UNITED STATES
SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-Q

(Mark One)

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

For the quarterly period ended June 30, 2023

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 Market

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

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

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

Large accelerated filer	☒	Accelerated filer	☐
Non-accelerated filer	☐	Smaller reporting company	☐

Emerging growth company ☐

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

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

As of July 31, 2023, there were 27,994,177 shares of the registrant's common stock issued and outstanding.

Krystal Biotech, Inc.
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PART I. FINANCIAL INFORMATION
ITEM 1. FINANCIAL STATEMENTS

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

(In thousands, except share and per share data)	June 30, 2023	December 31, 2022
Assets		
Current assets		
Cash and cash equivalents	$ 275,875	$ 161,900
Short-term investments	201,642	217,271
Prepaid expenses and other current assets	6,487	4,608
Total current assets	484,004	383,779
Property and equipment, net	163,737	161,684
Long-term investments	28,410	4,621
Right-of-use assets	7,590	8,042
Other non-current assets	285	324
Total assets	$ 684,026	$ 558,450
Liabilities and Stockholders' Equity		
Current liabilities		
Accounts payable	$ 4,446	$ 3,981
Current portion of lease liability	1,529	1,561
Accrued expenses and other current liabilities	17,923	23,305
Total current liabilities	23,898	28,847
Lease liability	7,014	7,372
Total liabilities	30,912	36,219
Commitments and contingencies (Note 6)		
Stockholders' equity		
Common stock; $0.00001 par value; 80,000,000 shares authorized at June 30, 2023 and December 31, 2022; 27,974,916 shares issued and outstanding at June 30, 2023; and 25,763,743 shares issued and outstanding at December 31, 2022	—	—
Additional paid-in capital	1,012,616	803,718
Accumulated other comprehensive loss	(236)	(728)
Accumulated deficit	(359,266)	(280,759)
Total stockholders' equity	653,114	522,231
Total liabilities and stockholders' equity	$ 684,026	$ 558,450

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

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

(In thousands, except share and per share data)	Three Months Ended June 30, 2023	Three Months Ended June 30, 2022	Six Months Ended June 30, 2023	Six Months Ended June 30, 2022
Expenses				
Research and development	$ 12,144	$ 10,890	$ 24,432	$ 20,204
General and administrative	25,904	17,863	49,939	33,771
Litigation settlement	—	—	12,500	25,000
Total operating expenses	38,048	28,753	86,871	78,975
Loss from operations	(38,048)	(28,753)	(86,871)	(78,975)
Other Income				
Interest and other income, net	4,838	645	8,364	902
Net loss	$ (33,210)	$ (28,108)	(78,507)	(78,073)
Unrealized gain (loss) on available-for-sale securities and currency translation adjustment	(82)	(348)	492	(1,382)
Comprehensive loss	$ (33,292)	$ (28,456)	$ (78,015)	$ (79,455)
Net loss per common share: Basic and diluted	$ (1.25)	$ (1.10)	$ (3.00)	$ (3.08)
Weighted-average common shares outstanding: Basic and diluted	26,656,883	25,545,167	26,187,161	25,331,000

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

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

(In thousands, except shares)	Common Stock Shares	Common Stock Amount	Additional Paid-in Capital	Accumulated Other Comprehensive (Loss)	Accumulated Deficit	Total Stockholders' 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
Shares surrendered for taxes and forfeitures	—	—	—	—	—	—
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

(In thousands, except shares)	Common Stock Shares	Common Stock Amount	Additional Paid-in Capital	Accumulated Other Comprehensive (Loss)	Accumulated Deficit	Total Stockholders' 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 surrendered for taxes and forfeitures	(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

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

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

(In thousands)	Six Months Ended June 30, 2023	2022
Operating Activities		
Net loss	$ (78,507)	$ (78,073)
Adjustments to reconcile net loss to net cash used in operating activities		
Depreciation and amortization	1,743	1,991
Stock-based compensation expense	21,768	14,619
Other, net	(2,371)	(166)
Changes in operating assets and liabilities		
Prepaid expenses and other current assets	(1,524)	848
Other non-current assets	110	15
Lease liability	(380)	(286)
Accounts payable	481	24
Accrued expenses and other current liabilities	(1,666)	2,476
Net cash (used in) operating activities	(60,346)	(58,552)
Investing Activities		
Purchases of property and equipment	(8,171)	(33,706)
Purchases of investments	(319,969)	(147,255)
Proceeds from maturities of investments	315,746	86,829
Net cash used in investing activities	(12,394)	(94,132)
Financing Activities		
Issuance of common stock, net	187,492	30,807
Taxes paid related to settlement of restricted stock awards	(749)	(649)
Net cash provided by financing activities	186,743	30,158
Effect of exchange rate changes on cash and cash equivalents	(28)	—
Net increase (decrease) in cash and cash equivalents	113,975	(122,526)
Cash and cash equivalents at beginning of period	161,900	341,246
Cash and cash equivalents at end of period	$ 275,875	$ 218,720
Supplemental Disclosures of Non-Cash Investing and Financing Activities		
Unpaid purchases of property and equipment included in accounts payable and accrued expenses	$ 10,998	$ 22,234
Initial recognition of right-of-use assets	$ —	$ 1,394

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, and December 2022, the Company incorporated wholly-owned subsidiaries in Switzerland, Netherlands, and France, respectively, for the purpose of establishing initial operations in Europe for the development and commercialization of Krystal's product pipeline.

We are a commercial-stage biotechnology company focused on the discovery, development and commercialization of genetic medicines to treat diseases with high unmet medical needs. Our approach leverages our patented platform that is based on engineered Herpes Simplex Virus-1 (“HSV-1”) vector to 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 vectors 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") manufacturing facilities.

On May 19, 2023, the Company received U.S. Food and Drug Administration (“FDA”) approval for its first candidate, 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. VYJUVEK became commercially available upon approval, and the Company expects to begin generating revenue from VYJUVEK product sales in 3Q 2023.

Liquidity

As of June 30, 2023, the Company had an accumulated deficit of $359.3 million. As the Company continues to incur losses, a transition to profitability is dependent upon the successful commercialization of VYJUVEK as well as successful development, approval, and commercialization of its other product candidates and the achievement of a level of revenues adequate to support the Company’s cost structure. The Company may never achieve profitability and unless and until it does, the Company will continue to need to raise additional capital or obtain financing from other sources. Management intends to fund future operations through its on hand cash and cash equivalents, the sale of equity, debt financings, and may also seek additional capital through arrangements with strategic partners 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 expand its commercialization capabilities in advance of the potential global regulatory approvals of its lead product, VYJUVEK. The Company believes that its cash, cash equivalents and short-term investments of approximately $477.5 million as of June 30, 2023 will be sufficient to allow the Company to fund its planned operations for at least the next 12 months from the date of this Quarterly Report on Form 10-Q.

2. Summary of Significant Accounting Policies

Basis of Presentation

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

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, as filed with the U.S. Securities and Exchange Commission ("SEC") on February 27, 2023.

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. Estimates are used in the following areas: stock-based compensation expense, accrued expenses, 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.

Concentrations of Credit Risk and Off-Balance Sheet Risk

Financial instruments that potentially subject the Company to credit risk consist of cash, cash equivalents and investments. The Company's policy is to invest its cash, cash equivalents and investments in money market funds, corporate bonds, commercial paper, U.S. government agency securities and various other bank deposit accounts. The counterparties to the agreements relating to the Company's investments consist of financial institutions of high credit standing. 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 has no financial instruments with off-balance sheet risk of loss.

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 consolidated statements of operations.

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, 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, which consist of commercial paper, corporate bonds, and U.S. government agency securities are considered to be Level 2 financial instruments. The fair value of Level 2 financial assets is determined using inputs that are observable in the market or can be derived principally from or corroborated by observable market data, such as pricing for similar securities, recently executed transactions, cash flow models with yield curves, and benchmark securities. In addition, Level 2 financial instruments are valued using comparisons to like-kind financial instruments and models that use readily observable market data as their basis.

Inventories

The Company capitalizes inventory costs associated with products when future commercialization is considered probable, and a future economic benefit is expected to be realized. These costs consist of raw materials, manufacturing-related costs, personnel costs including stock-based compensation, facility costs, transportation and freight, and other indirect overhead costs. Prior to receiving FDA approval for VYJUVEK in May 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, using the 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 six months ended June 30, 2023 and 2022, there were no inventory impairment adjustments. As of June 30, 2023, the Company recorded $1.1 million of inventory consisting of raw materials and work-in-process within prepaid expense and other current assets 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 from the accounts 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 from the estimated undiscounted future cash flows expected to result from its use and eventual disposition. 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 six months ended June 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 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.

For lease arrangements where it has been determined that the Company has control over an asset that is under construction and is thus considered the accounting owner of the asset during the construction period, the Company records a construction in progress asset and corresponding financial obligation on the condensed consolidated balance sheet. Once the construction is complete, an assessment is performed to determine whether the lease meets certain sale-leaseback criteria. If the sale-leaseback criteria are determined to be met, the Company will remove the asset and related financial obligation from the condensed consolidated balance sheet and treat the lease as either an operating or finance lease based on an assessment of the guidance. If, upon completion of construction, the project does not meet the "sale-leaseback" criteria, the lease will be treated as a financing obligation and the Company will depreciate the asset over its estimated useful life for financial reporting purposes once the asset has been placed into service.

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. 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 Loss

Comprehensive 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 is a component of other comprehensive gains or losses and is 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 during the three and six months ended June 30, 2023 and 2022.

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 the Company's condensed consolidated financial statements, and no recently issued accounting pronouncements that are expected to have a material impact on the Company's condensed consolidated financial statements.

3. Net Loss Per Share Attributable to Common Stockholders

Basic net loss per share attributable to common stockholders is calculated by dividing net loss attributable to common stockholders by the weighted average shares outstanding during the period, without consideration for common stock equivalents. Diluted net loss per share attributable to common stockholders is computed by dividing the net loss by the weighted-average number of shares of common stock and common share equivalents outstanding for the period. Common share equivalents consist of common stock issuable upon exercise of stock options and vesting of restricted stock awards. There were 3,274,066 and 3,686,862 common share equivalents outstanding as of June 30, 2023 and 2022, respectively, in the form of stock options and unvested restricted stock awards, that have been excluded from the calculation of diluted net loss per common share as their effect would be anti-dilutive for all periods presented.

	Three Months Ended June 30,		Six Months Ended June 30,	
(In thousands, except share and per share data)	**2023**	**2022**	**2023**	**2022**
Numerator:				
Net loss	$ (33,210)	$ (28,108)	$ (78,507)	$ (78,073)
Denominator:				
Weighted-average basic and diluted common shares	26,656,883	25,545,167	26,187,161	25,331,000
Basic and diluted net loss per common share	$ (1.25)	$ (1.10)	$ (3.00)	$ (3.08)

4. Fair Value Instruments

The following tables show the Company's cash, cash equivalents and available-for-sale securities by significant investment category as of June 30, 2023 and December 31, 2022, respectively (in thousands):

	June 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]
Level 1:							
Cash and cash equivalents	$ 275,875	$ —	$ —	$ 275,875	$ 275,875	$ —	$ —
Subtotal	275,875	—	—	275,875	275,875	—	—
Level 2:							
Commercial paper	62,089	18	(21)	62,086	—	62,086	—
Corporate bonds	58,880	47	(122)	58,805	—	47,716	11,089
U.S. government agency securities	109,268	179	(286)	109,161	—	91,840	17,321
Subtotal	230,237	244	(429)	230,052	—	201,642	28,410
Total	$ 506,112	$ 244	$ (429)	$ 505,927	$ 275,875	$ 201,642	$ 28,410

	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]
Level 1:							
Cash and cash equivalents	$ 161,900	$ —	$ —	$ 161,900	$ 161,900	$ —	$ —
Subtotal	161,900	—	—	161,900	161,900	—	—
Level 2:							
Commercial paper	63,624	5	(23)	63,606	—	63,606	—
Corporate bonds	82,241	13	(419)	81,835	—	77,214	4,621
U.S. government agency securities	76,683	161	(393)	76,451	—	76,451	—
Subtotal	222,548	179	(835)	221,892	—	217,271	4,621
Total	$ 384,448	$ 179	$ (835)	$ 383,792	$ 161,900	$ 217,271	$ 4,621

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

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

5. Balance Sheet Components

Property and Equipment, Net

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

	June 30, 2023	December 31, 2022
Construction in progress	$ 97,759	$ 131,331
Building and building improvements	33,984	—
Leasehold improvements	24,597	24,217
Manufacturing equipment	12,323	9,783
Laboratory equipment	2,285	2,089
Furniture and fixtures	1,460	957
Computer equipment and software	473	100
Total property and equipment	172,881	168,477
Accumulated depreciation	(9,144)	(6,793)
Property and equipment, net	$ 163,737	$ 161,684

Depreciation expense was $1.2 million and $2.3 million for the three and six months ended June 30, 2023 and $494 thousand and $956 thousand for the three and six months ended June 30, 2022, respectively.

On March 27, 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 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 June 30, 2023 as it was determined that additional assets were ready for their intended use. As certain building improvements are not yet complete and certain qualification activities are still underway, the Company will continue to hold the remaining assets within construction in progress until validation has been completed and the assets are ready for their intended use. Validation of the facility is expected to be completed in 2023.

Accrued Expenses and Other Current Liabilities

Accrued expenses and other current liabilities consisted of the following (in thousands):

	June 30, 2023	December 31, 2022
Accrued construction in progress	7,518	11,452
Accrued payroll and benefits	4,428	6,781
Accrued professional fees	3,721	3,397
Accrued preclinical and clinical expenses	1,582	1,365
Other current liabilities	652	267
Accrued taxes	22	43
Total	$ 17,923	$ 23,305

6. Commitments and Contingencies

Agreements with Contract Research Organizations and Contract Manufacturing Organizations

The Company enters into various agreements in the normal course of business with Contract Research Organizations ("CROs"), Contract Manufacturing Organizations ("CMOs") and other third parties for preclinical research studies, clinical trials and testing and manufacturing services. The agreements with CMOs primarily relate to the manufacturing of our 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 preclinical 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 commitment as of June 30, 2023 under these agreements is approximately $3.0 million. The Company has incurred research and development expenses under these agreements of $1.1 million and $3.1 million for the three and six months ended June 30, 2023 and $1.2 million and $3.0 million for the three and six ended June 30, 2022, respectively.

ASTRA Contractual Obligations

The Company has contracted with various third parties to complete the interior build-out of 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 June 30, 2023 is $10.6 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 June 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.

Legal Proceedings

In May 2020, a complaint was filed against the Company in the United States District Court for the Western District of Pennsylvania by PeriphaGen, Inc. ("PeriphaGen") alleging breach of contract and misappropriation of trade secrets. On April 27, 2022, the Company and PeriphaGen entered into a final settlement agreement, and the Company paid PeriphaGen an upfront payment of $25.0 million on April 28, 2022 for: (i) the release of all claims in the 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 Company 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 the settlement payments of zero and $12.5 million for the three and six months ended June 30, 2023, respectively, and zero and $25.0 million for the three and six months ended June 30, 2022, respectively, under litigation settlement expense on the condensed consolidated statements of operations for the six months ended June 30, 2023 and June 30, 2022. In accordance with ASC 450, as of the June 30, 2023, Company has not recorded an accrual for the remaining contingent milestone payments.

The Company did not receive insurance proceeds during the three and six months ended June 30, 2023 and received zero and $768 thousand during the three and six months ended June 30, 2022, respectively. The reimbursements have been recorded as an offset to our legal fees included in general and administrative expenses on the condensed consolidated statements of operations and within operating activities on the condensed consolidated statements of cash flows.

7. Leases

As of June 30, 2023, 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 six months)	$ 828
2024	1,539
2025	1,277
2026	1,277
2027	1,300
Thereafter	10,763
Future minimum operating lease payments	$ 16,984
Less: Interest	(8,441)
Present value of lease liability	$ 8,543

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

	June 30, 2023	December 31, 2022
Operating leases:		
Right-of-use assets	$ 7,590	$ 8,042
Current portion of lease liability	1,529	1,561
Lease liability	7,014	7,372
Total lease liability	$ 8,543	$ 8,933
Weighted average remaining lease term, in years	12.3	12.5
Weighted average discount rate	9.4 %	9.4 %

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2023	2022	2023	2022
Lease cost:				
Operating lease expense	$ 440	$ 391	$ 902	$ 800
Variable lease expense	29	71	88	120
Total lease expense	$ 469	$ 462	$ 990	$ 920

8. Capitalization

ATM Program

On December 31, 2020, the Company entered into a sales agreement with Cowen and Company, LLC ("Cowen") with respect to an at-the-market equity offering program ("2020 ATM 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 per share, having an aggregate offering price up to $150.0 million ("Placement Shares"). The issuance and sale of the Placement Shares were made pursuant to the Company's effective "shelf" registration statement on Form S-3 that was filed with the Securities and Exchange Commission (the “SEC”) on May 4, 2020 (the “2020 Shelf Registration Statement”). During the six months ended June 30, 2022, 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.

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 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 having an aggregate offering price of up to $150.0 million (the “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

ended June 30, 2023, no shares of 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.

9. Stock-Based Compensation

In 2017, the Company adopted the 2017 IPO Stock Plan (the "Plan"), which governs the issuance of stock options and restricted stock 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 to its employees. In February 2023, the Company began issuing restricted stock units and performance-based restricted stock units to certain employees.

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 101,680 and 389,280 stock options to employees, non-employees, and directors of the Company during the three and six months ended June 30, 2023 and 589,500 and 1,769,000 to employees, non-employees, and directors of the Company during the three and six months ended June 30, 2022, respectively.

The following table summarizes the Company's stock option activity:

	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	$ 61.15	8.7	$ 64,880
Granted	389,280	$ 88.74		
Exercised	(490,995)	$ 56.41		
Cancelled or forfeited	(250,800)	$ 63.14		
Expired	—	$ —		
Outstanding at June 30, 2023	3,229,666	$ 65.04	8.4	$ 169,121
Exercisable at June 30, 2023	778,737	$ 55.83	7.5	$ 47,952

(1) Aggregate intrinsic value represents the difference between the closing stock price of our common stock on June 30, 2023 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 during the three and six months ended June 30, 2023 was $26.7 million and $27.9 million, respectively, and during the three and six months ended June 30, 2022 was $704 thousand and $739 thousand, respectively.

The weighted-average grant-date fair value per share of options granted to employees, non-employees, and directors during the three and six months ended June 30, 2023 was $72.95 and $61.06, respectively, and during the three and six months ended June 30, 2022 was $42.90 and $43.03, respectively.

There was $98.2 million of unrecognized stock-based compensation expense related to employees', non-employees', and directors' option awards that is expected to be recognized over a weighted-average period of 2.6 years as of June 30, 2023.

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 six months ended June 30, 2023 and 2022 as follows (in thousands):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2023	2022	2023	2022
Research and development	$ 2,472	$ 1,995	$ 4,825	$ 3,363
General and administrative	6,978	5,776	14,086	10,357
Total stock-based compensation	$ 9,450	$ 7,771	$ 18,911	$ 13,720

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 six months ended June 30, 2023 and 2022:

	Three Months Ended June 30,		Six Months Ended June 30,	
	2023	2022	2023	2022
Expected stock price volatility	75 %	78 %	75 %	78 %
Expected term of the award (years)	5.9	6.2	6.1	6.2
Risk-free interest rate	3.84 %	2.92 %	4.00 %	2.17 %
Weighted average exercise price	$ 108.30	$ 61.51	$ 88.74	$ 62.19
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 the Company during each of the three and six months ended June 30, 2023 and June 30, 2022.

	Number of Shares	Weighted Averag Grant Date Fair Value
Non-vested RSAs as of December 31, 2022	66,600	$ 7
Granted	—	$
Vested	(12,649)	$ 7
Surrendered for taxes	(9,551)	$ 7
Non-vested RSAs as of June 30, 2023	$ 44,400	$ 7

There was $2.9 million of unrecognized stock-based compensation expense related to employees' RSAs that is expected to be recognized over a weighted-average period of 1.7 years as of June 30, 2023.

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2023	2022	2023	2022
General and administrative	$ 436	$ 418	$ 868	$
Total stock-based compensation	$ 436	$ 418	$ 868	$

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 the Company during the three and six months ended June 30, 2023, and zero RSUs during the three and six months ended June 30, 2022, respectively.

	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	(14,200)	$ 81.91
Non-vested RSUs as of June 30, 2023	172,700	$ 81.91

There was $13.0 million of unrecognized stock-based compensation expense related to employees' RSU awards that is expected to be recognized over a weighted-average period of 3.7 years as of June 30, 2023.

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2023	2022	2023	2022
Research and Development	$ 391	$ —	$ 534	$
General and administrative	441	—	627	
Total stock-based compensation	$ 832	$ —	$ 1,161	$

Performance-Based Restricted Stock Units

Performance-based restricted stock units ("PSUs") granted to employees 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 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 June 30, 2023, the Company estimates that 100% of the PSUs granted will be eligible to vest.

The Company granted zero and 60,000 PSUs to employees of the Company during the three and six months ended June 30, 2023 and zero PSUs during the three and six months ended June 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	—	
Non-vested PSUs as of June 30, 2023	60,000	$ 81.91

There was $4.1 million of unrecognized stock-based compensation expense related to employees' PSU awards that is expected to be recognized over a weighted-average period of 1.7 years as of June 30, 2023.

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2023	2022	2023	2022
General and administrative	$ 613	$ —	$ 828	$
Total stock-based compensation	$ 613	$ —	$ 828	$

Shares remaining available for grant under the Plan were 1,126,321, with a sublimit for incentive stock options of 4,282, at June 30, 2023.

Following 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 six months ended June 30, 2023, the Company capitalized $112 thousand in prepaid expenses and other current assets.

Historically, the Company capitalized the portion of stock-based compensation related to work performed on the construction of manufacturing facilities. There was zero and $162 thousand and of stock-based compensation that was capitalized in property and equipment during the three and six months ended June 30, 2023 and $146 thousand and $287 thousand capitalized during the three and six months ended June 30, 2022, respectively.

10. 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, other than noted below, that would require recognition or disclosure in the condensed consolidated financial statements.

Andrew Orth, the Company's Chief Commercial Officer, notified the Company that he was resigning from his position and his last day with the Company was August 2, 2023.

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, as filed with the SEC on February 27, 2023.

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 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") and other regulatory approval of our product candidates;
- our ability to achieve certain accelerated or orphan drug designations from the FDA;
- 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;
- our ability to raise capital to fund our operations;
- increases in costs associated with our research and development programs for our product candidates;
- increases in our general and administrative expenses;
- risks related to our ability to successfully develop and commercialize our product candidates;
- our ability to identify and develop 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 cost of building a medical affairs and commercial organization, including a sales force in anticipation of commercialization of any of our product candidates;
- 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 financial performance;
- our ability to establish and maintain collaborations;
- our estimates regarding expenses, future revenue, capital requirements and needs for additional financing;
- 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, (iii) Exhibit 99.2 to our Form 8-K filed with the Securities and Exchange Commission ("SEC") on May 19, 2023, and (iv) 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.

Except as required by law, we assume no obligation to update these forward-looking statements publicly, or to update the reasons actual results could differ materially from those anticipated in these forward-looking statements, even if new information becomes available in the future.

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

Overview

We are a commercial-stage biotechnology company focused on the discovery, development and commercialization of genetic medicines to treat diseases with high unmet medical needs. Our approach leverages our patented platform that is based on engineered Herpes Simplex Virus-1 ("HSV-1") vector to 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 vectors 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 manufacturing facilities.

Our US FDA Approved Product

VYJUVEK

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

Commercialization in the US began immediately following FDA approval, including: (i) accepting Patient Start Forms; (ii) efforts to support physician education and utilization; and (iii) facilitating patients' access to therapy through Krystal Connect™, our personalized support program. Through Krystal Connect, we have been accepting Patient Start Forms submitted by healthcare professionals, and we are working with payers for coverage authorization by way of prior authorizations and medical exceptions. As of June 30, 2023, the Company received 121 Patient Start Forms of which 30 Patient Start Forms were generated for patients with dominant DEB. Going forward, we intend to report on the number of Patient Start Forms submitted to Krystal Connect in our quarterly reports for the first three quarters following VYJUVEK approval and then transition to reporting the number of Patients on Therapy starting in Q1 2024.

In July 2023, we received a positive opinion from the European Medicines Agency (the "EMA") Pediatric Committee on the Pediatric Investigation Plan for B-VEC for the treatment of DEB with no additional studies required. We plan to submit a market authorization application to the EMA in the second half of 2023 and anticipate a potential launch in the European Union in the second half of 2024.

In July 2023, the Pharmaceuticals and Medical Agency in Japan officially accepted the open label extension study of B-VEC, and we intend to start an open label extension study of B-VEC in Japan in the second half of 2023 and file for approval in Japan in 2024.

Pipeline Highlights and Recent Developments

Respiratory

KB407 is an inhaled (nebulized) formulation of our novel vector designed to deliver two copies of the full-length cystic fibrosis transmembrane conductance regulator ("CFTR") transgene for the treatment of Cystic Fibrosis ("CF"), a serious rare lung disease caused by missing or mutated CFTR protein. On July 3, 2023, we announced that we dosed the first patient in a Phase 1 clinical trial ("CORAL-1/US") of KB407 for the treatment of CF, regardless of a patient's underlying genotype, We anticipate announcing data from the Phase 1 study in 2024. Details of the Phase 1 study can be found at www.clinicaltrials.gov under NCT identifier NCT05504837. We faced pandemic, logistical and recruiting challenges with respect to our intended Phase 1 clinical study in Australia (CORAL-AU) and, as we were successful in initiating our CORAL-1/US study prior to the initiation of our CORAL-AU study, we have terminated our study in Australia to focus on completing the Phase 1 study entirely in the US. The Company is using the Aerogen Solo® Nebulizer System and Aerogen® Ultra in its Phase 1 CORAL-1/US study evaluating KB407.

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"). We presented preclinical pharmacology data for KB408 at the European Society of Gene & Cell Therapy Virtual Congress that was held October 19-22, 2021. We are planning to file an Investigational New Drug ("IND") application for KB408 to treat AATD patients in the second half of 2023.

Oncology

On July 26, 2023, we announced that we expanded 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 designed to deliver genes encoding both human IL-12 and IL-2 to the tumor microenvironment and promote systemic immune-mediated tumor clearance. KB707 targets solid tumors that are accessible via intratumoral injection or inhalation, and we intend to advance both routes of administration into clinical studies.

The FDA has accepted our IND application to evaluate 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 the treatment of patients with anti-PD-1 relapsed/refractory locally advanced or metastatic melanoma. Details of the Phase 1 study can be found www.clinicaltrials.gov under NCT identifier NCT05970497. We expect to dose our first patient in the second half of 2023. We plan to file an amendment to our existing KB707 IND in the second half of 2023 to allow us to evaluate inhaled (nebulized) KB707 in a clinical trial to treat tumors in a patient's lungs. We expect to dose our first patient with inhaled KB707 in the first half of 2024.

Dermatology

KB105 is a topical gel containing our novel vector designed to deliver two copies of the *TGM1* transgene for the treatment of TGM1-ARCI, a serious rare skin disorder caused by missing or mutated TGM1 protein. In July 2021, we announced complete data from the Phase 1/2 trial, a randomized placebo-controlled study, showing repeat topical KB105 dosing continued to be well tolerated with no adverse events or evidence of immune response. Details of the Phase 1/2 study can be found at www.clinicaltrials.gov under NCT identifier NCT04047732. We are working with the FDA to ensure that we are aligned on the clinical endpoints in the pivotal trial prior to initiating a Phase 2 study in pediatric patients. As such, we now intend to commence the KB105 Phase 2 study in 2024.

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. The FDA has granted KB104 rare pediatric designation for the treatment of Netherton Syndrome. As we continually evaluate the priority of our expanding pipeline portfolio, we now anticipate filing an IND application and initiating a clinical trial of KB104 to treat patients with Netherton Syndrome in late 2024.

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 treated the first subject in the Phase 1, Cohort 3 study of KB301 for the improvement of lateral canthal lines at rest. The Phase 1, Cohort 3 study is being conducted at a single center as an open label study to evaluate two different doses of KB301 in up to 20 subjects. Improvement of lateral canthal lines at rest ("LCL") 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, and Jeune plans to announce results from this study in the second half of 2023. Following completion of this study, Jeune plans to initiate a Phase 2 study of KB301 in LCL. Details of the Phase 1 study can be found at www.clinicaltrials.gov under NCT identifier (NCT04540900).

Jeune has several other aesthetic genetic medicine product candidates in various stages of preclinical development.

Business Highlights and Recent Developments

In May 2023, the Company entered into a securities purchase agreement for the sale of 1,729,729 shares of its common stock at $92.50 per share in a private placement (the "PIPE") to certain qualified institutional buyers. The PIPE financing was led by Avoro Capital Advisors and Redmile Group, LLC with participation from Braidwell LP and Frazier Life Sciences. Net proceeds from the PIPE were $160.0 million.

COVID-19 Update

To date the impact of the COVID-19 pandemic on our business and clinical trials in the U.S. has been minimal. Outside of the U.S., we experienced pandemic-related delays in clinical trial initiation in Australia. We will closely monitor any potential impact that future public health crises may have on our clinical trials. For additional information, please see "The effect of the COVID-19 pandemic or similar public health crises on our operations and the operations of our third-party partners could cause a disruption of the development efforts for our product candidates and adversely impact our business" in Item 1A. Risk Factors" in our Annual Report on Form 10-K for the fiscal year ended December 31, 2022, as filed with the SEC on February 27, 2023.

Financial Overview

Revenue

On May 19, 2023, we received FDA approval for VYJUVEK for the treatment of DEB in patients six months or older. We have not generated any revenue from the sale of products or other sources as of June 30, 2023. VYJUVEK became commercially available upon approval and we expect to begin generating revenue from VYJUVEK product sales in 3Q 2023. Our future revenue will fluctuate from quarter to quarter for many reasons, including the uncertain timing and amount of any such sales.

Cost of Goods Sold

As noted above, we expect to generate revenue and cost of goods sold for VYJUVEK product sales in 3Q 2023. Due to the timing of FDA approval and the Company's manufacturing schedule, a majority of the costs associated with VYJUVEK inventory manufactured for the commercial launch was previously expensed as research and development. The Company anticipates that the previously expensed inventory will favorably impact the Company's gross margin. The Company is still evaluating the impact of previously expensed inventories on future costs of goods sold.

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 expand our product portfolio. In the near term, we expect that our research and development expenses will increase as we resume dosing with KB105 Phase 1/2 clinical trial, continue the Phase 1, Cohort 3 study and initiate a Phase 2 trial for KB301, continue the Phase 1 trial for KB407, initiate a Phase 1 trial for KB707, initiate a Phase 1 trial for KB104, 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.

General and Administrative Expenses

General and administrative expenses consist principally of salaries and other related costs, including stock-based compensation for personnel in our executive, commercial, business development and other administrative functions. General and administrative expenses also include professional fees associated with corporate and intellectual property-related legal expenses, consulting and accounting services, facility-related costs and expenses associated with obtaining and maintaining patents. Other general and administrative costs include travel expenses.

We anticipate that our general and administrative expenses will increase in the future to support the continued research and development of our product candidates and our commercial and operational goals. 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 preparation for commercial operations.

ASTRA Capital Expenditures

In March 2021, we closed on the purchase of the building that was constructed to house our second CGMP facility, ASTRA and we are currently in the process of completing the interior build-out of this facility.. The Company placed a portion

of ASTRA into service during the three and six months ended June 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 of the building. As certain building improvements and certain qualification activities are still underway, the Company will continue to hold the remaining assets within construction in progress until validation has been completed and the assets are ready for their intended use. Validation of the facility is expected to be completed in 2023.

Interest and Other Income

Interest and other income 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 and six months ended June 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 June 30, 2023 and 2022

	Three Months Ended June 30,		
	2023	2022	Change
(In thousands)	**(unaudited)**		
Expenses			
Research and development	$ 12,144	$ 10,890	$ 1,254
General and administrative	25,904	17,863	8,041
Total operating expenses	38,048	28,753	9,295
Loss from operations	(38,048)	(28,753)	(9,295)
Other Income			
Interest and other income, net	4,838	645	4,193
Net loss	$ (33,210)	$ (28,108)	$ (5,102)

Research and Development Expenses

Research and development expenses increased $1.3 million in the three months ended June 30, 2023 compared to the three months ended June 30, 2022. The increase was primarily due to increased payroll related expenses of $2.3 million, which were primarily driven by an increase in headcount to support overall growth, and includes a $868 thousand increase in stock-based compensation, an increase in depreciation of $644 thousand, and increased other research and development expenses of $188 thousand, which consisted of increased facilities and license and regulatory fees, offset by decreased outsourced research and development costs. The increase was partially offset by decreases in preclinical, clinical and pre-commercial manufacturing expenses of $1.8 million, due to the costs related to the manufacturing of VYJUVEK following FDA approval being recorded as inventory, and fewer receipts of raw materials and lab supplies period over period that were purchased for planned manufacturing runs of the Company's products.

Research and development expenses consist primarily of costs relating to the preclinical and clinical development of our product candidates and preclinical 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 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 expense by product candidate or program, and for unallocated expenses, by type, for the three months ended June 30, 2023 and 2022:

	Three Months Ended June 30,		
(in thousands)	**2023**	**2022**	**Change**
VYJUVEK	$ 2,130	$ 1,730	$ 400
KB105	10	90	(80)
KB407	442	535	(93)
KB301	77	160	(83)
KB707	943	—	943
Other dermatology programs	6	68	(62)
Other respiratory programs	309	535	(226)
Other aesthetics programs	1	57	(56)
Other research programs	131	233	(102)
Other development programs	194	141	53
Stock-based compensation	2,863	1,995	868
Other unallocated manufacturing expenses[(1)]	3,536	4,369	(833)
Other unallocated expenses[(2)]	1,502	977	525
Research and development expense	$ 12,144	$ 10,890	$ 1,254

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

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

As noted above, research and development expense increased $1.3 million in the three months ended June 30, 2023 compared to the three months ended June 30, 2022. Expenses for VYJUVEK increased $400 thousand, due to increased OLE clinical trial costs, license and regulatory costs and increased allocated research and development expenses. Spending on KB707 programs increased by $943 thousand due primarily to increased internal resources and other payroll related costs to support continued research and increases in contract research expenses. Stock-based compensation increased $868 thousand due to an increase in internal resources to support overall research and development growth. Additionally, other unallocated expenses increased $525 thousand primarily related to increases in depreciation expense. These increases were offset by a decrease in other unallocated manufacturing expenses of $833 thousand due to the costs related to the manufacturing of VYJUVEK following FDA approval being recorded as inventory and fewer receipts of raw materials period over period that were purchased for planned manufacturing runs of the Company's products.

General and Administrative Expenses

General and administrative expenses increased $8.0 million in the three months ended June 30, 2023 as compared to the three months ended June 30, 2022. Higher general and administrative spending was due largely to increases in payroll related expenses of approximately $5.9 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 $2.3 million increase in stock-based compensation, increased legal and professional costs of $525 thousand, an increase in costs in costs due to preparation for commercialization and launch of VYJUVEK including increased information technology infrastructure costs of $643 thousand, increased software-related costs of $411 thousand, increased marketing costs of $404 thousand, and increased travel related costs of $274 thousand, and an increase in other general and administrative expenses of $165 thousand. These increases were partially offset by a decreased business development costs of $307 thousand.

Interest and Other Income

Interest and other income for the three months ended June 30, 2023 and 2022 was $4.8 million and $645 thousand, 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.

Six Months Ended June 30, 2023 and 2022

(In thousands)	Six Months Ended June 30, 2023	2022	Change
	(unaudited)		
Expenses			
Research and development	$ 24,432	$ 20,204	$ 4,228
General and administrative	49,939	33,771	16,168
Litigation settlement	12,500	25,000	(12,500)
Total operating expenses	86,871	78,975	7,896
Loss from operations	(86,871)	(78,975)	(7,896)
Other Income			
Interest and other income, net	8,364	902	7,462
Net loss	$ (78,507)	$ (78,073)	$ (434)

Research and Development Expenses

Research and development expenses increased $4.2 million in the six months ended June 30, 2023 compared to the six months ended June 30, 2022. The increase was primarily due to increased payroll related expenses of $4.7 million, which were primarily driven by an increase in headcount to support overall growth, and includes a $2.0 million increase in stock-based compensation, an increase in depreciation of $1.3 million, and increased other research and development expenses of $1.1 million, primarily due to facilities expenses and licenses and regulatory fees. These increases were partially offset by decreases in preclinical, clinical and pre-commercial manufacturing expenses of $2.9 million, due to the costs related to the manufacturing of VYJUVEK following FDA approval being recorded as inventory and and due fewer receipts of raw materials and lab supplies period over period that were purchased for planned manufacturing runs of the Company's products.

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

(in thousands)	Six Months Ended June 30, 2023	2022	Change
VYJUVEK	4,520	3,332	$ 1,188
KB105	243	106	137
KB407	819	931	(112)
KB301	329	382	(53)
KB707	1,408	—	1,408
Other dermatology programs	14	67	(53)
Other respiratory programs	419	397	22
Other aesthetics programs	14	67	(53)
Other research programs	258	440	(182)
Other development programs	529	286	243
Stock-based compensation	5,359	3,363	1,996
Other unallocated manufacturing expenses[(1)]	7,456	8,972	(1,516)
Other unallocated expenses[(2)]	3,064	1,861	1,203
Research and development expense	$ 24,432	$ 20,204	$ 4,228

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

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

As noted above, research and development expense increased $4.2 million in the six months ended June 30, 2023 compared to the six months ended June 30, 2022. Expenses for VYJUVEK increased $1.2 million, due to increased payroll related expenses to support pre-approval activities, clinical trial costs, license and regulatory costs and increased allocated research and development expenses. Due to the expansion of our research and development pipeline to oncology, KB707 spending increased $1.4 million primarily due to increased payroll related costs and increased contract research costs. Stock-based compensation increased $2.0 million due to an increase in internal resources to support overall research and development growth. Additionally, other unallocated expenses increased $1.2 million primarily related to increases in depreciation expense. These increases were offset by a decrease in other unallocated manufacturing expenses of $1.5 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 the Company's products.

General and Administrative Expenses

General and administrative expenses increased $16.2 million in the six months ended June 30, 2023 as compared to the six months ended June 30, 2022. Higher general and administrative spending was due largely to increases in payroll related expenses of approximately $12.9 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 $5.2 million increase in stock-based compensation, an increase in costs due to preparation for commercialization and launch of VYJUVEK including increased marketing costs of $1.3 million, increased information technology infrastructure costs of $1.1 million, increased software-related costs of $664 thousand, increased travel related costs of $505 thousand, and an increase in other general and administrative expenses of $557 thousand, which consisted primarily of increased conference costs and utilities costs. These increases were partially offset by decreases in business development costs of $517 thousand and medical affairs costs of $397 thousand.

Litigation Settlement

Litigation settlement for the six months ended June 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 6 of the notes to condensed consolidated financial statements included in this Form 10-Q for more information.

Interest and Other Income

Interest and other income for the six months ended June 30, 2023 and 2022 was $8.4 million and $902 thousand, 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.

Liquidity and Capital Resources

Overview

At June 30, 2023, our cash, cash equivalents and short-term investments balance was approximately $477.5 million. Since operations began, we have incurred operating losses. Our net losses were $33.2 million and $78.5 million for the three and six months ended June 30, 2023, respectively, and $28.1 million and $78.1 million for the three and six months ended June 30, 2022, respectively. At June 30, 2023, we had an accumulated deficit of $359.3 million. We believe that our cash, cash equivalents and short-term investments as of June 30, 2023 will be sufficient to allow us to fund operations for at least 12 months from the filing date of this Quarterly Report on Form 10-Q.

As we continue to incur losses, a transition to profitability is dependent upon the successful development, approval and commercialization of our product candidates and the achievement of a level of revenues adequate to support our cost structure. Furthermore, we expect to incur increasing costs associated with satisfying regulatory and quality standards, maintaining product and clinical trials, and furthering our efforts around our current and future product candidates. We may never achieve profitability, and unless and until we do, we will continue to need to raise additional capital 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 commercialization of VYJUVEK, our continued clinical studies of KB105, KB407, KB301 or our planned clinical and preclinical studies for our other product candidates, or our operations. Further, we do not expect to generate any product revenues until the third quarter of 2023, at the earliest. 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 $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 launch of VYJUVEK, KB105, KB407, KB301, KB707 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, third-party clinical trial research and development services, laboratory and related supplies, pre-commercialization costs, legal and other regulatory 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 and commercialization of pharmaceutical products, 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 cost of our OLE study for 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 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 we may develop, including the costs and timing of establishing product sales, medical affairs, marketing, distribution and manufacturing capabilities;
- subject to receipt of marketing approval, if any, revenue received from commercial sale of our current and future product candidates;
- the terms and timing of any future collaborations, licensing, consulting or other arrangements that we may establish;
- the amount and timing of any payments we may be required to make, or that we may receive, in connection with the licensing, filing, prosecution, maintenance, defense and enforcement of any patents or other intellectual property rights, including milestone and royalty payments and patent prosecution fees that we are obligated to pay pursuant to our license agreements;
- our current license agreements remaining in effect and our achievement of milestones under those agreements;
- our ability to establish and maintain collaborations and licenses on favorable terms, if at all; and
- the extent to which we acquire or in-license other product candidates and technologies.

We may need to obtain substantial additional funding in order to receive regulatory approval and to commercialize our product candidates. To the extent that we raise additional capital through the sale of common stock, convertible securities or other equity securities, the ownership interests of our existing stockholders may be materially diluted and the terms of these securities could include liquidation or other preferences that could adversely affect the rights of our existing stockholders. In addition, debt financing, if available, would result in increased fixed payment obligations and may involve agreements that include restrictive covenants that limit our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends, that could adversely affect our ability to conduct our business. If we are unable to raise capital when needed or on attractive terms, we could be forced to significantly delay, scale back or discontinue the development or commercialization of our product candidates, seek collaborators at an earlier stage than otherwise would be desirable or on terms that are less favorable than might otherwise be available, and relinquish or license, potentially on unfavorable terms, our rights to our product candidates that we otherwise would seek to develop or commercialize ourselves.

Sources and Uses of Cash

The following table summarizes our sources and uses of cash for the six months ended June 30, 2023 and 2022 (in thousands):

	Six Months Ended June 30,	
	2023	**2022**
	(unaudited)	
Net cash used in operating activities	(60,346)	(58,552)
Net cash used in investing activities	(12,394)	(94,132)
Net cash provided by financing activities	186,743	30,158
Effect of exchange rate changes on cash and cash equivalents	(28)	—
Net increase (decrease) in cash	$ 113,975	$ (122,526)

Operating Activities

Net cash used in operating activities for the six months ended June 30, 2023 was $60.3 million and consisted primarily of a net loss of $78.5 million adjusted for non-cash items primarily comprised of stock-based compensation expense of $21.8 million, depreciation and amortization of $1.7 million and other adjustments of $2.4 million, and cash used by increases in net working capital of approximately $3.0 million.

Net cash used in operating activities for the six months ended June 30, 2022 was $58.6 million and consisted primarily of a net loss of $78.1 million adjusted for non-cash items primarily comprised of depreciation and amortization and stock-based compensation expense of $16.4 million, and including increases from net changes in operating assets and liabilities of approximately $3.1 million.

Investing Activities

Net cash provided by investing activities for the six months ended June 30, 2023 was $12.4 million and consisted primarily of proceeds of $315.7 million received from the maturities of short-term investments, partially offset by expenditures of $8.2 million on the build-out of our ASTRA facility, leasehold improvement of new office space, and purchases of computer and laboratory equipment, and $320.0 million on the purchase of short-term and long-term investments.

Net cash used in investing activities for the six months ended June 30, 2022 was $94.1 million and consisted primarily of expenditures of $33.7 million on the build-out of our ASTRA facility, leasehold improvement of new office space, and purchases of computer and laboratory equipment, $147.3 million on the purchase of short-term and long-term investments, partially offset by proceeds of $86.8 million received from the maturities of short-term investments.

Financing Activities

Net cash provided by financing activities for the six months ended June 30, 2023 was $186.7 million and consisted primarily of proceeds of $160.0 million received from a private placement equity offering and proceeds of $27.7 million from exercises of stock options, partially offset by $749 thousand used for the employee tax withholding payment for settlement of vested restricted stock awards.

Net cash provided by financing activities for the six months ended June 30, 2022 was $30.2 million and consisted primarily of proceeds of $30.8 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. During the six months

ended June 30, 2022, the Company issued and sold 434,782 shares of common stock at a weighted average price of $69.00 per share for net proceeds of $29.1 million after deducting underwriting discounts and commissions of approximately $900 thousand. For the six months ended June 30, 2022, the Company received proceeds of $1.7 million from the exercise of stock options.

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 $477.5 million at June 30, 2023, which consist primarily of money market, bank deposits, 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 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 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 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 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 second quarter of 2023, we completed the implementation of the inventory module within our enterprise resource planning software, Microsoft Dynamics D365 ("Dynamics"), as part of our plan to upgrade our systems and processes as we prepared for the commercial launch of VYJUVEK. We expect the implementation of this additional module to strengthen our internal controls by automating a number of accounting and reporting controls supporting our manufacturing, inventory and commercial processes. 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, no change in our internal control over financial reporting occurred during the quarter ended June 30, 2023 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 6 of the notes to condensed consolidated financial statements included in Item 1 of Part I of this Form 10-Q is incorporated by reference in response to this item.

Item 1A. Risk Factors.

There are a number of risks and uncertainties that may have a material effect on 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 in conjunction with “Summary Risk Factors” and “Item 1A. Risk Factors” in our Annual Report on Form 10-K for the fiscal year ended December 31, 2022, as filed with the SEC on February 27, 2023, and Exhibit 99.2 to our Form 8-K filed with the SEC on May 19, 2023. Except as set forth below, the Risk Factors included in our Annual Report on Form 10-K for the year ended December 31, 2022 and in Exhibit 99.2 to our Form 8-K filed with the SEC on May 19, 2023 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 profitability.

Since inception, we have incurred recurring losses and negative cash flows from operations and, at June 30, 2023, we had an accumulated deficit of $359.3 million. Our ability to achieve 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, EU 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. 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 could be delayed by many years in our ability to achieve profitability, if ever, and our business prospects and financial condition could be materially adversely affected. Moreover, if we decide to leverage any success with 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 will be materially adversely affected.

We currently have one FDA-approved product, VYJUVEK, 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 profitability. If we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and 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 products and biological development, we are unable to accurately predict the timing or amount of increased expenses or when, or if, we will be able to achieve 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 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 anticipate that we 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 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 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 and product candidates and growth potential and the market price per share of our common stock.

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, with respect to the development of our product candidates have been limited 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, and obtaining FDA approval for 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

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 FDA approval for VYJUVEK 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. 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 and the sales, marketing, and distribution of VYJUVEK or any future approved products may be unsuccessful or less successful than anticipated.

We recently received FDA approval 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 of such capabilities. For example, our commercial launch may not develop as planned 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. 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, and we may not be able to compete against more established companies in our industry.

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

To successfully commercialize any products that may result from our development activities, we need to continue to expand our marketing and distribution capabilities, either on our own or in collaboration with others. The development of our marketing and distribution effort is, and will continue to be, expensive and time-consuming and could delay any further product launch. We cannot be certain that we will be able to develop this capability successfully. We may enter into collaborations regarding VYJUVEK, or any future approved product candidates, with other entities to utilize their established marketing and distribution capabilities. However, we may be unable to enter into such agreements on favorable terms, if at all. If any future collaborators do not commit sufficient resources to commercialize VYJUVEK or any future product candidates, or we are unable to develop the necessary capabilities on our own, we will be unable to generate sufficient product revenue to sustain our business. We compete 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 with the sales and marketing efforts of VYJUVEK and any future product candidates, if approved.

The market for VYJUVEK, 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 a variety of factors, including our estimates of the number of people who have these diseases, the potential scope of our approved product labels, the subset of people with these diseases who have the potential to benefit from treatment with VYJUVEK or any product candidates, various pricing scenarios, and our understanding of reimbursement policies in particular countries. These estimates are based on many assumptions and may prove incorrect, and new studies may reduce the estimated incidence or prevalence of these diseases. Estimating market opportunities can be particularly challenging for rare indications, 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 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 revenues may be smaller than anticipated, and our business may suffer.

Our products 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 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 the product candidate, 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, 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 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 our product or 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 a product or 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 our product or product candidates and could significantly harm our business, financial condition, results of operations and prospects.

Our product candidates will 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 our product to lose 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") 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. 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 prevent, limit or delay regulatory approval of 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 profitability, which would materially and adversely affect our business, financial condition, results of operations and prospects.

While we have obtained orphan drug designation for VYJUVEK, KB105, and KB407, it may not effectively protect us from competition, and we may be unable to obtain orphan drug designation for our future 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. Regulatory authorities in some jurisdictions, including the United States and the EU, may designate drugs for relatively small patient populations as orphan drugs. Under the Orphan Drug Act of 1983, 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. In the EU, 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 would be sufficient to justify the necessary investment in developing the drug or biologic product.

Generally, if a product candidate with an orphan drug designation receives the first marketing approval for the indication for which it has such designation, the product is entitled to a period of marketing exclusivity, which precludes the FDA or the EMA from approving another marketing application for a product that constitutes the same drug treating the same indication for that marketing 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 be reduced to six years if a product no longer meets the criteria for orphan drug designation or if the product is sufficiently profitable so that market exclusivity is no longer justified. Orphan drug exclusivity may be revoked if any regulatory agency determines that 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.

Even though we have obtained orphan drug exclusivity for VYJUVEK, KB105 and KB407, that 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 orphan drug is approved, the FDA may subsequently approve another drug for the same condition if the FDA concludes that the latter drug is not the same drug 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 be granted to 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 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.

Breakthrough therapy designation, Regenerative Medicine Advanced Therapy designation, Fast Track designation or Rare Pediatric Disease designation by the FDA, even if granted for any of our product candidates, may not lead to a faster development, regulatory review or approval process, and it does not increase the likelihood that any of our product candidates will receive marketing approval in the United States.

A RMAT/PRIME therapy product candidate is defined as a product candidate that is intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening disease. Drugs designated as RMAT therapies by the FDA are eligible for accelerated approval and increased interaction and communication with the FDA designed to expedite the development and review process. If a drug, or biologic in our case, is intended for the treatment of a serious or life-threatening condition and the biologic demonstrates the potential to address unmet medical needs for this condition, the biologic sponsor may apply for FDA Fast Track designation. Even after having received Fast Track designation, we may not experience a faster

development process, review or approval compared to conventional FDA procedures. In addition, the FDA may withdraw Fast Track designation if it believes that the designation is no longer supported by data from our clinical development program. Many biologics that have received Fast Track designation have failed to obtain approval. A sponsor who receives an approval for a drug or biologic for a "rare pediatric disease" 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 approval of VYJUVEK, the FDA issued us 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, we may not experience a faster review or approval for a subsequent marketing application.

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 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 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 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 future product candidate uneconomical or obsolete, and we may not be successful in marketing VYJUVEK or any future product candidate 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 our approved product, use of our approved product and 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 our approved product;
- 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
- the inability to commercialize our product candidates.

With respect to our approved product 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. 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.

Risks Related to Manufacturing

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

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 will 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. The CGMP requirements govern quality control of the manufacturing process and documentation policies and procedures. In complying with CGMP, we will be 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 be subject to possible regulatory action and may not be permitted to sell any product candidate that we may develop.

In addition, the manufacturing process used to produce VYJUVEK and our product candidates is complex and novel. 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 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. 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. 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 utilize a third-party for manufacturing of the sterile gel that is mixed with our in-house produced vector for VYJUVEK. In the event that any third-party manufacturers do not successfully carry out their contractual duties, meet expected deadlines or manufacture in accordance with regulatory requirements or if there are disagreements between us and any third-party manufacturers, we may not be able to manufacture our products for commercial or regulatory purposes. In such instances, we may need to locate an appropriate replacement third-party relationship, which may not be readily available or on acceptable terms, which would cause additional delay or increased expense and would thereby have a material adverse effect on our business, financial condition, results of operations and prospects.

If we or a third-party 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 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 clinical development or marketing schedules.

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 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.

Risks Related to Commercialization of Our Product or Product Candidates

If we are unable to expand our market development capabilities or enter into agreements with third parties to market and sell VYJUVEK or our product candidates, we may be unable to generate any product 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, we will need to further expand our market development organization, either on our own or with a third-party. The development of our own market development team is expensive and time-consuming and could delay any product launch. Moreover, we cannot be certain that we will be able to successfully develop this capability. We may enter into collaboration agreements regarding any of our product candidates with third parties to utilize their established marketing and distribution capabilities, but we may be unable to enter into such agreements on favorable terms, if at all. If any future collaborators do not commit sufficient resources to commercialize our products, or we are unable to develop the necessary capabilities on our own, we will be unable to generate sufficient product revenue to sustain our business. 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 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 any of our product candidates is approved but fails to achieve market acceptance among physicians, patients or third-party payors, we will not be able to generate significant revenues 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 may be adversely impacted, and our business may suffer.

We have mainly focused our research and product development efforts to date on VYJUVEK for DEB. Our understanding of both the number of people who have this disease, as well as the subset of people with this disease who have the potential to benefit from treatment with VYJUVEK, are based on estimates in published literature. These estimates may prove to be incorrect and new studies may reduce the estimated incidence or prevalence of this disease. The number of patients in the United States, the EU and elsewhere may turn out to be lower than expected or these patients may not be otherwise amenable to treatment with VYJUVEK or may become increasingly difficult to identify and access, all of which would adversely affect our business, financial condition, results of operations and prospects.

Further, there are several factors that could contribute to making the actual number of patients who receive VYJUVEK less than the potentially addressable market. These include the lack of widespread availability of, and limited reimbursement for, new therapies in many underdeveloped markets. Further, the severity of the progression of a disease up to the time of treatment 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.

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

We anticipate that prior to receiving certain gene therapies, 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 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 other future product candidates 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. 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 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 EU 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.

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 develop our own manufacturing facilities, build our sales, marketing and distribution infrastructure that we believe will be necessary to commercialize VYJUVEK, and increase 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. It is likely that our management, finance, development personnel, systems and facilities currently in place may not be adequate to support this 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 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 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 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.

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 U.S. and foreign patent protection for our approved product and product candidates, 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. For example, there is no assurance that our existing patents or any other patent we are granted will prevent third parties from developing competing technologies. 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 dermatological or pulmonary 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 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 our approved product and product candidates, 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 develop, manufacture, market and sell VYJUVEK and our product candidates, and to freely use our proprietary technologies (e.g., 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 The United States Patent and Trademark Office. Our competitors or other third parties may assert infringement claims against us, alleging that our therapeutics, manufacturing methods, formulations or administration methods are covered by their patents. Moreover, we may face patent infringement claims from non-practicing entities that have no relevant product revenue, and against whom our patent portfolio may therefore have no deterrent effect.

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

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

Risks Related to Ownership of Our Common Stock

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 may not be able to sell your

common stock at or above the price that you paid for it. The market price of our common stock may be influenced by many factors, including:

- our ability to successfully commercialize VYJUVEK
- our ability to successfully proceed to and conduct clinical trials;
- results of clinical trials of our product candidates or those of our competitors;
- our ability to obtain regulatory approval for our product candidates and our ability to successfully commercialize any of our approved product candidates;
- the success of competitive products or technologies;
- commencement or termination of collaborations;
- regulatory or legal developments in the United States and other countries;
- 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 any approved product or inability to do so at acceptable prices;
- disputes or other developments relating to proprietary rights, including patents, litigation matters and our ability to obtain patent protection for our 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” in our Annual Report on Form 10-K for the fiscal year ended December 31, 2022.

ITEM 2. UNREGISTERED SALES OF EQUITY SECURITIES AND USE OF PROCEEDS

None.

ITEM 3. DEFAULTS UPON SENIOR SECURITIES

None.

ITEM 4. MINE SAFETY DISCLOSURES

Not applicable.

ITEM 5. OTHER INFORMATION

Andrew Orth, the Company’s Chief Commercial Officer, notified the Company that he was resigning from his position and his last day with the Company was August 2, 2023.

Insider Trading Arrangements

On June 12, 2023, Krish Krishnan, our Chief Executive Officer and Chairman of our Board of Directors,adopted a Rule 10b5-1 trading arrangement that is intended to satisfy the affirmative defense conditions of Rule 10b5-1(c) under the Exchange Act for the sale of up to 100,000 shares of our Common Stock until September 11, 2024.

On June 12, 2023, Suma Krishnan, our President, R&D and a member of our Board of Directors, adopted a Rule 10b5-1 trading arrangement that is intended to satisfy the affirmative defense conditions of Rule 10b5-1(c) under the Exchange Act for the sale of up to 100,000 shares of our Common Stock until September 11, 2024.

ITEM 6. EXHIBITS

Exhibit Number	
10.1	Securities Purchase Agreement, by and among Krystal Biotech, Inc. and the institutional investors listed on the signature pages thereto, dated as of May 21, 2023 (incorporated by reference to Exhibit 10.1 to the Company's Current Report on Form 8-K, as filed with the SEC on May 22, 2023).
10.2	Registration Rights Agreement, by and among Krystal Biotech, Inc. and the institutional investors listed on the signature pages thereto, dated as of May 21, 2023 (incorporated by reference to Exhibit 10.2 to the Company's Current Report on Form 8-K, as filed with the SEC on May 22, 2023).
31.1	Certification of Periodic Report by Chief Executive Officer under Section 302 of the Sarbanes-Oxley Act of 2002.
31.2	Certification of Periodic Report by Chief Accounting Officer under Section 302 of the Sarbanes-Oxley Act of 2002.
32.1	Certification of Chief Executive Officer and Chief Accounting Officer Pursuant to 18 U.S.C. Section 1350 as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
101	Inline XBRL (Extensible Business Reporting Language). The following materials from this Quarterly Report on Form 10-Q for the period ended June 30, 2023, 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 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 consolidated financial statements of Krystal Biotech, Inc. The instance document does not appear in the interactive data file because its XBRL tags are embedded within the Inline XBRL document.
104	Cover Page Interactive Data File - the cover page XBRL tags are embedded within the Inline XBRL.

SIGNATURES

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

KRYSTAL BIOTECH, INC.
(Registrant)

Date: August 7, 2023	By:	/s/ Krish S. Krishnan
		Krish S. Krishnan President and Chief Executive Officer (Principal executive officer)
	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: August 7, 2023

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

Exhibit 31.2

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

I, Kathryn A. Romano, certify that:

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

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the Condensed financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) for the registrant and have:

   a. Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

   b. Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

   c. Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

   a. All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

   b. Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: August 7, 2023

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

Exhibit 32.1

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

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

1. the Quarterly Report on Form 10-Q for the three months ended June 30, 2023, (the "Periodic Report") fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and

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

Date: August 7, 2023

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

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

1. the Quarterly Report on Form 10-Q for the three months ended June 30, 2023, (the "Periodic Report") fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and

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

Date: August 7, 2023

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