturers and their facilities are subject to payment of user fees and continual review and periodic inspections by the FDA and other regulatory authorities for compliance with CGMP requirements and adherence to commitments made in the BLA or foreign marketing application. If we, or a regulatory authority, discover previously unknown problems with an approved product, such as adverse events of unanticipated severity or frequency, or problems with the facility where the product is manufactured or a regulatory authority disagrees with the promotion, marketing or labeling of that product, a regulatory authority may impose restrictions relative to that product, the manufacturing facility or us, including requiring recall or withdrawal of the product from the market or suspension of manufacturing.

If we fail to comply with applicable regulatory requirements, a regulatory authority may:

- issue a warning letter asserting that we are in violation of the law;
- seek an injunction or impose administrative, civil, or criminal penalties or monetary fines;
- suspend or withdraw regulatory approval;
- suspend any ongoing clinical trials;
- refuse to approve a pending BLA or comparable foreign marketing application (or any supplements thereto) submitted by us or our strategic partners, if any;
- restrict the marketing or manufacturing of the product;
- seize or detain the product or otherwise require the withdrawal of the product from the market;
- refuse to permit the import or export of product candidates; or
- refuse to allow us to enter into government contracts.

Any government investigation of alleged violations of law could require us to expend significant time and resources in response and could generate negative publicity. The occurrence of any event or penalty described above may inhibit our ability to commercialize our approved product, VYJUVEK, and product candidates and adversely affect our business, financial condition, results of operations and prospects.

The FDA's policies, and those of equivalent foreign regulatory agencies, may change and additional government regulations may be enacted that could negatively impact the existing marketing approval for VYJUVEK and prevent, limit, or delay regulatory approval of our product candidates. We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action, either in the United States or abroad. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to

maintain regulatory compliance, we may lose any marketing approval that we may have obtained, which would materially and adversely affect our business, financial condition, results of operations and prospects.

While we have obtained orphan drug exclusivity for VYJUVEK in the U.S., and orphan drug designation for certain of our product candidates in the U.S. and other jurisdictions, it may not effectively protect us from competition, and we may be unable to obtain orphan drug designation for other product candidates. If our competitors are able to obtain orphan drug exclusivity before us, we may not be able to have competing products approved by the applicable regulatory authority for a significant period of time.

Regulatory authorities in some jurisdictions, including the United States, the European Union, and Japan may designate drugs for relatively small patient populations as orphan drugs.

Under the Orphan Drug Act of 1983, as amended, the FDA may designate a product candidate as an orphan drug if it is intended to treat a rare disease or condition, which is generally defined as having a patient population of fewer than 200,000 individuals in the United States, or a patient population greater than 200,000 in the United States where there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the United States. Orphan drug designation does not convey any advantage in or shorten the duration of the regulatory review and approval process, but it can lead to financial incentives, such as opportunities for grant funding toward clinical trial costs, tax advantages and user-fee waivers. In addition, if a product that has orphan designation subsequently receives the first FDA approval for the disease or condition for which it has such designation, the drug is entitled to orphan drug marketing exclusivity for a period of seven years. Orphan drug marketing exclusivity generally prevents the FDA from approving another application to market the same drug or biological product for the same disease or condition for seven years, except in limited circumstances, including if the FDA concludes that the later drug is safer, more effective or makes a major contribution to patient care. A designated orphan drug may not receive orphan drug marketing exclusivity if it is approved for a use that is broader than the indication for which it received orphan designation. Orphan drug marketing exclusivity rights in the United States 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 quantity of the drug to meet the needs of patients with the rare disease or condition.

In the European Union, the European Commission, upon a recommendation from the EMA's Committee for Orphan Medicinal Products, grants orphan drug designation to promote the development of products that are intended for the diagnosis, prevention, or treatment of a life-threatening or chronically debilitating condition affecting not more than 5 in 10,000 persons in the EU. Additionally, orphan designation is granted for products intended for the diagnosis, prevention, or treatment of a life-threatening, seriously debilitating, or serious and chronic condition and when, without incentives, it is unlikely that sales of the drug in the European Union would be sufficient to justify the necessary investment in developing the drug or biologic product. In the European Union, orphan medicinal product designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process, but orphan drug designation may entitle an applicant to financial incentives such as reduction of fees or fee waivers, protocol assistance, and access to the centralized marketing authorization procedure. Upon grant of a marketing authorization, orphan products are entitled to ten years of market exclusivity for the approved therapeutic indication, which means that the EMA and European Commission cannot accept another marketing authorization application, grant a marketing authorization, or accept an application to extend a marketing authorization for a similar product for the same indication for a period of ten years. The period of market exclusivity is extended by two years for orphan medicinal products that have also complied with an agreed Pediatric Investigation Plan, or PIP. The ten-year market exclusivity in the European Union may be reduced to six years if, at the end of the fifth year, it is established that the product no longer meets the criteria for which it received orphan designation, including where it is shown that the product is sufficiently profitable not to justify maintenance of market exclusivity, or where the prevalence of the condition has increased above the threshold. Additionally granting of an authorization for another similar orphan medicinal product where another product has market exclusivity can happen at any time: (i) the second applicant can establish that its product, although similar, is safer, more effective, or otherwise clinically superior; (ii) the applicant cannot supply enough orphan medicinal product, or (iii) where the applicant consents to a second orphan medicinal product application.

The orphan drug designation system in Japan aims to support the development of drugs for diseases that affect fewer than 50,000 patients in Japan, for which significant unmet medical need exists. An investigational therapy is eligible to qualify for orphan drug designation in Japan if there is no approved alternative treatment option or if there is high efficacy or safety compared to existing treatment options expected. Specific measures to support the development of orphan drugs in Japan include subsidies for research and development expenditures, prioritized consultation regarding clinical development, reduced consultation fees, tax incentives, priority review of applications, reduced application fees, and extended registration validity period. Up to 10 years of orphan exclusivity, known as the re-examination period, is granted for the product after approval. The orphan drug exclusivity may be rescinded by the Japanese government in certain circumstances.

Even though we have obtained orphan drug exclusivity for VYJUVEK in the United States; orphan drug designation for B-VEC in the European Union and Japan; orphan drug designation for KB105 and KB407 in the United States and the European Union; and orphan drug designation for KB408 in the United States, we cannot assure you that we will be able to

obtain or maintain orphan drug exclusivity and if we are able to maintain the orphan drug exclusivity, the exclusivity may not effectively protect the product from competition because different drugs can be approved for the same condition. Further, we cannot assure you that any of our other product candidates will be approved for any orphan-designated use in any jurisdiction, in a timely manner or at all, or that a competitor will not obtain orphan drug exclusivity that could block the regulatory approval of any of our drug candidates for several years. If we are unable to maintain or obtain orphan drug exclusivity, our ability to generate sufficient revenue may be negatively affected. If a competitor is able to obtain orphan drug exclusivity that would block our product candidates' regulatory approval, our ability to generate revenue could be significantly reduced, which would harm our business prospects, financial condition and results of operations. We do not know if, when, or how the FDA or other regulators may change the applicable orphan drug regulations and policies in the future, and it is uncertain how any changes might affect our business. Depending on what changes may be made to orphan drug regulations and policies, our business could be adversely impacted.

Accelerated approval by the FDA, even if granted for any of our product candidates, may not lead to a faster development or regulatory review or approval process and it does not increase the likelihood that our product candidates will receive marketing approval.

We may seek approval of our current or future product candidates using the FDA's accelerated approval pathway. This pathway may not lead to a faster development, regulatory review or approval process and does not increase the likelihood that our product candidates will receive marketing approval. A product may be eligible for accelerated approval if it treats a serious or life-threatening condition, generally provides a meaningful advantage over available therapies, and demonstrates an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit. As a condition of approval, the FDA may require that a sponsor of a product receiving accelerated approval perform adequate and well-controlled post-marketing confirmatory clinical trials. These confirmatory trials must be completed with due diligence. Under the Food and Drug Omnibus Reform Act of 2022, or FDORA, the FDA is permitted to require, as appropriate, that a post-approval confirmatory trial or trials be underway prior to approval or within a specified time after the date accelerated approval was granted. FDORA also requires sponsors to send updates to the FDA every 180 days on the status of such studies, including progress toward enrollment targets, and the FDA must promptly post this information publicly. Furthermore, under FDORA, the FDA is empowered to take action, such as issuing fines, against companies that fail to conduct with due diligence any post-approval confirmatory trial or submit timely reports to the agency on their progress. In addition, for products under consideration for accelerated approval, the FDA currently requires, unless otherwise requested by the agency, pre-approval of promotional materials intended for dissemination or publication within 120 days of marketing approval be submitted to the agency for review during the review period, which could adversely impact the timing of the commercial launch of the product. There can be no assurance that the FDA would allow any of our product candidates to proceed on an accelerated approval pathway, and even if the FDA did allow such pathway, there can be no assurance that any expedited development, review, or approval will be granted on a timely basis, or at all.

Breakthrough Therapy Designation, Fast Track Designation, Regenerative Medicine Advanced Therapy Designation or Priority Review by the FDA, or PRIME Scheme by the EMA, even if granted for any of our product candidates, may not lead to a faster development, regulatory review or approval process, and such designations may not increase the likelihood that any of our product candidates will receive marketing approval.

We may seek a Breakthrough Therapy Designation for some of our product candidates. A breakthrough therapy is defined as a therapy that is intended, alone or in combination with one or more other therapies, to treat a serious or life-threatening disease or condition, and preliminary clinical evidence indicates that the therapy may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. For therapies that have been designated as breakthrough therapies, interaction and communication between the FDA and the sponsor of the trial can help to identify the most efficient path for clinical development while minimizing the number of patients placed in ineffective control regimens. Therapies designated as breakthrough therapies by the FDA may also be eligible for priority review and accelerated approval. Designation as a breakthrough therapy is within the discretion of the FDA. Accordingly, even if we believe one of our product candidates meets the criteria for designation as a breakthrough therapy, the FDA may disagree and instead determine not to make such designation. In any event, the receipt of a Breakthrough Therapy Designation for a product candidate may not result in a faster development process, review or approval compared to therapies considered for approval under conventional FDA procedures and does not assure ultimate approval by the FDA. In addition, even if one or more of our product candidates qualify as breakthrough therapies, the FDA may later decide that such product candidates no longer meet the conditions for qualification or decide that the time for FDA review or approval will not be shortened.

We have obtained and may seek Fast Track Designation for some of our product candidates. For instance, VYJUVEK, KB105, and KB707 (intratumoral and inhaled) were granted Fast Track Designation by the FDA. If a therapy is intended for the treatment of a serious or life-threatening condition and the therapy demonstrates the potential to address unmet medical needs for this condition, the sponsor may apply for Fast Track Designation. The FDA has broad discretion whether or not to grant this

designation, so even if we believe a particular product candidate is eligible for this designation, we cannot assure you that the FDA would decide to grant it. Even if we do receive Fast Track Designation, we may not experience a faster development process, review or approval compared to conventional FDA procedures. For products that receive Fast Track Designation, sponsors may have greater interactions with the FDA and the FDA may initiate review of sections of the marketing application before the application is complete. This rolling review may be available if the FDA determines, after preliminary evaluation of clinical data submitted by the sponsor, that a Fast Track product may be effective. The sponsor must also provide, and the FDA must approve, a schedule for the submission of the remaining information and the sponsor must pay applicable user fees. However, the FDA's time period goal for reviewing an application does not begin until the last section of the application is submitted. The FDA may withdraw Fast Track Designation if it believes that the designation is no longer supported by data from clinical programs. Many biologics that have received Fast Track Designation have failed to obtain marketing approval. Fast Track Designation alone does not guarantee qualification for the FDA's priority review procedures.

We were granted Regenerative Medicine Advanced Therapy ("RMAT") designation for B-VEC from the FDA, and we may seek RMAT designation for some of our product candidates. In 2017, the FDA established the RMAT designation as part of its implementation of the 21st Century Cures Act to expedite review of any drug that meets the following criteria: it qualifies as a RMAT, which is defined as a cell therapy, therapeutic tissue engineering product, human cell and tissue product, or any combination product using such therapies or products, with limited exceptions; it is intended to treat, modify, reverse, or cure a serious or life-threatening disease or condition; and preliminary clinical evidence indicates that the drug has the potential to address unmet medical needs for such a disease or condition. Like Breakthrough Therapy Designation, RMAT designation provides potential benefits that include more frequent meetings with the FDA to discuss the development plan for the product candidate, and eligibility for rolling review and priority review. Products granted RMAT designation may also be eligible for accelerated approval on the basis of a surrogate or intermediate endpoint reasonably likely to predict long-term clinical benefit, or reliance upon data obtained from a meaningful number of sites, including through expansion to additional sites. RMAT-designated products that receive accelerated approval may, as appropriate, fulfill their post-approval requirements through the submission of clinical evidence, clinical trials, patient registries, or other sources of real-world evidence, such as electronic health records; through the collection of larger confirmatory data sets; or via post-approval monitoring of all patients treated with such therapy prior to approval of the therapy. There is no assurance that we will be able to obtain RMAT designation for our product candidates. RMAT designation does not change the FDA's standards for product approval, and there is no assurance that such designation will result in expedited review or approval or that the approved indication will not be narrower than the indication covered by the designation. Additionally, RMAT designation can be revoked if the criteria for eligibility cease to be met as clinical data emerges.

If the FDA determines that a product candidate offers a treatment for a serious condition and, if approved, the product would provide a significant improvement in safety or effectiveness, the FDA may designate the product candidate for priority review. A priority review designation means that the goal for the FDA to review an application is six months, rather than the standard review period of ten months. The FDA has broad discretion with respect to whether or not to grant priority review status to a product candidate, so even if we believe a particular product candidate is eligible for such designation or status, the FDA may decide not to grant it. Moreover, a priority review designation does not necessarily result in an expedited regulatory review or approval process or necessarily confer any advantage with respect to approval compared to conventional FDA procedures. Receiving priority review from the FDA does not guarantee approval within the six-month review cycle, or at all.

We have obtained and may seek to qualify our product candidates under the PRIority MEdicines ("PRIME") scheme from the EMA. For instance, VYJUVEK was granted PRIME designation. The PRIME scheme is open to medicines under development and for which the applicant intends to apply for an initial MAA through the centralized procedure. Eligible products must target conditions for which where is an unmet medical need (there is no satisfactory method of diagnosis, prevention or treatment in the European Union or, if there is, the new medicine will bring a major therapeutic advantage) and they must demonstrate the potential to address the unmet medical need by introducing new methods or therapy or improving existing ones. There is no assurance that we will be able to obtain PRIME qualification for our product candidates. PRIME does not change the standards for product approval, and there is no assurance that such qualification will result in expedited review or approval. Moreover, where, during the course of development, a product no longer meets the eligibility criteria, support under the PRIME scheme may be withdrawn.

We have obtained a rare pediatric disease designation for certain of our product candidates; however, there is no guarantee that FDA approval will result in issuance of a priority review voucher.

In 2012, Congress authorized the FDA to award priority review vouchers to sponsors of certain rare pediatric disease product applications. This program is designed to encourage development of new drug and biological products for prevention and treatment of certain rare pediatric diseases. Specifically, under this program, a sponsor that receives an approval for a drug or biologic for a "rare pediatric disease" that meets certain criteria may qualify for a voucher that can be redeemed to receive a priority review of a subsequent marketing application for a different product. The sponsor of a rare pediatric disease drug product receiving a priority review voucher may transfer (including by sale) the voucher to another sponsor. The voucher may

be further transferred any number of times before the voucher is used, as long as the sponsor making the transfer has not yet submitted the application. The FDA may also revoke any priority review voucher if the rare pediatric disease drug for which the voucher was awarded is not marketed in the United States within one year following the date of approval. We received rare pediatric disease designation for VYJUVEK and were awarded a priority review voucher following FDA approval of VYJUVEK in May 2023. The priority review voucher was sold in August 2023. We have also obtained a rare pediatric disease designation for KB105, KB104, and for KB407. In addition, in May 2024, intratumoral KB707 was also granted rare pediatric disease designation by the FDA for the treatment of osteosarcoma. However, there is no guarantee that we will be able to obtain a priority review voucher if these product candidates are approved by the FDA. Congress included a sunset provision in the statute authorizing the rare pediatric disease priority review voucher program. Under the current statutory sunset provisions, after September 30, 2024, the FDA may only award a voucher for an approved rare pediatric disease product application if the sponsor has rare pediatric disease designation for the product candidate, and that designation was granted by September 30, 2024. After September 30, 2026, the FDA may not award any rare pediatric disease priority review vouchers.

We may seek designation for our platform technology as a designated platform technology, but we might not receive such designation, and even if we do, such designation may not lead to a faster development or regulatory review or approval process.

We may seek designation for our platform technology as a designated platform technology. Under the Food and Drug Omnibus Reform Act of 2022, or FDORA, a platform technology incorporated within or utilized by a drug or biologic is eligible for designation as a designated platform technology if (1) the platform technology is incorporated in, or utilized by, a product approved under a New Drug Application, or NDA, or BLA; (2) preliminary evidence submitted by the sponsor of the approved product, or a sponsor that has been granted a right of reference to data submitted in the application for such product, demonstrates that the platform technology has the potential to be incorporated in, or utilized by, more than one product without an adverse effect on quality, manufacturing, or safety; and (3) data or information submitted by the sponsor indicates that incorporation or utilization of the platform technology has a reasonable likelihood to bring significant efficiencies to the product development or manufacturing process and to the review process. A sponsor may request the FDA to designate a platform technology as a designated platform technology concurrently with, or at any time after, submission of an IND application for a product that incorporates or utilizes the platform technology that is the subject of the request. If so designated, the FDA may expedite the development and review of any subsequent NDA or BLA for a product that uses or incorporates the platform technology. Even if we believe our platform technology meets the criteria for such designation, the FDA may disagree and instead determine not to grant such designation. In addition, the receipt of such designation for a platform technology does not ensure that our applicable product candidates will be developed more quickly or receive a faster FDA review process or ultimate FDA approval. Moreover, the FDA may revoke a designation if the FDA determines that a designated platform technology no longer meets the criteria for such designation.

Risks Related to Manufacturing

Delays in obtaining regulatory approvals of the process, or changes to the process, and facilities needed to manufacture VYJUVEK or our product candidates or disruptions in our manufacturing process may disrupt our production of VYJUVEK or delay or disrupt our development and commercialization efforts with respect to our product candidates.

Before we can begin to commercially manufacture our product candidates, we must pass a pre-approval inspection of our manufacturing facilities by the FDA. A manufacturing authorization must also be obtained from the appropriate EU regulatory authorities. The timeframe required for us to obtain such approvals is uncertain. To obtain approval, we need to ensure that all our processes, methods and equipment are compliant with CGMP, and perform extensive audits of vendors, contract laboratories, and suppliers. If any of our vendors, contract laboratories, or suppliers is found to be out of compliance with CGMP, we may experience delays or disruptions in manufacturing while we work with these third parties to remedy the violation or while we work to identify suitable replacement vendors, contract laboratories, or suppliers. The CGMP requirements govern quality control of the manufacturing process and documentation policies and procedures. In complying with CGMP, we are obligated to expend time, money and effort in production, record keeping and quality control to assure that the product meets applicable specifications and other requirements. If we fail to comply with these requirements, we will be subject to possible regulatory action and may not be permitted to sell any approved product that we may develop.

In addition, the manufacturing process used to produce VYJUVEK and our product candidates is complex and novel and demand for an approved product may require the need for us to change the manufacturing process, which may require regulatory approval before we are able to sell the product manufactured by the changed process. The production of VYJUVEK and our product candidates require processing steps that are more complex than those required for most chemical pharmaceuticals. Moreover, unlike chemical pharmaceuticals, the physical and chemical properties of a biologic such as ours generally cannot be fully characterized. As a result, assays of the finished product may not be sufficient to ensure that the product will perform in the intended manner. Accordingly, we employ multiple steps to control our manufacturing process to assure that the process works and that VYJUVEK and our product candidates are made strictly and consistently in compliance with the process. Problems with an approved manufacturing process, even minor deviations from the normal process, could

result in product defects or manufacturing failures that result in lot failures, product recalls, product liability claims or insufficient inventory. In addition to the potential impacts of problems with an approved manufacturing process, changes to an approved manufacturing process may result in problems with the process design, process reproducibility, stability, or batch consistency, and may require regulatory approval before we are permitted to sell products manufactured with the changed manufacturing process, which could potentially delay commercial availability of an approved product. We may encounter problems achieving adequate quantities and quality of materials that meet FDA, EMA or other applicable standards or specifications with consistent and acceptable production yields and costs.

Although we have established our own manufacturing facilities for VYJUVEK and our product candidates, we may also utilize third parties to conduct our product manufacturing or components thereof. We are also dependent on a limited number of third-party suppliers for some of the components and materials used in manufacturing VYJUVEK and our product candidates and in commercially supplying VYJUVEK. Therefore, we are subject to the risk that these third parties may not perform satisfactorily.

We may maintain third-party manufacturing capabilities in order to provide multiple sources of supply of VYJUVEK or a product candidate that is approved for sale. In addition, we may utilize third parties to manufacture components of VYJUVEK or our product candidates. For example, we use a third-party to manufacture the sterile gel that is mixed with our in-house produced vector for VYJUVEK. Our ability to commercially supply VYJUVEK depends, in part, on the ability of third parties to supply and manufacture the raw materials and other important components related to our manufacture of VYJUVEK. For some materials and components related to our manufacture of VYJUVEK and our product candidates, there are, in general, relatively few alternative sources of supply, and our use of a limited number of suppliers for some of the components and materials used in manufacturing VYJUVEK and our product candidates and commercially supplying VYJUVEK exposes us to several risks, including disruptions in supply, price increases, late deliveries and an inability to meet demand. If we fail to develop and maintain supply relationships with these third parties, we may be unable to successfully commercialize VYJUVEK or any approved product candidate. Any of our existing suppliers may:

- fail to supply us 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 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 materials that fail to meet regulatory requirements;
- become unavailable through business interruption or financial insolvency;
- lose regulatory status as an approved supply source;
- be unable or unwilling to (i) honor current supply agreements or (ii) renew current supply agreements when such agreements expire on a timely basis, on acceptable terms or at all; or
- discontinue production or manufacturing of materials that we acquire through such third-party supplier.

In the event of any of the foregoing, if we do not have an alternative supplier or manufacturer in place, we may not be able to manufacture our products for commercial, regulatory, or clinical purposes and would be required to expend substantial management time and expense to identify, qualify and transfer to alternative suppliers or manufacturers. There can be no assurance that replacements would be available to us on a timely basis, on acceptable terms, or at all. Any need to find and qualify new suppliers or manufacturers could significantly delay production of VYJUVEK or any product candidate, if approved, adversely impact our ability to market VYJUVEK or any product candidate, if approved, and have a material adverse effect on our business, financial condition, results of operations and prospects.

If we or a third-party supplier or manufacturer fails to comply with applicable CGMP regulations, the FDA and foreign regulatory authorities can impose regulatory sanctions including, among other things, refusal to approve a pending application for a new product candidate or suspension or revocation of a pre-existing approval. Such an occurrence may cause our business, financial condition, results of operations and prospects to be materially harmed.

Any contamination in, or changes to, our manufacturing process, shortages of raw materials or failure of any of our key suppliers to deliver necessary components could result in delays in our ability to produce VYJUVEK or any other approved product for commercial supply or any product candidate for clinical development.

Given the nature of biologics manufacturing, there is a risk of contamination. Any contamination could materially adversely affect our ability to produce VYJUVEK or our product candidates on schedule and could, therefore, harm our results of operations and cause reputational damage.

Some of the raw materials required in our manufacturing process are derived from biologic sources. Such raw materials are difficult to procure and may be subject to contamination or recall. A material shortage, contamination, recall or restriction on the use of biologically derived substances in the manufacture of VYJUVEK or our product candidates could adversely impact or disrupt the commercial manufacturing or the production of clinical material, which could materially and adversely affect our development timelines and our business, financial condition, results of operations and prospects.

Failure to increase manufacturing capacity and at higher yields in a timely or cost-effective manner, or at all, to sufficiently meet our commercial needs could result in delays in commercial availability of an approved product. If a manufacturing process is approved by the FDA, implementing a new or changed manufacturing processes is difficult, time consuming, and would require regulatory approvals, which could potentially delay commercial availability of an approved product, which, in turn, could harm our results of operations and cause reputational damage.

Our failure to maintain or continuously improve our quality management program could have an adverse effect upon our business, subject us to regulatory actions and cause patients to lose confidence in us or our products, among other negative consequences.

Quality management plays an essential role in the manufacturing of VYJUVEK and our product candidates, conducting clinical trials, preventing defects, improving our product candidates, and assuring the safety and efficacy of VYJUVEK and our product candidates. We seek to maintain a robust quality management program which includes the following broad pillars of quality:

- monitoring and assuring regulatory compliance for clinical trials, manufacturing, and testing of good applicable practice ("GxP") (e.g., CGCP and CGMP regulated) products;
- monitoring and providing oversight of all GxP suppliers;
- establishing and maintaining an integrated, robust quality management system for clinical, manufacturing, supply chain and distribution operations; and
- cultivating a proactive, preventative quality culture and employee and supplier training to ensure quality.

Our success depends on our ability to maintain and continuously improve our quality management program. Any change to an approved manufacturing process will put strain on our quality management program. A quality or safety issue may result in adverse inspection reports, warning letters, monetary sanctions, injunctions to halt manufacture and distribution of VYJUVEK or our product candidates, if approved, civil or criminal sanctions, costly litigation, refusal of a government to grant approvals and licenses, restrictions on operations, or withdrawal, suspension or variation of existing approvals and licenses. An inability to address a quality or safety issue in an effective and timely manner may also cause negative publicity, or a loss of patient confidence in us or VYJUVEK or our product candidates, which may result in difficulty in successfully launching products and the loss of potential future sales, which could have an adverse effect on our business, financial condition, and results of operations.

If VYJUVEK demand increases more than previously estimated or we wish to improve manufacturing efficiencies or to lower cost of production, we may need or choose to scale up the current FDA-approved VYJUVEK commercial manufacturing process, which is subject to risks and uncertainties and will require us to submit a Prior Approval Supplement ("PAS") to the FDA and obtain the agency's approval for the manufacturing process changes before they can be implemented.

We may desire or need to make manufacturing process changes to scale-up manufacturing to meet increased demand, to improve efficiencies or costs, or otherwise. Scaling up a manufacturing process carries regulatory, financial, and operational risks, which could potentially impact product availability. For example, as a result of the strong and increasing commercial demand for VYJUVEK, we designed a revised commercial manufacturing process that should more than quadruple the output of each production batch. Before we can commercially sell VYJUVEK manufactured with the revised commercial manufacturing process, the change to the manufacturing process must be validated and we need to preparee and submit to the FDA a PAS, which must be approved by the FDA. In addition to requiring FDA approval of a PAS, there are risks associated with scaling up a manufacturing process, including, among others, cost overruns, potential problems with the process scale-up design, process reproducibility, stability issues, and batch consistency. Furthermore, studies or tests to demonstrate comparability of product manufactured under an existing and under a revised manufacturing process, or any other studies on a revised process, such as validation studies, may uncover findings that result in the FDA delaying or refusing to approve a new process. We cannot guarantee when the FDA will approve the PAS for the scaled-up VYJUVEK manufacturing process. Until an enhanced VYJUVEK manufacturing process is approved by the FDA, we will need to meet any additional demand for VYJUVEK from batches manufactured with our existing FDA-approved commercial manufacturing process, which could potentially delay the commercial availability of VYJUVEK and adversely affect our business, results of operations, and financial condition.

We may need or desire to transfer VYJUVEK or an approved product candidate manufacturing from ANCORIS, our commercial scale CGMP-compliant manufacturing facility where VYJUVEK is currently manufactured to ASTRA, our recently completed and qualified state-of-the-art CGMP manufacturing facility, or transfer an approved product manufacturing from ASTRA to ANCORIS, and technical transfer of a manufacturing process is subject to risks and uncertainties and requires FDA inspection and approval of the facility where manufacturing is planned to be transferred.

We plan to complete a technical transfer process to allow us to commercially manufacture VYJUVEK at ASTRA, our recently completed and qualified state-of-the-art CGMP manufacturing facility, in addition to ANCORIS. This process may be time consuming and will require an FDA inspection of ASTRA. We cannot provide any assurance of the timing of such FDA approval, or if the FDA will approve commercial manufacturing of VYJUVEK at ASTRA. We have never completed a technical transfer process to an in-house manufacturing facility, and there is no guarantee that we will be successful doing so. Failure or delay in technical transfer of VYJUVEK or another approved product from ANCORIS to ASTRA, or from ASTRA to ANCORIS, could impair our ability to supply sufficient product to meet commercial demand and successfully commercialize and generate revenue from sales of approved products, which could adversely affect our business, financial condition, and results of operations.

Risks Related to Commercialization of VYJUVEK and Our Product Candidates

We have limited experience as a commercial company and the sales, marketing, and distribution of VYJUVEK or any future approved products may be unsuccessful or less successful than anticipated.

We received FDA approval of VYJUVEK in May 2023 and initiated a commercial launch of VYJUVEK in the United States in the second quarter of 2023. As a company, we have no prior experience commercializing a biologic. The success of our commercialization efforts is difficult to predict and subject to the effective execution of our business plan, including, among other things, the continued development of our internal sales, marketing, manufacturing, and distribution capabilities and our ability to navigate the significant expenses and risks involved with the development and management of such capabilities. For example, our commercial launch of VYJUVEK may not develop as planned or anticipated, which may require us to, among others, adjust or amend our business plan, including scale up of our manufacturing process, and incur significant expenses. Further, given our lack of experience commercializing products, we do not have a track record of successfully executing a commercial launch. There is a risk that we underestimate the level of demand for a product, which could require us to change a manufacturing process to increase production yields and changes to a manufacturing process are time consuming and subject to regulatory, financial, and operational risks. If we are unsuccessful in accomplishing our objectives and executing on our business plan, or if our commercialization efforts do not develop as planned, we may not be able to successfully commercialize VYJUVEK and any future approved products, we may require significant additional capital and financial resources, we may not become profitable on a consistent basis, and we may not be able to compete against more established companies in our industry.

If we are unable to maintain our agreements with third parties to distribute VYJUVEK to patients in the United States, our results of operations and business could be adversely affected.

We rely on a small number of third parties to commercially distribute VYJUVEK to patients in the United States. We have contracted with a third-party packaging company to package VYJUVEK, a third-party logistics company to warehouse, process, and ship VYJUVEK to a limited number of specialty pharmacies that mix the medication and administer it to patients in the patient's home by a healthcare professional and a specialty distributor that distributes VYJUVEK to hospitals and outpatient clinics where patients are administered the medication at a healthcare professional's office. This distribution network requires significant coordination with our sales and marketing and finance organizations. In addition, failure to coordinate financial systems could negatively impact our ability to accurately report product revenue from VYJUVEK. If we are unable to effectively manage the distribution process, the sales of VYJUVEK could be compromised and our results of operations may be harmed.

If the third parties involved in the commercial distribution of VYJUVEK in the United States do not fulfill their contractual obligations to us or refuse or fail to adequately or to properly distribute VYJUVEK and serve patients, or the agreements with them are terminated without adequate notice, shipments of VYJUVEK, and associated revenue, could be adversely affected. In addition, if we were required to replace such third-parties, it could take time to locate an appropriate replacement third-party on acceptable terms, which could cause delays in our distribution network and increased expenses, and thereby adversely impact our commercial sales of VYJUVEK in the United States and result in a material adverse effect on our business, financial condition, results of operations, and prospects.

We plan on using local distributors to market and sell VYJUVEK in certain jurisdictions outside of the U.S., the U.K., certain EU countries, and Japan, which subjects us to certain risks.

We plan on using local distributors to market and sell VYJUVEK outside of the U.S., the U.K., certain EU countries, and Japan. We may be unable to enter into appropriate supply, marketing, and distribution arrangements on favorable terms, if at all. Our use of distributors in these markets to market and sell VYJUVEK involves certain risks, including, but not limited to, risks that these organizations will not comply with applicable laws and regulations, not effectively sell or support VYJUVEK or

reduce or discontinue their efforts to sell or support VYJUVEK, not devote the resources necessary to market and sell VYJUVEK in the volumes and within the time frame we expect, not be able to satisfy financial obligations to us or others, not provide us with accurate or timely information regarding their inventories of VYJUVEK or the number of patients who are using VYJUVEK, or not provide us with accurate or timely information regarding serious adverse events and/or product complaints. Any such events may result in regulatory actions that may include suspension or termination of the distribution and sale of our products in a certain country, loss of revenue, and/or reputational damage, which could harm our results of operations and business.

In connection with the commercial launch of VYJUVEK in the United States, we recruited a sales force and established marketing, market access and medical affairs teams and distribution capabilities and if the commercial launch of VYJUVEK is not successful for any reason, we could incur substantial costs and our investment would be lost if we cannot retain or reposition our sales, marketing, market access and medical affairs personnel.

To achieve commercial success for VYJUVEK, we have devoted and anticipate that we will continue to devote significant resources to support our sales force, marketing, market access, and medical affairs teams and distribution capabilities. There are risks involved with establishing our own sales, marketing, distribution, training, and support capabilities. For example, recruiting and training sales and marketing personnel is expensive and time-consuming and could delay our ability to focus on other priorities. If the commercial launch of VYJUVEK in the United States is not successful for any reason, this would be costly, and our investment would be lost if we cannot retain or reposition our sales, marketing, market access and medical affairs personnel or terminate on favorable terms any agreements entered into with third parties to support our commercialization efforts.

Factors that may inhibit our efforts to commercialize VYJUVEK or any other product candidates, if approved, on our own in the United States or elsewhere include:

- our inability to train and retain adequate numbers of effective sales, marketing, training, and support personnel;
- the inability of sales personnel to obtain access to physicians, including key opinion leaders, or to educate an adequate number of physicians of the benefits of VYJUVEK or any approved product candidate;
- the lack of complementary products to be offered by our sales personnel, which may put us at a competitive disadvantage relative to companies with more extensive or integrated product offerings; and
- unforeseen costs and expenses associated with establishing and maintaining an independent sales, marketing, training, and support organization.

If our sales force, marketing, market access, and medical affairs teams and distribution capabilities fail, or are otherwise unsuccessful, it would materially adversely impact the commercial launch of VYJUVEK, impact our ability to generate revenue and harm our business.

If we are unable to expand our medical affairs, marketing, market access, sales, and distribution capabilities or collaborate with third parties to market and sell our product candidates for which we obtain marketing approval, we may be unable to generate sufficient product revenue.

To successfully commercialize any products for which we obtain marketing approvals, we will need to expand our sales force, marketing, market access, and medical affairs teams and distribution capabilities, either on our own or in collaboration with others. The development of a sales force, marketing, market access, and medical affairs teams and distribution capabilities effort is expensive and time-consuming, and our expenses associated with maintaining our sales force may be disproportional compared to the revenue we may be able to generate on sales of VYJUVEK and future products. We cannot be certain that we will be able to internally develop this capability successfully. We may enter into collaborations regarding VYJUVEK or any future approved product candidates with other entities to utilize their established marketing and distribution capabilities. However, we may be unable to enter into such agreements on favorable terms, if at all.

We compete with many companies that currently have extensive, experienced, and well-funded medical affairs, marketing, market access, distribution, and sales operations to recruit, hire, train and retain personnel, and we may not be able to hire or retain such talent on commercially reasonable terms, if at all. We also face competition in our search for third parties to assist us with the sales and marketing efforts. If any future collaborators do not commit sufficient resources to commercialize our product candidates, if approved, or we are unable to develop the necessary capabilities on our own, we will be unable to generate sufficient product revenue to sustain our business.

Our efforts to educate the medical community and third-party payors on the benefits of VYJUVEK or our product candidates, if approved, may require significant resources and may never be successful. Such efforts may require more resources than are typically required due to the complexity and uniqueness of our products. If VYJUVEK or any of our product candidates that are approved fails to achieve market acceptance among physicians, patients, or third-party payors, we will not

be able to generate significant revenue from such product, which could have a material adverse effect on our business, financial condition, results of operations and prospects.

If the market opportunities for VYJUVEK or our product candidates are smaller than we believe they are, our product revenue may be adversely impacted, and our business may suffer.

We focus our research and product development primarily on genetic medicines for patients with debilitating diseases. We base our market opportunity estimates on a variety of factors, including our estimates of the number of people who have these diseases, the potential scope of our approved product labels, the subset of people with these diseases who have the potential to benefit from treatment with VYJUVEK or our product candidates, various pricing scenarios, and our understanding of reimbursement policies in particular countries. These estimates are based on many assumptions and may prove incorrect, and new studies may reduce the estimated incidence or prevalence of these diseases. Estimating market opportunities can be particularly challenging for rare indications, as epidemiological data is often more limited than for more prevalent indications and can require additional assumptions to assess potential patient populations. For example, as we commercialize VYJUVEK in the United States and learn more about market dynamics and engage with regulators on additional potential marketing approvals, our view of VYJUVEK's initial potential market opportunity will become more refined. The addressable patient population in the United States and internationally may turn out to be lower than expected, patients may not be otherwise amenable to treatment with VYJUVEK or our product candidates, if approved, or may become increasingly difficult to identify and access, all of which would adversely affect our business, financial condition, results of operations and prospects. If we are unable to successfully commercialize VYJUVEK or any future product candidates with attractive market opportunities, our future product revenue may be smaller than anticipated, and our business may suffer.

Further, there are several factors that could contribute to making the actual number of patients who receive VYJUVEK less than the potentially addressable market. These include the lack of widespread availability of, and limited reimbursement for, new therapies in many underdeveloped markets. Further, the severity of the progression of a disease up to the time of treatment may diminish the therapeutic benefit conferred by a gene therapy due to irreversible cell damage. Lastly, certain patients' immune systems might prohibit the successful delivery of certain gene therapy products to the target tissue, thereby limiting the treatment outcomes.

In addition to determining market opportunities for our products, we need to accurately forecast demand and the timing of the demand, which is difficult. Incorrect demand estimates could adversely impact the Company. For example, if product demand is higher than we initially estimate, we may need to spend time and money on increasing our manufacturing capabilities and/or changing our manufacturing processes, which could require greater capital expenditures than initially forecasted and potentially delay commercial availability of an approved product, which could adversely affect our business, financial condition, and results of operations.

The commercial success of VYJUVEK and our product candidates will depend upon their degree of market acceptance by physicians, patients, third-party payors, and others in the medical community.

Even with the requisite approvals from the FDA in the United States, potential approvals of VYJUVEK from the EMA in the European Union, PMDA in Japan and other regulatory authorities internationally (and potential approvals of any of our product candidates by regulatory authorities), the commercial success of VYJUVEK and our product candidates will depend, in part, on the acceptance of physicians, patients and health care payors of gene therapy products in general, and VYJUVEK and our product candidates, in particular, as medically necessary, cost-effective, and safe. VYJUVEK and any product candidate that we commercialize may not gain acceptance by physicians, patients, health care payors and others in the medical community. If these products do not achieve an adequate level of acceptance, we may not generate significant product revenue and may not become consistently profitable. The degree of market acceptance of gene therapy products and VYJUVEK and our product candidates, if approved for commercial sale, will depend on several factors, including:

- the efficacy and safety of VYJUVEK and our product candidates as demonstrated in clinical trials;
- the potential and perceived advantages of VYJUVEK and our product candidates over alternative treatments, if available;
- the cost of VYJUVEK and our product candidates relative to alternative treatments if any are available;
- the clinical indications for which VYJUVEK and our product candidates are approved by the FDA and other regulatory authorities;
- the willingness of physicians to prescribe new therapies;
- the willingness of the target patient population to try new therapies;
- the prevalence and severity of any side effects;

- product labeling or product insert requirements of the FDA, the EMA, the PMDA, or other regulatory authorities, including any limitations or warnings contained in a product's approved labeling;
- relative convenience and ease of administration;
- the strength of marketing and distribution support;
- the timing of market introduction of competitive products;
- the availability of products and their ability to meet market demand;
- publicity concerning VYJUVEK and our product candidates or competing products and treatments;
- any restrictions on the use of VYJUVEK and our products together with other medications; and
- favorable third-party payor coverage and adequate reimbursement.

Even if a product candidate displays a favorable efficacy and safety profile in preclinical studies and clinical trials, market acceptance of the product will not be fully known until after it is launched.

Government price controls or other changes in pricing regulation could restrict the amount that we are able to charge for VYJUVEK and our product candidates, if approved, which would adversely affect our revenue and results of operations.

We expect that coverage and reimbursement of pharmaceuticals may be increasingly restricted both in the United States and internationally. The escalating cost of health care has led to increased pressure on the health care industry to reduce costs. Drug pricing by pharmaceutical companies recently has come under increased scrutiny and continues to be subject to intense political and public debate in the United States and abroad. Government and private third-party payors have proposed health care reforms and cost reductions. A number of federal and state proposals to control the cost of health care, including the cost of drug treatments, have been made in the United States. Specifically, there have been several recent U.S. Congressional inquiries and proposed bills designed to, among other things, bring more transparency to drug pricing, review the relationship between pricing and manufacturer patient programs and reform government program reimbursement methodologies for drugs. In some international markets, the government controls the pricing, which can affect the profitability of drugs. Current government regulations and possible future legislation regarding health care may affect coverage and reimbursement for medical treatment by third-party payors, which may render VYJUVEK or our product candidates, if approved, not commercially viable or may adversely affect our anticipated future revenue and gross margins.

We cannot predict the extent to which our business may be affected by these or other potential future legislative or regulatory developments. However, future price controls or other changes in pricing regulation or negative publicity related to the pricing of drugs or biologics generally could restrict the amount that we are able to charge for VYJUVEK or our product candidates, if approved, which would adversely affect our anticipated revenue and results of operations.

The insurance coverage and reimbursement status of newly approved products is uncertain. Failure to obtain or maintain adequate coverage and reimbursement for VYJUVEK or our product candidates, if approved, could limit our ability to market those products and decrease our ability to generate product revenue.

We expect that coverage and reimbursement by government and private payors will be essential for most patients to be able to afford our approved genetic medicine products. Accordingly, sales of VYJUVEK and our product candidates, if approved, will depend substantially, both domestically and abroad, on the extent to which the costs of our product or product candidates will be paid by health maintenance, managed care, pharmacy benefit and similar healthcare management organizations, or will be reimbursed by government authorities, private health coverage insurers and other third-party payors. Coverage and reimbursement by a third-party payor may depend upon several factors, including the third-party payor's determination that use of a product is:

- a covered benefit under its health plan;
- safe, effective, and medically necessary;
- appropriate for the specific patient;
- cost-effective; and
- neither experimental nor investigational.

Obtaining coverage and reimbursement for a product from third-party payors is a time-consuming and costly process that could require us to provide to the payor supporting scientific, clinical and cost-effectiveness data. We may not be able to provide data sufficient to gain acceptance with respect to coverage and reimbursement. If coverage and reimbursement are not available, or are available only at limited levels, we may not be able to successfully commercialize our product candidates, if approved. Even if coverage is provided, the coverage may be more limited than the purposes for which the product is approved

by the FDA or comparable foreign regulatory authorities. Moreover, eligibility for coverage and reimbursement does not imply that a product will be paid for in all cases or at a rate that covers our costs, including research, development, intellectual property protection, manufacture, sale, and distribution expenses, and therefore, the approved reimbursement amount may not be adequate to realize a sufficient return on our investment.

There is significant uncertainty related to third-party coverage and reimbursement of newly approved drug products. In the United States, third-party payors, including government payors such as the Medicare and Medicaid programs, play an important role in determining the extent to which new drugs and biologics will be covered and reimbursed. The Medicare and Medicaid programs increasingly are used as models for how private payors and government payors develop their coverage and reimbursement policies. It is difficult to predict what CMS will decide with respect to coverage and reimbursement for fundamentally novel products such as our product candidates. Moreover, reimbursement agencies in the European Union may be more conservative than CMS. For example, several cancer drugs have been approved for reimbursement in the United States and have not been approved for reimbursement in certain European Union Member States. It is difficult to predict what third-party payors will decide with respect to the coverage and reimbursement for our product candidates.

Outside the United States, international operations generally are subject to extensive government price controls and other market regulations and increasing emphasis on cost-containment initiatives in the European Union and other countries may put pricing pressure on us. In many countries, the prices of medical products are subject to varying price control mechanisms as part of national health systems. It also can take a significant amount of time after approval of a product to secure pricing and reimbursement for such product in many counties outside the United States. In general, the prices of medicines under such systems are substantially lower than in the United States. Other countries allow companies to fix their own prices for medical products but monitor and control company profits. Additional foreign price controls or other changes in pricing regulation could restrict the amount that we are able to charge for our product candidates. Accordingly, in markets outside the United States, the reimbursement for our approved products may be reduced compared with the United States and may be insufficient to generate commercially reasonable product revenue.

Moreover, increasing efforts by government and third-party payors in the United States and abroad to cap or reduce healthcare costs may cause such organizations to limit both coverage and the level of reimbursement for new products approved and, as a result, they may not cover or provide adequate payment for our product candidates. Payors increasingly are considering new metrics as the basis for reimbursement rates, such as Average Sales Price, Average Manufacturer Price, and Actual Acquisition Cost. The existing data for reimbursement based on some of these metrics is relatively limited, although certain states have begun to survey acquisition cost data for the purpose of setting Medicaid reimbursement rates, and CMS has begun making pharmacy National Average Drug Acquisition Cost and National Average Retail Price data publicly available on at least a monthly basis. Therefore, it may be difficult to project the impact of these evolving reimbursement metrics on the willingness of payors to cover product candidates that we are able to commercialize. We expect to experience pricing pressures in connection with the sale of any of our product candidates due to the trend toward managed healthcare, the increasing influence of health maintenance organizations, additional legislative changes, statements by elected officials, and administrative changes. The downward pressure on healthcare costs in general, particularly prescription drugs, has become intense. As a result, increasingly high barriers are being erected to the entry of new products such as ours.

Ethical, legal, and social issues related to genetic testing may reduce demand for our product candidates, if approved .

Prior to receiving VYJUVEK, patients are required to undergo genetic testing, and we anticipate that prior to receiving certain of our other product candidates, if approved, patients may be required to undergo genetic testing. Genetic testing has raised concerns regarding the appropriate utilization and the confidentiality of information provided by genetic testing. Genetic tests for assessing a person's likelihood of developing a chronic disease have focused public attention on the need to protect the privacy of genetic information. For example, concerns have been expressed that insurance carriers and employers may use these tests to discriminate based on genetic information, resulting in barriers to the acceptance of genetic tests by consumers. Concerns have also been raised about the accuracy of genetic testing. This could lead to governmental authorities restricting genetic testing or calling for additional regulation of genetic testing, particularly for diseases for which there is no known cure. Any of these scenarios could decrease demand for VYJUVEK and our product candidates, if approved.

Increasing demand for compassionate use or expanded access of our unapproved therapies could negatively affect our reputation and harm our business.

We are developing our product candidates principally for illnesses for which there are currently limited to no available therapeutic options. At least one other company has been the target of disruptive social media campaigns related to a request for access to unapproved drugs for patients with significant unmet medical need. If we experience a similar social media campaign regarding our decision to provide or not provide our product candidates under an expanded access corporate policy, our reputation may be negatively affected, and our business may be harmed. Recent media attention to individual patients' expanded access requests has resulted in the introduction of legislation at the local and national level referred to as "Right to Try" laws, such as the Right to Try Act, which are intended to give patients access to unapproved therapies. New and emerging

legislation regarding expanded access to unapproved drugs for life-threatening illnesses could negatively impact our business in the future.

A possible consequence of both activism and legislation in this area is the need for us to initiate an unanticipated expanded access program or to make our product candidates more widely available sooner than anticipated. We are a small company with limited resources and unanticipated trials or access programs could result in diversion of resources from our primary goals.

In addition, some patients who receive access to unapproved drugs through compassionate use or expanded access programs have life-threatening illnesses and have exhausted all other available therapies. The risk for serious adverse events in this patient population is high which could have a negative impact on the safety profile of our product candidates if we were to provide them to these patients in accordance with our expanded access corporate policy, which could cause significant delays or an inability to successfully commercialize our product candidates, which could materially harm our business. If we were to provide patients with our product candidates under our expanded access corporate policy, we may in the future need to restructure or pause ongoing compassionate use and/or expanded access programs in order to perform the controlled clinical trials required for regulatory approval and successful commercialization of our product candidates, which could prompt adverse publicity or other disruptions related to current or potential participants in such programs.

Risks Related to Our Intellectual Property

If we are unable to obtain and maintain adequate United States and foreign patent protection for VYJUVEK, our current product candidates, and any future product candidates we may develop, and/or our vector platform, or if the scope of the patent protection obtained is not sufficiently broad, our competitors could develop and commercialize products and technologies similar or identical to ours, and our ability to successfully commercialize VYJUVEK, our current product candidates, any future product candidates we may develop, and our platform technologies may be adversely affected.

Our success depends, in large part, on our ability to obtain and maintain patent protection in the United States and other countries with respect to our approved product, current product candidates, additional product candidates in our pipeline and current and future innovations related to our vector platform. The patent prosecution process is expensive, time-consuming, and complex; we may not be able to file, prosecute, maintain, and/or enforce all necessary or desirable patent applications and issued patents at a reasonable cost or in a timely manner.

Even if we are granted the patents we are currently pursuing, they may not issue in a form that will provide us with the full scope of protection we desire, they may not prevent competitors or other third parties from competing with us, and/or they may not otherwise provide us with a competitive advantage. Our competitors, or other third parties, may be able to circumvent our patents by developing similar or alternative technologies or products in a non-infringing manner. Moreover, our patent estate does not preclude third parties from having intellectual property rights that could interfere with our freedom to use our platform, including for our intended indications. Even assuming patents issue from our pending and future patent applications, changes in either the patent laws or interpretation of the patent laws in the United States and foreign jurisdictions may diminish the value of our patents or narrow their scope of protection.

We also may not be aware of all third-party intellectual property rights potentially relating to technologies similar to our own. Publications of discoveries in the scientific literature often lag their actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after earliest priority date or, in some cases, not at all until patents are issued. Therefore, it is impossible to be certain that we were the first to develop the specific technologies as claimed in any owned patents or pending patent applications, or that we were the first to file for patent protection of such inventions.

We may not be able to protect our intellectual property rights throughout the world.

Filing, prosecuting, and defending patents on VYJUVEK, each and every one of our product candidates, and current and future innovations related to our vector platform, in all countries throughout the world would be prohibitively expensive, and intellectual property rights in some countries outside the United States may differ in scope from those eventually granted in the United States. Thus, in some cases, we may not have the opportunity to obtain patent protection for certain technologies in some jurisdictions outside the United States. In addition, the laws of some foreign countries do not protect intellectual property rights to the same extent as federal and state laws in the United States. Consequently, we may not be able to prevent third parties from practicing our inventions in all countries outside the United States, even in jurisdictions where we do pursue patent protection. Competitors may use our technologies in jurisdictions where we have not pursued and obtained patent protection to develop their own products and, further, may export otherwise infringing products to territories where we have patent protection, but enforcement is not as strong as that in the United States. These products may compete with VYJUVEK and our product candidates that are approved, and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents, trade secrets, and other intellectual property protection, particularly those relating to biotechnology products. Such challenges in enforcing rights in these countries could make it difficult for us to stop the infringement of our patents, if pursued and obtained, or marketing of competing products in violation of our proprietary rights generally. Proceedings to enforce our current and future patent rights in foreign jurisdictions could result in substantial costs and may divert our efforts and attention from other aspects of our business; could put our patents at risk of being invalidated or interpreted narrowly; could put any future patent applications, including continuation and divisional applications, at risk of not issuing; and could provoke third parties to assert claims against us. We may not prevail in any lawsuits, and the damages or other remedies awarded, if any, may not be commercially meaningful. Accordingly, our efforts to enforce any intellectual property rights around the world stemming from intellectual property that we develop may be inadequate to obtain a significant commercial advantage in these foreign jurisdictions.

Third parties may initiate legal proceedings alleging that we are infringing their intellectual property rights, the outcome of which would be uncertain and could have a material adverse effect on the success of our business.

Our commercial success depends upon our ability (and the ability of any potential future collaborators) to market and sell VYJUVEK and to develop, manufacture, market and sell our product candidates, and to freely use our proprietary technologies without infringing the rights and intellectual property of others. Many companies and institutions have filed, and continue to file, patent applications related to various aspects of gene therapy. Because patent applications can take many years to issue, may be confidential for 18 months or more after filing, and can be revised before issuance, there may be applications now pending which may later result in issued patents that a third-party asserts are infringed by the manufacture, use, sale, or importation of VYJUVEK or any of our product candidates, if approved. The biotechnology and pharmaceutical industries are characterized by extensive and complex litigation regarding patents and other intellectual property rights. We may become party to, or be threatened with, adversarial proceedings or litigation regarding intellectual property rights with respect to VYJUVEK or our product candidates or related technologies, including, for example, interference proceedings, post grant review challenges, and *inter partes* review before The United States Patent and Trademark Office. Our competitors or other third parties may assert infringement claims against us, alleging that our products, manufacturing methods, formulations or administration methods are covered by their patents. Moreover, we may face patent infringement claims from non-practicing entities that have no relevant product revenue, and against whom our patent portfolio may therefore have no deterrent effect.

There is a risk that third parties may choose to engage in litigation with us to enforce or to otherwise assert their patents or other intellectual property rights against us. Even if we believe such claims are without merit, a court of competent jurisdiction could hold that these third-party patents are valid, enforceable, and infringed, which could materially and adversely affect our ability to commercialize VYJUVEK or any of our product candidates, if approved. In order to successfully challenge the validity of any such U.S. patent in federal court, we would need to overcome a presumption of validity. As this burden is a high one requiring us to present clear and convincing evidence as to the invalidity of any such U.S. patent claim, there is no assurance that a court of competent jurisdiction would invalidate the claims of any such U.S. patent. In such a hypothetical situation, there is no assurance that a court of competent jurisdiction would find that our product, product candidates or technologies do not infringe a third-party patent.

Patent and other types of intellectual property litigation can involve complex factual and legal questions, and their outcomes are uncertain. If we are found, or believe there is a risk that we may be found, to infringe a third-party's valid and enforceable intellectual property rights, we could be required (or may choose) to obtain a license from such a third-party to continue developing, manufacturing and marketing our approved product, product candidates and technologies. However, we may not be able to obtain any required license on commercially reasonable terms, if at all. Even if we were able to obtain a license, it could be non-exclusive, thereby giving our competitors and other third parties access to the same technologies licensed to us, and further, it could require us to make substantial licensing and royalty payments. We could be forced, including by court order, to cease developing, manufacturing, and commercializing the infringing product or technologies. We also could be found liable for monetary damages, including treble damages and attorneys' fees, if we are found to have willfully infringed a patent or other intellectual property right. A finding of infringement could prevent us from manufacturing and commercializing our products and technologies or force us to cease some or all our business operations. Claims that we have misappropriated the confidential information or trade secrets of third parties could have a similar negative impact on our business, financial condition, results of operations and prospects.

Intellectual property litigation could cause us to spend substantial resources and distract our personnel from their normal responsibilities.

Litigation or other legal proceedings relating to intellectual property claims, with or without merit, is unpredictable and generally expensive and time consuming. Competitors may infringe our current or future patents, should such patents issue, or we may be required to defend against claims of infringement or other unauthorized use of intellectual property. Even if resolved in our favor, litigation or other legal proceedings relating to intellectual property claims may cause us to incur significant

expenses and could distract our scientific and management personnel from their normal responsibilities. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation. In addition, there could be public announcements of the results of hearings, motions, or other interim proceedings or developments, and if securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our common stock. Such litigation or proceedings could substantially and adversely impact our financial results and reduce the resources available for development activities or any future sales, marketing, or distribution activities.

We may not have sufficient financial or other resources to adequately conduct such litigation or proceedings. Some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their greater financial resources. Accordingly, despite our efforts, we may not be able to prevent third parties from infringing, misappropriating, or successfully challenging our intellectual property rights. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could have a material adverse effect on our ability to compete in the marketplace.

We have been subject to claims asserting that we, our employees or our advisors have wrongfully used or disclosed alleged trade secrets of other parties, and we may face such claims in the future or claims asserting ownership of what we regard as our own intellectual property.

Certain of our employees or advisors are currently, or were previously, employed at universities or other biotechnology or pharmaceutical companies, including potential competitors, and we have and may in the future enter into agreements providing us with rights to intellectual property of third parties for limited purposes. Although we endeavor to observe the terms of agreements under which we obtain access to third-party intellectual property and to ensure that our employees and advisors do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that these individuals, or we, have used or disclosed intellectual property, including trade secrets or other proprietary information, of third parties or the current or former employers of employees or advisors. For instance, as described in Note 7 of the Notes to Condensed Consolidated Financial Statements (unaudited) included in Part I, Item 1 of this Quarterly Report on Form 10-Q, in April of 2022, we entered into a settlement agreement with PeriphaGen, Inc., which had alleged breach of contract and misappropriation of trade secrets. If we fail to successfully defend any such claims, in addition to paying monetary damages, we may be subject to an injunction and may lose valuable intellectual property rights or personnel. Moreover, any such litigation, or the threat thereof, may adversely affect our ability to hire new employees or contract with independent contractors. A loss of key personnel or their work product could hamper or prevent our ability to commercialize VYJUVEK or our product candidates, which could have an adverse effect on our business, results of operations, and financial condition. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management.

While it is our policy to require our employees and contractors who may be involved in the conception of intellectual property to execute agreements assigning such intellectual property rights to us, unforeseen complications may arise when fully and adequately executing such an agreement with each party who, in fact, conceives of intellectual property that we regard as our own. Examples of such complications may include, for example, when we obtain agreements assigning intellectual property to us, the assignment of intellectual property rights may not be self-executing, or the assignment agreements may be breached. Such complications may lead to us being forced to bring claims against third parties or current and former employees, or defend claims that they may bring against us, to determine the ownership of what we regard as our intellectual property. Moreover, individuals executing agreements with us may have preexisting or competing obligations to a third-party, such as an academic institution, and thus an agreement with us may be insufficient in fully perfecting ownership of inventions developed by that individual. Disputes about the ownership of intellectual property may have a material adverse effect on our business.

Changes in U.S. patent law could diminish the value of patents in general, thereby impairing our ability to protect VYJUVEK or our product candidates.

Patent reform legislation could increase the uncertainties and costs surrounding the prosecution of patent applications and the enforcement or defense of issued patents. For example, in September 2011, the Leahy-Smith America Invents Act, or the Leahy-Smith Act, was signed into law. The Leahy-Smith Act included several significant changes to U.S. patent law, including provisions that affected the way patent applications are prosecuted, and altered strategies regarding patent litigation. These provisions also switched the United States from a “first-to-invent” system to a “first-to-file” system, allowed third-party submissions of prior art to the United States Patent and Trademark Office (“USPTO”) during patent prosecution, and set forth additional procedures to attack the validity of a patent through various post grant proceedings administered by the USPTO. As patent reform legislation can inject serious uncertainty into the patent prosecution and litigation processes, it is not clear what impact future patent reform legislation will have on the operation of our business. However, such future legislation, and its implementation, could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of any issued patents, all of which could have a material adverse effect on our business, financial condition, results of operations and prospects.

Moreover, the patent positions of companies engaged in the development and commercialization of biologics and pharmaceuticals are particularly uncertain given the ever evolving and constantly shifting nature of precedential patent cases decided by both the U.S. Court of Appeals for the Federal Circuit and the U.S. Supreme Court. We cannot assure you that our efforts to seek patent protection for our technology and product candidates will not be negatively impacted by future court decisions or changes in guidance or procedures issued by the USPTO. These decisions, and any guidance issued by the USPTO (or changes thereto), could have a material adverse effect on our existing patent portfolio and our ability to protect and enforce our intellectual property rights in the future.

If our trademarks and trade names are not adequately protected, then we may not be able to build name recognition in our markets of interest and our business may be adversely affected.

Although we have registered certain of our trademarks and trade names, they may be challenged, infringed, circumvented, or declared generic or determined to be infringing on other marks. We may not be able to protect our rights to these trademarks and trade names, which are important for building name recognition among potential partners or customers in our markets of interest. At times, competitors may adopt trade names or trademarks similar to ours, thereby impeding our ability to build brand identity and possibly leading to market confusion. There also could be potential trade name or trademark infringement claims brought by owners of other registered trademarks or trade names that incorporate variations of our registered or unregistered trademarks or trade names. Over the long term, if we are unable to establish name recognition based on our trademarks and trade names, then we may not be able to compete effectively, and our business may be adversely affected. Our efforts to enforce or protect our proprietary rights related to patents, trademarks, trade secrets, domain names, copyrights or other intellectual property may be ineffective and could result in substantial costs and diversion of resources and could adversely impact our financial condition or results of operations.

Intellectual property rights and regulatory exclusivity rights do not necessarily address all potential threats.

The degree of current and future protection afforded by our intellectual property rights is uncertain because intellectual property rights have limitations and may not adequately protect our business or permit us to maintain our competitive advantage. For example:

- others may be able to make gene therapy products that are similar to our approved product or any of our product candidates but that are not covered by the claims of our current patents, or of patents that we may own or license in the future;
- we, or any future license partners or collaborators, might not have been the first to file patent applications covering certain aspects of the concerned technologies;
- others may independently develop similar or alternative technologies, or duplicate any of our technologies, potentially without falling within the scope of our current or future issued claims, thus not infringing our intellectual property rights;
- it is possible that our filed or future patent applications will not lead to issued patents;
- issued patents to which we currently hold rights or to which we may hold rights in the future may be held invalid or unenforceable, including as a result of legal challenges by third parties or our competitors;
- others may have access to any future intellectual property rights licensed to us on a non-exclusive basis;
- our competitors might conduct research and development activities in countries where we do not have or pursue patent rights, and then use the information learned from such activities to develop competitive products for sale in our major commercial markets;
- we may not develop additional proprietary technologies that are patentable;
- the patents or other intellectual property rights of others may have an adverse effect on our business; and
- we may choose not to file a patent application covering certain of our trade secrets or know-how, and a third-party may subsequently file a patent covering such intellectual property.

Should any of these events occur, they could significantly harm our business, financial condition, results of operations and prospects.

Risks Related to Our Financial Position and Need for Additional Capital

We have incurred net losses in the past and may not sustain profitability.

Although we generated net income for the year ended December 31, 2023 and for the quarters ended March 31, 2024 and June 30, 2024, we have otherwise previously incurred recurring losses and negative cash flows from operations since inception. Our transition to operating profitability depends on our ability to (i) successfully commercialize VYJUVEK in the

U.S. and obtain the necessary regulatory approvals to commercialize VYJUVEK outside of the U.S. and then successfully commercialize VYJUVEK outside the U.S., and (ii) complete the development of, and obtain the regulatory approvals necessary to successfully commercialize our product candidates with significant market potential. We have devoted substantially all our efforts to date to (i) research and development of our gene therapy platform, product candidates and our manufacturing infrastructure, and, more recently, (ii) commercializing VYJUVEK in the U.S. We expect to continue to incur significant expenses for the foreseeable future and our operating results may fluctuate significantly from quarter to quarter. We anticipate that our expenses will increase substantially if, and as, we:

- manufacture, market and sell our lead product, VYJUVEK, in the U.S. and prepare for regulatory approvals outside of the U.S. and if such approvals are received, commercialize VYJUVEK in those geographies;
- continue our research, preclinical studies, and the clinical development of our current product candidates, including our current clinical trials and planned clinical trials;
- initiate preclinical studies and clinical trials for any additional product candidates that we may pursue in the future;
- prepare for regulatory approvals for our product candidates in the United States, EU and in other key geographies;
- continue to operate our in-house commercial-scale CGMP manufacturing facilities, ANCORIS and ASTRA, and as we seek to obtain FDA approval for commercial manufacture of VYJUVEK at ASTRA, which approval may not be granted;
- manufacture material for commercial sales of VYJUVEK and clinical trials or potential commercial sales of our product candidates;
- further develop our gene therapy platform;
- further establish our sales, marketing and distribution infrastructure to commercialize VYJUVEK and product candidates for which we may obtain marketing approval;
- develop, maintain, expand and protect our intellectual property portfolio; and
- acquire or in-license other product candidates and technologies.

To remain profitable, we must be successful in a range of challenging activities, including designing, initiating, and completing clinical trials for our product candidates, developing, validating, and maintaining commercial scale manufacturing processes, obtaining marketing approvals, manufacturing, marketing, and selling VYJUVEK and any product candidates for which we may obtain marketing approval, and satisfying any post-marketing requirements. If we were required to discontinue development of any of our product candidates, if VYJUVEK does not receive regulatory approvals outside the U.S., or any of our product candidates do not receive regulatory approvals, or if VYJUVEK or any of our product candidates, if approved, fails to achieve sufficient market acceptance for any indication, our ability to remain profitable, our business prospects and financial condition could be materially adversely affected. Moreover, if we decide to leverage any success with VYJUVEK or any of our current product candidates to develop other product opportunities, we may not be successful in such efforts. In any such event, our business may be materially adversely affected.

We currently have one product, VYJUVEK, approved by the FDA and several product candidates in the clinical trials stages. However, we may never develop, acquire or in-license additional product candidates. We may never generate revenue from any of our product candidates. We may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to remain profitable would decrease the value of our company and could impair our ability to raise capital, maintain our research and development efforts, expand our business, or continue our operations. A decline in the value of our company also could cause stockholders to lose all or part of their investment.

Because of the numerous risks and uncertainties associated with pharmaceutical product and biological development, we are unable to accurately predict the timing or amount of increased expenses. If we are required by the FDA, the EMA, the PMDA, or other regulatory authorities to perform studies in addition to those currently expected, or if there are any delays in completing our clinical trials or the development of our product candidates, our expenses could increase and potential revenue from product candidates in development could be delayed.

We may need to raise additional funding to maintain and expand our commercialization capabilities and to complete the development of, and obtain the regulatory approvals necessary to, commercialize our product candidates. Such funding may not be available on acceptable terms, or at all. Failure to obtain this necessary capital when needed may force us to delay, limit or terminate certain of our product development efforts or other operations.

To complete the process of obtaining regulatory approval for our product candidates and to continue building the manufacturing, sales, marketing, and distribution infrastructure that we believe is or will be necessary to successfully

commercialize VYJUVEK and our product candidates, if approved, we may require substantial additional funding. We expect to continue to incur significant expenses related to sales, medical affairs, marketing, manufacturing, and distribution of VYJUVEK. In addition, if we obtain marketing approval for our product candidates, we expect to incur significant additional expenses related to product sales, medical affairs, marketing, manufacturing and distribution. We may need additional funding to complete the development of our product candidates and to commercialize any such approved products. Our future capital requirements will depend on many factors, including:

- the ability of VYJUVEK to generate sufficient revenue;
- the costs of product sales, medical affairs, marketing, manufacturing, and distribution for VYJUVEK;
- the outcome, timing and costs of seeking regulatory approvals for VYJUVEK outside the U.S.;
- the progress, timing, results, and costs of our current and planned clinical trials of B-VEC (in Japan), ophthalmic B-VEC, KB105, KB301, KB407, KB707, and KB408;
- the continued development and the filing of IND applications for KB104 and other product candidates;
- the initiation, scope, progress, timing, costs and results of drug discovery, laboratory testing, manufacturing, preclinical studies, and clinical trials for any product candidates that we may pursue in the future;
- the costs of maintaining our own commercial-scale CGMP manufacturing facilities;
- the outcome, timing, and costs of seeking regulatory approvals for any of our product candidates;
- the costs associated with the manufacturing process development and evaluation of third-party suppliers or manufacturers, if necessary;
- the costs of future activities, including product sales, medical affairs, marketing, manufacturing, and distribution, in the event we receive marketing approval for any of our current and future product candidates;
- the extent to which the costs of our product candidates, if approved, will be paid by health maintenance, managed care, pharmacy benefit and similar healthcare management organizations, or will be reimbursed by government authorities, private health coverage insurers and other third-party payors;
- subject to receipt of marketing approval, if any, revenue received from commercial sale of our current and future product candidates;
- the terms and timing of any current or future collaborations, distribution, licensing, consulting, or other arrangements;
- the amount and timing of any payments we may be required to make, or that we may receive, in connection with the licensing, filing, prosecution, maintenance, defense and enforcement of any patents or other intellectual property rights, including milestone and royalty payments and patent prosecution fees that we are obligated to pay pursuant to our license agreements, if any;
- the terms of our license agreements, if any, and our achievement of milestones under those agreements;
- our ability to establish and maintain collaborations and licenses on favorable terms, if at all; and
- the extent to which we acquire or in-license other product candidates and technologies.

Identifying product candidates and conducting preclinical testing and clinical trials is a time-consuming, expensive and uncertain process that takes years to complete, and we may never generate the necessary data or results required to obtain marketing approval and achieve product sales for our product candidates in development or future product candidates. Revenue will be derived from VYJUVEK until we have another product candidate receive marketing approval. Accordingly, we may need to continue to rely on additional financing to achieve our business objectives. Any additional fundraising efforts may divert our management from their day-to-day activities, which may adversely affect our ability to develop and commercialize our product candidates. Moreover, the terms of any financing may adversely affect the holdings or the rights of our stockholders and the issuance of additional securities, whether equity or debt, by us, or the possibility of such issuance, may cause the market price of our shares to decline. The sale of additional equity or convertible securities would dilute all of our existing stockholders. Existing stockholders may not agree with our financing plans or the terms of such financings. Adequate additional financing may not be available to us on acceptable terms, or at all. The terms of additional financing may be impacted by, among other things, general market conditions, the market's perception of our approved product, VYJUVEK, and product candidates, our growth potential, and the market price per share of our common stock. See "Raising additional capital could cause the price of our common stock to decline and cause dilution to our stockholders, restrict our operations or require us to relinquish rights."

Changes in tax law may adversely affect our business and financial condition

We are subject to evolving and complex tax laws in the U.S. and the foreign jurisdictions in which we operate. New income, sales, use or other tax laws, statutes, rules, regulations, or ordinances could be enacted at any time, or interpreted, changed, modified, or applied adversely to us, any of which could adversely affect our business operations and financial performance. Changes to tax laws (which could apply retroactively) could adversely affect us and our stockholders. In recent years, such changes have been made and changes are likely to occur in the future, which could have a material adverse effect on our business, cash flow, financial condition, and results of operations.

Our ability to use our net operating loss carryforwards and certain tax credit carryforwards may be subject to limitation.

We have U.S. federal and state net operating loss carryforwards, which are available to reduce future taxable income. Federal net operating loss carryforwards generated in tax years beginning after December 31, 2017 may be carried forward indefinitely but are limited to offset 80% of taxable income in any tax year. All of our remaining federal net operating loss carryforwards may be carried forward indefinitely. Our state net operating loss carryforwards expire beginning in 2037. We also have federal research and development tax credits which may be used to offset future tax liabilities and expire beginning in 2039. We also have federal orphan drug tax credits which may be used to offset future tax liabilities, which expire beginning in 2039.

Under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended, changes in our ownership may limit the amount of our net operating loss carryforwards and tax credit carryforwards that could be utilized annually to offset our future taxable income. This limitation would generally apply in the event of a cumulative change in ownership of our company of more than 50% within a three-year period. Any such limitation may significantly reduce our ability to utilize our net operating loss carryforwards and tax credit carryforwards before they expire. Private placements and other transactions that have occurred since our inception, as well as our initial public offering, may trigger such an ownership change pursuant to Sections 382 and 383. Any such limitation, whether as the result of the initial public offering, prior private placements, sales of our common stock by our existing stockholders or additional sales of our common stock by us, could have a material adverse effect on our results of operations in future years.

Our limited operating history may make it difficult for you to evaluate the success of our business to date and to assess our future viability.

We commenced operations in 2016. Our efforts to date have been primarily related to organizing and staffing our company, business planning, raising capital, developing our vector platform and related technologies, identifying potential gene therapy product candidates, undertaking preclinical studies and clinical trials, scaling our manufacturing capabilities, obtaining FDA approval for VYJUVEK, and commercializing VYJUVEK. Consequently, any predictions you make about our future success, performance or viability may not be as accurate as they could be if we had more experience developing and commercializing gene therapy products.

We expect our financial condition and operating results to continue to fluctuate from quarter to quarter and year to year due to a variety of factors, many of which are beyond our control. We are transitioning from a company with a research and development focus to a company undertaking commercial activities. We may encounter unforeseen expenses, difficulties, complications and delays and may not be successful in such a transition. Accordingly, you should not rely upon the results of any particular quarterly or annual period as indications of future operating performance.

Risks Related to Ownership of Our Common Stock

Our Chief Executive Officer and Chairman of the Board of Directors and our Founder, President, Research & Development and Director will have the ability to substantially influence all matters submitted to stockholders for approval.

Krish S. Krishnan and Suma M. Krishnan, our Chief Executive Officer and Chairman of the Board and our Founder, President, Research & Development and Director, respectively, in the aggregate, beneficially own over 10% of our outstanding common stock. As a result, they will be able to substantially influence all matters submitted to our stockholders for approval, as well as our management and affairs. For example, these persons would substantially influence the election of directors and approval of any merger, consolidation, or sale of all or substantially all our assets. This concentration of voting power could delay or prevent an acquisition of our company on terms that other stockholders may desire or result in management of our company that our public stockholders disagree with.

If securities analysts publish negative evaluations of our stock, the price of our stock could decline.

The trading market for our common stock relies in part on the research and reports that industry or financial analysts publish about us or our business. If securities analysts covering our business downgrade their evaluations of our stock, the price of our stock could decline. If one or more of these analysts cease to cover our stock, we could lose visibility in the market for our stock, which in turn could cause our stock price to decline.

Raising additional capital could cause the price of our common stock to decline and cause dilution to our stockholders, restrict our operations or require us to relinquish rights.

Until such time, if ever, as we can generate substantial and consistent product revenue, we may need to finance our cash needs through a combination of private and public equity offerings, debt financings, collaborations, strategic alliances, and licensing arrangements. We may issue additional common stock or restricted securities as part of such financing activities and any such issuances may have a dilutive effect on our then-existing stockholders. Sales of substantial amounts of our common stock in the open market, or the availability of such shares for sale, could adversely affect the price of our common stock.

The incurrence of indebtedness would result in fixed payment obligations and a portion of our operating cash flows, if any, being dedicated to the payment of principal and interest on such indebtedness, and we may be required to agree to certain restrictive covenants, such as limitations on our ability to incur additional debt, limitations on our ability to acquire, sell or license intellectual property rights and other operating restrictions that could adversely impact our ability to conduct our business.

If we are unable to raise additional funds through equity or debt financings when needed, and instead raise additional capital through marketing and distribution agreements or other collaborations, strategic alliances, or licensing arrangements with third parties, we may have to relinquish certain valuable rights to our current and future product candidates, technologies, future revenue streams or discovery programs or grant licenses on terms that may not be favorable to us.

The price of our common stock may be volatile and fluctuate substantially, which could result in substantial losses for holders of our common stock.

The price of our common stock has been and is likely to continue to be volatile. The stock market in general and the market for biopharmaceutical or pharmaceutical companies specifically has experienced extreme volatility that has often been unrelated to the operating performance of such companies. As a result of this volatility, a stockholder may not be able to sell their common stock at or above the price that they paid for it. The market price of our common stock may be influenced by many factors, including:

- our ability to successfully commercialize VYJUVEK;
- our ability to successfully proceed to and conduct clinical trials;
- results of clinical trials of our product candidates or those of our competitors;
- our ability to obtain regulatory approval for our product candidates and our ability to successfully commercialize any of our approved product candidates;
- the success of competitive products or technologies;
- commencement or termination of collaborations;
- regulatory or legal developments in the United States and other countries;
- the recruitment or departure of key personnel;
- the level of expenses related to VYJUVEK or any of our product candidates or clinical development programs;
- the results of our efforts to discover, develop, acquire or in-license additional product candidates;
- actual or anticipated changes in estimates as to financial results, development timelines or recommendations by securities analysts;
- our inability to manufacture adequate product supply for VYJUVEK and any other approved product or inability to do so at acceptable prices;
- disputes or other developments relating to proprietary rights, including patent applications, and issued patents;
- our ability to obtain patent protection for our product candidates and technologies;
- significant lawsuits, including patent or stockholder litigation;
- variations in our financial results or those of companies that are perceived to be similar to us;
- changes in the structure of healthcare payment systems;
- market conditions in the pharmaceutical and biotechnology sectors;
- general economic, industry and market conditions; and
- the other factors described in this “Risk Factors” section.

If we fail to maintain effective internal control over financial reporting, we may not be able to accurately report our financial results, which may adversely affect investor confidence in our company and, as a result, the value of our common stock.

Our management is responsible for establishing and maintaining adequate internal control over financial reporting and is required to have an independent auditor assess the effectiveness of our internal control over financial reporting, pursuant to Section 404 of the Sarbanes-Oxley Act of 2002, as amended (the "Sarbanes-Oxley Act"). We cannot give any assurances that material weaknesses will not be identified in the future in connection with our compliance with the provisions of Section 404 of the Sarbanes-Oxley Act. The existence of any material weakness would preclude a conclusion by management and our independent auditors that we maintained effective internal control over financial reporting. Our management may be required to devote significant time and expense to remediate any material weaknesses that may be discovered and may not be able to remediate any material weakness in a timely manner. The existence of any material weakness in our internal control over financial reporting could also result in errors in our financial statements that could require us to restate our financial statements, cause us to fail to meet our reporting obligations and cause investors to lose confidence in our reported financial information, all of which could lead to a decline in the market price of our common stock.

Provisions in our corporate charter documents and under Delaware law could make an acquisition of us, which may be beneficial to our stockholders, more difficult and may prevent attempts by our stockholders to replace or remove our current management.

Provisions in our corporate charter and our bylaws may discourage, delay, or prevent a merger, acquisition, or other change in control of us that stockholders may consider favorable, including transactions in which stockholders might otherwise receive a premium for their shares. These provisions also could limit the price that investors might be willing to pay in the future for shares of our common stock, thereby depressing the market price of our common stock. In addition, because our board of directors is responsible for appointing the members of our management team, these provisions may frustrate or prevent any attempts by our stockholders to replace or remove our current management by making it more difficult for stockholders to replace members of our board of directors. Among other things, these provisions:

- establish a classified board of directors such that not all members of the board are elected at one time;
- allow the authorized number of our directors to be changed only by resolution of our board of directors;
- limit the manner in which stockholders can remove directors from the board;
- establish advance notice requirements for stockholder proposals that can be acted on at stockholder meetings and nominations to our board of directors;
- require that stockholder actions must be effected at a duly called stockholder meeting and prohibit actions by our stockholders by written consent;
- limit who may call stockholder meetings;
- authorize our board of directors to issue preferred stock without stockholder approval, which could be used to institute a stockholder rights plan, or so-called "poison pill," that would work to dilute the stock ownership of a potential hostile acquirer, effectively preventing acquisitions that have not been approved by our board of directors; and
- require the approval of the holders of at least 80% of the votes that all our stockholders would be entitled to cast to amend or repeal certain provisions of our bylaws.

Moreover, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporation Law, which prohibits a person who owns in excess of 15% of our outstanding voting stock from merging or combining with us for a period of three years after the date of the transaction in which the person acquired in excess of 15% of our outstanding voting stock, unless the merger or combination is approved in a prescribed manner.

We have broad discretion in the use of our cash, cash equivalents and marketable securities and may not use them effectively.

Our management has broad discretion in the application of our cash, cash equivalents and marketable securities and could spend these funds in ways that do not improve our results of operations or enhance the value of our common stock. The failure by our management to apply these funds effectively could result in financial losses that could have a material adverse effect on our business, cause the price of our common stock to decline and delay the development of our product candidates. Pending their use, we may invest our cash and cash equivalents in a manner that does not produce income or that loses value.

Because we do not anticipate paying any cash dividends on our common stock in the foreseeable future, capital appreciation, if any, will be stockholders' sole source of gain.

We have never declared or paid cash dividends on our common stock. We currently intend to retain all our future earnings to finance the growth and development of our business. In addition, the terms of any future debt agreements may preclude us from paying dividends. As a result, capital appreciation, if any, of our common stock will be stockholders' sole source of gain for the foreseeable future.

Issuing additional shares of our common stock could cause the price of our common stock to decline and cause dilution to our stockholders.

To the extent we raise additional capital by issuing additional shares of our common stock, or securities convertible into or exchangeable or exercisable for common stock, our existing stockholders may experience substantial dilution. Additionally, if we issue additional shares of our common stock or instruments convertible into our common stock, the trading price of our common stock could decline. We cannot predict whether we will raise additional capital by issuing shares of our common stock, or securities convertible into or exchangeable or exercisable for common stock, the size of any future issuances, or the effect, if any, that they may have on the market price for our common stock. We also have stock options, restricted common stock, restricted stock units, and performance-based restricted stock units outstanding, and we expect to issue additional equity awards to directors and employees. The issuance of restricted common stock, common stock upon exercise of outstanding options, common stock upon vesting of restricted stock units, or common stock upon vesting of performance-based restricted stock units would be dilutive and may cause the market price for our common stock to decline. If we issue preferred stock in the future, the holders of that preferred stock could gain rights superior to our existing stockholders, such as liquidation and other preferences, or the market price of our common stock could be adversely affected.

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.

ITEM 5. OTHER INFORMATION

Insider Trading Arrangements

On June 14, 2024, Krish Krishnan, our Chief Executive Officer and Chairman of our Board of Directors , adopted a Rule 10b5-1 trading arrangement that is intended to satisfy the affirmative defense conditions of Rule 10b5-1(c) under the Exchange Act for the sale of up to 100,000 shares of our Common Stock until September 13, 2025.

On June 14, 2024, Suma Krishnan, our President, R&D and a member of our Board of Directors , adopted a Rule 10b5-1 trading arrangement that is intended to satisfy the affirmative defense conditions of Rule 10b5-1(c) under the Exchange Act for the sale of up to 100,000 shares of our Common Stock until September 13, 2025.

Executive Change in Control Severance Plan

On August 2, 2024, upon recommendation of the Compensation Committee (the "Compensation Committee") of the Company's Board of Directors (the "Board"), the Board adopted the Krystal Biotech, Inc. Executive Change in Control Severance Plan (the "Change in Control Plan"), covering eligible executives, including each of the Company's named executive officers, Krish S. Krishnan, Chairman and Chief Executive Officer, Suma M. Krishnan, Founder and President, R&D, and Kathryn A. Romano, Chief Accounting Officer (each a "Participant").

Pursuant to the Change in Control Plan, upon a termination of a Participant's employment (i) by the Company without Cause or (ii) due to a Participant's resignation for Good Reason (each as defined in the Change in Control Plan) that occurs within the 24-month period following the consummation of a Change in Control (as defined in the Change in Control Plan), Participants are entitled to receive: (A) accrued amounts, including unpaid salary and reimbursement for all incurred but unreimbursed expenses through the date of the Participant's termination of employment, and (B) subject to the Participant's execution of a release of claims and compliance with restrictive covenant obligations: (1) a lump sum cash payment equal to the sum of the Participant's base salary and target annual cash bonus (or, if greater, the average of the annual cash bonus earned for the three fiscal years preceding the year in which termination occurs) multiplied by the multiple applicable for such Participant (2.0 for each of Mr. Krishnan and Ms. Krishnan, and 1.5 for all other named executive officers); (2) a cash payment equal to the Participant's target annual bonus for the calendar year in which termination occurs, prorated to reflect the number of days the Participant remained employed in such calendar year; (3) payment of any unpaid annual cash bonus earned for the year prior to the year in which termination occurs; (4) Company-paid (or reimbursed) healthcare coverage under the Consolidated Omnibus Budget Reconciliation Act of 1985, as amended (COBRA) or other applicable law for up to 24 months following the date of termination of the employment of Mr. Krishnan or Ms. Krishnan (18 months for other named executive officers); and (5) treatment of outstanding equity awards as set forth under the Krystal Biotech, Inc. 2017 IPO Stock Incentive Plan (the "Stock Incentive Plan"). The Change in Control Plan contains a modified cutback provision whereby payments payable to a Participant may be reduced if doing so would put the Participant in a more advantageous after-tax position than if payments were not reduced and the Participant became subject to excise taxes under Section 4999 of the Internal Revenue Code of 1986, as amended.

The foregoing description is only a summary and is qualified in its entirety by reference to the full text of the Change in Control Plan, a copy of which is filed as Exhibit 10.1 to this Form 10-Q and incorporated herein by reference.

Amendment to the Krystal Biotech, Inc. 2017 IPO Stock Incentive Plan

On August 2, 2024, upon recommendation of the Compensation Committee, the Board approved an amendment (the "Amendment") to the Stock Incentive Plan. The Amendment provides that in the event of a Change in Control (as defined in the Stock Incentive Plan) in which outstanding equity awards under the Stock Incentive Plan are assumed or replaced by a successor entity on the same terms and conditions as the original awards (such awards, "Assumed or Replaced Awards"), such Assumed or Replaced Awards shall not vest solely as a result of the Change in Control. The Amendment further provides that outstanding equity awards under the Stock Incentive Plan will immediately vest and be exercisable, and any restrictions then in force will lapse: (i) if a successor entity fails to assume or replace an outstanding equity award under the Stock Incentive Plan on the same terms and conditions as the original awards, or (ii) with respect to Assumed or Replaced Awards, upon a

participant's termination of employment without Cause (as defined in the Stock Incentive Plan) that occurs within the 24-month period following the consummation of a Change in Control.

The foregoing description is only a summary and is qualified in its entirety by reference to the full text of the Amendment, a copy of which is filed as Exhibit 10.2 to this Form 10-Q and incorporated herein by reference.

ITEM 6. EXHIBITS

Exhibit Number	
10.1	Krystal Biotech, Inc. Executive Change in Control Severance Plan
10.2	First Amendment to the Krystal Biotech, Inc. 2017 IPO S tock Incentive Plan
31.1	Certification of Periodic Report by Chief Executive Officer under Section 302 of the Sarbanes-Oxley Act of 2002.
31.2	Certification of Periodic Report by Chief Accounting Officer under Section 302 of the Sarbanes-Oxley Act of 2002.
32.1	Certification of Chief Executive Officer and Chief Accounting Officer Pursuant to 18 U.S.C. Section 1350 as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
101	Inline XBRL (Extensible Business Reporting Language). The following materials from this Quarterly Report on Form 10-Q for the period ended June 30, 2024, are formatted in Inline XBRL: (i) consolidated balance sheets of Krystal Biotech, Inc., (ii) consolidated statements of operations of Krystal Biotech, Inc., (iii) consolidated statements of operations and comprehensive income/(loss) of Krystal Biotech, Inc., (iv) consolidated statements of changes in equity of Krystal Biotech, Inc., (v) consolidated statements of cash flows of Krystal Biotech, Inc. and (vi) notes to condensed consolidated financial statements of Krystal Biotech, Inc. The instance document does not appear in the interactive data file because its XBRL tags are embedded within the Inline XBRL document.
104	Cover Page Interactive Data File - the cover page XBRL tags are embedded within the Inline XBRL.

SIGNATURES

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

KRYSTAL BIOTECH, INC.
(Registrant)

Date: August 5, 2024	By:	/s/ Krish S. Krishnan
		Krish S. Krishnan
		President and Chief Executive Officer
		(Principal executive officer)

Date: August 5, 2024	By:	/s/ Kathryn A. Romano
		Kathryn A. Romano
		Chief Accounting Officer
		(Principal financial and accounting officer)

KRYSTAL BIOTECH, INC.
EXECUTIVE CHANGE IN CONTROL SEVERANCE PLAN

1. Purpose. Krystal Biotech, Inc., a Delaware corporation (the "Company"), has adopted the Krystal Biotech, Inc. Executive Change in Control Severance Plan (the "Plan") to provide severance pay and benefits to Eligible Executives whose employment is terminated under qualifying circumstances during the Change in Control Protection Period on or after August 2, 2024 (the "Effective Date"). The Plan is intended to be maintained primarily for the purpose of providing benefits for a select group of management or highly compensated employees. Capitalized terms that are used but not defined in other sections below will have the meanings ascribed to such terms in Section 9 hereof.

2. Administration of the Plan.

(a) Administration by the Committee. The Committee shall be responsible for the management and control of the operation and the administration of the Plan, including interpretation of the Plan, decisions pertaining to eligibility to participate in the Plan, computation of severance benefits, granting or denial of severance benefit claims and review of claims denials. The Committee has absolute discretion in the exercise of its powers and responsibilities. For this purpose, the Committee's powers shall include the following authority, in addition to all other powers provided by the Plan:

(i) to make and enforce such rules and regulations as it deems necessary or proper for the efficient administration of the Plan;

(ii) to interpret the Plan, the Committee's interpretation thereof to be final and conclusive on all persons claiming benefits under the Plan;

(iii) to decide all questions concerning the Plan and the eligibility of any person to participate in the Plan and at what Tier level;

(iv) to make a determination as to the right of any person to a benefit under the Plan (including to determine whether and when there has been a termination of an Eligible Executive's employment and the cause of such termination;

(v) to appoint such agents, counsel, accountants, consultants, claims administrators and other persons as may be required to assist in administering the Plan (and the Committee hereby appoints (x) the General Counsel and Corporate Secretary of the Company and the most senior officer of the Company overseeing the Human Resources function to perform ministerial and administrative tasks under the Plan, and (y) the most senior officer of the Company overseeing the Human Resources function of the Company to serve as claims administrator under the Plan, subject to Section 7 hereof);

(vi) to allocate and delegate its responsibilities under the Plan and to designate other persons to carry out any of its responsibilities under the Plan, any such allocation, delegation or designation to be in writing;

(vii) to sue or cause suit to be brought in the name of the Plan; and

(viii) to obtain from the Company, its Affiliates and from Eligible Executives such information as is necessary for the proper administration of the Plan.

(b) Indemnification of the Committee. The Company shall, without limiting any rights that the Committee may have under the Company's charter or bylaws, applicable law or otherwise, indemnify and hold harmless the Committee and each member thereof (and any other individual acting on behalf of the Committee or any member thereof) against any and all expenses and liabilities arising out of such person's administrative functions or fiduciary responsibilities, excepting only expenses and liabilities arising out of the person's own gross negligence or willful misconduct. Expenses against which such person shall be indemnified hereunder include the amounts of any settlement, judgment, attorneys' fees, costs of court, and any other related charges reasonably incurred in connection with a claim, proceeding, settlement, or other action under the Plan.

(c) Compensation and Expenses. Members of the Committee shall not receive additional compensation with respect to services for the Plan. To the extent required by applicable law, but not otherwise, the members of the Committee shall furnish a bond or security for the performance of their duties hereunder. Any expenses properly incurred by the Committee incident to the administration, termination or protection of the Plan, including the cost of furnishing a bond, shall be paid by the Company.

3. Eligibility. Only individuals who are Eligible Executives may participate in the Plan. The Committee has full and absolute discretion to determine and select which employees of the Company and its Subsidiaries and Affiliates are Eligible Executives. Once an employee becomes an Eligible Executive, such individual shall automatically continue to be an Eligible Executive until the Eligible Executive ceases to be an employee or is removed as an Eligible Executive by the Committee; provided, however, that any change in eligibility will be considered a Plan amendment subject to the restrictions on Plan amendments set forth in Section 8(d) below. The Plan shall supersede all prior agreements, practices, policies, procedures and plans relating to severance payments or benefits provided in connection with a Change in Control from all members of the Company Group with respect to the Eligible Executives (other than with respect to any awards outstanding under the Stock Incentive Plan).

4. Plan Benefits.

(a) Qualifying Termination During a Change in Control Protection Period. In the event an Eligible Executive's employment with any member of the Company Group is terminated due to a Qualifying Termination that occurs during the Change in Control Protection

Period, such Eligible Executive shall be entitled to receive the Accrued Amounts, and so long as such Eligible Executive fully satisfies the Release Requirement and abides by the terms of the Restrictive Covenant Obligations, such Eligible Executive shall also be entitled to receive:

(i) A cash payment equal to the product of (A) the Applicable Severance Multiple, multiplied by (B) the sum of such Eligible Executive's (1) Base Salary and (2) Bonus Opportunity, payable in a lump sum as soon as administratively practicable following the date the Release Requirement is fully satisfied; provided, that, if the consideration period associated with the Release Requirement (including any revocation period) commences in one calendar year and ends in a second calendar year, such payment shall be made on the later of the first payroll date of the second calendar year or the date the Release Requirement is fully satisfied (including the expiration of any revocation period); provided, further, that in no event shall such payment be made later than seventy four (74) days after such Date of Termination;

(ii) The Pro-Rated Bonus, payable in a lump sum on the date the annual bonus for the calendar year that includes the Date of Termination would otherwise have been paid, but in any event no later than March 15 of the calendar year following the end of the calendar year that includes the Date of Termination;

(iii) Payment of any earned but unpaid annual bonus for the year prior to the year in which the Date of Termination occurs, if any, payable in a lump sum at the same time that any such annual bonuses are paid to similarly-situated employees of the Company Group generally; and

(iv) Subject to such Eligible Executive's timely election of continuation coverage under COBRA or other applicable law, during the Applicable Benefit Period, except to the extent otherwise provided by applicable law, the applicable member of the Company Group shall, at its option, pay or reimburse such Eligible Executive on a monthly basis for the amount such Eligible Executive is required to pay for such Eligible Executive and his or her dependents to effect and continue coverage as contemplated by this Section 4(a)(iv); provided, that, if such continued coverage would be discriminatory and would result in the imposition of excise taxes or other liabilities on the Company for failure to comply with any requirements of the Patient Protection and Affordable Care Act of 2010, as amended, the Health Care and Education Reconciliation Act of 2010, as amended (to the extent applicable), or any other applicable law, the Company will provide such Eligible Executive with a cash payment equal to any COBRA premiums, inclusive of any taxes thereon, for the remainder of the Applicable Benefit Period. For the avoidance of doubt, the Applicable Benefit Period shall be deemed to run concurrent with (and shall not extend) the coverage continuation period mandated by COBRA or any other legally mandated and applicable coverage period, and following the Applicable Benefit Period, such Eligible Executive shall be responsible for the full cost associated with any continued coverage, whether under COBRA, any insurance policy conversion rights or otherwise. The Company Group's obligation to provide continuation coverage under this Plan shall immediately terminate if such Eligible Executive becomes eligible for group medical coverage provided by another employer. Such Eligible Executive shall give

prompt notice to the Company if he or she becomes eligible for group medical coverage offered by another employer during the Applicable Benefit Period.

(b) Equity Incentive Awards. Notwithstanding anything to the contrary contained in any award agreement or other agreement between an Eligible Executive and the Company, in the event that an Eligible Executive's employment with any member of the Company Group terminates (pursuant to a Qualifying Termination or otherwise), all outstanding and unvested equity incentive awards then held by the Eligible Executive, pursuant to the Stock Incentive Plan or otherwise, will be treated in accordance with the Stock Incentive Plan, provided, that, for any Eligible Executive, the treatment specified under the Stock Incentive Plan with respect to a termination without Cause shall be applied for any Eligible Executive, with respect to any Qualifying Termination (but, for the avoidance of doubt, shall remain subject to all other terms and conditions as set forth in the Stock Incentive Plan).

(c) Non-Qualifying Terminations of Employment. In the event that an Eligible Executive's employment with any member of the Company Group terminates other than pursuant to a Qualifying Termination that occurs during the Change in Control Protection Period, then all compensation and benefits to the Eligible Executive pursuant to this Plan shall terminate contemporaneously with such termination of employment, except that the Eligible Executive shall be entitled to the Accrued Amounts.

(d) No Duplication. Except as otherwise expressly provided in this Plan, this Plan shall be construed and administered in a manner which avoids duplication of compensation and benefits which may be provided under any other plan, program, policy or other arrangement or individual contract or agreement or under any statute, rule or regulation, including the Worker Adjustment and Retraining Notification Act of 1988 or any similar law. In the event an Eligible Executive is covered by any other plan, program, policy, individually negotiated agreement (including, without limitation, an Employment Agreement), statutory entitlement or other arrangement in effect as of the Date of Termination, that may duplicate the payments and benefits provided for in Section 4(a), the Committee shall reduce or eliminate the duplicative benefits provided for under the Plan.

5. Certain Excise Taxes. Notwithstanding anything to the contrary in the Plan, if an Eligible Executive is a "disqualified individual" (as defined in Section 280G(c) of the Code), and the payments and benefits provided for in the Plan, together with any other payments and benefits which the Eligible Executive has the right to receive from the Company or any of its Affiliates (or any other person who is a party to the transaction constituting a Change in Control), and taking into account reductions in respect of reasonable compensation for personal services to be rendered by the Eligible Executive on or following the date of the relevant "change in ownership or control" (within the meaning of Section 280G of the Code), including pursuant to applicable non-competition and other restrictive covenant obligations, would constitute a "parachute payment" (as defined in Section 280G(b)(2) of the Code), then the payments and benefits provided for in the Plan shall be either (a) reduced (but not below zero) so that the present value of such total amounts and benefits received by the Eligible Executive from the

Company and its Affiliates will be one dollar less than three times the Eligible Executive's "base amount" (as defined in Section 280G(b)(3) of the Code) and so that no portion of such amounts and benefits received by the Eligible Executive shall be subject to the excise tax imposed by Section 4999 of the Code or (b) paid in full, whichever produces the better net after-tax position to the Eligible Executive (taking into account any applicable excise tax under Section 4999 of the Code and any other applicable taxes). The reduction of payments and benefits hereunder, if applicable, shall be made by reducing, first, payments or benefits to be paid in cash hereunder in the order in which such payment or benefit would be paid or provided (beginning with such payment or benefit that would be made last in time and continuing, to the extent necessary, through to such payment or benefit that would be made first in time) and, then, reducing any benefit to be provided in-kind hereunder in a similar order. The determination as to whether any such reduction in the amount of the payments and benefits provided hereunder is necessary shall be made by the Company in good faith. If a reduced payment or benefit is made or provided and through error or otherwise that payment or benefit, when aggregated with other payments and benefits from the Company (or its Affiliates) used in determining if a "parachute payment" exists, exceeds one dollar less than three times the Eligible Executive's base amount, then the Eligible Executive shall promptly repay such excess to the Company upon receipt of a notification that an overpayment has been made. Nothing in this Section 5 shall require the Company to be responsible for, or have any liability or obligation with respect to, the Eligible Executives' excise tax liabilities under Section 4999 of the Code, if any.

6. Restrictive Covenant and Post-Termination Obligations. If the Eligible Executive (i) is party to an Employment Agreement that contains restrictive covenant obligations, including, but not limited to, confidentiality, non-competition, non-solicitation, and non-disparagement covenants, the Eligible Executive shall comply with, and be subject to, such restrictive covenant obligations as a condition of the Eligible Executive's participation in the Plan, or (ii) is not party to an Employment Agreement that contains such restrictive covenant obligations, the Eligible Executive's participation in this Plan shall be subject to (x) the Eligible Executive's execution of the Employee Proprietary Information and Invention Assignment Agreement attached hereto as Exhibit A and (y) the restrictive covenants set forth in this Section 6, and in respect of clause (i) herein, the restrictive covenant obligations set forth herein shall be in addition to, and not in lieu of, any such restrictive obligations set forth in an Employment Agreement (such restrictive covenant obligations set forth in (i) and (ii), as applicable, the "Restrictive Covenant Obligations"). The terms of this Section 6 shall survive the expiration or termination of this Plan for any reason.

(a) Permitted Disclosures. The Eligible Executive understands, agrees and acknowledges that the provisions in this Plan or any other Restrictive Covenant Obligations do not prohibit or restrict Executive from (i) communicating with the Department of Justice, Securities and Exchange Commission, Department of Labor, National Labor Relations Board, Equal Employment Opportunity Commission or any other governmental authority, (ii) exercising the Eligible Executive's rights, if any, under the National Labor Relations Act to engage in protected concerted activity, (iii) making a report in good faith and with a reasonable

belief of any violations of law or regulation to a governmental authority, cooperating with or participating in a legal proceeding relating to such violations including providing documents or other information, or (iv) making any other disclosures that are protected under the whistleblower provisions of any applicable law, rule or regulation. The Eligible Executive is hereby provided notice that under the 2016 Defend Trade Secrets Act: (A) no individual will be held criminally or civilly liable under Federal or State trade secret law for the disclosure of a trade secret (as defined in the Economic Espionage Act) that: (y) is made in confidence to a Federal, State, or local government official, either directly or indirectly, or to an attorney; and made solely for the purpose of reporting or investigating a suspected violation of law, or (z) is made in a complaint or other document filed in a lawsuit or other proceeding, if such filing is made under seal so that it is not made public; and (B) an individual who pursues a lawsuit for retaliation by an employer for reporting a suspected violation of the law may disclose the trade secret to the attorney of the individual and use the trade secret information in the court proceeding, if the individual files any document containing the trade secret under seal, and does not disclose the trade secret, except as permitted by court order.

(b) Non-Competition. To further preserve the Company Group's goodwill, specialized training expertise, and legitimate business interests, the Eligible Executive agrees that during the Restricted Period, the Eligible Executive will not, directly or indirectly, on the Eligible Executive's own behalf or on behalf of any third party, in any capacity (whether as a proprietor, investor, stockholder, partner, officer, director, manager, employee, consultant, contractor, advisor, agent or otherwise, and whether or not for compensation), work for, be a consultant for, be employed or engaged by, lend one's name or assistance to, or provide strategic advice to any Competitor, where the services the Employee would render to the Competitor are similar to those which the Employee performed for the Company. This Section 6(b) does not apply to (1) the Eligible Executive's passive ownership of not more than 2% of the outstanding, publicly traded securities of another company; and (2) employment or provision of services following the Date of Termination in a role that does not have responsibility for, or require performance of, any of the same or similar functions, duties or activities that the Eligible Executive performed or had oversight for on behalf of the Company Group, or is in connection with an independent business line of a Competitor that is wholly separate and unrelated to the confidential information of the Company Group and its products and technologies in effect or under development within the twelve (12) month period immediately preceding the Date of Termination.

(c) Non-Disparagement. Subject to Section 6(a) above, each Eligible Executive agrees that the Eligible Executive, during their employment and at all times thereafter, will not, and will cause the Eligible Executive's Affiliates to not, make, publish, or communicate any disparaging or defamatory comments regarding (whether verbally or in writing) any member of the Company Group or each of their current or former directors, officers, members, managers, partners, executives or direct or indirect owners (including equityholders), investors, businesses, investments, agents products or services.

(d) Cooperation. Each Eligible Executive agrees that during the Eligible Executive's employment with any member of the Company Group and thereafter (regardless of whether the Eligible Executive resigns or the Eligible Executive's employment is terminated by such member of the Company Group or the reason for such resignation or termination), the Eligible Executive shall provide reasonable and timely cooperation in connection with: (i) any actual or threatened litigation, inquiry, review, investigation, process, or other matter, action, or proceeding (whether conducted by or before any court, regulatory, or governmental entity, or by or on behalf of the Company Group, or otherwise), that relates to events occurring during the Eligible Executive's employment by any member of the Company Group or about which the Company Group otherwise believes the Eligible Executive may have relevant information; (ii) the transitioning of the Eligible Executive's role and responsibilities to other personnel; and (iii) the provision of information in response to the Company Group's requests and inquiries in connection with the Eligible Executive's separation of employment. Each Eligible Executive's cooperation shall include being available, at reasonable and mutually agreed times and locations (when possible) to (A) meet with and provide information to the Company Group and its counsel or other agents in connection with fact-finding, investigatory, discovery, and/or pre-litigation or other proceeding issues, at the Company's expense, and (B) provide truthful testimony (including via affidavit, deposition, at trial, or otherwise) in connection with any such matter, all without the requirement of being subpoenaed.

(e) Prior Obligations. Each Eligible Executive hereby represents and warrants that the Eligible Executive is not the subject of, or a party to, any non-competition, non-solicitation, restrictive covenant or non-disclosure agreement, or any other agreement, obligation, restriction or understanding, that would prohibit the Eligible Executive from complying with the Plan or fully performing each of the Eligible Executive's duties and responsibilities for the Company Group, or would in any manner, directly or indirectly, limit or affect any of the duties and responsibilities that may now or in the future be assigned to the Eligible Executive by any member of the Company Group. Each Eligible Executive expressly acknowledges and agrees that the Eligible Executive is strictly prohibited from using or disclosing any confidential information belonging to any prior employer in the course of performing services for any member of the Company Group, and the Eligible Executive promises that the Eligible Executive shall not do so. Each Eligible Executive shall not introduce documents or other materials containing confidential information of any prior employer or any other person, excluding any member of the Company Group, to the premises or property (including computers and computer systems) of any member of the Company Group.

(f) Consent to Notification. If an Eligible Executive ceases to be employed by any member of the Company Group, the Eligible Executive hereby grants consent to notification by the Company Group to any new employer, any third party engaging the Eligible Executive's services, or any entity to which the Eligible Executive becomes a partner, member, employee or otherwise engaged about the Eligible Executive's rights and obligations under the Plan.

7. Claims Procedure and Review. Any claim for Plan benefits shall be made in accordance with this Section 7.

(a) Filing a Claim. Any Eligible Executive that the Committee determines is entitled to severance benefits under the Plan is not required to file a claim for benefits. The Committee shall inform any Eligible Executive upon such Eligible Executive's Qualifying Termination that such Eligible Executive will be eligible for benefits under this Plan, so long as the Eligible Executive satisfies the conditions set forth in Section 4 of this Plan. Any Eligible Executive (i) who is not paid severance benefits hereunder and who believes that such Eligible Executive is entitled to severance benefits hereunder or (ii) who has been paid severance benefits hereunder and believes that such Eligible Executive is entitled to greater benefits hereunder may file a claim for severance benefits under the Plan in writing with the Committee within ninety (90) days after such Date of Termination.

(b) Initial Determination of a Claim. If a claim for severance benefits hereunder has been properly filed, the Committee shall evaluate it and notify the Eligible Executive of the approval, whole denial or partial denial of the claim, within a reasonable period of time but no later than ninety (90) days after receipt of the claim (or one-hundred and eighty (180) days after receipt of the claim if special circumstances require an extension of time for processing the claim). If a claim for severance benefits hereunder is wholly or partially denied, such notice shall (i) be in writing; (ii) be written in a manner calculated to be understood by the Eligible Executive; (iii) contain the specific reason or reasons for denial of the claim; (iv) refer specifically to the pertinent Plan provisions upon which the denial is based; (v) describe any additional material or information necessary for the Eligible Executive to perfect the claim (and explain why such material or information is necessary); and (vi) explain the Eligible Executive's right to appeal the claim and describe the Plan's claim appeal procedures and time limits applicable to such procedures, including a statement of the Eligible Executive's right to bring a civil action under Section 502(a) of ERISA following an adverse benefit determination on review.

(c) Appeal of a Denied Claim. Within sixty (60) days of the receipt by the Eligible Executive of the notice of denial or partial denial of a claim in accordance with Section 7(b), the Eligible Executive may file a written appeal with the Committee. In connection with the appeal, the Eligible Executive may review Plan documents and may submit written issues and comments. The Eligible Executive may also submit issues, arguments and other comments in writing to the Committee, along with any documentary evidence to support their claim. The Committee shall deliver to the Eligible Executive a written decision on the appeal promptly, but not later than sixty (60) days after the receipt of the Eligible Executive's appeal (or one-hundred and twenty (120) days after receipt of the Eligible Executive's appeal if there are special circumstances which require an extension of time for processing). Such decision shall (i) be in writing; (ii) be written in a manner calculated to be understood by the Eligible Executive; (iii) include specific reasons for the decision; (iv) refer specifically to the Plan provisions upon which the decision is based; (v) state that the Eligible Executive is entitled to receive, on request

and free of charge, reasonable access to and copies of all documents, records, and other information relevant to the Eligible Executive's claim for benefits; and (vi) a statement of the Eligible Executive's right to bring an action under Section 502(a) of ERISA.

(d) Special Circumstances. If special circumstances require an extension of up to one-hundred and eighty (180) days for an initial claim or one-hundred and twenty (120) days for an appeal, whichever applies, the Committee shall send written notice of the extension within ninety (90) days after receipt of the initial claim and within sixty (60) days after receipt of an appeal. This notice shall indicate the special circumstances requiring the extension and state when the Committee expects to render the decision.

(e) Claims Limitation Period. Eligible Executives must follow the claims and appeals procedures detailed in this Section 7 before taking legal action or any action in any other form regarding claims for Plan benefits. If the Eligible Executive has completed the entire above claims procedure, and said Eligible Executive disagrees with the Committee's final decision, the Eligible Executive may commence a civil court action under ERISA Section 502(a). Subject to this Section 7, the Eligible Executive shall be required to commence any such civil action in the federal courts of Pennsylvania within one (1) year of the Committee's final decision, and the Eligible Executive hereby consents to the exclusive jurisdiction of such courts for these purposes. If the Eligible Executive does not commence a civil action in such courts and within the claim's limitation period described in the immediately preceding sentence, the Eligible Executive waives all rights to relief under ERISA. An Eligible Executive shall have no right to seek review of a denial of Plan benefits, or to bring any legal action or proceeding to enforce a claim, prior to filing a claim and exhausting his or her administrative remedies under this Section 7.

(f) Compliance with ERISA. The benefits claim procedure provided in this Section 7 is intended to comply with the provisions of 29 C.F.R. §2560.503-1 and ERISA Section 503. All provisions of this Section 7 shall be interpreted, construed, and limited in accordance with such intent.

8. General Provisions.

(a) Taxes. The Company is authorized to withhold from all payments made hereunder amounts of withholding and other taxes due or potentially payable in connection therewith, and to take such other action as the Company may deem advisable to enable the Company and the Eligible Executive to satisfy obligations for the payment of withholding taxes and other tax obligations relating to any payments made under the Plan.

(b) No Mitigation. No Eligible Executive shall have any duty to mitigate the amounts payable under the Plan by seeking or accepting new employment or self-employment following a Qualifying Termination.

(c) Offset. The Company may set off against, and each Eligible Executive authorizes the Company to deduct from, any payments due to the Eligible Executive, or to his or her estate, heirs, legal representatives, or successors, any amounts which may be due and owing to the Company or an Affiliate of the Company by the Eligible Executive, whether arising under the Plan or otherwise; provided, however, that no such offset may be made with respect to amounts payable that are subject to the requirements of Section 409A unless the offset would not result in a violation of the requirements of Section 409A.

(d) Amendment and Termination. The Plan may be amended or terminated by the Board or the Committee from time to time in its discretion and for any reason or no reason; provided, that, during the Change in Control Protection Period, no amendment or termination of the Plan shall impair any rights or obligations to any Eligible Executive under the Plan unless the Eligible Executive expressly consents to such amendment or termination. Notwithstanding the foregoing, the Board or the Committee is required to provide written notice of any amendment to any Eligible Executives who are then participating in the Plan at least eighteen (18) months prior to the effective date of such amendment, and in the event that a Change in Control occurs during such eighteen (18)-month period, then upon any Qualifying Termination following such Change in Control and during the Change in Control Protection Period that follows, such Eligible Executive shall be entitled to receive the greater level of severance benefits provided for under the amended Plan or the Plan prior to such amendment.

(e) Successors. The Plan will be binding upon any successor to the Company, its assets, its businesses or its interest (whether as a result of the occurrence of a Change in Control or otherwise), in the same manner and to the same extent that the Company would be obligated under the Plan if no succession had taken place. All payments and benefits that become due to an Eligible Executive under the Plan will inure to the benefit of his or her beneficiaries, executors, heirs, assigns, designees or legal representatives.

(f) Transfer and Assignment. Neither an Eligible Executive nor any other person shall have any right to sell, assign, transfer, pledge, anticipate or otherwise encumber, transfer, hypothecate or convey any amounts payable under the Plan prior to the date that such amounts are paid.

(g) Unfunded Obligation. All benefits due an Eligible Executive under the Plan are unfunded and unsecured and are payable out of the general assets of the Company. The Company is not required to segregate any monies or other assets from its general funds with respect to these obligations. Eligible Executives shall not have any preference or security interest in any assets of the Company other than as a general unsecured creditor.

(h) Severability; Reformation. If any provision of the Plan (or portion thereof) is held to be illegal, unenforceable or invalid for any reason, the illegality, unenforceability or invalidity of such provision (or portion thereof) will not affect the remaining provisions (or portions thereof) of the Plan, but such provision (or portion thereof) will be fully severable and the Plan will be construed and enforced as if the illegal, unenforceable or invalid

provision (or portion thereof) had never been included herein. The parties agree that a court or arbitrator shall have the power to modify any such illegal, unenforceable or invalid term or provision to the maximum extent permitted by applicable law to render it legal, enforceable and valid while preserving the intention of the parties.

(i) Section 409A. The Plan is intended to comply with Section 409A or an exemption thereunder and shall be construed and administered in accordance with Section 409A. Notwithstanding any other provision of the Plan, payments provided under the Plan may only be made upon an event and in a manner that complies with Section 409A or an applicable exemption. Any payments under the Plan that may be excluded from Section 409A either as separation pay due to an involuntary separation from service or as a short-term deferral shall be excluded from Section 409A to the maximum extent possible. Any payments to be made under the Plan upon the termination of an Eligible Executive's employment shall only be made if such termination of employment constitutes a "separation from service" under Section 409A. In no event may an Eligible Executive, directly or indirectly, designate the calendar year of any payment under this Plan. Each installment payment under the Plan is intended to be a separate payment for purposes of Section 409A. Notwithstanding any provision in the Plan to the contrary, if any payment or benefit provided for herein would be subject to additional taxes and interest under Section 409A if an Eligible Executive's receipt of such payment or benefit is not delayed until the earlier of (i) the date of the Eligible Executive's death; or (ii) the date that is six months after the Date of Termination (such earlier date, the "Section 409A Payment Date"), then such payment or benefit shall not be provided to the Eligible Executive (or the Eligible Executive's estate, if applicable) until the Section 409A Payment Date. Notwithstanding the foregoing, the Company makes no representations that the payments and benefits provided under the Plan are exempt from, or compliant with, Section 409A and in no event shall the Company or any of its Affiliates be liable for all or any portion of any taxes, penalties, interest or other expenses that may be incurred by any Eligible Executive on account of non-compliance with Section 409A.

(j) Governing Law. All questions arising with respect to the provisions of the Plan and payments due hereunder will be determined by application of the laws of the Commonwealth of Pennsylvania, without giving effect to any conflict of law provisions thereof, except to the extent preempted by federal law.

(k) Status. The Company intends that the Plan constitute an unfunded "employee welfare benefit plan" as such term is defined under ERISA for the benefit of a select group of management and highly compensated employees. No Eligible Executive, employee of the Company or any other person shall have any rights to or interest in any specific assets or accounts of the Company or any of its Affiliates by reason of the Plan.

(l) Third-Party Beneficiaries. Each member of the Company Group shall be a third-party beneficiary of the Eligible Executive's covenants and obligations under the Restrictive Covenant Obligations and shall be entitled to enforce such obligations.

(m) No Right to Continued Employment The adoption and maintenance of the Plan shall not be deemed to be a contract of employment between the Company or any of its Affiliates and any person, or to have any impact whatsoever on the at-will employment relationship between the Company or any of its Affiliates and the Eligible Executives. Nothing in the Plan shall be deemed to give any person the right to be retained in the employ of the Company or any of its Affiliates for any period of time or to restrict the right of the Company or any of its Affiliates to terminate the employment of any person at any time.

(n) Title and Headings; Construction. Titles and headings to Sections hereof are for the purpose of reference only and shall in no way limit, define or otherwise affect the provisions hereof. Unless the context requires otherwise, all references herein to laws, regulations, contracts, documents, agreements and instruments refer to such laws, regulations, contracts, documents, agreements and instruments as they may be amended from time to time, and references to particular provisions of laws or regulations include a reference to the corresponding provisions of any succeeding law or regulation. The word “or” as used herein is not exclusive and is deemed to have the meaning “and/or.” The words “herein”, “hereof”, “hereunder” and other compounds of the word “here” shall refer to the entire Plan, and not to any particular provision hereof. Wherever the context so requires, the masculine gender includes the feminine or neuter, and the singular number includes the plural and conversely. The use herein of the word “including” following any general statement, term or matter shall not be construed to limit such statement, term or matter to the specific items or matters set forth immediately following such word or to similar items or matters, whether or not non-limiting language (such as “without limitation”, “but not limited to”, or words of similar import) is used with reference thereto, but rather shall be deemed to refer to all other items or matters that could reasonably fall within the broadest possible scope of such general statement, term or matter. Neither the Plan nor any uncertainty or ambiguity herein shall be construed or resolved against any party hereto, whether under any rule of construction or otherwise. On the contrary, the Plan has been reviewed by each of the parties hereto and shall be construed and interpreted according to the ordinary meaning of the words used so as to fairly accomplish the purposes and intentions of the parties hereto.

(o) Overpayment. If, due to mistake or any other reason, a person receives severance payments or benefits under the Plan in excess of what the Plan provides, such person shall repay the overpayment to the Company in a lump sum within thirty (30) days of receipt of notice of the amount of overpayment. If such person fails to so repay the overpayment, then without limiting any other remedies available to the Company, the Company may deduct the amount of the overpayment from any other amounts which become payable to such person under the Plan or otherwise.

(p) Clawback. Notwithstanding anything in this Plan or any other agreement between the Company and/or its related entities and an Eligible Executive to the contrary, the Eligible Executive acknowledges and agrees that any amounts payable under the Plan to the Eligible Executive are subject to (i) any right that the Company may have under any policy or

other agreement or arrangement with the Eligible Executive (whether in existence as of the Effective Date or later adopted) established by the Company providing for clawback or recovery of amounts that were paid to the Eligible Executive; and (ii) any right or obligation that the Company may have regarding the clawback of "incentive-based compensation" under Section 10D of the Exchange Act and any applicable rules and regulations promulgated thereunder from time to time by the U.S. Securities and Exchange Commission, the listing standards of any national securities exchange or association on which the Company's securities are listed, or any other applicable law. The Company will make any determination for clawback or recovery in its sole discretion and in accordance with applicable laws, regulations, and securities exchange listing standards.

(q) Agent for Service of Legal Process. Legal process may be served on the Committee, which is the plan administrator, at the following address: Krystal Biotech, Inc., 2100 Wharton Street, Suite 701, Pittsburgh, Pennsylvania, 15203.

9. Definitions. For purposes of the Plan, the following terms shall have the respective meanings set forth below:

(a) "Accrued Amounts" means (i) all unpaid Base Salary and/or other earned wages accrued through the Date of Termination, which shall be paid on the Company's first regularly scheduled pay date following the Date of Termination (or earlier if required by applicable law); (ii) reimbursement for all incurred but unreimbursed expenses for which an Eligible Executive is entitled to reimbursement in accordance with the expense reimbursement policies of the Company in effect as of the Date of Termination; and (iii) benefits to which an Eligible Executive may be entitled pursuant to the terms of any plan or policy sponsored by the Company or any of its Affiliates as in effect from time to time (excluding, for the avoidance of doubt, the Stock Incentive Plan).

(b) "Affiliate" means with respect to any person, any other person that directly or indirectly through one or more intermediaries controls, is controlled by or is under common control with, the person in question. As used herein, the term "control" means the possession, direct or indirect, of the power to direct or cause the direction of the management and policies of a person, whether through ownership of voting securities, by contract or otherwise.

(c) "Applicable Benefit Period" means, for each Eligible Executive, the period beginning on the first day of the first month that commences following the Date of Termination and extending for the number of months corresponding to such Eligible Executive's Tier, as set forth on Exhibit B attached hereto.

(d) "Applicable Severance Multiplier" means, for each Eligible Executive, the applicable multiple corresponding to such Eligible Executive's Tier, as set forth on Exhibit B attached hereto.

(e) "Base Salary" means the amount an Eligible Executive is entitled to receive as base salary on an annualized basis, in effect as of the Date of Termination, excluding all annual cash incentive awards, bonuses, equity awards, and incentive compensation payable by the Company or any member of the Company Group as consideration for an Eligible Executive's services. Notwithstanding the foregoing, in the event of a reduction in an Eligible Executive's Base Salary resulting in the Eligible Executive's resignation for Good Reason, for purposes of determining the Eligible Executive's cash severance payments set forth in Section 4(a), the Eligible Executive's Base Salary shall be deemed to be that in effect immediately prior to such reduction.

(f) "Board" means the Board of Directors of the Company.

(g) "Bonus Opportunity" means the dollar amount equal to the greater of (i)(A) an Eligible Executive's target annual bonus percentage for the calendar year that includes the Date of Termination, multiplied by (B) such Eligible Executive's Base Salary, in each case pro-rated for any changes in Base Salary or target annual bonus percentage, as applicable, during the calendar year that includes the Date of Termination (determined without giving effect to any reduction that constituted the basis for a termination for Good Reason pursuant to this Plan); and (ii) the average of the annual bonuses earned by the Eligible Executive under any executive bonus plan in the three fiscal years immediately preceding the calendar year that includes the Date of Termination, provided, that if the Eligible Executive was employed by a member of the Company Group for less than three years prior to the Date of Termination, such average shall be calculated based on the average of such lesser number of whole years immediately preceding the Date of Termination.

(h) "Cause" shall have the meaning set forth in the Employment Agreement; provided, that, if no such agreement exists or no such definition is provided therein, "Cause" means the Eligible Executive's (i) willful misconduct, including, without limitation, violation of sexual or other harassment policy, misappropriation of or material misrepresentation regarding property of the Company, other than customary and de minimis use of the Company property for personal purposes, as determined in the reasonable discretion of the Company; (ii) willful and repeated non-performance of duties (other than by reason of Disability); (iii) willful and repeated failure to follow lawful directives; (iv) a felony conviction, a plea of nolo contendere to a felony by the Eligible Executive, or other conduct by the Eligible Executive that has or would result in material injury to the Company's reputation, including conviction of fraud, theft, embezzlement, or a crime involving moral turpitude; (v) a material breach of any agreement between the Eligible Executive and the Company; or (vi) a significant violation of the Company's employment and management policies made known to the Eligible Executive on the Company's intranet website or otherwise. If the Company elects to terminate for Cause under prongs (ii), (iii), (v) or (vi), the Eligible Executive shall have thirty (30) days to cure to the reasonable satisfaction of the Company after written notice by the Company specifying the alleged conduct giving rise to Cause within thirty (30) days of learning of the alleged conduct,

except where such cause, by its nature, is not curable as determined by the Company or the termination is based upon a recurrence of an act previously cured by the Eligible Executive.

(i) "Change in Control" means the occurrence of any of the following events after the Effective Date:

(i) Any "person," as such term is used in Sections 13(d) and 14(d) of the Exchange Act (other than the Company, any trustee or other fiduciary holding securities under any employee benefit plan of the Company or its Affiliates, or any company owned, directly or indirectly, by the stockholders of the Company in substantially the same proportions as their ownership of Common Stock of the Company), becoming the beneficial owner (as defined in Rule 13d-3 under the Exchange Act), directly or indirectly, of securities of the Company representing 50% or more of the combined voting power of the Company's then outstanding securities;

(ii) During any period of two consecutive years, individuals who at the beginning of such period constitute the Board, and any new director whose election by the Board or nomination for election by the Company's stockholders was approved by a vote of at least two-thirds of the directors then still in office who either were directors at the beginning of the two-year period or whose election or nomination for election was previously so approved, cease for any reason to constitute at least a majority of the Board (excluding any person whose election or nomination for election was a result of either an actual or threatened election contest as such term is used in Rule 14a-11 of Regulation 14A promulgated under the Exchange Act);

(iii) A merger or consolidation of the Company or a subsidiary of the Company with any other corporation, other than a merger or consolidation which would result in the voting securities of the Company outstanding immediately prior thereto continuing to represent (either by remaining outstanding or by being converted into voting securities of the surviving entity) more than 50% of the combined voting power of the voting securities of the Company or the ultimate parent company of the Company outstanding immediately after such merger or consolidation; provided, however, that a merger or consolidation effected to implement a recapitalization of the Company (or similar transaction) in which no person (other than those covered by the exceptions in clause (i)) acquires more than 50% of the combined voting power of the Company's then outstanding securities; and

(iv) A complete liquidation or dissolution of the Company or the consummation of a sale or disposition by the Company of all or substantially all of the Company's assets other than the sale or disposition of all or substantially all of the assets of the Company to a person or persons who beneficially own, directly or indirectly, 50% or more of the combined voting power of the outstanding voting securities of the Company at the time of the sale. Notwithstanding the foregoing, for purposes of any payment made to an Eligible Executive under this Plan that is characterized as "nonqualified deferred compensation" within the meaning of Section 409A, to the extent the impact of a Change in Control on such payment would subject the Eligible Executive to additional taxes under Section 409A, a Change in Control for purposes

of such payment will mean both a Change in Control and a "change in the ownership of a corporation," "change in the effective control of a corporation," or a "change in the ownership of a substantial portion of a corporation's assets" within the meaning of Section 409A as applied to the Company.

(j) "Change in Control Protection Period" means the twenty-four (24)-month period following the consummation of a Change in Control.

(k) "COBRA" means the Consolidated Omnibus Budget Reconciliation Act of 1985, as amended.

(l) "Code" means the Internal Revenue Code of 1986, as amended.

(m) "Committee" means the Compensation Committee of the Board or such other committee duly authorized by the Board to manage and control the operation and administration of the Plan. To the extent the Board elects to administer the Plan or if no committee is duly authorized by the Board to administer this Plan, the term "Committee" shall be deemed to refer to the Board for all purposes under this Plan.

(n) "Common Stock" has the meaning ascribed to such term in the Stock Incentive Plan.

(o) "Company Group" means the Company and each of its direct and indirect Subsidiaries and Affiliates; provided, however, that for purposes of Section 6(b), "Company Group" shall mean the Company and each of its direct and indirect Subsidiaries.

(p) "Competitor" means any person that (i) is engaged in the development of products or technologies which compete with the products or technologies offered by a member of the Company Group, including any products or technologies under development by a member of the Company Group; and (ii) is located within any state, county, municipality, city, or any other jurisdiction or locale in the United States where the Company Group conducts business or has material plans to conduct business and the Eligible Executive has a material presence or influence, in each case of (i) and (ii), at any time during the Eligible Executive's employment with the Company or a member of the Company Group or, for the post-termination portion of the Restricted Period, within the twelve (12) month period immediately preceding the Date of Termination.

(q) "Date of Termination" means the effective date of the termination of an Eligible Executive's employment with the Company or any other member of the Company Group, as applicable, as designated by the Committee or its permitted delegate(s) under this Plan (or, in the case of a termination for Good Reason, the resignation date designated by the Eligible Executive within the applicable time period specified in Section 9(w)), such that the Eligible Executive is no longer employed by, any member of the Company Group as of such designated date.

(r) "Disability" shall have the meaning as set forth in the long-term disability policy of the Company or the member of the Company Group to which the Eligible Executive provides services regardless of whether the Eligible Executive is covered by such policy. If the Company or member of the Company Group to which the Eligible Executive provides services does not have a long-term disability plan in place, "Disability" means that an Eligible Executive is unable to carry out the responsibilities and functions of the position held by the Eligible Executive by reason of any medically determinable physical or mental impairment for a period of not less than ninety (90) consecutive days. An Eligible Executive will not be considered to have incurred a Disability unless he or she furnishes proof of such impairment sufficient to satisfy the Committee in its discretion.

(s) "Eligible Executive" means any employee of the Company Group who (i) has executed and returned a Participation Agreement to the Company; and (ii) is not covered under any other severance plan, policy, program or arrangement sponsored or maintained by any member of the Company Group (other than with respect to an Employment Agreement or an award agreement pursuant to which awards are outstanding under the Stock Incentive Plan). The Committee shall have the sole discretion to determine whether an employee is an Eligible Executive. Notwithstanding anything to the contrary set forth herein, Eligible Executives shall be limited to a select group of management or highly compensated employees within the meaning of Sections 201, 301 and 401 of ERISA.

(t) "Employment Agreement" means (i) an employment agreement, service agreement, or similar agreement or (ii) a proprietary information and invention assignment, or similar agreement, in each case, between the Eligible Executive and the Company.

(u) "ERISA" means the Employee Retirement Income Security Act of 1974, as amended.

(v) "Exchange Act" means the Securities Exchange Act of 1934, as amended from time to time, including the guidance, rules and regulations promulgated thereunder and successor provisions, guidance, rules and regulations thereto.

(w) "Good Reason" shall have the meaning set forth in the Employment Agreement; provided, that, if no such agreement exists or no such definition or similar definition (including, without limitation, "Good Cause") is provided therein, "Good Reason" means the occurrence of any of the following events, unless the Eligible Executive consents to the applicable event: (i) a relocation of the Eligible Executive's principal place of employment outside a fifty (50)-mile radius from the Eligible Executive's office location in effect on the effective date of the Participation Agreement (which, for the avoidance of doubt, shall not include reasonable business travel); (ii) a material diminution in position, responsibilities or authority; or (iii) a material reduction in the Eligible Executive's annual Base Salary or annual bonus target. If the Eligible Executive elects to terminate the Eligible Executive's employment for "Good Reason," the Eligible Executive must provide the Company written notice within thirty (30) days, after which the Company shall have thirty (30) days to cure. If the Company

has not cured and the Eligible Executive elects to terminate the Eligible Executive's employment, the Eligible Executive must do so within ten (10) days after the end of the cure period.

(x) "Participation Agreement" means the participation agreement delivered to each Eligible Executive by the Company prior to the Eligible Executive's entry into the Plan evidencing the Eligible Executive's agreement to participate in the Plan and to comply with all terms, conditions and restrictions within the Plan, in substantially the form set forth on Exhibit C attached hereto.

(y) "Pro-Rated Bonus" means the Eligible Executive's target annual bonus amount for the calendar year that includes the Date of Termination, with the amount paid prorated based on the number of days that such Eligible Executive was employed by any member of the Company Group during the calendar year in which the Date of Termination occurs.

(z) "Qualifying Termination" means the termination of an Eligible Executive's employment (i) by any member of the Company Group without Cause (which, for the avoidance of doubt, does not include a termination due to death or Disability); provided, that such Eligible Executive remains an employee in good standing with the applicable member of the Company Group through the Date of Termination; or (ii) due to an Eligible Executive's resignation for Good Reason.

(aa) "Release Requirement" means the requirement that an Eligible Executive (or his or her beneficiary or executor, in the case of the Eligible Executive's death) execute and deliver to the Company a general release of claims, in substantially the form set forth on Exhibit D attached hereto, on or prior to the date that is twenty-one (21) days following the date upon which the Company delivers the release to the Eligible Executive (which shall occur no later than seven (7) days following the Date of Termination) or, in the event that such termination of employment is "in connection with an exit incentive or other employment termination program" (as such phrase is defined in the Age Discrimination in Employment Act of 1967, as amended), the date that is forty-five (45) days following such delivery date. Notwithstanding the foregoing or any other provision in the Plan to the contrary, the Release Requirement shall not be considered satisfied if the release described in the preceding sentence is (i) executed by the Eligible Executive (or his or her beneficiary or executor, if applicable) before the close of business on the Date of Termination, or (ii) revoked by the Eligible Executive (or his or her beneficiary or executor, as applicable) within any time provided by the Company for such revocation.

(bb) "Restricted Period" means the period during which an Eligible Executive is employed by any member of the Company Group and continuing through the number of months corresponding to such Eligible Executive's Tier, as set forth on Exhibit B attached hereto, following such Date of Termination, regardless of the reason for such termination of employment.

(cc) "Section 409A" means Section 409A of the Code and the Department of Treasury regulations and other interpretive guidance issued thereunder, including any such regulations or guidance that may be amended or issued after the Effective Date.

(dd) "Stock Incentive Plan" means the Krystal Biotech, Inc. 2017 IPO Stock Incentive Plan, as may be amended, restated or otherwise modified from time to time, or any successor equity incentive plan established by the Company.

(ee) "Subsidiary" means any subsidiary corporation of the Company within the meaning of Section 424(f) of the Code.

(ff) "Tier" means the "Tier Level" used for purposes of determining the level of severance benefits an Eligible Executive is eligible to receive, as specified on Exhibit B attached hereto. The level of severance payments and benefits an Eligible Executive shall be eligible to receive under this Plan shall depend upon such Eligible Executive's corresponding "Tier Level" at the time a Qualifying Termination occurs, as specified on Exhibit B attached hereto.

Exhibit A

KRYSTAL BIOTECH, INC.

EMPLOYEE
PROPRIETARY INFORMATION AND INVENTION ASSIGNMENT AGREEMENT

Employee Name: _____

In consideration of my employment or continued employment by Krystal Biotech, Inc. (together with its subsidiaries, the "Company"), which I acknowledge to be good and valuable consideration for my obligations hereunder, intending to be legally bound, I hereby agree to the restrictions and obligations placed by the Company on my use and development of certain information, technology, ideas, inventions and other materials, as set forth in this Employee Proprietary Information and Invention Assignment Agreement (the "Agreement").

1. At-Will Employment. I acknowledge that nothing in this Agreement shall be construed to imply that the term of my employment is guaranteed for any period of time. Unless otherwise stated in a written agreement signed by a duly authorized representative of the Company other than me, my employment is "at-will" and may be terminated for any or no reason, with or without cause and with or without notice.

2. Proprietary Information.

(a) Definition. I understand that the term "Proprietary Information" in this Agreement means any and all information and materials, in whatever form, tangible or intangible, whether disclosed to or learned or developed by me before or after the execution of this Agreement, whether or not marked or identified as confidential or proprietary, pertaining in any manner to the business of or used by the Company and its affiliates, or pertaining in any manner to any person or entity to whom the Company owes a duty of confidentiality. Proprietary Information includes, but is not limited to, the following types of information and materials: (i) research, development, technical or engineering information, know-how, data processing or computer software, programs, tools, data, analyses, designs, diagrams, drawings, schematics, sketches or other visual representations, plans, projects, manuals, documents, files, photographs, results, specifications, trade secrets, inventions, discoveries, compositions, ideas, concepts, structures, improvements, products, prototypes, instruments, machinery, equipment, processes, formulas, algorithms, methods, techniques, works in process, systems, technologies, disclosures, applications and other materials; (ii) financial information and materials, including, without limitation, information and materials relating to costs, vendors, suppliers, licensors, profits, markets, sales, distributors, joint venture partners, customers, subscribers, members and bids, whether existing or potential; (iii) business and marketing information and materials, including, without limitation, information and materials relating to future development and new product concepts; (iv) personnel files and information about the Company's other employees and independent contractors; and (v) any other information or materials relating to the past,

present, planned or foreseeable business, products, developments, technology or activities of the Company.

(b) Exclusions. Proprietary Information does not include any information or materials that I can prove by written evidence (i) is or becomes publicly known through lawful means and without breach of this Agreement by me; (ii) was rightfully in my possession or part of my general knowledge prior to my employment by the Company; or (iii) is disclosed to me without confidential or proprietary restrictions by a third party who rightfully possesses the information or materials without confidential or proprietary restrictions. However, to the extent the Company owes a duty of confidentiality to a third party with respect to such information, idea or material, such information, idea or material shall continue to be Proprietary Information until such time as the Company's duty of confidentiality terminates or expires. If I am uncertain as to whether particular information or materials are Proprietary Information, I will request the Company's written opinion as to their status.

(c) Prior Knowledge. Except as disclosed on Schedule A to this Agreement, I have no information or materials pertaining in any manner to the business of or used by the Company and its affiliates, other than information I have learned from the Company in the course of being hired and employed.

3. Restrictions on Proprietary Information.

(a) Restrictions on Use and Disclosure. I agree that all originals and all copies of manuscripts, letters, notes, notebooks, reports, models, computer files, and other materials containing, representing, evidencing, recording, or constituting any Proprietary Information (created by myself or others) shall be the sole property of the Company or the property of third parties who lawfully disclosed the Proprietary Information under obligations of confidentiality. I agree that, during my employment and at all times thereafter, I will hold the Proprietary Information in strict confidence, and I will not use, reproduce, disclose or deliver, directly or indirectly, any Proprietary Information except to the extent necessary to perform my duties as an employee of the Company or as permitted by a duly authorized representative of the Company. I will use my best efforts to prevent the unauthorized use, reproduction, disclosure or delivery of Proprietary Information by others. Notwithstanding the foregoing, nothing herein or in any other agreement or policy of the Company will prohibit or restrict me from lawfully (i) initiating communications directly with, cooperating with, providing information to, causing information to be provided to, or otherwise assisting in an investigation by any governmental or regulatory agency, entity, or official(s) (collectively, "Governmental Authorities") regarding a possible violation of any law; (ii) responding to any inquiry or legal process from any such Governmental Authorities; (iii) testifying, participating or otherwise assisting in an action or proceeding by any such Governmental Authorities relating to a possible violation of law; or (iv) making any other disclosures that are protected under the whistleblower provisions of any applicable law, rule or regulation. Additionally, pursuant to the federal Defend Trade Secrets Act of 2016, I shall not be held criminally or civilly liable under any federal or state trade secret law for the disclosure of a trade secret that is made: (A)(I) in confidence to a federal, state, or local government official, either directly or indirectly, or to an attorney; and (II) solely for the

purpose of reporting or investigating a suspected violation of law; (B) to my attorney in relation to a lawsuit for retaliation against me for reporting a suspected violation of law; or (C) in a complaint or other document filed in a lawsuit or other proceeding, if such filing is made under seal. Nothing in this Agreement requires me to obtain prior authorization from the Company, or any other person or entity before engaging in any conduct described in this paragraph, or to notify the Company that I have engaged in any such conduct.

(b) Location. I agree to maintain at my work station and/or any other place under my control only such Proprietary Information as I have a current "need to know." I agree to return to the appropriate person or location or otherwise properly dispose of Proprietary Information once that need to know no longer exists.

(c) Third Party Information. I recognize that the Company has received and will receive Proprietary Information from third parties to whom or which the Company owes a duty of confidentiality. In addition to the restrictions set forth in this Section 3, I will not use, reproduce, disclose or deliver such Proprietary Information except as permitted by the Company's agreement with such third party.

(d) Interference with Business. I acknowledge that, because of my responsibilities at the Company, I will help to develop, and will be exposed to, the Company's business strategies, information on customers and clients, and other valuable Proprietary Information and trade secrets, and that use or disclosure of such Proprietary Information and trade secrets in breach of this Agreement would be extremely difficult to detect or prove. I also acknowledge that the Company's relationships with its employees, customers, clients, vendors, and other persons are valuable business assets. Therefore, I agree as follows:

(i) I shall not, during my employment and for a period of one (1) year following termination of my employment with the Company for any reason (the "Restricted Period"), directly or indirectly, solicit, induce, recruit, or encourage any officer, director, employee, independent contractor or consultant of the Company who was employed or engaged by the Company (during the final one (1) year of my employment with respect to the post-termination portion of the Restricted Period) to leave the Company or terminate his or her employment or relationship with the Company.

(ii) I shall not, during the Restricted Period, directly or indirectly, solicit any of the Company's customers, clients, vendors, business partners, or suppliers, or otherwise interfere with any business relationship or contract between the Company and any of its customers, clients, vendors, business partners, or suppliers.

(iii) I shall not, during the Restricted Period, directly or indirectly, solicit, for the purpose of selling products or services offered by the Company, any actual or prospective customer or client of the Company with whom I dealt on behalf of the Company or whose dealings with the Company I coordinated or supervised or about whom I obtained Proprietary Information (during the final one (1) year of my employment with respect to the post-termination portion of the Restricted Period).

I understand and agree that nothing in this Section 3 limits or modifies in any way my duties under any other Section of this Agreement or any applicable law regarding the Company's Proprietary Information.

4. Privacy; Protection of Personal Information.

(a) Privacy. I acknowledge that the Company may access all information and materials generated, received or maintained by or for me on the premises or equipment of the Company (including, without limitation, computer systems and electronic or voice mail systems), and I hereby waive any privacy rights I may have with respect to such information and materials.

(b) Protection of Personal Information. During my employment with the Company and thereafter, I shall hold Personal Information in the strictest confidence and shall not disclose or use Personal Information about other individuals, except in connection with my work for the Company, or unless expressly authorized in writing by an authorized representative of the Company. I understand that there are laws in the United States and other countries that protect Personal Information, and that I must not use Personal Information about other individuals other than for the purposes for which it was originally used or make any disclosures of other individuals' Personal Information to any third party or from one country to another without prior approval of an authorized representative of the Company. I understand that nothing in this Agreement prevents me from discussing my wages or other terms and conditions of my employment with coworkers or others, unless such discussion would be for the purpose of engaging in unfair competition or other unlawful conduct.

(c) Definition of Personal Information. "Personal Information" means personally identifiable information about employees, independent contractors, or third-party individuals, including names, addresses, telephone or facsimile numbers, email addresses, Social Security Numbers, background information, credit card or banking information, health information, or other information entrusted to the Company.

5. Inventions.

(a) Definitions.

(i) I understand that the term "Inventions" in this Agreement means any and all ideas, concepts, inventions, discoveries, developments, modifications, improvements, know-how, trade secrets, data, designs, diagrams, plans, specifications, methods, processes, techniques, formulas, algorithms, tools, works of authorship, derivative works, software, content, textual or artistic works, mask works, video, graphics, sound recordings, structures, products, prototypes, systems, applications, creations and technologies in any stage of development, whether or not patentable or reduced to practice and whether or not copyrightable.

(ii) I understand that the term "Intellectual Property Rights" in this Agreement means any and all (A) patents, utility models, industrial rights and similar intellectual property rights registered or applied for in the United States and all other countries throughout

the world (including all reissues, divisions, continuations, continuations-in-part, renewals, extensions and reexaminations thereof); (B) rights in trademarks, service marks, trade dress, logos, domain names, rights of publicity, trade names and corporate names (whether or not registered) in the United States and all other countries throughout the world, including all registrations and applications for registration of the foregoing and all goodwill related thereto; (C) copyrights (whether or not registered) and rights in works of authorship, databases and mask works, and registrations and applications for registration thereof in the United States and all other countries throughout the world, including all renewals, extensions, reversions or restorations associated with such copyrights, now or hereafter provided by law, regardless of the medium of fixation or means of expression; (D) rights in trade secrets and other confidential information and know-how in the United States and all other countries throughout the world; (E) other intellectual property or proprietary rights in the United States and all other countries throughout the world, including all neighboring rights and *sui generis* rights; (F) rights to apply for, file, register establish, maintain, extend or renew any of the foregoing; (G) rights to enforce and protect any of the foregoing, including the right to bring legal actions for past, present and future infringement, misappropriation or other violations of any of the foregoing; and (H) rights to transfer and grant licenses and other rights with respect to any of the foregoing, in the Company's sole discretion and without a duty of accounting.

(b) Assignment. I hereby assign, and agree to assign automatically upon creation, to the Company, without additional compensation, my entire right, title and interest (including, without limitation, all Intellectual Property Rights) in and to (i) all Inventions that are made, conceived, discovered or developed by me (either alone or jointly with others), or result from or are suggested by any work performed by me (either alone or jointly with others) for or on behalf of the Company or its affiliates, (A) during the period of my employment with the Company, whether before or after the execution of this Agreement and whether or not made, conceived, discovered or developed during regular business hours or (B) during or after the period of my employment with the Company, whether before or after the execution of this Agreement, if based on or using Proprietary Information or otherwise in connection with my activities as an employee of the Company (collectively, the "Company Inventions"), and (ii) all benefits, privileges, causes of action and remedies relating to the Company Inventions, whether before or hereafter accrued (including, without limitation, the exclusive rights to apply for and maintain all registrations, renewals and/or extensions; to sue for all past, present or future infringements or other violations of any rights in the Invention; and to settle and retain proceeds from any such actions), free and clear of all liens and encumbrances. I agree that all such Company Inventions are the sole property of the Company, or any other entity designated by it, and all Intellectual Property Rights shall vest in and inure to the benefit of the Company or such other entity. I agree and acknowledge that all copyrightable Company Inventions shall be considered works made for hire prepared within the scope of my employment.

(c) License. If, under applicable law notwithstanding the foregoing, I retain any right, title or interest (including any Intellectual Property Right) with respect to any Company Invention, I hereby grant and agree to grant to the Company, without any limitations or additional remuneration, a worldwide, exclusive, royalty-free, irrevocable, perpetual, transferable and sublicensable (through multiple tiers) license to make, have made, use, import,

sell, offer to sell, practice any method or process in connection with, copy, distribute, prepare derivative works of, display, perform and otherwise exploit such Company Invention, and I agree not to make any claim against the Company or its affiliates, suppliers, or customers with respect to such Company Invention.

(d) Records; Disclosure. I agree to keep and maintain adequate and current written records regarding all Inventions made, conceived, discovered or developed by me (either alone or jointly with others) during my period of employment or after the termination of my employment if based on or using Proprietary Information or otherwise in connection with my activities as an employee of the Company. I agree to make available such records and disclose promptly and fully in writing to the Company all such Inventions, regardless of whether I believe the Invention is a Company Invention subject to this Agreement, and the Company will examine such disclosure in confidence to make such determination. Any such records related to Company Inventions shall be the sole property of the Company.

(e) Assistance and Cooperation. I agree to cooperate with and assist the Company, and perform, during and after my employment, all acts deemed necessary or desirable by the Company, to apply for, obtain, establish, perfect, maintain, evidence, enforce or otherwise protect any of the full benefits, enjoyment, right, title and interest throughout the world in the Company Inventions. Such acts may include, but are not limited to, execution of assignments of title and other documents and assistance or cooperation in legal proceedings. Should the Company be unable to secure my signature on any such document, whether due to my mental or physical incapacity or any other cause, I hereby irrevocably designate and appoint the Company and each of its duly authorized representatives as my agent and attorney-in-fact, with full power of substitution and delegation, to undertake such acts in my name as if executed and delivered by me (which appointment is coupled with an interest), and I waive and quitclaim to the Company any and all claims of any nature whatsoever that I may have or may later have for infringement of any Intellectual Property Rights in or to the Company Inventions.

(f) Moral Rights. To the extent allowed by applicable law, the assignment of the Company Inventions includes all rights of paternity, integrity, disclosure and withdrawal and any other rights that may be known as or referred to as “moral rights,” “artist’s rights,” “droit moral,” or the like (collectively “Moral Rights”). To the extent I retain any such Moral Rights under applicable law, I hereby waive and agree not to institute, support, maintain or permit any action or proceeding on the basis of, or otherwise assert, such Moral Rights. I hereby authorize the Company to publish the Company Inventions in the Company’s sole discretion with or without attributing any of the foregoing to me or identifying me in connection therewith and regardless of the effect on such Company Inventions or my relationship thereto. I agree to ratify and consent to any action that may be taken or authorized by the Company with respect to such Company Inventions, and I will confirm any such ratifications and consents from time to time as requested by the Company.

(g) Excluded Inventions. I agree to identify inSchedule A all Inventions, if any, that I made, conceived, discovered, or developed (either alone or jointly with others) prior to my employment by the Company that relate to the current or planned conduct of the

Company's business (collectively, "Excluded Inventions"), which I wish to exclude from the scope of this Agreement. I represent and warrant that such list is complete and accurate, and I understand that by not listing an Invention on Schedule A as an Excluded Invention, I am acknowledging that such Invention was not made, conceived, discovered, or developed prior to my employment by the Company.

(h) Employee Inventions and Third-Party Inventions. I shall not, without prior written approval by the Company, make any disclosure to the Company of or incorporate into Company property or Company Inventions any Invention owned by me or in which I have an interest ("Employee Invention") or any Invention owned by a third party. If, in the course of my employment with the Company, I make any disclosure to the Company of or incorporate into Company property or Company Inventions an Employee Invention, with or without Company approval, I hereby grant and agree to grant to the Company a worldwide, nonexclusive, royalty-free, irrevocable, perpetual, transferable and sublicensable (through multiple tiers) license to make, have made, use, import, sell, offer to sell, practice any method or process in connection with, copy, distribute, prepare derivative works of, display, perform and otherwise exploit such Employee Invention, and I agree not to make any claim against the Company or its affiliates, suppliers, or customers with respect to any such Employee Invention.

(i) Representations; Warranties, and Covenants. I represent, warrant, and covenant that: (i) I have the right to grant the rights and assignments granted herein, without the need for any assignments, releases, consents, approvals, immunities, or other rights not yet obtained; (ii) any Company Inventions that are copyrightable works are my original works of authorship; and (iii) neither the Company Inventions nor any element thereof are subject to any restrictions or to any mortgages, liens, pledges, security interests, encumbrances or encroachments.

(j) Adequate Consideration. I acknowledge that the Company Inventions and the associated Intellectual Property Rights may have substantial economic value, that any and all proceeds resulting from use and exploitation thereof shall belong solely to the Company, and that the salary, equity incentives and/or other compensation I receive from the Company for my employment with the Company includes fair and adequate consideration for all assignments, licenses, and waivers hereunder.

6. Prohibition on Disclosure or Use of Third-Party Confidential Information I will not disclose to the Company or induce the Company to use any confidential, proprietary, or trade secret information or materials belonging to others (including without limitation any former employers) at any time, nor will I use any such information or materials in the course of my employment with the Company. I acknowledge and agree that any violation of this provision shall be grounds for my immediate termination and could subject me to substantial civil liabilities and criminal penalties. I acknowledge that no officer or other employee or representative of the Company has requested or instructed me to disclose or use any such information or materials, and I will immediately inform my supervisor in the event I believe that my work at the Company would make it difficult for me not to disclose to the Company any such information or materials.

7. No Conflicts; Former Agreements. I represent and warrant that I have no other agreements or relationships with or commitments to any other person or entity that conflict with my obligations to the Company as an employee of the Company or under this Agreement, and that my employment and my performance of the terms of this Agreement will not require me to violate any obligation to or confidence with another. I agree that I will not enter into any oral or written agreement in conflict with this Agreement. Except as disclosed on Schedule A to this Agreement, I represent and warrant that I have not entered into any other agreements or relationships with or commitments to any other person or entity that obligates me to disclose to any such other person or entity any Proprietary Information or that assigns or obligates me to assign to any such other person or entity any Company Inventions.

8. Third Party and Government Contracts. I understand that the Company has or may enter into contracts with other persons or entities, including the United States or foreign government or their agents, under which certain Intellectual Property Rights will be required to be protected, assigned, licensed, or otherwise transferred. I hereby agree to be bound by all such agreements, and to execute such other documents and agreements as are necessary to enable the Company to meet its obligations under any such contracts.

9. Termination; Return of Materials. I agree to promptly return all property of the Company, including, without limitation, (a) all source code, books, manuals, records, models, drawings, reports, notes, contracts, lists, blueprints, and other documents or materials and all copies thereof, (b) all equipment furnished to or prepared by me in the course of or incident to my employment, and (c) all written or tangible materials containing Proprietary Information in my possession upon termination of my employment for any reason or at any other time at the Company's request. Following my termination, I will not retain any written or other tangible material containing any Proprietary Information or information pertaining to any Company Invention. I understand that my obligations contained in this Agreement will survive the termination of my employment and I will continue to make all disclosures required of me by Section 5(d) above. Following any termination of employment, I will cooperate with the Company in the winding up of pending work on behalf of the Company and the orderly transfer of work to other employees. I will also cooperate with the Company in the defense of any action brought by any third party against the Company that relates to my employment by the Company. In the event of the termination of my employment, I agree, if requested by the Company, to sign and deliver the Termination Certificate attached as Schedule B hereto. I agree that after the termination of my employment, I will not enter into any agreement that conflicts with my obligations under this Agreement and will inform any subsequent employers of my obligations under this Agreement. The termination of any employment or other agreement between the Company and me shall not terminate this Agreement and each and all of the terms and conditions hereof shall survive and remain in full force and effect.

10. Remedies. I recognize that nothing in this Agreement is intended to limit any remedy of the Company under prevailing law governing the protection of trade secrets or other Intellectual Property Rights. In addition, I acknowledge that any breach by me of this Agreement would cause irreparable injury to the Company for which pecuniary compensation would not afford adequate relief and for which it would be extremely difficult to ascertain the amount of

compensation which would afford adequate relief to the Company. Therefore, I agree that if I breach any provision of this Agreement, the Company shall be entitled to injunctive or other equitable relief to remedy any breach or prevent any threatened breach of this Agreement, without the necessity of posting bond or other security or proving it has sustained any actual damage. This remedy will be in addition to any other remedies available to the Company at law or in equity.

11. Miscellaneous Provisions.

(a) Assignment; Binding Effect. I acknowledge and agree that my performance is personal hereunder, and that I shall have no right to assign, delegate or otherwise transfer and shall not assign, delegate or otherwise transfer any rights or obligations under this Agreement. Any such assignment, delegation or other transfer shall be null and void. This Agreement may be freely assigned or transferred by the Company, and nothing in this Agreement shall prevent the consolidation, merger or sale of the Company or a sale of any or all or substantially all of its assets. Subject to the foregoing, this Agreement shall inure to the benefit of the Company and its affiliates, successors, and assigns, and shall be binding on me and my heirs, executors, administrators, devisees, spouses, agents, legal representatives and successors in interest.

(b) Governing Law. This Agreement will be governed by and construed in accordance with the laws of the Commonwealth of Pennsylvania, without giving effect to its conflict of law rules.

(c) Jurisdiction. Except for actions for injunctive or other equitable relief, which may be brought in any court of competent jurisdiction, any legal suit, action or proceeding arising out of or relating to this Agreement shall be commenced in a federal court in the Western District of Pennsylvania or in Pennsylvania state court in the City of Pittsburgh, Pennsylvania in Allegheny County, and each party hereto irrevocably submits to the exclusive jurisdiction and venue of any such court in any such suit, action, or proceeding.

(d) Severability. If any provision of this Agreement, or application thereof to any person, place, or circumstance, shall be held by a court of competent jurisdiction to be invalid, illegal or unenforceable, an arbitrator or reviewing court of appropriate jurisdiction shall have the authority to “blue pencil” or otherwise modify such provision so as to render it enforceable while maintaining the parties’ original intent as reflected herein to the greatest extent possible and the remainder of this Agreement shall remain in full force and effect.

(e) Waivers. Delay or failure to exercise any right or remedy under this Agreement shall not constitute a waiver of such right or remedy nor shall any single or partial exercise of any right or remedy under this Agreement preclude any other or further exercise thereof or the exercise of any other right or remedy. Any waiver of any breach of this Agreement shall not operate as a waiver of any subsequent breaches. No waiver by any party of any of the provisions of this Agreement shall be effective unless explicitly set out in writing and signed by the party so waiving. No waiver by any party shall operate or be construed as a waiver in respect of any failure, breach, or default not expressly identified by such written

waiver, whether of a similar or different character, and whether occurring before or after that waiver. All rights or remedies specified for a party herein shall be cumulative and in addition to all other rights and remedies of the party hereunder or under applicable law.

(f) Interpretation. This Agreement shall be construed as a whole, according to its fair meaning, and not in favor of or against any party. Sections and section headings contained in this Agreement are for reference purposes only and shall not affect in any manner the meaning or interpretation of this Agreement. Whenever the context requires, references to the singular shall include the plural and the plural the singular and any gender shall include any other gender.

(g) Entire Agreement; Amendment. This Agreement, including without limitation the Schedules hereto, constitutes the entire agreement between the Company and me with respect to the subject matter hereof and replaces and supersedes any prior or existing agreement entered into by me and the Company with respect to the subject matter hereof; provided, however, that any other restrictive covenants between the parties shall continue on in full force and effect. This Agreement may not be modified or amended, in whole or in part, except by a writing signed by me and a duly authorized representative of the Company other than me. I agree that any subsequent change in my duties or compensation for employment will not affect the validity or scope of this Agreement.

(h) Counterparts and Electronic Signatures. This Agreement may be executed in two (2) counterparts, each of which shall be deemed an original, and both of which shall constitute one and the same instrument. Either Party may sign this Agreement by (i) providing a scanned image of a handwritten signature that is attached to an electronic document, or (ii) through the use of e-signature software such as DocuSign. A signed copy of this Agreement delivered by email or other means of electronic transmission shall be deemed to have the same legal effect as delivery of an original signed copy of this Agreement.

IF YOU HAVE ANY QUESTIONS CONCERNING THIS AGREEMENT, YOU MAY WISH TO CONSULT AN ATTORNEY. MANAGERS, LEGAL COUNSEL AND OTHERS AT THE COMPANY ARE NOT AUTHORIZED TO GIVE YOU LEGAL ADVICE CONCERNING THIS AGREEMENT.

I HAVE READ THIS AGREEMENT CAREFULLY AND I UNDERSTAND AND ACCEPT THE OBLIGATIONS THAT IT IMPOSES UPON ME WITHOUT RESERVATION. NO PROMISES OR REPRESENTATIONS HAVE BEEN MADE TO ME TO INDUCE ME TO SIGN THIS AGREEMENT. I SIGN THIS AGREEMENT VOLUNTARILY AND FREELY.

Date: _____

_____
Employee Name

_____
Employee Signature

ACKNOWLEDGED AND ACCEPTED:

Krystal Biotech, Inc.

By: _____
Name:
Title

SCHEDULE A
EMPLOYEE DISCLOSURE

1. PROPRIETARY INFORMATION

Except as set forth below, I acknowledge that at this time I know nothing about the business or Proprietary Information [1] of the Company, other than informatior I have learned from the Company in the course of being hired:

(Check here: _____ if continued on additional attached sheets)

2. EXCLUDED INVENTIONS

_____ I have made no Inventions prior to my employment with the Company that relate to the current or planned conduct of the Company's business and that are owned by me (either alone or jointly with others), and I do not wish to exclude any Excluded Inventions from the scope of the Agreement.

_____ The following is a complete and accurate list of all Inventions I have made, conceived, discovered or developed prior to my employment with the Company that relate to the current or planned conduct of the Company's business and that are owned by me (either alone or jointly with others), which I wish to exclude from the scope of the Agreement:

(Check here: _____ if continued on additional attached sheets)

3. CONFLICTING AGREEMENTS

The following information is provided in accordance with Section 7 of the Agreement:

_____ I am not party to any agreement or have any relationship with or commitment to any other person or entity that obligates me to disclose to any such other person or entity any Proprietary Information or that assigns or obligates me to assign to any such other person or entity any Company Inventions.

_____ The following is a complete and accurate list of all agreements, relationships with or commitments to any other person or entity any that obligates me to disclose to any such other person or entity any Proprietary Information or that assigns or obligates me to assign to any such other person or entity any Company Inventions.

(Check here: _____ if continued on additional attached sheets)

Date: _____

_____
Employee Name

_____
Employee Signature

[1] Capitalized terms used herein shall have the meanings set forth in the Proprietary Information and Invention Assignment Agreement between the Company and the Employee.

SCHEDULE B

TERMINATION CERTIFICATE CONCERNING
PROPRIETARY INFORMATION AND COMPANY INVENTIONS

This document is to certify that I have returned all property of Krystal Biotech, Inc. (together with its subsidiaries, the "Company"), including, without limitation, (a) all source code, books, manuals, records, models, drawings, reports, notes, contracts, lists, blueprints, and other documents or materials and all copies thereof, (b) all equipment furnished to or prepared by me in the course of or incident to my employment, and (c) all written and tangible materials containing Proprietary Information (as defined in the Agreement) in my possession.

I further certify that I have reviewed the Employee Proprietary Information and Invention Assignment Agreement (the "Agreement") signed by me and that I have complied with and will continue to comply with all of its terms, including, without limitation, (i) the disclosure of any Inventions (as defined in the Agreement) made, conceived, discovered or developed by me (either alone or jointly with others) during my period of employment or after the termination of my employment if based on or using Proprietary Information or otherwise in connection with my activities as an employee of the Company, and (ii) the preservation as confidential of all Proprietary Information pertaining to the Company. This certificate in no way limits my responsibilities or the Company's rights under the Agreement.

On termination of my employment with the Company, I will be employed by _____in the position of _____.

Date: _____

_____
Employee Name

_____
Employee Signature

Exhibit B

Applicable Severance Multiple and Applicable Benefit Period

Qualifying Termination During a Change in Control Protection Period

Tier Level	Executive Position/ Level	Applicable Severance Multiple	Applicable Benefit Period	Restricted Period
Tier 1	Chief Executive Officer; President, R&D	2x	24 Months	24 Months
Tier 2	Named Executive Officers other than the Chief Executive Officer and President, R&D	1.5x	18 Months	18 Months
Tier 3	Designated Senior Vice Presidents and Designated Vice Presidents*	1x	12 Months	12 Months
*As designated by the Committee				

Exhibit C

Participation Agreement

[DATE]

Re: Participation Agreement – Krystal Biotech, Inc. Executive Change in Control Severance Plan

Dear [NAME]:

We are pleased to inform you that you are eligible to participate in the Krystal Biotech, Inc. Executive Change in Control Severance Plan (as it may be amended from time to time, the "Plan"). Your participation in the Plan is subject to your execution and delivery of this Participation Agreement, and to the terms and conditions of the Plan, which is incorporated herein and deemed to be part of this Participation Agreement for all purposes. Unless otherwise defined herein, capitalized terms used in this Participation Agreement shall have the meanings set forth in the Plan.

As [TITLE] of the Company, you are eligible to receive Tier [●] severance payments and benefits under the Plan, subject to your execution and delivery of this Participation Agreement and the terms and conditions of the Plan.

In signing below, you expressly agree to be bound by, and promise to abide by, the terms of the Restrictive Covenant Obligations, which create certain obligations and restrictions, including, but not limited to, confidentiality, non-competition, non-solicitation, non-disparagement and post-termination cooperation. You agree that the Restrictive Covenant Obligations are reasonable in all respects.

You acknowledge and agree that the Plan and this Participation Agreement supersede all prior severance benefit policies, plans, agreements and arrangements of the Company or any other member of the Company Group and supersede all prior oral or written communications by the Company or any other member of the Company Group with respect to severance benefits provided in connection with or following a Change in Control (other than with respect to any awards outstanding under the Stock Incentive Plan), and all such prior policies, plans, arrangements and communications are hereby null and void and of no further force and effect, solely with respect to your severance entitlements set forth therein.

You further acknowledge and agree that (i) you have fully read, understand and voluntarily enter into this Participation Agreement and (ii) you have had a sufficient opportunity to consult with your personal tax, financial planning advisor and attorney about the tax, financial and legal consequences of your participation in the Plan before signing this Participation Agreement.

This Participation Agreement may be executed in separate counterparts, each of which shall be deemed an original, but all of which taken together shall constitute one and the same instrument. Please execute this Participation Agreement in the space provided below and send a fully executed copy to [NAME] no later than [DATE].

Sincerely,

KRYSTAL BIOTECH, INC.

By: _____
Name
Title

AGREED AND ACCEPTED

_____
(NAME)

Exhibit D

Separation Agreement and General Release

This Separation Agreement and General Release of Claims (this "Release" or "Agreement") is entered into by and between [NAME] ("Executive") and Krystal Biotech, Inc., a Delaware corporation (the "Company").

WHEREAS:

(i) Executive has been employed by the Company;

(ii) Executive's employment has been terminated as of the Separation Date, as defined below; and

(iii) In connection with Executive's termination of employment, Executive is eligible to receive certain severance payments and benefits pursuant to the Company's Executive Change in Control Plan Severance Plan, effective as of August 2, 2024 (the "Plan") and Executive's Participation Agreement entered into under the Plan, dated as of [DATE] (the "Participation Agreement"), subject to the terms and conditions set forth therein including the Release Requirement. Unless otherwise defined herein, capitalized terms used in this Agreement shall have the meanings set forth in the Plan.

NOW THEREFORE, the Company and Executive agree as follows:

1. **Separation Date**. Executive's employment with the Company was or will be permanently terminated on [DATE] (the "Separation Date").

2. **Release**.

(a) For good and valuable consideration set forth in the Plan and Participation Agreement, Executive knowingly and voluntarily (for Executive and Executive's spouses, heirs, executors, administrators, beneficiaries, trustees, successors, and assigns) releases and forever discharges the Company and each of its respective parents, subsidiaries and affiliates, and each of their present, former and future direct or indirect owners, managers, directors, officers, employees, attorneys, agents, members, insurers, shareholders and representatives, and each of their predecessors, successors and assigns (collectively, the "Released Parties") from any and all claims, suits, controversies, actions, causes of action, cross-claims, counter claims, commitments, demands, debts, compensatory damages, liquidated damages, punitive or exemplary damages, other damages, claims for costs and attorneys' fees, or liabilities of any nature whatsoever in law, contract, statute, equity or otherwise, both past and present and whether known or unknown, suspected, unsuspected or claimed (collectively, "Claims") against the Released Parties that Executive or any of Executive's heirs, executors, administrators or assigns, may have (i) from the beginning of time through the date upon which Executive executes this Release; (ii) arising out of, or relating to, Executive's employment with any Released Parties through the date upon which Executive executes this Release; (iii) arising out

of, or relating to, any agreement with any Released Parties, including, but not limited to, any other awards, policies, plans, programs or practices of the Released Parties that may apply to Executive or in which Executive may participate, including, but not limited to, any rights under bonus plans or programs of Released Parties and/or any other short-term or long-term equity-based or cash-based incentive plans or programs of the Released Parties; (iv) arising out of, or relating to, Executive's termination of employment from any of the Released Parties; and/or (v) arising out of, or relating to, Executive's status as an employee, member, officer, or director of any of the Released Parties, including, but not limited to, any allegation, claim or violation, arising under Title VII of the Civil Rights Act of 1964, as amended; the Civil Rights Act of 1991; the Equal Pay Act of 1963, as amended; the Americans with Disabilities Act of 1990; the Family and Medical Leave Act of 1993; the Worker Adjustment and Retraining Notification Act of 1988, as amended; the Employee Retirement Income Security Act of 1974 (with respect to unvested benefits); the Fair Labor Standards Act; the Equal Pay Act, as amended; Section 1981 of U.S.C. Title 42; the Age Discrimination in Employment Act, as amended (including the Older Workers Benefit Protection Act); or their federal, state or local counterparts; or under any other federal, state or local civil or human rights law, or under any other local, state, or federal law, regulation or ordinance; or under any public policy, contract or tort, any doctrine of good faith and fair dealing, or under common law; or arising under any policies, practices or procedures of the Released Parties; or any Claim for wrongful discharge, breach of contract, infliction of emotional distress, defamation; or any Claim for costs, fees, or other expenses, including attorneys' fees incurred in these matters. This is a general release that is intended to apply to all Claims Executive may have against the Released Parties through the date Executive executes this Release, except those Claims that cannot be waived pursuant to applicable laws.

(b) Executive understands that Executive may later discover Claims or facts that may be different than, or in addition to, those which Executive now knows or believes to exist with regards to the subject matter of this Release and the releases in this Section, and which, if known at the time of executing this Release, may have materially affected this Release or Executive's decision to enter into it. Executive hereby waives any right or Claim that might arise as a result of such different or additional Claims or facts.

(c) Executive warrants and represents to the Company that there are no circumstances of which the Executive is aware or of which the Executive ought reasonably to be aware which would amount to a repudiatory breach by the Executive of any express or implied terms of the Executive's employment which would entitle (or would have entitled) the Company to terminate the Executive's employment without notice or payment in lieu of notice and any payment to the Executive pursuant to this Release is conditional on this being so.

(d) Nothing in this Section or this Release shall release or impair: (i) Executive's right to make Claims arising out of any acts or omissions of the Released Parties after the date Executive executes this Release; (ii) any right that cannot be waived by private agreement under law (including the right to file any Claim for workers' compensation or unemployment insurance); (iii) any Claim to vested benefits under the Company's benefit, stock and/or equity plans; (iv) any right to indemnification pursuant to the Employment Agreement, if applicable, or the Company's organizational documents (if any); (v) any right to the benefits

provided pursuant to the Participation Agreement or the Plan; or (vi) any Claim or right that Executive may have under the Participation Agreement or the Plan.

(e) Nothing in this Release is intended to prohibit or restrict Executive's right to file a charge with or participate in a charge by the Equal Employment Opportunity Commission or any other local, state, or federal administrative body or government agency prohibiting waiver of such right; provided, however, that Executive hereby waives the right to recover any monetary damages or other relief against any Released Parties excepting any benefit or remedy to which Executive is or becomes entitled to pursuant to Section 922 of the Dodd-Frank Wall Street Reform and Consumer Protection Act.

(f) Executive acknowledges, understands and agrees that Executive has no knowledge of any actions or inactions by any of the Released Parties or by Executive that Executive believes constitutes a basis for a claimed violation of any federal, state, or local law, any common law or any rule promulgated by an administrative body.

(g) Executive represents that Executive has made no assignment or transfer of any right or Claim covered by this Section and that Executive further agrees that Executive is not aware of any such right or Claim covered by this Section.

(h) Executive acknowledges and agrees that the releases set forth in this Section are an essential and material term of this Release and that without such waiver the Company would not have agreed to the terms of the Participation Agreement or the Plan.

3. **Cooperation; No Cooperation with Non-Governmental Third Parties** Executive shall not knowingly encourage, counsel or assist any non-governmental attorneys or their clients in the presentation or prosecution of any disputes, differences, grievances, claims, charges or complaints by any non-governmental third party against any of the Released Parties, unless compelled to do so by valid subpoena or other court order, and in such case only after first notifying the Company sufficiently in advance of such subpoena or court order to reasonably allow the Company an opportunity to object to the same. Executive agrees to promptly notify the Company via email to [_____] ([EMAIL ADDRESS]) in the event of any requests for information or testimony that Executive receives in connection with any of the foregoing.

4. **Voluntary Agreement**. Executive has carefully read and fully understands all of the provisions of this Release and that Executive is expressly waiving valuable rights. Executive is entering into this Release knowingly, freely and voluntarily in exchange for good and valuable consideration to which Executive would not be entitled in the absence of executing and not revoking this Release.

5. **Consultation; Consideration and Revocation Period**

(a) Executive acknowledges that the Company has advised Executive of Executive's right to consult with an attorney prior to executing this Release.

(b) Executive acknowledges that Executive has been provided with [at least twenty-one (21) calendar days] // [at least forty-five (45) calendar days] (the "Review Period") to consider the offer of this Release prior to entering into it. Any modifications made to this Release, whether material or not, shall not extend or re-start the Review Period. Executive agrees to notify the Company of acceptance of this Release by delivering a signed copy of the Release to the Company, to [NAME], [TITLE], at [EMAIL], within the Review Period. Executive understands that the entire Review Period may be taken to consider this Agreement. Executive may execute and return this Release in less than the full Review Period, but not before Executive's Separation Date. By signing and returning this Release, Executive acknowledges that the Review Period afforded Executive was a reasonable period of time to consider fully each and every term of this Release, including the general release set forth in Section 2. Executive has seven (7) calendar days after the date on which Executive executes this Release to revoke Executive's consent to the Release.

6. **Return of Company Property**. Upon Executive's execution of this Release, Executive acknowledges and agrees that Executive has returned to the Company all documents and information (and all copies thereof) belonging or relating to the business of Company and its affiliates as well as any other Company property or equipment which Executive has or has had in Executive's possession at any time, including, but not limited to, files, notes, drawings, passwords, records, business plans and forecasts, financial information, specifications, computer-recorded information, tangible property (including, but not limited to, computers and/or cell phones), credit cards, entry cards, identification badges and keys, and any other materials of any kind which contain or embody any proprietary or confidential information of the Company or its affiliates (and all reproductions thereof).

7. **Confidentiality, Restrictive Covenant Obligations, and Defend Trade Secrets Act**

(a) Executive acknowledges and warrants that Executive shall remain bound by all continuing obligations set forth in any agreements or other documents with the Company, including, without limitation, the Restrictive Covenant Obligations.

(b) Nothing in this Release or any other agreement with or policy of the Company shall prohibit or restrict Executive or Executive's attorneys from: (i) making any disclosure of relevant and necessary information or documents in any action, investigation, or proceeding relating to this Release, or as required by law or legal process, including with respect to possible violations of law; (ii) participating, cooperating, or testifying in any action, investigation, or proceeding with, or providing information to, any governmental agency or legislative body, any self-regulatory organization, and/or pursuant to the Sarbanes-Oxley Act; (iii) seeking or accepting any U.S. Securities and Exchange Commission awards or other relief in connection with protected whistleblower activity; (iv) initiating communications with, or responding to any inquiry from, any regulatory or supervisory authority regarding any good faith concerns about possible violations of law or regulation; or (v) making any other disclosures that are protected under the whistleblower provisions of any applicable law, rule or regulation. Pursuant to 18 U.S.C. § 1833(b), Executive will not be held criminally or civilly liable under any

Federal or state trade secret law for any disclosure of a trade secret of the Company or its subsidiaries or affiliates that (A) is made (x) in confidence to a Federal, state, or local government official, either directly or indirectly, or to an attorney and (y) solely for the purpose of reporting or investigating a suspected violation of law; or (B) is made in a complaint or other document that is filed under seal in a lawsuit or other proceeding. If Executive files a lawsuit for retaliation by the Company for reporting a suspected violation of law, Executive may disclose the trade secret to Executive's attorney and use the trade secret information in the court proceeding, if Executive files any document containing the trade secret under seal, and does not disclose the trade secret, except pursuant to court order. Nothing in this Release is intended to conflict with 18 U.S.C. § 1833(b) or create liability for disclosures of trade secrets that are expressly allowed by such section.

8. **Future Cooperation**. Executive agrees to be available to and cooperate with the Company as provided in the Plan or Employment Agreement, as applicable.

9. **Resignation from Office**. Executive shall on the Separation Date immediately resign from all offices held by Executive in, or on behalf of, the Company and/or any affiliate by delivering to the Company a letter of resignation in a form acceptable to the Company. Executive shall promptly on request do all and any acts and things as the Company may reasonably require to effect and/or register Executive's resignation under this Section and from all other offices, trusteeships or appointments which Executive holds in connection with or by reason of Executive's employment by the Company and/or any affiliate.

10. **No Admission of Wrongdoing**. Executive agrees that neither this Release, nor the furnishing of the consideration for this Release, shall be deemed or construed at any time to be an admission by any Released Party of any improper or unlawful conduct.

11. **Savings Clause**. If any term or provision of this Release is invalid, illegal or unenforceable in any jurisdiction, such invalidity, illegality or unenforceability shall not affect any other term or provision of this Release or invalidate or render unenforceable such term or provision in any other jurisdiction. Upon such determination that any term or other provision of this Release is invalid, illegal or unenforceable, this Release shall be enforceable as closely as possible to its original intent, which is to provide the Released Parties with a full release of all legally releasable claims through the date upon which Executive signs this Release.

12. **Governing Law and Jurisdiction**. This Release shall be governed by the laws of the Commonwealth of Pennsylvania and Executive expressly consents to the personal jurisdiction of the Pennsylvania state and federal courts for any lawsuit relating to this Release.

13. **Each Party the Drafter**. This Release, and the provisions contained in it, shall not be construed or interpreted for, or against, any party to this Release because that party drafted or caused that party's legal representatives to draft any of its provisions.

14. **Assignment; Third-Party Beneficiaries**. This Release is personal to Executive and may not be assigned by Executive. This Release is binding on, and will inure to the benefit of, the Released Parties. The Released Parties are expressly intended to be third-party

beneficiaries of the releases set forth in the "Release" Section, and it may be enforced by each of them.

15. **Entire Agreement; No Oral Modifications; Counterparts**. This Release sets forth the parties' entire agreement with respect to the subject matter and shall supersede all prior and contemporaneous communications, agreements and understandings, written or oral, with respect hereto and thereto. This Release may not be modified or amended unless mutually agreed to in writing by the parties. This Release may be executed in two or more counterparts, each of which will be an original and all of which together will constitute one and the same instrument. A. .pdf-ed or electronic signature shall operate the same as an original signature. All references to a "Section" of this Release are intended to refer to all paragraph(s) under a single numbered Section.

IN WITNESS THEREOF, the parties have executed this Release as of the date(s) set forth beneath their signatures below.

DO NOT SIGN THIS RELEASE BEFORE THE CLOSE OF BUSINESS ON THE SEPARATION DATE SET FORTH ABOVE.

EXECUTIVE		KRYSTAL BIOTECH, INC.	
Signed:	_____	Signed:	_____
Name:	_____	Name:	_____
Date:	_____	Title:	_____
		Date:	_____

FIRST AMENDMENT TO THE
KRYSTAL BIOTECH, INC.
2017 IPO STOCK INCENTIVE PLAN

This First Amendment (the "Amendment") to the Krystal Biotech, Inc. 2017 IPO Stock Incentive Plan (the "Plan") is made and entered into effective as of August 2, 2024 (the "Effective Date"). Capitalized terms used but not defined herein shall have the meanings ascribed to such terms in the Plan.

RECITALS

WHEREAS, the Company previously established the Plan;

WHEREAS, the Board has the authority to amend the Plan pursuant to Section 13 of the Plan; and

WHEREAS, the Board desires to amend the Plan as set forth in this Amendment.

NOW, THEREFORE, the Board does hereby amend the Plan, effective as of the Effective Date, as follows:

1. Revision of certain definitions contained in Section 2 of the Plan.

(a) Section 2(j) of the Plan is hereby amended by deleting the present Section 2(j) in its entirety and substituting the following in lieu thereof:

"(j) "Change in Control" means the occurrence of any of the following events:

(i) Any "person," as such term is used in Sections 13(d) and 14(d) of the Exchange Act (other than the Company, any trustee or other fiduciary holding securities under any employee benefit plan of the Company or its Affiliates, or any company owned, directly or indirectly, by the stockholders of the Company in substantially the same proportions as their ownership of Common Stock of the Company), becoming the beneficial owner (as defined in Rule 13d-3 under the Exchange Act), directly or indirectly, of securities of the Company representing 50% or more of the combined voting power of the Company's then outstanding securities;

(ii) During any period of two consecutive years, individuals who at the beginning of such period constitute the Board, and any new director whose election by the Board or nomination for election by the Company's stockholders was approved by a vote of at least two-thirds of the directors then still in office who either were directors at the beginning of the two-year period or whose election or nomination for election was previously so approved, cease for any reason to constitute at least a majority of the Board (excluding any person whose election or nomination for election

was a result of either an actual or threatened election contest as such term is used in Rule 14a-11 of Regulation 14A promulgated under the Exchange Act);

(iii) A merger or consolidation of the Company or a Subsidiary with any other corporation, other than a merger or consolidation which would result in the voting securities of the Company outstanding immediately prior thereto continuing to represent (either by remaining outstanding or by being converted into voting securities of the surviving entity) more than 50% of the combined voting power of the voting securities of the Company or the ultimate parent company of the Company outstanding immediately after such merger or consolidation; provided, however, that a merger or consolidation effected to implement a recapitalization of the Company (or similar transaction) in which no person (other than those covered by the exceptions in clause (i) above) acquires more than 50% of the combined voting power of the Company's then outstanding securities; and

(iv) A complete liquidation or dissolution of the Company or the consummation of a sale or disposition by the Company of all or substantially all of the Company's assets other than the sale or disposition of all or substantially all of the assets of the Company to a person or persons who beneficially own, directly or indirectly, 50% or more of the combined voting power of the outstanding voting securities of the Company at the time of the sale. Notwithstanding the foregoing, for purposes of any payment made to a Grantee under this Plan that is characterized as "nonqualified deferred compensation" within the meaning of Section 409A of the Code ("Section 409A"), to the extent the impact of a Change in Control on such payment would subject the Grantee to additional taxes under Section 409A, a Change in Control for purposes of such payment will mean both a Change in Control and a "change in the ownership of a corporation," "change in the effective control of a corporation," or a "change in the ownership of a substantial portion of a corporation's assets" within the meaning of Section 409A as applied to the Company."

(b) Section 2(p) of the Plan is hereby amended by deleting the present Section 2(p) in its entirety and substituting the following in lieu thereof: "(p) Intentionally Blank."

(c) Section 2(r) of the Plan is hereby amended by deleting the present Section 2(r) in its entirety and substituting the following in lieu thereof: "(r) Intentionally Blank." Any other references in the Plan to "Corporate Transaction" shall be replaced in each instance with "Change in Control."

(d) Section 2(kk) of the Plan is hereby amended by deleting the present Section 2(kk) in its entirety and substituting the following in lieu thereof: "(kk) Intentionally Blank." Any other references in the Plan to "Registration Date" shall be replaced in each instance with "effective date of the Plan."

2. Amendment and Restatement of Section 11. Section 11 of the Plan is hereby amended by deleting the present section in its entirety and substituting the following in lieu thereof:

"11. Change in Control.

(a) Treatment of Awards Assumed or Replaced by a Successor Entity. Unless otherwise provided by the Administrator in an Award Agreement or any other written agreement between the Grantee and the Company or a Related Entity, in the event of a Change in Control in which outstanding Awards are Assumed or Replaced under the successor entity's or its Parent's (together, the "Successor Entity") equity compensation plan for outstanding Awards on the same terms and conditions as the original Awards, such Awards that are Assumed or Replaced shall not vest solely as a result of the occurrence of the Change in Control.

(b) Treatment of Awards Assumed or Replaced upon a Termination of Continuous Service Following a Change in Control. If, within twenty-four (24) months following the date of such Change in Control, Grantee's Continuous Service is terminated by the Successor Entity without Cause, any outstanding Assumed Awards or Replaced Awards shall become immediately vested and exercisable. Unless the applicable Award Agreement specifically provides for different treatment, upon the circumstances described in this Section 11(b), Awards that vest based on performance shall be settled at the greater of (i) the target level of performance as set forth in the Award Agreement, and (ii) the actual performance achieved, measured and calculated as of the date of such Change in Control, pursuant to a shortened performance period ending on the occurrence of such Change in Control.

(c) Treatment of Awards not Assumed or Replaced on a Change in Control. Unless otherwise provided by the Administrator in an Award Agreement or any or any other written agreement between the Grantee and the Company or a Related Entity, upon a Change in Control, in which outstanding Awards are not Assumed or Replaced by the Successor Entity as provided in Section 11(a) above, any such Awards shall become immediately vested and exercisable, and any restrictions then in force will lapse, with performance-based Awards deemed earned at the greater of (i) the target level of performance as set forth in the Award Agreement, and (ii) the actual performance achieved, measured and calculated as of the date of the Change in Control, pursuant to a shortened performance period ending on the occurrence of such Change in Control.

(d) Notwithstanding any other provision herein to the contrary, the Administrator may, in its sole discretion, provide for accelerated vesting or lapse of restrictions, of an Award at any time."

3. Miscellaneous. Except as expressly set forth herein, all terms and provisions contained in the Plan shall remain in full force and effect and are hereby ratified and confirmed. The provisions of this Amendment shall be binding upon, and shall inure to the benefit of, the successors and assigns of the Company and Grantees, respectively. This Amendment shall be governed by and construed in accordance with the laws of the State of Delaware.

Exhibit 31.1

CERTIFICATION OF CHIEF EXECUTIVE OFFICER
PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, Krish S. Krishnan, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Krystal Biotech, 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 Condensed 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)) 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. 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

   c. 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: August 5, 2024

By: /s/ Krish S. Krishnan
Krish S. Krishnan
President and Chief Executive Officer

Exhibit 31.2

CERTIFICATION OF CHIEF ACCOUNTING OFFICER
PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, Kathryn A. Romano, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Krystal Biotech, 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 Condensed 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) 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. 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

   c. 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: August 5, 2024

By: /s/ Kathryn A. Romano
Kathryn A. Romano
Chief Accounting Officer

Exhibit 32.1

CERTIFICATION OF CHIEF EXECUTIVE OFFICER AND CHIEF ACCOUNTING OFFICER
PURSUANT TO
18 U.S.C. SECTION 1350,
AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

I, Krish S. Krishnan, Chief Executive Officer, certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that:

1. the Quarterly Report on Form 10-Q for the three months ended June 30, 2024, (the "Periodic Report") fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and

2. information contained in the Periodic Report fairly presents, in all material respects, the financial condition and results of operations of Krystal Biotech, Inc.

Date: August 5, 2024

By: /s/ Krish S. Krishnan
Krish S. Krishnan
President and Chief Executive Officer

I, Kathryn A. Romano, Chief Accounting Officer, certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that:

1. the Quarterly Report on Form 10-Q for the three months ended June 30, 2024, (the "Periodic Report") fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and

2. information contained in the Periodic Report fairly presents, in all material respects, the financial condition and results of operations of Krystal Biotech, Inc.

Date: August 5, 2024

By: /s/ Kathryn A. Romano
Kathryn A. Romano
Chief Accounting Officer