UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-Q

(Mark One)

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

For the quarterly period ended September 30, 2026

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: 001-39264

KEROS THERAPEUTICS, INC.

(Exact name of registrant as specified in its charter)

Delaware	**81-1173868**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification Number)**
1050 Waltham Street, Suite 302 Lexington, Massachusetts	**02421**
(Address of principal executive offices)	**(Zip Code)**

Tel: (617) 314-6297

(Registrant's telephone number, including area code)

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

Title of each class	Trading Symbol	Name of each exchange on which registered
Common Stock, $0.0001 par value per share	**KROS**	**The Nasdaq Stock Market LLC**

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

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

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

Large Accelerated Filer	☐	Accelerated Filer	☐
Non-Accelerated Filer	☑	Smaller Reporting Company	☑
		Emerging Growth Company	☐

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

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

As of October 30, 2026, there were 19,624,374 outstanding shares of the registrant's common stock, par value $0.0001 per share.

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SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. All statements other than statements of historical facts contained in this Quarterly Report on Form 10-Q are forward-looking statements, including statements about:

- the timing of announcement of data from our ongoing Phase 2 clinical trial of our lead product candidate, rinvatercept (KER-065), in patients with Duchenne muscular dystrophy;
- the timing of engagement with regulators on the planned Phase 2 clinical trial of rinvatercept in patients with amyotrophic lateral sclerosis;
- plans of Takeda Pharmaceuticals U.S.A., Inc., or Takeda, related to the development of elritercept (KER-050) in patients with myelodysplastic syndromes and in patients with myelofibrosis;
- risks associated with public health crises, which may adversely impact our business, nonclinical studies and clinical trials;
- our ability to receive the required regulatory approvals and clearances to successfully market and sell our products, if approved in the United States and certain other countries;
- our ability to successfully advance our pipeline of additional product candidates;
- our ability to develop sales and marketing capabilities;
- the rate and degree of market acceptance of any products we are able to commercialize;
- the effects of increased competition as well as innovations by new and existing competitors in our market;
- our ability to obtain funding for our operations;
- the sufficiency of our existing capital resources;
- our ability to establish and maintain collaborations;
- our ability to effectively manage our anticipated growth;
- our ability to maintain, protect and enhance our intellectual property rights and proprietary technologies;
- our ability to operate our business without infringing the intellectual property rights and proprietary technology of third parties;
- costs associated with defending intellectual property infringement, product liability and other claims;
- regulatory developments in the United States, Australia, New Zealand, Georgia, Europe, the United Kingdom and other foreign countries;
- our ability to attract and retain qualified employees;
- statements regarding future revenue, hiring plans, expenses, capital expenditures, capital requirements and stock performance; and
- the future trading prices of our common stock and the impact of securities analysts' reports on these prices.

In some cases, you can identify forward-looking statements by the words "anticipate," "believe," "continue," "could," "estimate," "expect," "intend," "may," "might," "objective," "ongoing," "plan," "predict," "project," "potential," "should," "will," or "would," or the negative of these terms, or other comparable terminology intended to identify statements about the future. These statements involve known and unknown risks, uncertainties and other factors that may cause our actual results, levels of activity, performance or achievements to be materially different from the information expressed or implied by these forward-looking statements.

In addition, statements that "we believe" and similar statements reflect our beliefs and opinions on the relevant subject. These statements are based upon information available to us as of the date of this report, and while we believe such information forms a reasonable basis for such statements, such information may be limited or incomplete, and our statements should not be read to indicate that we have conducted an exhaustive inquiry into, or review of, all potentially available relevant information. These statements are inherently uncertain, and investors are cautioned not to unduly rely upon these statements.

You should read the section titled "Risk Factors" set forth in Part II, Item 1A of this Quarterly Report on Form 10-Q for a discussion of important factors that may cause our actual results to differ materially from those expressed or implied by our forward-looking statements. Moreover, we operate in an evolving environment. New risk factors and uncertainties may emerge

from time to time, and it is not possible for management to predict all risk factors and uncertainties. As a result of these factors, we cannot assure you that the forward-looking statements in this Quarterly Report on Form 10-Q will prove to be accurate. Except as required by applicable law, we do not plan to publicly update or revise any forward-looking statements contained herein, whether as a result of any new information, future events, changed circumstances or otherwise.

You should read this Quarterly Report on Form 10-Q completely and with the understanding that our actual future results may be materially different from what we expect. We qualify all of our forward-looking statements by these cautionary statements.

SPECIAL NOTE REGARDING COMPANY REFERENCES

Throughout this Quarterly Report on Form 10-Q, "Keros," the "Company," "we," "us" and "our" refer to Keros Therapeutics, Inc. and its subsidiaries.

SPECIAL NOTE REGARDING TRADEMARKS

All trademarks, trade names and service marks appearing in this Quarterly Report on Form 10-Q are the property of their respective owners.

SUMMARY OF SELECTED RISKS ASSOCIATED WITH OUR BUSINESS

Our business faces significant risks and uncertainties. If any of the following risks are realized, our business, financial condition and results of operations could be materially and adversely affected. You should carefully review and consider the full discussion of our risk factors in the section titled “Risk Factors” in Part II, Item 1A of this Quarterly Report on Form 10-Q. Some of the more significant risks include the following:

- We have a limited operating history, have incurred net losses in almost every year since our inception and anticipate that we will continue to incur net losses in the future.
- We will need substantial additional funding in order to complete the development and commence commercialization of our product candidates. Failure to obtain this necessary capital when needed may force us to delay, reduce or eliminate certain of our product development or research operations.
- We are heavily dependent on the success of our product candidates, which are in clinical development. If we are unable to advance our current or future product candidates through clinical trials, obtain marketing approval and ultimately commercialize any product candidates we develop, or experience significant delays in doing so, our business will be materially harmed.
- All of our product candidates are in nonclinical or clinical development stages. Clinical trials are difficult to design and implement, and they involve a lengthy and expensive process with uncertain outcomes. We may experience delays in completing, or ultimately be unable to complete, the development and commercialization of rinvatercept (KER-065), elritercept (KER-050) or any future product candidates.
- If we are unable to successfully commercialize any product candidate for which we receive regulatory approval, or experience significant delays in doing so, our business will be materially harmed.
- We face significant competition from other biotechnology and pharmaceutical companies, and our operating results will suffer if we fail to compete effectively.
- Our success depends in part on our ability to protect our intellectual property. It is difficult and costly to protect our proprietary rights and technology, and we may not be able to ensure their protection.
- We rely, and expect to continue to rely, on third parties, including independent clinical investigators, contracted laboratories and contract research organizations, to conduct our nonclinical studies and clinical trials. If these third parties do not successfully carry out their contractual duties or meet expected deadlines, we may not be able to obtain regulatory approval for or commercialize our product candidates and our business could be substantially harmed.
- We rely on third parties to supply and manufacture our product candidates, and we expect to continue to rely on third parties to manufacture our products, if approved. The development of such product candidates and the commercialization of any products, if approved, could be stopped, delayed or made less profitable if any such third party fails to provide us with sufficient quantities of product candidates or products or fails to do so at acceptable quality levels or prices or fails to maintain or achieve satisfactory regulatory compliance.
- We are dependent on our existing third-party collaborations with Takeda Pharmaceuticals U.S.A., Inc., or Takeda, and Hansoh (Shanghai) Healthtech Co., Ltd., or Hansoh, to commercialize elritercept, and if Takeda and Hansoh are not successful in commercializing elritercept in their respective licensed territories, we will lose a significant source of potential revenue.
- Our current and future collaborations are and will be important to our business. If we are unable to enter into new collaborations, or if these or our current collaborations are not successful, our business could be adversely affected.
- Public health crises could adversely impact our business, including the timing or results of our nonclinical studies and clinical trials.

PART I. FINANCIAL INFORMATION

Item 1. FINANCIAL STATEMENTS (unaudited)

KEROS THERAPEUTICS, INC.
Condensed Consolidated Balance Sheets
(In thousands, except share and per share data)
(Unaudited)

	SEPTEMBER 30, 2026	DECEMBER 31, 2025
ASSETS		
CURRENT ASSETS:		
Cash and cash equivalents	$ 259,464	$ 287,415
Accounts receivable	20,000	3,567
Prepaid expenses and other current assets	5,920	22,202
Current income tax receivable	2,250	2,250
Total current assets	287,634	315,434
Operating lease right-of-use assets	14,887	16,841
Property and equipment, net	3,390	4,297
Restricted cash	1,449	1,449
Other long-term assets	11	—
TOTAL ASSETS	$ 307,371	$ 338,021
LIABILITIES AND STOCKHOLDERS' EQUITY		
CURRENT LIABILITIES:		
Accounts payable	$ 3,787	$ 1,967
Current portion of operating lease liabilities	2,695	2,408
Accrued expenses and other current liabilities	8,761	16,039
Current tax liability	615	—
Total current liabilities	15,858	20,414
Operating lease liabilities, net of current portion	12,415	14,475
Total liabilities	28,273	34,889
STOCKHOLDERS' EQUITY:		
Preferred stock, par value of $0.0001 per share; 10,000,000 shares authorized as of September 30, 2026 and December 31, 2025; no shares issued and outstanding	—	—
Series A junior participating preferred stock, par value of $0.0001 per share; 500,000 shares authorized as of September 30, 2026 and December 31, 2025; no shares issued and outstanding	—	—
Common stock, par value of $0.0001 per share; 200,000,000 shares authorized as of September 30, 2026 and December 31, 2025; 41,103,773 shares issued and 19,830,671 shares outstanding as of September 30, 2026 and 40,670,466 shares issued and 19,543,706 shares outstanding as of December 31, 2025	4	4
Treasury stock, at cost; 21,273,102 shares as of September 30, 2026 and 21,126,760 shares as of December 31, 2025	(386,211)	(384,558)
Additional paid-in capital	1,185,335	1,169,451
Accumulated deficit	(520,030)	(481,765)
Total stockholders' equity	279,098	303,132
TOTAL LIABILITIES AND STOCKHOLDERS' EQUITY	$ 307,371	$ 338,021

See notes to condensed consolidated financial statements.

KEROS THERAPEUTICS, INC.
Condensed Consolidated Statements of Operations
(In thousands, except share and per share data)
(Unaudited)

	THREE MONTHS ENDED SEPTEMBER 30,		NINE MONTHS ENDED SEPTEMBER 30,	
	2026	**2025**	**2026**	**2025**
REVENUE:				
Service and other revenue	$ 9	$ 4,262	$ 376	$ 38,321
License revenue	40,000	10,000	40,000	205,355
Total revenue	40,009	14,262	40,376	243,676
OPERATING EXPENSES:				
Research and development	(20,491)	(19,519)	(58,919)	(111,731)
General and administrative	(6,700)	(10,127)	(25,453)	(35,106)
Total operating expenses	(27,191)	(29,646)	(84,372)	(146,837)
INCOME (LOSS) FROM OPERATIONS	12,818	(15,384)	(43,996)	96,839
OTHER INCOME (EXPENSE), NET				
Interest income, net	55	—	55	—
Dividend income	2,220	6,933	6,842	20,845
Other expense, net	(168)	(253)	(394)	(812)
Total other income, net	2,107	6,680	6,503	20,033
Income (loss) before income taxes	14,925	(8,704)	(37,493)	116,872
Income tax (provision) benefit	(772)	1,424	(772)	(6,397)
Net income (loss)	$ 14,153	$ (7,280)	$ (38,265)	$ 110,475
Net income (loss) attributable to common stockholders — basic and diluted (Note 9)	$ 14,153	$ (7,280)	$ (38,265)	$ 110,475
Weighted-average shares of common stock outstanding — basic	19,886,097	40,623,364	19,773,047	40,598,777
Weighted-average shares of common stock outstanding — diluted	20,341,140	40,623,364	19,773,047	41,202,371
Net income (loss) per share of common stock — basic	$ 0.71	$ (0.18)	$ (1.94)	$ 2.72
Net income (loss) per share of common stock — diluted	$ 0.70	$ (0.18)	$ (1.94)	$ 2.68

See notes to condensed consolidated financial statements.

KEROS THERAPEUTICS, INC.
Condensed Consolidated Statements of Stockholders' Equity
(In thousands, except share and per share data)
(Unaudited)

	COMMON STOCK $0.0001 PAR VALUE		TREASURY STOCK		ADDITIONAL PAID-IN CAPITAL	ACCUMULATED DEFICIT	TOTAL STOCKHOLDERS' EQUITY
	SHARES	AMOUNT	SHARES	AMOUNT			
As of December 31, 2025	40,670,466	$ 4	(21,126,760)	$ (384,558)	$ 1,169,451	$ (481,765)	$ 303,132
Exercise of common stock options	15,304	—	—	—	7	—	7
Vesting of restricted stock	173,827	—	—	—	—	—	—
Stock-based compensation	—	—	—	—	6,199	—	6,199
Net income (loss)	—	—	—	—	—	(23,708)	(23,708)
As of March 31, 2026	40,859,597	$ 4	(21,126,760)	$ (384,558)	$ 1,175,657	$ (505,473)	$ 285,630
Exercise of common stock options	67,000	—	—	—	23	—	23
Vesting of restricted stock	27,351	—	—	—	—	—	—
Stock-based compensation	—	—	—	—	5,377	—	5,377
Net income (loss)	—	—	—	—	—	(28,710)	(28,710)
As of June 30, 2026	40,953,948	$ 4	(21,126,760)	$ (384,558)	$ 1,181,057	$ (534,183)	$ 262,320
Issuance of common stock under the employee stock purchase plan	7,712	—	—	—	73	—	73
Exercise of common stock options	4,000	—	—	—	2	—	2
Vesting of restricted stock	138,113	—	—	—	—	—	—
Repurchase of treasury stock, including commissions of $3 thousand	—	—	(146,342)	(1,653)	—	—	(1,653)
Stock-based compensation	—	—	—	—	4,203	—	4,203
Net income (loss)	—	—	—	—	—	14,153	14,153
As of September 30, 2026	41,103,773	$ 4	(21,273,102)	$ (386,211)	$ 1,185,335	$ (520,030)	$ 279,098

	COMMON STOCK $0.0001 PAR VALUE		TREASURY STOCK		ADDITIONAL PAID-IN CAPITAL	ACCUMULATED DEFICIT	TOTAL STOCKHOLDERS' EQUITY
	SHARES	AMOUNT	SHARES	AMOUNT			
As of December 31, 2024	40,554,705	$ 4	—	$ —	$ 1,140,328	$ (568,779)	$ 571,553
Exercise of common stock options	7,342	—	—	—	4	—	4
Stock-based compensation	—	—	—	—	8,863	—	8,863
Net income (loss)	—	—	—	—	—	148,451	148,451
As of March 31, 2025	40,562,047	$ 4	—	$ —	$ 1,149,195	$ (420,328)	$ 728,871
Exercise of common stock options	53,367	—	—	—	17	—	17
Stock-based compensation	—	—	—	—	8,542	—	8,542
Net income (loss)	—	—	—	—	—	(30,696)	(30,696)
As of June 30, 2025	40,615,414	$ 4	—	$ —	$ 1,157,754	$ (451,024)	$ 706,734
Issuance of common stock under the employee stock purchase plan	11,806	—	—	—	138	—	138
Exercise of common stock options	4,944	—	—	—	2	—	2
Stock-based compensation	—	—	—	—	3,986	—	3,986
Net income (loss)	—	—	—	—	—	(7,280)	(7,280)
As of September 30, 2025	40,632,164	$ 4	—	$ —	$ 1,161,880	$ (458,304)	$ 703,580

See notes to condensed consolidated financial statements.

KEROS THERAPEUTICS, INC.
Condensed Consolidated Statements of Cash Flows
(In thousands)
(Unaudited)

	NINE MONTHS ENDED SEPTEMBER 30,	
	2026	**2025**
CASH FLOWS FROM OPERATING ACTIVITIES:		
Net income (loss)	$ (38,265)	$ 110,475
Adjustments to reconcile net income (loss) to net cash used in operating activities:		
Depreciation expense	1,189	1,109
Gain on disposal of fixed asset	—	(14)
Stock-based compensation expense	15,779	21,391
Non-cash lease expense	1,954	1,788
Changes in operating assets and liabilities:		
Accounts receivable	(16,433)	(750)
Prepaid expenses and other current assets	16,282	3,877
Other assets	(11)	2,056
Accounts payable	2,186	(2,304)
Right-of-use assets and operating lease liabilities	(1,773)	(1,432)
Deferred revenue	—	155
Current tax liability	615	4,147
Accrued expenses and other current liabilities	(2,083)	(5,701)
Net cash provided by (used in) operating activities	(20,560)	134,797
CASH FLOWS FROM INVESTING ACTIVITIES:		
Purchase of property and equipment	(287)	(1,417)
Net cash used in investing activities	(287)	(1,417)
CASH FLOWS FROM FINANCING ACTIVITIES:		
Proceeds from issuance of common stock under the employee stock purchase plan	73	138
Repurchase of treasury stock	(1,545)	—
Payment of direct expenses associated with the treasury stock repurchase	(5,664)	—
Proceeds from exercise of stock options	32	23
Net cash provided by (used in) financing activities	(7,104)	161
NET INCREASE (DECREASE) IN CASH, CASH EQUIVALENTS AND RESTRICTED CASH	(27,951)	133,541
Cash, cash equivalents and restricted cash at beginning of period	288,864	561,380
Cash, cash equivalents and restricted cash at end of period	$ 260,913	$ 694,921
SUPPLEMENTAL DISCLOSURE OF CASH FLOW INFORMATION:		
Cash paid for taxes	$ —	$ 2,250
Property and equipment purchases in accounts payable and accrued expenses	$ —	$ 5
Repurchases of treasury stock in accrued expenses	$ 105	$ —

The following table provides a reconciliation of the ending cash, cash equivalents and restricted cash as of each of the periods shown above:

	SEPTEMBER 30,	
	2026	**2025**
Cash and cash equivalents	$ 259,464	$ 693,472
Restricted cash	1,449	1,449
Total cash, cash equivalents and restricted cash	$ 260,913	$ 694,921

See notes to condensed consolidated financial statements.

1. NATURE OF BUSINESS AND BASIS OF PRESENTATION

Keros Therapeutics, Inc. (“Keros” or the “Company”) was incorporated in 2015 as a Delaware corporation. Its principal offices are in Lexington, Massachusetts. The Company is a clinical-stage biopharmaceutical company focused on developing and commercializing novel therapeutics to treat a wide range of patients with disorders that are linked to dysfunctional signaling of the transforming growth factor-beta (“TGF-ß”) family of proteins.

The Company's lead product candidate, rinvatercept (KER-065), is being developed for the treatment of Duchenne muscular dystrophy and for the treatment of amyotrophic lateral sclerosis. The Company’s most advanced product candidate, elritercept (KER-050), is being developed for the treatment of low blood cell counts (“cytopenias”), including anemia and thrombocytopenia, in patients with myelodysplastic syndromes (“MDS”) and in patients with myelofibrosis.

Since its inception in 2015, the Company has devoted the majority of its resources to business planning, research and development of its product candidates, including conducting clinical trials and nonclinical studies, raising capital and recruiting management and technical staff to support these operations. To date, the Company has not generated any revenue from product sales as none of its product candidates have been approved for commercialization.

Liquidity and Capital Resources

The Company’s condensed consolidated financial statements have been prepared on the basis of the Company continuing as a going concern for the next 12 months. Management believes that the Company’s existing cash and cash equivalents as of September 30, 2026 will allow the Company to continue its operations for at least the next 12 months. In the absence of a significant source of recurring revenue, the continued viability of the Company is dependent on its ability to continue to raise additional capital to finance its operations. If the Company is unable to obtain additional funding, the Company may be forced to delay, reduce or eliminate some or all of its research and development programs, product portfolio expansion or commercialization efforts, which could adversely affect its business prospects, or the Company may be unable to continue operations.

The accompanying unaudited interim condensed consolidated financial statements as of September 30, 2026 and for the three and nine months ended September 30, 2026 and 2025 have been prepared by the Company in conformity with generally accepted accounting principles in the United States of America (“U.S. GAAP”) and, pursuant to the rules and regulations of Article 10 of Regulation S-X of the Securities Act published by the Securities and Exchange Commission (“SEC”) for interim financial statements. Certain information and footnote disclosures normally included in financial statements prepared in accordance with U.S. GAAP have been condensed or omitted pursuant to such rules and regulations. However, the Company believes the disclosures are adequate to make the information presented not misleading. These unaudited interim condensed consolidated financial statements should be read in conjunction with the Company’s audited financial statements and notes thereto for the year ended December 31, 2025 included in the Company’s Annual Report on Form 10-K for the year ended December 31, 2025 filed with the SEC on March 4, 2026 (the “Annual Report”).

2. SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES

Significant Accounting Policies

The significant accounting policies and estimates used in preparation of the unaudited interim condensed consolidated financial statements are described in the Company’s audited consolidated financial statements as of and for the year ended December 31, 2025, and the notes thereto, which are included in the Annual Report. Except as detailed below, there have been no changes to the Company’s significant accounting policies during the nine months ended September 30, 2026.

Stock-Based Compensation

The Company accounts for all stock-based awards granted to employees and non-employees as stock-based compensation expense at fair value. The Company’s stock-based awards include stock options and restricted stock unit ("RSU") awards. The measurement date for employee and non-employee awards is the date of grant. For stock-based awards that vest based on service conditions, stock-based compensation costs are recognized as expense over the requisite service period, which is the vesting period, on a straight-line basis. For stock options and RSUs with performance conditions, stock-based compensation costs are recognized as expense using the accelerated attribution method when it is probable that the performance condition will be achieved. For stock options with market-based conditions, stock-based compensation costs are

recognized ratably for each vesting tranche from the grant date through the requisite service period using the correlated grant date fair value, regardless of when and if ever the market condition is met. If there is a non-substantive explicit service condition, the total compensation expense is recognized upon the date of grant. Stock-based compensation expense is classified in the accompanying consolidated statement of operations based on the function to which the related services are provided. Forfeitures are recorded as they occur.

The fair value of each service-based and performance-based stock option grant is estimated on the date of grant using the Black-Scholes option-pricing model. The Company calculates expected volatility based solely on the historical volatilities of the common stock of the Company. The expected term of the Company's stock options has been determined utilizing the "simplified" method for awards that qualify as "plain-vanilla" options. The risk-free interest rate is determined by reference to the U.S. Treasury yield curve in effect at the time of grant of the award for time periods approximately equal to the expected term of the award. Expected dividend yield is based on the fact that the Company has never paid cash dividends on common stock and does not expect to pay any cash dividends in the foreseeable future.

The fair value of the market-based awards is estimated using a Monte Carlo Simulation, incorporating various option pricing inputs. The effect of the market condition is reflected in the grant-date fair value with valuation techniques developed to value path-dependent options.

Recently Issued Accounting Pronouncements

In November 2024, the Financial Accounting Standards Board issued ASU No. 2024-03, *Income Statement—Reporting Comprehensive Income —Expense Disaggregation Disclosures (Subtopic 220-40): Disaggregation of Income Statement Expenses* ("ASU No. 2024-03"), which intends to improve financial reporting by requiring disclosure of additional information about specific expense categories. This guidance is effective for annual reporting periods beginning after December 15, 2026 and interim reporting periods beginning after December 15, 2027. Early adoption is permitted and the guidance is to be applied prospectively and may be applied retrospectively. The Company is currently evaluating the impact of this guidance on its consolidated financial statements and related disclosures.

Risks and Uncertainties

There have been significant disruptions to global financial markets that have contributed to a general global economic slowdown. Recent trends towards rising inflation and prices may materially affect the Company's business and corresponding financial position and cash flows. Inflationary factors, such as increases in the cost of materials and supplies relating to the Company's nonclinical studies and clinical trials, interest rates and overhead costs may adversely affect its operating results. Rising interest rates and implementation of tariffs present a recent challenge impacting the U.S. economy and could make it more difficult for the Company to obtain traditional financing on acceptable terms, if at all, in the future. Although the Company does not believe that inflation, higher interest rates or tariffs have had a material impact on its financial position or results of operations to date, the Company may experience increases in the near future (especially if inflation rates rise more quickly) on its operating costs, including its labor costs and research and development costs, due to supply chain constraints, consequences associated with public health crises and global geopolitical tensions, such as the ongoing war between Russia and Ukraine and the wars in the Middle East, worsening global macroeconomic conditions and employee availability and wage increases, which may result in additional stress on the Company's working capital resources. As of the date of issuance of these financial statements, the Company is not aware of any specific event or circumstance that would require the Company to update its estimates, assumptions and judgments or revise the carrying value of its assets or liabilities. Actual results could differ from those estimates, and any such differences may be material to the Company's financial statements.

In addition, the Company is subject to other challenges and risks specific to its business and its ability to execute on its business plan and strategy, as well as risks and uncertainties common to companies in the biopharmaceutical industry with research and development operations, including, without limitation, risks and uncertainties associated with: obtaining regulatory approval of its product candidates; delays or problems in obtaining clinical supply, loss of single source suppliers or failure to comply with manufacturing regulations; product development and the inherent uncertainty of clinical success; the challenges of protecting and enhancing its intellectual property rights; the challenges of complying with applicable regulatory requirements; and identifying, acquiring or in-licensing additional products or product candidates.

3. FAIR VALUE MEASUREMENTS

The following table presents information about the Company's financial assets and liabilities measured at fair value on a recurring basis and indicates the level of the fair value hierarchy utilized to determine such fair values (in thousands):

DESCRIPTION	SEPTEMBER 30, 2026	QUOTED PRICES IN ACTIVE MARKETS FOR IDENTICAL ASSETS (LEVEL 1)	SIGNIFICANT OTHER OBSERVABLE INPUTS (LEVEL 2)	SIGNIFICANT UNOBSERVABLE INPUTS (LEVEL 3)
Assets				
Money market funds	$ 253,772	$ 253,772	$ —	$ —
Total financial assets	$ 253,772	$ 253,772	$ —	$ —

DESCRIPTION	DECEMBER 31, 2025	QUOTED PRICES IN ACTIVE MARKETS FOR IDENTICAL ASSETS (LEVEL 1)	SIGNIFICANT OTHER OBSERVABLE INPUTS (LEVEL 2)	SIGNIFICANT UNOBSERVABLE INPUTS (LEVEL 3)
Assets				
Money market funds	$ 270,713	$ 270,713	$ —	$ —
Total financial assets	$ 270,713	$ 270,713	$ —	$ —

There have been no transfers between fair value levels during the nine months ended September 30, 2026. The carrying values of other current assets, accounts payable and accrued expenses approximate their fair values due to the short-term nature of these assets and liabilities.

4. PREPAID EXPENSES AND OTHER CURRENT ASSETS

Prepaid expenses and other current assets consisted of the following (in thousands):

	SEPTEMBER 30, 2026	DECEMBER 31, 2025
Prepaid external R&D costs	$ 2,053	$ 1,609
Prepaid external manufacturing costs	13	398
Prepaid insurance	704	609
Prepaid subscriptions	1,361	947
Interest and dividend receivable	587	555
Prepaid consultants	30	1,207
Refunds due from contract research organizations	584	14,480
Other	588	2,397
Total prepaid expenses and other current assets	$ 5,920	$ 22,202

5. ACCRUED EXPENSES AND OTHER CURRENT LIABILITIES

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

	SEPTEMBER 30, 2026	DECEMBER 31, 2025
Accrued external R&D costs	$ 1,015	$ 2,662
Accrued external manufacturing costs	1,774	429
Accrued compensation and benefits	4,989	5,870
Accrued excise tax	129	3,377
Accrued legal and consulting fees	538	3,484
Other	316	217
Total accrued expenses and other current liabilities	$ 8,761	$ 16,039

Accrued compensation and benefits consisted primarily of accrued payroll, bonus and vacation.

6. STOCKHOLDERS' EQUITY

As of September 30, 2026, the Company’s amended and restated certificate of incorporation authorized the Company to issue 200,000,000 shares of common stock at a par value of $0.0001 per share.

In May 2021, the Company entered into a Sales Agreement (the "ATM Sales Agreement") with Leerink Partners LLC ("Leerink"), as sales agent, under which the Company may offer and sell, from time to time, shares of its common stock (the "ATM Shares") through Leerink (the "ATM Offering"). In September 2026, the Company filed a shelf registration statement on Form S-3 (File No. 333-298854) with the SEC, and a prospectus supplement, which registered the offering, issuance and sale of up to $350.0 million of various equity and debt securities, including the issuance and sale, if any, of up to $100.0 million of shares of its common stock under the ATM Sales Agreement pursuant to the prospectus supplement. The SEC declared the registration statement effective on September 16, 2026. During the three and nine months ended September 30, 2026, the Company did not sell any shares of its common stock pursuant to the ATM Offering. As of September 30, 2026, the Company may offer and sell ATM Shares at an aggregate offering price of up to $100.0 million (less any sales agent commissions) available under the ATM Offering.

7. TREASURY STOCK

The Company's board of directors (the “Board”) may authorize the repurchase of shares of the Company’s common stock in the open market or through negotiated transactions, at such times and at such prices as management deems appropriate.

On October 15, 2025, the Company entered into stock purchase agreements with certain entities affiliated with ADAR1 Capital Management (collectively, the “ADAR1 Parties”) and certain entities affiliated with Pontifax Venture Capital (collectively, the “Pontifax Parties”). Such stock purchase agreements for each of the Pontifax and ADAR1 Parties are collectively referred to as “Repurchase Agreements”. Pursuant to the terms and conditions of the Repurchase Agreements, the ADAR1 Parties and the Pontifax Parties sold all of the shares of common stock beneficially owned by them, being an aggregate of 10,176,595 shares of common stock, to the Company at a price of $17.75 per share for an aggregate purchase price of $180.6 million, excluding excise tax, direct fees and expenses related to the repurchases. The total cost of the repurchase was recorded as treasury stock using the cost method and is included as treasury stock in the Company’s consolidated balance sheets.

On October 20, 2025, the Company commenced a tender offer to repurchase shares of its common stock at a cash purchase price of $17.75 per share (the “Tender Offer”). The Tender Offer expired at 5:00 p.m. Eastern time on November 18, 2025, and following such expiration the Company accepted for purchase a total of 10,950,165 shares, for an aggregate purchase price of approximately $194.4 million, excluding excise tax, direct fees and expenses related to the Tender Offer. More than 10,950,165 shares were tendered in the Tender Offer, and as such, shares were accepted for purchase on a pro rata basis, except for conditional tenders that were automatically regarded as withdrawn if the condition was not satisfied. The total cost of the repurchase was recorded as treasury stock using the cost method and is included as treasury stock in the accompanying consolidated balance sheets.

The completion of the Repurchase Agreements in October 2025 and the Tender Offer in November 2025 concluded the Company’s previously announced $375.0 million capital return program. In connection with the Repurchase Agreements and Tender Offer, the Company incurred $9.6 million in excise tax, direct fees and expenses that were included in the cost of treasury stock in the accompanying consolidated balance sheets as of December 31, 2025.

On September 8, 2026, the Board approved a share repurchase program for up to $5.0 million of its common stock, following receipt of a $20.0 million development milestone payment under the license agreement with Takeda in September 2026. The Board approval authorizes the Company to repurchase outstanding shares of the Company's common stock, from time to time, in the open market and/or by such other means in accordance with applicable state and federal securities laws.

The timing and amount of any repurchases will depend on a variety of factors, including the market price of the Company's common stock, general market and economic conditions, contractual limitations and other considerations. The repurchase program does not obligate the Company to acquire any particular amount of common stock and may be suspended, modified or discontinued at any time at the Company's discretion.

Repurchases of common stock are recorded on the trade date. During the three and nine months ended September 30, 2026, the Company repurchased 146,342 shares of its common stock under the September 2026 Repurchase Program for an aggregate cost of $1.7 million, including commissions. The weighted-average purchase price was $11.27 per share. Of the $1.7 million, $0.1 million related to 9,168 shares with a trade date of September 30, 2026, settled on October 1, 2026 and is included in accrued expenses as of September 30, 2026.

8. STOCK-BASED COMPENSATION

2017 Stock Incentive Plan

The Board adopted the 2017 Stock Incentive Plan (the "2017 Plan") in February 2017, and the stockholders approved the 2017 Plan in March 2017. The 2017 Plan was most recently amended in March 2020.

As of September 30, 2026, there was an aggregate of 325,795 shares of common stock issuable upon the exercise of outstanding options under the 2017 Plan. Any options or awards outstanding under the 2017 Plan remain outstanding and effective.

2020 Equity Incentive Plan

In April 2020, the 2020 Equity Incentive Plan (the "2020 Plan") became effective, and, as a result, no further awards will be made under the 2017 Plan. The 2020 Plan provides for the grant of stock options qualifying as incentive stock options, to employees and for the grant of nonstatutory stock options, restricted stock awards, RSU awards, stock appreciation rights, performance stock awards and other forms of stock compensation to employees, consultants and directors. The 2020 Plan also provides for the grant of performance cash awards to employees, consultants and directors. Any previously granted awards under the 2017 Plan will remain outstanding in accordance with their respective terms.

Under the 2020 Plan, there is an annual increase on January 1 of each year from January 1, 2021 continuing through January 1, 2030, by 4.0% of the total number of shares of common stock outstanding on December 31 of the preceding calendar year, or a lesser number of shares as may be determined by the Board. On January 1, 2026, the Company increased the number of shares available for future grant under the 2020 Plan by 781,748 shares. The awards granted under the 2020 Plan typically vest over a two- to four-year period and have a 10-year contractual term.

As of September 30, 2026, there was an aggregate of 4,053,704 shares of common stock issuable upon the exercise of outstanding options and 572,263 shares of common stock issuable upon the vesting of the outstanding RSU awards under the 2020 Plan. Additionally, there were an aggregate of 3,169,001 shares reserved for future issuance under the 2020 Plan, including shares forfeited from the 2017 Plan.

Restricted Stock Units

A summary of RSU award activity during the nine months ended September 30, 2026 is as follows (in thousands except share and per share data):

	Number of Units	Weighted-Average Grant-Date Fair Value
Outstanding RSU awards as of December 31, 2025	716,850	$ 12.37
Granted	264,513	14.05
Vested	(339,291)	11.24
Forfeited	(69,809)	12.10
Outstanding RSU awards as of September 30, 2026	572,263	$ 13.84

The summary of RSU award activity includes 90,500 performance-based awards granted during the year ended December 31, 2025. As of September 30, 2026, there has been no expense recognized for the performance-based awards, for which the performance criteria was not deemed probable as of September 30, 2026. As of September 30, 2026, there was approximately $6.8 million of unrecognized stock-based compensation expense related to RSU awards that is expected to vest, including $1.4 million unrecognized stock-based compensation expense related to performance-based awards. These costs are expected to be recognized over a weighted-average remaining vesting period of approximately 2.23 years.

The aggregate fair value of RSU awards that vested during the nine months ended September 30, 2026 and 2025 was $4.6 million and zero, respectively.

Stock Options

A summary of option activity during the nine months ended September 30, 2026 is as follows (in thousands except share and per share data):

	NUMBER OF OPTIONS	WEIGHTED-AVERAGE EXERCISE PRICE
Outstanding as of December 31, 2025	3,901,706	$ 38.52
Granted	964,123	14.83
Exercised	(86,304)	0.37
Cancelled or Forfeited	(232,882)	40.39
Expired	(167,144)	$ 40.02
Outstanding as of September 30, 2026	4,379,499	$ 33.90
Options exercisable as of September 30, 2026	3,200,492	$ 36.92

The weighted-average grant-date fair value price per share of options granted during the nine months ended September 30, 2026 and 2025 was $10.72 and $10.36, respectively. As of September 30, 2026, there was $16.9 million of unrecognized stock-based compensation expense related to unvested service-based stock options and $0.1 million of unrecognized stock-based compensation expense related to performance-based and market-based stock options. The unrecognized stock-based compensation expense is estimated to be recognized over a period of 2.44 years.

The summary of option activity includes 125,000 performance-based stock options, 62,500 of which were cancelled during the nine months ended September 30, 2026. The options include performance-based and service-based vesting criteria. During the nine months ended September 30, 2026, the Company recognized $0.3 million of stock-based compensation expense related to these performance-based options. As of September 30, 2026, there was $0.1 million of unrecognized stock-based compensation expense related to service-based vesting criteria that the Company expects to recognize.

The summary of option activity also includes 100,000 market-based stock options that were granted during the nine months ended September 30, 2026. The Company measured the fair value of the market-based awards on the grant date using a Monte Carlo Simulation, incorporating various option pricing inputs. The weighted-average grant date fair value of the market-based awards is estimated as $10.85 per share and the total compensation expense is $1.1 million. Due to a non-substantive explicit service condition, the total compensation expense was recognized during the nine months ended September 30, 2026.

The aggregate intrinsic value of stock options is calculated as the difference between the exercise price of the stock options and the fair value of the Company’s common stock for those stock options that had exercise prices lower than the fair value of

the Company's common stock. The aggregate intrinsic value of the stock options outstanding and stock options exercisable was $3.7 million and $3.6 million as of September 30, 2026, respectively. The aggregate intrinsic value of stock options exercised was $1.0 million and $0.7 million during the nine months ended September 30, 2026 and 2025, respectively.

2020 Employee Stock Purchase Plan

In March 2020, the Board adopted and the Company's stockholders approved the 2020 Employee Stock Purchase Plan ("ESPP"). The ESPP became effective on April 7, 2020. Under the ESPP, eligible employees can purchase shares of the Company's common stock, based on a percentage of their compensation, subject to certain limits. The purchase price per share is equal to the lower of 85% of the fair market value of the Company's common stock on the first trading day of the twenty-four month offering period, or on the applicable purchase date. Each offering under the ESPP consists of two twelve-month purchase periods.

Under the ESPP, there is an annual increase on January 1 of each year from January 1, 2021 continuing through January 1, 2030, by the lesser of (i) 1.0% of the total number of shares of common stock outstanding on December 31 of the preceding calendar year, (ii) 455,852 shares or (iii) a lesser number of shares as may be determined by the Board. On January 1, 2026, the Company increased the number of shares available for future grant under the ESPP by 195,437 shares. If purchase rights granted under the ESPP terminate without having been exercised, the shares of our common stock not purchased under such purchase rights will again become available for issuance under the ESPP.

As of September 30, 2026, 19,518 shares of common stock were purchased under the ESPP. As of September 30, 2026, there was an aggregate of 1,829,327 shares reserved for future issuance under the ESPP.

Stock-Based Compensation Expense

Total stock-based compensation expense recorded for employees, directors and non-employees during the three and nine months ended September 30, 2026 and 2025 was as follows (in thousands):

	THREE MONTHS ENDED SEPTEMBER 30,		NINE MONTHS ENDED SEPTEMBER 30,	
	2026	2025	2026	2025
Research and development	$ 2,025	$ 992	$ 6,792	$ 11,230
General and administrative	2,178	2,994	8,987	10,161
Total stock-based compensation expense	$ 4,203	$ 3,986	$ 15,779	$ 21,391

9. INCOME (LOSS) PER SHARE

Basic and diluted income (loss) per share of common stock are calculated as follows (in thousands, except share and per share data):

	THREE MONTHS ENDED SEPTEMBER 30,		NINE MONTHS ENDED SEPTEMBER 30,	
	2026	2025	2026	2025
Numerator:				
Net income (loss) attributable to common stockholders - basic and diluted	$ 14,153	$ (7,280)	$ (38,265)	$ 110,475
Denominator:				
Weighted-average shares of common stock outstanding - basic	19,886,097	40,623,364	19,773,047	40,598,777
Effect of potentially dilutive stock options	316,990	—	—	424,342
Effect of potentially dilutive employee stock purchase plan shares	—	—	—	—
Effect of potentially dilutive restricted stock units	138,053	—	—	179,252
Total potentially dilutive securities	455,043	—	—	603,594
Weighted-average shares of common stock outstanding used to compute diluted net loss[1]	20,341,140	40,623,364	19,773,047	41,202,371
Net income (loss) per share of common stock				
Basic	$ 0.71	$ (0.18)	$ (1.94)	$ 2.72
Diluted	$ 0.70	$ (0.18)	$ (1.94)	$ 2.68

Shares repurchased under the 2026 Repurchase Program are included in the calculation of weighted-average shares as a reduction of common stock outstanding from their respective trade dates, including 9,168 shares with a trade date of September 30, 2026 that settled in October 2026.

The Company excluded the following from the computation of diluted net income (loss) per share attributable to common stockholders as of September 30, 2026 and 2025 because including them would have had an anti-dilutive effect:

	THREE MONTHS ENDED SEPTEMBER 30,		NINE MONTHS ENDED SEPTEMBER 30,	
	2026	2025	2026	2025
Restricted stock unit awards	275,860	55,087	176,128	18,564
Options to purchase common stock	4,087,183	4,407,885	3,967,552	4,711,081
Employee stock purchase plan shares	9,773	8,976	3,293	8,976
Total	4,372,816	4,471,948	4,146,973	4,738,621

[1] In periods where there is a net loss, zero incremental shares are included as the effect would be anti-dilutive.

10. INCOME TAXES

The Company calculates income taxes at each interim reporting period based on the estimated annual effective tax rate for the full year, adjusted for any discrete events which are recorded in the period they occur. Cumulative adjustments to the income tax provision are recorded in the interim reporting period in which a change in the estimated annual effective tax rate is determined. The Company's income tax (provision) benefit and effective tax rate are presented below (income tax (provision) benefit in thousands):

	THREE MONTHS ENDED SEPTEMBER 30,		NINE MONTHS ENDED SEPTEMBER 30,	
	2026	2025	2026	2025
Income tax (provision) benefit	(772)	1,424	(772)	(6,397)
Effective tax rate	5.2 %	16.4 %	(2.1)%	5.5 %

The income tax (provision) benefit and the effective tax rate for the three and nine months ended September 30, 2026 and September 30, 2025 are primarily attributable to taxable income during the period resulting from the Takeda Agreement (as defined below), partially offset by available net operating loss and tax credit carryforwards. The Company has evaluated the positive and negative evidence bearing upon its ability to realize its deferred tax assets. Management has considered the Company's history of cumulative net losses incurred since inception and its lack of commercialization of any products that would generate revenue from product sales and has concluded that it is more likely than not that the Company will not realize the benefits of its deferred tax assets. Accordingly, a full valuation allowance is maintained against its net deferred tax assets.

11. COMMITMENTS AND CONTINGENCIES

Purchase Commitments

The Company enters into agreements in the normal course of business with contract manufacturing organizations for process development, raw material purchases and manufacturing services. These contracts typically do not contain minimum purchase commitments and are generally cancellable by the Company upon written notice. Payments due upon cancellation consist of payments for services provided or expenses incurred, including noncancellable obligations of the Company's service providers, up to the date of cancellation and, in the case of certain arrangements with contract manufacturing organizations, may include noncancellable fees. Under such agreements, the exact amounts owed by the Company in the event of termination will be based on the timing of the termination and the exact terms of the agreement. As of September 30, 2026, the Company has committed up to approximately $4.6 million under these agreements which are expected to be paid through 2028.

Distribution of Proceeds

The Company plans to distribute 25% of any net cash proceeds it receives on or before December 31, 2028 from the Takeda Agreement (as defined below) to its stockholders. In September 2026, the Company committed to repurchasing up to $5.0 million of its common stock following the receipt of a $20.0 million development milestone payment under the Takeda Agreement. The Company began repurchasing its common stock in September 2026 and has repurchased $1.6 million of its common stock, excluding commissions, during the three and nine months ended September 30, 2026. As of September 30, 2026, the Company plans to repurchase the remaining $3.4 million of its common stock prior to February 28, 2027.

12. REVENUE FROM CONTRACTS WITH CUSTOMERS

Hansoh License Agreement

On December 12, 2021, the Company entered into a license agreement (the "Hansoh Agreement") with Hansoh (Shanghai) Healthtech Co., Ltd. ("Hansoh"). Under the Hansoh Agreement, the Company granted to Hansoh the exclusive right to develop, manufacture and commercialize elritercept and licensed products containing elritercept within the territories of mainland China, Hong Kong and Macau (the "Hansoh Territory").

In connection with the Hansoh Agreement, Hansoh will purchase clinical trial supply of elritercept from the Company, and the parties will also negotiate in good faith to enter into an agreement for commercial supply prior to any anticipated commercialization in the Hansoh Territory. In addition, Hansoh will use commercially reasonable efforts to develop, obtain regulatory approval for, and commercialize licensed products in any region in the Hansoh Territory.

Pursuant to the Hansoh Agreement, the Company received a one-time, net $18.0 million upfront license payment in January 2022. In addition to the upfront payment and development milestones achieved to date, the Company will also be eligible to

receive up to an aggregate of (i) $23.5 million upon the achievement of specified development milestones and (ii) $144.0 million upon the achievement of specified net sales thresholds for all licensed products in the Hansoh Territory. If a licensed product is approved for marketing in the Hansoh Territory, the Company will be entitled to receive royalty payments based on a tiered percentage of annual net sales in each region within the Hansoh Territory, with such percentage ranging from the low double digit to high teens, subject to specified potential royalty reductions.

Hansoh's obligation to pay royalties for a given licensed product in a given region in the Hansoh Territory will begin on the date of the first commercial sale for such licensed product in such region and continue until the latest of (i) 10 years from the date of the first commercial sale for such licensed product in such region, (ii) the expiration of the last valid claim of certain licensed patents or joint patents, and (iii) expiration of regulatory exclusivity in such region. During the royalty term, neither party will directly nor indirectly commercialize a competing product in the Hansoh Territory.

The Hansoh Agreement will continue in force on a region-by-region basis until the expiration of the royalty term. Hansoh may terminate the Hansoh Agreement in its entirety for convenience, with notice. The Company may terminate the Hansoh Agreement in its entirety for a patent challenge brought by Hansoh or its affiliates or their sublicensees. Either party may terminate the Hansoh Agreement in its entirety (i) if the other party materially breaches the Hansoh Agreement and fails to cure such breach or (ii) upon the bankruptcy of the other party.

The Company evaluated the Hansoh Agreement and concluded that it was subject to ASC 606, as the Company viewed the Hansoh Agreement as a contract with a customer. As such, the Company assessed the terms of the Hansoh Agreement and identified a single performance obligation for the Company to provide Hansoh an exclusive license to develop, manufacture and commercialize elritercept and licensed products containing elritercept in the Hansoh Territory, including the underlying know-how related to such licenses. All other promised goods/services were deemed immaterial in the context of the Hansoh Agreement. Under the Hansoh Agreement, the Company recognized a one-time, upfront license fee of $20.0 million as revenue and $2.0 million in withholding tax expense during the year ended December 31, 2021. The Company received the net payment of $18.0 million during the three months ended March 31, 2022.

The Company will recognize development milestone payments as revenue at the point in time when it is determined that it is probable such milestones will be achieved as all performance obligations will have been satisfied at the point which a milestone might occur (i.e., Hansoh will have assumed all responsibility for the activities under the Hansoh Agreement). The Company will recognize royalty payments and commercial milestone payments as the associated sales of licensed products are recorded by Hansoh, as they predominantly relate to the license granted with the Hansoh Agreement. The Company did not recognize any revenue related to milestones for the three and nine months ended September 30, 2026 or 2025.

In connection with the Hansoh Agreement, the Company entered into a manufacturing technology transfer agreement (the "Tech Transfer Agreement") with Hansoh, effective in June 2023. The Tech Transfer Agreement governs the transfer to Hansoh, by the Company of all documents and information required to complete the manufacturing technology transfer. Under the Tech Transfer Agreement, Hansoh is obligated to make certain payments to the Company, at the rates set forth in the Tech Transfer Agreement, as manufacturing technology transfer services are provided over the term of the Tech Transfer Agreement. The Company did not recognize any revenue during the three and nine months ended September 30, 2026. The Company recognized zero and $0.1 million of service and other revenue during the three and nine months ended September 30, 2025, respectively.

In connection with the Hansoh Agreement, the Company entered into a clinical product supply agreement (the "Supply Agreement") with Hansoh, effective February 2024. The Company evaluated the Supply Agreement and concluded that it was subject to ASC 606, as the Company viewed the Supply Agreement as a contract with a customer. As such, the Company assessed the terms of the Supply Agreement and identified a single performance obligation for the Company to supply Hansoh with clinical product supply. The Company will recognize revenue at a point in time when control transfers, which is deemed to be at the shipping point when the clinical product supply is ready for shipment. The Company recognized zero and $0.3 million of service and other revenue during the three and nine months ended September 30, 2026, respectively. The Company recognized $0.1 million of service and other revenue during the three and nine months ended September 30, 2025. The Company did not have any accounts receivable due from Hansoh as of September 30, 2026 and as of December 31, 2025.

Takeda License Agreement

On December 3, 2024, the Company entered into a license agreement with Takeda Pharmaceuticals U.S.A., Inc. ("Takeda"), which became effective on January 16, 2025. Under the terms of the license agreement with Takeda (the "Takeda Agreement"), the Company granted to Takeda the exclusive right to develop, manufacture and commercialize elritercept and certain derivative compounds globally, excluding the territories of mainland China, Hong Kong and Macau (collectively, the "Takeda Territory"). Concurrent with the Takeda Agreement, the Company entered into a separate Transition Services Agreement with Takeda ("TSA"), pursuant to which the Company will provide certain transitional services to Takeda, including, but not limited to, clinical trial and manufacturing services, during the term of the TSA (the "Transition Services"). In January

2026, the initial fifteen month term of the TSA was extended for six months through October 2026. The Company will receive reimbursement of certain costs incurred by the Company in connection with providing the Transition Services.

Pursuant to the terms of the Takeda Agreement, the Company received a $200.0 million upfront payment in February 2025. In July 2025, a one-time $10.0 million development milestone was achieved upon dosing of the first patient in the Phase 3 RENEW clinical trial of elritercept. In July 2026, a one-time $20.0 million development milestone was achieved upon dosing of the first patient in the open-label, randomized Phase 3 ELRiSE MDS clinical trial. In September 2026, a one-time $20.0 million development milestone was achieved upon dosing of the first patient in the double-blind, randomized Phase 3 ELRiSE MF clinical trial. In addition to the upfront and development milestone payments, the Company is entitled to receive up to an aggregate of (i) $40.0 million upon the achievement of specified development milestones; (ii) $280.0 million upon the achievement of specified commercial milestones; and (iii) $740.0 million upon the achievement of specified sales milestones. If a licensed product is approved for marketing in the Takeda Territory, the Company will be entitled to receive royalty payments based on tiered increments of annual net sales in the Takeda Territory, with such percentage ranging from the low double-digits to high teens, subject to specified potential royalty reductions. The Company recognized $10.0 million as revenue upon the achievement of the first development milestone related to the Takeda Agreement on its consolidated statement of operations for the year ended December 31, 2025. Payment was received during the year ended December 31, 2025. The Company recognized $40.0 million as revenue upon the achievement of development milestones related to the Takeda Agreement on its consolidated statement of operations for the nine months ended September 30, 2026. Payment of $20.0 million was received during the nine months ended September 30, 2026. The Company expects to receive the remaining $20.0 million during the three months ended December 31, 2026.

Takeda's obligation to pay royalties for a given licensed product in a given country in the Takeda Territory will begin on the date of the first commercial sale for such licensed product in such country and continue until the latest of (i) 10 years from the date of the first commercial sale for such licensed product in such region, (ii) the expiration of the last valid claim of certain licensed patents, and (iii) expiration of regulatory exclusivity in such region.

The Takeda Agreement will continue in force until the expiration of the royalty term. Takeda may terminate the Takeda Agreement (i) in its entirety or on a country-by-country basis for convenience, with notice or (ii) if Takeda reasonably determines that the development, manufacture, and commercialization of the licensed compound or licensed product pose a safety or public health risk. The Company may terminate the Takeda Agreement in its entirety in the event that Takeda or its affiliates bring a patent challenge. Either party may terminate the Takeda Agreement in its entirety (i) if the other party materially breaches the Takeda Agreement and fails to cure such breach; or (ii) upon the bankruptcy of the other party.

The Company assessed this arrangement in accordance with ASC 606 and concluded it is a contract with a customer as defined in ASC 606. As the Company entered into the Takeda Agreement and the TSA concurrently, they were evaluated as a single contract under ASC 606. The Company identified two performance obligations in the Takeda Agreement and TSA including the license to its intellectual property ("License and Know-How") and the Transition Services. The Company determined that the License and Know-How were distinct from the Transition Services as Takeda is able to derive benefit from the License and Know-How upon delivery and the Transition Services do not modify the License and Know-How.

The transaction price at contract inception included fixed consideration consisting of the upfront fee of $200.0 million. The transaction price at inception also included estimated variable consideration of $51.4 million relating to the Transition Services revenue that was not constrained. The transaction price has been allocated to the License and Know-How and the Transition Services on a relative standalone selling price basis. The Company allocated $195.4 million of the transaction price to the License and Know-How, which was recognized at a point in time, when control of the License and Know-How was transferred to Takeda on the effective date of the Takeda Agreement. The remaining $56.1 million of the transaction price was allocated to the Transition Services, which will be recognized over time as services are provided using the input method, based on costs incurred to provide the Transition Services, as the level of costs incurred over-time best reflect the transfer of services. As of December 31, 2025, the Company recognized $38.5 million of service revenue for the Transition Services and such services are substantially complete. Any remaining services to be performed pursuant to the TSA will be recognized as performed and based on rates agreed to in the TSA. The $10.0 million, $20.0 million and $20.0 million development milestones were recognized as license revenue upon achievement in July 2025, July 2026 and September 2026, respectively.

The remaining $40.0 million of specified development milestones, $280.0 million of specified commercial milestones and $740.0 million of specified sales milestones were considered constrained at contract inception and excluded from the transaction price since the Company could not conclude it is probable that a significant revenue reversal in the amount recognized will not occur. The sales-based milestone payments and royalties will be recognized upon occurrence based on the sales and usage-based royalty exception. The Company will reevaluate the transaction price at the end of each reporting period and will adjust the estimate of the transaction price as uncertain events are resolved or other changes in circumstances occur. The Company determined that the Takeda Agreement and TSA did not contain a significant financing component.

Accounts receivable due from Takeda were $20.0 million as of September 30, 2026 and $3.6 million as of December 31, 2025.

Revenue recognized from Takeda for the three and nine months ended September 30, 2026 and 2025 is as follows (in thousands):

	THREE MONTHS ENDED SEPTEMBER 30,		NINE MONTHS ENDED SEPTEMBER 30,	
	2026	2025	2026	2025
License revenue	$ 40,000	$ 10,000	$ 40,000	$ 205,355
Service revenue	$ 9	$ 4,124	$ 41	$ 38,129
Total revenue from Takeda	$ 40,009	$ 14,124	$ 40,041	$ 243,484

13. SEGMENT REPORTING

The following table presents segment revenue and significant segment expenses (in thousands) for the three and nine months ended September 30, 2026 and 2025:

	THREE MONTHS ENDED SEPTEMBER 30,		NINE MONTHS ENDED SEPTEMBER 30,	
	2026	2025	2026	2025
REVENUE:				
Total segment revenue	40,009	14,262	40,376	243,676
LESS:				
Rinvatercept program expenses	(5,048)	(1,345)	(11,799)	(4,293)
Elritercept program expenses	4	(3,341)	(13)	(37,872)
Cibotercept program expenses	152	(1,218)	209	(11,185)
Nonclinical & development expenses	(4,387)	(1,700)	(12,504)	(5,425)
Compensation costs (excluding stock based compensation)	(6,955)	(9,568)	(22,870)	(37,187)
Other external costs	(6,287)	(7,731)	(20,208)	(27,229)
Other segment items[2]	(3,335)	3,361	(11,456)	(10,010)
Net income (loss)	14,153	(7,280)	(38,265)	110,475

The Company manages operations on a consolidated basis as a single reportable and single operating segment focused on developing novel therapeutics. Segment revenue above is 100 percent attributed to the Company's agreements with Takeda and Hansoh and is equal to consolidated total revenue. All revenues are derived in the United States where the agreements originated. The Company manages expenses on a program level. The significant expense categories outlined above align with the segment-level information that is regularly provided to the chief operating decision maker ("CODM") to allocate resources, assess performance of the reportable segment, and make key operating decisions. On a quarterly basis, the Company's CODM, who is its Chief Executive Officer, reviews financial information, including consolidated net income, clinical expenses by program, other company expenses and a long-range cash flow projection, to make resource decisions for the Company's programs to achieve the approved corporate goals. No segment asset information is provided above as the CODM is focused on how expenses impact ending cash by period and overall cash runway. Any review of segment assets, which would focus on cash and cash equivalents, would be at the same level as the consolidated balance sheet. All long-lived assets are held in the United States.

14. CORPORATE RESTRUCTURING

In May 2025, the Board formally approved a plan to reduce the overall workforce by approximately 45% (the "2025 Restructuring"). In connection with the 2025 Restructuring, the Company incurred one-time employee termination benefits, including severance, retention and benefits costs. The total cost associated with the 2025 Restructuring was $3.1 million, including severance, retention and benefits costs, of which $2.5 million and $0.5 million were recognized as part of total research and development and general and administrative expenses, respectively, during the year ended December 31, 2025.

[2] Other segment items include other expense, income tax provision, bank fees, depreciation expense, dividend income, interest income and stock-based compensation.

All costs have been incurred under the Company's single reportable segment. The 2025 Restructuring was complete as of December 31, 2025.

Accrued compensation and benefits costs as of September 30, 2026 is as follows (in thousands):

	RESTRUCTURING LIABILITY
Balance as of December 31, 2025	145
Expenses incurred	—
Cash payments	(145)
Balance as of September 30, 2026	—

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

You should read the following discussion and analysis of our financial condition and results of operations together with (1) our unaudited interim condensed consolidated financial statements and the related notes and other financial information included elsewhere in this Quarterly Report on Form 10-Q and (2) the audited consolidated financial statements and the related notes and management's discussion and analysis of financial condition and results of operations for the fiscal year ended December 31, 2025 included in our Annual Report on Form 10-K for the year ended December 31, 2025, and filed with the Securities and Exchange Commission, or SEC, on March 4, 2026, which we refer to as the Annual Report.

Some of the information contained in this discussion and analysis or set forth elsewhere in this Quarterly Report on Form 10-Q, including information with respect to our plans and strategy for our business, includes forward-looking statements that involve risks and uncertainties. As a result of many factors, including those factors set forth in the "Risk Factors" section of this Quarterly Report on Form 10-Q, our actual results could differ materially from the results described in or implied by the forward-looking statements contained in the following discussion and analysis. You should carefully read the section titled "Risk Factors" set forth in Part II, Item 1A of this Quarterly Report on Form 10-Q to gain an understanding of the important factors that could cause actual results to differ materially from our forward-looking statements. Please also see the section entitled "Special Note Regarding Forward-Looking Statements." You should, therefore, not rely on these forward-looking statements as representing our views as of any date subsequent to the date of this Quarterly Report on Form 10-Q.

Overview

We are a clinical-stage biopharmaceutical company focused on developing and commercializing novel therapeutics to treat a wide range of patients with disorders that are linked to dysfunctional signaling of the transforming growth factor-beta, or TGF-ß, family of proteins. We are a leader in understanding the role of the TGF-ß family of proteins, which are master regulators of the growth, repair and maintenance of a number of tissues, including skeletal muscle, bone, adipose, heart tissue and blood. By leveraging this understanding, we have discovered and are developing protein therapeutics that have the potential to provide meaningful and potentially disease-modifying benefit to patients.

Our lead product candidate, rinvatercept (KER-065), is designed to bind to and inhibit TGF-ß ligands, including myostatin (GDF8) and activin A, which are negative regulators of muscle and bone mass and strength. Through inhibition of these TGF-ß ligands, we believe that rinvatercept has the potential to increase skeletal muscle regeneration, increase muscle size and strength, reduce body fat, reduce fibrosis of the skeletal muscle and increase bone strength. We are developing rinvatercept for the treatment of Duchenne muscular dystrophy, or DMD, and for the treatment of amyotrophic lateral sclerosis, or ALS. Glucocorticoids, the standard of care in DMD, can have significant side effects when used long-term, including catabolism of muscle, increased fat and accelerated bone loss. In March 2025, we announced initial topline results from the Phase 1 clinical trial of rinvatercept in healthy adult male volunteers. We have commenced our Phase 2 clinical trial of rinvatercept in patients with DMD, and expect to announce initial data from this trial in the first half of 2027. We also plan to engage regulators on the design of a Phase 2 clinical trial of rinvatercept in patients with ALS in the second half of 2026.

Our most advanced product candidate, elritercept (KER-050), is an engineered ligand trap comprised of a modified ligand-binding domain of the TGF-ß receptor known as activin receptor type IIA, or ActRIIA, that is fused to the portion of the human antibody known as the Fc domain. Elritercept is being developed for the treatment of low blood cell counts, or cytopenias, including anemia and thrombocytopenia, in patients with myelodysplastic syndromes, or MDS, and in patients with myelofibrosis. In December 2024, we entered into an exclusive license agreement with Takeda Pharmaceuticals U.S.A., Inc., or Takeda, to further develop, manufacture and commercialize elritercept worldwide outside of mainland China, Hong Kong and Macau, which became effective on January 16, 2025. Elritercept is designed to increase red blood cell and platelet

production by inhibiting the signaling of a subset of the TGF-ß family of proteins to promote hematopoiesis. We believe elritercept has the potential to provide benefit to patients suffering from red blood cell and platelet differentiation and maturation defects occurring across the spectrum from early through terminal stages of hematopoiesis, and consequently may be effective for many patients that have limited treatment options or are refractory to available therapies. In July 2025, we announced that the first patient was dosed in the placebo-controlled Phase 3 RENEW clinical trial in patients with very low-, low-, or intermediate-risk MDS, which we refer to as lower-risk MDS. The dosing of the first patient triggered a $10 million milestone payment to us under the license agreement with Takeda. In July 2026, we announced that the first patient was dosed in the open-label, randomized Phase 3 ELRiSE MDS clinical trial evaluating the efficacy and safety of elritercept versus epoetin alfa for the treatment of anemia due to lower-risk MDS. The dosing of the first patient triggered a $20 million milestone payment payable to us under the license agreement with Takeda. In September 2026, we announced that the first patient was dosed in the double-blind, randomized Phase 3 ELRiSE MF clinical trial evaluating the efficacy and safety of elritercept to placebo in patients with myelofibrosis and anemia on concurrent ruxolitinib therapy. The dosing of the first patient triggered a $20 million milestone payment payable to us under the license agreement with Takeda. We plan to distribute 25% of the net cash proceeds we receive on or before December 31, 2028 from the license agreement with Takeda to our stockholders.

Since our inception in 2015, we have devoted the majority of our efforts to business planning, research and development of our product candidates, including by conducting clinical trials and nonclinical studies, raising capital and recruiting management and technical staff to support these operations. To date, we have not generated any revenue from product sales as none of our product candidates have been approved for commercialization. We have historically financed our operations primarily through the sale of convertible preferred stock, common stock and cash received from licensing agreements.

ATM Sales Agreement

In May 2021, we entered into a Sales Agreement with Leerink Partners LLC, or Leerink, as sales agent, which we refer to as the ATM Sales Agreement, under which we may offer and sell, from time to time, shares of our common stock, or the ATM Shares, through Leerink, which we refer to as the ATM Offering. In September 2026, we filed a shelf registration statement on Form S-3 (File No. 333-298854) with the Securities and Exchange Commission, or the SEC, and a prospectus supplement, which registered the offering, issuance and sale of up to $350.0 million of various equity and debt securities, including the issuance and sale, if any, of up to $100.0 million of shares of our common stock under the ATM Sales Agreement pursuant to the prospectus supplement. The SEC declared the registration statement effective on September 16, 2026. During the three and nine months ended September 30, 2026, we did not sell any shares of our common stock pursuant to the ATM Offering.

Under the ATM Sales Agreement, Leerink may sell the ATM Shares by methods deemed to be an “at-the-market offering” as defined in Rule 415(a)(4) promulgated under the Securities Act of 1933, as amended. We may sell the ATM Shares in amounts and at times to be determined by us from time to time subject to the terms and conditions of the ATM Sales Agreement, but we have no obligation to sell any of the ATM Shares in the ATM Offering. As of September 30, 2026, we had sold a total of 4,290,096 shares of our common stock pursuant to the ATM Offering for aggregate net proceeds of approximately $228.6 million after deducting sales agent commissions and estimated offering expenses. As of September 30, 2026, we may offer and sell ATM Shares at an aggregate offering price of up to $100.0 million (less any sales agent commissions) available under the ATM Offering.

October 2025 Share Repurchases

On October 15, 2025, we entered into the Repurchase Agreements with the ADAR1 Parties and the Pontifax Parties. Pursuant to the terms and conditions of the Repurchase Agreements, the ADAR1 Parties and the Pontifax Parties sold all of the shares of our common stock beneficially owned by them, being an aggregate of 10,176,595 shares of common stock, to us at a per share purchase price of $17.75 per share, for an aggregate purchase price of $180.6 million. In addition, concurrently with the execution of the Pontifax Repurchase Agreement, each of Tomer Kariv and Ran Nussbaum resigned from our board of directors and all committees thereof.

Pursuant to the Repurchase Agreements, each of the ADAR1 Parties and the Pontifax Parties agreed to customary standstill restrictions and voting commitments, which will remain in effect until immediately following the final certification of the voting results for our 2028 annual stockholder meeting. We and each of the ADAR1 Parties and the Pontifax Parties also agreed to customary mutual non-disparagement obligations to remain in effect during the same period.

November 2025 Issuer Tender Offer

On October 20, 2025, we announced that our board of directors authorized the Tender Offer, which we launched on October 20, 2025 and completed on November 21, 2025.

On November 20, 2025, we disclosed that a total of 17,712,262 shares of our common stock were validly tendered and not validly withdrawn. In accordance with the terms and conditions of the Tender Offer, we accepted for purchase a total of

10,950,165 Tender Offer Shares at a purchase price of $17.75 per share, for an aggregate purchase price of approximately $194.4 million. As a result of the Tender Offer, we had 19,543,706 shares of common stock outstanding immediately following the closing of the Tender Offer.

September 2026 Share Repurchase Program

On September 8, 2026, we announced that our board of directors approved a share repurchase program for up to $5.0 million of our common stock, following receipt of a $20 million development milestone payment under the license agreement with Takeda in September 2026.

Financial Overview

We have incurred recurring operating losses each fiscal year through December 31, 2024. Our net income for the year ended December 31, 2025 was primarily driven by revenue related to the Takeda Agreement. Our ability to generate product revenue sufficient to achieve profitability will depend on the successful development and commercialization of one or more of our product candidates. We had net income of $14.2 million for the three months ended September 30, 2026, which was primarily driven by the achievement of two milestones related to our Takeda Agreement. Our net loss was $38.3 million for the nine months ended September 30, 2026. As of September 30, 2026, we had an accumulated deficit of $520.0 million. We expect to continue to generate operating losses and negative operating cash flows for the foreseeable future in connection with our ongoing activities. As of September 30, 2026, we had cash and cash equivalents of $259.5 million.

Nonclinical Update

In October 2026, we presented a nonclinical poster on a research version of KER-206, or RKER-206, at the American Society of Bone and Mineral Research 2026 Annual Meeting. KER-206 is a nonclinical asset that was nominated out of our proprietary library of ActRII ligand traps for further investigation. KER-206 is a novel ligand trap comprised of a modified ligand-binding domain derived from ActRIIB that is fused to the portion of the human antibody known as the Fc domain. KER-206 is designed to bind select ligands from the TGF-β superfamily, including activin A, activin B, GDF8 and GDF11, which regulate bone remodeling through effects on differentiation and activity of bone-resorptive cells (osteoclasts) and bone-forming cells (osteoblasts). Through inhibition of these TGF-ß ligands, we believe that KER-206 has the potential to increase bone mass and improve skeletal integrity in disorders characterized by pathological bone loss.

Known Trends, Events and Uncertainties

Recent trends towards rising inflation and prices may materially affect our business and corresponding financial position and cash flows. Inflationary factors, such as increases in the cost of materials and supplies relating to our nonclinical studies, clinical trials, interest rates and overhead costs may adversely affect our operating results. Rising interest rates and implementation of tariffs present a recent challenge impacting the U.S. economy and could make it more difficult for us to obtain traditional financing on acceptable terms, if at all, in the future. Additionally, the general consensus among economists suggests that we should expect a higher recession risk to continue over the next year due in part to ongoing tariff and trade issues, which, together with the foregoing, could result in further economic uncertainty and volatility in the capital markets in the near term, and could negatively affect our operations. Furthermore, such economic conditions have produced downward pressure on share prices. Although we do not believe that inflation, higher interest rates or tariffs have had a material impact on our financial position or results of operations to date, we may experience increases in the near future (especially if inflation rates rise more quickly) on our operating costs, including our labor costs and research and development costs, due to supply chain constraints, consequences associated with public health crises and global geopolitical tensions, such as the ongoing war between Russia and Ukraine and the wars in the Middle East, worsening global macroeconomic conditions and employee availability and wage increases, which may result in additional stress on our working capital resources.

Licensing Agreements

2024 License Agreement with Takeda Pharmaceuticals U.S.A., Inc.

In December 2024, we entered into a license agreement with Takeda, which became effective on January 16, 2025. Under the terms of the license agreement with Takeda, or the Takeda Agreement, we granted to Takeda the exclusive right to develop, manufacture and commercialize elritercept and certain derivative compounds globally, excluding the territories of mainland China, Hong Kong and Macau, which we refer to collectively as the Takeda Territory.

Pursuant to the terms of the Takeda Agreement, we received a $200.0 million upfront payment in February 2025. In July 2025, we announced that the first patient was dosed in the Phase 3 RENEW clinical trial of elritercept, which triggered a $10 million milestone payment to us under the Takeda Agreement. We received the $10.0 million payment for the achievement of this development milestone in August 2025. In July 2026, we announced that the first patient was dosed in the open-label, randomized Phase 3 ELRiSE MDS clinical trial, which triggered a one-time $20.0 million development milestone under the Takeda Agreement. We received the $20.0 million payment for the achievement of this development milestone in September 2026. In September 2026, we announced that the first patient was dosed in the double-blind, randomized Phase 3 ELRiSE

MF clinical trial, which triggered a one-time $20.0 million milestone payment payable to us under the Takeda Agreement. We have not received the payment for this milestone as of September 30, 2026. In addition to the upfront payment and milestone payments, we are entitled to receive up to an aggregate of (i) $40.0 million upon the achievement of specified development milestones, (ii) $280.0 million upon the achievement of specified commercial milestones and (iii) $740.0 million upon the achievement of specified sales milestones. If a licensed product is approved for marketing in the Takeda Territory, we will be entitled to receive royalty payments based on tiered increments of annual net sales in the Takeda Territory, with such percentage ranging from the low double-digits to high teens, subject to specified potential royalty reductions.

Takeda's obligation to pay royalties for a given licensed product in a given country in the Takeda Territory will begin on the date of the first commercial sale for such licensed product in such country and continue until the latest of (i) 10 years from the date of the first commercial sale for such licensed product in such region, (ii) the expiration of the last valid claim of certain licensed patents, and (iii) expiration of regulatory exclusivity in such region.

The Takeda Agreement will continue in force until the expiration of the royalty term. Takeda may terminate the Takeda Agreement (i) in its entirety or on a country-by-country basis for convenience, with notice or (ii) if Takeda reasonably determines that the development, manufacture, and commercialization of the licensed compound or licensed product pose a safety or public health risk. We may terminate the Takeda Agreement in its entirety in the event that Takeda or its affiliates bring a patent challenge. Either party may terminate the Takeda Agreement in its entirety (i) if the other party materially breaches the Takeda Agreement and fails to cure such breach; or (ii) upon the bankruptcy of the other party.

2021 License Agreement with Hansoh (Shanghai) Healthtech Co., Ltd.

On December 12, 2021, we entered into a license agreement with Hansoh (Shanghai) Healthtech Co., Ltd., or Hansoh. Under the terms of the license agreement with Hansoh, or the Hansoh Agreement, we granted to Hansoh the exclusive right to develop, manufacture and commercialize elritercept and licensed products containing elritercept within the territories of mainland China, Hong Kong and Macau, which we refer to collectively as the Hansoh Territory.

In connection with the Hansoh Agreement, Hansoh will purchase clinical trial supply of elritercept from us, and the parties will also negotiate in good faith to enter into an agreement for commercial supply prior to any anticipated commercialization in the Hansoh Territory. In addition, Hansoh will use commercially reasonable efforts to develop, obtain regulatory approval for, and commercialize licensed products in any region in the Hansoh Territory.

Pursuant to the terms of the Hansoh Agreement, we received a net $18.0 million upfront payment in January 2022. In addition to the upfront payment and development milestones achieved to date, we are entitled to receive up to an aggregate of (i) $23.5 million upon the achievement of specified development milestones and (ii) $144.0 million upon the achievement of specified net sales thresholds for all licensed products in the Hansoh Territory. If a licensed product is approved for marketing in the Hansoh Territory, we will be entitled to receive royalty payments based on a tiered percentage of annual net sales in each region within the Hansoh Territory, with such percentage ranging from the low double digit to high teens, subject to specified potential royalty reductions. We did not recognize any revenue related to milestones for the three and nine months ended September 30, 2026 or 2025.

Hansoh's obligation to pay royalties for a given licensed product in a given region in the Hansoh Territory will begin on the date of the first commercial sale for such licensed product in such region and continue until the latest of (i) ten years from the date of the first commercial sale for such licensed product in such region, (ii) the expiration of the last valid claim of certain licensed patents or joint patents, and (iii) expiration of regulatory exclusivity in such region. During the royalty term, neither party will directly or indirectly commercialize a competing product in the Hansoh Territory.

Effective in June 2023, in connection with the Hansoh Agreement, we entered into a manufacturing technology transfer agreement, or the Tech Transfer Agreement, with Hansoh. The Tech Transfer Agreement governs the transfer to Hansoh of all documents and information required to complete the manufacturing technology transfer. Under the Tech Transfer Agreement, Hansoh is obligated to make certain payments to us, at the rates set forth in the Tech Transfer Agreement, as manufacturing technology transfer services are provided over the term of the Tech Transfer Agreement. We did not recognize any revenue during the three and nine months ended September 30, 2026. We recognized zero and $0.1 million of service and other revenue during the three and nine months ended September 30, 2025, respectively.

Effective in February 2024, in connection with the Hansoh Agreement, we entered into a clinical product supply agreement with Hansoh, or the Supply Agreement. We recognized zero and $0.3 million of service and other revenue during the three and nine months ended September 30, 2026, respectively. We recognized $0.1 million of service and other revenue during the three and nine months ended September 30, 2025.

2016 Exclusive Patent License Agreement with The General Hospital Corporation

In April 2016, we entered into an exclusive patent license agreement with The General Hospital Corporation, or MGH, and such agreement was subsequently amended in May 2017 and February 2018. Under the license agreement with MGH, or the

MGH Agreement, we obtained an exclusive, worldwide license, with the right to sublicense, under certain patents and technical information of MGH, to make, have made, use, have used, sell, have sold, lease, have leased, import, have imported or otherwise transfer licensed products and processes for use in the treatment, diagnosis, palliation and prevention of diseases and disorders in humans and animals. We are required to use commercially reasonable efforts to develop and commercialize licensed products and processes, and must achieve certain required diligence milestones.

Additionally, we are required to pay a low-five digit to mid-five digit annual maintenance fee prior to the first commercial sale of our first product or process, a mid-five digit annual maintenance fee after the first commercial sale of our first product or process that is creditable against royalties, certain clinical and regulatory milestone payments for the first three products or indications to achieve such milestones, which milestone payments are $8.6 million in the aggregate, and certain commercial milestone payments for the first three products or indications to achieve such milestones, which milestone payments are $18.0 million in the aggregate. We made payments of $50,000 and $300,000 in 2020 and 2021, respectively, for the achievement of the clinical and regulatory milestones of (i) filing of an IND in the first country and (ii) the completion of a Phase 1 clinical trial, respectively. We are also obligated to pay tiered royalties on net sales of licensed products ranging in the low-single digits to mid-single digits. The royalty rates are subject to up to a maximum 50% reduction for lack of a valid claim, in the event that it is necessary for us to obtain a license to any third-party intellectual property related to the licensed products, and generic competition. The obligation to pay royalties under the MGH Agreement expires on a licensed product-by-licensed product and country-by-country basis upon the later of expiry of the last valid claim of the licensed patents that cover such licensed product in such country and ten years from the first commercial sale of such product in such country. We are also obligated to pay a percentage of non-royalty-related payments received by us from sublicensees ranging in the sub-teen double digits and a change of control fee equal to a low-single digit percentage of the payments received as part of any completed transaction up to a low-seven digit amount.

Components of Our Results of Operations

Revenue

To date, we have not generated any revenue, and do not expect to generate any revenue in the foreseeable future, from product sales. We have generated revenue solely from research collaborations or licensing of intellectual property and related transition services. We may in the future generate revenue from other strategic collaborations or licensing arrangements.

Operating Expenses

Research and Development Expenses

Research and development expenses consist primarily of costs incurred for our research activities, including our discovery efforts and the nonclinical and clinical development of our current and potential future product candidates, and include:

- salaries, benefits and other related costs, including stock-based compensation expense, for personnel engaged in research and development functions;
- expenses incurred under agreements with third parties, including CROs that conduct research, nonclinical and clinical activities on our behalf, as well as contract manufacturing organizations, or CMOs, that manufacture drug product for use in our nonclinical studies and clinical trials;
- license fees incurred in connection with license agreements;
- research and development supplies and services expenses;
- facility-related expenses, which include direct depreciation costs and allocated expenses for rent and maintenance of facilities and other operating costs;
- cost of outside consultants, including their fees and related travel expenses, engaged in research and development functions;
- expenses related to regulatory affairs; and
- fees related to our scientific advisory board.

We expense research and development costs as incurred. Costs for external development activities are recognized based on an evaluation of the progress to completion of specific tasks using information provided to us by our vendors. Payments for these activities are based on the terms of the individual agreements, which may differ from the pattern of costs incurred, and are reflected in our unaudited interim condensed consolidated financial statements as prepaid or accrued research and development expenses. Nonrefundable advance payments for goods or services to be received in the future for use in

research and development activities are recorded as prepaid expenses and expensed as the related goods are delivered or the services are performed.

Research and development activities are central to our business model. We expect that our research and development expenses will continue to increase for the foreseeable future, with the exception of elritercept-related expenses which are expected to decrease once transitioned to Takeda, as we continue ongoing and initiate new clinical trials for our product candidates and continue to discover and develop additional product candidates. We expect research and development expenses to fluctuate from quarter to quarter depending on the timing of clinical trial activities, clinical manufacturing and other development activities. If any of our product candidates enter into later stages of clinical development, they will generally have higher development costs than those in earlier stages of clinical development, primarily due to the increased size and duration of later-stage clinical trials. There are numerous factors associated with the successful commercialization of any product candidates we may develop in the future, including future trial design and various regulatory requirements, many of which cannot be determined with accuracy at this time based on our stage of development. Additionally, future commercial and regulatory factors beyond our control will impact our clinical development program and plans.

General and Administrative Expenses

General and administrative expenses consist primarily of salaries and other related costs, including stock-based compensation, for personnel in our executive, finance, corporate and business development and administrative functions. General and administrative expenses also include professional fees for legal, patent, accounting, information technology, auditing, tax and consulting services, travel expenses and facility-related expenses, which include direct depreciation costs and allocated expenses for rent and maintenance of facilities and other operating costs.

We expect that our general and administrative expenses will increase in the future as we increase our headcount to support our continued research and development and potential commercialization of our product candidates. We have incurred and expect to continue to incur increased expenses associated with being a public company, including costs of accounting, audit, legal, regulatory and tax compliance services, director and officer insurance costs, and investor and public relations costs.

Other Income (Expense), Net

Dividend Income

Dividend income consists of income earned on our money market funds that are recorded as cash equivalents on our consolidated balance sheets.

Other Expense, Net

Other expense, net primarily consists of unrealized and realized gains and losses on foreign currency and other taxes and fees.

Interest Income, Net

Interest income, net consists of income earned on our bank accounts.

Income Tax (Provision) Benefit

The income tax (provision) benefit has historically resulted from income tax related to taxable income generated from the Takeda Agreement. We have not recorded any income tax benefits for the losses incurred to date as it is more likely than not that these benefits will not be realized based on our history of losses and expected future losses.

Results of Operations

Comparison for the three months ended September 30, 2026 and 2025

The following table summarizes our results of operations for the three months ended September 30, 2026 and 2025 (in thousands):

	THREE MONTHS ENDED SEPTEMBER 30,	
	2026	2025
REVENUE:		
Service and other revenue	$ 9	$ 4,262
License revenue	40,000	10,000
Total revenue	40,009	14,262
OPERATING EXPENSES:		
Research and development	(20,491)	(19,519)
General and administrative	(6,700)	(10,127)
Total operating expenses	(27,191)	(29,646)
INCOME (LOSS) FROM OPERATIONS	12,818	(15,384)
OTHER INCOME (EXPENSE), NET		
Interest income, net	55	—
Dividend income	2,220	6,933
Other expense, net	(168)	(253)
Total other income, net	2,107	6,680
Income (loss) before income taxes	14,925	(8,704)
Income tax (provision) benefit	(772)	1,424
Net income (loss)	$ 14,153	$ (7,280)

Revenue

We recognized $9.0 thousand of service and other revenue related to the transition services agreement with Takeda, or the TSA, and $40.0 million of license revenue related to the achievement of two development milestones under the Takeda Agreement for the three months ended September 30, 2026, compared to $4.3 million of service and other revenue primarily related to the TSA, and $10.0 million of license revenue related to the achievement of a development milestone under the Takeda Agreement for the three months ended September 30, 2025. The increase in total revenue of $25.7 million was primarily due to recognition of license revenue for the two development milestones achieved under the Takeda Agreement during the three months ended September 30, 2026.

Research and Development Expenses

The following table summarizes our research and development expenses for the three months ended September 30, 2026 and 2025 (in thousands):

	THREE MONTHS ENDED SEPTEMBER 30,		INCREASE / (DECREASE)
	2026	2025	
Rinvatercept	$ 5,048	$ 1,345	$ 3,703
Elritercept	(4)	3,341	(3,345)
Cibotercept	(152)	1,218	(1,370)
Nonclinical and development fees	4,387	1,700	2,687
Personnel expenses (including stock-based compensation)	7,202	7,872	(670)
Professional fees	1,442	1,720	(278)
Facilities and supplies	2,084	1,841	243
Other expenses	484	482	2
	$ 20,491	$ 19,519	$ 972

Research and development expenses were $20.5 million for the three months ended September 30, 2026, compared to $19.5 million for the three months ended September 30, 2025. The increase of $1.0 million was primarily due to (i) an increase of $3.7 million of rinvatercept-related expenses, primarily driven by an increase of $1.9 million in manufacturing and nonclinical activities and an increase of $1.8 million in clinical spend associated with our Phase 2 clinical trial in patients with DMD and our planned Phase 2 clinical trial in patients with ALS; and (ii) an increase of $2.7 million in nonclinical pipeline and development activities. These increases were partially offset by (a) a decrease of $3.3 million of elritercept-related expenses as activities transitioned to Takeda during 2025; (b) a decrease of $1.4 million of cibotercept-related expenses related to our deprioritization of cibotercept; and (c) a decrease of $0.7 million related to personnel expenses, primarily driven by a net decrease of $1.7 million in salaries, severance, bonus and retention bonus and partially offset by an increase of $1.0 million of stock-based compensation costs. We expect research and development expenses to fluctuate from quarter to quarter depending on the timing of clinical trial activities, clinical manufacturing and other development activities.

General and Administrative Expenses

General and administrative expenses were $6.7 million for the three months ended September 30, 2026, compared to $10.1 million for the three months ended September 30, 2025. The decrease of approximately $3.4 million was primarily due to (i) a net decrease of $1.7 million in professional fees, insurance, facilities, supplies and other expenses; and (ii) a decrease of $1.7 million in personnel expenses, which includes a decrease of $0.8 million of stock-based compensation costs.

Total Other Income, Net

Total other income, net was $2.1 million for the three months ended September 30, 2026, compared to $6.7 million for the three months ended September 30, 2025. The decrease of $4.6 million was primarily driven by a decrease of $4.7 million in dividend income.

Income Tax (Provision) Benefit

Income tax provision was $0.8 million for the three months ended September 30, 2026, compared to an income tax benefit of $1.4 million for the three months ended September 30, 2025. The prior period benefit was driven by deductible expenses incurred that reduced the taxable liability associated with the Takeda Agreement.

Comparison for the nine months ended September 30, 2026 and 2025

The following table summarizes our results of operations for the nine months ended September 30, 2026 and 2025 (in thousands):

	NINE MONTHS ENDEDSEPTEMBER 30,	
	2026	2025
REVENUE:		
Service and other revenue	$ 376	$ 38,321
License revenue	40,000	205,355
Total revenue	40,376	243,676
OPERATING EXPENSES:		
Research and development	(58,919)	(111,731)
General and administrative	(25,453)	(35,106)
Total operating expenses	(84,372)	(146,837)
INCOME (LOSS) FROM OPERATIONS	(43,996)	96,839
OTHER INCOME (EXPENSE), NET		
Interest income, net	55	—
Dividend income	6,842	20,845
Other expense, net	(394)	(812)
Total other income, net	6,503	20,033
Income (loss) before income taxes	(37,493)	116,872
Income tax (provision) benefit	(772)	(6,397)
Net income (loss)	$ (38,265)	$ 110,475

Revenue

We recognized $0.4 million of service and other revenue primarily related to the Hansoh Agreement and $40.0 million of license revenue related to the achievement of two development milestones under the Takeda Agreement for the nine months ended September 30, 2026, compared to $38.3 million of service and other revenue primarily related to the TSA and $205.4 million of license revenue related to the upfront payment and achievement of a development milestone under the Takeda Agreement for the nine months ended September 30, 2025. The decrease in total revenue of $203.3 million was primarily due to the recognition of license revenue for the upfront payment under the Takeda Agreement and service and other revenue related to the TSA during the nine months ended September 30, 2025.

Research and Development Expenses

The following table summarizes our research and development expenses for the nine months ended September 30, 2026 and 2025 (in thousands):

	NINE MONTHS ENDED SEPTEMBER 30,		INCREASE / (DECREASE)
	2026	2025	
Rinvatercept	$ 11,799	$ 4,293	$ 7,506
Elritercept	13	37,872	(37,859)
Cibotercept	(209)	11,185	(11,394)
Nonclinical and development fees	12,504	5,425	7,079
Personnel expenses (including stock-based compensation)	23,333	39,634	(16,301)
Professional fees	3,780	4,846	(1,066)
Facilities and supplies	6,057	6,981	(924)
Other expenses	1,642	1,495	147
	$ 58,919	$ 111,731	$ (52,812)

Research and development expenses were $58.9 million for the nine months ended September 30, 2026, compared to $111.7 million for the nine months ended September 30, 2025. The decrease of $52.8 million was primarily due to (i) a decrease of $37.9 million of elritercept-related expenses as activities transitioned to Takeda during 2025; (ii) a decrease of $11.4 million of cibotercept-related expenses related to our deprioritization of cibotercept; (iii) a decrease of $16.3 million related to personnel expenses, including a decrease of $4.4 million of stock-based compensation costs, driven by a reduction in headcount; and (iv) a net decrease of $1.8 million in facilities, supplies, professional fees and other expenses. These decreases were partially offset by (a) an increase of $7.5 million of rinvatercept-related expenses, primarily driven by an increase of $4.9 million in clinical spend associated with our Phase 2 clinical trial in patients with DMD and our planned Phase 2 clinical trial in patients with ALS, and a net increase of $2.6 million in manufacturing and nonclinical activities; and (b) an increase of $7.1 million in nonclinical pipeline and development activities. We expect research and development expenses to fluctuate from quarter to quarter depending on the timing of clinical trial activities, clinical manufacturing and other development activities.

General and Administrative Expenses

General and administrative expenses were $25.5 million for the nine months ended September 30, 2026, compared to $35.1 million for the nine months ended September 30, 2025. The decrease of approximately $9.7 million was primarily due to (i) a net decrease of $6.0 million in professional fees, insurance, facilities, supplies and other expenses; and (ii) a decrease of $3.6 million in personnel expenses, which included a decrease of $1.2 million of stock-based compensation costs.

Total Other Income, Net

Total other income, net was $6.5 million for the nine months ended September 30, 2026, compared to $20.0 million for the nine months ended September 30, 2025. The decrease of $13.5 million was driven by a decrease of $14.0 million in dividend income, partially offset by (i) a decrease of $0.4 million in other expense, net and (ii) an increase of $0.1 million in interest income, net.

Income Tax (Provision) Benefit

Income tax provision was $0.8 million for the nine months ended September 30, 2026, compared to $6.4 million for the nine months ended September 30, 2025. The decrease in income tax provision was attributable to greater taxable income generated during the nine months ended September 30, 2025 related to the revenue recognized from the Takeda Agreement.

Liquidity and Capital Resources

Since our inception, we have incurred significant operating losses each fiscal year through December 31, 2024. Our net income for the year ended December 31, 2025 was primarily driven by revenue related to the Takeda Agreement. Our net loss was $38.3 million for the nine months ended September 30, 2026 compared to net income of $110.5 million for the nine months ended September 30, 2025, which was primarily driven by the one-time upfront fee related to the Takeda Agreement. As of September 30, 2026 and December 31, 2025, we had an accumulated deficit of $520.0 million and $481.8 million, respectively. To date, we have devoted the majority of our efforts to business planning, research and development of our product candidates, including by conducting clinical trials and nonclinical studies, raising capital and recruiting management and technical staff to support these operations. Our primary uses of cash are to fund operating expenses, which are primarily research and development expenditures. We expect to continue to incur substantial expenses in connection with our ongoing activities, particularly as we advance the nonclinical studies and clinical trials of our product candidates. Furthermore, we expect to incur costs associated with operating as a public company, including significant legal, accounting, investor relations, director and officer insurance premiums and other expenses. In addition, if we obtain marketing approval for any of our product candidates, we expect to incur significant commercialization expenses related to establishing sales, marketing, distribution and other commercial infrastructure to commercialize such products.

We currently do not have any products approved for sale. We do not expect to generate any revenue from product sales unless and until we successfully complete development and obtain regulatory approval for one or more of our product candidates, which we expect will take a number of years. Since our inception, we have funded our operations primarily through equity financings, research collaborations, or licensing of intellectual property.

In September 2026, we filed a shelf registration statement on Form S-3 with the SEC and a prospectus supplement, which registered the offering, issuance and sale of up to $350.0 million of various equity and debt securities, including the issuance and sale, if any, of up to $100.0 million of shares of our common stock under the ATM Sales Agreement pursuant to the prospectus supplement. The SEC declared the registration statement effective on September 16, 2026. As of September 30, 2026, we had sold a total of 4,290,096 shares of our common stock pursuant to the ATM Offering. During the three and nine months ended September 30, 2026, we did not sell any shares of our common stock pursuant to the ATM Offering. As of September 30, 2026, we may offer and sell ATM Shares at an aggregate offering price of up to $100.0 million (less any sales agent commissions) available under the ATM Offering.

In October 2025, we entered into the Repurchase Agreements, pursuant to which the ADAR1 Parties and the Pontifax Parties sold all of the shares of our common stock beneficially owned by them, being an aggregate of 10,176,595 shares of common stock, to us at a per share purchase price of $17.75 per share, for an aggregate purchase price of $180.6 million.

In November 2025, we completed our Tender Offer and accepted for purchase a total of 10,950,165 shares of our common stock at a purchase price of $17.75 per share, for an aggregate purchase price of approximately $194.4 million.

On September 8, 2026, we announced that our board of directors approved a share repurchase program for up to $5.0 million of our common stock, following receipt of a $20 million development milestone payment under the license agreement with Takeda in September 2026.

We have also committed to distribute 25% of any net cash proceeds we receive on or before December 31, 2028 under the Takeda Agreement, including the $20 million milestone payment triggered in September 2026 upon dosing of the first patient in the ELRiSE MF clinical trial, to our stockholders.

As of September 30, 2026, we had cash and cash equivalents of $259.5 million. Based on our current operating assumptions, we believe that our existing cash and cash equivalents will be sufficient to fund our projected liquidity requirements into the second half of 2028. We have based this estimate on assumptions that may prove to be wrong, and we could use our capital resources sooner than we currently expect. Due to the numerous risks and uncertainties associated with the development of our product candidates and programs, and because the extent to which we may enter into collaborations with third parties for development of our product candidates is unknown, we are unable to estimate the timing and amounts of increased capital outlays and operating expenses associated with completing the research and development of our product candidates. Our future funding requirements, both near and long-term, will depend on many factors, including:

- the progress, timing and completion of nonclinical studies and clinical trials for our current or any future product candidates, as well as the associated costs, including any unforeseen costs we may incur as a result of nonclinical study or clinical trial delays due to public health crises or other causes;
- the timing and amount of milestone and royalty payments we are required to make or are eligible to receive under our license agreements with each of The General Hospital Corporation, Hansoh, and Takeda;
- the number of potential new product candidates we identify and decide to develop;

- the need for additional or expanded nonclinical studies and clinical trials beyond those that we plan to conduct with respect to our current and future product candidates;
- the costs involved in growing our organization to the size needed to allow for the research, development and potential commercialization of our current or any future product candidates;
- the costs involved in filing patent applications, maintaining and enforcing patents or defending against infringement or other claims raised by third parties;
- the maintenance of our existing license and collaboration agreements and the entry into new license and collaboration agreements;
- the time and costs involved in obtaining regulatory approval for our product candidates and any delays we may encounter as a result of evolving regulatory requirements or adverse results with respect to any of our product candidates;
- the effect of competing technological and market developments;
- the costs of operating as a public company;
- the cost of manufacturing rinvatercept and future product candidates for clinical trials in preparation for marketing approval and in preparation for commercialization;
- the cost of establishing sales, marketing and distribution capabilities for any product candidates for which we may receive regulatory approval in regions where we choose to commercialize our products, if approved, on our own;
- the amount of revenues, if any, we may derive either directly or in the form of royalty payments from future sales of our product candidates, if approved; and
- market acceptance of any approved product candidates.

In addition, implementation of tariffs, public health crises, bank failures, geopolitical tensions and resulting global slowdown of economic activity continue to rapidly evolve and have already resulted in a significant disruption of global financial markets. If the disruption persists and deepens, we could experience an inability to access additional capital when and if needed. If we are unable to obtain funding, we could be forced to delay, reduce or eliminate some or all of our research and development programs and clinical development efforts, which would adversely affect our business prospects, or we may be unable to continue operations. We do not have any committed external source of funds or other support for our development efforts and we cannot be certain that additional funding will be available on acceptable terms, or at all. Until we can generate sufficient product or royalty revenue to finance our cash requirements, which we may never do, we expect to finance our future cash needs through a combination of public or private equity offerings, debt financings, collaborations, strategic alliances, licensing arrangements and other marketing or distribution arrangements. Adequate additional funding may not be available to us on acceptable terms, or at all. Any failure to raise capital as and when needed could have a negative impact on our financial condition and on our ability to pursue our business plans and strategies.

Cash Flows

The following table summarizes our cash flows for the nine months ended September 30, 2026 and 2025 (in thousands):

	NINE MONTHS ENDED SEPTEMBER 30,	
	2026	**2025**
Net cash provided by (used in) operating activities	$ (20,560)	$ 134,797
Net cash used in investing activities	(287)	(1,417)
Net cash provided by (used in) financing activities	(7,104)	161
Net increase (decrease) in cash, cash equivalents and restricted cash	$ (27,951)	$ 133,541

Cash Provided by (Used in) Operating Activities

Net cash used in operating activities was $20.6 million for the nine months ended September 30, 2026, which was driven by (i) a net loss of $38.3 million; and (ii) $1.2 million in net cash used in operating assets and liabilities, partially offset by non-cash charges including $15.8 million of stock-based compensation expense, $2.0 million in lease expenses and $1.2 million in depreciation. The $1.2 million of net cash used in operating assets and liabilities was comprised of (i) a $16.4 million increase in accounts receivable related to the Takeda Agreement; and (ii) a $1.8 million change in operating lease liabilities, which was partially offset by (a) a $16.3 million decrease in prepaid expenses and other current assets due to timing of expense recognition for our research and development costs; (b) a $0.1 million increase in accounts payable and accrued expenses; and (c) a $0.6 million increase in current tax liability.

Net cash provided by operating activities was $134.8 million for the nine months ended September 30, 2025, which was driven by (i) net income of $110.5 million; (ii) $48.0 thousand in net cash provided by operating assets and liabilities; and (iii) non-cash charges including $21.4 million of stock-based compensation expense, $1.8 million in lease expenses and $1.1 million in depreciation. The $48.0 thousand of net cash provided by operating assets and liabilities was comprised of (i) a $4.1 million increase in current tax liability; (ii) a $3.9 million decrease in prepaid expenses and other current assets due to timing of expense recognition for our research and development costs; (iii) a $2.1 million decrease in other long-term assets; and (iv) a $0.2 million increase in deferred revenue; offset by (a) an $8.0 million decrease in accounts payable and accrued expenses to support the advancement of our programs, primarily driven by the transition of elritercept-related expenses to Takeda and the deprioritization of cibotercept, (b) a $1.4 million change in operating lease liabilities; and (c) a $0.8 million increase in accounts receivable related to the Takeda Agreement.

Cash Used in Investing Activities

Net cash used in investing activities was $0.3 million for the nine months ended September 30, 2026 and $1.4 million for the nine months ended September 30, 2025. The cash used in investing activities in both periods was due to purchases of property and equipment.

Cash Provided by (Used in) Financing Activities

Net cash used in financing activities was $7.1 million for the nine months ended September 30, 2026, which was primarily related to (i) $5.7 million in cash paid for direct expenses associated with the Repurchase Agreements and the Tender Offer, and (ii) $1.5 million used to repurchase shares of common stock via the September 2026 share repurchase program, partially offset by issuances of common stock under the 2020 Employee Stock Purchase Plan and exercises of options to purchase common stock.

Net cash provided by financing activities was $0.2 million for the nine months ended September 30, 2025 and was primarily related to issuances of common stock under the 2020 Employee Stock Purchase Plan and exercises of options to purchase common stock.

Contractual Obligations and Commitments

The disclosure of our contractual obligations and commitments was included in the Annual Report. There have been no material changes from the contractual commitments and obligations previously disclosed in the Annual Report, except as described herein.

Purchase Commitments

We enter into agreements in the normal course of business with contract manufacturing organizations for process development, raw material purchases and manufacturing services. These contracts typically do not contain minimum purchase commitments and are generally cancellable by us upon written notice. Payments due upon cancellation consist of payments for services provided or expenses incurred, including noncancellable obligations of our service providers, up to the date of cancellation and, in the case of certain arrangements with contract manufacturing organizations, may include noncancellable fees. Under such agreements, the exact amounts owed by us in the event of termination will be based on the timing of the termination and the exact terms of the agreement. As of September 30, 2026, we have committed up to approximately $4.6 million under these agreements which are expected to be paid through 2028.

Distribution of Proceeds

We plan to distribute 25% of any net cash proceeds we receive on or before December 31, 2028 from the Takeda Agreement to our stockholders. In September 2026, we committed to repurchasing up to $5.0 million of our common stock following the receipt of a $20.0 million development milestone payment under the Takeda Agreement. We began repurchasing our common stock in September 2026 and have repurchased $1.6 million of our common stock, excluding commissions, during the three and nine months ended September 30, 2026. As of September 30, 2026, we plan to repurchase the remaining $3.4 million of our common stock prior to February 28, 2027.

Critical Accounting Estimates

Our unaudited interim condensed consolidated financial statements are prepared in accordance with generally accepted accounting principles in the United States. The preparation of our unaudited interim condensed consolidated financial statements and related disclosures requires us to make estimates and judgments that affect the reported amounts of assets, liabilities, costs and expenses, and the disclosure of contingent assets and liabilities in our condensed financial statements. We base our estimates on historical experience, known trends and events and various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of

assets and liabilities that are not readily apparent from other sources. We evaluate our estimates and assumptions on an ongoing basis. Our actual results may differ from these estimates under different assumptions or conditions.

There have been no significant changes to our critical accounting policies from those described in “Management’s Discussion and Analysis of Financial Condition and Results of Operations,” included in our Annual Report.

Recently Issued Accounting Pronouncements

Refer to Note 2 in the accompanying notes to our unaudited interim condensed consolidated financial statements appearing elsewhere in this Quarterly Report on Form 10-Q for a discussion of recent accounting pronouncements. In the nine months ended September 30, 2026, there were no newly applicable recently issued accounting pronouncements.

ITEM 3. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK

We are a “smaller reporting company,” as defined in Item 10(f)(1) of Regulation S-K. As a result, pursuant to Item 305(e) of Regulation S-K, we are not required to provide the information required by this Item 3.

ITEM 4. CONTROLS AND PROCEDURES

Evaluation of Disclosure Controls and Procedures

We maintain “disclosure controls and procedures,” as defined in Rule 13a-15(e) and Rule 15d-15(e) under the Exchange Act that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported, within the time periods specified in the SEC’s rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is accumulated and communicated to our management, including our principal executive and principal financial officers, as appropriate to allow timely decisions regarding required disclosure.

Our management, with the participation of our Chief Executive Officer and our Corporate Controller and Vice President, Finance, evaluated the effectiveness of our disclosure controls and procedures as of September 30, 2026. Based on the evaluation of our disclosure controls and procedures as of September 30, 2026, our Chief Executive Officer and Corporate Controller and Vice President, Finance concluded that, as of such date, our disclosure controls and procedures were effective at the reasonable assurance level.

Changes in Internal Control Over Financial Reporting

There was no change in our internal control over financial reporting identified in connection with the evaluation required by Rule 13a-15(d) and 15d-15(d) of the Exchange Act that occurred during the period covered by this Quarterly Report on Form 10-Q that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

Inherent Limitations on Effectiveness of Controls

Our disclosure controls and procedures and internal control over financial reporting are designed to provide reasonable assurance of achieving the desired control objectives. Our management recognizes that any control system, no matter how well designed and operated, is based upon certain judgments and assumptions and cannot provide absolute assurance that its objectives will be met. Similarly, an evaluation of controls cannot provide absolute assurance that misstatements due to error or fraud will not occur or that all control issues and instances of fraud, if any, have been detected.

PART II – OTHER INFORMATION

ITEM 1. LEGAL PROCEEDINGS

From time to time, we may become subject to arbitration, litigation or claims arising in the ordinary course of business. We are not currently a party to any material arbitration or legal proceedings. The results of any future claims or proceedings cannot be predicted with certainty, and regardless of the outcome, litigation can have an adverse impact on us because of defense and litigation costs, diversion of management resources, and other factors.

ITEM 1A. RISK FACTORS

Our business is subject to numerous risks. You should consider carefully the risks and uncertainties described below, in addition to other information contained in this Quarterly Report on Form 10-Q as well as our other public filings with the Securities and Exchange Commission, or the SEC. Any of the following risks could have a material adverse effect on our business, financial condition, results of operations and growth prospects and cause the trading price of our common stock to decline.

Risks Related to Our Financial Position and Need for Additional Capital

We have a limited operating history, have incurred net losses in almost every year since our inception and anticipate that we will continue to incur net losses in the future.

We are a clinical-stage biopharmaceutical company with a limited operating history. Since our inception in 2015, we have invested most of our resources in developing our product candidates, building our intellectual property portfolio, developing our supply chain, conducting business planning, raising capital and providing general and administrative support for these operations. Consequently, we have no meaningful operations upon which to evaluate our business and predictions about our future success or viability may not be as accurate as they could be if we had a longer operating history or a history of successfully developing and commercializing drug products. Investment in biopharmaceutical product development is highly speculative because it entails substantial upfront capital expenditures and significant risk that any potential product candidate will fail to demonstrate adequate effect or an acceptable safety profile, gain regulatory approval and become commercially viable. We have not yet demonstrated the ability to progress any product candidate through late-stage clinical trials, we have no products approved for commercial sale and we have not generated any revenue from product sales to date. While we were profitable in the year ended December 31, 2025, this was primarily driven by the one-time upfront payment from our license agreement with Takeda Pharmaceuticals U.S.A., Inc., or Takeda. We continue to incur significant research and development and other expenses related to our ongoing operations. As a result, we have incurred losses in all fiscal years prior to the fiscal year ended December 31, 2025. We reported net income of $14.2 million for the three months ended September 30, 2026, which was primarily driven by the achievement of two milestones related to our Takeda Agreement. We reported a net loss of $38.3 million for the nine months ended September 30, 2026. As of September 30, 2026, we had an accumulated deficit of $520.0 million. We expect to continue to incur significant losses for the foreseeable future, and we expect these losses to increase as we continue our research and development of, and seek regulatory approvals for, our lead product candidate, rinvatercept, our most advanced product candidate, elritercept, and any future product candidates we may develop.

We anticipate that our expenses will increase substantially if, and as, we:

- progress our Phase 2 clinical trial of rinvatercept in patients with Duchenne muscular dystrophy, or DMD;
- commence our Phase 2 clinical trial of rinvatercept in patients with amyotrophic lateral sclerosis, or ALS;
- continue the research and development of our other clinical- and nonclinical-stage product candidates and discovery-stage programs;
- increase the amount of research and development activities to identify and develop product candidates using our proprietary discovery approach;
- make milestone, royalty or other payments under in-license or collaboration agreements;
- maintain, expand and protect our intellectual property portfolio;
- expand our operational, financial and management systems and increase personnel, including personnel to support our clinical development, manufacturing and commercialization efforts and our operations as a public company;
- establish a sales, marketing, medical affairs and distribution infrastructure to commercialize any products for which we may obtain marketing approval and intend to commercialize on our own or jointly with third parties;
- invest in or in-license other technologies; and
- experience any delays or encounter any issues with any of the above, including but not limited to failed studies, complex results, manufacturing challenges, safety issues or other regulatory challenges.

To become and remain profitable, we, our collaborators and any potential future collaborators must develop and eventually commercialize products with significant market potential. This will require us to be successful in a range of challenging activities, including completing nonclinical studies and clinical trials, obtaining marketing approval for product candidates, manufacturing, marketing and selling products for which we may obtain marketing approval and satisfying any post-marketing requirements. We, our collaborators and any potential future collaborators may never succeed in any or all of these activities and, even if we do, we may never generate revenue that is significant or large enough to achieve profitability. Revenue we generate from our collaborations with Takeda and Hansoh (Shanghai) Healthtech Co., Ltd., or Hansoh, and any future collaboration arrangements may not be sufficient to sustain our operations. If we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and remain profitable would decrease

the value of our company and could impair our ability to raise capital, maintain our research and development efforts, expand our business or continue our operations. A decline in the value of our company also could cause you to lose all or part of your investment.

Even if we succeed in commercializing one or more of our product candidates, we will continue to incur substantial research and development and other expenditures to develop and market additional product candidates. We may encounter unforeseen expenses, difficulties, complications, delays and other unknown factors that may adversely affect our business. The size of our future net losses will depend, in part, on the rate of future growth of our expenses and our ability to generate revenue. Our prior losses and expected future losses have had and will continue to have an adverse effect on our stockholders' equity and working capital.

We will need substantial additional funding in order to complete the development and commence commercialization of our product candidates. Failure to obtain this necessary capital when needed may force us to delay, reduce or eliminate certain of our product development or research operations.

To date, we have funded our operations primarily through private placements of our equity securities, upfront, milestone and expense reimbursement payments received from our collaborators, from our initial public offering, or IPO, in April 2020, from our public offerings of common stock in November 2020 and January 2024, and from our "at-the-market offering," in connection with our Sales Agreement with Leerink Partners LLC, or Leerink, as agent, pursuant to which we may offer and sell, from time to time, shares of our common stock through Leerink, or the ATM Offering. We expect our expenses to increase in connection with our ongoing activities, particularly as we progress our Phase 2 clinical trial of rinvatercept in patients with DMD and commence our Phase 2 clinical trial of rinvatercept in patients with ALS, and continue to research, develop and initiate clinical trials of any other future product candidates. In addition, if we obtain regulatory approval for any of our product candidates, we expect to incur significant commercialization expenses related to product manufacturing, marketing, sales and distribution. Furthermore, we expect to incur additional costs associated with operating as a public company. Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations. If we are unable to raise capital when needed or on attractive terms, we could be forced to delay, reduce or eliminate our product development programs or any future commercialization efforts.

As of September 30, 2026, we had $259.5 million in cash and cash equivalents. Based on our current operating assumptions, we expect that our existing cash and cash equivalents as of September 30, 2026 will enable us to fund our operating expenses and capital expenditure requirements into the second half of 2028. We have based this estimate on assumptions that may prove to be wrong, and we could use our capital resources sooner than we currently expect. Our future capital requirements for rinvatercept, elritercept or our other nonclinical programs will depend on many factors, including:

- the progress, timing and completion of nonclinical studies and clinical trials for our current or any future product candidates, as well as the associated costs, including any unforeseen costs we may incur as a result of nonclinical study or clinical trial delays due to public health crises or other causes;
- the timing and amount of milestone and royalty payments we are required to make or are eligible to receive under our license agreements with each of The General Hospital Corporation, Takeda and Hansoh;
- the number of potential new product candidates we identify and decide to develop;
- the need for additional or expanded nonclinical studies and clinical trials beyond those that we plan to conduct with respect to our current and future product candidates;
- the costs involved in growing our organization to the size needed to allow for the research, development and potential commercialization of our current or any future product candidates;
- the costs involved in filing patent applications, maintaining and enforcing patents or defending against infringement or other claims raised by third parties;
- the maintenance of our existing license and collaboration agreements and the entry into new license and collaboration agreements;
- the time and costs involved in obtaining regulatory approval for our product candidates and any delays we may encounter as a result of evolving regulatory requirements or adverse results with respect to any of our product candidates;
- the effect of competing technological and market developments;
- the cost of manufacturing rinvatercept and future product candidates for clinical trials in preparation for marketing approval applications and in preparation for commercialization;
- the cost of establishing sales, marketing and distribution capabilities for any product candidates for which we may receive regulatory approval in regions where we choose to commercialize our products, if approved, on our own;

- the amount of revenues, if any, we may derive either directly or in the form of royalty payments from future sales of our product candidates, if approved; and
- market acceptance of any approved product candidates.

We do not have any committed external source of funds or other support for our development efforts and we cannot be certain that additional funding will be available on acceptable terms, or at all. Further, in the event that the license agreement we entered into with Takeda, or the Takeda Agreement, is terminated, we may not receive any additional fees or milestone payments under that agreement. Absent the funding support obtained under the Takeda Agreement, our further development of elritercept would require significant additional capital from us, or the establishment of alternative collaborations with third parties, which may not be possible. Until we can generate sufficient product or royalty revenue to finance our cash requirements, which we may never do, we expect to finance our future cash needs through a combination of public or private equity offerings, debt financings, collaborations, strategic alliances, licensing arrangements and other marketing or distribution arrangements.

Our ability to raise additional funds will depend on financial, economic and market conditions and other factors, over which we may have no or limited control. Disruptions in the financial markets in general, geopolitical conflicts and economic instability may make equity and debt financing more difficult to obtain, and may have a material adverse effect on our ability to meet our fundraising needs. We cannot guarantee that future financing will be available in sufficient amounts or on terms acceptable to us, if at all. If we are unable to obtain additional funding, we could be forced to delay, reduce or eliminate some or all of our research and development programs and clinical development efforts, which would adversely affect our business prospects, or we may be unable to continue operations.

Raising additional capital may cause dilution to holders of our common stock, restrict our operations or require us to relinquish rights to our technologies or product candidates.

Until such time, if ever, as we can generate substantial product revenues, we expect to finance our operations with our existing cash and cash equivalents and revenue from our collaborations. In order to further advance development of our product candidates, discover additional product candidates and pursue our other business objectives, we will need to seek additional funds.

We cannot guarantee that future financing will be available in sufficient amounts or on commercially reasonable terms, if at all. Moreover, the terms of any financing may adversely affect the holdings or the rights of holders of our common stock and the issuance of additional securities, whether equity or debt, by us, or the possibility of such issuance, may cause the market price of our common stock to decline. The sale of additional common stock or securities convertible or exchangeable into common stock would dilute all of our existing stockholders and the terms of these securities may include liquidation or other preferences that adversely affect your rights as a holder of our common stock. The incurrence of indebtedness could result in increased fixed payment obligations and we may be required to agree to certain restrictive covenants, such as limitations on our ability to incur additional debt or declare dividends, limitations on our ability to acquire, sell or license intellectual property rights and other operating restrictions that could adversely impact our ability to conduct our business. If we raise additional capital through marketing and distribution arrangements or other collaborations, strategic alliances or licensing arrangements with third parties, we may have to relinquish certain valuable rights to our product candidates, technologies, future revenue streams or research programs or grant licenses on terms that may not be favorable to us. We also could be required to seek collaborators for rinvatercept or any future product candidate at an earlier stage than otherwise would be desirable or relinquish our rights to product candidates or technologies that we otherwise would seek to develop or commercialize ourselves. Further, any additional fundraising efforts may divert our management from its day-to-day activities, which may adversely affect our ability to develop and commercialize our product candidates.

If we are unable to raise additional capital in sufficient amounts or on terms acceptable to us, we may have to significantly delay, scale back or discontinue the development or commercialization of one or more of our product candidates or one or more of our other research and development initiatives. Any of the above events could significantly harm our business, prospects, financial condition and results of operations and cause the price of our common stock to decline.

Risks Related to the Discovery, Development and Regulatory Approval of our Product Candidates

We are heavily dependent on the success of our product candidates, which are in clinical development. If we are unable to advance our current or future product candidates through clinical trials, obtain marketing approval and

ultimately commercialize any product candidates we develop, or experience significant delays in doing so, our business will be materially harmed.

Before obtaining marketing approval from regulatory authorities for the sale of our product candidates, we must conduct extensive clinical trials to demonstrate the safety and efficacy of the product candidates in humans. We are early in our product candidate development efforts, as rinvatercept and elritercept are still in clinical trials. If either of rinvatercept or elritercept encounters safety or efficacy problems, development delays or regulatory issues or other problems, our development plans and business would be significantly harmed.

Our ability to generate product revenues, which we do not expect will occur for several years, if ever, will depend heavily on the successful development and eventual commercialization of rinvatercept, elritercept and any future product candidates we develop, which may never occur. Rinvatercept, elritercept and any future product candidates we develop will require additional nonclinical and clinical development, management of clinical, nonclinical and manufacturing activities, marketing approval in the United States and other jurisdictions for specific indications for use, demonstrating effectiveness to pricing and reimbursement authorities, obtaining sufficient manufacturing supply for both clinical development and commercial production, building of a commercial organization and substantial investment and significant marketing efforts before we generate any revenues from product sales. The success of our current and future product candidates will depend on several factors, including the following:

- successful and timely completion of clinical trials and nonclinical studies for which the U.S. Food and Drug Administration, or the FDA, or any comparable foreign regulatory authority agree with the design, endpoints or implementation;
- sufficiency of our financial and other resources to complete the necessary nonclinical studies and clinical trials;
- receiving regulatory approvals or authorizations for conducting our planned clinical trials or future clinical trials;
- commencement of and successful patient enrollment in, and completion of, additional clinical trials on a timely basis;
- our ability to demonstrate to the satisfaction of the FDA or any comparable foreign regulatory authority that the applicable product candidate is safe and effective as a treatment for our targeted indications or, in the case of an applicable product candidate which is regulated as a biological product, that the applicable product candidate is safe, pure, and potent for our targeted indications;
- our ability to demonstrate to the satisfaction of the FDA or any comparable foreign regulatory authority that the applicable product candidate's risk-benefit ratio for its proposed indication is acceptable;
- timely receipt of marketing approvals for our product candidates from applicable regulatory authorities;
- the extent of any required post-marketing approval commitments to applicable regulatory authorities;
- establishing and scaling up, either alone or with third-party manufacturers, manufacturing capabilities of clinical supply for our clinical trials and commercial manufacturing, if any of our product candidates are approved;
- obtaining and maintaining patent and trade secret protection or regulatory exclusivity for our product candidates, both in the United States and internationally;
- successfully scaling a sales and marketing organization and launching commercial sales of our product candidates, if approved;
- acceptance of our product candidates' benefits and uses, if approved, by patients, the medical community and third-party payors;
- maintaining a continued acceptable safety profile of our product candidates following approval;
- effectively competing with companies developing and commercializing other therapies in the indications which our product candidates target;
- obtaining and maintaining healthcare coverage and adequate reimbursement from third-party payors; and
- enforcing and defending intellectual property rights and claims.

If we or our collaborators are not successful with respect to one or more of these factors in a timely manner or at all, we or our collaborators could experience significant delays or an inability to successfully commercialize rinvatercept, elritercept or any future product candidates we develop, which would materially harm our business. If we do not receive marketing approvals for our current and future product candidates, we may not be able to continue our operations.

All of our product candidates are in nonclinical or clinical development stages. Clinical trials are difficult to design and implement, and they involve a lengthy and expensive process with uncertain outcomes. We may experience delays in completing, or ultimately be unable to complete, the development and commercialization of rinvatercept, elritercept or any future product candidates.

Clinical testing is expensive and can take many years to complete, and its outcome is inherently uncertain. Failure can occur at any time during the clinical trial process and our future clinical trial results may not be successful. We cannot guarantee that any of our ongoing and planned clinical trials will be conducted as planned or completed on schedule, if at all. Moreover, even if these trials are initiated or conducted on a timely basis, issues may arise that could result in the suspension or termination of such clinical trials. For example, in January 2025, we announced the early termination of our Phase 2 clinical trial evaluating cibotercept in patients with pulmonary arterial hypertension, or PAH, which we refer to as the TROPOS trial, based on an ongoing safety review due to the unanticipated observation of pericardial effusion adverse events in the trial.

To date, we have not completed any pivotal clinical trials required for the approval of any of our product candidates. Although we have completed our Phase 1 clinical trial of rinvatercept and our Phase 1 clinical trial of elritercept, both in healthy volunteers, we may experience delays in our ongoing clinical trials or nonclinical studies and we do not know whether planned clinical trials will begin on time, need to be redesigned, enroll patients on time, have sufficient drug supply for our product candidates on a timely basis or be completed on schedule, if at all. A failure of one or more clinical trials can occur at any stage of testing, and our ongoing and future clinical trials may not be successful. We or our collaborators also may experience numerous unforeseen events during our clinical trials that could delay or prevent our ability to receive marketing approval or commercialize rinvatercept, elritercept or any future product candidates, including:

- delays in or failure to obtain regulatory authorizations to commence a trial;
- delays in reaching a consensus with regulatory agencies as to the design or implementation of our clinical trials;
- delays in or failure to reach agreement on acceptable terms with prospective contract research organizations, or CROs, and clinical trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and trial sites;
- delays in or failure to obtain institutional review board, or IRB, or positive ethics committee opinions at each site;
- delays in or failure to recruit a sufficient number of suitable patients to participate in a trial;
- failure to have patients complete a trial or return for post-treatment follow-up, including disruptions in our ability to treat patients or conduct post-treatment follow-up due to public health crises;
- clinical sites deviating from trial protocol, missing data or dropping out of a trial;
- delays in adding new clinical trial sites;
- failure to manufacture sufficient quantities of our product candidates for use in clinical trials in a timely manner;
- occurrence of adverse events associated with the product candidate that are viewed to outweigh its potential benefits, or safety or tolerability concerns that could cause us or our collaborators, as applicable, to suspend or terminate a trial if we or our collaborators find that the participants are being exposed to unacceptable health risks;
- failure to perform clinical trials in accordance with the FDA's or any comparable foreign regulatory authority's good clinical practices, or GCP, requirements, or regulatory guidelines in other countries;
- changes in regulatory requirements, policies and guidelines;
- failure of our third-party research contractors to comply with regulatory requirements or meet their contractual obligations to us in a timely manner, or at all;
- delays in establishing the appropriate dosage levels and frequency of dosing in clinical trials;
- the quality or stability of our product candidates falling below acceptable standards; and
- business interruptions resulting from geopolitical actions, including war, such as the current Russia-Ukraine war and the wars in the Middle East, and terrorism or the perception that such hostilities may be imminent, another outbreak of a contagious disease, or natural disasters including earthquakes, typhoons, floods and fires.

In addition, public health crises may increase the likelihood that we encounter additional difficulties and delays in the future. We could also encounter delays if a clinical trial is suspended or terminated by us, the IRBs of the institutions in which such trials are being conducted, or the FDA or comparable foreign regulatory authorities, or recommended for suspension or termination by the Safety Review Committee for such trial. A suspension or termination may be imposed due to a number of factors, including failure to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols, inspection of the clinical trial operations or trial site by the FDA or comparable foreign regulatory authorities resulting in the imposition of a clinical hold, unforeseen safety issues or adverse side effects, failure to demonstrate a benefit from using a product or treatment, failure to establish or achieve clinically meaningful trial endpoints, changes in governmental regulations or administrative actions or lack of adequate funding to continue the clinical trial. Many of the factors that cause, or lead to, a delay in the commencement or completion of clinical trials may also ultimately lead to the denial of regulatory approval of our product candidates. Further, the FDA or comparable foreign regulatory authorities may disagree with our clinical trial design and our interpretation of data from clinical trials, or may change the requirements for approval even after they have reviewed and commented on the design for our clinical trials.

Our product development costs will increase if we experience delays in clinical testing or marketing approvals. We do not know whether any of our clinical trials will begin as planned, will need to be restructured or will be completed on schedule, or at all. Significant clinical trial delays also could shorten any periods during which we may have the exclusive right to commercialize our product candidates and may allow our competitors to bring products to market before we do, potentially impairing our ability to successfully commercialize our product candidates and harming our business and results of operations. Any delays in our clinical development programs may harm our business, financial condition and results of operations significantly.

Our clinical trials may fail to demonstrate substantial evidence of the safety and efficacy or safety, purity and potency of our product candidates or any future product candidates, which would prevent or delay or limit the scope of regulatory approval and commercialization.

To obtain the requisite regulatory approvals to market and sell any of our product candidates, including rinvatercept, elritercept and any other future product candidates, we must demonstrate through extensive nonclinical studies and clinical trials that our investigational drug products are safe and effective for use in each targeted indication, and in the case of our product candidates regulated as biological products, such as rinvatercept and elritercept, that the product candidate is safe, pure and potent for use in its targeted indication. Clinical testing is expensive and can take many years to complete, and its outcome is inherently uncertain. Failure can occur at any time during the clinical development process. Most product candidates that begin clinical trials are never approved by regulatory authorities for commercialization. We may be unable to establish clinical endpoints that applicable regulatory authorities would consider clinically meaningful, and a clinical trial can fail at any stage of testing. Further, the process of obtaining regulatory approval is expensive, often takes many years following the commencement of clinical trials and can vary substantially based upon the type, complexity and novelty of the product candidates involved, as well as the target indications, patient population and regulatory agency. Prior to obtaining approval to commercialize rinvatercept, elritercept and any future product candidates in the United States or abroad, we, our collaborators or our potential future collaborators must demonstrate with substantial evidence from adequate and well-controlled clinical trials, and to the satisfaction of the FDA or comparable foreign regulatory authorities, that such product candidates are safe and effective for their intended uses.

Clinical trials that we conduct may not demonstrate the efficacy and safety necessary to obtain regulatory approval to market our product candidates. In some instances, there can be significant variability in safety or efficacy results between different clinical trials of the same product candidate due to numerous factors, including changes in trial procedures set forth in protocols, differences in the size and type of the patient populations, changes in and adherence to the clinical trial protocols and the rate of dropout among clinical trial participants. If the results of our ongoing or future clinical trials are inconclusive with respect to the efficacy of our product candidates, if we do not meet the clinical endpoints with statistical and clinically meaningful significance, or if there are safety concerns associated with our product candidates, we may be delayed in obtaining marketing approval, if at all. Additionally, any safety concerns observed in any one of our clinical trials in our targeted indications could limit the prospects for regulatory approval of our product candidates in those and other indications. For example, in January 2025, we announced the early termination of our TROPOS trial evaluating cibotercept in patients with PAH, based on an ongoing safety review due to the unanticipated observation of pericardial effusion adverse events in the trial. Consequently, we determined to deprioritize cibotercept, and pause all material, internal development activities associated with this asset.

Even if the trials are successfully completed, clinical data are often susceptible to varying interpretations and analyses, and we cannot guarantee that the FDA or comparable foreign regulatory authorities will interpret the results as we do, and more trials could be required before we submit our product candidates for approval. We cannot guarantee that the FDA or comparable foreign regulatory authorities will view our product candidates as having efficacy even if positive results are observed in clinical trials. Moreover, results acceptable to support approval in one jurisdiction may be deemed inadequate by another regulatory authority to support regulatory approval in that other jurisdiction. To the extent that the results of the trials are not satisfactory to the FDA or comparable foreign regulatory authorities for support of a marketing application, approval of rinvatercept, elritercept and any future product candidates may be significantly delayed, or we may be required to expend significant additional resources, which may not be available to us or our collaborators, to conduct additional trials in support of potential approval of our product candidates. Even if regulatory approval is secured for a product candidate, the terms of such approval may limit the scope and use of the specific product candidate, which may also limit its commercial potential.

The results of nonclinical studies and early-stage clinical trials of our product candidates may not be predictive of the results of later-stage clinical trials. Initial success in our ongoing clinical trials may not be indicative of results obtained when these trials are completed or in later-stage trials.

The results of nonclinical studies and clinical trials may not be predictive of the results of later-stage clinical trials. Product candidates in later stages of clinical trials may fail to show the desired safety and efficacy traits despite having progressed through nonclinical studies and initial clinical trials. For example, in a Phase 1 clinical trial evaluating cibotercept in healthy volunteers, cibotercept was generally well tolerated at doses up to 4.5 mg/kg, and there were no reported pericardial effusion adverse events. However, in January 2025, we announced the early termination of our TROPOS trial evaluating cibotercept in patients with PAH, based on an ongoing safety review due to the unanticipated observation of pericardial effusion adverse events in the trial. Furthermore, there can be no assurance that any of our clinical trials will ultimately be successful or support further clinical development of any of our product candidates. There is a high failure rate for product candidates proceeding through clinical trials. Many companies in the biotechnology and pharmaceutical industries have suffered significant setbacks in late-stage clinical trials after achieving positive results in early-stage development and we cannot be certain that we will not face similar setbacks. These setbacks have been caused by, among other things, nonclinical findings made while clinical trials were underway, or safety or efficacy observations made in nonclinical studies and clinical trials, including previously unreported adverse events. Moreover, nonclinical and clinical data are often susceptible to varying interpretations and analyses and many companies that believed their product candidates performed satisfactorily in nonclinical studies and clinical trials nonetheless failed to obtain FDA or comparable foreign regulatory authority approval. Any such setbacks in our clinical development could have a material adverse effect on our business, financial condition and results of operations.

Additionally, some of the clinical trials we conduct may include open-label trials conducted at a limited number of clinical sites on a limited number of patients. For example, our ongoing Phase 2 clinical trial of rinvatercept in patients with DMD, is an open-label trial. An “open-label” clinical trial is one where both the patient and investigator know whether the patient is receiving the investigational product candidate or either an existing approved product or placebo. Most typically, open-label clinical trials test only the investigational product candidate and sometimes may do so at different dose levels. For example, in our ongoing Phase 2 clinical trial for rinvatercept in patients with DMD, rinvatercept is being administered at a starting dose of 0.5 mg/kg, and, based on individual titration rules, the dose may be escalated in 0.5 mg/kg increments after two doses, up to a maximum dose of 2.0 mg/kg.

Open-label clinical trials are also subject to various limitations that may exaggerate any therapeutic effect as patients in open-label clinical trials are aware when they are receiving treatment. Open-label clinical trials may be subject to a “patient bias” where patients perceive their symptoms to have improved merely due to their awareness of receiving an experimental treatment. In addition, open-label clinical trials may be subject to an “investigator bias” where those assessing and reviewing the physiological outcomes of the clinical trials are aware of which patients have received treatment and may interpret the information of the treated group more favorably given this knowledge. Given that one open-label Phase 2 clinical trial is ongoing for rinvatercept in patients with DMD and two open-label Phase 2 clinical trials are ongoing for elritercept, one in patients with lower-risk MDS and one in patients with myelofibrosis, the results from these clinical trials may not be predictive of future clinical trial results with these or other product candidates for which we include an open-label clinical trial, when studied in a controlled environment with a placebo or active control.

Our product candidates may be associated with serious adverse, undesirable or unacceptable side effects or other properties or safety risks, which may delay or halt their clinical development, or prevent marketing approval. If such side effects are identified during the development of our product candidates or following approval we may suspend or abandon our development of such product candidates, the commercial profile of any approved label may be limited, or we may be subject to other significant negative consequences following marketing approval.

Undesirable side effects that may be caused by our product candidates could cause us or regulatory authorities to interrupt, delay or halt clinical trials and could result in a more restrictive label or the delay or denial of regulatory approval by the FDA or comparable foreign regulatory authorities. While rinvatercept and elritercept have generally been well tolerated in our nonclinical studies and clinical trials to date, the results from future nonclinical studies and clinical trials, including of cibotercept and our other product candidates, may identify additional safety concerns or other undesirable properties of our product candidates.

The results of our ongoing Phase 2 clinical trial of rinvatercept, the Phase 2 and Phase 3 clinical trials of elritercept and future clinical trials of these and other product candidates may show that our product candidates cause undesirable or unacceptable side effects or even death. In such an event, our trials could be suspended or terminated and the FDA or comparable foreign regulatory authorities could order us to cease further development of or deny approval of our product candidates for any or all targeted indications. The drug-related side effects could affect patient recruitment or the ability of enrolled patients to complete

the trial or result in potential product liability claims. Any of these occurrences may harm our business, financial condition and results of operations significantly.

Moreover, if our product candidates are associated with undesirable side effects in nonclinical studies or clinical trials or have characteristics that are unexpected, we may elect to abandon their development or limit their development to more narrow uses or subpopulations in which the undesirable side effects or other characteristics are less prevalent, less severe or more acceptable from a risk-benefit perspective, which may limit the commercial expectations for the product candidate, if approved. Additionally, adverse developments in clinical trials of pharmaceutical and biopharmaceutical products conducted by others may cause the FDA or other foreign regulatory oversight bodies to suspend or terminate our clinical trials or to change the requirements for approval of any of our product candidates.

In addition, if any of our product candidates receives marketing approval and we or others later identify undesirable or unacceptable side effects caused by such products, a number of potentially significant negative consequences could result, including:

- regulatory authorities may withdraw approvals of such product and require us to take our approved product off the market;
- regulatory authorities may require the addition of labeling statements, specific warnings, a contraindication or field alerts to physicians and pharmacies;
- regulatory authorities may require a medication guide outlining the risks of such side effects for distribution to patients, or that we implement a risk evaluation and mitigation strategy, or REMS, plan to ensure that the benefits of the product outweigh its risks;
- we may be required to conduct additional clinical trials, which may lead to additional interactions with regulatory authorities;
- we may be required to change the way the product is administered, conduct additional clinical trials or change the labeling of the product;
- we may be subject to limitations on how we may promote the product;
- sales of the product may decrease significantly;
- we may be subject to litigation or product liability claims; and
- our reputation may suffer.

Any of these events could prevent us, our collaborators or our potential future partners from achieving or maintaining market acceptance of the affected product or could substantially increase commercialization costs and expenses, which in turn could delay or prevent us from generating significant revenue from the sale of our product candidates, if approved.

We may find it difficult to enroll patients in our clinical trials, which could delay or prevent us from proceeding with, or otherwise adversely affect, clinical trials of our product candidates.

Identifying and qualifying patients to participate in clinical trials of our product candidates is critical to our success. The timely completion of our clinical trials in accordance with their protocols depends, among other things, on our ability to recruit a sufficient number of eligible patients to participate and remain in the trial until its conclusion. Patients may be unwilling to participate in our clinical trials because of negative publicity from adverse events related to novel therapeutic approaches, competitive clinical trials for similar patient populations, the existence of current treatments or for other reasons, including public health crises. Any delays related to patient enrollment or difficulties related to patient retention could result in increased costs, delays in advancing our product candidates, delays in testing the effectiveness of our product candidates or termination of the clinical trials altogether. We may not be able to identify, recruit and enroll a sufficient number of patients, or those with the required or desired characteristics, to complete our clinical trials in a timely manner. Patient enrollment and trial completion is affected by many factors, including the:

- size and nature of the patient population and process for identifying patients;
- proximity and availability of clinical trial sites for prospective patients;
- ability of patients to travel to clinical trial sites;
- eligibility and exclusion criteria for the trial;
- design of the clinical trial;
- safety profile, to date, of the product candidate under study;
- perceived risks and benefits of the product candidate under study;
- perceived risks and benefits of our approach;

- approval of competing product candidates currently under investigation for the treatment of similar diseases or conditions, or competing clinical trials for similar product candidates or targeting patient populations meeting our patient eligibility criteria;
- severity of the disease under investigation;
- degree of progression of the patient's disease at the time of enrollment and throughout the clinical trial;
- ability to obtain and maintain patient consent;
- risk that enrolled patients will drop out before completion of the trial;
- patient referral practices of physicians; and
- ability to adequately monitor patients during and after treatment.

Enrollment risks are heightened with respect to indications that are rare or orphan diseases, which may limit the pool of patients that may be enrolled in our planned clinical trials. In addition, our clinical trials will compete with other clinical trials for product candidates that are in the same therapeutic areas as our product candidates, and this competition will reduce the number and types of patients available to us, because some patients who might have opted to enroll in our trials may instead opt to enroll in a trial being conducted by one of our competitors. Since the number of qualified clinical investigators is limited, we expect to conduct some of our clinical trials at the same clinical trial sites that some of our competitors use, which will reduce the number of patients who are available for our clinical trials in such clinical trial site.

Delays related to patient enrollment and difficulties related to patient retention may result in increased costs or may affect the timing or outcome of our future clinical trials, which could prevent completion of these trials and adversely affect our ability to advance the development of our product candidates.

Interim, topline and preliminary data from our clinical trials that we announce or publish from time to time may change as more patient data become available and are subject to audit and verification procedures that could result in material changes in the final data.

From time to time, we may publish interim, topline or preliminary data from our clinical trials. Preliminary and interim data from our clinical trials may change as more participant data become available. For example, in December 2024, we announced preliminary efficacy results from Parts 1 and 2 of the Phase 2 clinical trial evaluating elritercept for the treatment of anemia and thrombocytopenia in patients with lower-risk MDS. Preliminary or interim data from our clinical trials are not necessarily predictive of final results. Preliminary and interim data are subject to the risk that one or more of the clinical outcomes may materially change as enrollment continues, more trial data become available and we issue our final clinical trial report. Interim, topline and preliminary data also remain subject to audit and verification procedures that may result in the final data being materially different from the preliminary data we previously published. As a result, preliminary, topline and interim data should be viewed with caution until the final data are available. Material adverse changes in the final data compared to the interim data could significantly harm our business prospects.

Further, others, including regulatory agencies, may not accept or agree with our assumptions, estimates, calculations, conclusions or analyses or may interpret or weigh the importance of data differently, which could impact the value of the particular program, the approvability or commercialization of the particular product candidate or product, if any, and our company in general. In addition, the information we choose to publicly disclose regarding a particular nonclinical study or clinical trial is based on what is typically extensive information, and you or others may not agree with what we determine is the material or otherwise appropriate information to include in our disclosure, and any information we determine not to disclose may ultimately be deemed significant with respect to future decisions, conclusions, views, activities or otherwise regarding a particular product, if any, product candidate or our business. If the preliminary and interim data that we report differ from actual results, or if others, including regulatory authorities, disagree with the conclusions reached, our ability to obtain approval for, and commercialize, our product candidates may be harmed, which could harm our business, operating results, prospects or financial condition.

Nonclinical development is uncertain. Our nonclinical programs may experience delays or may never advance to clinical trials, which would adversely affect our ability to obtain regulatory approvals or commercialize these programs on a timely basis or at all, which would have an adverse effect on our business.

Before we can commence clinical trials for any product candidate, we must complete extensive nonclinical studies that support any future Investigational New Drug, or IND, applications in the United States, or similar applications in other jurisdictions. All of our completed clinical trials have, to date, been conducted outside of the United States. However, we submitted and cleared an IND with the FDA for the Phase 2 clinical trial for elritercept in patients with MDS in October 2022

and submitted and cleared an IND with the FDA for the Phase 2 clinical trial for elritercept in patients with myelofibrosis in February 2025. Conducting nonclinical testing is a lengthy, time-consuming and expensive process and delays associated with product candidates for which we are directly conducting nonclinical testing and studies may cause us to incur additional operating expenses. While we are initially conducting our Phase 2 clinical trial of rinvatercept in patients with DMD outside of the United States, we cannot be certain of the timely completion or outcome of our nonclinical testing and studies for our other product candidates and cannot predict if the FDA will accept our proposed clinical programs or if the outcome of our nonclinical testing and foreign clinical trials will ultimately support the further development of our other product candidates. As a result, we cannot be sure that we will be able to submit INDs or similar applications for our nonclinical programs on the timelines we expect, if at all, and we cannot be sure that submission of INDs or similar applications will result in the FDA or comparable foreign regulatory authorities allowing clinical trials to begin.

Our research and development activities could be affected or delayed as a result of shortages in animal availability or possible restrictions on animal testing.

Certain laws and regulations require us to test our product candidates on animals before initiating clinical trials involving humans. Failure to access or a significant delay in accessing animal research models that meet our needs or that fulfill regulatory requirements may materially adversely affect our ability to advance our nonclinical and clinical programs and successfully develop our product candidates, and this could result in significant harm to our business.

Additionally, animal testing activities have been the subject of controversy and adverse publicity. Animal rights groups and other organizations and individuals have attempted to stop animal testing activities by pressing for legislation and regulation in these areas and by disrupting these activities through protests and other means. To the extent the activities of these groups are successful, our research and development activities may be interrupted, delayed or become more expensive.

The regulatory approval processes of the FDA and comparable foreign regulatory authorities are lengthy, time-consuming and inherently unpredictable, and if we are ultimately unable to obtain regulatory approval for our product candidates, our business will be substantially harmed.

The time required to obtain approval by the FDA and comparable foreign regulatory authorities is unpredictable but typically takes many years following the commencement of clinical trials and depends upon numerous factors, including the substantial discretion of the regulatory authorities. In addition, approval policies, laws or regulations, or the type and amount of clinical data necessary to gain approval may change during the course of a product candidate's clinical development and may vary among jurisdictions. We have not obtained regulatory approval for any product candidate, and it is possible that none of our existing product candidates or any product candidates we may seek to develop in the future will ever obtain regulatory approval.

Our product candidates could fail to receive regulatory approval for many reasons, including the following:

- the FDA or comparable foreign regulatory authorities may disagree with the design or implementation of our clinical trials;
- we may be unable to demonstrate to the satisfaction of the FDA or comparable foreign regulatory authorities that a product candidate is safe and effective as a treatment for our targeted indications, or, in the case of a product candidate regulated as a biological product, that the product candidate is safe, pure and potent for its proposed indication;
- the population studied may not be sufficiently broad or representative to assure safety or efficacy in the population for which we seek approval;
- the results of clinical trials may not meet the level of statistical significance required by the FDA or comparable foreign regulatory authorities for approval;
- we may be unable to demonstrate that a product candidate's clinical and other benefits outweigh its safety risks;
- the FDA or comparable foreign regulatory authorities may disagree with our interpretation of data from nonclinical studies or clinical trials;
- the FDA or comparable foreign regulatory authorities may require additional nonclinical studies or clinical trials beyond those that we currently anticipate;
- the data collected from clinical trials of our product candidates may not be sufficient to support the submission of a New Drug Application, or NDA, or a Biologics License Application, or BLA, as applicable, to the FDA or other submission or to obtain regulatory approval in the United States or elsewhere;

- the FDA or comparable foreign regulatory authorities may find deficiencies with or fail to approve the manufacturing processes or facilities of third-party manufacturers with which we contract for clinical and commercial supplies; and
- the approval policies or regulations of the FDA or any comparable foreign regulatory authorities or the laws they enforce may significantly change in a manner rendering our clinical data insufficient for approval.

This lengthy approval process as well as the unpredictability of future clinical trial results may result in our failing to obtain regulatory approval to market any of our product candidates, which would significantly harm our business, financial condition and results of operations. The FDA and comparable foreign regulatory authorities have substantial discretion in the approval process, and determining when or whether regulatory approval will be obtained for any of our product candidates. Even if we believe the data collected from clinical trials of our product candidates are promising, such data may not be sufficient to support approval by the FDA or comparable foreign regulatory authorities. If global health concerns such as a pandemic prevent the FDA or other regulatory authorities from conducting their regular inspections, reviews or other regulatory activities, it could significantly impact the ability of the FDA or comparable foreign regulatory authorities to timely review and process our regulatory submissions, which could have a material adverse effect on our business.

In addition, even if we were to obtain approval, regulatory authorities may approve any of our product candidates for fewer or more limited indications than we request, may not approve the price we intend to charge for our products, if any, may grant approval contingent on the performance of costly post-marketing clinical trials, or may approve a product candidate with a label that does not include the labeling claims necessary or desirable for the successful commercialization of that product candidate. Any of the foregoing scenarios could materially harm the commercial prospects for our product candidates.

The FDA and any comparable foreign regulatory authorities may not accept data from trials conducted in locations outside of their jurisdiction.

The acceptance of trial data by the FDA or any comparable foreign regulatory authority from clinical trials conducted outside of their respective jurisdictions may be subject to certain conditions or may not be accepted at all. In cases where data from foreign clinical trials are intended to serve as the basis for marketing approval in the United States, the FDA will generally not approve the application on the basis of foreign data alone unless (i) the data are applicable to the United States population and United States medical practice, (ii) the trials are performed by clinical investigators of recognized competence and pursuant to compliance with current GCP requirements and (iii) the FDA is able to validate the data through an on-site inspection or other appropriate means. Similar requirements and limitations regarding the acceptability of foreign clinical trial data may apply in other countries. Additionally, the FDA's clinical trial requirements, including the adequacy of the patient population studied and statistical powering, must be met. In addition, such foreign trials are subject to the applicable local laws of the foreign jurisdictions where the trials are conducted. There can be no assurance that the FDA or any applicable foreign regulatory authority will accept data from trials conducted outside of its applicable jurisdiction. If the FDA or any applicable foreign regulatory authority does not accept such data, it would result in the need for additional trials, which would be costly and time-consuming and delay aspects of our business plan, and which may result in our product candidates not receiving approval for commercialization in the applicable jurisdiction.

Even if we receive regulatory approval of a product candidate, we will be subject to ongoing regulatory obligations and continued regulatory review, which may result in significant additional expense and we may be subject to penalties if we fail to comply with regulatory requirements or experience unanticipated problems with such product candidate.

If any of our product candidates are approved, they will be subject to ongoing regulatory requirements for manufacturing, labeling, packaging, storage, advertising, promotion, sampling, record-keeping, conduct of post-marketing studies and submission of safety, efficacy and other post-market information, including both federal and state requirements in the United States and requirements of comparable foreign regulatory authorities. In addition, we will be subject to continued compliance with current Good Manufacturing Practices, or cGMPs, and GCP requirements for any clinical trials that we conduct post-approval.

Manufacturers and their facilities are required to comply with extensive FDA and comparable foreign regulatory authority requirements, including ensuring that quality control and manufacturing procedures conform to cGMP regulations. As such, we and our contract manufacturers will be subject to continual review and inspections to assess compliance with cGMP and adherence to commitments made in any NDA or BLA, other foreign marketing authorization application, and previous responses to inspection observations. Accordingly, we and others with whom we work must continue to expend time, money, and effort in all areas of regulatory compliance, including manufacturing, production and quality control.

Any regulatory approvals that we receive for our product candidates may be subject to 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 safety and efficacy of the product candidate. The FDA may also require a REMS program as a condition of approval of our product candidates, which could entail requirements for long-term patient follow-up, a medication guide, physician communication plans or additional elements to ensure safe use, such as restricted distribution methods, patient registries and other risk minimization tools. Comparable foreign regulatory authorities may impose similar requirements to the FDA REMS program. As an example, the European Commission may require the implementation of a risk mitigation plan in order to collect additional information on a medicine's safety profile which may include plans for pharmacovigilance activities and measures to minimize risks. In addition, if the FDA or a comparable foreign regulatory authority approves our product candidates, we will have to comply with requirements including submissions of safety and other post-marketing information and reports and registration.

The FDA or comparable foreign regulatory authorities may impose consent decrees, or similar requirements, or withdraw approval if compliance with regulatory requirements and standards is not maintained or if problems occur after the product reaches the market. Later discovery of previously unknown problems with our product candidates, including adverse events of unanticipated severity or frequency, or with our third-party manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may result in revisions to the approved labeling to add new safety information; imposition of post-market studies or clinical trials to assess new safety risks; or imposition of distribution restrictions or other restrictions under a REMS program, or comparable foreign program. Other potential consequences include, among other things:

- restrictions or suspensions on operations including on the marketing or manufacturing of our products, if approved, withdrawal of the product from the market or voluntary or mandatory product recalls;
- fines, warning letters or holds on clinical trials;
- refusal by the FDA or comparable foreign regulatory authorities to approve pending applications or supplements to approved applications filed by us or suspension or revocation of license approvals;
- product seizure or detention or refusal to permit the import or export of our product candidates; and
- injunctions or the imposition of civil or criminal penalties.

The FDA and comparable foreign regulatory authorities strictly regulate marketing, labeling, advertising, and promotion of products that are placed on the market. Products may be promoted only for the approved indications and in accordance with the provisions of the approved label. The FDA and other agencies and comparable foreign regulatory authorities actively enforce the laws and regulations prohibiting the promotion of off-label uses and a company that is found to have improperly promoted off-label uses may be subject to significant liability including, among other things, adverse publicity, warning letters, corrective advertising and potential civil and criminal penalties. Physicians may prescribe, in their independent professional medical judgment, legally available products for uses that are not described in the product's labeling and that differ from those tested by us and approved by the FDA or comparable foreign regulatory authorities. Physicians may believe that such off-label uses are the best treatment for many patients in varied circumstances. The FDA and comparable foreign regulatory authorities do not govern the behavior of physicians in their choice of treatments. The FDA and comparable foreign regulatory authorities do, however, restrict manufacturer's communications on the subject of off-label use of their products. As an example, the United States federal government has levied large civil and criminal fines against companies for alleged improper promotion of off-label use and has enjoined companies from engaging in off-label promotion. The FDA and other foreign regulatory agencies have also required that companies enter into consent decrees or permanent injunctions under which specified promotional conduct is changed or curtailed. However, companies may share truthful and not misleading information that is otherwise consistent with a product's approved labeling.

The holder of an NDA or BLA and equivalent foreign applications must submit new or supplemental applications and obtain approval for certain changes to the approved product, product labeling, or manufacturing process. We could also be asked to conduct post-marketing clinical trials to confirm the safety and efficacy of our products, if approved, in general or in specific patient subsets. If original marketing approval was obtained via the accelerated approval pathway, we could be required to conduct a successful post-marketing clinical trial to confirm clinical benefit for our products, if approved. An unsuccessful post-marketing study or failure to complete such a study could result in the withdrawal of marketing approval.

The policies of the FDA and of comparable foreign regulatory authorities may change and additional government regulations may be enacted that could 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. As an example, the regulatory landscape related to clinical trials in the EU has evolved. The EU Clinical Trials Regulation (EU) No 536/2014, or CTR, which repealed the EU Clinical Trials Directive, or CTD, became

applicable on January 31, 2022. The CTR permits trial sponsors to make a single submission to both the competent authority and an ethics committee in each EU Member State, leading to a single decision for each EU Member State. The assessment procedure for the authorization of clinical trials has been harmonized as well, including a joint assessment of some elements of the application by all EU Member States in which the trial is to be conducted, and a separate assessment by each EU Member State with respect to specific requirements related to its own territory, including ethics rules. Each EU Member State's decision is communicated to the sponsor through a centralized EU portal, the Clinical Trial Information System, or CTIS. In addition, the CTR requires the publication of certain data and documents in relation to the conduct of a clinical trial, which will take place in accordance with specific timelines. The timelines are established by the European Medicines Agency, or the EMA, and are determined based on the documents and the categorization of the clinical trial. The CTR provided a three-year transition period that ended on January 31, 2025. Since this date, all new or ongoing trials are subject to the provisions of the CTR. Our compliance with the CTR requirements and that of our third-party service providers, such as CROs, may impact our development plans.

The UK regulatory framework in relation to clinical trials is governed by the Medicines for Human Use (Clinical Trials) Regulations 2004, as amended, which is derived from the CTD, as implemented into UK national law through secondary legislation. The United Kingdom government has enacted new legislation to overhaul the clinical trials regulatory framework. In April 2025, the UK adopted the Medicines for Human Use (Clinical Trials) (Amendment) Regulations 2025, intended to support a more streamlined and flexible regulation of clinical trials, remove unnecessary administrative burdens on trial sponsors, and protect the interests of trial participants. The amendment became applicable on April 28, 2026 following a one-year transition period. While these changes introduce efficiencies, the new requirements may impose additional compliance burdens on us and our CROs conducting trials in the United Kingdom and could result in increased costs or delays in trial initiation or conduct. Any significant divergence between the United Kingdom and EU regulatory systems could affect the cost and complexity of conducting clinical trials in the United Kingdom and may impact the acceptability of United Kingdom-based trial data for seeking marketing authorizations in the EU, and vice versa.

In addition, on December 11, 2025, the European Commission, the European Parliament and the European Council reached a political agreement on a comprehensive overhaul of EU pharmaceutical legislation, or the Pharma Package. The reform has been under negotiation since the European Commission submitted its proposal in April 2023. This package – comprised of a new directive and regulation to replace existing legislation – aims to modernize the EU framework. The political agreement is currently subject to formal approval by the European Parliament and Council. If adopted substantially in its proposed form, the Pharma Package could, among other changes, reduce the baseline market protection period by one year, reshape the incentives regime for orphan medicinal products (including by reducing the baseline orphan market exclusivity from ten to nine years and replacing the current indication-based system with a product-based approach under which subsequent orphan indications for the same active substance will no longer benefit from separate periods of market exclusivity), and expand the Bolar exemption to permit generic and biosimilar manufacturers to conduct preparatory activities for regulatory submissions, including pricing and reimbursement, and participate in procurement tenders while patent protection remains in force. Under the agreed transitional provisions, marketing authorizations already granted, and marketing authorization applications submitted before the new regulation becomes applicable, will generally continue to be governed by current protection rules; however, any marketing authorization application we submit after that date, any subsequent application for an additional orphan indication, and the expanded Bolar exemption (for which no transitional protection applies) could reduce the period of market exclusivity available to our product candidates in the EU and accelerate exposure to generic or biosimilar competition, potentially materially impacting the commercial prospects of our product candidates. The new framework is expected to enter into force in 2026/2027 and to be subject to transitional arrangements, with full application not anticipated before 2028.

If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may lose any marketing approval that we may have obtained and we may not achieve or sustain profitability.

If approved, our investigational products regulated as biologics, including rinvatercept and elritercept, may face competition from biosimilars approved through an abbreviated regulatory pathway.

We or our collaborators, as applicable, are developing rinvatercept for the treatment of DMD and for the treatment of ALS, and elritercept for the treatment of cytopenias, including anemia and thrombocytopenia, in patients with MDS and myelofibrosis, both of which we anticipate will be regulated as biological products. The Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010, or collectively the ACA, includes a subtitle called the Biologics Price Competition and Innovation Act of 2009, or BPCIA, which created an abbreviated approval pathway for biological products that are biosimilar to or interchangeable with an FDA-licensed reference biological product. Under the

BPCIA, an application for a biosimilar product may not be submitted to the FDA until four years following the date that the reference product was first licensed by the FDA. In addition, the approval of a biosimilar product may not be made effective by the FDA until 12 years from the date on which the reference product was first licensed. During this 12-year period of exclusivity, another company may still market a competing version of the reference product if the FDA approves a BLA for the competing product containing the sponsor's own nonclinical data and data from adequate and well-controlled clinical trials to demonstrate the safety, purity, and potency of the other company's product. The law is complex and is still being interpreted and implemented by the FDA. As a result, its ultimate impact, implementation, and meaning are subject to uncertainty.

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

The EU also provides opportunities for data and market exclusivity related to marketing authorizations. Upon receiving a marketing authorization, innovative medicinal products are generally entitled to receive eight years of data exclusivity and 10 years of market exclusivity. Data exclusivity, if granted, prevents regulatory authorities in the EU from referencing the innovator's data to assess a generic application or biosimilar application for eight years from the date of authorization of the innovative product, after which a generic or biosimilar marketing authorization application can be submitted, and the innovator's data may be referenced. The market exclusivity period prevents a successful generic or biosimilar applicant from commercializing its product in the EU until 10 years have elapsed from the initial marketing authorization of the reference product in the EU. The overall 10-year period may, occasionally, be extended for a further year to a maximum of 11 years if, during the first eight years of those 10 years, the marketing authorization holder obtains an authorization for one or more new therapeutic indications which, during the scientific evaluation prior to their authorization, are held to bring a significant clinical benefit in comparison with existing therapies. However, there is no guarantee that a product will be considered by the EU's regulatory authorities to be a new chemical/biological entity, and products may not qualify for data exclusivity.

In the EU, there is a special regime for biosimilars, or biological medicinal products that are similar to a reference medicinal product but that do not meet the definition of a generic medicinal product. For such products, the results of appropriate nonclinical or clinical trials must be provided in support of an application for marketing authorization. Guidelines from the EMA detail the type and quantity of supplementary data to be provided for different types of biological product.

We also believe that our product candidates in the EU should benefit from this data and market exclusivity. As with the United States, however, if competitors obtain marketing authorization for their biosimilar products, our products may become subject to competition from these biosimilars, with the attendant competitive pressure and consequences.

We may become exposed to costly and damaging liability claims, either when testing our product candidates in the clinic or at the commercial stage, and our product liability insurance may not cover all damages from such claims.

We are exposed to potential product liability and professional indemnity risks that are inherent in the research, development, manufacturing, marketing and use of biopharmaceutical products. Currently, we have no products that have been approved for commercial sale; however, the current and future use of product candidates by us and our collaborators in clinical trials, and the potential sale of any approved products in the future, may expose us to liability claims. These claims might be made by patients who use the product, healthcare providers, pharmaceutical companies, our collaborators or others selling such products. Any claims against us, regardless of their merit, could be difficult and costly to defend and could materially adversely affect the market for our product candidates or any prospects for commercialization of our product candidates. Regardless of the merits or eventual outcome, liability claims may result in:

- decreased demand for our products, if approved, due to negative public perception;
- injury to our reputation;
- withdrawal of clinical trial participants or difficulties in recruiting new trial participants;
- initiation of investigations by regulators;

- costs to defend or settle the related litigation;
- a diversion of management's time and our resources;
- substantial monetary awards to trial participants or patients;
- product recalls, withdrawals or labeling, marketing or promotional restrictions;
- loss of revenues from product sales; and
- the inability to commercialize any of our product candidates, if approved.

Although we believe we maintain adequate product liability insurance for our product candidates, it is possible that our liabilities could exceed our insurance coverage. We intend to expand our insurance coverage to include the sale of commercial products if we obtain marketing approval for any of our product candidates. However, we may not be able to maintain insurance coverage at a reasonable cost or obtain insurance coverage that will be adequate to satisfy any liability that may arise. If a successful product liability claim or series of claims is brought against us for uninsured liabilities or in excess of insured liabilities, our assets may not be sufficient to cover such claims and our business operations could be impaired.

Should any of the events described above occur, this could have a material adverse effect on our business, financial condition and results of operations.

Due to our limited resources and access to capital, we must, and have in the past decided to, prioritize development of certain product candidates over other potential product candidates. These decisions may prove to have been wrong and may adversely affect our ability to develop our own programs, our attractiveness as a commercial partner and may ultimately have an impact on our commercial success.

Because we have limited resources and access to capital to fund our operations, we must decide which product candidates to pursue and the amount of resources to allocate to each. For example, in August 2025, we announced the deprioritization of cibotercept, including our decision to discontinue all material, internal development activities related to this asset. This decision came after the termination of the development of cibotercept in PAH following the analysis of all available safety and efficacy data from the TROPOS Phase 2 clinical trial in patients with PAH. Our decisions concerning the allocation of research, collaboration, management and financial resources toward particular proprietary molecules in our library, product candidates or therapeutic areas may not lead to the development of viable commercial products and may divert resources away from better opportunities. Similarly, our decisions to delay, terminate or collaborate with third parties in respect of certain product development programs may also prove not to be optimal and could cause us to miss valuable opportunities. If we make incorrect determinations regarding the market potential of our product candidates or misread trends in the biopharmaceutical industry, in particular for rinvatercept and elritercept, our business, financial condition and results of operations could be materially adversely affected.

We may seek Fast Track Designation by the FDA for product candidates that we develop, and we may be unsuccessful. If we are successful, the designation may not actually lead to a faster development or regulatory review or approval process.

We may seek Fast Track Designation for product candidates we develop. If a product is intended for the treatment of a serious or life-threatening condition and nonclinical or clinical data demonstrate the potential to address an unmet medical need for this condition, the product sponsor may apply for Fast Track Designation. The FDA has broad discretion whether or not to grant this designation, so even if we believe a particular product candidate is eligible for this designation, we cannot assure you that the FDA would decide to grant it. We received Fast Track Designation for elritercept for the treatment of anemia in adults with lower-risk MDS, however we may not experience a faster development process, review or approval compared to conventional FDA procedures. The FDA may rescind the Fast Track Designation if it believes that the designation is no longer supported by data from our clinical development activities.

We may seek orphan drug designation for product candidates we develop, and we may be unsuccessful or may be unable to maintain the benefits associated with orphan drug designation, including the potential for market exclusivity.

As part of our business strategy, we may seek orphan drug designation for any product candidates we develop, which meets the related applicable criteria, and we may be unsuccessful. For example, in August 2025, we announced that the FDA granted orphan drug designation for rinvatercept for the treatment of DMD. Regulatory authorities in some jurisdictions, including the United States, may designate drugs for relatively small patient populations as orphan drugs. Under the Orphan Drug Act in the United States, the FDA may designate a drug as an orphan drug if it is a drug intended to treat a rare disease

or condition, which is generally defined as a patient population of fewer than 200,000 individuals annually in the United States, or a patient population greater than 200,000 in the United States where there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the United States. In the United States, orphan drug designation entitles a party to financial incentives such as opportunities for grant funding towards certain clinical trial costs, tax advantages and user-fee waivers.

Generally in the United States, if a drug with an orphan drug designation subsequently receives the first marketing approval for the indication for which it has such designation, the drug is entitled to a period of marketing exclusivity, which precludes the FDA from approving another marketing application for the same drug and indication for seven years, except in limited circumstances.

Even if we obtain orphan drug exclusivity for any of our product candidates, that exclusivity may not effectively protect the product candidate from competition because different therapies can be approved for the same condition and the same therapies can be approved for different conditions but used off-label. Even after an orphan drug is approved, the FDA can subsequently approve the same drug for the same condition if the FDA concludes that the later drug is clinically superior in that it is shown to be safer, more effective or makes a major contribution to patient care. In addition, a designated orphan drug may not receive orphan drug exclusivity if it is approved for a use that is broader than the indication for which it received orphan designation. Moreover, orphan drug exclusive marketing rights in the United States may be lost if the FDA later determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the drug to meet the needs of patients with the rare disease or condition.

In the EU, the European Commission, on the basis of the opinion of the EMA Committee for Orphan Medicinal Products, grants orphan drug designation for medicines to be developed for the diagnosis, prevention or treatment of diseases that are life-threatening or chronically debilitating, for which either no satisfactory method of diagnosis, prevention, or treatment exists, or if such method exists, the medicine is of significant benefit to those affected by such condition. To benefit from such designation, either the prevalence of such condition must not be more than five in 10,000 people across the EU or, if more prevalent, it must be unlikely that the marketing of the medicine would generate sufficient returns to justify the investment needed for its development.

Orphan drug designation neither shortens the development time or regulatory review time of a drug nor gives the drug any advantage in the regulatory review or approval process. While we may seek orphan drug designation for applicable indications for our current and any future product candidates, we may never receive such designations. Even if we do receive such designations, there is no guarantee that we will benefit from those designations.

Risks Related to Commercialization of Our Product Candidates

If we are unable to successfully commercialize any product candidate for which we receive regulatory approval, or experience significant delays in doing so, our business will be materially harmed.

If we are successful in obtaining marketing approval from applicable regulatory authorities for rinvatercept, elritercept or any other product candidate, our ability to generate revenues from any such products will depend on our success in:

- launching commercial sales of such products, whether alone or in collaboration with others;
- receiving approved labels with claims that are necessary or desirable for successful marketing, and that do not contain safety or other limitations that would impede our ability to market such products;
- creating market demand for such products through marketing, sales and promotion activities;
- hiring, training, and deploying a sales force or contracting with third parties to commercialize such products in the United States;
- creating strategic collaborations with, or offering licenses to, third parties to promote and sell such products in foreign markets where we receive marketing approval;
- manufacturing such products in sufficient quantities and at acceptable quality and cost to meet commercial demand at launch and thereafter;
- establishing and maintaining agreements with wholesalers, distributors, and group purchasing organizations on commercially reasonable terms;
- maintaining patent and trade secret protection and regulatory exclusivity for such products;
- achieving market acceptance of such products by patients, the medical community, and third-party payors;
- achieving coverage and adequate reimbursement from third-party payors for such products;

- patients' willingness to pay out-of-pocket in the absence of such coverage and adequate reimbursement from third-party payors;
- effectively competing with other therapies; and
- maintaining a continued acceptable safety profile of such products following launch.

To the extent we are not able to do any of the foregoing, our business, financial condition, results of operations, stock price and prospects will be materially harmed.

We face significant competition from other biotechnology and pharmaceutical companies, and our operating results will suffer if we fail to compete effectively.

The biopharmaceutical industry is characterized by intense competition and rapid innovation. Our competitors may be able to develop other compounds or drugs that are able to achieve similar or better results. Our potential competitors include major multinational pharmaceutical companies, established biotechnology companies, specialty pharmaceutical companies and universities and other research institutions. Many of our competitors have substantially greater financial, technical and other resources, such as larger research and development staff and experienced marketing and manufacturing organizations and well-established sales forces. Smaller or early-stage companies may also prove to be significant competitors, particularly as they develop novel approaches to treating disease indications that our product candidates are also focused on treating. Established pharmaceutical companies may also invest heavily to accelerate discovery and development of novel therapeutics or to in-license novel therapeutics that could make the product candidates that we develop obsolete. Mergers and acquisitions in the biotechnology and pharmaceutical industries may result in even more resources being concentrated in our competitors. Competition may increase further as a result of advances in the commercial applicability of technologies and greater availability of capital for investment in these industries. Our competitors, either alone or with collaborative partners, may succeed in developing, acquiring or licensing on an exclusive basis drug or biologic products that are more effective, safer, more easily commercialized or less costly than our product candidates or may develop proprietary technologies or secure patent protection that we may need for the development of our technologies and products. We believe the key competitive factors that will affect the development and commercial success of our product candidates are efficacy, safety, tolerability, reliability, convenience of use, price and reimbursement.

We compete in the segments of the biotechnology, pharmaceutical and other related industries that develop and market therapies in our target indications. There are many other companies, including large biotechnology and pharmaceutical companies, that have commercialized and/or are developing therapies for the same therapeutic areas that our product candidates target.

Currently, patients with DMD are treated with corticosteroids to manage the inflammatory component of the disease. EMFLAZA (deflazacort) is an FDA-approved corticosteroid marketed by PTC Therapeutics, Inc. and Agamree (vamorolone) is an FDA-approved corticosteroid marketed by Catalyst Pharmaceuticals in the United States. In addition, there are four FDA-approved exon skipping drugs: EXONDYS 51 (eteplirsen), VYONDYS 53 (golodirsen), and AMONDYS 45 (casimersen), which are phosphorodiamidate morpholino oligomers, or PMOs, approved for the treatment of patients with DMD who are amenable to exon 51, exon 53 and exon 45 skipping, respectively, and are marketed by Sarepta Therapeutics, Inc., or Sarepta, and VILTEPSO (vitolarsen), a PMO approved for the treatment of patients with DMD who are amenable to exon 53 skipping, which is marketed by Nippon Shinyaku Co. Ltd. Additionally, in June 2023, Sarepta announced that the FDA accelerated approval of its product, ELEVIDYS, an adeno-associated virus based gene therapy for the treatment of ambulatory pediatric patients aged 4 through 5 years with DMD with a confirmed mutation in the DMD gene. In June 2024, the FDA granted ELEVIDYS full approval for the treatment of ambulatory individuals aged four years and older, and accelerated approval for the treatment of non-ambulatory individuals aged four years and older. In July 2025, Sarepta announced its decision to voluntarily and temporarily pause all shipments of ELEVIDYS for patients with DMD in the United States. Subsequently, in July 2025, Sarepta announced that the FDA notified Sarepta that it may lift its voluntary pause on shipments of ELEVIDYS for ambulatory patients age four years and older with DMD, and Sarepta resumed shipping to such patients immediately.

In March 2024, Italfarmaco S.p.A. announced that the FDA approved Duvyzat (givinostat), a histone deacetylase inhibitor for the treatment of DMD in patients aged six years and older.

In addition, several companies are developing gene therapies to treat DMD, including REGENXBIO Inc. and Solid Biosciences Inc. RNA-targeted treatments that are in clinical and nonclinical development are also being pursued by several companies, including Avidity Biosciences, Inc. (which was acquired by Novartis AG in February 2026), Wave Life Sciences

Ltd., Dyne Therapeutics, Inc. and Sarepta. Edgewise Therapeutics, Inc. (which was acquired by Servier in July 2026) is developing sevasemten, a myosin ATPase inhibitor, for both DMD and Becker muscular dystrophy. Satellos Bioscience Inc. is developing SAT-3277, an AAK1 inhibitor, for the treatment of DMD. Capricor Therapeutics, Inc. is developing Deramiocel, a cell therapy, for the treatment of DMD.

There are three therapies approved by FDA to treat ALS and its symptoms: riluzole, an anti-glutamatergic agent; edaravone, a free-radical scavenger; and tofersen, an antisense oligonucleotide treatment of SOD1-ALS. One additional therapeutic, sodium phenylbutyrate and taurursodiol, was approved under an accelerated pathway by the FDA but subsequently withdrawn from the market following negative results from a confirmatory Phase 3 trial. To the best of our knowledge, there are no other companies developing product candidates to treat ALS by targeting skeletal muscle. However, several companies are developing product candidates to treat ALS through other mechanisms, including Otsuka Pharmaceutical Co., Ltd., Novartis AG and Genervon Biopharmaceuticals LLC.

Kyntra Bio Inc. (formerly FibroGen Inc.) and Astellas Pharma Inc. are developing product candidates for the treatment of anemia, and Merck & Co. Inc., or Merck, Bristol-Myers Squibb Company and Disc Medicine are developing product candidates targeting diseases associated with MDS and myelofibrosis, including chronic anemia. Additionally, in April 2020, Merck and Bristol-Myers Squibb Company received FDA approval of its product, Reblozyl, for the treatment of anemia failing an erythropoiesis stimulating agent and requiring two or more red blood cell units over eight weeks in adult patients with very low- to intermediate-risk MDS with ring sideroblasts or with myelodysplastic/myeloproliferative neoplasm with ring sideroblasts and thrombocytosis. In June 2020, Merck further announced that the European Commission approved Reblozyl for the treatment of transfusion-dependent anemia in adult patients with MDS or beta thalassemia and in September 2020, Merck announced that Health Canada approved Reblozyl for the treatment of adult patients with red blood cell transfusion-dependent anemia associated with beta thalassemia. In August 2023, Bristol-Myers Squibb Company announced that the FDA approved Reblozyl for the treatment of anemia without previous erythropoiesis stimulating agent use (ESA-naïve) in adult patients with very low- to intermediate-risk MDS who may require regular red blood cell transfusions. In April 2024, Bristol-Myers Squibb Company further announced that the European Commission expanded approval of Reblozyl to include treatment of adult patients with and without ring sideroblasts with transfusion-dependent anemia due to lower-risk MDS. In June 2024, Geron Corporation announced that the FDA approved imetelstat (RYTELO) for the treatment of adult patients with low- to intermediate-1 risk MDS with transfusion-dependent anemia requiring four or more red blood cell units over eight weeks who have not responded to or have lost response to or are ineligible for erythropoiesis-stimulating agents.

In March 2022, CTI BioPharma Corp. (which was acquired by Swedish Orphan Biovitrum AB in June 2023) received FDA accelerated approval of its product, pacritinib (Vonjo), for the treatment of adults with intermediate or high-risk primary or secondary (post-polycythemia vera or post-essential thrombocythemia) myelofibrosis with a platelet count below 50×10^9/L. In September 2023, GSK plc announced that the FDA approved its product, Ojjaara, for the treatment of intermediate or high-risk myelofibrosis, including primary myelofibrosis or secondary myelofibrosis (post-polycythaemia vera and post-essential thrombocythaemia), in adults with anemia. Additionally, MorphoSys AG (which was acquired by Novartis AG in July 2024) is also developing a product candidate as a treatment for myelofibrosis, and Incyte Corporation is developing an ALK2 inhibitor product candidate for the treatment of myelofibrosis. Geron Corporation is also developing imetelstat as a treatment for myelofibrosis.

Other companies that are developing product candidates or have approved products that are designed to target the TGF-ß signaling pathways include Scholar Rock Holding Corporation and Regeneron Pharmaceuticals, Inc.

We anticipate that we will continue to face intense and increasing competition as new treatments enter the market and advanced technologies become available. There can be no assurance that our competitors are not currently developing, or will not in the future develop, products that are equally or more effective or are more economically attractive than any of our current or future product candidates. Competing products may gain faster or greater market acceptance than our products, if any, and medical advances or rapid technological development by competitors may result in our product candidates becoming non-competitive or obsolete before we are able to recover our research and development and commercialization expenses. If we or our product candidates do not compete effectively, it may have a material adverse effect on our business, financial condition and results of operations.

We do not have a sales or marketing infrastructure and have no experience in the sale or marketing of biopharmaceutical products. To achieve commercial success for any approved product, we must develop or acquire a sales and marketing organization, outsource these functions to third parties or enter into strategic collaborations.

We may decide to establish our own sales and marketing capabilities and promote our product candidates if and when regulatory approval has been obtained in the United States or in other jurisdictions. There are risks involved if we decide to establish our own sales and marketing capabilities or enter into arrangements with third parties to perform these services. Even if we establish sales and marketing capabilities, we may fail to launch our products, if approved, effectively or to market our products effectively since we have no experience in the sales and marketing of biopharmaceutical products. In addition, recruiting and training a sales force is expensive and time-consuming and could delay any product launch. In the event that any such launch is delayed or does not occur for any reason, we would have prematurely or unnecessarily incurred these commercialization expenses, and our investment would be lost if we cannot retain or reposition our sales and marketing personnel. Factors that may inhibit our efforts to commercialize our products, if approved, on our own include:

- our inability to recruit, train and retain adequate numbers of effective sales and marketing personnel;
- the inability of sales personnel to obtain access to or educate adequate numbers of physicians on the benefits of our products, if approved;
- the lack of complementary products to be offered by sales personnel, which may put us at a competitive disadvantage relative to companies with more extensive product lines;
- unforeseen costs and expenses associated with creating an independent sales and marketing organization; and
- costs of marketing and promotion above those anticipated by us.

If we enter into arrangements with third parties to perform sales and marketing services, our product revenues or the profitability of these product revenues to us could be lower than if we were to market and sell any products that we develop ourselves. Such collaborative arrangements with partners may place the commercialization of our products, if approved, outside of our control and would make us subject to a number of risks including that we may not be able to control the amount or timing of resources that our collaborative partner devotes to our products, if any, or that our collaborator's willingness or ability to complete its obligations, and our obligations under our arrangements may be adversely affected by business combinations or significant changes in our collaborator's business strategy. In addition, we may not be successful in entering into arrangements with third parties to sell and market our products, if approved, or may be unable to do so on terms that are favorable to us. Acceptable third parties may fail to devote the necessary resources and attention to sell and market our products, if any, effectively.

If we do not establish sales and marketing capabilities successfully, either on our own or in collaboration with third parties, we may not be successful in commercializing our products, if any, which in turn would have a material adverse effect on our business, financial condition and results of operations.

Even if a product candidate we develop receives marketing approval, it may fail to achieve the degree of market acceptance by physicians, patients, third-party payors and others in the medical community necessary for commercial success. The revenues that we generate from their sales may be limited, and we may never become profitable.

We have never commercialized a product candidate for any indication. Even if our product candidates are approved by the appropriate regulatory authorities for marketing and sale, they may not gain acceptance among physicians, patients, third-party payors and others in the medical community. If any product candidates for which we obtain regulatory approval do not gain an adequate level of market acceptance, we could be prevented from or significantly delayed in achieving profitability. Market acceptance of our product candidates by the medical community, patients and third-party payors will depend on a number of factors, some of which are beyond our control. For example, physicians are often reluctant to switch their patients and patients may be reluctant to switch from existing therapies even when new and potentially more effective or safer treatments enter the market.

Efforts to educate the medical community and third-party payors on the benefits of our product candidates may require significant resources and may not be successful. If any of our product candidates are approved but do not achieve an adequate level of market acceptance, we could be prevented from or significantly delayed in achieving profitability. The degree of market acceptance of any product for which we receive marketing approval will depend on a number of factors, including:

- the clinical indications for which our product candidates are approved;
- physicians, hospitals and patients considering our product candidates as a safe and effective treatment;
- the potential and perceived advantages of our product candidates over alternative treatments;
- the prevalence and severity of any side effects;
- product labeling or product insert requirements of the FDA or comparable foreign regulatory authorities;

- limitations or warnings contained in the labeling approved by the FDA or comparable foreign regulatory authorities;
- the timing of market introduction of our product candidates in relation to other potentially competitive products;
- the cost of our product candidates in relation to alternative treatments;
- the amount of upfront costs or training required for physicians to administer our product candidates;
- the availability of coverage and adequate reimbursement from third-party payors and government authorities;
- the willingness of patients to pay out-of-pocket in the absence of comprehensive coverage and reimbursement by third-party payors and government authorities;
- the relative convenience and ease of administration, including as compared to alternative treatments and competitive therapies;
- the effectiveness of our sales and marketing efforts and distribution support; and
- the presence or perceived risk of potential product liability claims.

Enacted and future healthcare legislation may increase the difficulty and cost for us to progress our clinical programs and obtain marketing approval of and commercialize our product candidates and may affect the prices we may set.

In the United States and other jurisdictions, there have been, and we expect there will continue to be, a number of legislative and regulatory changes and proposed changes to the healthcare system that could affect our future results of operations. In particular, there have been and continue to be a number of initiatives at the U.S. federal and state levels that seek to reduce healthcare costs and improve the quality of healthcare. For example, in March 2010, the ACA was enacted, which substantially changed the way healthcare is financed by both governmental and private insurers.

Since its enactment, there have been judicial, Congressional and executive branch challenges and amendments to certain aspects of the ACA. For example, on July 4, 2025, the One Big Beautiful Bill Act, or the OBBBA, was signed into law, which narrowed access to ACA marketplace exchange enrollment and declined to extend the ACA enhanced advanced premium tax credits that expired at the end of 2025, which, among other provisions in the law, are anticipated to reduce the number of Americans with health insurance. The OBBBA also is expected to reduce Medicaid spending and enrollment by implementing work requirements for some beneficiaries, capping state-directed payments, reducing federal funding, and limiting provider taxes used to fund the program. Congress is considering proposed legislation intended to further reduce healthcare costs with alternatives to replace the expired ACA subsidies. Other legislative changes have also been proposed and adopted in the United States since the ACA was enacted. For example, in August 2011, the Budget Control Act of 2011, among other things, led to aggregate reductions of Medicare payments to providers of 2% per fiscal year. These reductions went into effect in April 2013 and, due to subsequent legislative amendments to the statute, including the Infrastructure Investment and Jobs Act and the Consolidated Appropriations Act of 2023, will remain in effect until 2032, unless additional action is taken by Congress. These new laws or any other similar laws introduced in the future may result in additional reductions in Medicare and other health care funding, which could negatively affect our customers and accordingly, our financial operations.

The current administration is pursuing policies to reduce regulations and expenditures across government agencies including at the U.S. Department of Health and Human Services, or HHS, the FDA, the U.S. Centers for Medicare & Medicaid Services, or CMS, and related agencies. These actions, presently directed by executive orders or memoranda from the Office of Management and Budget, may propose policy changes that create additional uncertainty for our business. For example, the current administration has announced agreements with certain pharmaceutical companies that require the drug manufacturers to offer, through a direct-to-consumer platform, or TrumpRx, U.S. patients and Medicaid programs prescription drug Most-Favored Nation pricing equal to or lower than those paid in other developed nations, with additional mandates for direct-to-patient discounts and repatriation of foreign revenues. Other recent actions, for example, include (1) directing agencies to reduce agency workforce and cut programs; (2) directing HHS and other agencies to lower prescription drug costs through a variety of initiatives, including by improving upon the Medicare Drug Price Negotiation Program and establishing Most-Favored-Nation pricing for pharmaceutical products; (3) imposing tariffs on certain imported pharmaceutical products; and (4) as part of the Make America Healthy Again Commission's Strategy Report released in September 2025, working across government agencies to increase enforcement on direct-to-consumer pharmaceutical advertising. Additionally, the current administration recently called on Congress to enact "The Great Healthcare Plan," to codify and expand Most-Favored Nation pricing, lower government subsidies to private insurance companies, increase healthcare price transparency, expand pharmaceutical drugs available for over-the-counter purchase, and enact restrictions on pharmacy benefit manager payment methodologies, among other things. In addition, the administration is pursuing other most-favored-nation drug pricing policies for certain prescription drugs. As part of these initiatives, the administration is implementing or considering various CMS Center for Medicare and Medicaid Innovation models, under which manufacturers may owe rebates for certain prescription drugs under Medicaid or Medicare if the price of a drug included in an applicable model exceeds an international benchmark

calculated based on prices available in certain reference countries. These include the finalized Global Benchmark for Efficient Drug Pricing Model (GLOBE) Model for certain Medicare Part B drugs; and the proposed Guarding United States Medicare Against Rising Drug Costs (GUARD) Model for certain Medicare Part D drugs, among others. These actions and policies may significantly reduce U.S. drug prices, potentially impacting manufacturers' global pricing strategies and profitability, while increasing their operational costs and compliance risks. In June 2024, Loper Bright Enterprises v. Raimondo, the U.S. Supreme Court greatly reduced judicial deference to regulatory agencies, which could increase successful legal challenges to federal regulations affecting our operations. Congress may introduce and ultimately pass health care related legislation that could impact the drug approval process and make changes to the Medicare Drug Price Negotiation Program.

Individual states in the United States have also increasingly passed legislation and implemented regulations designed to control pharmaceutical and biological 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. For example, on June 15, 2026, the FDA approved Colorado's Section 804 Importation Program proposal to import certain drugs from Canada for specific state healthcare programs. It is unclear how this and Florida's similar program, approved by the FDA in 2024, will be implemented and whether they will overcome potential legal, regulatory, or industry challenges in the United States and/or Canada. Legally mandated price controls on payment amounts by third-party payors or other restrictions could harm our business, results of operations, financial condition and prospects. In addition, regional healthcare authorities and individual hospitals are increasingly using bidding procedures to determine what pharmaceutical products and which suppliers will be included in their prescription drug and other healthcare programs. This could reduce the ultimate demand for our product candidates or put pressure on our product pricing.

In markets outside of the United States, reimbursement and healthcare payment systems vary significantly by country, and many countries have instituted price ceilings on specific products and therapies. For example, the EU provides options for EU Member States to restrict the range of medicinal products for which their national health insurance systems provide reimbursement and to control the prices of medicinal products for human use. An EU Member State may approve a specific price for the medicinal product, it may refuse to reimburse a product at the price set by the manufacturer or it may instead adopt a system of direct or indirect controls on the profitability of the company placing the medicinal product on the market. Many EU Member States also periodically review their reimbursement procedures for medicinal products, which could have an adverse impact on reimbursement status. We expect that legislators, policymakers and healthcare insurance funds in the EU Member States will continue to propose and implement cost-containing measures, such as lower maximum prices, lower or lack of reimbursement coverage and incentives to use cheaper, usually generic, products as an alternative to branded products, and/or branded products available through parallel import to keep healthcare costs down.

Moreover, in order to obtain reimbursement for our products in some European countries, including some EU Member States, we may be required to compile additional data comparing the cost-effectiveness of our products to other available therapies. The Health Technology Assessment, or HTA, of medicinal products is becoming an increasingly common part of the pricing and reimbursement procedures in some EU Member States, including those representing the larger markets. The HTA process is the procedure to assess therapeutic, economic and societal impact of a given medicinal product in the national healthcare systems of the individual country. The outcome of an HTA will often influence the pricing and reimbursement status granted to these medicinal products by the competent authorities of individual EU Member States. The extent to which pricing and reimbursement decisions are influenced by the HTA of the specific medicinal product currently varies between EU Member States. On January 12, 2025, Regulation (EU) 2021/2282 on HTA entered into application through a phased implementation. The HTA Regulation initially applies to new active substances for oncology products and advanced therapy medicinal products, and will expand to orphan medicinal products in January 2028 and to all centrally authorized medicinal products by 2030. The Regulation is intended to boost cooperation among EU Member States in assessing health technologies, including new medicinal products, and establishes a framework for EU-level joint clinical assessments. The regulation permits EU Member States to use common HTA tools, methodologies, and procedures across the EU to identify promising technologies early, and continuing voluntary cooperation in other areas. Individual EU Member States will continue to be responsible for assessing non-clinical (e.g., economic, social, ethical) aspects of health technologies, and making decisions on pricing and reimbursement. As the HTA Regulation applies to orphan medicinal products from 2028, joint clinical assessments conducted at the EU level may result in adverse or delayed outcomes that negatively affect our ability to obtain or maintain favorable reimbursement status across EU Member States. If we are unable to maintain favorable pricing and reimbursement status in EU Member States for product candidates that we may successfully develop and for which we may obtain regulatory approval, any anticipated revenue from and growth prospects for those products in the EU could be negatively affected.

We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action in the United States or any other jurisdiction. The implementation of cost containment measures or other healthcare reforms may negatively impact our operations. If we or any third parties we may engage are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we or such third parties are not able to maintain regulatory compliance, our product candidates may lose any regulatory approval that may have been obtained and we may not achieve or sustain profitability.

Disruptions at the FDA, the SEC and other government agencies or comparable regulatory authorities caused by funding shortages or global health concerns could hinder their ability to hire and retain key leadership and other personnel, otherwise prevent new products and services from being developed, approved or commercialized in a timely manner or at all, 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 or comparable foreign regulatory authorities 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, statutory, regulatory and policy changes, and other events that may otherwise affect the FDA's or comparable foreign regulatory authorities' ability to perform routine functions. Average review times at the FDA have fluctuated in recent years as a result. In addition, government funding of the Securities and Exchange Commission, or the SEC, and other government agencies or comparable foreign regulatory authorities on which our operations may rely, including those that fund research and development activities is subject to the political process, which is inherently fluid and unpredictable.

Disruptions at the FDA, other agencies or comparable foreign regulatory authorities may also slow the time necessary for new drugs to be reviewed and/or approved, which would adversely affect our business. For example, the U.S. government has shut down several times, including in October 2025, and certain regulatory agencies, such as the FDA and the SEC,
have had to furlough critical employees and stop critical activities during such shutdowns. If a prolonged government shutdown occurs, or if global health concerns prevent the FDA or other regulatory authorities from conducting their regular inspections, reviews, or other regulatory activities, it could significantly impact the ability of the FDA or comparable foreign regulatory authorities to timely review and process our regulatory submissions, which could have a material adverse effect on our business. Further, future government shutdowns or delays could impact our ability to access the public markets and obtain necessary capital in order to properly capitalize and continue our operations.

Our business operations and current and future relationships with investigators, healthcare professionals, consultants, third-party payors, patient organizations and customers may be subject to applicable healthcare regulatory laws, which could expose us to penalties.

Our business operations and current and future arrangements with investigators, healthcare professionals, consultants, third-party payors, patient organizations and customers, may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations. These laws may constrain the business or financial arrangements and relationships through which we conduct our operations, including how we research, market, sell and distribute our product candidates, if approved. Such laws include:

- the U.S. federal Anti-Kickback Statute, which prohibits, among other things, persons or entities from knowingly and willfully soliciting, offering, receiving or providing any remuneration (including any kickback, bribe, or certain rebate), directly or indirectly, overtly or covertly, in cash or in kind, to induce or reward, or in return for, either the referral of an individual for, or the purchase, lease, order or recommendation of, any good, facility, item or service, for which payment may be made, in whole or in part, under U.S. federal and state healthcare programs such as Medicare and Medicaid. A person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation;
- the U.S. federal civil and criminal false claims laws, including the civil False Claims Act, which can be enforced by private individuals on behalf of the government through civil whistleblower or qui tam actions, and civil monetary penalties laws prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, to the U.S. federal government, claims for payment or approval that are false or fraudulent, knowingly making, using or causing to be made or used, a false record or statement material to a false or fraudulent claim, or from knowingly making a false statement to avoid, decrease or conceal an obligation to pay money to the U.S. federal government. In addition, the government may assert that a claim including items and services resulting from a violation of the U.S. federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the civil False Claims Act;

- the U.S. federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which created additional federal civil and criminal liability for, among other things, knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program, or knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false statement, in connection with the delivery of, or payment for, healthcare benefits, items or services. Similar to the U.S. federal Anti-Kickback Statute, a person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation;
- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009, or HITECH, and their implementing regulations, which impose certain obligations, including mandatory contractual terms, with respect to safeguarding the privacy, security and transmission of individually identifiable health information without appropriate authorization by covered entities subject to the rule, such as health plans, healthcare clearinghouses and certain healthcare providers and their business associates, independent contractors of a covered entity that perform certain services involving the use or disclosure of individually identifiable health information, as well as their covered subcontractors;
- the Federal Food, Drug, and Cosmetic Act, which prohibits, among other things, the adulteration or misbranding of drugs, biologics and medical devices;
- the U.S. Public Health Service Act, which prohibits, among other things, the introduction into interstate commerce of a biological product unless a biologics license is in effect for that product;
- the U.S. Physician Payments Sunshine Act and its implementing regulations, which require certain manufacturers of drugs, devices, biologics and medical supplies that are reimbursable under Medicare, Medicaid, or the Children's Health Insurance Program, with specific exceptions, to report annually to CMS information related to certain payments and other transfers of value made in the prior year to physicians (defined to include doctors, dentists, optometrists, podiatrists, and chiropractors), other healthcare professionals (such as physician assistants and nurse practitioners), and teaching hospitals, as well as ownership and investment interests held by such physicians and their immediate family members; and
- analogous U.S. state and foreign laws and regulations, including: anti-kickback and false claims laws, which may apply to our business practices, including but not limited to, research, distribution, sales and marketing arrangements and claims involving healthcare items or services reimbursed by any third-party payor, including private insurers; laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the U.S. federal government or foreign regulatory authorities, or otherwise restrict payments that may be made to healthcare providers and other potential referral sources; laws and regulations that require drug manufacturers to file reports relating to drug pricing and marketing information, which requires tracking gifts and other remuneration and items of value provided to healthcare professionals and entities; laws that require certain regulatory licenses to manufacture or distribute our products commercially and/or the registration of pharmaceutical sales representatives; and laws governing the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

In addition, our activities are also subject to certain federal, state and foreign consumer-protection and unfair-competition laws, which broadly regulate marketplace activities and activities that potentially harm consumers.

Ensuring that our internal operations and future business arrangements with third parties comply with applicable healthcare laws and regulations will involve substantial costs. It is possible that governmental authorities will conclude that our business practices do not comply with current or future statutes, regulations, agency guidance or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of the laws described above or any other governmental laws and regulations that may apply to us, we may be subject to significant penalties, including civil, criminal and administrative penalties, damages, fines, exclusion from government-funded healthcare programs, such as Medicare and Medicaid or similar programs in other countries or jurisdictions, integrity oversight and reporting obligations to resolve allegations of non-compliance, disgorgement, imprisonment, contractual damages, reputational harm, diminished profits and the curtailment or restructuring of our operations. If any of the physicians or other providers or entities with whom we expect to do business are found to not be in compliance with applicable laws, they may be subject to significant criminal, civil or administrative sanctions, including exclusions from government funded healthcare programs and imprisonment, which could affect our ability to operate our business. Further, defending against any such actions can be costly, time-consuming and may require significant personnel resources. Therefore, even if we are successful in defending against any such actions that may be brought against us, our business may be impaired.

If the market opportunities for our product candidates are smaller than we believe they are, even assuming approval of a product candidate, our business may suffer.

Our projections of both the number of people who are affected by disease within our potential target indications, as well as the subset of these people who have the potential to benefit from treatment with our product candidates, are based on our beliefs and estimates. These estimates have been derived from a variety of sources, including the scientific literature, healthcare utilization databases and market research, and may prove to be incorrect. Further, new studies may change the estimated incidence or prevalence of these diseases. The number of patients may turn out to be lower than expected. Likewise, the potentially addressable patient population for each of our product candidates may be limited or may not be amenable to treatment with our product candidates, and new patients may become increasingly difficult to identify or gain access to, which would adversely affect our business, financial condition and results of operations.

Any product candidates we develop may become subject to unfavorable third-party coverage and reimbursement practices, as well as pricing regulations.

The availability and extent of coverage and adequate reimbursement by third-party payors, including government health administration authorities, private health coverage insurers, managed care organizations and other third-party payors is essential for most patients to be able to afford expensive treatments. Sales of any of our product candidates that receive marketing approval will depend substantially, both in the United States and internationally, on the extent to which the costs of our product candidates will be covered and reimbursed by third-party payors. If reimbursement is not available, or is available only to limited levels, we may not be able to successfully commercialize our product candidates. Even if coverage is provided, the approved reimbursement amount may not be high enough to allow us to establish or maintain pricing sufficient to realize an adequate return on our investment. Coverage and reimbursement may impact the demand for, or the price of, any product candidate for which we obtain marketing approval. If coverage and reimbursement are not available or reimbursement is available only to limited levels, we may not successfully commercialize any product candidate for which we obtain marketing approval.

There is significant uncertainty related to third-party payor coverage and reimbursement of newly approved products. In the United States, for example, principal decisions about reimbursement for new products are typically made by the CMS. The CMS decide whether and to what extent a new product will be covered and reimbursed under Medicare, and private third-party payors often follow CMS's decisions regarding coverage and reimbursement to a substantial degree. However, one third-party payor's determination to provide coverage for a product candidate does not assure that other payors will also provide coverage for the product candidate. As a result, the coverage determination process is often time-consuming and costly. This process will require us to provide scientific and clinical support for the use of our products, if approved, to each third-party payor separately, with no assurance that coverage and adequate reimbursement will be applied consistently or obtained in the first instance.

Increasingly, third-party payors are requiring that drug companies provide them with predetermined discounts from list prices and are challenging the prices charged for medical products. Further, such payors are increasingly challenging the price, examining the medical necessity and reviewing the cost effectiveness of medical product candidates. There may be especially significant delays in obtaining coverage and reimbursement for newly approved drugs. Third-party payors may limit coverage to specific product candidates on an approved list, known as a formulary, which might not include all FDA-approved drugs for a particular indication. We may need to conduct expensive pharmaco-economic studies to demonstrate the medical necessity and cost effectiveness of our products, if any. Nonetheless, our product candidates may not be considered medically necessary or cost effective. We cannot be sure that coverage and reimbursement will be available for any product that we commercialize and, if reimbursement is available, what the level of reimbursement will be.

Furthermore, obtaining coverage and adequate reimbursement for products administered under the supervision of a physician may be particularly difficult because of the higher prices often associated with such drugs. Additionally, separate reimbursement for the product itself or the treatment or procedure in which the product is used may not be available, which may impact physician utilization. In addition, companion diagnostic tests require coverage and reimbursement separate and apart from the coverage and reimbursement for their companion pharmaceutical or biological products. Similar challenges to obtaining coverage and reimbursement, applicable to pharmaceutical or biological products, will apply to companion diagnostics.

Further, HHS imposes rebates on many Medicare Part B and Medicare Part D products to penalize price increases that outpace inflation on an annual basis. HHS has also been empowered to negotiate the price of certain single-source drugs that have been on the market for at least seven years covered under Medicare as part of the Medicare Drug Price Negotiation Program. Each year up to 20 products will be selected by HHS for the Medicare Drug Price Negotiation Program. Products

subject to the Medicare Drug Price Negotiation Program are expected to experience a significant reduction in reimbursement from the Medicare program on a per unit basis.

Outside the United States, international operations are generally subject to extensive governmental price controls and other market regulations, and we believe the increasing emphasis on cost containment initiatives in Europe, Canada and other countries has and will continue to put pressure on the pricing and usage of therapeutics such as our product candidates. In many countries, particularly the countries of the EU, medical product prices are subject to varying price control mechanisms as part of national health systems. In these countries, pricing negotiations with governmental authorities can take considerable time after a product receives marketing approval. To obtain reimbursement or pricing approval in some countries, we may be required to conduct a clinical trial that compares the cost-effectiveness of our product candidate to other available therapies. In general, product prices under such systems are substantially lower than in the United States. Other countries allow companies to fix their own prices for products, but monitor and control company profits. Additional foreign price controls or other changes in pricing regulation could restrict the amount that we are able to charge for our product candidates. Accordingly, in markets outside the United States, the reimbursement for our products, if approved, may be reduced compared with the United States and may be insufficient to generate commercially reasonable revenue and profits.

If we are unable to establish or sustain coverage and adequate reimbursement for any future product candidates from third-party payors, the adoption of those products and sales revenue will be adversely affected, which, in turn, could adversely affect the ability to market or sell those product candidates, if approved. Coverage policies and third-party payor reimbursement rates may change at any time. Even if favorable coverage and reimbursement status is attained for one or more products for which we receive regulatory approval, less favorable coverage policies and reimbursement rates may be implemented in the future.

Risks Related to Our Intellectual Property

Our success depends in part on our ability to protect our intellectual property. It is difficult and costly to protect our proprietary rights and technology, and we may not be able to ensure their protection.

Our commercial success will depend in large part on obtaining and maintaining patent, trademark and trade secret protection of our proprietary technologies and our product candidates, their respective components, formulations, combination therapies, methods used to manufacture them and methods of treatment, as well as successfully defending these patents against third-party challenges. Our ability to stop unauthorized third parties from making, using, selling, offering to sell or importing our product candidates is dependent upon the extent to which we have rights under valid and enforceable patents that cover these activities. If we are unable to secure and maintain patent protection for any product or technology we develop, or if the scope of the patent protection secured is not sufficiently broad, our competitors could develop and commercialize products and technology similar or identical to ours, and our ability to commercialize any product candidates we may develop may be adversely affected.

The patenting process is expensive and time-consuming, and we may not be able to file, prosecute and maintain all necessary or desirable patent applications at a reasonable cost or in a timely manner. In addition, we may not pursue, obtain or maintain patent protection in all relevant markets. It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection. Moreover, in some circumstances, we may not have the right to control the preparation, filing and prosecution of patent applications, or to maintain the patents, covering technology that we license from or license to third parties and are reliant on our licensors or licensees. Therefore, these patents and applications may not be prosecuted and enforced in a manner consistent with the best interests of our business.

The strength of patents in the biotechnology and pharmaceutical field involves complex legal and scientific questions and can be uncertain. The patent applications that we own or in-license may fail to result in issued patents with claims that cover our product candidates or uses thereof in the United States or in other foreign countries. Even if the patents do successfully issue, third parties may challenge the validity, enforceability or scope thereof, which may result in such patents being narrowed, invalidated or held unenforceable. Furthermore, even if they are unchallenged, our patents and patent applications may not adequately protect our intellectual property or prevent others from designing around our claims. If the breadth or strength of protection provided by the patent applications we hold with respect to our product candidates is threatened, it could dissuade companies from collaborating with us to develop, and threaten our ability to commercialize, our product candidates. Further, if we encounter delays in our clinical trials, the period of time during which we could market our product candidates under patent protection would be reduced.

Since patent applications in the United States and most other countries are confidential for a period of time after filing, we cannot be certain that we were the first to file any patent application related to our product candidates. Furthermore, for United States applications in which all claims are entitled to a priority date before March 16, 2013, an interference proceeding can be provoked by a third party or instituted by the United States Patent and Trademark Office, or USPTO, to determine who was the first to invent any of the subject matter covered by the patent claims of our applications.

We cannot be certain that we are the first to invent the inventions covered by pending patent applications and, if we are not, we may be subject to priority disputes. We may be required to disclaim part or all of the term of certain patents or all of the term of certain patent applications. There may be prior art of which we are not aware that may affect the validity or enforceability of a patent claim, and we may be subject to a third-party preissuance submission of prior art to the USPTO. There also may be prior art of which we are aware, but which we do not believe affects the validity or enforceability of a claim, which may, nonetheless, ultimately be found to affect the validity or enforceability of a claim. No assurance can be given that if challenged, our patents would be declared by a court to be valid or enforceable or that even if found valid and enforceable, a competitor's technology or product would be found by a court to infringe our patents. We may analyze patents or patent applications of our competitors that we believe are relevant to our activities, and consider that we are free to operate in relation to our product candidates, but our competitors may achieve issued claims, including in patents we consider to be unrelated, which block our efforts or may potentially result in our product candidates or our activities infringing such claims. The possibility exists that others will develop products which have the same effect as our products, if any, on an independent basis which do not infringe our patents or other intellectual property rights, or will design around the claims of patents that we have had issued that cover our products, if approved.

Recent or future patent reform legislation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents. Under the enacted Leahy-Smith America Invents Act, or America Invents Act, enacted in 2013, the United States moved from a "first to invent" to a "first-to-file" system. Under a "first-to-file" system, assuming the other requirements for patentability are met, the first inventor to file a patent application generally will be entitled to a patent on the invention regardless of whether another inventor had made the invention earlier. The America Invents Act includes a number of other significant changes to U.S. patent law, including provisions that affect the way patent applications are prosecuted, redefine prior art and establish a new post-grant review system. The effects of these changes are currently unclear as the USPTO only recently developed new regulations and procedures in connection with the America Invents Act and many of the substantive changes to patent law, including the "first-to-file" provisions, only became effective in March 2013. In addition, the courts have yet to address many of these provisions and the applicability of the act and new regulations on specific patents discussed herein have not been determined and would need to be reviewed. However, the America Invents Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could have a material adverse effect on our business and financial condition.

The degree of future protection for our proprietary rights is uncertain because legal means afford only limited protection and may not adequately protect our rights or permit us to gain or keep our competitive advantage. For example:

- others may be able to make or use compounds or cells that are similar to the biological compositions of our product candidates but that are not covered by the claims of our patents;
- the active biological ingredients in our current product candidates will eventually become commercially available in biosimilar drug products, and no patent protection may be available with regard to formulation or method of use;
- we or our licensors, as the case may be, may fail to meet our obligations to the U.S. government in regards to any in-licensed patents and patent applications funded by U.S. government grants, leading to the loss of patent rights;
- we or our licensors, as the case may be, might not have been the first to file patent applications for these inventions;
- others may independently develop similar or alternative technologies or duplicate any of our technologies;
- it is possible that our pending patent applications will not result in issued patents;
- it is possible that there are prior public disclosures that could invalidate our or our licensors' patents, as the case may be, or parts of our or their patents;
- it is possible that others may circumvent our owned or in-licensed patents;
- it is possible that there are unpublished applications or patent applications maintained in secrecy that may later issue with claims covering our products, if any, or technology similar to ours;
- the laws of foreign countries may not protect our or our licensors', as the case may be, proprietary rights to the same extent as the laws of the United States;
- the claims of our owned or in-licensed issued patents or patent applications, if and when issued, may not cover our product candidates;

- our owned or in-licensed issued patents may not provide us with any competitive advantages, may be narrowed in scope, or be held invalid or unenforceable as a result of legal challenges by third parties;
- the inventors of our owned or in-licensed patents or patent applications may become involved with competitors, develop products or processes which design around our patents, or become hostile to us or the patents or patent applications on which they are named as inventors;
- it is possible that our owned or in-licensed patents or patent applications omit individual(s) that should be listed as inventor(s) or include individual(s) that should not be listed as inventor(s), which may cause these patents or patents issuing from these patent applications to be held invalid or unenforceable;
- we have engaged in scientific collaborations in the past, and will continue to do so in the future. Such collaborators may develop adjacent or competing products to ours that are outside the scope of our patents;
- we may not develop additional proprietary technologies for which we can obtain patent protection;
- it is possible that product candidates or diagnostic tests we develop may be covered by third parties' patents or other exclusive rights; or
- the patents of others may have an adverse effect on our business.

We depend on intellectual property licensed from third parties and termination of any of these licenses could result in the loss of significant rights, which would harm our business.

We are dependent on patents, know-how and proprietary technology, both our own and licensed from others. Any termination of these licenses could result in the loss of significant rights and could harm our ability to commercialize our product candidates. See the section titled "Management's Discussion and Analysis of Financial Condition and Results of Operations—Licensing Agreements" set forth in Part I, Item 2 of this Quarterly Report on Form 10-Q for additional information regarding our license agreements.

Disputes may also arise between us and our licensors regarding intellectual property subject to a license agreement, including:

- the scope of rights granted under the license agreement and other interpretation-related issues;
- whether and the extent to which our technology and processes infringe on intellectual property of the licensor that is not subject to the licensing agreement;
- our right to sublicense patents and other rights to third parties under collaborative development relationships;
- our diligence obligations with respect to the use of the licensed technology in relation to our development and commercialization of our product candidates, and what activities satisfy those diligence obligations; and
- the inventorship or ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and us and our partners.

In addition, intellectual property license agreements are complex, and certain provisions in such agreements may be susceptible to multiple interpretations. The resolution of any contract interpretation disagreement that may arise could narrow what we believe to be the scope of our rights to the relevant intellectual property or technology, or increase what we believe to be our financial or other obligations under the relevant agreement, either of which could have a material adverse effect on our business, financial condition, results of operations and prospects. If disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on acceptable terms, we may be unable to successfully develop and commercialize the affected product candidates.

We are generally also subject to all of the same risks with respect to protection of intellectual property that we license, as we are for intellectual property that we own, which are described below. If we or our licensors fail to adequately protect this intellectual property, our ability to commercialize products could suffer.

If we fail to comply with our obligations under our patent license with a third party, we could lose license rights that are important to our business.

We are a party to a license agreement pursuant to which we in-license key patent and patent applications for our product candidates. These existing licenses impose various diligence, milestone payment, royalty, insurance and other obligations on us. If we fail to comply with these obligations, our licensor may have the right to terminate the license, in which event we would not be able to develop or market the products covered by such licensed intellectual property. Termination of these agreements or reduction or elimination of our rights under these agreements, or restrictions on our ability to freely assign or

sublicense our rights under such agreements when it is in the interest of our business to do so, may impede, delay or prohibit the further development or commercialization of one or more product candidates that rely on such agreements.

We may have limited control over the maintenance and prosecution of these in-licensed patents and patent applications, activities or any other intellectual property that may be related to our in-licensed intellectual property. For example, we cannot be certain that such activities by our licensor have been or will be conducted in compliance with applicable laws and regulations or will result in valid and enforceable patents and other intellectual property rights.

If we are unable to protect the confidentiality of our trade secrets, our business and competitive position would be harmed.

In addition to patent protection, we rely heavily upon know-how and to some extent trade secret protection, as well as non-disclosure agreements and invention assignment agreements with our employees, consultants and third-parties, to protect our confidential and proprietary information, especially where we do not believe patent protection is appropriate or obtainable. In addition to contractual measures, we try to protect the confidential nature of our proprietary information using physical and technological security measures. Such measures may not, for example, in the case of misappropriation of a trade secret by an employee or third party with authorized access, provide adequate protection for our proprietary information. Our security measures may not prevent an employee or consultant from misappropriating our trade secrets and providing them to a competitor, and recourse we take against such misconduct may not provide an adequate remedy to protect our interests fully. Enforcing a claim that a party illegally disclosed or misappropriated a trade secret can be difficult, expensive, and time-consuming, and the outcome is unpredictable. In addition, trade secrets may be independently developed by others in a manner that could prevent legal recourse by us. If any of our confidential or proprietary information, such as our trade secrets, were to be disclosed or misappropriated, or if any such information was independently developed by a competitor, our competitive position could be harmed.

In addition, courts outside the United States are sometimes less willing to protect trade secrets. If we choose to go to court to stop a third party from using any of our trade secrets, we may incur substantial costs. These lawsuits may consume our time and other resources even if we are successful. Although we take steps to protect our proprietary information and trade secrets, including through contractual means with our employees and consultants, third parties may independently develop substantially equivalent proprietary information and techniques or otherwise gain access to our trade secrets or disclose our technology. Thus, we may not be able to meaningfully protect our trade secrets. It is our policy to require our employees, consultants, outside scientific collaborators, sponsored researchers and other advisors to execute confidentiality agreements upon the commencement of employment or consulting relationships with us. These agreements provide that all confidential information concerning our business or financial affairs developed or made known to the individual or entity during the course of the party's relationship with us is to be kept confidential and not disclosed to third parties except in specific circumstances. In the case of employees, the agreements provide that all inventions conceived by the individual, and which are related to our current or planned business or research and development or made during normal working hours, on our premises or using our equipment or proprietary information, are our exclusive property. In addition, we take other appropriate precautions, such as physical and technological security measures, to guard against misappropriation of our proprietary technology by third parties. We have also adopted policies and conduct training that provides guidance on our expectations, and our advice for best practices, in protecting our trade secrets.

Third-party claims of intellectual property infringement may prevent or delay our product discovery and development efforts.

Our commercial success depends in part on our ability to develop, manufacture, market and sell our product candidates and use our proprietary technologies without infringing the proprietary rights of third parties. There is a substantial amount of litigation involving patents and other intellectual property rights in the biotechnology and pharmaceutical industries, as well as administrative proceedings for challenging patents, including interference, derivation, *inter partes* review, post grant review, and reexamination proceedings before the USPTO or oppositions and other comparable proceedings in foreign jurisdictions. We may be exposed to, or threatened with, future litigation by third parties having patent or other intellectual property rights alleging that our product candidates and/or proprietary technologies infringe their intellectual property rights. Numerous U.S. and foreign issued patents and pending patent applications, which are owned by third parties, exist in the fields in which we are developing our product candidates. There may be third-party patents or patent applications with claims to materials, formulations, methods of manufacture or methods for treatment related to our product candidates and programs. As the biotechnology and pharmaceutical industries expand and more patents are issued, the risk increases that our product candidates may give rise to claims of infringement of the patent rights of others. Moreover, it is not always clear to industry participants, including us, which patents cover various types of drugs, products or their methods of use or manufacture. Thus,

because of the large number of patents issued and patent applications filed in our fields, there may be a risk that third parties may allege they have patent rights encompassing our product candidates, technologies or methods. We are aware of issued patents, in the United States and abroad, relating to methods of treating DMD. If any such patent were to be asserted against us, we believe that we have defenses against any such action, including that these patents would not be infringed by our product candidates and/or that these patents are not valid. However, if these patents were asserted against us and our defenses to such an action were unsuccessful, unless we obtain a license to these patents, which may not be available on commercially reasonable terms, or at all, we could be liable for damages and precluded from commercializing rinvatercept in certain indications, which could have a material adverse effect on our business, financial condition, cash flows or results of operations.

If a third-party claims that we infringe its intellectual property rights, we may face a number of issues, including, but not limited to:

- infringement and other intellectual property claims which, regardless of merit, may be expensive and time-consuming to litigate and may divert our management's attention from our core business;
- substantial damages for infringement, which we may have to pay if a court decides that the product candidate or technology at issue infringes on or violates the third party's rights, and, if the court finds that the infringement was willful, we could be ordered to pay treble damages and the patent owner's attorneys' fees;
- a court prohibiting us from developing, manufacturing, marketing or selling our product candidates, or from using our proprietary technologies, unless the third party licenses its product rights to us, which it is not required to do;
- if a license is available from a third party, we may have to pay substantial royalties, upfront fees and other amounts, and/or grant cross-licenses to intellectual property rights for our products, if any; and
- redesigning our product candidates or processes so they do not infringe, which may not be possible or may require substantial monetary expenditures and time.

Some of our competitors may be able to sustain the costs of complex patent litigation more effectively than we can because they have substantially greater resources. In addition, any uncertainties resulting from the initiation and continuation of any litigation could have a material adverse effect on our ability to raise the funds necessary to continue our operations or could otherwise have a material adverse effect on our business, results of operations, financial condition and prospects.

Third parties may assert that we are employing their proprietary technology without authorization. Generally, conducting clinical trials and other development activities in the United States is protected under the Safe Harbor exemption as set forth in 35 U.S.C. § 271. If and when rinvatercept, elritercept or another one of our product candidates is approved by the FDA, that certain third party may then seek to enforce its patent by filing a patent infringement lawsuit against us. While we do not believe that any claims of such patent that could otherwise materially adversely affect commercialization of our product candidates, if approved, are valid and enforceable, we may be incorrect in this belief, or we may not be able to prove it in a litigation. In this regard, patents issued in the United States by law enjoy a presumption of validity that can be rebutted only with evidence that is "clear and convincing," a heightened standard of proof. There may be third-party patents of which we are currently unaware with claims to materials, formulations, methods of manufacture or methods for treatment related to the use or manufacture of our product candidates. Because patent applications can take many years to issue, there may be currently pending patent applications which may later result in issued patents that our product candidates may infringe. In addition, third parties may obtain patents in the future and claim that use of our technologies infringes upon these patents. If any third-party patents were held by a court of competent jurisdiction to cover the manufacturing process of our product candidates, constructs or molecules used in or formed during the manufacturing process, or any final product itself, the holders of any such patents may be able to block our ability to commercialize the product candidate unless we obtained a license under the applicable patents, or until such patents expire or they are finally determined to be held invalid or unenforceable. Similarly, if any third-party patent were held by a court of competent jurisdiction to cover aspects of our formulations, processes for manufacture or methods of use, the holders of any such patent may be able to block our ability to develop and commercialize the product candidate unless we obtained a license or until such patent expires or is finally determined to be held invalid or unenforceable. In either case, such a license may not be available on commercially reasonable terms or at all. If we are unable to obtain a necessary license to a third-party patent on commercially reasonable terms, or at all, our ability to commercialize our product candidates may be impaired or delayed, which could in turn significantly harm our business. Even if we obtain a license, it may be non-exclusive, thereby giving our competitors access to the same technologies licensed to us. In addition, if the breadth or strength of protection provided by our patents and patent applications is threatened, it could dissuade companies from collaborating with us to license, develop or commercialize current or future product candidates.

Parties making claims against us may seek and obtain injunctive or other equitable relief, which could effectively block our ability to further develop and commercialize our product candidates. Defense of these claims, regardless of their merit, would

involve substantial litigation expense and would be a substantial diversion of employee resources from our business. In the event of a successful claim of infringement against us, we may have to pay substantial damages, including treble damages and attorneys' fees for willful infringement, obtain one or more licenses from third parties, pay royalties or redesign our infringing products, which may be impossible or require substantial time and monetary expenditure. We cannot predict whether any such license would be available at all or whether it would be available on commercially reasonable terms. Even if such a license is available, it may be non-exclusive, which could result in our competitors gaining access to the same intellectual property. Furthermore, even in the absence of litigation, we may need to obtain licenses from third parties to advance our research or allow commercialization of our product candidates. We may fail to obtain any of these licenses at a reasonable cost or on reasonable terms, if at all. In that event, we would be unable to further develop and commercialize our product candidates, which could harm our business significantly.

Lastly, we may need to indemnify our customers and distributors against claims relating to the infringement of intellectual property rights of third parties related to our product candidates, including rinvatercept and elritercept. Third parties may assert infringement claims against our customers or distributors. These claims may require us to initiate or defend protracted and costly litigation on behalf of our customers or distributors, regardless of the merits of these claims. If any of these claims succeed, we may be forced to pay damages on behalf of our customers, suppliers or distributors, or may be required to obtain licenses for the product candidates or services they use. If we cannot obtain all necessary licenses on commercially reasonable terms, our customers may be forced to stop using our products, if approved, or services.

Third parties may assert that our employees or consultants have wrongfully used or disclosed confidential information or misappropriated trade secrets.

As is common in the biotechnology and pharmaceutical industries, we employ individuals who were previously employed at universities or other biopharmaceutical or pharmaceutical companies, including our competitors or potential competitors. Although no claims against us are currently pending, and although we try to ensure that our employees and consultants do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that we or our employees, consultants or independent contractors have inadvertently or otherwise used or disclosed intellectual property, including trade secrets or other proprietary information, of a former employer or other third parties. Litigation may be necessary to defend against these claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel. Even if we are successful in defending against such claims, litigation or other legal proceedings relating to intellectual property claims may cause us to incur significant expenses, and could distract our technical and management personnel from their normal responsibilities. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments, and, if securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our common stock. This type of litigation or proceeding could substantially increase our operating losses and reduce our resources available for development activities. We may not have sufficient financial or other resources to adequately conduct such litigation or proceedings. Some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their substantially greater financial resources. Uncertainties resulting from the initiation and continuation of patent litigation or other intellectual property related proceedings could adversely affect our ability to compete in the marketplace.

We may not be successful in obtaining or maintaining necessary rights to develop any future product candidates on acceptable terms.

Because our programs may involve additional product candidates that may require the use of proprietary rights held by third parties, the growth of our business may depend in part on our ability to acquire, in-license or use these proprietary rights.

Our product candidates may also require specific formulations to work effectively and efficiently and these rights may be held by others. We may develop products containing our compounds and pre-existing pharmaceutical compounds. We may be required by the FDA or comparable foreign regulatory authorities to provide a companion diagnostic test or tests with our product candidates. These diagnostic test or tests may be covered by intellectual property rights held by others. We may be unable to acquire or in-license any compositions, methods of use, processes or other third-party intellectual property rights from third parties that we identify as necessary or important to our business operations. We may fail to obtain any of these licenses at a reasonable cost or on reasonable terms, if at all, which would harm our business. We may need to cease use of the compositions or methods covered by such third-party intellectual property rights, and may need to seek to develop alternative approaches that do not infringe on such intellectual property rights which may entail additional costs and development delays, even if we were able to develop such alternatives, which may not be feasible. Even if we are able to

obtain a license, it may be non-exclusive, thereby giving our competitors access to the same technologies licensed to us. In that event, we may be required to expend significant time and resources to develop or license replacement technology.

Additionally, we sometimes collaborate with academic institutions to accelerate our nonclinical research or development under written agreements with these institutions. In certain cases, these institutions provide us with an option to negotiate a license to any of the institution's rights in technology resulting from the collaboration. Regardless of such option, we may be unable to negotiate a license within the specified timeframe or under terms that are acceptable to us. If we are unable to do so, the institution may offer the intellectual property rights to others, potentially blocking our ability to pursue our program. If we are unable to successfully obtain rights to required third-party intellectual property or to maintain the existing intellectual property rights we have, we may have to abandon development of such program and our business and financial condition could suffer.

The licensing and acquisition of third-party intellectual property rights is a competitive area, and companies, which may be more established, or have greater resources than we do, may also be pursuing strategies to license or acquire third-party intellectual property rights that we may consider necessary or attractive in order to commercialize our product candidates. More established companies may have a competitive advantage over us due to their size, cash resources and greater clinical development and commercialization capabilities. There can be no assurance that we will be able to successfully complete such negotiations and ultimately acquire the rights to the intellectual property surrounding the additional product candidates that we may seek to acquire.

We may be involved in lawsuits to protect or enforce our patents or the patents of our licensors, which could be expensive, time-consuming and unsuccessful.

Competitors may infringe our patents or the patents of our licensors. To counter infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time-consuming. In addition, in an infringement proceeding, a court may decide that one or more of our patents is not valid or is unenforceable, or may refuse to stop the other party from using the technology at issue on the grounds that our patents do not cover the technology in question. An adverse result in any litigation or defense proceedings could put one or more of our patents at risk of being invalidated, held unenforceable, or interpreted narrowly and could put our patent applications at risk of not issuing. Defense of these claims, regardless of their merit, would involve substantial litigation expense and would be a substantial diversion of employee resources from our business.

We may choose to challenge the patentability of claims in a third party's U.S. patent by requesting that the USPTO review the patent claims in an *ex-parte* re-exam, *inter partes* review or post-grant review proceedings. These proceedings are expensive and may consume our time or other resources. We may choose to challenge a third party's patent in patent opposition proceedings in the foreign patent offices. The costs of these opposition proceedings could be substantial, and may consume our time or other resources. If we fail to obtain a favorable result at the USPTO or other patent office then we may be exposed to litigation by a third party alleging that the patent may be infringed by our product candidates or proprietary technologies.

In addition, because some patent applications in the United States may be maintained in secrecy until the patents are issued, patent applications in the United States and many foreign jurisdictions are typically not published until 18 months after filing, and publications in the scientific literature often lag behind actual discoveries, we cannot be certain that others have not filed patent applications for technology covered by our owned and in-licensed issued patents or our pending applications, or that we or, if applicable, a licensor were the first to invent the technology. Our competitors may have filed, and may in the future file, patent applications covering our products, if approved, or technology similar to ours. Any such patent application may have priority over our owned and in-licensed patent applications or patents, which could require us to obtain rights to issued patents covering such technologies. If another party has filed a U.S. patent application on inventions similar to those owned by or in-licensed to us, we or, in the case of in-licensed technology, the licensor may have to participate in an interference proceeding declared by the USPTO to determine priority of invention in the United States. If we or one of our licensors is a party to an interference proceeding involving a U.S. patent application on inventions owned by or in-licensed to us, we may incur substantial costs, divert management's time and expend other resources, even if we are successful.

Interference proceedings provoked by third parties or brought by the USPTO may be necessary to determine the priority of inventions with respect to our patents or patent applications or those of our licensors. An unfavorable outcome could result in a loss of our current patent rights and could require us to cease using the related technology or to attempt to license rights to it from the prevailing party. Our business could be harmed if the prevailing party does not offer us a license on commercially reasonable terms or at all. Litigation or interference proceedings may result in a decision adverse to our interests and, even if we are successful, may result in substantial costs and distract our management and other employees. We may not be able to

prevent, alone or with our licensors, misappropriation of our trade secrets or confidential information, particularly in countries where the laws may not protect those rights as fully as in the United States.

Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our common stock.

Obtaining and maintaining our patent protection depends on compliance with various procedural, document submission, fee payment and other requirements imposed by governmental patent agencies, and our patent protection could be reduced or eliminated for non-compliance with these requirements.

Periodic maintenance fees on any issued patent are due to be paid to the USPTO and foreign patent agencies in several stages over the lifetime of the patent. The USPTO and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other provisions during the patent application process and following the issuance of a patent. While an inadvertent lapse can in many cases be cured by payment of a late fee or by other means in accordance with the applicable rules, there are situations in which noncompliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. Noncompliance events that could result in abandonment or lapse of a patent or patent application include, but are not limited to, failure to respond to official actions within prescribed time limits, non-payment of fees and failure to properly legalize and submit formal documents. In such an event, our competitors might be able to enter the market, which would have a material adverse effect on our business.

Issued patents covering our product candidates could be found invalid or unenforceable if challenged in court or the USPTO.

If we or one of our licensing partners initiate legal proceedings against a third party to enforce a patent covering one of our product candidates, the defendant could counterclaim that the patent covering our product candidate, as applicable, is invalid and/or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity and/or unenforceability are commonplace, and there are numerous grounds upon which a third-party can assert invalidity or unenforceability of a patent. Third parties may also raise similar claims before administrative bodies in the United States or abroad, even outside the context of litigation. Such mechanisms include re-examination, post grant review, and equivalent proceedings in foreign jurisdictions (e.g., opposition proceedings). Such proceedings could result in revocation or amendment to our patents in such a way that they no longer cover our product candidates. The outcome following legal assertions of invalidity and unenforceability is unpredictable. With respect to the validity question, for example, we cannot be certain that there is no invalidating prior art, of which we, our patent counsel and the patent examiner were unaware during prosecution. If a defendant were to prevail on a legal assertion of invalidity and/or unenforceability, or if we are otherwise unable to adequately protect our rights, we would lose at least part, and perhaps all, of the patent protection on our product candidates. Such a loss of patent protection could have a material adverse impact on our business and our ability to commercialize or license our technology and product candidates.

Moreover, the patents included in our patent portfolio may expire before, or soon after, our first product achieves marketing approval in the United States or foreign jurisdictions. For example, the patents related to novel ALK2 inhibitors in the patent family that we license from The General Hospital Corporation are expected to expire in April 2038, without taking into account any possible patent term adjustments or extensions. Upon the expiration of our current or future owned or licensed patents, we may lose the right to exclude others from practicing these inventions. The expiration of these patents could also have a similar material adverse effect on our business, results of operations, financial condition and prospects. We own pending patent applications covering our proprietary technologies or our product candidates that if issued as patents are expected to expire from 2037 through 2047, without taking into account any possible patent term adjustments or extensions. However, we cannot be assured that the USPTO or relevant foreign patent offices will grant any of these patent applications.

Changes in patent law in the U.S. and in ex-U.S. jurisdictions could diminish the value of patents in general, thereby impairing our ability to protect our products, if approved.

As is the case with other biopharmaceutical companies, our success is heavily dependent on intellectual property, particularly patents. Obtaining and enforcing patents in the biopharmaceutical industry involve both technological and legal complexity,

and is therefore costly, time-consuming and inherently uncertain. Changes in either the patent laws or interpretation of the patent laws in the United States or in ex-U.S. jurisdictions could increase the uncertainties and costs surrounding the prosecution of patent applications and the enforcement or defense of issued patents. In addition, the United States has recently enacted and is currently implementing wide-ranging patent reform legislation. Recent U.S. Supreme Court rulings have narrowed the scope of patent protection available in certain circumstances and weakened the rights of patent owners in certain situations. In addition to increasing uncertainty with regard to our ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents, once obtained. Depending on decisions by the U.S. Congress, the federal courts, and the USPTO, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce our existing patents and patents that we might obtain in the future. For example, in the case *Amgen Inc. v. Sanofi,* the Federal Circuit held that a well-characterized antigen is insufficient to satisfy the written description requirement of certain claims directed to a genus of antibodies that are solely defined by function; and in the case of *Assoc. for Molecular Pathology v. Myriad Genetics, Inc.,* the U.S. Supreme Court held that certain claims to DNA molecules are not patentable. We cannot predict how these decisions or any future decisions by the courts, the U.S. Congress or the USPTO may impact the value of our patents. Similarly, any adverse changes in the patent laws of other jurisdictions could have a material adverse effect on our business and financial condition.

Some of our in-licensed intellectual property that is discovered through government-funded programs may be subject to federal regulation such as "march-in" rights, certain reporting requirements and a preference for U.S. industry. Compliance with such regulations may limit our exclusive rights, subject us to expenditure of resources with respect to reporting requirements and limit our ability to contract with foreign manufacturers.

It is possible that patent filings we may choose to in-license in the future may be subject to the Bayh-Dole Act. In particular, under the Bayh-Dole Act, the federal government retains a "nonexclusive, nontransferable, irrevocable, paid-up license" for its own benefit to inventions produced with its financial assistance. The Bayh-Dole Act also provides federal agencies with "march-in rights." March-in rights allow the government, in specified circumstances, to require the contractor or successors in title to the patent to grant a "nonexclusive, partially exclusive, or exclusive license" to a "responsible applicant or applicants." If the patent owner refuses to do so, the government may grant the license itself. Intellectual property discovered under government-funded programs are also subject to certain reporting requirements, compliance with which may require us or our licensors to expend substantial resources. Such intellectual property is also subject to a preference for U.S. industry, which may limit our ability to contract with foreign product manufacturers for products covered by such intellectual property. Moreover, we sometimes collaborate with academic institutions to accelerate our nonclinical research or development. While it is our policy to avoid engaging our university partners in projects in which there is a risk that federal funds may be commingled, we cannot be sure that any co-developed intellectual property will be free from government rights pursuant to the Bayh-Dole Act. Further, we may choose to license intellectual property in the future that may be subject to government rights pursuant to the Bayh-Dole Act. If, in the future, we co-own or license in technology which is critical to our business that is developed in whole or in part with federal funds subject to the Bayh-Dole Act, our ability to enforce or otherwise exploit patents covering such technology may be adversely affected.

We have limited foreign intellectual property rights and may not be able to protect our intellectual property rights throughout the world.

We have limited intellectual property rights outside the United States. Filing, prosecuting and defending patents on product candidates in all countries throughout the world would be prohibitively expensive, and our intellectual property rights in some countries outside the United States can be less extensive than those in the United States. In addition, the laws of some foreign countries do not protect intellectual property rights to the same extent as do federal and state laws in the United States. Consequently, we may not be able to prevent third parties from practicing our inventions in all countries outside the United States, or from selling or importing products made using our inventions in and into the United States or other jurisdictions. Many countries have compulsory licensing laws under which a patent owner may be compelled to grant licenses to third parties. In addition, many countries limit the enforceability of patents against government agencies or government contractors. In these countries, the patent owner may have limited remedies, which could materially diminish the value of such patent. If we or any of our licensing partners are forced to grant a license to third parties with respect to any patents relevant to our business, our competitive position may be impaired, and our business, financial condition, results of operations and prospects may be adversely affected. Also, competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products and, further, may export otherwise infringing products to territories where we have patent protection but where enforcement is not as strong as that in the United States. These products may compete with our products, if approved, in jurisdictions where we do not have any issued patents and our patent claims or other intellectual property rights may not be effective or sufficient to prevent them from competing.

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

We may incur substantial costs as a result of litigation or other proceedings relating to patents, and we may be unable to protect our rights to our products, if approved, and technology.

If we or our licensing partners choose to go to court to stop a third party from using the inventions claimed in our owned or in-licensed patents, that third party may ask the court to rule that the patents are invalid and/or should not be enforced against that third party. These lawsuits are expensive and would consume time and other resources even if we or they, as the case may be, were successful in stopping the infringement of these patents. In addition, there is a risk that the court will decide that these patents are not valid and that we or they, as the case may be, do not have the right to stop others from using the inventions.

There is also the risk that, even if the validity of these patents is upheld, the court will refuse to stop the third party on the ground that such third party's activities do not infringe our owned or in-licensed patents. In addition, the U.S. Supreme Court has recently changed some legal principles that affect patent applications, granted patents and assessment of the eligibility or validity of these patents. As a consequence, issued patents may be found to contain invalid claims according to the newly revised eligibility and validity standards. Some of our owned or in-licensed patents may be subject to challenge and subsequent invalidation or significant narrowing of claim scope in proceedings before the USPTO, or during litigation, under the revised criteria which could also make it more difficult to obtain patents.

We, or our licensing partners, may not be able to detect infringement against our owned or in-licensed patents, as the case may be, which may be especially difficult for manufacturing processes or formulation patents. Even if we or our licensing partners detect infringement by a third party of our owned or in-licensed patents, we or our licensing partners, as the case may be, may choose not to pursue litigation against or settlement with the third party. If we, or our licensing partners, later sue such third party for patent infringement, the third party may have certain legal defenses available to it, which otherwise would not be available except for the delay between when the infringement was first detected and when the suit was brought. Such legal defenses may make it impossible for us or our licensing partners to enforce our owned or in-licensed patents, as the case may be, against such third party.

If another party questions the patentability of any of our claims in our owned or in-licensed U.S. patents, the third-party can request that the USPTO review the patent claims such as in an *inter partes* review, *ex parte* re-exam or post-grant review proceedings. These proceedings are expensive and may result in a loss of scope of some claims or a loss of the entire patent. In addition to potential USPTO review proceedings, we may become a party to patent opposition proceedings in foreign patent offices, where either our owned or in-licensed foreign patents are challenged.

In the future, we may be involved in similar proceedings challenging the patent rights of others, and the outcome of such proceedings is highly uncertain. An adverse determination in any such proceeding could reduce the scope of, or invalidate, our patent rights, allow third parties to commercialize our technology or products and compete directly with us, without payment to us, or result in our inability to manufacture or commercialize products without infringing third-party patent rights. The costs of these opposition or similar proceedings could be substantial, and may result in a loss of scope of some claims or a loss of the entire patent. An unfavorable result at the USPTO or other patent office may result in the loss of our right to exclude others from practicing one or more of our inventions in the relevant country or jurisdiction, which could have a material adverse effect on our business.

Patent terms may be inadequate to protect our competitive position on our product candidates for an adequate amount of time.

Patents have a limited lifespan. In the United States, if all maintenance fees are timely paid, the natural expiration of a patent is generally 20 years from its earliest U.S. non-provisional filing date. Various extensions such as patent term adjustments and/or extensions, may be available, but the life of a patent, and the protection it affords, is limited. Even if patents covering our product candidates are obtained, once the patent life has expired, we may be open to competition from competitive products, including biosimilars. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. As a result, our owned and licensed patent portfolio may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours.

If we do not obtain patent term extension and data exclusivity for any product candidates we may develop, our business may be materially harmed.

Depending upon the timing, duration and specifics of any FDA marketing approval of any product candidates we may develop, one or more of our U.S. patents may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Action of 1984 Hatch-Waxman Amendments. The Hatch-Waxman Amendments permit a patent extension term of up to five years as compensation for patent term lost during the FDA regulatory review process. A patent term extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval, only one patent may be extended and only those claims covering the approved drug, a method for using it, or a method for manufacturing it may be extended. However, we may not be granted an extension because of, for example, failing to exercise due diligence during the testing phase or regulatory review process, failing to apply within applicable deadlines, failing to apply prior to expiration of relevant patents, or otherwise failing to satisfy applicable requirements. Moreover, the applicable time period or the scope of patent protection afforded could be less than we request. If we are unable to obtain patent term extension or term of any such extension is less than we request, our competitors may obtain approval of competing products following our patent expiration, and our business, financial condition, results of operations, and prospects could be materially harmed. Further, for our licensed patents, we may not have the right to control prosecution, including filing with the USPTO, of a petition for patent term extension under the Hatch-Waxman Act. Thus, if one of our licensed patents is eligible for patent term extension under the Hatch-Waxman Act, we may not be able to control whether a petition to obtain a patent term extension is filed, or obtained, from the USPTO.

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

Our trademarks or trade names may be challenged, infringed, circumvented or declared generic or determined to be infringing on other marks. We may not be able to protect our rights to these trademarks and trade names or may be forced to stop using these names, which we need for name recognition by potential partners or customers in our markets of interest. At times, competitors may adopt trade names or trademarks similar to ours, thereby impeding our ability to build brand identity and possibly leading to market confusion. In addition, there could be potential trade name or trademark infringement claims brought by owners of other registered trade names or trademarks that incorporate variations of our unregistered trade names or trademarks. If we are unable to establish name recognition based on our trademarks and trade names, we may not be able to compete effectively and our business may be adversely affected.

Risks Related to Our Reliance on Third Parties

We rely, and expect to continue to rely, on third parties, including independent clinical investigators, contracted laboratories and CROs, to conduct our nonclinical studies and clinical trials. If these third parties do not successfully carry out their contractual duties or meet expected deadlines, we may not be able to obtain regulatory approval for or commercialize our product candidates and our business could be substantially harmed.

We have relied upon and plan to continue to rely upon third parties, including independent clinical investigators, contracted laboratories and third-party CROs, to conduct our nonclinical studies and clinical trials in accordance with applicable regulatory requirements and to monitor and manage data for our ongoing nonclinical and clinical programs. We rely on these parties for execution of our nonclinical studies and clinical trials, and control only certain aspects of their activities. Nevertheless, we are responsible for ensuring that each of our studies and trials is conducted in accordance with the applicable protocol, legal and regulatory requirements and scientific standards, and our reliance on these third parties does

not relieve us of our regulatory responsibilities. We and our third-party contractors and CROs are required to comply with good laboratory practices, or GLPs, as applicable, and GCP requirements, which are regulations and guidelines enforced by the FDA and comparable foreign regulatory authorities for conducting, recording and reporting the results of clinical trials to assure that data and reported results are credible, reproducible and accurate and that the rights, integrity, and confidentiality of trial participants are protected. Regulatory authorities enforce these GLPs and GCPs through periodic inspections of laboratories conducting GLP studies, trial sponsors, principal investigators and trial sites. If we, our investigators or any of our CROs or contracted laboratories fail to comply with applicable GLPs and GCPs, the clinical data generated in our clinical trials may be deemed unreliable and the FDA or comparable foreign regulatory authorities may require us to perform additional nonclinical studies or clinical trials before approving our marketing applications. We cannot assure you that upon inspection by a given regulatory authority, such regulatory authority will determine that any of our nonclinical studies or clinical trials comply with applicable GLP or GCP regulations. In addition, our clinical trials must be conducted with product, including biologic product, produced in compliance with applicable cGMP regulations. Our failure to comply with these regulations may require us to repeat nonclinical studies or clinical trials, which would delay the regulatory approval process.

Further, these laboratories, investigators and CROs are not our employees and we will not be able to control, other than by contract, the amount of resources, including time, which they devote to our product candidates and clinical trials. If independent laboratories, investigators or CROs fail to devote sufficient resources to the development of our product candidates, or if their performance is substandard, it may delay or compromise the prospects for approval and commercialization of any product candidates that we develop. In addition, the use of third-party service providers requires us to disclose our proprietary information to these parties, which could increase the risk that this information will be misappropriated.

Our CROs have the right to terminate their agreements with us in the event of an uncured material breach. In addition, some of our CROs have an ability to terminate their respective agreements with us if it can be reasonably demonstrated that the safety of the subjects participating in our clinical trials warrants such termination, if we make a general assignment for the benefit of our creditors or if we are liquidated.

Public health crises and government measures taken in response also impact our CROs, and may affect our ability to initiate and complete our nonclinical studies and clinical trials.

There is a limited number of third-party service providers that specialize or have the expertise required to achieve our business objectives. If any of our relationships with these third-party laboratories, CROs or clinical investigators terminate, we may not be able to enter into arrangements with alternative laboratories, CROs or investigators or to do so in a timely manner or on commercially reasonable terms. If laboratories, CROs or clinical investigators do not successfully carry out their contractual duties or obligations or meet expected deadlines, if they need to be replaced or if the quality or accuracy of the clinical data they obtain is compromised due to the failure to adhere to our nonclinical or clinical protocols, regulatory requirements or for other reasons, our nonclinical or clinical trials may be extended, delayed or terminated and we may not be able to obtain regulatory approval for or successfully commercialize our product candidates. As a result, our results of operations and the commercial prospects for our product candidates would be harmed, our costs could increase and our ability to generate revenues could be delayed.

Switching or adding additional laboratories or CROs (or investigators) involves additional cost and requires management time and focus. In addition, there is a natural transition period when a new laboratory or CRO commences work. As a result, delays occur, which can materially impact our ability to meet our desired clinical development timelines. Though we carefully manage our relationships with our contracted laboratories and CROs, there can be no assurance that we will not encounter similar challenges or delays in the future or that these delays or challenges will not have a material adverse impact on our business, financial condition and results of operations.

In addition, clinical investigators may serve as scientific advisors or consultants to us from time to time and may receive cash or equity compensation in connection with such services. If these relationships and any related compensation result in perceived or actual conflicts of interest, or the FDA or comparable foreign regulatory authorities conclude that the financial relationship may have affected the interpretation of the nonclinical study or clinical trial, the integrity of the data generated at the applicable nonclinical study or clinical trial site may be questioned and the utility of the nonclinical study or clinical trial itself may be jeopardized, which could result in the delay or rejection by the FDA or comparable foreign regulatory authorities. Any such delay or rejection could prevent us from commercializing our clinical-stage product candidate or any future product candidates.

We rely on third parties to supply and manufacture our product candidates, and we expect to continue to rely on third parties to manufacture our products, if approved. The development of such product candidates and the commercialization of any products, if approved, could be stopped, delayed or made less profitable if any such third party fails to provide us with sufficient quantities of product candidates or products or fails to do so at acceptable quality levels or prices or fails to maintain or achieve satisfactory regulatory compliance.

We do not currently have the infrastructure or capability internally to manufacture our product candidates for use in the conduct of our nonclinical studies and clinical trials or for commercial supply, if our products are approved. We rely on, and expect to continue to rely on, contract manufacturing organizations, or CMOs. Any replacement of our CMOs could require significant effort and expertise because there may be a limited number of qualified CMOs. This could be particularly problematic where we rely on a single-source supplier, as is currently the case for the manufacture of rinvatercept.

Reliance on third-party providers may expose us to more risk than if we were to manufacture our product candidates ourselves. We are dependent on our CMOs for the production of our product candidates in accordance with relevant regulations, such as cGMP, which includes, among other things, quality control, quality assurance and the maintenance of records and documentation. Moreover, many of the third parties with whom we contract may also have relationships with other commercial entities, including our competitors, for whom they may also be conducting product development activities that could harm our competitive position.

If we were to experience an unexpected loss of supply of or if any supplier were unable to meet our demand for any of our product candidates, we could experience delays in our research or planned clinical trials or commercialization. We could be unable to find alternative suppliers of acceptable quality, in the appropriate volumes and at an acceptable cost. Moreover, our suppliers are often subject to strict manufacturing requirements and rigorous testing requirements, which could limit or delay production. Any changes in regulatory requirements, policy and guidelines, including the imposition of additional regulatory oversight around manufacturing and testing requirements generally or with respect to our technology in particular, could also limit or delay production. The long transition periods necessary to switch manufacturers and suppliers, if necessary, could significantly delay our clinical trials and the commercialization of our products, if approved, which could materially adversely affect our business, financial condition and results of operation.

In complying with the applicable manufacturing regulations of the FDA and comparable foreign regulatory authorities, we and our third-party suppliers must spend significant time, money and effort in the areas of design and development, testing, production, record-keeping and quality control to assure that the products meet applicable specifications and other regulatory requirements. The facilities used by our contract manufacturers to manufacture our product candidates are subject to review by the FDA or comparable foreign regulatory authorities pursuant to inspections that will be conducted after we submit our NDA or BLA to the FDA, or similar applications to comparable foreign regulatory authorities. We do not control the manufacturing process of, and are completely dependent on, our contract manufacturing partners for compliance with cGMP requirements for manufacture of drug and biologic products. If our contract manufacturers cannot successfully manufacture material that conforms to our specifications and the strict regulatory requirements of the FDA or comparable foreign regulatory authorities, we will not be able to secure or maintain regulatory approval for our product candidates manufactured at these manufacturing facilities. In addition, we have limited control over the ability of our contract manufacturers to maintain adequate quality control, quality assurance and qualified personnel. Despite our efforts to audit and verify regulatory compliance, one or more of our third-party manufacturing partners may be found on regulatory inspection by the FDA or other comparable foreign regulatory authorities to be noncompliant with cGMP regulations. If the FDA or a comparable foreign regulatory authority does not approve these facilities for the manufacture of our product candidates or if any regulatory authority withdraws its approval in the future, we and they may need to find alternative manufacturing facilities, which would negatively impact the ability to develop, obtain regulatory approval for or market our product candidates, if approved. The failure of our manufacturers to comply with regulatory requirements could also result in an enforcement action against us, including the seizure of products and shutting down of production. If any of our third-party suppliers fails to comply with cGMP or other applicable manufacturing regulations, our ability to develop and commercialize the products could suffer significant interruptions. We face risks inherent in relying on a single CMO, as any disruption, such as a fire, natural hazards or vandalism at the CMO could significantly interrupt our manufacturing capability. All of our CMOs currently do not have alternative production plans in place or disaster-recovery facilities available. In case of a disruption, we will have to establish alternative manufacturing sources. This would require substantial capital on our part, which we may not be able to obtain on commercially acceptable terms or at all. Additionally, we would likely experience months of manufacturing delays as the CMO builds or locates replacement facilities and seeks and obtains necessary regulatory approvals. If this occurs, we will be unable to satisfy manufacturing needs on a timely basis, if at all.

We are dependent on our existing third-party collaborations with Takeda and Hansoh to commercialize elritercept, and if Takeda and Hansoh are not successful in commercializing elritercept in their respective licensed territories, we will lose a significant source of potential revenue.

Under the terms of the Takeda Agreement, Takeda will pay us milestone payments upon the achievement of specified development, commercial and sales milestones. In addition, if a licensed product is approved for marketing within the Takeda Territory, which includes all countries globally other than the territories of mainland China, Hong Kong and Macau, we will be entitled to receive royalty payments based on tiered increments of annual net sales in the Takeda Territory, with such percentage ranging from the low double-digits to high teens, subject to specified potential royalty reductions. However, under the terms of the Takeda Agreement, Takeda may terminate the agreement in its entirety or on a country-by-country basis for convenience and without cause on written notice of a certain period. In addition, Takeda generally has control over the further clinical development of elritercept and any other licensed compounds in the Takeda Territory. Takeda's decisions with respect to such development will affect the timing and availability of potential future payments under the Takeda Agreement, if any. If the Takeda Agreement is terminated early, or if Takeda's development activities are terminated early or suspended for an extended period of time, or are otherwise unsuccessful, our business and business prospects would be materially and adversely affected.

Additionally, under the terms of the license agreement with Hansoh, Hansoh will pay us milestone payments upon the achievement of specified development and commercial milestones. In addition, if elritercept, or a licensed product containing elritercept, is approved for marketing within the territories of mainland China, Hong Kong and Macau, which we refer to collectively as the Hansoh Territory, we will be entitled to receive royalty payments based on a tiered percentage of annual net sales in each region within the Hansoh Territory, with such percentage ranging from the low double digit to high teens, subject to specified potential royalty reductions. We are relying on Hansoh to commercialize elritercept in the Hansoh Territory, and if Hansoh is not able to commercialize elritercept in those countries, or determines not to pursue development or commercialization of elritercept in those countries, we will not receive any milestone or royalty payments under the agreement.

Our current and future collaborations are and will be important to our business. If we are unable to enter into new collaborations, or if these or our current collaborations are not successful, our business could be adversely affected.

Part of our strategy is to strategically evaluate and, as deemed appropriate, enter into additional strategic collaborations in the future when strategically attractive, including potentially with major biotechnology or pharmaceutical companies. We have limited capabilities for product development and do not yet have any capability for commercialization. Accordingly, we may enter into collaborations with other companies to provide us with important technologies and funding for our programs and technology. Other than our respective collaborations with Takeda and Hansoh for elritercept, we have no active collaborations for any of our product candidates. Our collaborations with Takeda and Hansoh and any future collaboration arrangements may not ultimately be successful, which could have a negative impact on our business, results of operations, financial condition and growth prospects. We do not maintain significant rights or control of future development and commercialization activities under our collaboration with Takeda. This could lead to potential disputes in the future over the terms of the collaboration and the respective rights of the parties, and these risks and uncertainties could be present with respect to our potential future collaborations as well.

If we fail to enter into or maintain collaborations on reasonable terms or at all, our ability to develop our existing or future research programs and product candidates could be delayed, the commercial potential of our product could change and our costs of development and commercialization could increase. Furthermore, we may find that our programs require the use of intellectual property rights held by third parties, and the growth of our business may depend in part on our ability to acquire or in-license these intellectual property rights.

Any future collaborations we enter into may pose a number of risks, including, but not limited to, the following:

- collaborators have significant discretion in determining the efforts and resources that they will apply;
- collaborators may not perform their obligations as expected;
- collaborators may not pursue development and commercialization of any product candidates that achieve regulatory approval or may elect not to continue or renew development or commercialization programs or license arrangements based on clinical trial results, changes in the collaborators' strategic focus or available funding, or external factors, such as a strategic transaction that may divert resources or create competing priorities;

- collaborators may delay clinical trials, provide insufficient funding for a clinical trial program, stop a clinical trial or abandon a product candidate, repeat or conduct new clinical trials or require a new formulation of a product candidate for clinical testing;
- collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our products, if approved, and product candidates if the collaborators believe that the competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours;
- product candidates discovered in collaboration with us may be viewed by our collaborators as competitive with their own product candidates or products, which may cause collaborators to cease to devote resources to the commercialization of our product candidates;
- collaborators may fail to comply with applicable regulatory requirements regarding the development, manufacture, distribution or marketing of a product candidate or product;
- collaborators with marketing and distribution rights to one or more of our product candidates that achieve regulatory approval may not commit sufficient resources to the marketing and distribution of such product or products;
- disagreements with collaborators, including disagreements over proprietary rights, contract interpretation or the preferred course of development, might cause delays or terminations of the research, development or commercialization of product candidates, might lead to additional responsibilities for us with respect to product candidates, or might result in litigation or arbitration, any of which would be time-consuming and expensive;
- collaborators may not properly maintain or defend our intellectual property rights or may use our proprietary information in such a way as to invite litigation that could jeopardize or invalidate our intellectual property or proprietary information or expose us to potential litigation;
- collaborators may infringe the intellectual property rights of third parties, which may expose us to litigation and potential liability;
- if a collaborator of ours is involved in a business combination, the collaborator might de-emphasize or terminate the development or commercialization of any product candidate licensed to it by us; and
- collaborations may be terminated by the collaborator, and, if terminated, we could be required to raise additional capital to pursue further development or commercialization of the applicable product candidates.

If our collaborations do not result in the successful discovery, development and commercialization of product candidates or if one of our collaborators terminates its agreement with us, we may not receive any future research funding or milestone or royalty payments under such collaboration. All of the risks relating to product development, regulatory approval and commercialization described in this Quarterly Report on Form 10-Q also apply to the activities of our therapeutic collaborators.

Additionally, if one of our collaborators terminates its agreement with us, we may find it more difficult to attract new collaborators and our perception in the business and financial communities could be adversely affected.

We face significant competition in seeking appropriate collaborative partners. Our ability to reach a definitive agreement for a collaboration will depend, among other things, upon an assessment of the collaborator's resources and expertise, the terms and conditions of the proposed collaboration and the proposed collaborator's evaluation of a number of factors. These factors may include the design or results of nonclinical studies or clinical trials, the likelihood of regulatory approval, the potential market for the subject product candidate, the costs and complexities of manufacturing and delivering such product candidate to patients, the potential of competing products, the existence of any uncertainty with respect to our ownership of technology (which can exist if there is a challenge to such ownership regardless of the merits of the challenge) and industry and market conditions generally. The collaborator may also consider alternative product candidates or technologies for similar indications that may be available to collaborate on and whether such a collaboration could be more attractive than the one with us.

We may not be able to negotiate collaborations on a timely basis, on acceptable terms, or at all. If we are unable to do so, we may have to curtail the development of the product candidate for which we are seeking to collaborate, reduce or delay its development program or one or more of our other development programs, delay its potential commercialization, reduce the scope of any sales or marketing activities or increase our expenditures and undertake development or commercialization activities at our own expense. If we elect to increase our expenditures to fund development or commercialization activities on our own, we may need to obtain additional capital, which may not be available to us on acceptable terms or at all. If we do not have sufficient funds, we may not be able to further develop product candidates or bring them to market and generate product revenue.

If we engage in future acquisitions or strategic collaborations, this may increase our capital requirements, dilute our stockholders, cause us to incur debt or assume contingent liabilities and subject us to other risks.

From time to time, we may evaluate various acquisition opportunities and strategic collaborations, including licensing or acquiring complementary products, intellectual property rights, technologies or businesses. Any potential acquisition or strategic collaboration may entail numerous risks, including:

- increased operating expenses and cash requirements;
- the assumption of additional indebtedness or contingent liabilities;
- the issuance of our equity securities;
- assimilation of operations, intellectual property and products of an acquired company, including difficulties associated with integrating new personnel;
- the diversion of our management's attention from our existing programs and initiatives in pursuing such a strategic merger or acquisition;
- retention of key employees, the loss of key personnel and uncertainties in our ability to maintain key business relationships;
- risks and uncertainties associated with the other party to such a transaction, including the prospects of that party and their existing products or product candidates and marketing approvals; and
- our inability to generate revenue from acquired technology and/or products sufficient to meet our objectives in undertaking the acquisition or even to offset the associated acquisition and maintenance costs.

In addition, if we undertake acquisitions or pursue collaborations in the future, we may issue dilutive securities, assume or incur debt obligations, incur large one-time expenses and acquire intangible assets that could result in significant future amortization expense. Moreover, we may not be able to locate suitable acquisition opportunities, and this inability could impair our ability to grow or obtain access to technology or products that may be important to the development of our business.

Risks Related to Our Employee Matters, Managing Our Growth and Other Risks Relating to Our Operations

We are highly dependent on our key personnel, including our Chief Executive Officer and Chief Scientific Officer. If we are not successful in attracting, motivating and retaining highly qualified personnel, we may not be able to successfully implement our business strategy.

Our ability to compete in the highly competitive biotechnology and pharmaceutical industries depends upon our ability to attract, motivate and retain highly qualified managerial, scientific and medical personnel. We are highly dependent on our management and particularly on the services of our scientific personnel including Jasbir Seehra, Ph.D., our Chief Executive Officer, and Lorena Lerner, Ph.D., our Chief Scientific Officer. We believe that their drug discovery and development experience and overall biopharmaceutical company management experience would be difficult to replace. Any of our executive officers could leave our employment at any time, as all of our employees are "at-will" employees. The loss of the services of our key personnel and any of our other executive officers, key employees, and scientific and medical advisors, and our inability to find suitable replacements, could result in delays in our research and development objectives and harm our business.

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

We will need to grow the size of our organization, and we may experience difficulties in managing this growth.

As of September 30, 2026, we had 87 full-time employees, including 67 employees engaged in research and development and 20 employees engaged in management or general and administrative activities. As our clinical development and commercialization plans and strategies develop, we expect we will need additional managerial, operational, sales, marketing, financial, legal and other personnel. Future growth would impose significant added responsibilities on members of management, including:

- identifying, recruiting, integrating, maintaining and motivating additional employees;
- managing our development efforts effectively, including the clinical and FDA review process for rinvatercept and any future product candidates, while complying with our contractual obligations to collaborators, contractors and other third parties; and
- improving our operational, financial and management controls, reporting systems and procedures.

Our future financial performance and our ability to commercialize rinvatercept, elritercept and any other product candidates we develop will depend, in part, on our ability to effectively manage any future growth, and our management may also have to divert a disproportionate amount of its attention away from day-to-day activities in order to devote a substantial amount of time to managing these growth activities.

We currently rely, and for the foreseeable future will continue to rely, in substantial part on certain independent organizations, advisors and consultants to provide certain services. The services include substantially all aspects of clinical trial management and manufacturing. We cannot assure you that the services of independent organizations, advisors and consultants will continue to be available to us on a timely basis when needed, or that we can find qualified replacements. In addition, if we are unable to effectively manage our outsourced activities or if the quality or accuracy of the services provided by consultants is compromised for any reason, our clinical trials may be extended, delayed or terminated, and we may not be able to obtain marketing approval of rinvatercept and our other product candidates or otherwise advance our business. We cannot assure you that we will be able to manage our existing consultants or find other competent outside contractors and consultants on economically reasonable terms, or at all.

If we are not able to effectively expand our organization by hiring qualified new employees and expanding our groups of consultants and contractors, we may not be able to successfully implement the tasks necessary to further develop and commercialize rinvatercept and our other product candidates and, accordingly, may not achieve our research, development and commercialization goals.

If our internal computer systems, or those used by our contract research organizations, or other contractors or consultants with whom we work, fail, suffer security incidents, or are or were otherwise compromised, we could experience adverse consequences, including but not limited to regulatory investigations or actions; litigation; fines and penalties; disruptions of our business operations; reputational harm; loss of revenue or profits; loss of customers or sales; and other adverse consequences.

In the ordinary course of our business, we and the third parties with whom we work, collect, receive, store, process, generate, use, transfer, disclose, make accessible, protect, secure, dispose of, transmit and share (collectively, process), proprietary, confidential and sensitive data, including personal data (such as health-related data), intellectual property and trade secrets (collectively, sensitive information).

Cyber-attacks, malicious internet-based activity, online and offline fraud, and other similar activities threaten the confidentiality, integrity, and availability of our sensitive information and information technology systems, and those of the third parties with whom we work. Such threats are prevalent and continue to rise, are increasingly difficult to detect, and come from a variety of sources, including traditional computer “hackers,” threat actors, “hacktivists,” organized criminal threat actors, personnel (such as through theft or misuse), sophisticated nation states, and nation-state-supported actors. Some actors now engage and are expected to continue to engage in cyber-attacks, including without limitation nation-state actors for geopolitical reasons and in conjunction with military conflicts and defense activities. During times of war and other major conflicts, we and the third parties with whom we work are vulnerable to a heightened risk of these attacks, including retaliatory cyber-attacks, that could materially disrupt our systems and operations, supply chain, and ability to produce, sell and distribute our goods and services. We and the third parties with whom we work are subject to a variety of evolving threats, including but not limited to social-engineering attacks (including through deep fakes, which are increasingly more difficult to identify as fake, and phishing attacks), malicious code (such as viruses and worms), malware (including as a result of advanced persistent threat intrusions), denial-of-service attacks, credential stuffing attacks, credential harvesting, personnel misconduct or error, ransomware attacks, supply-chain attacks, software bugs, server malfunctions, software or hardware failures, loss of data or other information technology assets, adware, telecommunications failures, attacks enhanced or facilitated by AI, and other similar threats. It may be difficult and/or costly to detect, investigate, mitigate, contain and remediate a security incident. Our efforts to do so may not be successful. Actions taken by us or the third parties with whom we work to detect, investigate, mitigate, contain and remediate a security incident could result in outages, data losses and disruptions of our business. Threat actors may also gain access to other networks and systems after a compromise of our networks and systems. For example, threat actors may use an initial compromise of one part of our environment to gain access to other parts of our environment, or

leverage a compromise of our networks or systems to gain access to the networks or systems of third parties with whom we work, such as through phishing or supply chain attacks. In particular, severe ransomware attacks are becoming increasingly prevalent and can lead to significant interruptions in our operations, ability to provide products and services, loss of sensitive data and income, reputational harm, and diversion of funds. Extortion payments may alleviate the negative impact of a ransomware attack, but we may be unwilling or unable to make such payments due to, for example, applicable laws or regulations prohibiting such payments.

We are increasingly dependent upon information technology systems, infrastructure and data to operate our business, particularly due to our hybrid-work policies. Hybrid work has become more common and has increased risks to our information technology systems and data, as more of our employees utilize network connections, computers and devices outside our premises or network, including working at home, while in transit and in public locations. Future or past business transactions (such as acquisitions or integrations) could expose us to additional cybersecurity risks and vulnerabilities, as our systems could be negatively affected by vulnerabilities present in acquired or integrated entities' systems and technologies. Furthermore, we may discover security issues that were not found during due diligence of such acquired or integrated entities, and it may be difficult to integrate companies into our information technology environment and security program.

We use third parties to operate critical business systems to process sensitive information in a variety of contexts, including, without limitation, cloud-based infrastructure, encryption and authentication technology, employee email and other functions. We also work with third-party service providers to provide other products, services, parts or otherwise to operate our business. Our ability to monitor these third parties' information security practices is limited, and these third parties may not have adequate information security measures in place. If our third-party service providers experience a security incident or other interruption, we could experience adverse consequences. While we may be entitled to damages if our third-party service providers fail to satisfy their privacy or security-related obligations to us, any award may be insufficient to cover our damages, or we may be unable to recover such award. In addition, supply-chain attacks have increased in frequency and severity, and we cannot guarantee that third parties' infrastructure in our supply chain or our third-party partners' supply chains have not been compromised.

While we have implemented security measures designed to protect against security incidents, there can be no assurance that these measures will be effective. We take steps to detect and remediate vulnerabilities in our information systems, but we have not always been and may not in the future be able to detect and remediate all vulnerabilities on a timely basis because the threats and techniques used to exploit the vulnerability change frequently and are often sophisticated in nature. Therefore, such vulnerabilities could be exploited and result in a security incident, but may not be detected until after the security incident has occurred. Unremediated high risk or critical vulnerabilities pose material risks to our business. Further, we have at times experienced and may in the future experience delays in developing and deploying remedial measures designed to address any such identified vulnerabilities.

Any of the previously identified or similar threats have in the past and may in the future cause a security incident or other interruption that have in the past and may in the future result in unauthorized, unlawful or accidental acquisition, modification, destruction, loss, alteration, encryption, disclosure of or access to our sensitive information or our information technology systems, or those of the third parties with whom we work. A security incident or other interruption could disrupt our ability (and that of third parties with whom we work) to operate our business. We may expend significant resources or modify our business activities (including our clinical trial activities) to try to protect against security incidents. Certain data privacy and security obligations have required us to implement and maintain specific security measures or industry-standard or reasonable security measures to protect our information technology systems and sensitive information.

The costs to respond to a security incident and/or to mitigate any security vulnerabilities that we identify could be significant, our efforts to address these problems may not be successful, and these problems could result in unexpected interruptions, delays, cessation of service, negative publicity, and other harm to our business and our competitive position.

If we (or a third party with whom we work) experience a security incident or are perceived to have experienced a security incident, we may experience adverse consequences. Additionally, our sensitive information could be leaked, disclosed, or revealed as a result of or in connection with our employee's, personnel's, or vendor's use of generative artificial intelligence, or AI, technologies, resulting in adverse consequences. In each case, these consequences may include: government enforcement actions (for example, investigations, fines, penalties, audits, and inspections); additional reporting requirements and/or oversight; restrictions on processing sensitive information (including personal data); litigation (including class action claims); indemnification obligations; negative publicity; reputational harm; monetary fund diversions; diversion of management

attention; interruptions in our operations (including availability of data); financial loss; and other similar harms. Security incidents and attendant consequences may prevent or cause individuals to stop conducting business with us or negatively impact our ability to grow and operate our business. For example, the loss of nonclinical or clinical data could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. Any security incident affecting us, our current and future CROs, collaborators, contractors, consultants or other partners or our industry, whether real or perceived, could harm our reputation, erode confidence in the effectiveness of our security measures and lead to regulatory scrutiny. Likewise, we use third parties for the manufacture of our product candidates and to conduct clinical trials, and similar events relating to their computer systems could also have a material adverse effect on our business. To the extent that any disruption or security incident were to result in a loss of, or damage to, our data or applications, or inappropriate disclosure of confidential or proprietary information, we could face governmental reporting obligations, fines, incur liability and the further development and commercialization of our product candidates could be delayed.

Furthermore, applicable data privacy and security obligations may require us, or we may voluntarily choose, to notify relevant stakeholders, including affected individuals, customers, regulators, and investors, of security incidents, or to take other actions, such as providing credit monitoring and identity theft protection services. Most jurisdictions have enacted laws requiring companies to notify individuals, regulatory authorities, and others of security incidents involving certain types of data. In addition, our agreements with collaborators may require us to notify them in the event of a security incident. Such mandatory disclosures and related actions can be costly, and the disclosure or the failure to comply with such applicable requirements could lead to adverse consequences such as negative publicity, may cause our collaborators to lose confidence in the effectiveness of our security measures and require us to expend significant capital and other resources to respond to and/or alleviate problems caused by the actual or perceived security incident.

In addition, any actual or perceived security incident could result in legal claims or proceedings, regulatory investigations or actions, and other types of liability under laws that protect the privacy and security of personal information, including federal, state and foreign data protection and privacy regulations, violations of which could result in significant penalties and fines, notably in the EU, UK and United States. In addition, security incidents and other incidents of unauthorized access to our information technology systems and data can be difficult to detect and any delay in identifying such breaches or incidents may lead to increased harm and legal exposure of the type described above.

Our contracts may not contain limitations of liability, and even where they do, there can be no assurance that limitations of liability in our contracts are sufficient to protect us from liabilities, damages, or claims related to our data privacy and security obligations. We cannot be sure that our insurance coverage will be adequate or sufficient to protect us from or to mitigate liabilities arising out of our privacy and security practices, that such coverage will continue to be available on commercially reasonable terms or at all, or that such coverage will pay future claims. The successful assertion of one or more large claims against us that exceeds our available insurance coverage, or results in changes to our insurance policies (including premium increases or the imposition of large deductible or co-insurance requirements), could have an adverse effect on our business. In addition to experiencing a security incident, third parties may gather, collect or infer sensitive information about us from public sources, data brokers or other means that reveals competitively sensitive details about our organization and could be used to undermine our competitive advantage or market position.

We and the third parties with whom we work are subject to stringent and evolving U.S. and foreign laws, regulations and rules, contractual obligations, industry standards, policies and other obligations related to data privacy and security. Our (or the third parties with whom we work) actual or perceived failure to comply with such obligations could lead to government enforcement actions, including administrative, civil or criminal fines or penalties, private litigation (including class claims) and mass arbitration demands; fines and penalties; disruptions of our business operations; reputational harm; loss of revenue or profits; other liabilities and adverse publicity and could negatively affect our operating results and business. Compliance or the failure to comply with such laws and regulations could increase the costs of our products, could limit their use or adoption, and could otherwise negatively affect our operating results and business.

In the ordinary course of business, we process personal data and other sensitive information. Our data processing activities subject us to numerous data privacy and security obligations, such as various laws, regulations, guidance, industry standards, external and internal privacy and security policies, contractual requirements and other obligations relating to data privacy and security.

In the United States, numerous federal and state laws and regulations, including federal and state health information privacy laws, state data breach notification laws, and federal and state consumer protection laws (including Section 5 of the Federal Trade Commission Act) and other similar laws (e.g., wiretapping laws), that govern the collection, use, disclosure and

protection of health information and other personal information could apply to our operations or the operations of our collaborators. In addition, we obtain health information from third parties, including research institutions from which we obtain clinical trial data, that are subject to privacy and security requirements under HIPAA, as amended by HITECH, which imposes specific requirements relating to the privacy, security, and transmission of individually identifiable protected health information. Depending on the facts and circumstances, we could be subject to civil, criminal and administrative penalties and fines if we violate HIPAA.

In addition, certain state and foreign laws govern the privacy and security of health information in certain circumstances, some of which are more stringent than U.S. federal law and many of which differ from each other in significant ways and may not have the same effect, thus complicating compliance efforts.

Numerous U.S. states have enacted comprehensive privacy laws that impose certain obligations on covered businesses, including providing specific disclosures in privacy notices and affording residents with certain rights concerning their personal data. As applicable, such rights include the right to access, correct, or delete certain personal data, and to opt-out of certain data processing activities, such as targeted advertising, profiling, and automated decision-making. The exercise of these rights may impact our business and ability to provide our products and services. Certain states also impose stricter requirements for processing certain personal data, including sensitive information, such as conducting data privacy impact assessments. These state laws allow for statutory fines for noncompliance. For example, the California Consumer Privacy Act of 2018, or the CCPA, applies to personal data of consumers, business representatives, and employees who are California residents, and requires businesses to provide specific disclosures in privacy notices and honor requests of such individuals to exercise certain privacy rights. The CCPA provides for fines and allows private litigants affected by certain data breaches to recover significant statutory damages.

The CCPA and other comprehensive U.S. state privacy laws exempt some data processed in the context of clinical trials, but these developments may further complicate compliance efforts, and increase legal risk and compliance costs for us and the third parties with whom we work. Similar laws are being considered in several other states, as well as at the federal and local levels, and we expect more states to pass similar laws in the future.

Other laws and regulations also apply to our business model. We are subject to new laws governing the privacy of consumer health data. For example, Washington’s My Health My Data Act, or MHMD, broadly defines consumer health data, places restrictions on processing consumer health data (including imposing stringent requirements for consents), provides consumers certain rights with respect to their health data, and creates a private right of action to allow individuals to sue for violations of the law. Other states have passed, are considering and may adopt similar laws. Additionally, under various privacy laws and other obligations, we may be required to obtain certain consents to process personal data. For example, some of our data processing practices may be challenged under wiretapping laws, if we obtain consumer information from third parties through various methods, including cookies, chatbot and session replay providers, or via third-party marketing pixels. These practices may be subject to increased challenges by class action plaintiffs. Our inability or failure to obtain consent for these practices could result in adverse consequences, including class action litigation and mass arbitration demands.

Outside the United States, an increasing number of laws, regulations, and industry standards govern data privacy and security. For example, the EU's General Data Protection Regulation, or EU GDPR, the United Kingdom’s GDPR, or UK GDPR (collectively referred to as the GDPR), and China’s Personal Information Protection Law impose strict requirements for processing personal data. For example, under the GDPR, companies may face temporary or definitive bans on data processing and other corrective actions; fines of up to €20 million under the EU GDPR, 17.5 million pounds sterling under the UK GDPR, or in each case, 4% of annual global revenue, whichever is greater; or private litigation related to processing of personal data brought by classes of data subjects or consumer protection organizations authorized at law to represent their interests.

In the ordinary course of business, we transfer personal data from Europe and other jurisdictions to the United States or other countries. Europe and other jurisdictions have enacted laws requiring data to be localized or limiting the transfer of personal data to other countries. In particular, the European Economic Area, or the EEA, and the UK have significantly restricted the transfer of personal data to countries whose privacy laws it believes are inadequate. Other jurisdictions may adopt or have already adopted similarly stringent data localization and cross-border data transfer laws. Although there are currently various mechanisms that may be used to transfer personal data from the EEA and UK to the United States in compliance with law, such as the EU-U.S. Data Privacy Framework and the UK Extension to the EU-U.S. Data Privacy Framework, under which we have self-certified to allow for transfers from the EEA and/or UK to the United States, these mechanisms are subject to legal

challenges, and there is no assurance that we can satisfy or rely on these measures to lawfully transfer personal data to the United States. If there is no lawful manner for us to transfer personal data 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 the interruption or degradation of our operations, the need to relocate part of or all of our business or data processing activities to other jurisdictions (such as Europe) at significant expense, 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 data necessary to operate our business. Additionally, companies that transfer personal data out of the EEA and UK to other jurisdictions, particularly to the United States, are subject to increased scrutiny from regulators, individual litigants, and activist groups. Some European regulators have ordered certain companies to suspend or permanently cease certain transfers out of Europe for allegedly violating the GDPR's cross-border data transfer limitations. Regulators in the United States, such as the Department of Justice, are also increasingly scrutinizing certain personal data transfers and have proposed and may enact certain data localization requirements, for example, the Biden Administration's executive order Preventing Access to Americans' Bulk Sensitive Personal Data and United States Government-Related Data by Countries of Concern.

Additionally, the U.S. Department of Justice issued a rule entitled the Preventing Access to U.S. Sensitive Personal Data and Government-Related Data by Countries of Concern or Covered Persons, which places additional restrictions on certain data transactions involving countries of concern (e.g., China, Russia, Iran) and covered persons (i.e., individuals and entities who are designated as such by the U.S. Attorney General or considered "foreign persons" and are majority owned by, organized under the laws of, a primary resident in, or a contractor of, a covered person or country of concern, as applicable) that may impact certain business activities such as vendor engagements, sale or sharing of data, employment of certain individuals and investor agreements. Violations of the rule could lead to significant civil and criminal fines and penalties. The rule applies regardless of whether data is anonymized, key-coded, pseudonymized, de-identified or encrypted, which presents particular challenges for companies like ours and may impact our ability to enter into certain transactions or agreements.

Laws such as these give rise to an increasingly complex set of compliance obligations on us. These data protection rules continue to evolve and may result in ever-increasing regulatory and public scrutiny and escalating levels of enforcement and sanctions and increased costs of compliance. We strive to comply with these rules and obligations to the extent possible. Such compliance is a rigorous and time-consuming process.

We are also bound by contractual obligations related to data privacy and security, and our efforts to comply with such obligations may not be successful. We publish privacy policies, marketing materials, whitepapers and other statements, such as statements related to compliance with certain certifications or self-regulatory principles, concerning data privacy and security. Although we endeavor to comply with our policies and other documentation, we may at times fail to do so or may be perceived to have failed to do so. Regulators in the United States are increasingly scrutinizing these statements, and if these policies, materials, statements or documentation are found to be deficient, lacking in transparency, deceptive, unfair, misleading or misrepresentative of our practices, we may be subject to investigation, enforcement actions by regulators or other adverse consequences.

Obligations related to data privacy and security (and individuals' data privacy expectations) are quickly changing, becoming increasingly stringent and creating uncertainty. Additionally, these obligations are subject to differing applications and interpretations, which may be inconsistent or conflict among jurisdictions. Preparing for and complying with these obligations requires us to devote significant resources. These obligations may necessitate changes to our services, information technologies, systems, and practices and to those of any third parties that process personal data on our behalf. In addition, these obligations may require us to change our business model. We may at times fail (or be perceived to have failed) in our efforts to comply with our data privacy and security obligations. Moreover, despite our efforts, our personnel or third parties with whom we work may fail to comply with such obligations, which could negatively impact our business operations. If we or the third parties with whom we work fail, or are perceived to have failed, to address or comply with applicable data privacy and 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) and mass arbitration demands; additional reporting requirements and/or oversight; bans or restrictions on processing personal data; orders to destroy or not use personal data; and imprisonment of company officials. In particular, plaintiffs have become increasingly more active in bringing privacy-related claims against companies, including class claims and mass arbitration demands. Some of these claims allow for the recovery of statutory damages on a per violation basis, and, if viable, carry the potential for monumental statutory damages, depending on the volume of data and the number of violations. Any of these events could have a material adverse effect on our reputation, business, or financial condition, including but not limited to: interruptions or stoppages in our business operations (including clinical trials); inability to process personal data or to operate in certain

jurisdictions; limited ability to develop or commercialize our products; expenditure of time and resources to defend any claim or inquiry; adverse publicity; or substantial changes to our business model or operations.

Our employees and personnel use generative AI technologies to perform their work, and the disclosure and use of personal information in AI technologies is subject to various privacy laws and other privacy obligations. Governments have passed and are likely to pass additional laws regulating AI. Our use of this technology could result in additional compliance costs, regulatory investigations and actions and lawsuits. If we are unable to use AI, it could make our business less efficient and result in competitive disadvantages.

Compliance with U.S. and foreign data protection laws and regulations could require us to take on more onerous obligations in our contracts, increase our costs of legal compliance, restrict our ability to collect, use and disclose data, or in some cases, impact our or our partners' or suppliers' ability to operate in certain jurisdictions. Failure to comply with these laws and regulations could result in government investigations and/or enforcement actions (which could include civil, criminal and administrative penalties), private litigation and/or adverse publicity and could negatively affect our operating results and business. Moreover, clinical trial subjects, employees and other individuals about whom we or our potential collaborators obtain personal information, as well as the providers who share this information with us, may limit our ability to collect, use and disclose the information. Claims that we have violated individuals' privacy rights, failed to comply with data protection laws, or breached our contractual obligations, even if we are not found liable, could be expensive and time-consuming to defend and could result in adverse publicity that could harm our business.

Our employees, independent contractors, vendors, principal investigators, CROs and consultants may engage in misconduct or other improper activities, including non-compliance with regulatory standards and requirements and insider trading.

We are exposed to the risk that our employees, independent contractors, vendors, principal investigators, CROs and consultants may engage in fraudulent conduct or other illegal activity. Misconduct by these parties could include intentional, reckless and/or negligent conduct or disclosure of unauthorized activities to us that violate the regulations of the FDA and comparable foreign regulatory authorities, including those laws requiring the reporting of true, complete and accurate information to such authorities; healthcare fraud and abuse laws and regulations in the United States and abroad; or laws that require the reporting of financial information or data accurately. In particular, 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 may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Activities subject to these laws also involve the improper use of information obtained in the course of clinical trials or creating fraudulent data in our nonclinical studies or clinical trials, which could result in regulatory sanctions and cause serious harm to our reputation. It is not always possible to identify and deter misconduct by employees and other third parties, 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 governmental investigations or other actions or lawsuits stemming from a failure to comply with these laws or regulations. Additionally, we are subject to the risk that a person could allege such fraud or other misconduct, even if none occurred. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, including the imposition of significant civil, criminal and administrative penalties, damages, monetary fines, imprisonment, possible exclusion from participation in Medicare, Medicaid and other federal or comparable foreign healthcare programs, additional reporting requirements and oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws, contractual damages, reputational harm, diminished profits and future earnings, and curtailment of our operations, any of which could adversely affect our ability to operate our business, financial condition and results of operations.

Public health crises could adversely impact our business, including the timing or results of our nonclinical studies and clinical trials.

As a result of any public health crises, we may experience disruptions that could severely impact our business, nonclinical studies and clinical trials, including:

- delays in receiving approval from local regulatory authorities to initiate our planned clinical trials;
- delays or difficulties in enrolling patients in our clinical trials;
- delays or difficulties in clinical site initiation, including difficulties in recruiting clinical site investigators and clinical site staff;

- delays in clinical sites receiving the supplies and materials needed to conduct our clinical trials, including interruption in global shipping that may affect the transport of clinical trial materials;
- changes in local regulations as part of a response to public health crises, which may require us to change the ways in which our clinical trials are conducted, which may result in unexpected costs, or to discontinue the clinical trials altogether;
- diversion of healthcare resources away from the conduct of clinical trials, including the diversion of hospitals serving as our clinical trial sites and hospital staff supporting the conduct of our clinical trials;
- interruption of key clinical trial activities, such as clinical trial site monitoring, due to limitations on travel imposed or recommended by federal or state governments, employers and others, or interruption of clinical trial subject visits and study procedures, the occurrence of which could affect the integrity of clinical trial data;
- risk that participants enrolled in our clinical trials will acquire any contagious diseases while the clinical trial is ongoing, which could impact the results of the clinical trial, including by increasing the number of observed adverse events;
- interruptions in nonclinical studies due to restricted or limited operations at our research and development laboratory facility or delays in receiving the supplies and materials needed to conduct our nonclinical studies;
- delays in necessary interactions with local regulators, ethics committees and other important agencies and contractors due to limitations in employee resources or forced furlough of government employees;
- limitations in employee resources that would otherwise be focused on the conduct of our clinical trials, including because of sickness of employees or their families or the desire of employees to avoid contact with large groups of people;
- refusal of the FDA or comparable foreign regulatory authorities to accept data from clinical trials in these affected geographies; and
- interruption or delays to our sourced discovery and clinical activities.

To the extent a public health crisis adversely affects our business, financial condition and results of operations, it may also have the effect of heightening many of the other risks described in this “Risk Factors” section.

A variety of risks are associated with operating our business internationally which could materially adversely affect our business.

We have conducted and conduct certain research and development and clinical operations in Australia, New Zealand, Georgia, Europe, the United Kingdom and other foreign countries, and may also conduct certain future clinical trials outside of the United States. Additionally, while we have not taken any steps to enter into any non-U.S. markets, we may do so in the future. Accordingly, we are subject to risks related to operating in foreign countries, including:

- different standards of care in various countries that could complicate the evaluation of our product candidates;
- different United States and foreign drug import and export rules;
- reduced protection for intellectual property rights in certain countries;
- unexpected changes in tariffs, trade barriers and regulatory requirements;
- economic weakness, including inflation, or political instability in particular foreign economies and markets;
- compliance with tax, employment, immigration, and labor laws for employees living or traveling abroad;
- compliance with the FCPA and other anti-corruption and anti-bribery laws;
- foreign taxes, including withholding of payroll taxes;
- foreign currency fluctuations, which could result in increased operating expenses and reduced revenue, and other obligations incident to doing business in another country;
- workforce uncertainty in countries where labor unrest is more common than in the United States;
- production shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad;
- different payor reimbursement regimes, governmental payors or patient self-pay systems and price controls;
- potential liability resulting from development work conducted by foreign partners;
- business interruptions resulting from natural disasters, outbreaks of contagious diseases and other public health crises, or geopolitical actions, including war and terrorism, or systems failure including cybersecurity breaches; and
- compliance with evolving and expansive foreign regulatory requirements, including data privacy laws (such as the GDPR).

Additionally, in connection with the ongoing war between Russia and Ukraine, the U.S. government and EU countries have imposed enhanced export controls on certain products and sanctions on certain industry sectors and parties in Russia. The U.S. government has also indicated it will consider imposing additional sanctions and other similar measures in the near future. Although we do not currently conduct any clinical trials in Russia or Ukraine, further escalation of geopolitical tensions

could have a broader impact that expands into other markets where we do business or conduct certain research and development operations, which could adversely affect our business, our supply chain for our product candidates, our collaborators or our ability to carry out our clinical trials.

We are subject to certain U.S. and foreign anti-corruption, anti-money laundering, export control, sanctions and other trade laws and regulations. We can face serious consequences for violations.

We are conducting clinical development in certain foreign countries, and may choose to conduct additional international clinical trials in the future. The U.S. Foreign Corrupt Practices Act, or FCPA, prohibits companies and their employees and third-party intermediaries from paying, offering, promising or authorizing others to pay or offer anything of value, directly or indirectly, to any foreign official, political party or candidate for the purpose of influencing any act or decision of the foreign entity in order to assist the individual or business in obtaining or retaining business. The FCPA also obligates companies whose securities are listed in the United States to comply with certain accounting provisions requiring the company to maintain books and records that accurately and fairly reflect all transactions of the corporation, including international subsidiaries, and to devise and maintain an adequate system of internal accounting controls. The FCPA presents particular challenges in the pharmaceutical industry, because, in many countries, hospitals are owned and operated by the government, and doctors and other hospital employees are considered foreign officials. We can be held liable for the corrupt or other illegal activities of our employees, representatives, contractors, business partners and agents, even if we do not explicitly authorize or have actual knowledge of such activities. Noncompliance with the FCPA and anti-corruption laws could subject us to whistleblower complaints, investigations, sanctions, settlements, prosecution, other enforcement actions, disgorgement of profits, significant fines, damages, other civil and criminal penalties or injunctions, suspension and/or debarment from contracting with certain persons, the loss of export privileges, reputational harm, adverse media coverage and other collateral consequences. If any subpoenas or investigations are launched, or governmental or other sanctions are imposed, or if we do not prevail in any possible civil or criminal litigation, our business, results of operations and financial condition could be materially harmed.

In addition, our products, if approved, may be subject to export controls, trade sanctions laws and regulations. Governmental regulation of the import or export of our products, or our failure to obtain any required import or export authorization for our products, when applicable, could harm our international sales and adversely affect our revenue. Compliance with applicable regulatory requirements regarding the export of our products may create delays in the introduction of our products in international markets or, in some cases, prevent the export of our products to some countries altogether. Furthermore, U.S. export control laws and economic sanctions prohibit the shipment of certain products and services to countries, governments, and persons targeted by U.S. sanctions. If we fail to comply with export and import regulations and such economic sanctions, penalties could be imposed, including fines and/or denial of certain export privileges. Moreover, any new export or import restrictions, new legislation or shifting approaches in the enforcement or scope of existing regulations, or in the countries, persons, or products targeted by such regulations, could result in decreased use of our products by, or in our decreased ability to export our products to, existing or potential customers with international operations. Any decreased use of our products or limitation on our ability to export or sell our products would likely adversely affect our business.

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

We are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the handling, use, storage, treatment and disposal of hazardous materials and wastes. Our research and development activities involve the use of biological and hazardous materials and produce hazardous waste products. We generally contract with third parties for the disposal of these materials and wastes. We cannot eliminate the risk of contamination or injury from these materials, which could cause an interruption of our commercialization efforts, research and development efforts and business operations, environmental damage resulting in costly clean-up and liabilities under applicable laws and regulations governing the use, storage, handling and disposal of these materials and specified waste products. Although we believe that the safety procedures utilized by our third-party manufacturers for handling and disposing of these materials generally comply with the standards prescribed by these laws and regulations, we cannot guarantee that this is the case or eliminate the risk of accidental contamination or injury from these materials. In such an event, we may be held liable for any resulting damages and such liability could exceed our resources and state or federal or other applicable authorities may curtail our use of certain materials and/or interrupt our business operations. Furthermore, environmental laws and regulations are complex, change frequently and have tended to become more stringent. We cannot predict the impact of such changes and cannot be certain of our future compliance. In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations. These current or future laws and regulations may

impair our research, development or production efforts. Failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions.

Although we maintain workers' compensation insurance to cover us for costs and expenses we may incur due to injuries to our employees resulting from the use of hazardous materials or other work-related injuries, this insurance may not provide adequate coverage against potential liabilities. We do not carry specific biological waste or hazardous waste insurance coverage, workers compensation or property and casualty and general liability insurance policies that include coverage for damages and fines arising from biological or hazardous waste exposure or contamination.

Changes in tax laws or regulations that are applied adversely to us may have a material adverse effect on our business, cash flow, financial condition or results of operations.

Legislation enacted in 2017, informally titled the Tax Cuts and Jobs Act of 2017, or the Tax Act, enacted many significant changes to the U.S. tax laws. Future guidance from the Internal Revenue Service and other tax authorities with respect to the Tax Act may affect us, and certain aspects of the Tax Act could be repealed or modified in future legislation. In addition, in response to the COVID-19 pandemic, the Coronavirus Aid, Relief, and Economic Security, or CARES Act, was signed into law in March 2020. The CARES Act modifies certain of the changes made by the Tax Act. Changes in corporate tax rates, the realization of net deferred tax assets relating to our U.S. operations, and the deductibility of expenses under the Tax Act, as amended by the CARES Act, or future tax reform legislation could have a material impact on the value of our deferred tax assets, could result in significant one-time charges in the current or future taxable years, and could increase our future U.S. tax expense. The foregoing items, as well as any other changes in tax laws, could have a material adverse effect on our business, cash flow, financial condition or results of operations. For example, the IRA, enacted in 2022, includes provisions that will impact the U.S. federal income taxation of corporations, including imposing a minimum tax on the book income of certain large corporations and an excise tax on certain corporate stock repurchases that would be imposed on the corporation repurchasing such stock, and the OBBBA, enacted in 2025, restores the current tax deductibility of domestic research and experimental expenses, which expenses had been required under the Tax Act to be capitalized and subsequently amortized over five years. In addition, it is uncertain if and to what extent various states will conform to the Tax Act, as amended by the CARES Act, or newly enacted federal tax legislation such as the OBBBA.

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

As of December 31, 2025, we had $159.1 million of U.S. federal and $27.9 million of state and no foreign NOL carryforwards. Under the Tax Act, as modified by the CARES Act, federal NOLs incurred in taxable years beginning after December 31, 2017 can be carried forward indefinitely, but the deductibility of federal NOLs in taxable years beginning after December 31, 2020, is limited.

Our NOL and tax credit carryforwards are subject to review and possible adjustment by the U.S. and state tax authorities. In addition, under Sections 382 and 383 of the Code and corresponding provisions of state law, if a corporation undergoes an "ownership change," which is generally defined as a greater than 50% change (by value) in its equity ownership over a three-year period, the corporation's ability to use its pre-change NOL and research and development, or R&D, tax credit carryforwards to offset its post-change taxable income and tax may be limited. This could limit the amount of NOLs or R&D tax credit carryforwards that we can utilize annually to offset future taxable income or tax liabilities. Subsequent ownership changes and changes to the U.S. tax rules in respect of the utilization of NOLs and R&D tax credits carried forward may further affect the limitation in future years. In addition, at the state level, there may be periods during which the use of NOLs is suspended or otherwise limited, which could accelerate or permanently increase state taxes owed.

Through November 21, 2025, we completed a study to assess whether an ownership change has occurred or whether there have been multiple ownership changes since our formation. The results of this study indicated that we experienced ownership changes as defined by Section 382 of the Code in 2016, 2020 and 2025. These ownership changes have subjected and will continue to subject our NOL and tax credit carryforwards to an annual limitation, which will significantly restrict our ability to use them to offset our taxable income and tax in periods following the ownership changes.

Risks Related to Our Common Stock

An active, liquid and orderly trading market may not develop for our common stock and as a result it may be difficult for you to sell your shares of our common stock.

Prior to our IPO in April 2020, there was no public market for shares of our common stock. Although our common stock is currently listed on the Nasdaq Global Market, we cannot assure you that an active trading market for our shares will develop or be sustained. In the absence of an active trading market for our common stock, investors may not be able to sell their shares of common stock without depressing the market price for the common stock, or may not be able to sell the shares at all. An inactive trading market may also impair our ability to raise capital to continue to fund operations by selling shares and may impair our ability to enter into collaborations or acquire other companies or technologies using our shares as consideration.

Our quarterly operating results may fluctuate significantly or may fall below the expectations of investors or securities analysts, each of which may cause our stock price to fluctuate or decline.

We expect our operating results to be subject to quarterly fluctuations. Our net loss and other operating results will be affected by numerous factors, including:

- variations in the level of expense related to the ongoing development of our product candidates and nonclinical and clinical development programs;
- results of nonclinical studies and ongoing and future clinical trials, or the addition or termination of clinical trials or funding support by us, or current or future collaborators or licensing partners;
- our execution of any collaboration, licensing or similar arrangements, and the timing of payments we may make or receive under existing or future arrangements or the termination or modification of any such existing or future arrangements;
- any intellectual property infringement lawsuit or opposition, interference or cancellation proceeding in which we may become involved;
- additions and departures of key personnel;
- strategic decisions by us, such as acquisitions, divestitures, spin-offs, joint ventures, strategic investments or changes in business strategy;
- if any of our product candidates receives regulatory approval, the terms of such approval and market acceptance and demand for such product candidates;
- regulatory developments affecting our product candidates; and
- general economic conditions, including as a result of bank failures, as well as economic conditions specifically affecting the biopharmaceutical industry.

If our quarterly operating results fall below the expectations of investors or securities analysts, the price of our common stock could decline substantially. Furthermore, any quarterly fluctuations in our operating results may, in turn, cause the price of our common stock to fluctuate substantially. We believe that quarterly comparisons of our financial results are not necessarily meaningful and should not be relied upon as an indication of our future performance.

The market price of our common stock has been and is likely to continue to be volatile and fluctuate substantially.

The market price of our common stock has been and is likely to continue to be highly volatile and may fluctuate substantially as a result of a variety of factors, some of which are related in complex ways. The market price for our common stock may fluctuate significantly in response to numerous factors, many of which are beyond our control, including the factors listed below and other factors described in this “Risk Factors” section:

- results of nonclinical studies and clinical trials of rinvatercept, elritercept and any other product candidate we may develop or those of our competitors;
- actual or anticipated changes in estimates as to financial results, development timelines or recommendations by securities analysts;
- commencement or termination of collaboration, licensing or similar arrangements for our development programs;
- announcements by our competitors of significant acquisitions, strategic partnerships, joint ventures, collaborations or capital commitments;
- failure or discontinuation of any of our development programs;
- results of clinical trials of product candidates of our competitors;
- regulatory or legal developments in the United States and other countries;
- developments or disputes concerning patent applications, issued patents or other proprietary rights;
- the recruitment or departure of key personnel;

- the level of expenses related to the development of rinvatercept and any other product candidate we may develop;
- variations in our financial results or those of companies that are perceived to be similar to us;
- announcements or expectations of additional financing efforts by us;
- sales of our common stock by us, our insiders or other stockholders;
- recommendations and changes in estimates or recommendations by securities analysts, if any, that cover our stock;
- changes in the structure of healthcare payment systems;
- market conditions in the pharmaceutical and biotechnology sectors;
- general economic, political, and market conditions and overall fluctuations in the financial markets in the United States and abroad, such as potential future disruptions in access to bank deposits or lending commitments due to bank failures; and
- investors' general perception of us and our business.

The stock market in general, and the markets for pharmaceutical, biopharmaceutical and biotechnology stocks in particular, have experienced extreme price and volume fluctuations that have been often unrelated or disproportionate to the operating performance of the issuer. In addition, the trading prices for common stock of other pharmaceutical, biopharmaceutical and biotechnology companies have been highly volatile as a result of public health crises, geopolitical tensions, a resulting global slowdown of economic activity, increased inflation, tariffs and other adverse effects or developments. The extent to which these factors may impact our business, nonclinical studies and clinical trials will depend on future developments, which are highly uncertain and cannot be predicted with confidence.

These and other market and industry factors may cause the market price and demand for our common stock to fluctuate substantially, regardless of our actual operating performance, which may negatively affect the liquidity of our common stock.

We could be subject to securities class action litigation.

In the past, securities class action litigation has often been brought against public companies following declines in the market prices of their securities. This risk is especially relevant for biopharmaceutical companies, which have experienced significant stock price volatility in recent years. If we face such litigation, it could result in substantial costs and a diversion of management's attention and our resources, which could harm our business, operating results, financial condition and cash flows.

Future sales and issuances of our common stock or rights to purchase common stock, including pursuant to our equity incentive plans, could result in additional dilution of the percentage ownership of our stockholders and could cause our stock price to fall.

Additional capital will be needed in the future to continue our planned operations. To the extent we raise additional capital by issuing equity securities, our stockholders may experience substantial dilution. We may sell common stock, convertible securities or other equity securities in one or more transactions at prices and in a manner, we determine from time to time. If we sell common stock, convertible securities or other equity securities in more than one transaction, investors may be materially diluted by subsequent sales. These sales may also result in material dilution to our existing stockholders, and new investors could gain rights superior to our existing stockholders.

Pursuant to our 2020 Equity Incentive Plan, or 2020 Plan, our management is authorized to grant stock options and other equity-based awards to our employees, directors and consultants. The number of shares of our common stock reserved for issuance under our 2020 Plan will automatically increase on January 1 of each year, for a period of ten years, from January 1, 2021 continuing through January 1, 2030, by 4.0% of the total number of shares of our common stock outstanding on December 31 of the preceding calendar year, or a lesser number of shares as may be determined by our board of directors. If our board of directors elects to increase the number of shares available for future grant by the maximum amount each year, our stockholders may experience additional dilution, which could cause our stock price to fall.

We do not intend to pay dividends on our common stock so any returns will be limited to the value of our stock.

You should not rely on an investment in our common stock to provide dividend income. We currently anticipate that we will retain future earnings for the development, operation and expansion of our business and do not anticipate declaring or paying any cash dividends for the foreseeable future. Any return to stockholders will therefore be limited to the appreciation of their stock, which may never occur, as the only way to realize any return on their investment.

Sales of a substantial number of shares of our common stock in the public market could cause our stock price to fall.

Sales of a substantial number of shares of our common stock in the public market could occur at any time. If our existing stockholders sell, or indicate an intention to sell, substantial amounts of our common stock in the public market, the market price of our common stock could decline. We are unable to predict the effect that such sales, particularly by our directors, executive officers and significant stockholders, may have on the prevailing market price of our common stock.

We have filed registration statements on Form S-8 registering the issuance of shares of common stock subject to options, restricted stock units or other equity awards issued or reserved for future issuance under our equity incentive plans. Shares registered under these registration statements on Form S-8 are available for sale in the public market subject to vesting arrangements and exercise of options and the restrictions of Rule 144 under the Securities Act in the case of our affiliates. In addition, certain holders of shares of our common stock have rights, subject to some conditions, to require us to file registration statements covering their shares or to include their shares in registration statements that we may file for ourselves or other stockholders. If we were to register the resale of these shares, they could be freely sold in the public market. If these additional shares are sold, or if it is perceived that they will be sold, in the public market, the trading price of our common stock could decline.

If securities or industry analysts publish inaccurate or unfavorable research about our business, our stock price and trading volume could decline.

The trading market for our common stock will rely, in part, on the research and reports that industry or financial analysts publish about us or our business. We do not have any control over these analysts. If one or more of the analysts covering our business downgrade their evaluations of our stock or publish inaccurate or unfavorable research about our business, the price of our stock would likely decline. If one or more of these analysts cease to cover our stock or fail to publish reports on us regularly, we could lose visibility in the market for our stock, which, in turn, could cause our stock price and trading volume to decline.

We will incur significantly increased costs as a result of operating as a public company, and our management will be required to devote substantial time to new compliance initiatives and corporate governance practices.

As a public company, we will incur significant legal, accounting and other expenses that we did not incur as a private company. The Sarbanes-Oxley Act of 2002, as amended, or the Sarbanes-Oxley Act, the Dodd-Frank Wall Street Reform and Consumer Protection Act, the listing requirements of the Nasdaq Stock Market and other applicable securities rules and regulations impose various requirements on public companies, including establishment and maintenance of effective disclosure and financial controls and corporate governance practices. Our management and other personnel will need to devote a substantial amount of time to these compliance initiatives. Moreover, we expect these rules and regulations to substantially increase our legal and financial compliance costs and to make some activities more time-consuming and costly. For example, we expect that these rules and regulations may make it more difficult and more expensive for us to obtain director and officer liability insurance and we may be required to incur substantial costs to maintain sufficient coverage. We are evaluating these rules and regulations, and cannot predict or estimate the amount or timing of additional costs we may incur or the timing of such costs. The impact of these requirements could also make it more difficult for us to attract and retain qualified persons to serve on our board of directors, our board committees or as executive officers. The increased costs may require us to reduce costs in other areas of our business or increase the prices of our services. Moreover, these rules and regulations are often subject to varying interpretations, in many cases due to their lack of specificity, and, as a result, their application in practice may evolve over time as new guidance is provided by regulatory and governing bodies. This could result in continuing uncertainty regarding compliance matters and higher costs necessitated by ongoing revisions to disclosure and governance practices.

We are a "smaller reporting company" and, as a result of the reduced disclosure requirements applicable to smaller reporting companies, our common stock may be less attractive to investors.

We are a "smaller reporting company," as defined in the Securities Exchange Act of 1934, as amended, or the Exchange Act, and we intend to take advantage of certain exemptions from various reporting requirements that are applicable to other public companies that are not "smaller reporting companies," including reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements. We are also a non-accelerated filer and are not required to comply with the auditor attestation requirements of Section 404(b) of the Sarbanes-Oxley Act of 2002. Our internal controls

over financial reporting will not receive the level of review provided by the process relating to the auditor attestation included in annual reports of issuers that are subject to these requirements.

We cannot predict if investors will find our common stock less attractive because we may rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile. We may take advantage of these reporting exemptions until we are no longer a “smaller reporting company.” We will remain a “smaller reporting company” until (a) the aggregate market value of our outstanding common stock held by non-affiliates as of the last business day of our most recently completed second fiscal quarter is $250 million or more and we reported annual net revenues as of our most recently completed fiscal year of $100 million or more, or (b) the aggregate market value of our outstanding common stock held by non-affiliates as of the last business day of our most recently completed second fiscal quarter is $700 million or more, regardless of annual revenue.

If we fail to maintain an effective system of internal control over financial reporting, we may not be able to accurately report our financial results or prevent fraud. As a result, stockholders could lose confidence in our financial and other public reporting, which would harm our business and the trading price of our common stock.

We are required, pursuant to Section 404 of the Sarbanes-Oxley Act, to assess the effectiveness of our internal control over financial reporting annually and disclosure controls and procedures quarterly. In particular, we must perform system and process evaluation and testing of our internal control over financial reporting to allow management to report on the effectiveness of our internal control over financial reporting, as required by Section 404 of the Sarbanes-Oxley Act.
When we become an accelerated filer or large accelerated filer, our independent registered public accounting firm
will be required to attest to the effectiveness of our internal control over financial reporting. The rules governing the standards that must be met for management to assess our internal control over financial reporting are complex and require significant documentation, testing and possible remediation. To comply with the requirements of being a reporting company under the Exchange Act, we will need to implement additional financial and management controls, reporting systems and procedures and hire additional accounting and finance staff.

We cannot assure you that there will not be material weaknesses or significant deficiencies in our internal control over financial reporting in the future. Any failure to maintain internal control over financial reporting could severely inhibit our ability to accurately report our financial condition, results of operations or cash flows. If we are unable to conclude that our internal control over financial reporting is effective, or if our independent registered public accounting firm determines we have a material weakness or significant deficiency in our internal control over financial reporting, investors may lose confidence in the accuracy and completeness of our financial reports, the market price of our common stock could decline, and we could be subject to sanctions or investigations by the Nasdaq Stock Market, the SEC or other regulatory authorities. Failure to remedy any material weakness in our internal control over financial reporting, or to implement or maintain other effective control systems required of public companies, could also restrict our future access to the capital markets.

Our disclosure controls and procedures may not prevent or detect all errors or acts of fraud.

We are subject to the periodic reporting requirements of the Exchange Act. We designed our disclosure controls and procedures to reasonably assure that information we must disclose in reports we file or submit under the Exchange Act is accumulated and communicated to management, and recorded, processed, summarized and reported within the time periods specified in the rules and forms of the SEC. We believe that any disclosure controls and procedures or internal controls and procedures, no matter how well-conceived and operated, can provide only reasonable, not absolute, assurance that the objectives of the control system are met.

These inherent limitations include the realities that judgments in decision-making can be faulty, and that breakdowns can occur because of simple error or mistake. For example, our directors or executive officers could inadvertently fail to disclose a new relationship or arrangement causing us to fail to make a required related party transaction disclosure. Additionally, controls can be circumvented by the individual acts of some persons, by collusion of two or more people or by an unauthorized override of the controls. Accordingly, because of the inherent limitations in our control system, misstatements due to error or fraud may occur and not be detected.

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

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

- establish a classified board of directors such that not all members of the board are elected at one time;
- allow the authorized number of our directors to be changed only by resolution of our board of directors;
- limit the manner in which stockholders can remove directors from the board;
- establish advance notice requirements for stockholder proposals that can be acted on at stockholder meetings and nominations to our board of directors;
- require that stockholder actions must be effected at a duly called stockholder meeting and prohibit actions by our stockholders by written consent;
- prohibit our stockholders from calling a special meeting of our stockholders;
- authorize our board of directors to issue preferred stock without stockholder approval, which could be used to institute a stockholder rights plan, or so-called "poison pill," that would work to dilute the stock ownership of a potential hostile acquirer, effectively preventing acquisitions that have not been approved by our board of directors; and
- require the approval of the holders of at least 66 2/3% of the votes that all our stockholders would be entitled to cast to amend or repeal certain provisions of our charter or bylaws.

For example, in April 2025, our board of directors adopted a stockholder rights plan that would cause substantial dilution to, and substantially increase the costs paid by, a stockholder who attempted to acquire us on terms not approved by our board of directors. This stockholder rights plan expired pursuant to its terms in April 2026.

Moreover, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporation Law, which prohibits a person who owns 15% or more of our outstanding voting stock from merging or combining with us for a period of three years after the date of the transaction in which the person acquired 15% or more of our outstanding voting stock, unless the merger or combination is approved in a prescribed manner. These provisions could discourage potential acquisition proposals and could delay or prevent a change in control transaction. They could also have the effect of discouraging others from making third-party tender offers for our common stock, including transactions that may be in your best interests. These provisions may also prevent changes in our management or limit the price that investors are willing to pay for our stock.

Our amended and restated certificate of incorporation designates the Court of Chancery of the State of Delaware as the exclusive forum for substantially all disputes between us and our stockholders, which could limit our stockholders' ability to obtain a favorable judicial forum for disputes with us or our directors, officers or employees.

Our amended and restated certificate of incorporation provides that the Court of Chancery of the State of Delaware (or, if and only if the Court of Chancery of the State of Delaware lacks subject matter jurisdiction, any state court located within the State of Delaware or, if and only if all such state courts lack subject matter jurisdiction, the federal district court for the District of Delaware) will be the exclusive forum for the following types of actions or proceedings under Delaware statutory or common law:

- any derivative action or proceeding brought on our behalf;
- any action or proceeding asserting a claim of breach of a fiduciary duty owed by any of our current or former directors, officers or other employees to us or our stockholders;
- any action or proceeding asserting a claim against us or any of our current or former directors, officers or other employees, arising out of or pursuant to any provision of the Delaware General Corporation Law, our certificate of incorporation or our bylaws;
- any action or proceeding to interpret, apply, enforce or determine the validity of our certificate of incorporation or our bylaws; and
- any action asserting a claim against us or any of our directors, officers or other employees governed by the internal affairs doctrine.

This provision would not apply to suits brought to enforce a duty or liability created by the Exchange Act or any other claim for which the U.S. federal courts have exclusive jurisdiction.

In addition, our amended and restated certificate of incorporation provides that the federal district courts of the United States will be the exclusive forum for resolving any complaint asserting a cause of action arising under the Securities Act, unless we consent in writing to the selection of an alternative forum.

These exclusive-forum provisions may limit a stockholder's ability to bring a claim in a judicial forum that it finds favorable for disputes with us or our directors, officers or other employees, which may discourage these types of lawsuits. Furthermore, the enforceability of similar choice of forum provisions in other companies' certificates of incorporation has been challenged in legal proceedings, and it is possible that a court could find these types of provisions to be inapplicable or unenforceable. For example, the Court of Chancery of the State of Delaware recently determined that a provision stating that U.S. federal district courts are the exclusive forum for resolving any complaint asserting a cause of action arising under the Securities Act is not enforceable. However, on March 18, 2020, this decision was ultimately overturned by the Delaware Supreme Court. If a court were to find the exclusive-forum provision contained in our amended and restated certificate of incorporation to be inapplicable or unenforceable in an action, we may incur additional costs associated with resolving such action in other jurisdictions, which could harm our business.

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

(a) Recent Sales of Unregistered Securities

None.

(b) Use of Proceeds

None.

(c) Issuer Purchases of Equity Securities

On September 8, 2026, our board of directors authorized a stock repurchase program to repurchase up to $5.0 million of our common stock from time to time prior to February 28, 2027 (the "September 2026 Repurchase Program"). We may repurchase shares from time to time through open market repurchases, privately negotiated transactions, or other means, including through Rule 10b5-1 trading plans. The actual timing, number and value of shares repurchased in the future will be determined by the Company in its discretion and will depend on a number of factors, including price, trading volume, market conditions and other general business conditions. Under the September 2026 Repurchase Program, as of September 30, 2026, we had repurchased 146,342 shares of our common stock for $1.6 million and we had $3.4 million remaining in share repurchase capacity as of September 30, 2026.

The table below provides information with respect to repurchases of shares of our common stock for each of the three months in the quarter ended September 30, 2026.

Period	Total Number of Shares Purchased (1)	Average Price Paid per Share(2)	Total Number of Shares Purchased as Part of Publicly Announced Plans or Programs	Approximate Dollar Value of Shares That May Yet Be Purchased Under the Plans or Programs
July 1-31, 2026	—	$ —	—	$ —
August 1-31, 2026	—	$ —	—	$ —
September 1-30, 2026	146,342	$ 11.27	146,342	$ 3,350,663
Total	146,342		146,342	

(1) All transactions are reported on a trade date basis.
(2) Average price per share excludes broker commissions.

ITEM 3. DEFAULTS UPON SENIOR SECURITIES

None.

ITEM 4. MINE SAFETY DISCLOSURES

Not applicable.

ITEM 5. OTHER INFORMATION

Rule 10b5-1 Trading Arrangements

During the three months ended September 30, 2026, the following directors and officers of the Company adopted a “Rule 10b5-1 trading arrangement” as that term is defined in Item 408(a) of Regulation S-K, all of which were entered into during an open trading window in accordance with the Company’s Insider Trading Policy and all of which were intended to satisfy Rule 10b5-1(c):

- On August 6, 2026, Esther Cho, our Chief Legal Officer, adopted a Rule 10b5-1 trading plan that provides for the sale of up to 7,000 shares of our common stock. The plan will expire on November 12, 2027, subject to early termination for certain specified events set forth in the plan.
- On August 6, 2026, Nima Farzan, a member of our board of directors, adopted a Rule 10b5-1 trading plan that provides for the sale of up to 8,821 shares of our common stock to be acquired upon the exercise of stock options or settlement of restricted stock units, or RSUs. The plan will expire on June 30, 2027, subject to early termination for certain specified events set forth in the plan.
- On August 6, 2026, Julius Knowles, a member of our board of directors, adopted a Rule 10b5-1 trading plan that provides for the sale of up to 3,571 shares of our common stock to be acquired upon the settlement of RSUs. The plan will expire on June 30, 2027, subject to early termination for certain specified events set forth in the plan.
- On August 6, 2026, Anne Prener, a member of our board of directors, adopted a Rule 10b5-1 trading plan that provides for the sale of up to 3,444 shares of our common stock to be acquired upon the settlement of RSUs. The plan will expire on November 30, 2027, subject to early termination for certain specified events set forth in the plan.

ITEM 6. EXHIBITS

Exhibit Number	Description	Incorporated by Reference			
		Schedule Form	File Number	Exhibit	Filing Date
3.1	Amended and Restated Certificate of Incorporation of the Registrant.	8-K	001-39264	3.1	April 13, 2020
3.2	Amended and Restated Bylaws of the Registrant.	8-K	001-39264	3.1	March 6, 2025
31.1*	Certification of Principal Executive Officer 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.				
31.2*	Certification of Principal Financial Officer 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.				
32.1+	Certification of Principal Executive Officer and Principal Financial Officer Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.				
101.INS	Inline XBRL Instance Document - the instance document does not appear in the Interactive Data File because its XBRL tags are embedded within the Inline XBRL document				
101.SCH*	Inline XBRL Taxonomy Extension Schema Document				
101.CAL*	Inline XBRL Taxonomy Extension Calculation Linkbase Document				
101.DEF*	Inline XBRL Taxonomy Extension Definition Linkbase Document				
101.LAB*	Inline XBRL Taxonomy Extension Label Linkbase Document				
101.PRE*	Inline XBRL Taxonomy Extension Presentation Linkbase Document				
104*	Cover Page formatted as Inline XBRL and included within the Exhibit 101 attachments				

* Filed herewith.

** Certain exhibits omitted pursuant to Item 601(b)(2) of Regulation S-K. The Registrant will furnish supplementally a copy of any omitted exhibit to the SEC upon request. The Registrant may request confidential treatment pursuant to Rule 24b-2 of the Exchange Act for any exhibits so furnished.

\+ This certification is being furnished solely to accompany this Quarterly Report on Form 10-Q pursuant to 18 U.S.C. Section 1350, and is not being filed for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, or otherwise subject to the liability of that section, nor shall it be deemed incorporated by reference into any filing of the registrant under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended, whether made before or after the date hereof, regardless of any general incorporation language in such filing.

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.

KEROS THERAPEUTICS, INC.

Date: November 5, 2026

By: /s/ Jasbir Seehra

Jasbir Seehra, Ph.D.

Chief Executive Officer
(*Principal Executive Officer*)

Date: November 5, 2026

By: /s/ Annita Tanini

Annita Tanini

Corporate Controller and Vice President, Finance
(*Interim Principal Financial Officer and Principal Accounting Officer*)

Exhibit 31.1

CERTIFICATION OF PRINCIPAL EXECUTIVE OFFICER 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, Jasbir Seehra, Ph.D., certify that:

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

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

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

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

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

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

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

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

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

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

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

Date: November 5, 2026

By: /s/ Jasbir Seehra, Ph.D.

Jasbir Seehra, Ph.D.

Chief Executive Officer

(Principal Executive Officer)

Exhibit 31.2

CERTIFICATION OF PRINCIPAL FINANCIAL OFFICER 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, Annita Tanini, certify that:

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

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

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

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

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

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

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

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

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

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

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

Date: November 5, 2026

By: /s/ Annita Tanini

Annita Tanini

Corporate Controller and Vice President, Finance

(Interim Principal Financial Officer and Principal Accounting Officer)

Exhibit 32.1

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

Pursuant to the requirement set forth in Rule 13a-14(b) of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), and Section 1350 of Chapter 63 of Title 18 of the United States Code (18 U.S.C. §1350), Jasbir Seehra, Ph.D., Chief Executive Officer of Keros Therapeutics, Inc. (the "Company") and Annita Tanini, Corporate Controller and Vice President, Finance of the Company, each hereby certifies that, to the best of his or her knowledge:

(1) The Company's Quarterly Report on Form 10-Q for the period ended September 30, 2026, to which this Certification is attached as Exhibit 32.1 (the "Quarterly Report"), fully complies with the requirements of Section 13(a) or Section 15(d) of the Exchange Act, and

(2) The information contained in the Quarterly Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

In Witness Whereof, the undersigned have set their hands hereto as of the 5th day of November, 2026.

/s/ Jasbir Seehra

Jasbir Seehra, Ph.D.

Chief Executive Officer

(Principal Executive Officer)

/s/ Annita Tanini

Annita Tanini

Corporate Controller and Vice President, Finance

(Interim Principal Financial Officer and Principal Accounting Officer)

This certification accompanies the Form 10-Q to which it relates, is not deemed filed with the Securities and Exchange Commission and is not to be incorporated by reference into any filing of Keros Therapeutics, Inc. under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended (whether made before or after the date of the Form 10-Q), irrespective of any general incorporation language contained in such filing.