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, 2025

or

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

For the transition period from_____ to_____

Commission file number: 001-38210

Krystal Biotech, Inc.

(Exact name of registrant as specified in its charter)

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

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

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

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

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

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

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

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

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

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

Emerging growth company ☐

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

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

As of July 30, 2025, there were 28,942,981 shares of the registrant's common stock issued and outstanding.

Krystal Biotech, Inc.
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PART I. FINANCIAL INFORMATION

ITEM 1. FINANCIAL STATEMENTS

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

(in thousands, except par value)	June 30, 2025	December 31, 2024
Assets		
Current assets		
Cash and cash equivalents	$ 353,829	$ 344,865
Short-term investments	328,157	252,652
Accounts receivable, net	111,439	104,746
Inventory	31,041	26,508
Prepaid expenses and other current assets	16,881	13,274
Total current assets	841,347	742,045
Property and equipment, net	150,388	155,168
Long-term investments	138,807	152,114
Right-of-use assets	7,655	6,280
Other non-current assets	197	231
Total assets	$ 1,138,394	$ 1,055,838
Liabilities and Stockholders' Equity		
Current liabilities		
Accounts payable	$ 7,124	$ 4,985
Current portion of lease liability	1,617	1,217
Accrued rebates	51,638	36,804
Accrued expenses and other current liabilities	26,517	58,989
Total current liabilities	86,896	101,995
Lease liability	8,064	6,044
Other long-term liabilities	2,787	1,419
Total liabilities	97,747	109,458
Commitments and contingencies (see note 7)		
Stockholders' equity		
Common stock; $0.00001 par value; 80,000 shares authorized as of June 30, 2025 and December 31, 2024; 28,927 and 28,794 shares issued and outstanding as of June 30, 2025 and December 31, 2024, respectively.	—	—
Additional paid-in capital	1,146,092	1,127,238
Accumulated other comprehensive gain (loss)	1,157	(190)
Accumulated deficit	(106,602)	(180,668)
Total stockholders' equity	1,040,647	946,380
Total liabilities and stockholders' equity	$ 1,138,394	$ 1,055,838

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

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

	Three Months Ended June 30,		Six Months Ended June 30, 2025	
(in thousands, except per share data)	**2025**	**2024**	**2025**	**2024**
Product revenue, net	$ 96,042	$ 70,284	$ 184,225	$ 115,535
Operating expenses				
Cost of goods sold	7,165	6,009	12,193	8,428
Research and development	14,410	15,583	28,666	26,539
Selling, general and administrative	35,160	27,626	67,883	53,685
Litigation settlement	—	12,500	—	25,000
Total operating expenses	56,735	61,718	108,742	113,652
Income from operations	39,307	8,566	75,483	1,883
Other income				
Interest and other income, net	7,468	7,479	14,889	15,095
Income before income taxes	46,775	16,045	90,372	16,978
Income tax expense	(8,442)	(477)	(16,305)	(477)
Net income	38,333	15,568	74,067	16,501
Unrealized (loss) gain on available-for-sale securities	(158)	(252)	186	(1,127)
Foreign currency translation	925	(83)	1,161	(145)
Comprehensive income	$ 39,100	$ 15,233	$ 75,414	$ 15,229
Net income per common share:				
Basic	$ 1.33	$ 0.54	$ 2.57	$ 0.58
Diluted	$ 1.29	$ 0.53	$ 2.48	$ 0.56
Weighted-average common shares outstanding:				
Basic	28,910	28,598	28,863	28,446
Diluted	29,749	29,637	29,819	29,504

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

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

(in thousands)	Common Stock Shares	Common Stock Amount	Additional Paid-in Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders' Equity
Balances as of January 1, 2025	28,794	$ —	$ 1,127,238	$ (190)	$ (180,668)	$ 946,380
Issuance of common stock upon exercise of stock options	17	—	1,462	—	—	1,462
Vesting of restricted stock units, net of shares withheld for taxes	98	—	(12,116)	—	—	(12,116)
Shares of restricted stock awards surrendered for taxes	(10)	—	(1,812)	—	—	(1,812)
Stock-based compensation	—	—	14,447		—	14,447
Unrealized gain on investments	—	—	—	344	—	344
Foreign currency translation	—	—	—	236	—	236
Net income	—	—	—	—	35,733	35,733
Balances as of March 31, 2025	28,899	$ —	$ 1,129,219	$ 390	$ (144,935)	$ 984,674
Issuance of common stock upon exercise of stock options	28	—	1,796	—	—	1,796
Stock-based compensation	—	—	15,077	—	—	15,077
Unrealized loss on investments	—	—	—	(158)	—	(158)
Foreign currency translation	—	—	—	925	—	925
Net income	—	—	—	—	38,333	38,333
Balances as of June 30, 2025	28,927	$ —	$ 1,146,092	$ 1,157	$ (106,602)	$ 1,040,647

(in thousands)	Common Stock Shares	Common Stock Amount	Additional Paid-in Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders' Equity
Balances as of January 1, 2024	28,237	$ —	$ 1,047,830	$ 638	$ (269,827)	$ 778,641
Issuance of common stock upon exercise of stock options	260	—	15,969	—	—	15,969
Vesting of restricted stock units, net of shares withheld for taxes	39	—	(4,181)	—	—	(4,181)
Shares of restricted stock awards surrendered for taxes	(8)	—	(1,205)	—	—	(1,205)
Stock-based compensation	—	—	10,023	—	—	10,023
Unrealized (loss) on investments	—	—	—	(875)	—	(875)
Foreign currency translation	—	—	—	(62)		(62)
Net income	—	—	—	—	932	932
Balances as of March 31, 2024	28,528	$ —	$ 1,068,436	$ (299)	$ (268,895)	$ 799,242
Issuance of common stock upon exercise of stock options	181	—	10,637	—	—	10,637
Stock-based compensation	—	—	13,781	—	—	13,781
Unrealized (loss) on investments	—	—	—	(252)	—	(252)
Foreign currency translation	—	—	—	(83)	—	(83)
Net income	—	—	—	—	15,568	15,568
Balances as of June 30, 2024	28,709	$ —	$ 1,092,854	$ (634)	$ (253,327)	$ 838,893

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, 2025	2024
Operating Activities		
Net income	$ 74,067	$ 16,501
Adjustments to reconcile net income to net cash provided by operating activities		
Depreciation	2,759	3,262
Amortization (accretion) on marketable securities	464	(1,104)
Amortization of operating lease right-of-use assets	418	368
Stock-based compensation expense, net	27,597	22,455
Realized gain on investments	(4,256)	(2,859)
Other, net	690	89
Changes in operating assets and liabilities		
Accounts receivable, net	(6,693)	(61,196)
Inventory	(515)	(1,708)
Prepaid expenses and other current assets	(2,957)	(2,503)
Lease liability	(258)	(406)
Other long-term liabilities	1,368	588
Accounts payable	5,029	1,511
Accrued expenses and other current liabilities	2,401	(4,034)
Accrued rebates	14,834	15,756
Accrued litigation settlement	(31,250)	25,000
Net cash provided by operating activities	83,698	11,720
Investing Activities		
Proceeds from disposal of assets	435	—
Purchases of property and equipment	(8,108)	(2,391)
Purchases of investments	(251,705)	(201,736)
Maturities of investments	194,084	158,794
Net cash (used in) investing activities	(65,294)	(45,333)
Financing Activities		
Proceeds from exercise of stock options	3,258	26,607
Taxes paid for employee tax withholding related to restricted stock units	(12,116)	(4,181)
Taxes paid related to settlement of restricted stock awards	(1,812)	(1,205)
Net cash (used in) provided by financing activities	(10,670)	21,221
Effect of exchange rate changes on cash and cash equivalents	1,230	(150)
Net increase (decrease) in cash and cash equivalents	8,964	(12,542)
Cash and cash equivalents at beginning of period	344,865	358,328
Cash and cash equivalents at end of period	$ 353,829	$ 345,786
Supplemental Disclosures of Non-Cash Activities		
Unpaid purchases of property and equipment included in accounts payable and accrued expenses	$ 1,329	$ 8,568
Initial recognition of right-of-use assets	$ 1,794	$ —
Supplemental Cash Flow Information		
Income taxes paid	$ 13,858	$ 2,002

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

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

1. Organization

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

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

Liquidity

As of June 30, 2025, the Company had an accumulated deficit of $106.6 million. Our operating profitability is dependent upon the continued successful commercialization of VYJUVEK, our U.S. Food and Drug Administration ("FDA"), European Medicines Agency ("EMA"), and Japan's Ministry of Health, Labour, and Welfare ("MHLW") approved product, as well as successful development, approval and commercialization of our product candidates. Management intends to fund future operations through its on hand cash and cash equivalents and revenue generated from the sale of VYJUVEK, and may also seek additional capital through arrangements with strategic partners, the sale of equity, debt financings or other sources.

The Company is subject to risks common to companies in the biotechnology industry, including but not limited to the failure of product candidates in clinical and preclinical studies, the development of competing product candidates or other technological innovations by competitors, dependence on key personnel, protection of proprietary technology, compliance with government regulations and the ability to commercialize product candidates. The Company expects to incur significant costs to further its pipeline and to expand its commercialization capabilities in advance of further potential global regulatory approvals of VYJUVEK. The Company believes that its cash, cash equivalents and short-term investments of approximately $682.0 million as of June 30, 2025 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 condensed consolidated financial statements have been prepared in conformity with generally accepted accounting principles in the United States of America ("GAAP"). In the opinion of management, all adjustments, which consist of all normal recurring adjustments necessary for a fair presentation of the Company's financial position and results of operations for the interim periods presented, are reflected in the interim condensed consolidated financial statements. All intercompany balances and transactions have been eliminated in consolidation.

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

The results of operations for the interim periods are not necessarily indicative of the results of operations to be expected for the full year. These unaudited 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, 2024 ("2024 10-K"), as filed with the U.S. Securities and Exchange Commission ("SEC") on February 19, 2025.

Use of Estimates

The preparation of condensed consolidated financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts in the condensed consolidated financial statements and

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

Summary of Significant Accounting Policies

See Note 2 to our consolidated financial statements included in our 2024 10-K. There were no material changes to the Company's significant accounting policies during the six months ended June 30, 2025.

Recently Issued Accounting Pronouncements, Not Yet Adopted

There were no accounting pronouncements issued or adopted during the six months ended June 30, 2025 that are expected to have a material impact on the Company's condensed consolidated financial statements.

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

In November 2024, the FASB issued *ASU 2024-03 Income Statement—Reporting Comprehensive Income—Expense Disaggregation Disclosures (Subtopic 220-40): Disaggregation of Income Statement Expenses.* This standard calls for enhanced disclosures about components of expense captions on the face of the income statement. This standard will be effective for fiscal years beginning after December 15, 2026, with the option to apply it retrospectively. Early adoption is allowed. Currently, the Company is assessing the potential impact of this guidance on its consolidated financial statement disclosures.

3. Product Revenue, Accounts Receivable and Reserves for Product Sales

The Company's product revenue, net of sales discounts and allowances totaled $96.0 million and $70.3 million for the three months ended June 30, 2025 and June 30, 2024, respectively, and $184.2 million and $115.5 million for the six months ended June 30, 2025 and June 30, 2024 respectively.

The Company's accounts receivable, net balance relating to VYJUVEK sales was $111.4 million as of June 30, 2025 and $104.7 million as of December 31, 2024. As of June 30, 2025 and December 31, 2024, approximately 78% and 97%, respectively, of the Company's accounts receivable, net was outstanding from a single customer. All other single customers represent less than 10% of outstanding in the applicable period.

The following table summarizes changes in allowances and discounts for the six months ended June 30, 2025:

(in thousands)	Rebates	Prompt Pay	Other Accruals	Total
Balance as of December 31, 2024	$ 38,223	$ 2,570	$ 326	$ 41,119
Provisions	30,918	6,245	257	37,420
Payments/Credits	(14,716)	(4,362)	(261)	(19,339)
Balance as of June 30, 2025	$ 54,425	$ 4,453	$ 322	$ 59,200

Rebates are included in accrued rebates and other long-term liabilities on the condensed consolidated balance sheets. Other long-term liabilities are comprised of $2.8 million and $1.4 million of long-term accrued rebates as of June 30, 2025 and December 31, 2024, respectively. Prompt pay is recorded as an allowance against accounts receivable, net on the condensed consolidated balance sheets. Other accruals are included in accrued expenses and other current liabilities on the condensed consolidated balance sheets. Provisions for rebates, prompt pay and other accruals are recorded as a reduction to product revenue, net on the condensed consolidated statements of operations and comprehensive income.

4. Net Income Per Share Attributable to Common Stockholders

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

For the three months ended June 30, 2025 and 2024, respectively, there were 604 thousand and 215 thousand common stock equivalents outstanding in the form of stock options and 275 thousand and 2 thousand in unvested restricted stock, that have been excluded from the calculation of diluted net income per common share as their effect would be anti-dilutive.

For the six months ended June 30, 2025 and 2024, respectively, there were 520 thousand and 169 thousand common stock equivalents outstanding in the form of stock options and 89 thousand and 1 thousand in unvested restricted stock, that have each been excluded from the calculation of diluted net income per common share as their effect would be anti-dilutive.

	Three Months Ended June 30,		Six Months Ended	
(in thousands, except per share data)	**2025**	**2024**	**2025**	**2024**
Numerator:				
Net income	$ 38,333	$ 15,568	$ 74,067	$ 16,501
Denominator:				
Weighted-average basic common shares	28,910	28,598	28,863	28,446
Dilutive effect of stock options and unvested restricted stock	839	1,039	956	1,058
Weighted-average diluted common shares	29,749	29,637	29,819	29,504
Net income per common share—basic	$ 1.33	$ 0.54	$ 2.57	$ 0.58
Net income per common share—diluted	$ 1.29	$ 0.53	$ 2.48	$ 0.56

5. Fair Value Instruments

The following tables show the Company's cash, cash equivalents and available-for-sale securities by significant investment category as of June 30, 2025 and December 31, 2024:

	June 30, 2025						
(in thousands)	**Amortized Cost**	**Gross Unrealized Gains**	**Gross Unrealized (Losses)**	**Aggregate Fair Value**	**Cash and Cash Equivalents**	**Short-term Marketable Securities[1]**	**Long-term Marketable Securities[2]**
Level 1:							
Cash and cash equivalents	$ 353,829	$ —	$ —	$ 353,829	$ 353,829	$ —	$ —
Subtotal	353,829	—	—	353,829	353,829	—	—
Level 2:							
Commercial paper	33,513	1	(4)	33,510	—	33,510	—
Corporate bonds	210,453	402	(53)	210,802	—	127,018	83,784
U.S. government agency securities	222,433	306	(87)	222,652	—	167,629	55,023
Subtotal	466,399	709	(144)	466,964	—	328,157	138,807
Total	$ 820,228	$ 709	$ (144)	$ 820,793	$ 353,829	$ 328,157	$ 138,807

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

(in thousands)	December 31, 2024						
	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	$ 344,865	$ —	$ —	$ 344,865	$ 344,865	$ —	$ —
Subtotal	344,865	—	—	344,865	344,865	—	—
Level 2:							
Commercial paper	15,373	4	(8)	15,369	—	15,369	—
Corporate bonds	177,771	423	(225)	177,969	—	86,693	91,276
U.S. government agency securities	211,283	318	(173)	211,428	—	150,590	60,838
Subtotal	404,427	745	(406)	404,766	—	252,652	152,114
Total	$ 749,292	$ 745	$ (406)	$ 749,631	$ 344,865	$ 252,652	$ 152,114

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

6. Balance Sheet Components

Inventory

Inventory consisted of the following:

(in thousands)	June 30, 2025	December 31, 2024
Raw materials	$ 14,053	$ 13,639
Work-in-process	15,285	10,743
Finished goods	1,703	2,126
Inventory	$ 31,041	$ 26,508

Property and Equipment, Net

Property and equipment, net consisted of the following:

(in thousands)	June 30, 2025	December 31, 2024
Building and building improvements	$ 109,150	$ 111,444
Manufacturing equipment	28,786	27,161
Leasehold improvements	27,487	25,673
Laboratory equipment	3,546	3,183
Construction in progress	3,438	5,778
Computer equipment and software	2,514	2,032
Furniture and fixtures	2,123	1,816
Total property and equipment	177,044	177,087
Accumulated depreciation	(26,656)	(21,919)
Property and equipment, net	$ 150,388	$ 155,168

Depreciation expense was $1.3 million and $1.8 million for the three months ended June 30, 2025 and 2024, respectively, and $2.8 million and $3.3 million for the six months ended June 30, 2025 and June 30, 2024, respectively. Depreciation expense capitalized into inventory was $1.1 million and $559 thousand for the three months ended June 30, 2025 and 2024, respectively, and $2.0 million and $1.4 million for the six months ended June 30, 2025 and June 30, 2024, respectively.

Accrued Expenses and Other Current Liabilities

Accrued expenses and other current liabilities consisted of the following as of June 30, 2025 and December 31, 2024:

(in thousands)	June 30, 2025	December 31, 2024
Accrued taxes	8,298	4,288
Accrued professional fees	5,293	2,659
Accrued payroll and benefits	5,107	9,558
Accrued preclinical and clinical expenses	3,322	2,537
Other current liabilities	2,173	2,403
Accrued inventory	1,288	1,217
Accrued construction in progress	1,036	5,077
Accrued litigation settlement	—	31,250
Accrued expenses and other current liabilities	$ 26,517	$ 58,989

In May 2020, PeriphaGen, Inc. ("PeriphaGen") commenced litigation against the Company alleging breach of contract and misappropriation of trade secrets. In April 2022, the Company and PeriphaGen entered into a final settlement. In accordance with the settlement agreement, the Company paid PeriphaGen total consideration of $75.0 million to settle the dispute, acquire certain assets and receive an exclusive license from PeriphaGen to certain intellectual property assets and biological materials which was paid over time upon completion of certain milestones, with the final payment occurring during the three months ended March 31, 2025. Refer to Note 7 of our consolidated financial statements in our 2024 10-K for additional information.

The Company recorded litigation settlement expense of zero and $12.5 million for the three months ended June 30, 2025 and 2024, respectively, and zero and $25.0 million for the six months ended June 30, 2025 and 2024, respectively on the condensed consolidated statements of operations and comprehensive income. As of March 31, 2025, the Company has fully paid the $75.0 million of total consideration discussed above.

7. Commitments and Contingencies

Agreements with Contract Manufacturing Organizations and Contract Research Organizations

The Company enters into various agreements in the normal course of business with Contract Manufacturing Organizations ("CMOs"), Contract Research Organizations ("CROs") and other third parties for preclinical research studies, clinical trials and testing and manufacturing services. The agreements with CMOs primarily relate to the manufacturing of our sterile gel that is mixed with in-house produced vectors as part of the final drug product for VYJUVEK. Agreements with third parties may also include research and development consulting activities, clinical-trial agreements, testing of our clinical-stage, pre-commercial and commercial stage products and/or storage, packaging and labeling. The Company is obligated to make milestone payments under certain of these contracts. The Company may also be responsible for the payment of a monthly service fee for project management services for the duration of any agreements. The estimated remaining commitments as of June 30, 2025 under these agreements was approximately $507 thousand. The Company has incurred research and development expenses related to commitments under these agreements of $2.2 million and $4.3 million for the three and six months ended June 30, 2025, respectively, and $1.0 million and $2.5 million for the three and six months ended June 30, 2024, respectively.

Contingencies

In the ordinary course of business, the Company may be subject from time to time to various proceedings, lawsuits, disputes, or claims. In accordance with FASB ASC Topic 450, *Contingencie*s ("ASC 450"), the Company accrues a liability for legal contingencies when it is probable that a liability has been incurred, and the amount of the loss can be reasonably estimated. If there is at least a reasonable possibility that a loss may be incurred, ASC 450 requires disclosure of a loss contingency.

In the first quarter of 2025, the Company and certain of its employees received subpoenas from the U.S. Department of Justice requesting that the Company produce certain documents regarding its sponsored genetic testing program relating to VYJUVEK and commercial practices relating thereto. The Company is cooperating and providing information in response to the subpoenas. It is not possible to estimate the amount of any loss or range of possible loss that might result from this inquiry, and because the final outcome cannot be predicted with certainty, unfavorable or unexpected developments or outcomes could result in a material impact to the Company's results of operations.

8. Leases

As of June 30, 2025, future minimum commitments under the Company's operating leases with lease terms in excess of 12 months were as follows:

(in thousands)	Operating Leases
2025 (remaining six months)	$ 783
2026	1,866
2027	1,919
2028	1,954
2029	1,990
Thereafter	9,227
Future minimum operating lease payments	17,739
Less: Interest	(8,058)
Present value of lease liability	$ 9,681

As of June 30, 2025 and December 31, 2024, the Company's weighted-average remaining lease term for operating leases was 10.4 years and 12.2 years, respectively, and the Company's weighted-average discount rate for operating leases was 9.7% and 9.5% as of June 30, 2025 and December 31, 2024, respectively.

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

	Three Months Ended June 30,		Six Months Ended June 30,	
(in thousands)	2025	2024	2025	2024
Lease cost:				
Operating lease expense	$ 388	$ 346	$ 814	$ 645
Variable lease expense	56	51	119	91
Total lease expense	$ 444	$ 397	$ 933	$ 736

9. Stock-Based Compensation

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

Shares remaining available for grant under the Plan were 2.0 million as of June 30, 2025.

Stock Options

The following table summarizes the Company's stock option activity for the six months ended June 30, 2025:

	Stock Options Outstanding	Weighted-Average Exercise Price	Weighted-Average Remaining Contractual Life *(in years)*	Aggregate Intrinsic Value[(1)] *(in thousands)*
Outstanding as of December 31, 2024	2,049,063	$ 82.69	7.3	$ 156,404
Granted	335,743	$ 166.90		
Exercised	(45,533)	$ 71.56		
Cancelled or forfeited	(63,985)	$ 105.98		
Outstanding as of June 30, 2025	2,275,288	$ 94.68	7.1	$ 117,142
Exercisable as of June 30, 2025	1,255,141	$ 70.95	6.2	$ 85,882

(1) Aggregate intrinsic value represents the difference between the closing stock price of our Common Stock on December 31, 2024 and June 30, 2025 and the exercise price of outstanding in-the-money options.

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

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

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

The total intrinsic value (the amount by which the fair market value exceeds the exercise price) of stock options exercised was $2.0 million and $19.8 million during the three months ended June 30, 2025 and 2024, respectively, and $3.4 million and $44.3 million for the six months ended June 30, 2025 and 2024, respectively.

The weighted-average grant-date fair value per share of options granted to employees and directors was $87.69 and $112.05 during the three months ended June 30, 2025 and 2024, respectively, and $109.93 and $109.18 for the six months ended June 30, 2025 and 2024, respectively.

There was $71.4 million of unrecognized stock-based compensation expense related to employees' and directors' options that is expected to be recognized over a weighted-average period of 2.8 years as of June 30, 2025.

Restricted Stock Awards

The following table summarizes the Company's RSA activity:

	Six Months Ended June 30,			
	2025		2024	
	Number of Shares	Weighted-Average Grant Date Fair Value	Number of Shares	Weighted-Average Grant Date Fair Value
Non-vested RSAs, beginning of period	22,200	$ 78.89	44,400	$ 78.89
Granted	—		—	
Vested	(11,925)	$ 78.89	(14,523)	$ 78.89
Surrendered for taxes	(10,275)	$ 78.89	(7,677)	$ 78.89
Non-vested RSAs, end of period	—	$ —	22,200	$ 78.89

Restricted Stock Units

The following table summarizes the Company's RSU activity:

	Six Months Ended June 30,			
	2025		2024	
	Number of Shares	Weighted-Average Grant Date Fair Value	Number of Shares	Weighted-Average Grant Date Fair Value
Non-vested RSUs, beginning of period	308,096	$ 135.22	160,900	$ 81.91
Granted	133,756	$ 178.66	224,890	$ 159.59
Vested	(84,124)	$ 129.58	(40,075)	$ 81.91
Forfeited	(18,850)	$ 148.92	(29,314)	$ 103.31
Non-vested RSUs, end of period	338,878	$ 153.01	316,401	$ 135.14

There was $46.4 million of unrecognized stock-based compensation expense related to employees' RSU awards that is expected to be recognized over a weighted-average period of 3.0 years as of June 30, 2025.

Performance-Based Restricted Stock Units

The following table summarizes the Company's PSU activity:

	Six Months Ended June 30,			
	2025		2024	
	Number of Shares	Weighted-Average Grant Date Fair Value	Number of Shares	Weighted-Average Grant Date Fair Value
Non-vested PSUs, beginning of period	137,500	$ 145.37	50,000	$ 81.91
Granted	—	$ —	112,500	$ 159.47
Vested	(81,250)	$ 135.61	(25,000)	$ 81.91
Forfeited	—	$ —	—	$ —
Non-vested PSUs, end of period	56,250	$ 159.47	137,500	$ 145.37

PSUs vest ratably over two years based upon continued service through the vesting date and the achievement of specific regulatory and commercial performance criteria as determined by the Compensation Committee of the Company's Board of Directors which were met by the end of the year in which the PSU awards were granted.

There was $6.0 million of unrecognized stock-based compensation expense related to employees' PSU awards that is expected to be recognized over a weighted-average period of eight months as of June 30, 2025.

Stock-Based Compensation Expense, Net

The Company recorded stock-based compensation expense, net related to its stock options and restricted stock in the condensed consolidated statements of operations and comprehensive income for the three and six months ended June 30, 2025 and 2024 as follows:

	Three Months Ended June 30,		Six Months Ended	
(in thousands)	2025	2024	2025	2024
Research and development	$ 2,627	$ 2,772	5,096	4,640
Selling, general and administrative	11,492	10,384	22,501	17,815
Total stock-based compensation	$ 14,119	$ 13,156	$ 27,597	$ 22,455

The Company capitalized stock-based compensation associated with the allocation of labor costs related to work performed to manufacture VYJUVEK of $958 thousand and $625 thousand for the three months ended June 30, 2025 and 2024, respectively, and $1.9 million and $1.3 million for the six months ended June 30, 2025 and 2024, respectively.

10. Income Taxes

The Company recorded an income tax provision of $8.4 million and $16.3 million for the three and six months ended June 30, 2025. The tax provision for interim periods is calculated using an estimate of the annual effective tax rate, adjusted for discrete items. If there are any changes to the estimated annual tax rate, the Company will make a cumulative adjustment to the income tax provision in the period the change becomes known. The Company recorded an income tax provision of $477 thousand for the three and six months ended June 30, 2024. At June 30, 2025, the Company maintains a full valuation allowance against its net deferred tax assets.

11. Segment Information

The Company operates as one operating segment, which is focused on the discovery, development, manufacturing and commercialization of genetic medicines to treat diseases with high unmet medical needs. The Company's chief operating decision maker ("CODM"), our chief executive officer, utilizes financial information presented on a consolidated basis to manage and allocate resources. The CODM uses consolidated gross margin, operating margin, net income and total research and development expenses by product candidate or program to assess performance, forecast future financial results and to allocate resources.

The following table presents selected financial information with respect to the Company's single operating segment for the three and six months ended June 30, 2025, and 2024:

	Three Months Ended		Six Months Ended	
(in thousands)	**June 30, 2025**	**June 30, 2024**	**June 30, 2025**	**June 30, 2024**
Product revenue, net	$ 96,042	$ 70,284	$ 184,225	$ 115,535
Cost of goods sold	7,165	6,009	12,193	8,428
Gross margin	93 %	91 %	93 %	93 %
B-VEC	2,378	3,594	4,353	5,815
KB301	24	223	62	383
KB304	424	823	667	958
KB407	307	713	655	1,451
KB408	219	249	516	495
KB707	2,413	1,770	5,147	3,156
KB801	426	155	879	205
KB803	408	214	894	214
Other dermatology programs	706	21	744	35
Other ophthalmology programs	15	243	42	293
Other research programs	351	393	745	643
Other development programs	233	193	459	425
Other research and development costs [(1)]	6,506	6,992	13,503	12,465
Total research and development	14,410	15,583	28,666	26,539
Selling, general and administrative	35,160	27,626	67,883	53,685
Litigation settlement	—	12,500	—	25,000
Income from operations	$ 39,307	$ 8,566	$ 75,483	$ 1,883
Other income				
Interest and other income, net	7,468	7,479	14,889	15,095
Income before income taxes	$ 46,775	$ 16,045	$ 90,372	$ 16,978
Income tax expense	$ (8,442)	$ (477)	$ (16,305)	$ (477)
Net income	$ 38,333	$ 15,568	$ 74,067	$ 16,501

(1) Includes stock-based compensation, other manufacturing expenses related to our product candidates and other unallocated expenses which largely relates to depreciation and other facilities and equipment related costs.

12. Subsequent Events

On July 4, 2025, the One Big Beautiful Bill Act was signed into law in the United States. This legislation includes changes to U.S. federal tax law, which may be subject to further clarification and the issuance of interpretive guidance. The Company is currently in the process of assessing the legislation and its effect on our consolidated financial statements, which we expect to account for in the applicable period.

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 additional subsequent events have occurred, that would require recognition or disclosure in the condensed consolidated financial statements except as discussed above.

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, 2024 ("2024 10-K"), as filed with the SEC on February 19, 2025.

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.

Forward-looking statements appearing in a number of places throughout this Quarterly Report on Form 10-Q include, but are not limited to, statements about the following, among other things:

- our commercialization plans in the United States, the European Union ("EU"), and Japan for our first commercial product, VYJUVEK® (beremagene geperpavec-svdt), which was approved by the U.S. Food and Drug Administration ("FDA") in May 2023, the European Commission ("EC") in April 2025, and Japan's Ministry of Health, Labour and Welfare ("MHLW") in July 2025 for the treatment of dystrophic epidermolysis bullosa ("DEB");
- our plans and expected timing of commercial launch of VYJUVEK in Europe and Japan;
- our plans for commercialization of B-VEC outside of the United States, major European markets, and Japan;
- the initiation, timing, progress, and results of clinical trials for our product candidates;
- the timing of regulatory filings;
- our expectations regarding revenue, research and development expenses, selling, general and administrative expenses, and our primary uses of capital;
- our plans and ability to successfully identify, develop and commercialize our product candidates;
- our commercialization, marketing, and manufacturing capabilities and strategy; and
- our business model and strategic plans for our business, product candidates and technology.

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

Forward-looking statements represent our management's beliefs and assumptions only as of the date of filing this Quarterly Report on Form 10-Q with the SEC. Except as required by law, we assume no obligation to update these forward-looking statements publicly as a result of subsequent events, developments or otherwise, or to update the reasons actual results could differ materially from those anticipated in these forward-looking statements, even if new information becomes available in the future.

Throughout this Quarterly Report on Form 10-Q, unless the context requires otherwise, all references to "Krystal," "the Company," "we," "our," "us" or similar terms refer to Krystal Biotech, Inc., together with its consolidated subsidiaries. Web links throughout this document are provided for convenience only and are not intended to be active hyperlinks to the

referenced websites. No content on the referenced websites shall be deemed incorporated by reference into this Quarterly Report on Form 10-Q.

Overview

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

Our Commercial Product

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

VYJUVEK is a non-invasive, topical, redosable gene therapy approved in the United States, Europe, and Japan for the treatment of 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 designed to deliver two copies of the *COL7A1* gene when applied directly to DEB wounds, providing the patient's skin cells the template to make normal type VII collagen protein and thereby addressing the fundamental disease-causing mechanism.

On July 24, 2025, Japan's MHLW approved VYJUVEK for the treatment of patients with DEB, starting from birth. With this approval, VYJUVEK is now the first and only genetic medicine approved in Japan for the treatment of DEB. The Japanese approval allows for dosing at home or in a healthcare setting, with the option for administration by patients or their family members. As per the approval issued by the MHLW, VYJUVEK is intended for use only in patients with a definite diagnosis of dystrophic epidermolysis bullosa. Genetic testing is not a requirement for treatment. The re-examination period for VYJUVEK in Japan is ten years.

VYJUVEK was also approved in Europe earlier this year. On April 23, 2025, the EC granted marketing authorization to VYJUVEK for the treatment of wounds in patients with DEB who have mutations in the *COL7A1* gene, starting from birth. VYJUVEK is currently the only corrective medicine approved in Europe for the treatment of DEB wounds. The approval granted by the EC also allows for flexible VYJUVEK dosing either at home or in a healthcare setting, with the option for patient or caregiver administration if deemed appropriate by a healthcare professional ("HCP").

Previously, in May 2023, the FDA approved VYJUVEK, the first ever redosable gene therapy, for the treatment of wounds in patients, six months of age or older, suffering from DEB. VYJUVEK is the first and only corrective medicine approved by the FDA for the treatment of both recessive and dominant subtypes of DEB, and is approved in the United States for administration by a HCP in either a clinical setting or in the home.

We possess exclusive rights to develop, manufacture, and commercialize VYJUVEK and all our pipeline candidates throughout the world and intend to commercialize VYJUVEK directly in the United States, major European markets, and Japan. We currently sell VYJUVEK in the United States to a limited number of specialty pharmacy providers that mix the medication to be administered by a HCP in either a healthcare or home setting and to a limited number of hospitals or specialty distributors who deliver to hospitals where patients are administered the medication in a healthcare setting. We intend to and have started entering into distribution arrangements with specialty distributors to commercialize VYJUVEK outside of the United States, in major European markets, and in Japan.

Net VYJUVEK product revenue was $96.0 million for the three months ended June 30, 2025, and $525.4 million in cumulative net product revenue since launch.

Gross margin for the three months ended June 30, 2025 was 93%. We define gross margin as product revenue, net less cost of goods sold expressed as a percentage of product revenue, net.

We seek to make the experience of starting and continuing on VYJUVEK treatment seamless for the patient. Since launch in the United States, infrastructure has been in place for patients to be treated in their homes by a HCP, reducing the need for regular visits to a clinic or hospital. Krystal Connect™, our United States in-house patient services call center, has been active since FDA approval and assists patients, care givers, and HCPs interested in accessing VYJUVEK.

Preparations and infrastructure buildout are underway to support our planned direct commercial launch in key European markets and Japan in 2025, starting with our first European launch in Germany expected in the third quarter of 2025.

Pipeline Highlights and Recent Developments

Respiratory

KB407 for Cystic Fibrosis ("CF")

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 CF, a serious rare lung disease caused by missing or mutated CFTR protein. In July 2023, we announced that we had dosed the first patient in CORAL-1, a Phase 1 multi-center, dose-escalation study evaluating KB407, delivered via a nebulizer, in patients with CF, regardless of their underlying genotype. In December 2024, we announced an interim safety data update for patients treated with KB407 in the first two dose escalation cohorts, in which we found single and repeat inhaled administration of KB407 to be safe and well tolerated. In January 2025, the Cystic Fibrosis Foundation Therapeutic Development Network Clinical Research Executive Committee granted full sanctioning of our KB407 Phase 1 CORAL-1 study protocol. Four patients have been enrolled in the third and final cohort of CORAL-1 and enrollment is ongoing. We expect to report safety and *CFTR* delivery data from patients in the third and final cohort before year end. Details of the Phase 1 study can be found at www.clinicaltrials.gov under NCT identifier NCT05504837.

KB408 for Alpha-1 Antitrypsin Deficiency ("AATD") Lung Disease

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 ("AAT") protein, for the treatment of AATD, a serious rare lung disease. In February 2024, we announced that we dosed the first patient in SERPENTINE-1, a Phase 1, open-label, single dose escalation study evaluating KB408, delivered via a nebulizer, in adult patients with AATD with a Pi*ZZ or a Pi*ZNull genotype. In December 2024, we announced an interim clinical update from the first two dose escalation cohorts of SERPENTINE-1. Inhaled KB408 was safe and well-tolerated at both tested dose levels and clear evidence of successful *SERPINA1* delivery and AAT expression was observed in both patients that underwent bronchoscopies. We have since confirmed *SERPINA1* delivery and functional AAT expression in a third patient dosed with KB408 in Cohort 2 and amended the SERPENTINE-1 protocol to investigate repeat dosing at the Cohort 2 dose level (the repeat dose cohort now referred to as "Cohort 2B"). A total of five patients were dosed in Cohort 2 of which three underwent bronchoscopy. The first patient in Cohort 2B was dosed earlier this month and enrollment in this repeat dose cohort is ongoing. Enrollment in single dose cohorts is now closed. Details of the Phase 1 study can be found at www.clinicaltrials.gov under NCT identifier: NCT06049082.

Ophthalmology

KB803 for Ocular Complications in Patients with DEB

KB803 is a redosable eye drop formulation of B-VEC, designed for the treatment of ocular complications that are thought to affect over 25% of DEB patients. These complications, which include corneal erosions, abrasions, blistering and scarring, can lead to progressive vision loss. There is currently no corrective therapy available.

B-VEC has been applied topically to the eye of one DEB patient under a compassionate use protocol. The clinical observations of this compassionate use case were published in the *New England Journal of Medicine* in February 2024. The patient presented with severe cicatrizing conjunctivitis secondary to DEB. Surgical symblepharon lysis of the patient's right eye with pannus removal was conducted and regular administration of B-VEC as an eye drop directly to the eye (5×10^9 PFU/mL) were added to routine post-surgical care, three times weekly for the first two weeks and then once weekly. B-VEC application frequency was further decreased to once monthly once the corneal epithelium was healed. B-VEC was well tolerated with no drug-related adverse events noted. Full corneal healing was observed at three months, as well as significant visual acuity improvement from hand motion to 20/25 by eight months.

In June 2025, we announced that we dosed the first patient in IOLITE, an intra-patient, double-blind, placebo-controlled, multicenter Phase 3 study with a crossover design to evaluate KB803 for the treatment and prevention of corneal abrasions in DEB patients, six months of age or older. We expect to enroll approximately 16 patients in the IOLITE study. Enrolled patients will initially receive either a single eye drop of placebo or KB803, at a concentration of 10^9 PFU/mL, to each eye once weekly for 12 weeks. At the conclusion of the first 12 weeks, patients will be switched from placebo to KB803, or vice versa, and continue with once weekly administration for a second 12 week period. IOLITE is a decentralized study and drug administration will occur at the patient's home by a HCP. The primary study endpoint will be the change in the average number of days per month with corneal abrasion symptoms while receiving KB803 versus placebo. Safety and secondary efficacy data, including weekly assessments of eye pain and monthly Epidermolysis Bullosa Eye Disease Index questionnaires, will be collected through to the end of the 24-week study period. Enrollment in IOLITE is ongoing. More details of the IOLITE study can be found at www.clinicaltrials.gov under NCT identifier NCT07016750.

Patients seeking to participate in IOLITE must first enroll in an ongoing natural history study and complete a 12-week run-in period, during which they report the number of days that they experience symptoms of corneal abrasions. Patients meeting the inclusion criteria following the 12-week run-in are eligible to participate in the IOLITE trial. The natural history study was initiated in August 2024 and remains open for enrollment. As of June 2025, we had enrolled 48 patients in the study. Details of the natural history study can be found at www.clinicaltrials.gov under NCT identifier NCT06563414.

KB801 for Neurotrophic Keratitis ("NK")

KB801 is an eye drop formulation of our novel HSV-1 based vector designed to deliver two transgene copies to the corneal epithelium for the sustained, localized expression and secretion of nerve growth factor ("NGF") and treatment of NK, a rare, degenerative corneal disease caused by nerve damage in the eye that leads to corneal epithelial defects, ulcers, and perforation. Recombinant NGF eye drops have been shown to significantly improve corneal healing and are approved for the treatment of NK in multiple jurisdictions worldwide, including the United States, but rapid clearance from the eye requires intensive administration six times a day, with eye pain frequently reported, and may result in suboptimal treatment outcomes. In preclinical studies presented the Association for Research in Vision and Ophthalmology 2025 Annual Meeting in May 2025, KB801 was shown to efficiently transduce corneal epithelial cells *in vitro* and *in vivo* leading to sustained NGF production in the front of the eye. By transducing the cells of the corneal epithelium to produce and secrete NGF, KB801 has the potential to significantly reduce the treatment burden for patients while also maintaining more consistent NGF levels in the front of the eye.

In July 2025, we announced that we dosed the first patient in EMERALD-1, a randomized, double-masked, multicenter, placebo-controlled Phase 1/2 study evaluating KB801, administered as an eye drop, for the treatment of NK. We expect to enroll up to 27 adult patients with Stage 2 or Stage 3 NK, as defined by the Mackie criteria, in the study. Patients will be randomized 2:1 to receive either KB801, at a concentration of 10^{10} PFU/mL, or placebo topically to the study eye twice weekly for eight weeks. The primary objective of EMERALD-1 is to evaluate the safety and tolerability of topical ocular administration of KB801 in patients with NK. The secondary objective is evaluation of efficacy based on the proportion of patients with complete durable healing of corneal epithelium at eight weeks. Additional exploratory efficacy measures will include change in corneal lesion size from baseline, each assessed at weeks 4, 6, 8, 10, and 20, as well as evaluations of corneal sensation and patient-reported symptom burden. Enrollment in EMERALD-1 is ongoing. More details of the EMERALD-1 study can be found at www.clinicaltrials.gov under NCT identifier NCT06999733.

Oncology

KB707 for Solid Tumors

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

Inhaled KB707 is currently under evaluation in KYANITE-1, an open-label, multi-center, dose escalation and expansion Phase 1/2 study, evaluating inhaled KB707, as monotherapy or in combination, in patients with locally advanced or metastatic solid tumors of the lung. In December 2024 and June 2025, we announced clinical updates for the monotherapy dose escalation and expansion cohorts of KYANITE-1. Early evidence of monotherapy activity was observed in the evaluable cohort of 11 patients with heavily pre-treated advanced non-small cell lung cancer, achieving an objective response rate of 36% and a disease control rate of 54% as of the latest data cut-off. Inhaled KB707 was also reported to be safe and generally well tolerated as monotherapy in the 39 patients included in the safety analysis. The majority of treatment-related adverse events have been mild to moderate in severity and transient with no Grade 4 or 5 adverse events observed. Enrollment in KYANITE-1 is ongoing. Details of the Phase 1/2 study can be found at www.clinicaltrials.gov under NCT identifier NCT06228326.

Intratumoral KB707 is currently under evaluation in OPAL-1, an open-label, multi-center, dose escalation and expansion Phase 1/2 study, evaluating intratumoral KB707, as monotherapy or in combination, in patients with locally advanced or metastatic solid tumors, who relapsed or are refractory to standard of care, with at least one measurable and injectable tumor accessible by transcutaneous route of administration. The final monotherapy dose escalation cohort was cleared in May 2024 and enrollment in OPAL-1 is ongoing. Details of the Phase 1/2 study can be found at www.clinicaltrials.gov under NCT identifier NCT05970497.

Dermatology

KB105 for Lamellar Ichthyosis ("LI")

KB105 is a topical gel containing our novel vector designed to deliver two copies of the *TGM1* transgene encoding the human enzyme transglutaminase-1 ("TGM1") for the treatment of LI, a serious rare skin disorder most often caused by missing or mutated TGM1 protein. We expect to resume enrollment in the Phase 2 portion of JADE-1, a randomized, placebo-controlled Phase 1/2 study evaluating KB105 for the treatment of LI in 2026. Details of the JADE-1 Phase 1/2 study can be found at www.clinicaltrials.gov under NCT identifier NCT04047732.

Pipeline Expansion

We presented preclinical data at the Society for Investigative Dermatology 2025 Annual Meeting in May 2025 on early-stage dermatology genetic medicine candidates for the treatment of Hailey-Hailey and Darier diseases.

Aesthetics

In addition to focusing on genetic medicines to treat patients with diseases with high unmet medical needs, we are leveraging the ability of our platform to deliver proteins of interest to cells in the skin in the context of aesthetic medicine via our wholly-owned subsidiary, Jeune Aesthetics, Inc. ("Jeune"). We recently expanded the senior leadership team at Jeune, with Marc Forth joining as Jeune CEO in April 2025 and Nishant Saxena joining as Jeune CFO in January 2025.

In July 2025, Jeune announced positive safety and efficacy results, including significant improvements in key skin aesthetic attributes such as wrinkles and elasticity, in PEARL-2, a 2:1 randomized, double-blind, placebo-controlled Phase 1 study evaluating KB304, for the treatment of wrinkles of the décolleté. KB304 is a solution formulation of our novel vector for intradermal injection designed to deliver two copies of the *COL3A1* transgene and one copy of the *ELN* transgene to address various signs of skin aging including elasticity loss. Meaningful aesthetic improvements in multiple skin attributes were reported by the investigator and subjects alike following KB304 treatment, with clear and statistically significant advantages over placebo. Improvements were reported not only for wrinkles but also multiple additional skin attributes, including elasticity, crepiness, hydration, and radiance. Increased subject satisfaction with wrinkle appearance was also reported, with clear separation from placebo. All adverse events were mild-to-moderate in severity and transient. The frequency and duration of adverse events also decreased with subsequent doses of KB304. No serious or severe adverse events were reported. Details of the study can be found at www.clinicaltrials.gov under NCT identifier NCT06724900.

Based on the broad aesthetic improvements observed following KB304 treatment in PEARL-2, Jeune has selected KB304 for progression into Phase 2 study for the treatment of wrinkles of the décolleté. In support of the Phase 2 study, Jeune recently completed development and validation of a décolleté-specific photonumeric scale ("JDWS"). Jeune intends to submit the JDWS to the FDA and align on the Phase 2 study protocol in the second half of 2025. Jeune currently expects to initiate the Phase 2 study in the first half of 2026.

Jeune's second clinical-stage program, 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 August 2024, Jeune announced positive interim safety and efficacy results from Cohorts 3 and 4 of the Phase 1 study PEARL-1, open label studies evaluating KB301 in the treatment of lateral canthal lines at rest and dynamic wrinkles of the décolleté, respectively. Meaningful and sustained improvements in multiple skin aesthetic attributes, as well as increased subject satisfaction with wrinkle appearance, were reported. Details of the Phase 1 study can be found at www.clinicaltrials.gov under NCT identifier NCT04540900. Jeune is currently evaluating aesthetic indications most suitable for the advanced clinical development of KB301.

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

Financial Overview

Product Revenue, Net

After FDA approval of VYJUVEK in May 2023, we began commercial marketing and sales and began recognizing revenue during the third quarter of 2023. Our future revenue will fluctuate from quarter to quarter for many reasons, including the uncertain timing and amount of any such sales. The transaction price that we recognize as revenue for VYJUVEK sales includes an estimate of variable consideration, which includes discounts, returns, copay assistance and rebates that are offered within contracts. Refer to Note 3 of the notes to the condensed consolidated financial statements included in this Quarterly Report on Form 10-Q for additional information.

Cost of Goods Sold

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

Research and Development Expenses

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

- agreements with contract research organizations, consultants and other third parties that conduct preclinical activities, clinical trials and other research and development activities on our behalf;
- costs of acquiring, developing and manufacturing product candidates and clinical trial materials, lab supplies and consumables;
- facility costs, depreciation and other related expenses, which include expenses for rent and the maintenance of our facilities;
- other testing and support costs and supplies; and
- payroll related expenses, including stock-based compensation expense.

We expense research and development costs to operations as incurred.

A significant portion of our research and development expenses are not allocated to individual products or programs, as certain expenses benefit multiple product candidates and preclinical development programs. For example, we do not allocate costs associated with stock-based compensation, certain manufacturing related costs, rent, storage, depreciation, or other facility related costs.

We expect our research and development expenses will increase as we continue the manufacturing of preclinical and clinical materials, manage the clinical trials of, and seek regulatory approval for our product candidates and as we expand our product portfolio. Due to the numerous risks and uncertainties associated with product development, we cannot determine with certainty the duration, costs and timing of clinical trials, and as a result, the actual costs to complete clinical trials may exceed the expected costs.

Selling, General and Administrative Expenses

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

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

Interest and Other Income, Net

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

Critical Accounting Policies, Significant Judgments and Estimates

There have been no significant changes during the six months ended June 30, 2025 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 2024 Form 10-K.

Results of Operations

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

Three Months Ended June 30, 2025 and 2024

	Three Months Ended June 30,		Change	
	2025	2024	$	%
(in thousands)	(unaudited)			
Product revenue, net	$ 96,042	$ 70,284	$ 25,758	37 %
Expenses				
Cost of goods sold	7,165	6,009	1,156	19 %
Research and development	14,410	15,583	(1,173)	(8)%
Selling, general and administrative	35,160	27,626	7,534	27 %
Litigation settlement	—	12,500	(12,500)	(100)%
Total operating expenses	56,735	61,718	(4,983)	(8)%
Income from operations	39,307	8,566	30,741	359 %
Other income				
Interest and other income, net	7,468	7,479	(11)	— %
Income before income taxes	46,775	16,045	30,730	192 %
Income tax expense	(8,442)	(477)	(7,965)	1670 %
Net income	$ 38,333	$ 15,568	$ 22,765	146 %

Product Revenue, Net

Product revenue, net was $96.0 million for the three months ended June 30, 2025, as compared to $70.3 million for the three months ended June 30, 2024. The increase in product revenue, net was driven by an increase in VYJUVEK sales as compared to the prior year.

Cost of Goods Sold

Cost of goods sold was $7.2 million for the three months ended June 30, 2025, as compared to $6.0 million for the three months ended June 30, 2024, due to an increase in VYJUVEK sales as compared to the prior year.

Research and Development Expenses

The following table summarizes our research and development expenses by product candidate or program, and for unallocated expenses, by type, for the three months ended June 30, 2025 and 2024.

	Three Months Ended June 30,		Change	
	2025	2024	$	%
(in thousands)	(unaudited)			
B-VEC	$ 2,378	$ 3,594	$ (1,216)	(34)%
KB301	24	223	(199)	(89)%
KB304	424	823	(399)	(48)%
KB407	307	713	(406)	(57)%
KB408	219	249	(30)	(12)%
KB707	2,413	1,770	643	36 %
KB801	426	155	271	175 %
KB803	408	214	194	91 %
Other dermatology programs	706	21	685	3262 %
Other ophthalmology programs	15	243	(228)	(94)%
Other research programs	351	393	(42)	(11)%
Other development programs	233	193	40	21 %
Stock-based compensation	2,627	2,772	(145)	(5)%
Other unallocated expenses[(1)]	3,879	4,220	(341)	(8)%
Research and development expense	$ 14,410	$ 15,583	$ (1,173)	(8)%

(1) Unallocated expenses consist of shared pre-commercial manufacturing costs, primarily relating to certain raw materials, process development, quality control and quality assurance activities, as well as other manufacturing and facility related costs including rent, storage and depreciation which support the development of multiple product candidates.

Research and development expenses decreased by $1.2 million for the three months ended June 30, 2025 as compared to the three months ended June 30, 2024. The decrease in research and development expenses was primarily attributable to:

- a net decrease of $1.6 million in manufacturing costs driven by the scheduling of production runs across our product candidates and programs, including payroll related expenses to support our research and development, primarily due to a decrease in B-VEC, KB304, KB407, KB707 and other unallocated expenses offset by an increase in other dermatology expenses.

This decrease was partially offset by:

- a net increase of $582 thousand in preclinical and clinical development costs, primarily due to an increase in KB707 costs related to our ongoing Phase 1/2 clinical trials for inhaled and intratumoral KB707.

Selling, General and Administrative Expenses

Selling, general and administrative expenses increased $7.5 million in the three months ended June 30, 2025 as compared to the three months ended June 30, 2024. The increase was primarily driven by the following:

- an increase of $3.3 million related to professional services, including legal and consulting services;
- an increase of $1.6 million in payroll-related costs, inclusive of $1.1 million in stock-based compensation, primarily driven by additional grants awarded during the year and an increase in headcount;
- an increase of $1.5 million in marketing costs to support commercial sales of VYJUVEK; and
- an increase of $1.3 million in other G&A costs, including $461 thousand in other taxes, $245 thousand in facilities expenses, and $243 thousand in subscription expense.

The increases were partially offset by:

- a decrease of $214 thousand in selling expenses related to our commercial launch of VYJUVEK, primarily related to our patient access program.

Litigation Settlement

Litigation settlement for the three months ended June 30, 2025 and 2024 was zero and $12.5 million, respectively, and consisted of amounts related to the settlement of litigation with PeriphaGen. See discussion in Note 7 of the notes to consolidated financial statements included in the December 31, 2024 Annual Report on Form 10-K and
in Item 1 of Part II of this Form 10-Q for more information.

Interest and Other Income, Net

Interest and other income, net was $7.5 million for both of the three months ended June 30, 2025 and 2024, and consisted of interest and dividend income earned from our cash, cash equivalents and investments. The increase in investment activity for the three months ended June 30, 2025 was offset by less favorable interest rates on investments as compared to the prior period.

Income Tax Expense

Income tax expense for the three months ended June 30, 2025 and 2024 was $8.4 million and $477 thousand, respectively, which relates to state, federal and foreign income taxes.

Six Months Ended June 30, 2025 and 2024

	Six Months Ended June 30,		Change	
	2025	2024	$	%
(in thousands)	(unaudited)			
Product revenue, net	$ 184,225	$ 115,535	$ 68,690	59 %
Expenses				
Cost of goods sold	12,193	8,428	3,765	45 %
Research and development	28,666	26,539	2,127	8 %
Selling, general and administrative	67,883	53,685	14,198	26 %
Litigation settlement	—	25,000	(25,000)	(100)%
Total operating expenses	108,742	113,652	(4,910)	(4)%
Income from operations	75,483	1,883	73,600	3909 %
Other income				
Interest and other income, net	14,889	15,095	(206)	(1)%
Income before income taxes	90,372	16,978	73,394	432 %
Income tax expense	(16,305)	(477)	(15,828)	3318 %
Net income	$ 74,067	$ 16,501	$ 57,566	349 %

Products Revenue, net

Product revenue, net was $184.2 million for the six months ended June 30, 2025 as compared to $115.5 million for the six months ended June 30, 2024. The increase in product revenue, net was driven by an increase in VYJUVEK sales as compared to the prior year.

Cost of Goods Sold

Cost of goods sold was $12.2 million for the six months ended June 30, 2025 as compared to $8.4 million for the six months ended June 30, 2024, due to an increase in VYJUVEK sales as compared to the prior year.

Research and Development Expenses

The following table summarizes our research and development expenses by product candidate or program, and for unallocated expenses, by type, for the six months ended June 30, 2025 and 2024:

	Six Months Ended June 30,		Change	
	2025	2024	$	%
(in thousands)	(unaudited)			
B-VEC	$ 4,353	$ 5,815	$ (1,462)	(25)%
KB301	62	383	(321)	(84)%
KB304	667	958	(291)	(30)%
KB407	655	1,451	(796)	(55)%
KB408	516	495	21	4 %
KB707	5,147	3,156	1,991	63 %
KB801	879	205	674	329 %
KB803	894	214	680	318 %
Other dermatology programs	744	35	709	2026 %
Other ophthalmology programs	42	293	(251)	(86)%
Other research programs	745	643	102	16 %
Other development programs	459	425	34	8 %
Stock-based compensation	5,096	4,640	456	10 %
Other unallocated expenses[(1)]	8,407	7,826	581	7 %
Research and development expense	$ 28,666	$ 26,539	$ 2,127	8 %

(1) Unallocated expenses consist of shared pre-commercial manufacturing costs, primarily relating to certain raw materials, process development, quality control and quality assurance activities, as well as other manufacturing and facility related costs including rent, storage and depreciation which support the development of multiple product candidates.

Research and development expenses increased by $2.1 million in the six months ended June 30, 2025 as compared to the six months ended June 30, 2024. The increase in research and development expenses was primarily attributable to:

- a net increase of $1.4 million in clinical development costs, primarily due to an increase in KB707 costs related to our Phase 1/2 clinical trials for inhaled and intratumoral KB707; and
- an increase of $605 thousand facilities costs and equipment costs included in other unallocated expenses.

These increases were partially offset by:

- a net decrease of $382 thousand in manufacturing costs mainly driven by the scheduling of production runs across our product candidates and programs, including payroll related expenses to support our research and development, primarily due to a decrease in B-VEC and KB407 costs offset by increases in KB801, KB803 and other dermatology programs costs.

Selling, General and Administrative Expenses

Selling, general and administrative expenses increased $14.2 million in the six months ended June 30, 2025 as compared to the six months ended June 30, 2024. The increase was primarily driven by the following:

- an increase of $5.9 million in payroll-related expenses, including stock-based compensation, primarily driven by an increase in headcount to support our growth;
- an increase of $4.5 million related to professional services, including legal and consulting services;
- an increase of $3.2 million in other G&A costs, including $1.0 million in charitable contributions; $503 thousand in facilities expenses, $432 thousand in subscription expenses, $471 thousand in other taxes, and $318 thousand in insurance expenses; and
- an increase of $1.7 million in marketing costs to support commercial sales of VYJUVEK.

The increases were partially offset by:

- a decrease of $1.1 million in selling expenses related to our commercial launch of VYJUVEK, primarily related to our patient access program.

Litigation Settlement

Litigation settlement for the six months ended June 30, 2025 and 2024 was zero and $25.0 million, respectively, and consisted of amounts related to the settlement of litigation with PeriphaGen. See discussion in Note 7 of the notes to consolidated financial statements included in the December 31, 2024 Annual Report on Form 10-K and
in Item 1 of Part II of this Form 10-Q for more information.

Interest and Other Income, Net

Interest and other income, net for the six months ended June 30, 2025 and 2024 was $14.9 million and $15.1 million, respectively, and consisted of interest and dividend income earned from our cash, cash equivalents and investments. The decrease in interest and dividend income is primarily the result of less favorable interest rates on investments as compared to the prior period.

Income Tax Expense

Income tax expense for the six months ended June 30, 2025 and 2024 was $16.3 million and $477 thousand, respectively, which relates to state, federal and foreign income taxes.

Liquidity and Capital Resources

Overview

As of June 30, 2025, our cash, cash equivalents and short-term investments balance was approximately $682.0 million. As of June 30, 2025, we had an accumulated deficit of $106.6 million. We believe that our cash, cash equivalents and short-term investments as of June 30, 2025 will be sufficient to allow us to fund our operations for at least 12 months from the filing date of this Quarterly Report on Form 10-Q.

Our operating profitability is dependent upon the continued successful commercialization of VYJUVEK, our U.S. Food and Drug Administration ("FDA"), European Medicines Agency ("EMA"), and Japan's Ministry of Health, Labour, and Welfare ("MHLW") approved product, as well as successful development, approval and commercialization of our product candidates. Management intends to fund future operations through its on hand cash and cash equivalents and revenue generated from the sale of VYJUVEK, and may also seek additional capital through arrangements with strategic partners, the sale of equity, debt financings or other sources.

Costs related to clinical trials can be unpredictable and, therefore, there can be no guarantee that we will have sufficient capital to fund the continued or planned pre-clinical and clinical studies for our product candidates, or our operations. Further, we expect future revenue to fluctuate from quarter to quarter for many reasons, including the uncertain timing and amount of any product sales. While we are in the process of building out our internal vector manufacturing capacity, some of our manufacturing activities will be contracted out to third parties. Additionally, we currently utilize third-party contract research organizations to carry out some of our clinical development activities. As we seek to obtain regulatory approval for our product candidates, we expect to continue to incur significant manufacturing and commercialization expenses as we prepare for product sales, marketing, commercial manufacturing, packaging, labeling and distribution. Our funds may not be sufficient to enable us to conduct pivotal clinical trials for, seek marketing approval for or commercially launch our product candidates. Accordingly, to obtain marketing approval for and to commercialize these or any other product candidates, we may be required to obtain further funding through public or private equity offerings, debt financings, collaboration and licensing arrangements or other sources. Adequate additional financing may not be available to us on acceptable terms, if at all. Our failure to raise capital when needed could have a negative effect on our financial condition and our ability to pursue our business strategy.

Operating Capital Requirements

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

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

- the costs needed to globally commercialize and market our lead product, VYJUVEK;

- the progress, timing and costs of clinical trials of our current product candidates;
- the progress, timing and costs of manufacturing VYJUVEK and revenue received from commercial sale of VYJUVEK;
- the continued development and the filing of investigational new drug applications for current and future product candidates;
- the initiation, scope, progress, timing, costs and results of drug discovery, laboratory testing, manufacturing, preclinical studies and clinical trials for any product candidates that we may pursue in the future, if any;
- the costs of maintaining our own commercial-scale CGMP manufacturing facilities;
- the outcome, timing and costs of seeking regulatory approvals;
- the costs associated with the manufacturing process development and evaluation of third-party manufacturers;
- the extent to which the costs of VYJUVEK and our product candidates, if approved, will be paid by health maintenance, managed care, pharmacy benefit and similar healthcare management organizations, or will be reimbursed by government authorities, private health coverage insurers and other third-party payors;
- the costs of commercialization activities for our current and future product candidates if we receive marketing approval for such product candidates, including the costs and timing of establishing product sales, medical affairs, marketing, distribution and manufacturing capabilities;
- the revenue received from commercial sale of our current and future product candidates, subject to receipt of marketing approval;
- 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, 2025 and 2024:

	Six Months Ended June 30,	
	2025	**2024**
(in thousands)	**(unaudited)**	
Net cash provided by operating activities	$ 83,698	$ 11,720
Net cash (used in) investing activities	(65,294)	(45,333)
Net cash (used in) provided by financing activities	(10,670)	21,221
Effect of exchange rate changes on cash and cash equivalents	1,230	(150)
Net increase (decrease) in cash	$ 8,964	$ (12,542)

Operating Activities

Net cash provided by operating activities for the six months ended June 30, 2025 was $83.7 million and consisted primarily of net income of $74.1 million adjusted for $27.7 million of non-cash items and a $18.0 million decrease in cash from increased working capital. Non-cash adjustments included depreciation of $2.8 million, amortization of operating lease right-of-use assets of $418 thousand, stock-based compensation expense of $27.6 million, amortization on marketable securities of $464 thousand, and other adjustments of $690 thousand, offset by realized gain on investments of $4.3 million..

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

Investing Activities

Net cash used in investing activities for the six months ended June 30, 2025 was $65.3 million and consisted of $251.7 million in purchases of short-term and long-term investments and $8.1 million in purchases of property and equipment, offset by $194.1 million received from the maturities and early calls of short and long-term investments and $435 thousand received in proceeds from disposal of assets.

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

Financing Activities

Net cash used in financing activities for the six months ended June 30, 2025 was $10.7 million and consisted of $12.1 million used for employee tax withholding payments related to vested restricted stock units and $1.8 million used for employee tax withholding payments for settlement of vested restricted stock awards partially offset by $3.3 million from exercises of stock options.

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

ITEM 3. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK

We had cash, cash equivalents and short-term investments of $682.0 million as of June 30, 2025, which consisted primarily of money market funds, commercial paper, corporate bonds and U.S. government agency securities. The investments in these financial instruments are made in accordance with an investment policy which specifies the categories, allocations and ratings of securities we may consider for investment. The primary objective of our investment activities is to preserve principal while at the same time maximizing the income we receive without significantly increasing risk. Some of the financial instruments in which we invest could be subject to market risk. This means that a change in prevailing interest rates may cause the value of the instruments to fluctuate. For example, if we purchase a security that was issued with a fixed interest rate and the prevailing interest rate later rises, the value of that security will probably decline. To minimize this risk, we intend to maintain a portfolio which may include cash, cash equivalents and short-term investment securities available-for-sale in a variety of securities which may include money market funds, government and non-government debt securities and commercial paper, all

with various maturity dates. Based on our current investment portfolio, we do not believe that our results of operations or our financial position would be materially affected by an immediate change of 10% in interest rates.

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

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

ITEM 4. CONTROLS AND PROCEDURES

Evaluation of Disclosure Controls and Procedures

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

Changes in Internal Control over Financial Reporting

In June 2025, we implemented a new procure to pay ("P2P") system that integrates with our existing enterprise resource planning system. As necessary, we are adding to or modifying the design and documentation of internal control processes and procedures relating to the new P2P system to simplify and strengthen our internal control over financial reporting.

Other than as discussed above, no change in our internal control over financial reporting occurred during the quarter ended June 30, 2025 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

In May 2020, PeriphaGen, Inc. ("PeriphaGen") commenced litigation against the Company alleging breach of contract and misappropriation of trade secrets. In April 2022, the Company and PeriphaGen entered into a final settlement. In accordance with the settlement agreement, the Company paid PeriphaGen total consideration of $75.0 million to settle the dispute, acquire certain assets and receive an exclusive license from PeriphaGen to certain intellectual property assets and biological materials which was paid over time upon completion of certain milestones. Refer to Note 7 of our consolidated financial statements in our 2024 10-K for additional information.

The Company recorded litigation settlement expense of zero and $12.5 million for the three months ended June 30, 2025 and 2024, respectively, on the condensed consolidated statements of operations and comprehensive income. During the three months ended March 31, 2025 and 2024, the Company paid $31.25 million and zero, respectively, and as such, the Company has fully paid the $75.0 million of total consideration discussed above.

We are not currently a party to any material legal proceedings. We may, however, in the ordinary course of business face various claims brought by third parties or government regulators and, from time to time, make claims or take legal actions to assert our rights, including claims relating to our directors, officers, stockholders, intellectual property rights, employment matters and the safety or efficacy of our products. Any of these claims could subject us to costly litigation and, while we generally believe that we have adequate insurance to cover many different types of liabilities, our insurance carriers may deny coverage, may be inadequately capitalized to pay on valid claims, or our policy limits may be inadequate to fully satisfy any damage awards or settlements. If this were to happen, the payment of any such awards could have a material adverse effect on our consolidated operations, cash flows and financial position. Additionally, any such claims, whether or not successful, could damage our reputation and business.

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 Japan's Ministry of Health, Labour and Welfare ("MHLW") approval of VYJUVEK on July 24, 2025, and should be reviewed in conjunction with "Summary Risk Factors" and "Part I, Item 1A. Risk Factors" in our 2024 10-K, and "Part II, Item 1A. Risk Factors" in our Quarterly Report on Form 10-Q for the quarter ended March 31, 2025, filed with the Securities and Exchange Commission (the "SEC") on May 6, 2025 ("Q1 2025 10-Q"). Except as set forth below, the above referenced risk factors included in our 2024 10-K and our Q1 2025 10-Q remain unchanged.

Risks Related to Our Business and Industry

We are substantially dependent on the commercial success of VYJUVEK

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

Although we received approval from the FDA, the European Commission, and Japan's MHLW for VYJUVEK for the treatment of DEB, 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 and involves risk, including some of the risks that are set forth under the caption "Risk Factors" in our annual and quarterly reports on file with the SEC. One or more of these factors, many of which are beyond our control could cause significant delays or an inability to successfully commercialize VYJUVEK.

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

Social media is increasingly being used by us, our employees, or others to communicate about our business, VYJUVEK, our clinical development programs, DEB, and the diseases our product candidates are being developed to treat. We use social media in connection with our commercialization efforts of VYJUVEK and intend to use it in connection with our commercialization efforts of our product candidates, if approved. Social media practices in the biotechnology and biopharmaceutical industries continue to evolve, and regulations and regulatory guidance relating to such use are evolving and not always clear. This evolution creates uncertainty and risk of noncompliance with regulations applicable to our business, resulting in potential regulatory actions against us, along with the potential for litigation and heightened scrutiny by the FDA, the EMA, the MHLW, the SEC, and other regulators. For example, patients may use social media channels to comment on their experience in an ongoing clinical trial of our product candidates, or to report an alleged adverse event. If such disclosures occur, there is a risk that clinical trial enrollment may be adversely impacted, that we may fail to monitor and comply with applicable adverse event reporting obligations, or that we may not be able to defend our business or the public's legitimate interests in the face of the political and market pressures generated by social media due to restrictions on what we may say about our product

candidates. There is also a risk of inappropriate disclosure of sensitive information, loss of trade secrets or other intellectual property, public exposure of personal information of our employees, patients who use VYJUVEK, clinical trial patients, and others, or negative or inaccurate posts or comments about us on any social networking website. In addition, we may encounter attacks on social media regarding our company, management, VYJUVEK, or our product candidates that seriously damage our reputation, brand image, and goodwill. If any of these events were to occur or we otherwise fail to comply with applicable regulations, we could incur liability, face regulatory actions, or incur other harm to our business that could have a material adverse effect on our business, prospects, operating results, and financial condition, and could adversely affect the price of our common stock.

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

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

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

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

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

In the United States, there have been and continue to be a number of legislative efforts to contain healthcare costs. Any legislative changes that result in price controls, reduce access to and reimbursement for care, or add additional regulations may have an adverse effect on our financial condition and results of operations. Any changes that reduce, or impede the ability to obtain, reimbursement for VYJUVEK or our product candidates that we intend to commercialize in the United States could adversely affect successful commercialization of VYJUVEK and our plans to introduce our product candidates in the United States.

The Budget Control Act of 2011, among other things, created measures for spending reductions by Congress. A Joint Select Committee on Deficit Reduction, tasked with recommending a targeted deficit reduction of at least $1.2 trillion for the years 2012 through 2021, was unable to reach required goals, thereby triggering the legislation's automatic reduction to several government programs. This includes aggregate reductions of Medicare payments to providers of up to 2% per fiscal year, which went into effect in April 2013 and will remain in effect through 2032 unless additional Congressional action is taken. In January 2013, the American Taxpayer Relief Act of 2012, among other things, further reduced Medicare payments to certain providers, and increased the time for Medicare contractors to recoup Medicare overpayments to providers from three to five years.

In August 2022, the Inflation Reduction Act ("IRA") was signed into law. The IRA includes several provisions to lower prescription drug costs for people with Medicare and reduce drug spending by the federal government. In relevant part, the IRA allows Medicare to negotiate prices for certain prescription drugs, requires drug manufacturers to pay a rebate to the federal government if prices for single-source drugs and biologicals covered under Medicare Part B and nearly all covered drugs under

Part D increase faster than the rate of inflation, caps out of pocket spending for Medicare Part D enrollees, and makes other benefit design changes to Medicare Part D intended to lower drug costs for enrollees and Medicare. Implementation of these changes began in 2023 and will continue to be implemented over the next several years. Beginning January 1, 2025, Medicare Part D enrollees now have a new annual out-of-pocket cap of $2,000 on prescription drugs. Other 2025 Medicare Part D changes include the elimination of the coverage gap phase and the replacement of the Coverage Gap Discount Program with the Manufacturer Discount Program, which requires drug manufacturers to pay a 10% discount for brand-name drugs and biologics during the initial coverage period and a 20% discount during the catastrophic phase. Multiple pharmaceutical manufacturers have challenged the law in court, largely on constitutional grounds. These suits will likely continue and the ultimate effects of such legal challenges are unclear. On April 15, 2025, President Trump issued an executive order directing the Secretary of the Department of Health and Human Services to take certain actions on drug pricing reform, including working with Congress on amendments to the IRA and rule making to establish new Medicare payment models for so-called "high-cost" prescription drugs and biological products. At this time, we continue to evaluate the effect of the IRA on our business operations and financial condition and results as the full impact of the IRA remains uncertain.

Furthermore, on July 4, 2025, legislation commonly referred to as the One Big Beautiful Bill Act was signed into law, which reduces funding to federal healthcare programs and imposes additional requirements to be eligible for healthcare, which may result in decreased access to healthcare, particularly in Medicaid programs.

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

Additional changes may affect our business, including those governing enrollment in federal healthcare programs, reimbursement changes, fraud and abuse enforcement, and expansion of new programs, such as Medicare payment for performance initiatives. Healthcare reform measures that may be adopted in the future, may result in more rigorous coverage criteria and in additional downward pressure on the price that we receive for any approved product. Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private payors. The implementation of cost containment measures or other healthcare reforms could result in reduced demand for our product and product candidates or additional pricing pressures and may adversely impact our ability to generate sufficient revenue, attain consistent profitability, or commercialize our product candidates, if approved.

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

With the FDA approval of VYJUVEK, our operations are directly, or indirectly through our prescribers, customers, and purchasers, subject to various federal and state fraud and abuse laws and regulations, including, without limitation, the federal Anti-Kickback Statute, federal civil and criminal false claims laws and the Physician Payments Sunshine Act and regulations. These laws impact, among other things, our sales, marketing, access assistance, sponsored genetic patient testing, and educational programs. In addition, we are subject to patient privacy laws by both the federal government and the states in which we conduct our business, as well as by foreign 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 (the "ACA") amended the intent requirement of the federal Anti-Kickback Statute to clarify that a person or entity does not have to have actual knowledge of this statute or specific intent to violate it;

- federal civil and criminal false claims laws and civil monetary penalty laws which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, claims for payment or approval from Medicare, Medicaid, or other government payors that are false or fraudulent. The ACA provides that a claim for items or services resulting from an Anti-Kickback Statute violation is a false claim under the 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 or attempting to execute 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 Rules ("HIPAA Rules"), which impose certain requirements relating to the privacy, security, and transmission of individually identifiable health information by certain entities subject to the HIPAA Rules, such as health plans, health care clearinghouses, and health care providers that engage in certain covered transactions, known as covered entities, as well as their business associates that perform certain services that involve the use or disclosure of individually identifiable health information for or on behalf of covered entities;
- 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 United States Centers for Medicare and Medicaid Services ("CMS") information related to: (i) payments or other "transfers of value" made to physicians and teaching hospitals, and (ii) ownership and investment interests held by physicians and their immediate family members;
- our operations in Europe and Japan may be directly or indirectly subject to European and Japanese law equivalents of each of the above U.S. federal laws, some of which may not have been applicable prior to the recent European Commission and MHLW approvals of VYJUVEK;
- U.S. 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 and foreign laws governing data privacy and security of health information, many of which differ from each other and require attention to frequently changing regulatory requirements, thus complicating compliance efforts in certain circumstances and increasing exposure to liability.

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 and drug manufacturers will resolve allegations in a settlement without admitting liability. Any such settlement could materially affect our business, financial operations, and reputation.

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

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

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

Privacy and data security have become significant areas of legal and regulatory focus in the United States, European Union, Japan, and in many other jurisdictions where we conduct or may conduct our operations. In our ordinary course of

business, we collect, receive, store, process, generate, use, transfer, disclose, make accessible, protect, secure, dispose of, transmit, and share (collectively, "process") personal information and other sensitive information, including, but not limited to, health information, individuals' financial information, as well as proprietary and confidential business data, including trade secrets, intellectual property, and sensitive third-party data (collectively, "sensitive data"). The legislative and regulatory landscape for privacy and data security continues to evolve, and there has been an increasing focus on privacy and data security issues, which may affect our business and is expected to increase our compliance costs and exposure to liability. Our data processing activities may subject us to numerous privacy and data security obligations, including, but not limited to, domestic and international laws, regulations, guidance, industry standards, external and internal privacy and security policies, and contractual requirements.

In the United States, federal, state, and local governments have enacted numerous data privacy and security laws, including data breach notification laws, personal information privacy laws, consumer protection laws, and other similar laws. Notably, HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act, imposes requirements on covered entities, as well as their business associates regarding the privacy, security, and transmission of individually identifiable health information. Further, states continue to adopt new laws or amend existing laws related to data privacy, requiring attention to frequently changing regulatory requirements. For example, and the California Consumer Privacy Act of 2018 ("CCPA") requires businesses to provide specific disclosures in their privacy notices and honor residents' privacy rights. The CCPA provides for civil penalties of up to $7,500 per violation and allows private litigants affected by certain data breaches to recover significant statutory damages. Although the CCPA does not apply to certain data that we process in the context of clinical trials, efforts to comply with the CCPA may increase our annual compliance costs and subject us to potential liability with respect to other personal information we may maintain about California residents. In addition, the California Privacy Rights Act of 2020 ("CPRA"), which came into effect on January 1, 2023, expanded the CCPA's requirements, extending it to cover personal information of business representatives and employees and the CPRA established a new regulatory agency to implement and enforce the law. Other states, such as Virginia, Nevada, Connecticut, Utah, Texas, and Colorado, have also passed comprehensive privacy laws, and similar laws are being considered in several other states, as well as at the federal and local levels, which impose similar obligations to those in the CCPA. Further, other states, such as Nevada and Washington, have enacted privacy laws specifically governing consumer health information, with Washington providing for a private right of action. Although many of these laws currently exempt certain health-related information, the laws may increase our potential liability related to our data processing activities, complicate our compliance efforts, and increase both legal risk and compliance costs for us and the third parties upon whom we rely.

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

In some circumstances, we may be unable to transfer personal information between certain jurisdictions due to data localization requirements or other limitations on cross-border data flows. Europe, Japan, and other jurisdictions have enacted laws requiring data to be localized or limiting the transfer of personal information to other countries. In particular, the European Economic Area ("EEA") and the UK have significantly restricted the transfer of personal information to the United States and other countries whose privacy laws they consider inadequate. Although there are various mechanisms that may be used to transfer personal information from the EEA and UK to the United States in compliance with the law, such as the EEA and UK's standard contractual clauses, these mechanisms are subject to legal challenges, and we may be unable to rely on these measures to lawfully transfer personal information to the United States in all cases. If there is no lawful manner for us to transfer personal information from the EEA, the UK, or other jurisdictions to the United States, or if the requirements for a legally compliant transfer are too onerous, we could face significant adverse consequences, including increased exposure to regulatory actions, substantial fines and penalties, the inability to transfer data and work with partners, vendors, and other third parties, and injunctions against our processing or transferring of personal information necessary to operate our business. Some European regulators have ordered certain companies to suspend or permanently cease certain transfers of personal information to recipients outside Europe for allegedly violating the EU GDPR's cross-border data transfer limitations. Additionally, companies that transfer personal information to recipients outside of the EEA and/or UK to other jurisdictions, particularly to the United States, are subject to increased scrutiny from regulators, individual litigants, and activist groups.

Compliance with applicable privacy and data security laws and regulations is a rigorous and time-intensive process, and we may be required to put in place additional mechanisms ensuring compliance with new data protection rules. Failure to comply with any such laws or regulations puts us at risk of facing significant fines and penalties that could adversely affect our business, financial condition, reputation, and results of our operations. Furthermore, conflicting requirements across applicable

privacy and data security laws would complicate our compliance efforts and increase both legal risk and compliance costs for us and the third parties upon whom we rely.

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

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

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

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

Companies across industries are facing increasing scrutiny from a variety of stakeholders related to their ESG, including diversity, equity, and inclusion ("DEI"), and sustainability practices. Investor advocacy groups, certain institutional investors, investment funds, and other influential investors have increasingly focused on ESG practices and have placed increasing importance on the non-financial impacts of their investments. Expectations regarding voluntary ESG initiatives and disclosures may result in increased costs (including but not limited to increased costs related to compliance, stakeholder engagement, contracting, and insurance), enhanced compliance or disclosure obligations, or other adverse impacts to our business, financial condition, or results of operations.

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

Certain market participants, including major institutional investors and capital providers, use third-party benchmarks and scores to assess companies' ESG profiles in making investment or voting decisions. Unfavorable ESG ratings could lead to increased negative investor sentiment towards us or our industry, which could negatively impact the price of our common stock. In addition, in recent years "anti-ESG" sentiment has gained momentum across the United States, with several states and Congress having proposed or enacted "anti-ESG" policies, legislation, or initiatives or issued related legal opinions, and the President having recently issued an executive order opposing DEI initiatives in the private sector. Such anti-ESG and anti-DEI-related policies, legislation, initiatives, litigation, legal opinions, and scrutiny could result in us facing additional compliance obligations, becoming the subject of investigations and enforcement actions, or sustaining reputational harm. Therefore, to the extent we take actions that are seen as positive to some investors, other investors may take issue with such actions or face regulatory pressure to refrain from investing in, or divest from, our business. To the extent ESG matters negatively impact our reputation, it may also impede our ability to compete as effectively to attract and retain employees, customers, or business partners which may adversely impact our operations.

In addition, there may be increasing levels of regulation, disclosure-related and otherwise, with respect to ESG matters. For example, in 2024 the SEC adopted new rules that require companies to provide significantly expanded climate-related disclosures in their periodic reporting. The new climate disclosure rules were the subject of multiple legal challenges,

and the SEC voluntarily stayed the climate disclosure rules pending the completion of judicial review. In March 2025, the SEC voted to cease defending the climate disclosure rules in court, and in April 2025, the U.S. Court of Appeals for the Eighth Circuit ordered the litigation challenging the SEC's climate disclosure rules to be held in abeyance. The court directed the SEC to file a status report by July 23, 2025, indicating whether it intends to review or reconsider the rules. On July 23, 2025, the SEC declined to review or reconsider the climate disclosure rules adopted in 2024, and directed the court to proceed with litigation and indicated it would not pre-judge whether it would enforce the rules if the court ultimately upholds them. Therefore, it is unknown whether the new rules will go into effect and if they do, whether there will be significant changes. If the new rules go into effect and are not substantially different than the rules adopted by the SEC, we may be required to incur significant additional costs to comply, including the implementation of significant additional internal controls processes and procedures regarding matters that have not been subject to such controls in the past, and impose increased oversight obligations on our management and board of directors. Even if the SEC rules are not adopted, states or ex-U.S. jurisdictions in which we currently or may in the future operate may also have or adopt ESG or climate-related disclosure rules requiring similar or broader disclosure obligations. These and other changes in stakeholder expectations will likely lead to increased costs as well as scrutiny that could heighten all of the risks identified in this risk factor. Additionally, our customers and suppliers may be subject to similar expectations, which may augment or create additional risks, including risks that may not be known to us.

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

On April 23, 2025, the European Commission granted marketing authorization to VYJUVEK, and we are planning for our first European launch in Germany in mid-2025. On July 24, 2025, Japan's MHLW granted marketing authorization to VYJUVEK, and we expect to launch in Japan before the end of 2025. We currently have operations and employees located outside the United States and our business strategy incorporates potential additional international expansion to target patient populations outside the United States. Doing business internationally involves a number of risks, including, but not limited to:

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

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

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

The ability of the FDA to review and approve new products can be affected by a variety of factors, including government budget and funding levels, ability to hire and retain key personnel and accept the payment of user fees, and statutory, regulatory and policy changes. Disruptions at the FDA and other agencies may also slow the time necessary for new product candidates to be reviewed and/or approved by necessary government agencies, which could adversely affect our business. For example, when the U.S. government has shut down in the past, certain regulatory agencies, such as the FDA and

the United States Securities and Exchange Commission have had to furlough critical employees and stop critical activities. If a prolonged government shutdown occurs, it could significantly impact the ability of the FDA to review and process our regulatory submissions in a timely manner, which could have a material adverse effect on our business. Further, future government shutdowns could impact our ability to access the public markets and obtain necessary capital. In addition, government funding of government agencies on which our operations may rely is subject to the political process, which is inherently fluid and unpredictable.

There remains substantial uncertainty as to how the current U.S. administration will seek to or continue to modify or revise the requirements and policies of the FDA and other regulatory agencies with jurisdiction over our product candidates. This uncertainty could present new challenges or potential opportunities as we navigate the clinical development and approval process for our product candidates. If we experience delays in obtaining approval or if we fail to obtain approval of our product candidates, the commercial prospects for our product candidates may be harmed and our ability to generate revenue could be materially impaired.

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

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

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

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

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

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

VYJUVEK, our first FDA, EMA, and MHLW-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, or BLA, is obligated to monitor and report adverse events and any failure of a product to meet the specifications in the BLA. The holder of an approved BLA also must submit new or supplemental applications and obtain FDA

approval for certain changes to the approved product, product labeling, or manufacturing process. For example, if demand for an approved product increases more than we previously estimate, we may need or desire to scale up an existing FDA-approved manufacturing process and the scaled-up manufacturing process would be subject to FDA review and approval. Advertising and promotional materials must comply with FDA rules and are subject to FDA review, in addition to other potentially applicable federal and state laws.

There remains substantial uncertainty as to how the current U.S. administration will seek to or continue to modify or revise the requirements and policies of the FDA and other regulatory agencies with jurisdiction over VYJUVEK and our product candidates. This uncertainty could present new challenges or potential opportunities as we navigate the clinical development and approval process for our product candidates. We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action or as a result of legal challenges, 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, our business could be materially harmed.

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, among other actions:

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

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

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

Risks Related to Commercialization of VYJUVEK and Our Product Candidates

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

We received FDA approval of VYJUVEK in May 2023, European Commission approval of VYJUVEK in April 2025, and MHLW approval in July 2025, and initiated a commercial launch of VYJUVEK in the United States in the second quarter of 2023. As a company, we have no prior experience commercializing a biologic. The success of our commercialization efforts

is difficult to predict and subject to the effective execution of our business plan, including, among other things, the continued development of our internal sales, marketing, manufacturing, and distribution capabilities and our ability to navigate the significant expenses and risks involved with the development and management of such capabilities. For example, our commercial launch of VYJUVEK in the United States, the European Union, or Japan 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. There is a risk that we underestimate the level of demand for a product, which could require us to change a manufacturing process to increase production yields and changes to a manufacturing process are time consuming and subject to regulatory, financial, and operational risks. If we are unsuccessful in accomplishing our objectives and executing on our business plan, or if our commercialization efforts do not develop as planned, we may not be able to successfully commercialize VYJUVEK and any future approved products, we may require significant additional capital and financial resources, we may not be profitable on a consistent basis, and we may not be able to compete against more established companies in our industry.

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

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

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

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

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

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

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

- the efficacy and safety of VYJUVEK and our product candidates as demonstrated in clinical trials;
- the potential and perceived advantages of VYJUVEK and our product candidates over alternative treatments, if available;

- the cost of VYJUVEK and our product candidates relative to alternative treatments if any are available;
- the clinical indications for which VYJUVEK and our product candidates are approved by the FDA and other regulatory authorities;
- the willingness of physicians to prescribe new therapies;
- the willingness of the target patient population to try new therapies;
- the prevalence and severity of any side effects;
- product labeling or product insert requirements of the FDA, the EMA, the MHLW, or other regulatory authorities, including any limitations or warnings contained in a product's approved labeling;
- relative convenience and ease of administration;
- the strength of marketing and distribution support;
- the timing of market introduction of competitive products;
- the availability of products and their ability to meet market demand;
- publicity concerning VYJUVEK and our product candidates or competing products and treatments;
- any restrictions on the use of VYJUVEK and our products together with other medications; and
- favorable third-party payor coverage and adequate reimbursement.

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

Risks Related to Our Financial Position and Need for Additional Capital

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

Although we generated net income starting with the year ended December 31, 2023, we previously incurred recurring losses and negative cash flows from operations since our inception. Our transition to consistent operating profitability depends on our ability to (i) successfully commercialize VYJUVEK in the United States and obtain the necessary regulatory approvals to commercialize VYJUVEK outside of the United States and then successfully commercialize VYJUVEK outside the United States, and (ii) complete the development of, and obtain the regulatory approvals necessary to successfully commercialize our product candidates with significant market potential. We have devoted substantially all our efforts to date to (i) research and development of our gene therapy platform, product candidates and our manufacturing infrastructure, and, more recently, (ii) commercializing VYJUVEK in the United States. We expect to continue to incur significant expenses for the foreseeable future and our operating results may fluctuate significantly from quarter to quarter. We anticipate that our expenses will increase substantially if, and as, we:

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

- acquire or in-license other product candidates and technologies.

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

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

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

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

None.

ITEM 3. DEFAULTS UPON SENIOR SECURITIES

None.

ITEM 4. MINE SAFETY DISCLOSURES

Not applicable.

ITEM 5. OTHER INFORMATION

Insider Trading Arrangements

During the three months ended June 30, 2025, none of our directors or officers (as that term is defined by the SEC in Rule 16a-1(f) under the Exchange Act) adopted or terminated a Rule 10b5-1 trading arrangement or a non-Rule 10b5-1 trading arrangement.

ITEM 6. EXHIBITS

Exhibit Number	
31.1	Certification of Periodic Report by Chief Executive Officer under Section 302 of the Sarbanes-Oxley Act of 2002.
31.2	Certification of Periodic Report by Chief Accounting Officer under Section 302 of the Sarbanes-Oxley Act of 2002.
32.1	Certification of Chief Executive Officer and Chief Accounting Officer Pursuant to 18 U.S.C. Section 1350 as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
101	Inline XBRL (Extensible Business Reporting Language). The following materials from this Quarterly Report on Form 10-Q for the period ended June 30, 2025, are formatted in Inline XBRL: (i) consolidated balance sheets of Krystal Biotech, Inc., (ii) consolidated statements of operations of Krystal Biotech, Inc., (iii) consolidated statements of operations and comprehensive income of Krystal Biotech, Inc., (iv) consolidated statements of changes in equity of Krystal Biotech, Inc., (v) consolidated statements of cash flows of Krystal Biotech, Inc. and (vi) notes to condensed consolidated financial statements of Krystal Biotech, Inc. The instance document does not appear in the interactive data file because its XBRL tags are embedded within the Inline XBRL document.
104	Cover Page Interactive Data File - the cover page XBRL tags are embedded within the Inline XBRL.

SIGNATURES

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

KRYSTAL BIOTECH, INC.
(Registrant)

Date: August 4, 2025	By:	/s/ Krish S. Krishnan
		Krish S. Krishnan President and Chief Executive Officer (Principal executive officer)
Date: August 4, 2025	By:	/s/ Kathryn A. Romano
		Kathryn A. Romano Chief Accounting Officer (Principal financial and accounting officer)

Exhibit 31.1

CERTIFICATION PURSUANT TO
RULES 13a-14(a) AND 15d-14(a) UNDER THE SECURITIES EXCHANGE ACT OF 1934,
AS ADOPTED 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)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

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

   b. Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

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

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

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

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

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

Date: August 4, 2025

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

Exhibit 31.2

CERTIFICATION PURSUANT TO
RULES 13a-14(a) AND 15d-14(a) UNDER THE SECURITIES EXCHANGE ACT OF 1934,
AS ADOPTED 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) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f) for the registrant and have:

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

   b. Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

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

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

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

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

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

Date: August 4, 2025

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, 2025, (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 4, 2025

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, 2025, (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 4, 2025

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