REFINITIV

DELTA REPORT

10-Q

KRYS - KRYSTAL BIOTECH, INC.

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

The following comparison report has been automatically generated

TOTAL DELTAS	2574
CHANGES	99
DELETIONS	1098
ADDITIONS	1377

UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549

FORM 10-Q

(Mark One)

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

For the quarterly period ended September 30, 2023 March 31, 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 October 30, 2023 April 29, 2024, there were 28,206,330 28,558,440 shares of the registrant's common stock issued and outstanding.

Krystal Biotech, Inc.
TABLE OF CONTENTS

PART I. FINANCIAL INFORMATION

ITEM 1. FINANCIAL STATEMENTS

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

(In thousands, except share and per share data)	September 30, 2023	December 31, 2022
(in thousands, except par value)		

(in thousands, except par value)			
(in thousands, except par value)		**March 31, 2024**	**December 31, 2023**
Assets	**Assets**		
Current assets	Current assets		
Current assets			
Current assets			
Cash and cash equivalents			
Cash and cash equivalents			
Cash and cash equivalents	Cash and cash equivalents	$ 373,241	$ 161,900
Short-term investments	Short-term investments	188,828	217,271
Accounts receivable, net	Accounts receivable, net	9,316	—
Inventory	Inventory	5,278	—
Prepaid expenses and other current assets	Prepaid expenses and other current assets	5,465	4,608
Total current assets	Total current assets	582,128	383,779
Property and equipment, net	Property and equipment, net	164,029	161,684
Long-term investments	Long-term investments	36,548	4,621
Right-of-use assets	Right-of-use assets	7,360	8,042
Other non-current assets	Other non-current assets	285	324
Total assets	Total assets	$ 790,350	$ 558,450
Liabilities and Stockholders' Equity			
Liabilities and Stockholders’ Equity			
Current liabilities			
Current liabilities			
Current liabilities	Current liabilities		
Accounts payable	Accounts payable	$ 4,340	$ 3,981
Accounts payable			
Accounts payable			

Current portion of lease liability	Current portion of lease liability	1,501	1,561
Accrued rebates			
Accrued expenses and other current liabilities	Accrued expenses and other current liabilities	21,742	23,305
Total current liabilities	Total current liabilities	27,583	28,847
Total current liabilities			
Total current liabilities			
Lease liability	Lease liability	6,819	7,372
Total liabilities	Total liabilities	34,402	36,219
Commitments and contingencies (Note 7)			
Commitments and contingencies (see note 7)	Commitments and contingencies (see note 7)		
Stockholders' equity	Stockholders' equity		
Common stock; $0.00001 par value; 80,000,000 shares authorized at September 30, 2023 and December 31, 2022; 28,194,655 shares issued and outstanding at September 30, 2023; and 25,763,743 shares issued and outstanding at December 31, 2022		—	—
Common stock; $0.00001 par value; 80,000 shares authorized as of March 31, 2024 and December 31, 2023; 28,528 and 28,237 shares issued and outstanding as of March 31, 2024 and December 31, 2023, respectively.			
Common stock; $0.00001 par value; 80,000 shares authorized as of March 31, 2024 and December 31, 2023; 28,528 and 28,237 shares issued and outstanding as of March 31, 2024 and December 31, 2023, respectively.			

Common stock; $0.00001 par value; 80,000 shares authorized as of March 31, 2024 and December 31, 2023; 28,528 and 28,237 shares issued and outstanding as of March 31, 2024 and December 31, 2023, respectively.			
Additional paid-in capital	Additional paid-in capital	1,034,849	803,718
Accumulated other comprehensive loss		(382)	(728)
Accumulated other comprehensive (loss) gain			
Accumulated deficit	Accumulated deficit	(278,519)	(280,759)
Total stockholders' equity		755,948	522,231
Total liabilities and stockholders' equity		$ 790,350	$ 558,450
Total stockholders' equity			
Total liabilities and stockholders' equity			

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) Loss
(unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
(In thousands, except share and per share data)	**2023**	**2022**	**2023**	**2022**
Product revenues, net	$ 8,556	$ —	$ 8,556	$ —
Expenses:				
(in thousands, except per share data)				
(in thousands, except per share data)				
(in thousands, except per share data)				
Product revenue, net				
Product revenue, net				
Product revenue, net				
Expenses				
Expenses				
Expenses				
Cost of goods sold				

Cost of goods sold					
Cost of goods sold	Cost of goods sold	223	—	223	—
Research and development	Research and development	10,629	11,516	35,061	31,720
Research and development					
Research and development					
Selling, general, and administrative					
Selling, general, and administrative					
Selling, general, and administrative	Selling, general, and administrative	23,697	19,935	73,637	53,705
Litigation settlement	Litigation settlement	—	—	12,500	25,000
Litigation settlement					
Litigation settlement					
Total operating expenses	Total operating expenses	34,549	31,451	121,421	110,425
(Loss) from operations		(25,993)	(31,451)	(112,865)	(110,425)
Other income:					
Gain from sale of priority review voucher		100,000	—	100,000	—
Total operating expenses					
Total operating expenses					
Loss from operations					
Loss from operations					
Loss from operations					
Other income					
Other income					
Other income					
Interest and other income, net	Interest and other income, net	6,740	1,601	15,105	2,502
Interest and other income, net					
Interest and other income, net					
Net income (loss)	Net income (loss)	$ 80,747	$ (29,850)	2,240	(107,923)
Unrealized (loss) gain on available-for-sale securities and currency translation adjustment		(146)	70	346	(1,312)
Comprehensive income (loss)		$ 80,601	$ (29,780)	$ 2,586	$ (109,235)
Net income (loss)					
Net income (loss)					
Unrealized (loss) gain on available-for-sale securities and other					
Unrealized (loss) gain on available-for-sale securities and other					
Unrealized (loss) gain on available-for-sale securities and other					
Comprehensive loss					
Comprehensive loss					

Comprehensive loss									
Net income (loss) per common share:									
Net income (loss) per common share:									
Net income (loss) per common share:	Net income (loss) per common share:								
Basic	Basic	$	2.88	$	(1.17)	$	0.08	$	(4.24)
Basic									
Basic									
Diluted									
Diluted									
Diluted	Diluted	$	2.79	$	(1.17)	$	0.08	$	(4.24)
Weighted-average common shares outstanding:	Weighted-average common shares outstanding:								
Weighted-average common shares outstanding:									
Weighted-average common shares outstanding:									
Basic									
Basic									
Basic	Basic		28,042,130		25,619,125		26,812,278		25,428,097
Diluted	Diluted		28,892,226		25,619,125		27,384,539		25,428,097
Diluted									
Diluted									

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

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

	Common Stock			Additional Paid-in		Accumulated Other Comprehensive		Accumulated		Total Stockholders'
(In thousands, except shares)	**Shares**		**Amount**		**Capital**		**Loss**		**Deficit**	**Equity**
Balances at January 1, 2023	25,763,743	$	—	$	803,718	$	(728)	$	(280,759)	$ 522,231
Issuance of common stock, net	42,021		—		2,208		—		—	2,208
Shares surrendered for taxes	(9,551)		—		(749)		—		—	(749)
Stock-based compensation expense	—		—		10,599		—		—	10,599
Unrealized gain on investments and other	—		—		—		574		—	574
Net loss	—		—		—		—		(45,297)	(45,297)
Balances at March 31, 2023	25,796,213	$	—	$	815,776	$	(154)	$	(326,056)	$ 489,566
Issuance of common stock, net	2,178,703		—		185,397		—		—	185,397
Stock-based compensation expense	—		—		11,443		—		—	11,443
Unrealized (loss) on investments and other	—		—		—		(82)		—	(82)
Net loss	—		—		—		—		(33,210)	(33,210)
Balances at June 30, 2023	27,974,916	$	—	$	1,012,616	$	(236)	$	(359,266)	$ 653,114
Issuance of common stock, net	219,739		—		13,511		—		—	13,511
Stock-based compensation expense	—		—		8,722		—		—	8,722
Unrealized (loss) on investments and other	—		—		—		(146)		—	(146)

Net income	—	—	—	—	80,747	80,747
Balances at September 30, 2023	28,194,655	$ —	$ 1,034,849	$ (382)	$ (278,519)	$ 755,948

	Common Stock		**Additional Paid-in**	**Accumulated Other Comprehensive**		**Total**
(in thousands)	**Shares**	**Amount**	**Capital**	**Income (Loss)**	**Accumulated Deficit**	**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, net	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 as of March 31, 2024	28,528	$ —	$ 1,068,436	$ (299)	$ (268,895)	$ 799,242

	Common Stock		**Additional Paid-in**	**Accumulated Other Comprehensive**	**Accumulated**	**Total Stockholders'**
(In thousands, except shares)	**Shares**	**Amount**	**Capital**	**(Loss)**	**Deficit**	**Equity**
Balances at January 1, 2022	25,207,985	$ —	$ 734,523	$ (163)	$ (140,784)	$ 593,576
Issuance of common stock, net	1,475	—	55	—	—	55
Shares surrendered for taxes and forfeitures	(10,379)	—	(649)	—	—	(649)
Stock-based compensation expense	—	—	6,571	—	—	6,571
Unrealized (loss) on investments and other	—	—	—	(1,034)	—	(1,034)
Net loss	—	—	—	—	(49,965)	(49,965)
Balances at March 31, 2022	25,199,081	$ —	$ 740,500	$ (1,197)	$ (190,749)	$ 548,554
Issuance of common stock, net	472,706	—	30,748	—	—	30,748
Shares forfeited	(7,500)	—	—	—	—	—
Stock-based compensation expense	—	—	8,335	—	—	8,335
Unrealized (loss) on investments and other	—	—	—	(348)	—	(348)
Net loss	—	—	—	—	(28,108)	(28,108)
Balances at June 30, 2022	25,664,287	$ —	$ 779,583	$ (1,545)	$ (218,857)	$ 559,181
Issuance of common stock, net	45,377	—	2,176	—	—	2,176
Stock-based compensation expense	—	—	9,195	—	—	9,195
Unrealized gain on investments and other	—	—	—	70	—	70
Net loss	—	—	—	—	(29,850)	(29,850)
Balances at September 30, 2022	25,709,664	$ —	$ 790,954	$ (1,475)	$ (248,707)	$ 540,772

	Common Stock		**Additional Paid-in**	**Accumulated Other Comprehensive**		**Total**
(in thousands)	**Shares**	**Amount**	**Capital**	**Income (Loss)**	**Accumulated Deficit**	**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, net	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 as of March 31, 2023	25,796	$ —	$ 815,776	$ (154)	$ (326,056)	$ 489,566

(1) Includes foreign currency translation losses of $62 thousand and $35 thousand for the three months ended March 31, 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)

		Nine Months Ended September 30, 2023	Nine Months Ended September 30, 2022		Three Months Ended March 31, 2024	Three Months Ended March 31, 2023
(In thousands)		**2023**	**2022**			
(in thousands)				*(in thousands)*	**2024**	**2023**
Operating Activities	**Operating Activities**					
Net income (loss)	Net income (loss)	$ 2,240	$(107,923)			
Adjustments to reconcile net income (loss) to net cash (used in) operating activities						
Gain from sale of priority review voucher		(100,000)	—			
Depreciation and amortization		2,569	2,966			
Stock-based compensation expense		30,080	23,678			
Loss on disposals of fixed assets		—	22			
Net income (loss)						
Net income (loss)						
Adjustments to reconcile net income (loss) to net cash provided by (used in) operating activities						
Depreciation						
Depreciation						
Depreciation						
Accretion on marketable securities						
Amortization of operating lease right-of-use assets						
Stock-based compensation expense, net						
Realized gain on investments						
Realized gain on investments						

Realized gain on investments			
Other, net	Other, net	(4,290)	(224)
Changes in operating assets and liabilities	Changes in operating assets and liabilities		
Accounts receivable	Accounts receivable	(9,316)	—
Accounts receivable			
Accounts receivable			
Inventory	Inventory	(3,983)	—
Prepaid expenses and other current assets	Prepaid expenses and other current assets	(137)	1,031
Other non-current assets	Other non-current assets	(48)	(31)
Lease liability	Lease liability	(603)	(459)
Accounts payable	Accounts payable	121	(316)
Accrued expenses and other current liabilities	Accrued expenses and other current liabilities	1,795	3,016
Net cash (used in) operating activities		(81,572)	(78,240)
Accrued rebates			
Accrued litigation settlement			
Net cash provided by (used in) operating activities			
Investing Activities	**Investing Activities**		
Proceeds from sale of priority review voucher		100,000	—
Investing Activities			
Investing Activities			
Purchases of property and equipment			
Purchases of property and equipment			
Purchases of property and equipment	Purchases of property and equipment	(9,952)	(47,762)
Purchases of investments	Purchases of investments	(425,870)	(214,712)
Proceeds from maturities of investments		428,620	153,599

Maturities of investments			
Net cash provided by (used in) investing activities		92,798	(108,875)
Net cash (used in) provided by investing activities			
Net cash (used in) provided by investing activities			
Net cash (used in) provided by investing activities			
Financing Activities	**Financing Activities**		
Issuance of common stock, net of issuance costs		200,880	32,927
Financing Activities			
Financing Activities			
Proceeds from exercise of stock options, net			
Proceeds from exercise of stock options, net			
Proceeds from exercise of stock options, net			
Taxes paid for employee tax withholding related to restricted stock units			
Taxes paid related to settlement of restricted stock awards	Taxes paid related to settlement of restricted stock awards	(749)	(649)
Net cash provided by financing activities	Net cash provided by financing activities	200,131	32,278
Net cash provided by financing activities			
Net cash provided by financing activities			
Effect of exchange rate changes on cash and cash equivalents			
Effect of exchange rate changes on cash and cash equivalents			
Effect of exchange rate changes on cash and cash equivalents	Effect of exchange rate changes on cash and cash equivalents	(16)	—

Net increase (decrease) in cash and cash equivalents	Net increase (decrease) in cash and cash equivalents	211,341	(154,837)
Net increase (decrease) in cash and cash equivalents			
Net increase (decrease) in cash and cash equivalents			
Cash and cash equivalents at beginning of period			
Cash and cash equivalents at beginning of period			
Cash and cash equivalents at beginning of period	Cash and cash equivalents at beginning of period	161,900	341,246
Cash and cash equivalents at end of period	Cash and cash equivalents at end of period	$373,241	$ 186,409
Supplemental Disclosures of Non-Cash Investing and Financing Activities			
Supplemental Disclosures of Non-Cash Investing Activities			
Supplemental Disclosures of Non-Cash Investing Activities			
Supplemental Disclosures of Non-Cash Investing Activities			
Unpaid purchases of property and equipment included in accounts payable and accrued expenses	Unpaid purchases of property and equipment included in accounts payable and accrued expenses	$ 11,103	$ 15,305
Initial recognition of right-of-use assets		$ —	$ 1,556
Unpaid purchases of property and equipment included in accounts payable and accrued expenses			
Unpaid purchases of property and equipment included in accounts payable and accrued expenses			

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, the Company converted from a California limited liability company to a Delaware C-corporation, and changed its 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, the

Company 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, and August 2023, and March 2024 the Company incorporated wholly-owned subsidiaries in Switzerland, Netherlands, France, Germany, and Germany, Japan, respectively, for the purpose of establishing initial operations in Europe and Japan for the commercialization of the Company’s 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. Our approach leverages Using our patented gene therapy technology platform that is based on an engineered Herpes Simplex Virus-1 herpes simplex virus-1 (“HSV-1”) vector to, 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. We formulate our 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 Practices ("CGMP" Practice (“CGMP”) manufacturing facilities.

On May 19, 2023, the Company received U.S. Food and Drug Administration (“FDA”) approval for its first product, VYJUVEK® (“VYJUVEK”) for the treatment of Dystrophic Epidermolysis Bullosa (“DEB”) in patients six months or older. Additionally, the Company received a Rare Pediatric Disease Priority Review Voucher (“PRV”) in connection with the VYJUVEK approval. VYJUVEK became commercially available upon approval, and we began generating revenue from VYJUVEK product sales in 3Q 2023.

Liquidity

As of September 30, 2023 March 31, 2024, the Company had an accumulated deficit of $278.5 million $268.9 million. As the Company continues to incur operating losses, a Our transition to operating profitability is dependent upon the continued successful commercialization of VYJUVEK as well as successful development, approval, and commercialization of its our other product candidates and the achievement of a level of revenues adequate to support the Company’s cost structure. The Company may never achieve operating profitability and unless and until it does, the Company will continue to need to raise additional capital or obtain financing from other sources. candidates. Management intends to fund future operations through its on hand cash and cash equivalents, revenue generated from the sale of VYJUVEK, the sale of equity, debt financings, and may also seek additional capital through 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 believes that its cash, cash equivalents and short-term investments of approximately $562.1 million $538.3 million as of September 30, 2023 March 31, 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 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, 2022 December 31, 2023 (“2023 10-K”), as filed with the U.S. Securities and Exchange Commission (“SEC”) on February 27, 2023 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.

Segment and Geographical Information

Operating segments are defined as components of an enterprise about which separate discrete information is available for evaluation by the chief operating decision maker, or decision-making group, in deciding how to allocate resources and in assessing performance. The Company and the Company's chief operating decision maker view the Company's operations and manage its business in one operating segment, which is the business of developing and commercializing pharmaceutical products.

Cash, Cash Equivalents and Investments

Cash and cash equivalents consist of money market funds and bank deposits. Cash equivalents are defined as short-term, highly liquid investments with original maturities of 90 days or less at the date of purchase.

Investments with maturities of less than one year are classified as short-term investments on the condensed consolidated balance sheets and consist of commercial paper, corporate bonds, and U.S. government agency securities. Investments with maturities of greater than one year are classified as long-term investments on the condensed consolidated balance sheets and consist of corporate bonds and government agency securities. Accrued interest on investments is also classified as short-term investments on the condensed consolidated balance sheets.

As our entire investment portfolio is considered available for use in current operations, we classify all investments as available-for-sale securities. Available-for-sale securities are carried at fair value, with unrealized gains and losses reported in accumulated other comprehensive loss, which is a separate component of stockholders' equity in the condensed consolidated balance sheets. Any premium arising at purchase is amortized to the earliest call date and any discount arising at purchase is accreted to maturity. Amortization and accretion of premiums and discounts are recorded in interest and other income, net, in the condensed consolidated statements of operations and comprehensive income (loss).

Fair Value of Financial Instruments

Fair value is defined as the price that would be received to sell an asset or paid to transfer a liability in an orderly transaction between market participants at the measurement date. There is a three-level hierarchy that prioritizes the inputs used in determining fair value by their reliability and preferred use, as follows:

- *Level 1*— Valuations based on quoted prices in active markets for identical assets or liabilities.
- *Level 2*— Valuations based on quoted prices in active markets for similar assets and liabilities, quoted prices for identical or similar assets or liabilities in inactive markets, or other inputs that are observable or can be corroborated by observable market data.
- *Level 3*— Valuations based on inputs that are both significant to the fair value measurement and unobservable.

To the extent that a valuation is based on models or inputs that are less observable, or unobservable in the market, the determination of fair value requires more judgment. Accordingly, the degree of judgment exercised by the Company in determining fair value is greatest for instruments categorized within Level 3. A financial instrument's level within the fair value hierarchy is based on the lowest level of any input that is significant to the fair value measurement.

There have been no significant changes to the valuation methods utilized by the Company during the periods presented. There have been no transfers between Level 1, Level 2, and Level 3 in any periods presented.

The carrying amounts of financial instruments consisting of cash and cash equivalents, investments, accounts receivable, net, prepaid expenses and other current assets, accounts payable, accrued expenses and other current liabilities included in the Company's condensed consolidated financial statements, approximate fair value, primarily due to their short maturities. Our available-for-sale, short-term and long-term investments are considered to be Level 2 financial instruments.

Revenue Recognition

The Company sells VYJUVEK to a limited number of specialty pharmacies ("SPs") that mix the medication and administer it to patients in the patient's home by a healthcare professional and a single specialty distributor ("SD"), that distributes VYJUVEK to hospitals and outpatient clinics where patients are administered the medication at a healthcare professional's office.

The Company recognizes product revenue under ASC Topic 606, *Revenue from Contracts with Customers ("Topic 606").* Under Topic 606, the Company is required to complete the following five steps:

(i) identify the contract(s) with a customer; (ii) identify the performance obligations in the contract; (iii) determine the transaction price, including variable consideration, if any; (iv) allocate the transaction price to the performance obligations in the contract; and (v) recognize revenue when (or as) the entity satisfies a performance obligation.

The Company recognizes revenue when the customer obtains control of the product, which occurs at a point in time, upon delivery to the customer. Sales and other taxes collected concurrent with revenue-producing activities are excluded from revenue. Revenue is measured as the amount of consideration the Company expects to receive in exchange for transferring VYJUVEK and is generally based upon a list or fixed price less allowances for returns, rebates and discounts. The Company's payment terms are generally 30 to 60 days from the invoice date.

Variable Consideration

Product revenues is recorded at the net sales price, or transaction price, upon delivery and transfer of control to the customer, and includes an estimate of variable consideration, which results from discounts, rebates, and returns that are offered within contracts between the Company and its customers.

– *Prompt Pay Discounts:* As an incentive for prompt payment, the Company offers a cash discount to customers. The Company estimates accrued prompt pay discounts using the most likely amount method. The Company expects that all eligible customers will comply with the contractual terms to earn the discount. The Company records the discount as an allowance against accounts receivable, net and a reduction of revenue.

– *Government Rebates:* The Company participates in certain government rebate programs including Medicaid. The Company estimates accrued government rebates using the expected value method. The Company accrues estimated rebates based on estimated percentages of VYJUVEK prescribed to qualified patients, estimated rebate percentages and estimated levels of inventory in the distribution channel that will be prescribed to qualified patients and records the rebates as a reduction of revenue. Accrued government rebates are recorded as a reduction of revenue and are included in other accrued liabilities on the condensed consolidated balance sheets.

– *Commercial Rebates:* The Company participates in certain commercial rebate programs. Under these rebate programs, the Company pays a rebate to the commercial entity or third-party administrator of the program. Accrued commercial rebates are estimated using the expected value method. The Company accrues estimated rebates based on contract prices, estimated percentages of VYJUVEK that will be prescribed to qualified patients and estimated levels of inventory in the distribution channel. Accrued commercial rebates are recorded as a reduction of revenue and are included in other accrued liabilities on the condensed consolidated balance sheets.

– *Copay Assistance:* The Company provides copay assistance to qualified patients, helping them meet copay obligations to their insurance provider. The Company reimburses pharmacies for this discount through third-party vendors. The Company estimates copay assistance costs using the expected value method. The estimate is based on contract prices, estimated percentages of VYJUVEK that will be prescribed to qualified patients, average assistance paid based on reporting from third-party vendors and estimated levels of inventory in the distribution channel. Copay assistance costs are recorded as reductions to revenue and are accrued in other accrued liabilities on the condensed consolidated balance sheets.

– *Returns:* The Company offers SPs and SDs limited return rights relating to product damage or defect and based on these provisions, the Company believes that there will be minimal returns.

Variable consideration is estimated and reduces the transaction price to reflect the Company's best estimate of the amount of consideration to which the Company is entitled based on the terms of the contracts and are recorded in the same period the related product revenues is recognized. The amount of variable consideration that is included in the transaction price may be constrained and is included in the net sales price only to the extent that it is considered probable that a significant reversal in the amount of the cumulative revenue recognized will not occur in a future period. Actual amounts of consideration ultimately received may differ from the Company's 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.

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.

Accounts Receivable

Accounts receivable is recorded net of allowances for prompt payment discounts, returns, and credit losses. The Company estimates an allowance for credit losses by considering factors such as credit quality, the age of the accounts receivable balances, and current economic conditions that may affect a customer's ability to pay. As of September 30, 2023, the credit profiles for the Company's customer was deemed to be in good standing, and as such an allowance for credit losses was not recorded.

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, net and marketable securities, which primarily consist of U.S. government agency securities and treasuries, equity securities, corporate bonds and commercial paper, potentially subject the Company to concentrations of credit risk. As of March 31, 2024, the credit profile for the Company's counterparty was deemed to be in good standing and, as such, an allowance for credit losses was not recorded. The Company had one customer for the three and nine months ended September 30, 2023March 31, 2024 and no product revenuesrevenue for the three and nine months ended September 30, 2022March 31, 2023. The Company has no financial instruments with off-balance sheet risk of loss.

InventoriesSummary of Significant Accounting Policies

The Company capitalizes inventory costs associated with products when future economic benefit is expected See Note 2 to be realized. These costs consist of raw materials, manufacturing-related costs, personnel costs including stock-based compensation, facility costs, and other indirect overhead costs. Prior to receiving FDA approval for VYJUVEK our consolidated financial statements included in May our 2023 the Company expensed costs related to inventory for clinical and pre-commercial purposes directly to research and development expense. Following the FDA's approval of VYJUVEK, the Company began capitalizing inventory related to commercialized products held for sale, in-process of production for sale, and raw materials to be used in the manufacturing of inventory.

The Company values its inventories at the lower-of-cost and net realizable value, on a first-in, first-out ("FIFO") basis. The Company adjusts the net realizable value of any excess, obsolete or unsalable inventories in the period in which an impairment is identified. For the three and nine months ended September 30, 2023 and 2022, there 10-K. There were no inventory impairment adjustments. As of September 30, 2023, material changes to the Company recorded $5.3 million of inventory, consisting of raw materials, work-in-process, and finished goods within inventory on the Company's condensed consolidated balance sheets.

Property and Equipment, net

Property and equipment, net, is stated at cost, less accumulated depreciation. Maintenance and repairs that do not improve or extend the lives of the respective assets are expensed to operations as incurred, while costs of major additions and betterments are capitalized. Upon disposal, the related cost and accumulated depreciation is removed and any resulting gain or loss is included in the results of operations. Depreciation is recorded using the straight-line method over the estimated useful lives of the respective assets, which are as follows:

Buildings and building improvements	7 - 47 years
Computer equipment and software	3 - 7 years
Manufacturing equipment	3 - 20 years
Laboratory equipment	3 - 15 years
Furniture and fixtures	3 - 7 years
Leasehold improvements	lesser of useful life or remaining life of lease

The Company reviews the estimated useful lives of its property and equipment on a continuing basis. In evaluating the useful lives, the Company considers how long assets will remain functionally effective, whether the technology continues to be relevant and considers other competitive and economic factors. If the assessment indicates that the assets will be used for a shorter or longer period than previously anticipated, the useful life of the assets is adjusted, resulting in a change in estimate. Changes in estimates are accounted for on a prospective basis by depreciating the current carrying values of the assets over their revised remaining useful lives.

Construction in progress is not depreciated until the asset is placed in service.

Impairment of Long-Lived Assets

The Company evaluates long-lived assets for potential impairment when events or changes in circumstances indicate the carrying value of the assets may not be recoverable. We review the recoverability of the net book value of long-lived assets whenever events and circumstances indicate ("triggering events") that the net book value of an asset may not be recoverable. In cases where a triggering event occurs and undiscounted expected future cash flows are less than the net book value, we recognize an impairment loss equal to an amount by which the net book value exceeds the fair value of the asset. Long-lived assets to be disposed of are reported at the lower of carrying amount or fair value less cost to sell. The Company has not identified any triggering events or recognized any impairment losses for the three and nine months ended September 30, 2023 and 2022.

Leases

The Company accounts for its lease agreements in accordance with Financial Accounting Standards Board ("FASB") Accounting Standards Codification, or ASC, Topic 842, *Leases*. Right-of-use lease assets represent the right to use an underlying asset during the lease term and the lease liabilities represent the commitment to make lease payments arising from the lease. Right-of-use lease assets and obligations are recognized based on the present value of remaining lease payments over the lease term. As the Company's existing lease agreements do not provide an implicit rate and as the Company does not have any external borrowings, the Company has used an estimated incremental borrowing rate based on the information available at lease commencement in determining the present value of lease payments. Operating lease expense is recognized on a straight-line basis over the lease term. Variable lease expense is recognized in the period in which the obligation for the payment is incurred. In addition, the Company also has made an Company's significant accounting policy election to exclude leases with an initial term of twelve months or less from its condensed consolidated balance sheets and to account for lease and non-lease components of its operating leases as a single component.

Research and Development Expenses

Research and development costs are charged to expense as incurred in performing research and development activities. These costs include employee compensation costs, facilities and overhead, preclinical and clinical activities, clinical manufacturing costs, contract management services, regulatory and other related costs.

The Company estimates contract research and manufacturing expenses based on the services performed pursuant to contracts with research organizations and manufacturing organizations that manufacture materials used in the Company's ongoing preclinical and clinical studies. Non-refundable advanced payments for goods or services to be received in the future for use in research and development activities are capitalized within prepaid expenses and other current assets on the condensed consolidated balance sheets. The capitalized amounts are expensed as the related goods are delivered or the services are performed.

In accruing service fees, the Company estimates the time period over which services will be performed and the level of effort to be expended in each period. These estimates are based on communications with third-party service providers and the Company's estimates of accrued expenses using information available at each balance sheet date. If the actual timing of the performance of services or the level of effort varies from the estimate, the Company will adjust the accrual accordingly.

Stock-Based Compensation Expense

The Company applies the fair value recognition provisions of FASB, ASC Topic 718, *Compensation—Stock Compensation* ("ASC 718"), to account for stock-based compensation. Compensation costs related to equity awards granted are based on the estimated fair value of the awards on the date of grant.

ASC 718 requires all stock-based payments, including grants of stock options and restricted stock, to be recognized in the consolidated statements of operations based on their grant-date fair values. Compensation expense for stock options, restricted stock awards, and restricted stock units is recognized on a straight-line basis based on the grant-date fair value over the associated service period of the award, which is generally the vesting term. Compensation expense for performance-based restricted stock units is recognized for the awards that are probable of vesting over the service period of the award. On a quarterly basis, management estimates the probable number of performance-based restricted stock units that would vest until such time that the ultimate achievement of the performance criteria are known.

The Company estimates the fair value of its stock options using the Black-Scholes option pricing model, which requires the input of subjective assumptions, including: (i) the expected stock price volatility; (ii) the expected term of the award; (iii) the risk-free interest rate; and (iv) expected dividends.

The Company estimates stock price volatility by using its own historical data. The expected term of the Company's stock options is estimated using the "simplified" method, whereby the expected term equals the arithmetic mean of the vesting term and the original contractual term of the option. The risk-free interest rates are based on U.S. Treasury securities with a maturity date commensurate with the expected term of the associated award. The Company has never paid and does not expect to pay dividends in the foreseeable future. The Company accounts for forfeitures as they occur. Stock-based compensation expense recognized in the financial statements is based on awards for which service conditions are expected to be satisfied.

Comprehensive Income (Loss)

Comprehensive income (loss) is defined as the change in equity during a period from transactions from non-owner sources. Unrealized gains or losses on available-for-sale securities and foreign currency translation are components of other comprehensive gains or losses and are presented net of taxes. We record reclassifications from other comprehensive gains or losses to interest and other income, net on the condensed consolidated statements of operations related to realized gains on sales of available-for-sale securities.

The Company reviews its securities quarterly to determine whether an other-than-temporary impairment has occurred. The Company determined that there were no other-than-temporary impairments policies during the three and nine months ended September 30, 2023 and 2022. March 31, 2024.

Recent Accounting Pronouncements

From time to time, new accounting pronouncements are issued by the FASB that the Company adopts as of the specified effective date. There were no recently adopted accounting pronouncements that had a material impact on issued or adopted during the Company's condensed consolidated financial statements, and no recently issued accounting pronouncements three months ended March 31, 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

The 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 3Q the third quarter of 2023. For the three and nine months ended September 30, 2023 and 2022, the Company recognized net product revenues of $8.6 million and zero, respectively. Accounts receivable balances were $9.3 million and zero as of September 30, 2023 and December 31, 2022, respectively.

The following table summarizes changes in allowances and discounts for the three months ended September 30, 2023 (in thousands) March 31, 2024:

	Rebates	Prompt Pay	Other Accruals	Total
Balance as of June 30, 2023	$ —	$ —	$ —	$ —

Provision	920	195	78	1,192
Payments/Credits	—	(5)	—	(5)
Balance, as of September 30, 2023	$ 920	$ 190	$ 78	$ 1,187

(in thousands)	Rebates	Prompt Pay	Other Accruals	Total
Balance as of December 31, 2023	$ 5,977	$ 858	$ 279	$ 7,114
Provisions	5,746	1,565	211	7,522
Payments/Credits	(434)	(953)	(75)	(1,462)
Balance, as of March 31, 2024	$ 11,289	$ 1,470	$ 415	$ 13,174

Rebates and other accruals are included in accrued rebates and accrued expenses and other current liabilities, respectively, 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 on the condensed consolidated statements of operations and comprehensive 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 share stock equivalents outstanding for the period. Common share stock equivalents consist of common stock issuable upon (1) exercise of stock options and (2) vesting of restricted stock awards, and restricted stock units. units and performance-based restricted stock units (collectively, “restricted stock”).

There were 378,299 and 1,277,313 common share equivalents, in the form of stock options, that have been excluded of from the calculation of diluted net income (loss) per common share for For the three and nine months ended September 30, 2023, March 31, 2024 and 2023, respectively, as their effect would be anti-dilutive. There there were 3,565,110 (1) 154 thousand and 3.8 million common share stock equivalents outstanding in the form of stock options and (2) 78 thousand and 291 thousand unvested restricted stock, awards as of September 30, 2022 that were 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 March 31,			
		Three Months Ended March 31,			
		Three Months Ended March 31,			
(in thousands, except per share data)					
(in thousands, except per share data)					
(in thousands, except per share data)					
		Three Months Ended September 30,		**Nine Months Ended September 30,**	
(In thousands, except share and per share data)		**2023**	**2022**	**2023**	**2022**
Numerator:					
Numerator:					
Numerator:	Numerator:				
Net income (loss)	Net income (loss)	$ 80,747	$ (29,850)	$ 2,240	$ (107,923)
Net income (loss)					
Net income (loss)					
Denominator:					
Denominator:					
Denominator:	Denominator:				

Weighted-average basic common shares	Weighted-average basic common shares	28,042,130	25,619,125	26,812,278	25,428,097
Weighted-average basic common shares					
Weighted-average basic common shares					
Dilutive effect of stock options and unvested restricted stock					
Dilutive effect of stock options and unvested restricted stock					
Dilutive effect of stock options and unvested restricted stock	Dilutive effect of stock options and unvested restricted stock	850,096	—	572,261	—
Weighted-average diluted common shares	Weighted-average diluted common shares	28,892,226	25,619,125	27,384,539	25,428,097
Weighted-average diluted common shares					
Weighted-average diluted common shares					
Net income (loss) per common share — Basic		$ 2.88	$ (1.17)	$ 0.08	$ (4.24)
Net income (loss) per common share — Diluted		$ 2.79	$ (1.17)	$ 0.08	$ (4.24)
Net income (loss) per common share—Basic					
Net income (loss) per common share—Basic					
Net income (loss) per common share—Basic					
Net income (loss) per common share—Diluted					
Net income (loss) per common share—Diluted					
Net income (loss) per common share—Diluted					

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 September 30, 2023 March 31, 2024 and December 31, 2022, respectively (in thousands) December 31, 2023:

		September 30, 2023						
		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)**
(in thousands)								
(in thousands)								
(in thousands)								
Level 1:								
Level 1:								
Level 1:	**Level 1:**							

Cash and cash equivalents	Cash and cash equivalents	$ 373,241	$ —	$ —	$ 373,241	$ 373,241	$ —	$ —
Cash and cash equivalents								
Cash and cash equivalents								
Subtotal	Subtotal	373,241	—	—	373,241	373,241	—	—
Subtotal								
Subtotal								
Level 2:								
Level 2:								
Level 2:	**Level 2:**							
Commercial paper	Commercial paper	43,496	1	(9)	43,488	—	43,488	—
Commercial paper								
Commercial paper								
Corporate bonds								
Corporate bonds								
Corporate bonds	Corporate bonds	70,465	4	(201)	70,268	—	56,463	13,805
U.S. government agency securities	U.S. government agency securities	111,635	225	(240)	111,620	—	88,877	22,743
U.S. government agency securities								
U.S. government agency securities								
Subtotal								
Subtotal								
Subtotal	Subtotal	225,596	230	(450)	225,376	—	188,828	36,548
Total	Total	$ 598,837	$ 230	$ (450)	$ 598,617	$ 373,241	$ 188,828	$ 36,548
Total								
Total								

		December 31, 2022						
		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)**
(in thousands)								
(in thousands)								
(in thousands)								
Level 1:	**Level 1:**							
Level 1:								
Level 1:								
Cash and cash equivalents								

Cash and cash equivalents								
Cash and cash equivalents	Cash and cash equivalents	$ 161,900	$ —	$ —	$ 161,900	$ 161,900	$ —	$ —
Subtotal	Subtotal	161,900	—	—	161,900	161,900	—	—
Subtotal								
Subtotal								
Level 2:								
Level 2:								
Level 2:	**Level 2:**							
Commercial paper	Commercial paper	63,624	5	(23)	63,606	—	63,606	—
Commercial paper								
Commercial paper								
Corporate bonds	Corporate bonds	82,241	13	(419)	81,835	—	77,214	4,621
Corporate bonds								
Corporate bonds								
U.S. government agency securities								
U.S. government agency securities								
U.S. government agency securities	U.S. government agency securities	76,683	161	(393)	76,451	—	76,451	—
Subtotal	Subtotal	222,548	179	(835)	221,892	—	217,271	4,621
Subtotal								
Subtotal								
Total	Total	$ 384,448	$ 179	$ (835)	$ 383,792	$ 161,900	$ 217,271	$ 4,621
Total								
Total								

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

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

See Note 2 to these unaudited condensed consolidated financial statements for additional discussion regarding the Company's fair value measurements.

6. Balance Sheet Components

Inventory

Inventory consisted of the following:

(in thousands)	March 31, 2024	December 31, 2023
Raw materials	$ 4,489	$ 3,154
Work-in-process	6,387	3,204
Finished goods	161	627

Inventory	$ 11,037	$ 6,985

Property and Equipment, Net

Property and equipment, net consist consisted of the following (in thousands): following:

		September 30, 2023	December 31, 2022
(in thousands)			
(in thousands)			
(in thousands)			
Building and building improvements	Building and building improvements	72,193	—
Construction in progress		$ 56,074	$ 131,331
Building and building improvements			
Building and building improvements			
Leasehold improvements			
Leasehold improvements			
Leasehold improvements	Leasehold improvements	24,835	24,217
Manufacturing equipment	Manufacturing equipment	16,827	9,783
Manufacturing equipment			
Manufacturing equipment			
Construction in progress			
Construction in progress			
Construction in progress			
Laboratory equipment	Laboratory equipment	2,339	2,089
Laboratory equipment			
Laboratory equipment			
Computer equipment and software			
Computer equipment and software			
Computer equipment and software			
Furniture and fixtures	Furniture and fixtures	1,518	957
Computer equipment and software		982	100
Furniture and fixtures			
Furniture and fixtures			
Total property and equipment			
Total property and equipment			
Total property and equipment	Total property and equipment	174,768	168,477
Accumulated depreciation	Accumulated depreciation	(10,739)	(6,793)
Accumulated depreciation			
Accumulated depreciation			
Property and equipment, net	Property and equipment, net	$ 164,029	$ 161,684
Property and equipment, net			
Property and equipment, net			

Depreciation expense was $1.2 million $1.4 million and $3.5 million $1.1 million for the three and nine months ended September 30, 2023 March 31, 2024 and $669 2023, respectively. Depreciation expense capitalized into inventory was $867 thousand and $1.6 million zero for the three and nine months ended

September 30, 2022, March 31, 2024 and 2023, respectively.

On March 27, 2023, 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, certain and as qualification of assets occurred through 2023 and the first quarter 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 during the first half of 2023. The Company placed additional portions of ASTRA into service during the three months ended September 30, 2023 as it was determined that additional assets were ready for their intended use. As certain building improvements pieces of equipment are not yet complete and certain qualification activities are still underway, qualified, the Company will continue to hold the remaining assets within construction in progress until validation qualification has been completed and the assets are ready for their intended use. Validation Estimated remaining payments related to ASTRA were $8.0 million as of March 31, 2024 and are recorded in accounts payable and accrued expenses and other current liabilities on the facility is expected to be completed in 2023.

condensed consolidated balance sheets.

Accrued Expenses and Other Current Liabilities

Accrued expenses and other current liabilities consisted of the following (in thousands) as of March 31, 2024 and December 31, 2023:

		September 30, 2023	December 31, 2022
Accrued construction in progress		$ 7,548	$ 11,452
(in thousands)			
(in thousands)			
(in thousands)			
Accrued litigation settlement			
Accrued litigation settlement			
Accrued litigation settlement			
Accrued construction-in-progress			
Accrued construction-in-progress			
Accrued construction-in-progress			
Accrued payroll and benefits			
Accrued payroll and benefits			
Accrued payroll and benefits	Accrued payroll and benefits	7,033	6,781
Accrued professional fees	Accrued professional fees	2,786	3,397
Accrued professional fees			
Accrued professional fees			
Other current liabilities	Other current liabilities	2,315	267
Other current liabilities			
Other current liabilities			
Accrued taxes			
Accrued taxes			
Accrued taxes			
Accrued preclinical and clinical expenses			
Accrued preclinical and clinical expenses			
Accrued preclinical and clinical expenses	Accrued preclinical and clinical expenses	1,908	1,365
Accrued taxes		152	43
Total	Total	$ 21,742	$ 23,305
Total			
Total			

7. Commitments and Contingencies

Agreements with Contract ResearchManufacturing Organizations and Contract ManufacturingResearch Organizations

The Company enters into various agreements in the normal course of business with Contract Research Organizations ("CROs"(“CROs”), Contract Manufacturing Organizations ("CMOs"(“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 cell and virus banks and for 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, storage, packaging, labeling, and/or testing of our preclinicalpre-commercial and clinical-stage or pre-commercial products. 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 commitmentcommitments as of September 30, 2023March 31, 2024 under these agreements is approximately $2.7 million$1.1 million. The Company has incurred research and development expenses under these agreements of $1.9 million and $5.0 million$1.5 million for the three and nine months ended September 30, 2023, respectively,March 31, 2024 and $2.1 million and $5.1 million$2.0 million for the three and nine months ended September 30, 2022, respectively.

ASTRA Contractual Obligations

The Company has contracted with various third parties to complete and qualify our second CGMP facility, ASTRA. These contracts typically call for the payment of fees for services or materials upon the achievement of certain milestones. The estimated remaining commitment as of September 30, 2023 is $11.1 million and primarily relates to the remaining building improvements and certain qualification activities of the facility. The Company has included costs incurred to-date associated with the ongoing build-out of ASTRA within construction in progress, except for the assets of the facility that have been placed in service.

As of September 30, 2023, Substantial Completion, as defined in the Standard Form of Contract for Construction and the corresponding General Conditions of the Contract for Construction (the “Agreement”) with Whiting-Turner Contracting Company (“Whiting-Turner”), the construction manager for ASTRA, had not been achieved. Whiting-Turner’s work under the Agreement represents a portion of the work necessary to complete construction of the ASTRA facility and, therefore the date of Substantial Completion of Whiting-Turner’s work under the Agreement may not equate to the date of in-service for portions of ASTRA or the date of full facility completion of ASTRA. March 31, 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"(“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 trade secret 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 total cumulative sales, $200.0 million in total cumulative sales and $300.0 million in total cumulative sales. As defined in the settlement agreement, cumulative sales shall include all revenue from sales of the CompanyCompany’s products by the Company and its affiliates and licensees, as reported by the

Company in its annual Form 10-K filings. 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 thelitigation settlement paymentsexpense of zero and $12.5 million for each of the three and nine months ended September 30, 2023, respectively,March 31, 2024 and zero and $25.0 million for the three and nine months ended September 30, 2022, respectively, under litigation settlement expense2023 on the condensed consolidated statements of operations.operations and comprehensive loss in accordance with the settlement agreement. During the three months ended March 31, 2024, in accordance with ASC 450, *Contingencies* (“ASC 450”), the Company determined that reaching $100.0 million in total 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 the $100.0 million in total cumulative sales. The Company recorded litigation

settlement expense of $12.5 million for the three months ended March 31, 2023, as it determined that FDA approval of B-VEC was probable. As of September 30, 2023March 31, 2024, the Company has not recorded an accrual for the remaining two contingent milestone payments.

The Company did not receive insurance proceeds during the three and nine months ended September 30, 2023 and received zero and $768 thousand during the three and nine months ended September 30, 2022, respectively. The reimbursements have been recorded as a reduction to our legal fees included in selling, general, and administrative expenses on the condensed consolidated statements of operations and within operating activities on the condensed consolidated statements of cash flows. payments totaling $25.0 million.

8. Leases

As of September 30, 2023March 31, 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
2023 (remaining three months)	$	413

2024		1,539
2025		1,277
2026		1,277
2027		1,300
Thereafter		10,763
Future minimum operating lease payments	$	16,569
Less: Interest		(8,249)
Present value of lease liability	$	8,320

Supplemental condensed consolidated balance sheet information related to leases is as follows:

	September 30, 2023		December 31, 2022	
Operating leases:				
Right-of-use assets	$	7,360	$	8,042
Current portion of lease liability		1,501		1,561
Lease liability		6,819		7,372
Total lease liability	$	8,320	$	8,933
Weighted average remaining lease term, in years		12.3		12.5
Weighted average discount rate		9.4 %		9.4 %

(in thousands)	Operating Leases	
2024 (remaining nine months)	$	1,157
2025		1,277
2026		1,277
2027		1,300
2028		1,325
Thereafter		9,438
Future minimum operating lease payments		15,774
Less: Interest		(7,879)
Present value of lease liability	$	7,895

As of March 31, 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 March 31, 2024 and December 31, 2023.

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2023	2022	2023	2022

(in thousands)
(in thousands)
(in thousands)
Lease cost:
Lease cost:

Lease cost:	Lease cost:				
Operating lease expense	Operating lease expense	$ 379	$ 399	$ 1,282	$ 1,200
Operating lease expense					
Operating lease expense					
Variable lease expense					
Variable lease expense					
Variable lease expense	Variable lease expense	62	48	150	168
Total lease expense	Total lease expense	$ 441	$ 447	$ 1,432	$ 1,368
Total lease expense					
Total lease expense					

9. Capitalization

ATM Program

On December 31, 2020, the Company entered into a sales agreement with Cowen and Company, LLC ("Cowen" (“Cowen”) with respect to an at-the-market equity offering program (" (“2020 ATM Program" Program”), under which the Company issued and sold from time to time through Cowen, acting as agent and/or principal, shares of its common stock, par value $0.0001 $0.00001 per share (“Common Stock”), having an aggregate offering price up to $150.0 million (" (“Placement Shares" Shares”). The issuance and sale of the Placement Shares were made pursuant to the Company's Company’s effective "shelf" “shelf” registration statement on Form S-3 that was filed with the Securities and Exchange Commission (the “SEC”) SEC on May 4, 2020 (the “2020 (“2020 Shelf Registration Statement”). During the nine three months ended September 30, 2022 March 31, 2023, no shares of Common Stock were issued pursuant to the Company issued and sold 434,782 Placement Shares at a weighted average price of $69.00 per share for net proceeds of $29.1 million after deducting selling commissions of approximately $900 thousand. 2020 ATM Program.

The Company’s 2020 Shelf Registration Statement expired on May 4, 2023, and the Company put in place a new at-the-market equity offering program under substantially the same terms as the 2020 ATM Program (the “New (“New ATM Program”). Accordingly, on May 8, 2023, the Company entered into a new sales agreement with Cowen to issue and sell shares of the Company’s common stock Common Stock having an aggregate offering price of up to $150.0 million (the “New (“New Placement Shares”) from time to time, under which Cowen will act as the Company’s agent and/or principal. The New 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 New Placement Shares that was filed with the SEC on May 8, 2023. During the quarter three months ended September 30, 2023 March 31, 2024, no shares of common stock Common Stock were issued pursuant to the New ATM Program, resulting in $150.0 million available for issuance under the New 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 (the “Plan” (“Plan”), which governs the issuance of stock options and restricted stock 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 March 31, 2024.

Stock Options

Options granted to employees and non-employees vest ratably over a four-year period and stock options granted to directors of the Company vest ratably over one-year to three-year periods. Stock options have a life of ten years.

The Company granted 30,500 and 419,780 stock options to employees, non-employees, and directors of the Company during the three and nine months ended September 30, 2023 and 189,000 and 1,958,000 to employees, non-employees, and directors of the Company during the three and nine months ended September 30, 2022, respectively.

The following table summarizes the Company's stock option activity: activity for the three months ended March 31, 2024:

	Stock Options Outstanding		Weighted-average Exercise Price	Weighted-average Remaining Contractual Life (Years)		Aggregate Intrinsic Value (In thousands) (1)
Outstanding at December 31, 2022	3,582,181	$	61.50	8.7	$	64,880
Granted	419,780	$	91.38			
Exercised	(710,734)	$	57.98			
Cancelled or forfeited	(623,967)	$	64.19			
Expired	—	$	—			
Outstanding at September 30, 2023	2,667,260	$	66.04	8.2	$	133,648
Exercisable at September 30, 2023	707,277	$	55.51	7.1	$	42,793

	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	126,742	$	155.82			
Exercised	(259,581)	$	61.52			
Cancelled or forfeited	(245,659)	$	67.89			
Outstanding as of March 31, 2024	2,228,094	$	71.88	7.7	$	236,279
Exercisable as of March 31, 2024	854,224	$	61.08	6.8	$	99,815

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

The following table summarizes the Company's stock option activity for the three months ended March 31, 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	287,600	$	81.83			
Exercised	(42,021)	$	53.54			
Cancelled or forfeited	(42,625)	$	65.59			
Outstanding as of March 31, 2023	3,785,135	$	62.75	8.5	$	66,066
Exercisable as of March 31, 2023	961,428	$	55.08	7.6	$	24,033

(1) Aggregate intrinsic value represents the difference between the closing stock price of our common stock on December 31, 2022 and March 31, 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 $24.5 million and $1.1 million during the three and nine months ended September 30, 2023 was $13.5 million March 31, 2024 and $41.4 million, respectively, and during the three and nine months ended September 30, 2022 was $1.3 million and $2.1 million, 2023, respectively.

The weighted-average grant-date fair value per share of options granted to employees, non-employees, and directors was $106.92 and $56.86 during the three and nine months ended September 30, 2023 was $85.93 March 31, 2024 and $62.87, respectively, and during the three and nine months ended September 30, 2022 was $49.59 and $43.66, 2023, respectively.

There was $77.5 $63.9 million of unrecognized stock-based compensation expense related to employees' employees', non-employees' non-employees', and directors' option awards options that is expected to be recognized over a weighted-average period of 2.6 years as of September 30, 2023 March 31, 2024.

The Company has recorded stock-based compensation expense related to the issuance of stock option awards in the condensed consolidated statements of operations for the three and nine months ended September 30, 2023 and 2022, respectively, as follows (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2023	2022	2023	2022
Research and development	$ 2,033	$ 2,184	$ 6,858	$ 5,547
Selling, general, and administrative	4,796	6,433	18,881	16,791
Total stock-based compensation	$ 6,829	$ 8,617	$ 25,739	$ 22,338

The fair value of options was estimated at the date of grant using the Black-Scholes valuation model with the following weighted-average assumptions for the three and nine months ended September 30, 2023 and 2022:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2023	2022	2023	2022
Expected stock price volatility	74 %	78 %	73 %	78 %
Expected term of the award (years)	6.2	6.2	6.0	6.2
Risk-free interest rate	4.26 %	3.20 %	3.94 %	2.27 %
Weighted average exercise price	$ 125.06	$ 70.91	$ 91.38	$ 63.03
Forfeiture rate	— %	— %	— %	— %
Dividend yield	— %	— %	— %	— %

Restricted Stock Awards

Restricted stock awards ("RSAs") granted to employees vest ratably over a four-year period. The Company granted zero RSAs to employees of following table summarizes the Company during each of the three and nine months ended September 30, 2023 and September 30, 2022, respectively.Company's RSA activity:

	Number of Shares	Weighted Average Grant Date Fair Value
Non-vested RSAs as of December 31, 2022	66,600	$ 78.89

	Three Months Ended March 31,
	Three Months Ended March 31,
	Three Months Ended March 31,
	2024
	2024

		2024		
		Number of Shares		
		Number of Shares		
		Number of Shares		
Non-vested RSAs, beginning of period				
Non-vested RSAs, beginning of period				
Non-vested RSAs, beginning of period				
Granted				
Granted				
Granted	Granted	—	$	—
Vested	Vested	(12,649)	$	78.89
Vested				
Vested				
Surrendered for taxes	Surrendered for taxes	(9,551)	$	78.89
Non-vested RSAs as of September 30, 2023		$44,400	$	78.89
Surrendered for taxes				
Surrendered for taxes				
Non-vested RSAs, end of period				
Non-vested RSAs, end of period				
Non-vested RSAs, end of period				

There was $2.5 million $1.6 million of unrecognized stock-based compensation expense related to employees' RSAs that is expected to be recognized over a weighted-average period of 1.4 years 11 months as of September 30, 2023 March 31, 2024.

The Company recorded stock-based compensation expense related to RSAs in the condensed consolidated statement of operations for the three and nine months ended September 30, 2023 and 2022, as follows (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2023	2022	2023	2022
Selling, general, and administrative	$ 437	$ 441	$ 1,305	$ 1,340
Total stock-based compensation	$ 437	$ 441	$ 1,305	$ 1,340

Restricted Stock Units

Restricted stock units ("RSUs") granted to employees vest ratably over a four-year period. The Company granted zero and 186,900 RSUs to employees of following table summarizes the Company during the three and nine months ended September 30, 2023, respectively, and zero RSUs during the three and nine months ended September 30, 2022, respectively. Company's RSU activity:

	Number of Shares	Weighted Average Grant Date Fair Value
Non-vested RSUs as of December 31, 2022	—	
Granted	186,900	$ 81.91
Vested	—	
Surrendered or forfeited	(24,700)	$ 81.91
Non-vested RSUs as of September 30, 2023	162,200	$ 81.91

	Three Months Ended March 31,			
	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	223,890	$ 159.59	186,900	$ 81.91
Vested	(40,075)	$ 81.91	—	
Forfeited	(14,383)	$ 86.27	—	
Non-vested RSUs, end of period	330,332	$ 134.37	186,900	$ 81.91

There was $11.3 million $43.4 million of unrecognized stock-based compensation expense related to employees' RSU awards that is expected to be recognized over a weighted-average period of 3.4 3.7 years as of September 30, 2023 March 31, 2024.

The Company recorded stock-based compensation expense related to RSUs in the condensed consolidated statement of operations for the three and nine months ended September 30, 2023 and 2022, as follows (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2023	2022	2023	2022
Research and Development	$ 303	$ —	$ 837	$ —
Selling, general, and administrative	370	—	998	—
Total stock-based compensation	$ 673	$ —	$ 1,835	$ —

Performance-Based Restricted Stock Units

Performance-based restricted stock units ("PSUs") The following table summarizes the Company's PSU activity:

	Three Months Ended March 31,			
	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	—	
Forfeited	—		—	
Non-vested PSUs, end of period	137,500	$ 145.37	60,000	$ 81.91

For the three months ended March 31, 2024, the Company granted to employees 112,500 of PSUs, which 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. Each PSU represents the right to receive one share As of the Company's common stock upon vesting. The Company recognizes stock-based compensation expense for the fair value of the PSU awards relating to the portion of the awards that are probable of vesting over the service period. On a quarterly basis, management estimates the probable number of PSU's that would vest until such time that the ultimate achievement of the performance criteria are known. As of September 30, 2023 March 31, 2024, the Company estimates estimated that 100% of the newly granted PSUs granted will be eligible to vest.

The Company granted zero and 60,000 PSUs to employees of the Company during the three and nine months ended September 30, 2023 and zero PSUs during the three and nine months ended September 30, 2022.

	Number of Shares	Weighted Average Grant Date Fair Value
Non-vested PSUs as of December 31, 2022	—	
Granted	60,000	$ 81.91
Vested	—	
Surrendered or forfeited	(10,000)	$ 81.91
Non-vested PSUs as of September 30, 2023	50,000	$ 81.91

There was $2.9 million $19.0 million of unrecognized stock-based compensation expense related to employees' PSU awards that is expected to be recognized over a weighted-average period of 1.4 1.8 years as of September 30, 2023 March 31, 2024.

Stock-Based Compensation Expense, Net

The Company recorded stock-based compensation expense related to its stock options, RSAs, RSUs, and PSUs in the condensed consolidated statement statements of operations and comprehensive loss for the three and nine months ended September 30, 2023 March 31, 2024 and 2022 2023 as follows (in thousands): follows:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2023	2022	2023	2022
Selling, general, and administrative	$ 373	$ —	$ 1,201	$ —
Total stock-based compensation	$ 373	$ —	$ 1,201	$ —

Shares remaining available for grant under the Plan were 1,489,488, with a sublimit for incentive stock options of 30,783, at September 30, 2023.

	Three Months Ended March 31,	
(in thousands)	2024	2023
Research and development	$ 1,868	$ 2,496
Selling, general, and administrative	7,431	7,941
Total stock-based compensation	$ 9,299	$ 10,437

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. For the three and nine months ended September 30, 2023, March 31, 2024 and 2023, the Company capitalized $410 stock-based compensation of $724 thousand and $522 thousand, zero, respectively, in into inventory.

Historically, the Company also capitalized the portion of stock-based compensation related to work performed on the construction of our manufacturing facilities. There was For the three months ended March 31, 2024 and 2023, the Company capitalized stock-based compensation of zero and $162 thousand, of stock-based compensation that was capitalized in respectively, into property and equipment, during the three and nine months ended September 30, 2023 and $137 thousand and $423 thousand of stock-based compensation that was capitalized in property and equipment during the three and nine months ended September 30, 2022, respectively. net.

11. Gain from Sale Income Taxes

The tax provision for interim periods is calculated using an estimate of Priority Review Voucher

In August 2023, the annual effective tax rate, adjusted for discrete items. If there are any changes to the estimated annual tax rate, the Company entered into an agreement to sell the rare pediatric disease voucher ("PRV"), which was awarded will make a cumulative adjustment to the income tax provision in the period the change becomes known. The Company in connection with did not record an income tax provision for the FDA's approval three months ended March 31, 2024 or 2023 as it generated sufficient tax losses, after consideration of VYJUVEK. The transaction closed in August 2023 and was not subject to any commissions or closing costs. The proceeds of $100.0 million from the sale discrete items, during each of the PRV were recorded as periods. The Company expects to maintain a gain from sale of priority review voucher on full valuation allowance against its net deferred tax assets for the Company's condensed consolidated statement of operations as it did not have a carrying value at the time of the sale. 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, 2022 December 31, 2023 ("2023 10-K"), as filed with the SEC on February 27, 2023 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; 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 U.S. Food and Drug Administration ("FDA") FDA and other regulatory approval of our product candidates;
- our ability to achieve certain accelerated or orphan drug designations from the FDA; FDA or other regulators;
- changes in our estimates regarding the potential market opportunity for our FDA-approved product, VYJUVEK (beremagene geperpavec-svdt) (also known as "B-VEC" where not approved), 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 recent bank failures; 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 (i) Item 1A of Part II of this Quarterly Report on Form 10-Q (ii) "Risk Factors" in our Annual Report on Form 10-K for the fiscal year ended December 31, 2022, and (iii) 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. Also, forward-looking statements represent our management's beliefs and assumptions only as of the date of this Quarterly Report. 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, "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. Our approach leverages Using our patented gene therapy technology platform that is based on an engineered Herpes Simplex Virus-1 herpes simplex virus-1 ("HSV-1") vector to, 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. We formulate our 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 CGMP Current Good Manufacturing Practice ("CGMP") manufacturing facilities.

Our US 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 United States Food and Drug Administration (the "FDA") FDA approved VYJUVEK, the first ever re-dosable redosable gene therapy, for treating patients, six months of age or older, suffering from Dystrophic Epidermolysis Bullosa ("DEB"), 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*. 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 healthcare professional 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 $45.3 million for the three months ended March 31, 2024, and $95.9 million in cumulative net product revenue since August 2023.

We have made steady progress securing access and reimbursement for VYJUVEK since launch. In January 2024, we announced that the U.S. Centers for Medicare & Medicaid Services had assigned a permanent and product-specific J-code (J3401) for VYJUVEK, effective on January 1, 2024, and as of April 2024, positive access has been achieved for 96% of lives covered under commercial and Medicaid plans.

As of September 30, 2023, the Company received 284 Patient Start Forms, of which 20% were generated April 2024, we have secured over 330 reimbursement approvals for patients with dominant DEB. In addition, the Company has received positive coverage determinations from all major commercial national health plans. Optional Medicaid fee-for service states initiated coverage in July, and the Company expects to receive positive coverage from most mandatory states VYJUVEK in the fourth quarter of 2023 and the balance in the first quarter of 2024. Consequently, we plan on reporting the number of Patients on Therapy starting in U.S.

Product revenue, net for the first quarter of 2024 was negatively impacted by the switchover of patients to the permanent J-code, while reimbursement approvals were delayed by a subsequent cybersecurity incident affecting our specialty pharmacy provider. In both instances, to ensure continuity of treatment, we bridged patients on VYJUVEK with free vials. We estimate that approximately 400 free vials were dispensed in the quarter which would otherwise have been commercial vials. No additional impact is expected moving forward relating to these events.

We seek to make the patient experience of starting and continuing on VYJUVEK treatment as seamless as possible. Since launch, the infrastructure has been in place for patients to be treated in their home by an HCP, reducing the need for regular visits to a clinic or hospital. Krystal ConnectTM, 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 first quarter of 2024, patient compliance with once weekly treatment while on VYJUVEK remains high at 91%.

In February 2024, our Senior Vice President and Head of North America Sales and Marketing joined the Company.

Preparations and infrastructure buildout are underway in Europe and Japan to support our planned direct commercial launch in these regions. In March 2024, we believe that Patients on Therapy completed the formation of Krystal Biotech Japan, G.K. ("Krystal Japan"), for the purpose of establishing initial operations in Japan, and we concluded our search for the President and General Manager of Krystal Japan who will be join the Company in June 2024. In March 2024, our General Manager of Germany and Austria also joined the Company in preparation for a better predictor of net revenue from VYJUVEK sales. European launch.

In October 2023, we filed for submitted a Marketing Authorization for B-VEC with Application ("MAA") to the European Medicines Agency (the "EMA") ("EMA") and anticipate approval in the EU in the second half of 2024. Previously, in July 2023, the Company received a positive opinion from the EMA Pediatric Committee on the Pediatric Investigation Plan for B-VEC for the treatment of DEB with no additional studies required. in patients from birth. 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. We expect to receive GMP certification from the EMA in the second half of 2024. 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 our the open label extension ("OLE") study of B-VEC. Following that acceptance, we initiated an open label extension study of B-VEC in 5 patients in Japan. Details The efficacy portion of the Japan OLE study can be found at jrct.niph.go.jp under JRCT ID jRCT2053230075. We intend to file a Japanese was completed in April 2024, and we anticipate filing our Japan New Drug Application for with Japan's PMDA in the second half of 2024, enabling a potential authorization by PMDA in 2025. Previously, in December 2023, B-VEC was granted orphan drug designation status for the treatment of DEB in by the first half Japan Ministry of 2024 following completion Health, Labour and Welfare, a designation which confers specific benefits for orphan drug development including priority review of the open label extension study.

applications, extended registration validity, and reduced development costs.

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"*CFTR*") transgene for the treatment of Cystic Fibrosis 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 (CORAL-1) clinical trial of KB407 for the treatment of CF. Cohort 1 of the CORAL-1 study has been enrolled and completed. The Company is working to initiate Cohort 2 of the CORAL-1 study following safety review by the Data Monitoring Committee. The CORAL-1 study is a multi-center, dose-escalation trial study of evaluating KB407, delivered via a nebulizer, in patients with CF, regardless of their underlying genotype. The Company anticipates announcing data from In March 2024, we completed dosing of the second cohort of CORAL-1, study and we intend to initiate the third and final cohort in the second quarter of 2024. Details of the Phase 1 study can be found at www.clinicaltrials.gov under NCT identifier NCT05504837.

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 September 2023, February 2024, we dosed the Company announced that the FDA had cleared the Company's Investigational New Drug ("IND") application for KB408. The Company plans to initiate 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 genotype. 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 second half of 2024. Recruitment is ongoing and details of the Phase 1 study can be found at www.clinicaltrials.gov under NCT identifier: NCT06049082.

Ophthalmology

In April 2023, we announced clinical trial (SERPINA-1) 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 dose Ophthalmology ("ARVO") 2023 Annual Meeting and subsequently published in the first patient *New England Journal of Medicine* in February 2024. Regular application of B-VEC to the eye was well tolerated and associated with KB408 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. Previously, 2024 on a potential clinical development path for ophthalmic B-VEC. In February 2024, we aligned with the FDA granted Orphan Drug Designation on our proposed single arm, open label study in approximately 10 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 this study in the second half of 2024.

In January 2024, the United States Patent and Trademark Office, or USPTO, issued U.S. Patent No. 11,865,148, covering methods of delivering human transgenes to the eye using replication-incompetent HSV-1. This patent covers the administration of B-VEC to the eye, as well as novel applications of our HSV-1 based platform to deliver genetic material to the eye via multiple routes of administration for KB408 the potential treatment of genetic eye diseases.

We will be presenting preclinical data at the ARVO 2024 Annual Meeting later this month highlighting the potential of Krystal's HSV-1-based gene delivery platform for back of the eye gene delivery, and we are actively evaluating multiple, preclinical-stage genetic medicine candidates for the treatment of AATD. front and back of the eye diseases.

Oncology

In July 2023, we announced expansion of our R&D pipeline to oncology, leveraging our prior experience in skin and lung tissue for local delivery of immune boosting cytokines to the tumor microenvironment. Our lead oncology product candidate, KB707 is a modified HSV-1 vector redosable, immunotherapy designed to deliver genes encoding both human IL-12 interleukin-2 ("IL-2") and IL-2 interleukin-12 ("IL-12") to the tumor microenvironment and promote systemic immune-mediated tumor clearance. Two formulations of KB707 targets solid tumors that are accessible via intratumoral in development, a solution formulation for transcutaneous injection or inhalation. and an inhaled (nebulized) formulation for lung delivery.

In July 2023, we announced that the FDA accepted our IND application to evaluate had granted intratumoral injection of KB707 in a clinical trial to treat patients with locally advanced or metastatic solid tumor malignancies. The FDA also granted KB707 fast track designation to delay disease progression in Fast Track Designation for the treatment of patients with anti-PD-1 relapsed/refractory locally advanced or metastatic melanoma.

melanoma and accepted our Investigational New Drug (IND) application to evaluate intratumoral KB707 in a clinical study. In October 2023, we dosed the first patient in the KB707 Phase 1 (OPAL-1) study to evaluate intratumoral KB707 in patients with locally advanced or metastatic solid tumor malignancies. The OPAL-1, study is an open-label, multi-center, monotherapy, dose escalation and expansion Phase 1 study, enrolling 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. The primary objective We have cleared the first two dose escalation cohorts of the study is OPAL-1 and have completed enrollment in Cohort 3. Data collected to evaluate safety and tolerability of KB707. Efficacy will also date have shown KB707 to be assessed by multiple measures including overall response rate, progression free survival, and overall survival, and the immune effects of KB707 monotherapy will be assessed in tumor tissue, lymph nodes, and blood. generally well tolerated with no patients experiencing dose-limiting toxicities or drug-related grade ≥3 adverse events. Details of the Phase 1 study can be found at www.clinicaltrials.gov under NCT identifier NCT05970497.

The Company presented preclinical data in multiple oncology models at Based on the Society for Immunotherapy current pace of Cancer's annual meeting on November 3 and 4, 2023. Combinatorial IL-2 and IL-12 expressed from the Company's platform technology was shown in one presentation, to provide a synergistic effect in a melanoma model, suppressing treated and non-treated tumor outgrowth, enhancing survival, and eliciting a durable memory response sufficient for recurrent tumor control. Similarly, the Company presented that non-invasive inhalation of vector-encoded IL-2 and IL-12 was found enrollment, we expect to be both safe and effective in treating lung tumors in a metastasis model, resulting in long-term survival after single or repeated cancer cell challenge, suggestive able to report interim data before the end of prolonged adaptive immunity. the year.

The Company is on track to file In January 2024, the FDA accepted an amendment to the existing KB707 our IND in the fourth quarter of 2023 to allow the Company application to evaluate inhaled KB707 in a clinical trial to treat tumors study, and, in a patient's lungs. The Company expects to dose April 2024, we dosed the first patient with 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 first half lung. Recruitment is ongoing. Details of the Phase 1 study can be found at www.clinicaltrials.gov under NCT identifier NCT06228326. Inhaled KB707 also received Fast Track Designation from the FDA in February 2024, for the treatment of patients with solid tumors with pulmonary metastases that are relapsed or refractory to standard of care therapy.

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 TGM1-ARCI, TGM1-deficient autosomal recessive congenital ichthyosis ("TGM1-ARCI"), a serious rare skin disorder caused by missing or mutated TMG1 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 intend expect to commence our resume enrollment in the Phase 2 trial portion of JADE-1, a randomized, placebo-controlled Phase 1/2 study evaluating KB105 (JADE-1) for the treatment of TGM1-ARCI, later in 2024, and we anticipate filing plan to file an IND application with the FDA and initiating initiate a clinical trial of KB104 to treat patients with Netherton Syndrome in late 2024.

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

We are also 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 (“LCL”) at rest. The Phase 1, rest in up to 20 subjects. In January 2024, Jeune initiated the PEARL-1 Cohort 3 4 clinical study, is being conducted at a single center as an open label study to evaluate two different doses KB301 for the improvement of KB301 dynamic wrinkles of the décolleté in up to 20 subjects. Improvement of lateral canthal lines at rest was selected as a target indication for KB301 based upon the Phase 1 safety, efficacy and durability studies, which evaluated KB301 in the lower and upper cheek, including the lateral canthal region. Subjects will be followed for three months after KB301 treatment. Jeune has encountered delays in enrollment in the Cohort 3 study and plans expects to announce results from this study for both cohorts in the first half of 2024.

mid-2024. Following completion of this study, both cohorts, Jeune plans to initiate a Phase 2 study of KB301 in LCL at rest. KB301. Details of the Phase 1 study can be found at www.clinicaltrials.gov under NCT identifier (NCT04540900).

Business Highlights and Recent Developments

On October 10, 2023, the United States Patent & Trademark Office issued U.S. Patent No. 11,779,660 entitled Viral Vectors for Cancer Therapy to Krystal Biotech, Inc. The issued patent covers the Company’s lead oncology NCT04540900. Jeune has several other aesthetic medicine product candidate, KB707.

In August 2023, the Company sold its Rare Pediatric Disease Priority Review Voucher (“PRV”) for $100.0 million. The Company was awarded the PRV candidates in connection with the FDA’s accelerated approval various stages of VYJUVEK. Under the Rare Pediatric Disease Priority Review Voucher Program, the FDA awards priority review vouchers to sponsors of rare pediatric disease product applications that meet certain criteria. The program is intended to encourage development of new drugs and biologics for the prevention and treatment of rare pediatric diseases. A PRV can be redeemed to receive priority review of a subsequent marketing application for a different product, or alternatively, sold or transferred.

In August 2023, we began research and development operations in our second CGMP biologics manufacturing facility, ASTRA. ASTRA is a 155,000 sq. ft. state-of-the-art CGMP facility with comprehensive end-to-end capabilities, which currently include or will include, process development, analytical testing, virus and cell banking, commercial scale drug product/substance manufacturing, fill/finish and packaging, labeling and distribution, waste handling, environmental monitoring, and warehousing with temperature controlled CGMP storage. ASTRA is equipped with multiple clean rooms with flexibility designed into its configuration for further scale-up and scale-out capabilities. preclinical development.

Financial Overview

Revenue

In After FDA approval of VYJUVEK in May 2023, we received FDA approval for VYJUVEK for the treatment of DEB in patients six months or older. After approval, we began commercial marketing and sales of the product throughout the United States and began recognizing revenue in 3Q 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 through agreements with to a limited number of specialty pharmacies 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”), that distributes VYJUVEK to hospitals and outpatient clinics where patients are administered the medication at a healthcare professional’s office. The transaction price that we recognized recognize as revenue for products VYJUVEK sales includes an estimate of variable consideration, which includes discounts, returns, copay assistance and rebates that are offered within contracts with our customers. contracts. Refer to Note 2 of our condensed consolidated financial statements in our 2023 10-K for additional information.

Cost of Goods Sold

We recognize cost Cost of goods sold for 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 for VYJUVEK in May 2023, costs associated with the manufacturing of VYJUVEK were expensed as research and development expense. As such, a portion of the cost of inventory sold during the period was expensed prior to FDA approval. We anticipate that the previously expensed inventory has and will continue to favorably impact the cost of goods sold and the Company’s gross margin until the remaining pre-approval inventory is sold.

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, continue complete the Phase 1, Cohort Cohorts 3 and 4 study and initiate a Phase 2 trial for KB301, continue the Phase 1 trial for KB407, continue the Phase 1 trial for intratumoral KB707 and initiate a Phase 1 trial for inhaled KB707, initiate a Phase 1 trial for KB408, initiate a Phase 1 trial for KB104, begin our open label study with 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 fees, costs, management service fees, marketing expenses, and other 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 continued research and development of our product candidates and our commercial and operational goals. 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 as a result of our commercial activities in the U.S. and preparation for commercial operations outside of the U.S. 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, and ASTRA. In March 2023, we are currently in the process of completing and qualifying this facility. The Company placed a portion of ASTRA into service during the three and nine months ended September 30, 2023 as it was determined that certain assets were ready for their intended use. On March 27, 2023, the Company received the permanent occupancy permit for ASTRA which allowed the Company to begin utilizing certain portions parts of the building. As certain building improvements for research and certain development operations once qualification activities are still underway, was completed and a portion of the Company will 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 hold incur capital expenditures related to ASTRA throughout the remaining assets within construction in progress until validation has been completed and the assets are ready for their intended use. Validation operational life of the facility is expected to be completed in 2023.

Gain from Sale of Priority Review Voucher

Gain from sale of priority review voucher relates to proceeds from sale of the rare pediatric PRV we received in connection with the FDA's approval of VYJUVEK. 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, and Significant Judgments and Estimates

There have been no significant changes during the three months ended March 31, 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 United States 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.

Revenue Recognition

After FDA approval of VYJUVEK in May 2023, we began commercial marketing and made our first product sales in 3Q 2023. *ASC 606Revenue from Contracts with Customers* requires us to make estimates of variable consideration, included in contracts with customers, to be included in the transaction price.

The transaction price that is recognized as revenue for products upon delivery and transfer of control to the customer includes an estimate of variable consideration for reserves, which result from discounts, rebates, and returns that are offered within contracts between us and our customers.

- *Prompt Pay Discounts:* As an incentive for prompt payment, we offer a cash discount to customers. We estimate accrued prompt pay discounts using the most likely amount method. We record the discount as an allowance against accounts receivable, net and a reduction of revenue.
- *Government Rebates:* We participate in certain government rebate programs including Medicaid. We estimate accrued government rebates using the expected value method. We accrue estimated rebates based on estimated percentages of VYJUVEK prescribed to qualified patients, estimated rebate percentages and estimated levels of inventory in the distribution channel that will be prescribed to qualified patients and record the rebates as a reduction of revenue. Accrued government rebates are included in other accrued liabilities on the condensed consolidated balance sheets.
- *Commercial Rebates:* We participate in certain commercial rebate programs. Under these rebate programs, we pay a rebate to the commercial entity or third-party administrator of the program. Accrued commercial rebates are estimated using the expected value method. We accrue estimated rebates based on contract prices, estimated percentages of medicine that will be prescribed to qualified patients and estimated levels of inventory in the distribution channel and record the rebate as a reduction of revenue. Accrued commercial rebates are included in other accrued liabilities on the condensed consolidated balance sheets.
- *Copay Assistance:* We reimburse pharmacies for this discount through third-party vendors. We estimate accrued copay assistance costs using the expected value method. The estimate is based on contract prices, estimated percentages of VYJUVEK that will be prescribed to qualified patients, average assistance paid based on reporting from the third-party vendors and estimated levels of inventory in the distribution channel. Copay assistance costs are recorded as reductions to revenue and are accrued in other accrued liabilities on the condensed consolidated balance sheets.
- *Returns:* We offer SPs and SDs limited return rights relating to product damage or defect and based on these provisions, we believe that there will be minimal returns.

Variable consideration is estimated and reduces the transaction price to reflect our best estimate of the amount of consideration to which we are entitled based on the terms of the contracts and are recorded in the same period the related product revenues is recognized. The amount of variable consideration that is included in the transaction price may be constrained and is included in the net sales price only to the extent that it is considered probable that a significant reversal in the amount of the cumulative revenue recognized will not occur in a future period. Actual amounts of consideration ultimately received may differ from our estimates. If actual results in the future vary from our estimates, we will adjust these estimates in the period these variances become known.

Aside from revenue recognition as noted above, there have been no significant changes during the three and nine months ended September 30, 2023 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 Annual Report on Form 10-K for the year ended December 31, 2022.

Results of Operations

Three Months Ended September 30, 2023 March 31, 2024 and 2022 2023

	Three Months Ended September 30,			Three Months Ended March 31,		
	2023	2022	Change	2024	2023	Change
(In thousands)	**(unaudited)**					
Product revenues, net	$ 8,556	$ —	8,556			
Expenses:						
(in thousands)				*(in thousands)*	**(unaudited)**	
Product revenue, net						
Expenses						
Cost of goods sold						
Cost of goods sold						
Cost of goods sold / Cost of goods sold	223	—	223			
Research and development / Research and development	10,629	11,516	(887)			

Selling, general, and administrative	Selling, general, and administrative	23,697	19,935	3,762	
Litigation settlement					
Total operating expenses	Total operating expenses	34,549	31,451	3,098	
Loss from operations	Loss from operations	(25,993)	(31,451)	5,458	
Other income	Other income				Other income
Gain from sale of priority review voucher		100,000	—	100,000	
Interest and other income, net					
Interest and other income, net					
Interest and other income, net	Interest and other income, net	6,740	1,601	5,139	
Net income (loss)	Net income (loss)	$80,747	$(29,850)	$110,597	
Net income (loss)					
Net income (loss)					

Product Revenues, net Revenue, Net

Product revenues, revenue, net was $8.6 million $45.3 million for the three months ended September 30, 2023 March 31, 2024, as compared to zero for the three months ended September 30, 2022 March 31, 2023 due to initial sales of VYJUVEK after FDA approval was obtained on May 19, 2023. As VYJUVEK was approved by initial product sales revenue did not begin until the FDA in May third quarter of 2023, there were was no comparative period revenues. To date, all of our product revenue has been generated in the United States. revenue.

Cost of Goods Sold

Cost of goods sold was $223 thousand $2.4 million for the three months ended September 30, 2023 March 31, 2024, as compared to zero for the three months ended September 30, 2022 March 31, 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. As such, a portion of the cost of inventory sold during the period was expensed prior to FDA approval.

Research and Development Expenses

Research and development expenses decreased $887 thousand $1.3 million in the three months ended September 30, 2023 March 31, 2024 compared to the three months ended September 30, 2022 March 31, 2023.

The decrease was primarily due to decreases driven by the following:

- a decrease of $1.1 million in pre-commercial manufacturing expenses of approximately $1.1 million due to costs related to the manufacturing of VYJUVEK following FDA approval being recorded as inventory, a decrease in outsourced research inventory; and development of $581 thousand, and
- a decrease of $482 $899 thousand from in allocated overhead allocations costs due to inventory. the capitalization of commercial batches of VYJUVEK after its approval in May 2023.

These decreases were partially offset by increased payroll related expenses of $859 thousand, which were primarily driven by increases in the following:

- an increase in headcount to support overall growth, of $287 thousand of clinical development costs; and includes a $152 thousand increase in stock-based compensation, and
- an increase of $272 thousand of depreciation due to the Company's second CGMP facility being placed into service in depreciation 2023 offset by the capitalization of $484 thousand. commercial batches of VYJUVEK after its approval in May 2023.

Research and development expenses consist primarily of costs relating to the our preclinical and clinical development, the development of our product candidates and preclinical 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 pre-clinical 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 September 30, 2023 March 31, 2024 and 2022: 2023.

		Three Months Ended September 30,								
	Three Months Ended March 31, 2024 2024 2024									
							2023		Change	
(in thousands)	(in thousands)	2023	2022	Change						
KB103[1]										
KB103[1]										
KB103[1]	KB103[1]	$ 1,749	$ 2,330	$ (581)	$	2,129	$2,387		$ (258)	
KB105	KB105	7	7	—	KB105	15	231	231	(216)	(216)
KB407	KB407	441	585	(144)	KB407	783	377	377	406	406
KB408					KB408	245	112		133	
KB301	KB301	150	549	(399)	KB301	176	250	250	(74)	(74)
KB707	KB707	1,265	68	1,197	KB707	1,419	—	—	1,419	1,419
Other dermatology programs	Other dermatology programs	1	437	(436)	Other dermatology programs	—	8	8	(8)	(8)
Other respiratory programs		340	128	212						
Other aesthetics programs										
Other aesthetics programs										
Other aesthetics programs	Other aesthetics programs	9	42	(33)		134	13	13	121	121
Other research programs	Other research programs	172	232	(60)	Other research programs	351	596	596	(245)	(245)
Other development programs	Other development programs	160	175	(15)	Other development programs	232	335	335	(103)	(103)
Stock-based compensation	Stock-based compensation	2,336	2,184	152	Stock-based compensation	1,868	2,496	2,496	(628)	(628)
Other unallocated manufacturing expenses[2] [1]	Other unallocated manufacturing expenses[2] [1]	2,682	3,639	(957)	Other unallocated manufacturing expenses[2] [1]	2,131	3,919	3,919	(1,788)	(1,788)

Other unallocated expenses(3) (2)	Other unallocated expenses(3) (2)	1,317	1,140	177	Other unallocated expenses(3) (2)	1,474	1,564	1,564	(90)		(90)
Research and development expense	Research and development expense	$ 10,629	$ 11,516	$ (887)	Research and development expense	$ 10,957		$ 12,288		$ (1,331)	

(1) For the three months ended September 30, 2023,KB103 expenses consist of post marketing study costs and overseas preclinical and clinical trial costs, licensing and regulatory costs.

(2) 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.

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

As noted above, The primary changes in our research and development expenses decreased $887 thousand by product candidate or program in the three months ended September 30, 2023 March 31, 2024 compared to the three months ended September 30, 2022. Expenses for KB103 decreased $581 thousand following the U.S. commercial launch of VYJUVEK. Other March 31, 2023 are as follows:

- a decrease in other unallocated manufacturing expenses decreased $957 thousand of $1.8 million due to the costs related to the manufacturing of VYJUVEK following FDA approval being recorded as inventory and fewer receipts cost of raw materials period over period that were purchased for planned manufacturing runs goods sold;
- a decrease in stock-based compensation of the Company's products. Other Dermatology decreased $436 $628 thousand due to the allocation of labor costs related to work performed to manufacture VYJUVEK to inventory following FDA approval; and
- a reduction net decrease in contract manufacturing expenses. These decreases were partially KB103 costs is due to research and development costs being expensed in 2023 prior to receiving FDA approval in May 2023 that are now included as part of the cost of inventory, offset by an increase in expenses overseas preclinical and clinical trial costs, licensing and regulatory costs, and on-going manufacturing efficiency and process optimization costs for which such processes have not yet been approved by the FDA; offset by
- an increase in KB707 costs of $1.2 million due primarily $1.4 million following the expansion of our research and development pipeline to increased internal resources and other oncology consisting of increase in payroll related costs to support continued our research and increases an increase in contract research expenses in preparation for the Phase 1 clinical trial. trial of inhaled KB707.

Selling, General, and Administrative Expenses

Selling, general, and administrative expenses increased $3.8 million $2.0 million in the three months ended September 30, 2023 March 31, 2024 as compared to the three months ended September 30, 2022 March 31, 2023. Higher

The increase was primarily driven by the following:

- an increase in selling general, expenses related to the launch of VYJUVEK of $2.1 million, which includes $1.5 million related to our patient access program;
- an increase of $706 thousand related to professional services incurred to support our commercial growth; and administrative spending was due largely to increases
- an increase in net payroll related expenses of approximately $2.2 million, $367 thousand, which was primarily driven by an increase in headcount in our commercial and other administrative functions to support overall growth and preparation for

  commercialization and includes an a partially offsetting $900 thousand decrease in stock-based compensation of $509 thousand due to the departure of certain employees increased selling expenses related to in the launch second half of VYJUVEK of $733 thousand, increased travel related costs of $452 thousand, increased information technology infrastructure costs of $423 thousand, increased software-related costs of $377 thousand, increased legal and professional costs of $327 thousand, and a $189 thousand increase in other selling, general, and administrative expenses. 2023.

These increases were partially offset by a decrease in marketing costs of $921 thousand $1.3 million due to the timing of marketing activities ahead of the VYJUVEK launch.

Litigation Settlement

Gain from sale Litigation settlement for each of Priority Review Voucher ("PRV") the three months ended March 31, 2024 and 2023 was $12.5 million, 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.

Gain from sale of priority review voucher *Interest and Other Income, Net*

Interest and other income, net for the three months ended September 30, 2023 March 31, 2024 and 2023 was $100.0 million $7.6 million and is related to the sale of our rare pediatric disease PRV, which was awarded to the Company in connection with the FDA's approval of VYJUVEK.

Interest and Other Income

Interest and other income for the three months ended September 30, 2023 and 2022 was $6.7 million and $1.6 million $3.5 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.

Nine Months Ended September 30, 2023 and 2022

	Nine Months Ended September 30,		
	2023	2022	Change
(In thousands)	(unaudited)		
Product revenues, net	$ 8,556	$ —	$ 8,556
Expenses:			
Cost of goods sold	223	—	223
Research and development	35,061	31,720	3,341
Selling, general, and administrative	73,637	53,705	19,932
Litigation settlement	12,500	25,000	(12,500)
Total operating expenses	121,421	110,425	10,996
Loss from operations	(112,865)	(110,425)	(2,440)
Other income:			
Gain from sale of priority review voucher	100,000	—	100,000
Interest and other income, net	15,105	902	14,203
Net income (loss)	$ 2,240	$ (109,523)	$ 111,763

Products Revenues, net

Product revenues, net was $8.6 million for the nine months ended September 30, 2023 as compared to zero for the nine months ended September 30, 2022 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 revenues. To date, all of our product revenue has been generated in the United States.

Cost of Goods Sold

Cost of goods sold was $223 thousand for the nine months ended September 30, 2023 as compared to zero for the nine months ended September 30, 2022 due to initial sales of VYJUVEK after FDA approval was obtained on May 19, 2023. Due to the timing of FDA approval and our manufacturing schedule, a majority of the costs associated with VYJUVEK inventory manufactured for the commercial launch were previously expensed as research and development. Prior to receiving FDA approval for VYJUVEK in May 2023, costs associated with the manufacturing of VYJUVEK were expensed as research and development expense. As such, a portion of the cost of inventory sold during the period was expensed prior to FDA approval.

Research and Development Expenses

Research and development expenses increased $3.3 million in the nine months ended September 30, 2023 compared to the nine months ended September 30, 2022. The increase was primarily due to increased payroll related expenses of $5.5 million, which were primarily driven by an increase in headcount to support overall growth and includes a $2.1 million increase in stock-based compensation, an increase in depreciation of $1.8 million, and increased other research and development expenses of $877 thousand primarily due to facilities costs and licenses and regulatory fees. These increases were partially offset by decreases in preclinical, clinical and pre-commercial manufacturing expenses of $4.0 million, due to the costs related to the manufacturing of VYJUVEK following FDA approval being recorded as inventory and due to fewer receipts of raw materials and lab supplies period over period that were purchased for planned manufacturing runs of our products, a decrease of

$635 thousand from overhead allocations to inventory, and a decrease in outsourced research and development of $338 thousand.

The following table summarizes our research and development expenses by product candidate or program, and for unallocated expenses, by type, for the nine months ended September 30, 2023 and 2022:

	Nine Months Ended September 30,		
(in thousands)	2023	2022	Change
KB103(1)	6,311	5,672	$ 639
KB105	260	116	144
KB407	1,259	1,522	(263)
KB301	480	923	(443)
KB707	2,633	66	2,567
Other dermatology programs	15	491	(476)
Other respiratory programs	749	531	218
Other aesthetics programs	23	109	(86)
Other research programs	429	672	(243)
Other development programs	689	461	228
Stock-based compensation	7,695	5,547	2,148
Other unallocated manufacturing expenses(2)	10,207	12,611	(2,404)
Other unallocated expenses(3)	4,311	2,999	1,312
Research and development expense	$ 35,061	$ 31,720	$ 3,341

(1) For the nine months ended September 30, 2023,KB103 expenses included costs related to post market study costs and overseas preclinical and clinical trial costs, licensing and regulatory costs.

(2) 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.

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

As noted above, research and development expenses increased $3.3 million in the nine months ended September 30, 2023 compared to the nine months ended September 30, 2022. Expenses for KB103 increased $639 thousand, due to increased payroll related expenses to support VYJUVEK's pre-approval activities, clinical trial costs, license and regulatory costs, costs associated with overseas clinical trials and regulatory agency filings, and increased allocated research and development expenses. Due to the expansion of our research and development pipeline to oncology, KB707 spending increased $2.6 million primarily due to increased payroll related costs and increased contract research costs in preparation for the Phase 1 clinical trial. Stock-based compensation increased $2.1 million due to an increase in internal resources to support overall research and development growth. Additionally, other unallocated expenses increased $1.3 million primarily related to increases in depreciation expense. These increases were offset by a decrease in other unallocated manufacturing expenses of $2.4 million due to the costs related to the manufacturing of VYJUVEK following FDA approval being recorded as inventory and due to fewer receipts of raw materials period over period that were purchased for planned manufacturing runs of our products and a $443 thousand decrease in KB301 due to the reduction of clinical research costs.

Selling, General, and Administrative Expenses

Selling, general, and administrative expenses increased $19.9 million in the nine months ended September 30, 2023 as compared to the nine months ended September 30, 2022. Higher selling, general, and administrative spending was due largely to increases in payroll related expenses of approximately $15.1 million, which was primarily driven by an increase in headcount in our commercial and other administrative functions to support overall growth and preparation for commercialization, and includes a $4.3 million increase in stock-based compensation, increased information technology infrastructure costs of $1.6 million, increased software-related costs of $1.0 million, increased travel related costs of $957 thousand, increased selling expenses related to the commercial launch of VYJUVEK of $733 thousand, increased legal and professional costs of $418 thousand, increased marketing costs of $401 thousand, and an increase in other selling, general, and administrative expenses of $769 thousand, which consisted primarily of increased conference costs and utilities costs. These increases were partially offset by decreases in business development costs of $531 thousand and medical affairs costs of $511 thousand.

Litigation Settlement

Litigation settlement for the nine months ended September 30, 2023 and 2022 was $12.5 million and $25.0 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.

Gain from sale of Priority Review Voucher

Gain from sale of priority review voucher for the nine months ended September 30, 2023 was $100 million related to the sale of our rare pediatric PRV, which was awarded to the Company in connection with the FDA's approval of VYJUVEK.

Interest and Other Income

Interest and other income for the nine months ended September 30, 2023 and 2022 was $15.1 million and $2.5 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.

Liquidity and Capital Resources

Overview

At September 30, 2023 As of March 31, 2024, our cash, cash equivalents and short-term investments balance was approximately $562.1 million $538.3 million. Since operations began, we have incurred operating losses. At September 30, 2023 As of March 31, 2024, we had an accumulated deficit of $278.5 $268.9 million. We believe that our cash, cash equivalents and short-term investments as of September 30, 2023 March 31, 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.

As we continue to incur operating losses, a Our transition to operating profitability is dependent upon the continued successful commercialization of VYJUVEK and the successful development, approval, manufacturing, and commercialization of our product candidates and the achievement of a level of revenues adequate to support our cost structure. candidates. Furthermore, we expect to incur increasing costs associated with satisfying regulatory and quality standards, maintaining and initiating product and clinical trials, and furthering our efforts around our 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 never achieve operating profitability, and unless and until we do, we will continue to need to raise also seek additional capital through arrangements with strategic partners or obtain financing from 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 our continued clinical studies of KB105, KB407, KB408, KB301, KB707 or our planned clinical and preclinical studies for our other 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 paid $12.5 million upon the approval of VYJUVEK by the FDA, and will be required to pay three additional $12.5 million contingent milestone payments upon reaching reporting $100.0 million in total cumulative sales, $200.0 million in total cumulative sales and $300.0 million in total cumulative sales. Our funds may not be sufficient to enable us to conduct pivotal clinical trials for, seek marketing approval for or commercial commercially launch of KB104, KB105, KB407, KB408, KB301, KB707, ophthalmic B-VEC or any other product candidate. 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 pharmaceutical products, 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 of VYJUVEK and revenue received from commercial sale of VYJUVEK;

- the continued development and the filing of an IND application 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 nine three months ended September 30, 2023 March 31, 2024 and 2022 (in thousands): 2023:

	Nine Months Ended September 30,	
	2023	2022
	(unaudited)	
Net cash used in operating activities	(81,572)	(78,240)
Net cash provided by (used in) investing activities	92,798	(108,875)
	2024	
	2024	
	2024	
(in thousands)		
(in thousands)		
(in thousands)		
Net cash provided by (used in) operating activities		

Net cash provided by (used in) operating activities			
Net cash provided by (used in) operating activities			
Net cash (used in) provided by investing activities			
Net cash (used in) provided by investing activities			
Net cash (used in) provided by investing activities			
Net cash provided by financing activities			
Net cash provided by financing activities			
Net cash provided by financing activities	Net cash provided by financing activities	200,131	32,278
Effect of exchange rate changes on cash and cash equivalents	Effect of exchange rate changes on cash and cash equivalents	(16)	—
Effect of exchange rate changes on cash and cash equivalents			
Effect of exchange rate changes on cash and cash equivalents			
Net increase (decrease) in cash	Net increase (decrease) in cash	$ 211,341	$ (154,837)
Net increase (decrease) in cash			
Net increase (decrease) in cash			

Operating Activities

Net cash used in provided by operating activities for the nine three months ended September 30, 2023 March 31, 2024 was $81.6 $15.9 million and consisted primarily of net income of $2.2 million $932 thousand adjusted for $8.7 million of non-cash items primarily comprised and a $6.3 million increase in working capital. Non-cash adjustments included depreciation of a gain on sale $1.4 million, amortization of the rare pediatric PRV operating lease right-of-use assets of $100.0 million, $182 thousand and stock-based compensation expense, net of $30.1 $9.3 million, depreciation and amortization offset by realized gain on investments of $2.6 $1.2 million, accretion on marketable securities of $802 thousand and other adjustments of $4.3 million, and cash used by increases in net working capital of approximately $12.2 million.

Net cash used in operating activities for the nine months ended September 30, 2022 was $78.2 million and consisted primarily of a net loss of $107.9 million adjusted for non-cash items primarily comprised of depreciation and amortization and stock-based compensation expense of $26.4 million, and including net changes in operating assets and liabilities of approximately $3.2 million. $238 thousand.

Investing Activities

Net cash provided by investing activities for the nine months ended September 30, 2023 was $92.8 million and consisted primarily of proceeds of $100.0 million from the sale of the rare pediatric PRV and $428.6 million received from the maturities of short-term investments, partially offset by expenditures of $10.0 million on the build-out of our ASTRA facility, leasehold improvement of new office space, and purchases of computer and laboratory equipment, and $425.9 million on the purchase of short-term and long-term investments.

Net cash used in investing activities for the nine three months ended September 30, 2022 March 31, 2024 was $108.9 million $26.0 million and consisted primarily of expenditures of $47.8 million on the build-out of our ASTRA facility, leasehold improvement of new office space, and $88.3 million in purchases of computer and laboratory equipment, $214.7 million on the purchase of short-term and long-term investments and $1.3 million in purchases of property and equipment, partially offset by proceeds of $153.6 million $63.6 million received from the maturities of short-term investments.

Financing Activities

Net cash provided by financing activities for the nine three months ended September 30, 2023 March 31, 2024 was $200.1 $10.6 million and consisted primarily of proceeds of $160.0 $16.0 million received from a private placement equity offering and proceeds of $40.9 million primarily from exercises of stock options, partially offset by $749 thousand $4.2 million used for the employee tax withholding payment 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 nine months ended September 30, 2022 was $32.3 million and consisted primarily of proceeds of $32.9 million received from our ATM Program and exercises of stock options and offset by $649 thousand used for the employee tax withholding payment for settlement of vested restricted stock awards.

ITEM 3. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK

Qualitative and Quantitative Disclosures About Market Risk

We had cash, cash equivalents and short-term investments of $562.1 million at September 30, 2023 $538.3 million as of March 31, 2024, which consist consisted primarily of money market bank deposits, 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 and long-term 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 a 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 the any investments we make in the future will not be subject to adverse changes in market value. Our cash, cash equivalents and short and long-term 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 ("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

During the three months ended September 30, 2023, in conjunction with the first sales of VYJUVEK, we implemented new procedures and controls related to the accounting and disclosures for revenue recognition, accounts receivable, and cost of goods sold. Management will continue to evaluate and monitor our internal controls as processes and procedures in each of the affected areas evolve.

Other than discussed above, There was no change in our internal control over financial reporting that occurred during the three months ended September 30, 2023 March 31, 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 ITEM 1A. Risk Factors. RISK FACTORS

There Our business involves significant risks, some of which are a number of described below. You should carefully consider the risks and uncertainties that may have a material effect on described below, together with all of the operating results of our business and our financial condition. The risk factors below reflect material changes to risks to our business following the FDA approval of VYJUVEK on May 19, 2023, and should be reviewed other information contained in conjunction with "Summary Risk Factors" and "Item 1A. Risk Factors" in our Annual this Quarterly Report on Form 10-K for 10-Q, including "Management's Discussion and Analysis of Financial Condition and Results of Operations" and the fiscal year ended December 31, 2022, as filed with condensed consolidated financial statements and the SEC on February 27, 2023. Except as set forth below, the Risk Factors related notes included in our Annual Report on Item 1 of Part I of this Form 10-K for the year ended December 31, 2022 remain unchanged.

Risks Related to Our Financial Position and Need for Additional Capital

We have incurred net losses since inception. We expect to incur losses for the foreseeable future and may never achieve or maintain operating profitability.

Since inception, we have incurred recurring losses and negative cash flows from operations and, at September 30, 2023, we had an accumulated deficit of $278.5 million. Our ability to achieve operating profitability depends on our ability to successfully complete the development of, and obtain the regulatory approvals necessary to commercialize, our product candidates. We have devoted substantially all our efforts to date to research and development of our gene therapy platform, product candidates and our manufacturing infrastructure. We expect to continue to incur significant expenses and operating losses for the foreseeable future. The net losses we incur may fluctuate significantly from quarter to quarter. We anticipate that our expenses will increase substantially if, and as, we:

- commercialize, manufacture and market our lead product, VYJUVEK;
- continue our research and the clinical development of our product candidates, including our current clinical trials and planned future trials;
- initiate clinical trials for certain of our product candidates and preclinical studies for any additional product candidates that we may pursue in the future;
- prepare for regulatory approvals for our product candidates in the United States, European Union and in other key geographies;
- continue to operate our in-house commercial-scale CGMP manufacturing facility, ANCORIS, and complete build out and startup of operations at our second CGMP manufacturing facility, ASTRA;
- manufacture material for clinical trials or potential commercial sales;
- further develop our gene therapy product candidate portfolio;
- further establish a sales, marketing and distribution infrastructure to commercialize any product candidate 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 become and remain profitable, we must develop and maintain commercial scale manufacturing processes for VYJUVEK or any other product candidates that become commercially available. 10-Q. If we were required to discontinue development of any of our product candidates, if any of our product candidates do not receive regulatory approval, if we do not obtain our targeted indications for our product candidates or if any of our product candidates, if approved, fails to achieve sufficient market acceptance for any indication, we the following risks actually occur, it could be delayed by many years in our ability to achieve operating profitability, if ever, and harm our business, prospects, operating results and financial condition could be materially adversely affected. Moreover, if we decide to leverage any success with any and future prospects. In such event, the market price of our current product candidates to develop other product opportunities, we may not be successful in such efforts. In any such event, our business will be materially adversely affected.

We currently have one FDA-approved product, VYJUVEK, common stock could decline, 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 revenues from VYJUVEK, or any other product candidate that may receive regulatory approval in the future, that is significant or large enough to achieve operating profitability. If we do achieve operating profitability, we may not be able to sustain or increase operating profitability on a quarterly or annual basis. Our failure to become and remain profitable would decrease the value of our company and you 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 your investment.

Because of the numerous Additional risks and uncertainties associated with pharmaceutical products and biological development, we are unable not presently known to accurately predict the timing or amount of increased expenses or when, or if, we will be able to achieve operating profitability. If we are required by the FDA, the EMA, 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 revenue from product candidates in development and operating profitability from commercially available products could be delayed.

We may need to raise additional funding in order to expand our marketing and distribution capabilities for VYJUVEK and receive approval for our other 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 sales, marketing and distribution infrastructure that we believe are necessary to successfully commercialize VYJUVEK, we may require substantial additional funding. We expect to incur significant expenses related to sales, medical affairs, marketing, manufacturing and distribution of VYJUVEK. We also may need additional funding to complete the development of our product candidates and to commercialize any additional products that receive regulatory approval. Our future capital requirements will depend on many factors, including:

- the ability of VYJUVEK to generate revenue;

- the costs of product sales, medical affairs, marketing, manufacturing and distribution for VYJUVEK;
- the progress, timing results and costs of our Phase 1/2 clinical trial for KB105;
- the progress, timing, results and costs of our Phase 2 clinical trial for KB301;
- the progress, timing, results and costs of our Phase 1 clinical trial for KB407;
- the progress, timing, results and costs of our planned Phase 1 clinical trial for KB707;
- the continued development and the filing of IND applications for KB104, KB408, 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 other product candidates that we may pursue in the future, if any;
- the costs of building and 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, 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 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 currently deem immaterial 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;
- our current license agreements, if any, 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.

Identifying potential 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, if any, 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 also impair 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 our 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 and growth potential and the market price per share of our common stock. operations.

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 limited to organizing and staffing our company, business planning, raising capital, developing our vector platform and related technologies, identifying and developing 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 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 will need to transition from a company with a research and development focus to a company capable of 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 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 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 approval 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", and the "Summary Risk Factors" and

"Item 1A. Risk Factors" in our Annual Report on Form 10-K for the fiscal year ended December 31, 2022. 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 have limited experience as a commercial company may not be successful in our efforts to identify, develop and the sales, marketing, commercialize additional product candidates, which may impair our ability to expand our business and distribution of VYJUVEK achieve our strategic objectives, and we may fail to capitalize on programs or any future approved products product candidates that may be unsuccessful a greater commercial opportunity or less successful than anticipated. for which there is a greater likelihood of success.

We recently received FDA approval Although a substantial amount of our efforts focuses on the commercialization of VYJUVEK and we have initiated a commercial launch of VYJUVEK in the United States. As a company, we have no prior experience commercializing a drug. 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, and distribution capabilities and our ability to navigate the significant expenses and risks involved with the development and management potential approval of such capabilities. For example, our commercial launch current product candidates, a key component 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 planned safe and effective by patients, the medical community, or anticipated, which may require us to, among others, adjust or amend our business plan 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. third-party payors.

If we are unsuccessful in accomplishing identifying and developing additional product candidates, our objectives 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 executing 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, plan, 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, or collectively, the ACA, includes a subtitle called the Biologics Price Competition and Innovation Act of 2009, or 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 are currently developing alternative autologous or palliative gene therapy approaches for our targeted indications, including DEB and cystic fibrosis. 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.

In the future, 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 will depend upon physicians who specialize in the treatment of genetic diseases targeted by our product candidates prescribing treatments that involve the use of our product candidates 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 do 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 develop as planned, 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 successfully 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 future of our product candidates that are approved products, we for sale, and, depending on demand, may require need to scale up the manufacturing process for any approved product, which is subject to risks and uncertainties. Future growth would impose significant additional capital added responsibilities on members of management. Our management, finance, development personnel, systems, and financial resources, we facilities currently in place may not become profitable, 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 compete 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 to us. 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 more established companies in our industry.

If us and we are unable 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 expand 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 distribution capabilities 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 collaborate with third parties delay marketing approval of our product candidates, restrict or regulate post-approval activities, and affect our ability to market and profitably sell VYJUVEK or future and any product candidates for which we obtain marketing approval, 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 may be 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 generate sufficient product revenues.

To successfully commercialize any products that may result 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 our development activities, we need three to continue five years. In August 2022, the Inflation Reduction Act of 2022 ("IRA") was signed into law. The IRA includes several provisions to expand our marketing lower prescription drug costs for people with Medicare and distribution capabilities, either on our own or 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 collaboration with others. The development of our marketing and distribution effort is, 2023, and will continue to be expensive 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 time-consuming 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 delay any lead to further drug pricing initiatives that could affect reimbursement for our product launch. We cannot and product candidates.

These initiatives, as well as other healthcare reform measures that may be certain adopted in the future, may result in more rigorous coverage criteria and in additional downward pressure on the price that we will be able to develop this capability successfully. We receive for any approved product. Any reduction in reimbursement from Medicare or other government programs may enter into collaborations regarding VYJUVEK, result in a similar reduction in payments from private payors. The implementation of cost containment measures or any future approved other healthcare reforms could result in reduced demand for our product and product candidates with other entities or additional pricing pressures and may adversely impact our ability to utilize their established marketing and distribution capabilities. However, we may be unable to enter into such agreements on favorable terms, if at all. If any future collaborators do not commit generate sufficient resources to revenue, attain consistent profitability, or commercialize VYJUVEK or any future 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 develop comply, or have not fully complied, with such laws, we could face substantial penalties.

With the necessary capabilities 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 own, 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 be unable resolve allegations in a settlement without admitting liability. Any such settlement could materially affect our business, financial operations, and reputation.

Efforts to generate sufficient product revenues to sustain ensure that our business. We compete business arrangements with many companies that currently have extensive, experienced and well-funded sales, distribution and marketing operations to recruit, hire, train and retain marketing and sales personnel. We also face competition in our search for third parties to assist us comply with the sales applicable healthcare laws and marketing efforts of VYJUVEK 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 any future product candidates, if approved.

abuse or other healthcare laws and regulations.

The market for VYJUVEK, 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 any future product candidates for which we obtain marketing approval, may be smaller than we expect.

We focus our research and product development on genetic medicines for patients with debilitating diseases. We base our market opportunity estimates on their provisions are open to a variety of factors, including 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 estimates 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 number of people who 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 these diseases, a material adverse effect on the potential scope success of our approved product labels, business.

We are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the subset generation, handling, use, storage, treatment, manufacture, transportation, and disposal of, people 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, and our property, casualty and general liability insurance policies specifically exclude coverage for damages and fines arising from biological or hazardous waste exposure or contamination. Accordingly, in the event of contamination or injury, we could be held liable for damages or be penalized with fines in an amount exceeding our resources, 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 do not maintain insurance for toxic tort claims that may be asserted against us in connection with our storage or disposal of biologic or hazardous materials. 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 diseases who have the potential to benefit from treatment with VYJUVEK laws and regulations also may result in substantial fines, penalties 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 other sanctions or prevalence of these diseases. Estimating market opportunities can be particularly challenging for rare indications, such as the ones we currently address, 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 begin to commercialize VYJUVEK in the United States and learn more about market dynamics and engage with regulators on additional potential marketing approvals, our view of our product'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, or patients may not be otherwise amenable to treatment with our products, all of liabilities, which would 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 may also 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 and manufacturing chain. 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 product and 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 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 has published proposed rules that would require companies to provide significantly expanded climate-related disclosures in their periodic reporting, which may require us 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, or the SEC, 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 VYJUVEK or any future 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 attractive 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 opportunities, could decrease our future ability to generate sufficient product revenues may be smaller than anticipated, revenue, and our business, may suffer. financial condition, results of operations and prospects would be materially and adversely affected.

Our products 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 in the future 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 revenues 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 such product;
- 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 development 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 patients 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 patients 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 patient 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 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 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 Investigational New Drug, or 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 patients.

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 patient data become 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 patient enrollment continues and more patient 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 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 Biologics License Application ("BLA") 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 supply contracts, including 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 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, and we may not achieve or sustain operating profitability, which would materially and adversely affect our business, financial condition, results of operations and prospects.

While we have obtained orphan drug exclusivity for VYJUVEK and orphan drug designation for VYJUVEK, KB105, KB407, and KB408, 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 for products that constitute the same drug and treat the same indications as our product candidates before us, we may not be able to have competing products approved by the applicable regulatory authority for a significant period of time.

On November 2, 2017, the FDA granted orphan drug designation to our lead product, VYJUVEK, for the treatment of DEB. On April 16, 2018, the European Commission granted the Orphan Medicinal Product Designation ("OMPD"), for VYJUVEK. On August 7, 2018, the FDA granted orphan drug designation to our product candidate, KB105, currently in clinical development for treatment of patients with TGM1-ARCI, and on October 10, 2019, the European Commission granted the OMPD for KB105. On August 17, 2020, the FDA granted orphan drug designation to our product candidate, KB407, currently in clinical development, for the treatment of cystic fibrosis, and on January 13, 2023, the European Commission granted the OMPD for KB407. On September 1, 2023, the FDA granted orphan drug designation to our product candidate, KB408 for the treatment of alpha-1 antitrypsin deficiency (AATD). Regulatory authorities in some jurisdictions, including the United States, the European Union, and the EU, 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 EU, 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 EU European Union would be sufficient to justify the necessary investment in developing the drug or biologic product.

Generally, if a In the European Union, orphan medicinal product candidate with an designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process, but orphan drug designation receives may entitle an applicant to financial incentives such as reduction of fees or fee waivers, protocol assistance, and access to the first centralized marketing approval authorization procedure. Upon grant of a marketing authorization, orphan products are entitled to ten years of market exclusivity for the approved therapeutic indication, for which it has such designation, the product is entitled to a period of marketing exclusivity, which precludes the FDA or means that the EMA from approving 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 that constitutes the same drug treating 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 marketing have also complied with an agreed Pediatric Investigation Plan, or PIP. The ten-year market exclusivity period, except in limited circumstances. If another sponsor receives such approval before we do (regardless of our orphan drug designation), we will be precluded from receiving marketing approval for our product for the applicable exclusivity period. The applicable period is seven years in the United States and 10 years in the EU. The exclusivity period in the EU can European Union may be reduced to six years if, a at the end of the fifth year, it is established that the product no longer meets the criteria for which it received orphan drug designation, or if including where it is shown that the product is sufficiently profitable so that 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 longer justified. Orphan 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 revoked if any regulatory agency determines that rescinded by the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the product to meet the needs of patients with the rare disease or condition. Japanese government in certain circumstances.

Even though we have obtained orphan drug exclusivity for VYJUVEK in the United States; orphan drug designation for VYJUVEK 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 or product candidate from competition because different drugs can be approved for the same condition. In the United States, even after an 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 approved, 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 subsequently approve another drug 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 same condition 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 concludes did allow such pathway, there can be no assurance that the latter drug is not the same drug any expedited development, review, or is clinically superior in that it is shown to be safer, more effective or makes a major contribution to patient care. In the EU, marketing authorization may approval will be granted to on a similar medicinal product for the same orphan indication if:

- the second applicant can establish in its application that its medicinal product, although like the orphan medicinal product already authorized, is safer, more effective timely basis, or otherwise clinically superior;
- the holder of the marketing authorization for the original orphan medicinal product consents to a second orphan medicinal product application; or
- the holder of the marketing authorization for the original orphan medicinal product cannot supply enough quantities of orphan medicinal product. at all.

Breakthrough therapy designation, Therapy Designation, Fast Track Designation, Regenerative Medicine Advanced Therapy designation, Fast Track designation Designation or Rare Pediatric Disease designation 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 it does such designations may not increase the likelihood that any of our product candidates will receive marketing approval in the United States. approval.

We may seek a Breakthrough Therapy Designation for some of our product candidates. A RMAT/PRIME breakthrough therapy product candidate is defined as a product candidate therapy that is intended, alone or in combination with one or more other drugs, therapies, to treat a serious or life-threatening disease. Drugs 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 RMAT 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 are may also be eligible for priority review and accelerated approval and increased interaction and communication with 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 designed may disagree and instead determine not to expedite 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 process. 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 were granted Fast Track Designation by the FDA. If a drug, or biologic in our case, therapy is intended for the treatment of a serious or life-threatening condition and the biologic therapy demonstrates the potential to address unmet medical needs for this condition, the biologic sponsor may apply for FDA Fast Track designation. 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 after having received if we do receive Fast Track designation, Designation, we may not experience a faster development process, review or approval compared to conventional FDA procedures. In addition, 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 Designation if it believes that the designation is no longer supported by data from our clinical development program. programs. Many biologics that have received Fast Track designation Designation have failed to obtain marketing approval. Fast Track Designation alone does not guarantee qualification for the FDA's priority review procedures.

We have obtained and may seek Regenerative Medicine Advanced Therapy, or RMAT, designation for some of our product candidates. For instance, VYJUVEK was granted RMAT designation by the FDA. 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 who 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. With 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 the FDA issued us in May 2023. The priority review voucher was sold in August 2023. We have also obtained a Rare Pediatric Priority Review Voucher. Also, we received the designation of "rare pediatric disease" for KB105, KB104, and for KB407, which could qualify us to receive a Rare Pediatric Priority Review Voucher.

There is no assurance we will receive RMAT, PRIME or breakthrough therapy or Fast Track designations for any of our other product candidates and the receipt of any of these designations for a product candidate may not result in a faster development process, review or approval and does not assure ultimate approval by the FDA. Further, even though we have received rare pediatric disease designation for KB105, KB104, and KB407, for KB407. 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 experience 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 for a subsequent marketing application. process.

We face significant competition 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, an environment or utilized by, a product approved under a New Drug Application, or NDA, or BLA; (2) preliminary evidence submitted by the sponsor of rapid technological change and the possibility approved product, or a sponsor 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 has been granted a right of reference to successfully commercialize and market our product candidates.

We are aware of several companies and institutions that are currently developing alternative autologous or palliative gene therapy approaches for DEB and cystic fibrosis. 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 data submitted 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 any application for such 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 ours, 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.

In the future, 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 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 our products or 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 our approved product 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 patients or other claimants;
- product recalls for 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

- demonstrates that the inability to commercialize our product candidates.

With respect to VYJUVEK and any of our product candidates that are approved for commercial sale in platform technology has 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 potential to be incorporated in, 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 utilized by, more than one 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. 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 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 business. A successful 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 liability claim candidates will be developed more quickly or series of claims brought against us, particularly receive a faster FDA review process or ultimate FDA approval. Moreover, the FDA may revoke a designation 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. 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 product development and commercialization efforts. 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. 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 would will be subject to possible regulatory action and may not be permitted to sell any approved product or product candidate that we may develop.

In addition, the manufacturing process used to produce VYJUVEK and our product candidates is complex and novel. novel and demand for an approved product may require the need for us to change to 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 the 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 clinical-grade 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. 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. We supply of VYJUVEK or a product candidate that is approved for sale. In addition, we may utilize third parties to manufacture components of our products. For example, we use a third-party for manufacturing of 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. 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 that of any third-party manufacturers of the foregoing, if we do not successfully carry out their contractual duties, meet expected deadlines have an alternative supplier or manufacture manufacturer in accordance with regulatory requirements or if there are disagreements between us and any third-party manufacturers, place, we may not be able to manufacture our products for commercial, regulatory, or regulatory purposes. In such instances, we may need clinical purposes and would be required to locate an appropriate replacement third-party relationship, which may not expend substantial management time and expense to identify, qualify and transfer to alternative suppliers or manufacturers. There can be readily no assurance that replacements would be available or to us on a timely basis, on acceptable terms, which would cause additional or at all. Any need to find and qualify new suppliers or manufacturers could significantly delay production of VYJUVEK or increased expense any product candidate, if approved, adversely impact our ability to market VYJUVEK or any product candidate, if approved, and would thereby 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 or marketing schedules. 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 drugs or drug products, conducting clinical trials, preventing defects, improving our product candidates, and assuring the safety and efficacy of our product and 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 our products, 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 our product or 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 a product manufactured with changes to an approved manufacturing process may be sold commercially, such change will require development, validation and documentation, and the preparation and submission to the FDA of a PAS, which must be approved by the FDA. With respect to our revised manufacturing process for VYJUVEK, we are planning to submit a PAS to the FDA in 2H 2024. The FDA will have up to four months to review the PAS, as well as the data and other information we submit to support the changes to the current FDA-approved manufacturing process. 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 Product Candidates 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 market 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 development access, sales, and distribution capabilities or enter into agreements collaborate with third parties to market and sell VYJUVEK or our product candidates for which we obtain marketing approval, we may be unable to generate sufficient product revenues. revenue.

To successfully commercialize VYJUVEK, and any other approved product candidates, we have expanded our capabilities to promote market access and build awareness. To successfully commercialize any products that may result from our development programs, for which we obtain marketing approvals, we will need to further expand our sales force, marketing, market development organization, access, and medical affairs teams and distribution capabilities, either on our own or in collaboration with a third-party. others. The development of our own a sales force, marketing, market development team access, and medical affairs teams and distribution capabilities effort is expensive and time-consuming, and could delay any product launch. Moreover, 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 successfully internally develop this capability. capability successfully. We may enter into collaboration agreements collaborations regarding VYJUVEK or any of our future approved product candidates with third parties other entities to utilize their established marketing and distribution capabilities, but 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 products, product candidates, if approved, or we are unable to develop the necessary capabilities on our own, we will be unable to generate sufficient product revenues revenue to sustain our business. We compete with many companies that currently have extensive, experienced and well-funded medical affairs, marketing and sales operations to recruit, hire, train and retain marketing and sales personnel. We also face competition in our search for third parties to assist us with the sales and marketing efforts of our product candidates. Without an effective internal team or the support of a third-party to perform marketing and sales functions, we may be unable to compete successfully against these more established companies.

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 is that are approved but fails to achieve market acceptance among physicians, patients, or third-party payors, we will not be able to generate significant revenues 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 revenues revenue may be adversely impacted, and our business may suffer.

We have mainly focused focus our research and product development efforts to date on VYJUVEK genetic medicines for DEB. Our understanding patients with debilitating diseases. We base our market opportunity estimates on a variety of both factors, including our estimates of the number of people who have this disease, as well as these diseases, the potential scope of our approved product labels, the subset of people with this disease 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 estimates in published literature. These estimates many assumptions and may prove to be incorrect, and new studies may reduce the estimated incidence or prevalence of this disease. The number of patients these diseases. Estimating market opportunities can be particularly challenging for rare indications, such as the ones we currently address, 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 European Union United States and elsewhere internationally may turn out to be lower than expected, or these patients may not be otherwise amenable to treatment with VYJUVEK or any of our products that receive regulatory approval 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 or any of our products that receive regulatory approval 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 will likely 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 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 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, 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 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, 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 ours, as there is no body of established practices and precedents for these types of products. 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 and surgical procedures and other treatments, 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 VYJUVEK and our product candidates, if approved.

We Prior to receiving VYJUVEK, patients are required to undergo genetic testing, and we anticipate that prior to receiving certain gene therapies, 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.

Even if we obtain Increasing demand for compassionate use or expanded access of our unapproved therapies could negatively affect our reputation and maintain approval for harm our business.

We are developing our product candidates from for illnesses for which there are currently limited to no available therapeutic options. At least one other company has been the FDA, target of disruptive social media campaigns related to a request for access to unapproved drugs for patients with significant unmet medical need. If we may never obtain approval for them outside of the United States, which would limit experience a similar social media campaign regarding our market opportunities and adversely affect our business.

Approval of a product candidate in the United States by the FDA does decision to provide or 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 provide our product candidates outside of the United States will under an expanded access corporate policy, our reputation may be subject negatively affected, and our business may be harmed. Recent media attention 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. Approval procedures vary among jurisdictions and can involve requirements and administrative review periods different from, and more onerous than, those individual patients' expanded access requests has resulted in the United States, including additional preclinical studies 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 clinical trials. 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 many countries outside 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 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 safety profile of our product candidates if approved, is also subject we were to approval. Obtaining a Marketing Authorization Application from 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 European Commission following future need to restructure or pause ongoing compassionate use and/or expanded access programs in order to perform 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 controlled clinical trials or reporting as conditions of approval. Regulatory authorities in countries outside of the United States required for regulatory approval 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 successful commercialization 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.

Risks Related to Our Business Operations

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 financial 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. 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.

We may be 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 which 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 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 Patient Protection and Affordable Care Act ("PPACA") 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 prompt adverse publicity or other government payors that are false or fraudulent. The PPACA 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, Modifications to the HIPAA Privacy, Security, Enforcement, and Breach;
- Notification Rules under HITECH and the Genetic Information Nondiscrimination Act; Other modifications to HIPAA, published in January 2013, which imposes certain requirements relating to the privacy, security and transmission of individually identifiable health information without appropriate authorization by entities subject to the rule, such as health plans, health care clearinghouses and health care providers;
- 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 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 disruptions 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 HIPAA, 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 will 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. potential participants in such programs.

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 a healthcare company may run afoul of one or more of the requirements.

Risks Related to Our Intellectual Property

If we are unable to obtain and maintain adequate United States and foreign patent protection for VYJUVEK, our approved product and 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, that we initiate, 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 revenues, 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 United States 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 United States U.S. patent claim, there is no assurance that a court of competent jurisdiction would invalidate the claims of any such United States 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, we have otherwise 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 current good manufacturing practice, or CGMP, manufacturing facilities, ANCORIS and ASTRA;
- 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, 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 for VYJUVEK 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 commercialize 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 any clinical trials required for VYJUVEK outside the U.S.;
- the progress, timing, results, and costs of our current and planned clinical trials of B-VEC (in Japan), 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 our 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. Our other federal net operating loss carryforwards and 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.

As of December 31, 2023, 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 owned shares representing approximately 14% 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 expect 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, you a stockholder may not be able to sell your their common stock at or above the price that you they paid for it. The market price of our common stock may be influenced by many factors, including:

- our ability to successfully commercialize VYJUVEK 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;
- developments or disputes concerning patent applications, issued patents or other proprietary rights;
- 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 patents, litigation matters 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 and the “Summary Risk Factors” and “Item 1A. 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 Annual Report 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 Form 10-K 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 fiscal year ended December 31, 2022. 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 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 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. The following table summarizes information with respect to purchases of our equity securities during the three months ended March 31, 2024:

Period	Total number of shares (or units) purchased	Average price paid per share (or unit)	Total number of shares (or units) purchased as part of publicly announced plans or programs	Maximum number (or approximate dollar value) of shares (or units) that may yet be purchased under the plans or programs
January 1, 2024 - January 31, 2024	—		—	—
February 1, 2024 - February 28, 2024	7,677	$ 78.89	—	—
March 1, 2024 - March 31, 2024	—		—	—
Total	7,677 [(1)]	$ 78.89	—	—

(1) Represents shares withheld from employees for taxes resulting from the vesting of restricted stock awards.

ITEM 3. DEFAULTS UPON SENIOR SECURITIES

None.

ITEM 4. MINE SAFETY DISCLOSURES

Not applicable.

ITEM 5. OTHER INFORMATION

Insider Trading Arrangements

On August 29, 2023 During the three months ended March 31, 2024, Kathryn Romano, none of our Chief Accounting Officer, directors or officers (as that term is defined by the SEC in Rule 16a-1(f) under the Exchange Act) adopted or terminated 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 25,000 shares of our Common Stock until June 28, 2024. or a non-Rule 10b5-1 trading arrangement.

ITEM 6. EXHIBITS

Exhibit Number	
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 September 30, 2023 March 31, 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: November 6, 2023 May 6, 2024	By:	/s/ Krish S. Krishnan
		Krish S. Krishnan
		President and Chief Executive Officer
		(Principal executive officer)
Date: May 6, 2024	By:	/s/ Kathryn A. Romano
		Kathryn A. Romano
		Chief Accounting Officer
		(Principal financial and accounting officer)

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: November 6, 2023 May 6, 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: November 6, 2023 May 6, 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 September 30, 2023 March 31, 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: November 6, 2023 May 6, 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 September 30, 2023 March 31, 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: November 6, 2023 May 6, 2024

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