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

FORM 10-Q

☒ **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 001-38501

SCHOLAR ROCK HOLDING CORPORATION
(Exact name of registrant as specified in its charter)

Delaware	82-3750435
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification No.)**
301 Binney Street, 3rd Floor Cambridge, Massachusetts	02142
(Address of principal executive offices)	**(Zip Code)**

(857) 259 3860
(Registrant's telephone number, including area code)

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

Title of each class	Trading symbol(s)	Name of each exchange on which registered
Common Stock, par value $0.001 per share	SRRK	The Nasdaq Global Select Market

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

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

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, 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 ☒

The number of outstanding shares of the Registrant's Common Stock as of November 6, 2026 was 122,004,478.

SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q ("Quarterly Report"), including the documents incorporated by reference, contains forward-looking statements within the meaning of the federal securities laws, Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended (the "Exchange Act"). We intend these forward-looking statements to be covered by the safe harbor provisions for forward-looking statements contained in the Private Securities Litigation Reform Act of 1995 and are including this statement for purposes of complying with those safe harbor provisions. All statements other than statements of historical facts contained in this Quarterly Report are forward-looking statements. In some cases, you can identify forward-looking statements by terminology such as "may," "will," "should," "expects," "intends," "plans," "anticipates," "believes," "estimates," "predicts," "potential," "continue" or the negative of these terms or other comparable terminology. Some of the risks and uncertainties that may cause our actual results, performance or achievements to differ materially from those expressed or implied by forward-looking statements include, among others, the following:

- the timing, scope or likelihood of our ability to obtain and maintain regulatory approval of apitegromab from the European Commission ("EC"), Japan's Pharmaceuticals and Medical Devices Agency ("PMDA") and other regulatory authorities;
- the ability to maintain regulatory approval of ISEMBYLD™ (apitegromab-mstn) from the U.S. Food and Drug Administration ("FDA") and successfully complete the post-marketing requirements from the FDA;
- our ability to successfully commercialize ISEMBYLD;
- our ability, through third-party manufacturers, to successfully manufacture commercial supply of ISEMBYLD;
- the rate and degree of market acceptance of ISEMBYLD;
- the size and growth potential of the markets for apitegromab, and our ability to serve those markets, either alone or in combination with others;
- the clinical utility of ISEMBYLD and its potential advantages over other therapeutic options;
- the potential benefit of orphan drug exclusivity and Orphan Medicinal Product designation for apitegromab;
- our success in identifying and executing development programs for additional formulations and indications for apitegromab;
- the success, cost and timing of clinical trials, including the progress, completion, timing of results, and actual results of our clinical trials;
- our ability, through third party manufacturers, to successfully manufacture our product candidates for clinical trials;
- the fact that top-line or interim data from our clinical studies may not be predictive of the final or more detailed results of such studies or the results of other ongoing or future studies;
- our success in identifying and executing a development program for our nonclinical product candidates, including SRK-373, SRK-256 and identifying additional product candidates from our noneclinical programs and research pipeline;
- our ability to retain our executives and highly skilled technical and managerial personnel, which could be affected due to any transition in management, or if we fail to recruit additional highly skilled personnel;
- our expectations regarding our ability to obtain and maintain intellectual property protection for our product candidates and the duration of such protection and our ability to operate our business without infringing on the intellectual property rights of others;
- our ability to obtain, generally or on terms acceptable to us, funding for our operations, including funding necessary to commercialize ISEMBYLD and complete further development of our current and future product candidates;
- our ability to establish or maintain collaborations or strategic relationships;
- our expectations relating to the potential of our proprietary platform technology;

- the impact of new laws and regulations or amendments to existing laws and regulations in the U.S. and foreign countries;
- risks associated with the impact of global economic and political developments on our business, including rising inflation and capital market disruptions, tariff policies, economic sanctions and economic slowdowns or recessions or public health pandemics;
- developments and projections relating to our competitors and our industry;
- our estimates and expectations regarding cash, cash reserves, and expense levels, future revenues, capital requirements and needs for additional financing, including our expected use of proceeds from our public offerings, and liquidity sources; and
- other risks and uncertainties, including those listed under the caption Part II, Item 1A "Risk Factors."

The risks set forth above are not exhaustive. Other sections of this report may include additional factors that could adversely affect our business and financial performance. Moreover, we operate in a very competitive and rapidly changing environment. New risk factors emerge from time to time and it is not possible for management to predict all risk factors, nor can we assess the impact of all risk factors on our business or the extent to which any factor, or combination of factors, may cause actual results to differ materially from those contained in any forward-looking statements. Given these risks and uncertainties, investors should not place undue reliance on forward-looking statements as a prediction of actual results. Investors should also refer to our most recent Annual Report on Form 10-K and our Quarterly Reports on Form 10-Q for future periods and Current Reports on Form 8-K as we file them with the United States Securities and Exchange Commission (the "SEC"), and to other materials we may furnish to the public from time to time through Current Reports on Form 8-K or otherwise, for a discussion of risks and uncertainties that may cause actual results, performance or achievements to differ materially from those expressed or implied by forward-looking statements. We expressly disclaim any responsibility to update any forward-looking statements to reflect changes in underlying assumptions or factors, new information, future events, or otherwise, and you should not rely upon these forward-looking statements after the date of this report.

We may from time to time provide estimates, projections and other information concerning our industry, the general business environment, and the markets for certain diseases, including estimates regarding the potential size of those markets and the estimated incidence and prevalence of certain medical conditions. Information that is based on estimates, forecasts, projections, market research or similar methodologies is inherently subject to uncertainties, and actual events, circumstances or numbers, including actual disease prevalence rates and market size, may differ materially from the information reflected in this Quarterly Report. Unless otherwise expressly stated, we obtained this industry data, business information, market data, prevalence information and other data from reports, research surveys, studies and similar data prepared by market research firms and other third parties, industry, medical and general publications, government data, and similar sources, in some cases applying our own assumptions and analysis that may, in the future, prove not to have been accurate.

SCHOLAR ROCK HOLDING CORPORATION
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PART I. FINANCIAL INFORMATION
Item 1. Financial Statements

SCHOLAR ROCK HOLDING CORPORATION
CONDENSED CONSOLIDATED BALANCE SHEETS

(Unaudited)
(In thousands, except share and per share data)

	September 30, 2026	December 31, 2025
Assets		
Current assets:		
Cash and cash equivalents	$ 366,269	$ 323,527
Marketable securities	47,330	44,036
Accounts receivable, net	677	—
Inventory	773	—
Prepaid expenses and other current assets	18,380	17,584
Total current assets	433,429	385,147
Property and equipment, net	984	1,705
Operating lease right-of-use asset	7,712	10,382
Restricted cash	1,400	3,807
Other long-term assets	2,447	3,231
Total assets	$ 445,972	$ 404,272
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 6,162	$ 10,303
Accrued expenses	68,141	39,566
Operating lease liability	5,821	5,550
Total current liabilities	80,124	55,419
Long-term portion of operating lease liability	960	3,656
Long-term debt	196,212	99,709
Total liabilities	277,296	158,784
Commitments and contingencies (Note 12)		
Stockholders' equity:		
Preferred stock, $0.001 par value; 10,000,000 shares authorized; no shares issued and outstanding at September 30, 2026 and December 31, 2025	—	—
Common stock, $0.001 par value; 300,000,000 shares authorized; 121,934,924 and 108,461,354 shares issued and outstanding as of September 30, 2026 and December 31, 2025, respectively	122	108
Additional paid-in capital	1,790,230	1,545,940
Accumulated other comprehensive income	1,623	94
Accumulated deficit	(1,623,299)	(1,300,654)
Total stockholders' equity	168,676	245,488
Total liabilities and stockholders' equity	$ 445,972	$ 404,272

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

SCHOLAR ROCK HOLDING CORPORATION
CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS

(Unaudited)
(In thousands, except share and per share data)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Revenue:				
Product revenue, net	$ 570	$ —	$ 570	$ —
Operating expenses:				
Cost of revenue	8	—	8	—
Research and development	54,128	50,486	164,176	161,565
Selling, general and administrative	52,495	53,064	153,364	131,184
Total operating expenses	106,631	103,550	317,548	292,749
Loss from operations	(106,061)	(103,550)	(316,978)	(292,749)
Other income (expense):				
Loss on extinguishment of debt	—	—	(3,337)	—
Interest income	4,096	2,733	11,124	10,374
Interest expense	(4,634)	(1,334)	(11,454)	(4,359)
Other expense, net	(640)	(69)	(2,000)	(240)
Total other income (expense), net	(1,178)	1,330	(5,667)	5,775
Net loss	$ (107,239)	$ (102,220)	$ (322,645)	$ (286,974)
Net loss per share, basic and diluted	$ (0.81)	$ (0.90)	$ (2.48)	$ (2.54)
Weighted average common shares outstanding, basic and diluted	132,372,289	113,729,850	130,106,052	112,763,974
Comprehensive loss:				
Net loss	$ (107,239)	$ (102,220)	$ (322,645)	$ (286,974)
Other comprehensive income (loss):				
Unrealized income (loss) on marketable securities	(7)	59	(116)	(43)
Foreign currency translation adjustments	489	—	1,645	—
Total other comprehensive income (loss)	482	59	1,529	(43)
Comprehensive loss	$ (106,757)	$ (102,161)	$ (321,116)	$ (287,017)

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

SCHOLAR ROCK HOLDING CORPORATION
CONDENSED CONSOLIDATED STATEMENTS OF STOCKHOLDERS' EQUITY

(Unaudited)
(In thousands, except share data)

	Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Income	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
Balance at December 31, 2025	**108,461,354**	**$ 108**	**$ 1,545,940**	**$ 94**	**$ (1,300,654)**	**$ 245,488**
Unrealized loss on marketable securities	—	—	—	(71)	—	(71)
Sale of common shares, net of issuance costs	2,576,299	3	111,771	—	—	111,774
Exercise of stock options	415,289	—	5,420	—	—	5,420
Issuance of common shares upon vesting of restricted stock units ("RSUs") and performance stock units ("PSUs")	846,766	1	(1)	—	—	—
Exercise of pre-funded warrants	6,804,066	7	(7)	—	—	—
Equity-based compensation expense	—	—	18,245	—	—	18,245
Foreign currency translation adjustments	—	—	—	667	—	667
Net loss	—	—	—	—	(105,510)	(105,510)
Balance at March 31, 2026	**119,103,774**	**$ 119**	**$ 1,681,368**	**$ 690**	**$ (1,406,164)**	**$ 276,013**
Unrealized loss on marketable securities	—	—	—	(38)	—	(38)
Sale of common shares, net of issuance costs	967,393	1	48,999			49,000
Exercise of stock options	932,079	1	11,405	—	—	11,406
Issuance of common shares upon RSU vesting	347,150	1	(1)	—	—	—
Exercise of common warrants	250,000	—	7,148	—	—	7,148
Equity-based compensation expense	—	—	19,662	—	—	19,662
Foreign currency translation adjustments	—	—	—	489	—	489
Net loss	—	—	—	—	(109,896)	(109,896)
Balance at June 30, 2026	**121,600,396**	**$ 122**	**$ 1,768,581**	**$ 1,141**	**$ (1,516,060)**	**$ 253,784**
Unrealized loss on marketable securities	—	—	—	(7)	—	(7)
Exercise of stock options	219,826	—	3,734	—	—	3,734
Issuance of common shares upon RSU vesting	114,702	—	—	—	—	—
Equity-based compensation expense	—	—	17,915	—	—	17,915
Foreign currency translation adjustments	—	—	—	489	—	489
Net loss	—	—	—	—	(107,239)	(107,239)
Balance at September 30, 2026	**121,934,924**	**$ 122**	**$ 1,790,230**	**$ 1,623**	**$ (1,623,299)**	**$ 168,676**

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

SCHOLAR ROCK HOLDING CORPORATION
CONDENSED CONSOLIDATED STATEMENTS OF STOCKHOLDERS' EQUITY (CONTINUED)

(Unaudited)
(In thousands, except share data)

	Common Stock		Additional Paid-in	Accumulated Other Comprehensive Income (Loss)	Accumulated	Total Stockholders'
	Shares	Amount	Capital		Deficit	Equity
Balance at December 31, 2024	**93,823,678**	**$ 94**	**$ 1,291,095**	**$ 160**	**$ (922,715)**	**$ 368,634**
Unrealized loss on marketable securities	—	—	—	(31)	—	(31)
Exercise of stock options	375,939	—	5,038	—	—	5,038
Issuance of common shares upon RSU vesting	673,790	1	(1)	—	—	—
Equity-based compensation expense	—	—	13,413	—	—	13,413
Other	—	—	2	—	—	2
Net loss	—	—	—	—	(74,723)	(74,723)
Balance at March 31, 2025	**94,873,407**	**$ 95**	**$ 1,309,547**	**$ 129**	**$ (997,438)**	**$ 312,333**
Unrealized loss on marketable securities	—	—	—	(71)	—	(71)
Exercise of stock options	275,874	—	3,262	—	—	3,262
Issuance of common shares upon RSU vesting	392,283	—	—	—	—	—
Exercise of pre-funded and common warrants	458,044	1	3,366	—	—	3,367
Equity-based compensation expense	—	—	24,433	—	—	24,433
Net loss	—	—	—	—	(110,031)	(110,031)
Balance at June 30, 2025	**95,999,608**	**$ 96**	**$ 1,340,608**	**$ 58**	**$ (1,107,469)**	**$ 233,293**
Unrealized gain on marketable securities	—	—	—	59	—	59
Exercise of stock options	308,064	—	3,874	—	—	3,874
Issuance of common shares upon RSU vesting	96,814	—	—	—	—	—
Sale of common shares, net of issuance costs	2,767,000	3	91,655	—	—	91,658
Equity-based compensation expense	—	—	18,331	—	—	18,331
Net loss	—	—	—	—	(102,220)	(102,220)
Balance at September 30, 2025	**99,171,486**	**$ 99**	**$ 1,454,468**	**$ 117**	**$ (1,209,689)**	**$ 244,995**

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

SCHOLAR ROCK HOLDING CORPORATION
CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS

(Unaudited)
(In thousands)

	Nine Months Ended September 30,	
	2026	**2025**
Cash flows from operating activities:		
Net loss	$ (322,645)	$ (286,974)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	890	1,233
Amortization of debt discount and debt issuance costs	300	325
Loss on disposal of property and equipment	3	—
Equity-based compensation	55,818	56,177
Accretion of investment securities	(689)	(3,791)
Non-cash operating lease expense	4,467	3,877
Loss on extinguishment of debt	3,337	—
Unrealized foreign currency exchange rate losses	1,776	—
Change in operating assets and liabilities:		
Accounts receivable	(677)	—
Inventory	(773)	—
Prepaid expenses and other current assets	(792)	(5,965)
Other assets	4,510	(372)
Accounts payable	(4,138)	(3,331)
Accrued expenses	28,597	18,797
Operating lease liabilities	(4,199)	(4,515)
Net cash used in operating activities	(234,215)	(224,539)
Cash flows from investing activities:		
Purchases of property and equipment	(170)	(570)
Purchases of marketable securities	(72,221)	(98,009)
Sales and maturities of marketable securities	69,500	278,298
Net cash provided by (used in) investing activities	(2,891)	179,719
Cash flows from financing activities:		
Proceeds from stock option exercises	20,560	10,945
Proceeds from pre-funded and common warrant exercises	7,148	3,367
Proceeds from sale of common shares, net	160,774	91,658
Proceeds from debt facility	197,729	49,992
Payment of debt issuance costs	(3,122)	—
Proceeds from debt refinancing	—	24,668
Payment of long-term debt	(103,058)	(25,520)
Other	—	2
Net cash provided by financing activities	280,031	155,112
Net increase (decrease) in cash, cash equivalents and restricted cash	42,925	110,292
Effect of exchange rate on cash, cash equivalents and restricted cash	(183)	—
Cash, cash equivalents and restricted cash, beginning of period	327,334	180,285
Cash, cash equivalents and restricted cash, end of period	$ 370,076	$ 290,577
Supplemental disclosure for non-cash items:		
Stock options exercised receivable	$ —	1,229
Right-of-use assets obtained in exchange for operating lease liabilities	$ 1,787	$ —
Supplemental cash flow information:		
Cash paid for interest	$ 11,230	4,038

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

SCHOLAR ROCK HOLDING CORPORATION
Notes to Condensed Consolidated Financial Statements
(Unaudited)

1. Nature of the Business

Scholar Rock Holding Corporation and its subsidiaries (collectively, the "Company") is a global biopharmaceutical company dedicated to improving the lives of children and adults with spinal muscular atrophy ("SMA") and additional rare, severe and debilitating neuromuscular diseases. As a leader in the biology of the transforming growth factor beta ("TGFβ") superfamily, the Company's novel understanding of the molecular mechanisms of growth factor activation enabled the development of a proprietary platform for the discovery and development of monoclonal antibodies that locally and selectively target the precursor, or latent, forms of growth factors. Based on the Company's innovative, proprietary, and scalable technology platform, the Company is building a world-leading anti-myostatin pipeline.

The Company has one product approved in the United States, ISEMBYLD™ (apitegromab-mstn), for the treatment of SMA. The Company's lead product candidates include a subcutaneous ("SC") formulation of apitegromab and SRK-439.

Apitegromab is a fully human monoclonal antibody that inhibits myostatin activation by selectively binding the pro- and latent forms of myostatin in skeletal muscle. Myostatin is a catabolic agent that functions as a negative regulator of muscle mass, therefore inhibition of myostatin results in increased muscle mass and strength. On September 11, 2026, the U.S. Food and Drug Administration ("FDA") approved ISEMBYLD for the treatment of SMA in adults and children two years of age and older who are currently receiving a survival motor neuron 2 ("SMN2")-targeted treatment. ISEMBYLD is the first and only muscle-targeted treatment approved for patients with SMA currently receiving SMN2-targeted therapy. The Company has initiated the U.S. commercial launch of ISEMBYLD and the Company plans to seek marketing authorizations for apitegromab for use in SMA in other jurisdictions. In October 2026, the Company resubmitted its Marketing Authorisation Application ("MAA") for apitegromab for the treatment of children and adults with SMA to the European Medicines Agency ("EMA"). In November 2026, the Company submitted a Japanese New Drug Application ("JNDA") for apitegromab for the treatment of children and adults with SMA to Japan's Pharmaceuticals and Medical Devices Agency ("PMDA").

The Company continues to evaluate ISEMBYLD in SMA with its ongoing long-term extension ONYX trial and its Phase 2 OPAL trial in SMA patients under two years of age, which was initiated in the third quarter of 2025. Additionally, the Company is evaluating apitegromab in patients with facioscapulohumeral muscular dystrophy ("FSHD") with its Phase 2 FORGE trial, which was initiated in the second quarter of 2026.

In addition to the current intravenous ("IV") formulation, the Company is developing a SC formulation of apitegromab. A Phase 1 study in healthy volunteers has been completed and demonstrated that SC apitegromab has favorable bioavailability and a comparable pharmacodynamic profile relative to IV administered apitegromab. Further development activities are ongoing, including planned EMA regulatory engagements following potential apitegromab approvals in Europe.

The Company's clinical-stage pipeline also includes SRK-439, a novel, investigational, subcutaneously administered fully human anti-pro/latent myostatin antibody that has high inhibitory potency while maintaining selectivity towards myostatin. SRK-439 is being developed for the treatment of patients with rare, severe, and debilitating neuromuscular diseases. A Phase 1 study of SRK-439 in healthy volunteers is currently underway. The company expects to have topline data by year end 2026 and plans to share data from this study in the first quarter of 2027.

Beyond these clinical-stage product candidates, the Company's early-stage pipeline includes additional nonclinical programs intended for the treatment of patients with rare, severe, and debilitating neuromuscular diseases. As the Company focuses its strategy on rare neuromuscular diseases, the Company is currently seeking partnerships for its non-neuromuscular disease programs.

The Company was formed in May 2012 and has principal offices in Cambridge, Massachusetts. Since its inception, the Company's operations have focused on research and development of monoclonal antibodies that selectively inhibit activation of growth factors for therapeutic effect, as well as establishing the Company's intellectual property portfolio, performing research and development activities and developing commercialization capabilities to support product sales, marketing and distribution activities and following the approval of ISEMBYLD in September 2026, commercializing ISEMBYLD in the U.S. The Company has primarily financed its operations through various equity financings, the exercise of stock options and warrants, as well as research and development collaboration agreements and the Company's debt facility (Note 13).

Revenue generation activities have historically been limited to two collaborations, both containing research services and the issuance of a license. Following the FDA approval of ISEMBYLD in September 2026, the Company has commenced commercial activities and begun generating product revenue in the U.S.

The Company is subject to a number of risks similar to those of other life science companies, including, but not limited to, successful commercialization of its approved product, successful discovery and development of its drug candidates including new indications, raising additional capital, development by competitors of new technological innovations, protection of proprietary technology and regulatory approval and market acceptance of the Company's product and product candidates. The Company anticipates that it will continue to incur significant operating losses for the next several years as it commercializes ISEMBYLD and continues to develop and seek regulatory approval for its new product candidates.

The Company believes that its existing cash, cash equivalents, marketable securities and cash available under the Company's debt facility as of September 30, 2026 will be sufficient to allow the Company to fund its current operations through at least a period of one year after the date these financial statements are issued.

2. Summary of Significant Accounting Policies

Summary of Significant Accounting Policies

The significant accounting policies used in preparation of the unaudited 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 Company's Annual Report on Form 10-K. With the exception of the policies disclosed below for accounts receivable, net, inventory and product revenue, net there have been no material changes to the significant accounting policies previously disclosed in the Company's Annual Report on Form 10-K for the year ended December 31, 2025.

Unaudited Interim Financial Information

The condensed consolidated financial statements of the Company included herein have been prepared pursuant to the rules and regulations of the Securities and Exchange Commission (the "SEC"). The unaudited condensed consolidated financial statements include the accounts of Scholar Rock Holding Corporation and its wholly owned subsidiaries. All intercompany transactions and balances have been eliminated in consolidation. In the opinion of management, the information furnished reflects all adjustments, all of which are of a normal and recurring nature, necessary for a fair presentation of the results for the reported interim periods. The Company considers events or transactions that occur after the balance sheet date but before the financial statements are issued to provide additional evidence relative to certain estimates or to identify matters that require additional disclosure. The results of operations for interim periods are not necessarily indicative of results to be expected for the full year or any other interim period.

Use of Estimates

The preparation of financial statements in accordance with accounting principles generally accepted in the U.S. ("GAAP") requires management to make estimates and judgments that may affect the reported amounts of assets and liabilities and related disclosures of contingent assets and liabilities at the date of the financial statements and the related reporting of revenues and expenses during the reporting period. Management bases its estimates on historical experience and on various assumptions that are believed to be reasonable under the circumstances. Actual results could differ from those estimates.

Accounts Receivable, Net

Accounts receivable, net consists of trade receivables which arise from product sales and represent amounts due from specialty pharmacy and specialty distributor customers in the U.S. (collectively, the "Customers"). The Company records trade receivables net of discounts, chargebacks, and any allowances for potential credit losses. An allowance for credit losses is determined based on the financial condition and creditworthiness of customers and the Company considers economic factors and events or trends expected to affect future collections experience, including payment history. For the three and nine months ended September 30, 2026, the Company did not record any expected credit losses related to outstanding accounts receivable.

Inventory

Inventory is comprised of raw materials, work-in-process and finished goods, and includes costs related to third-party contract manufacturing, packaging, freight-in and overhead. Inventory is stated at the lower of cost or net realizable value with cost based on the first-in-first-out method. Materials that may be used for either research and development or commercial purposes where the intended use is not yet known are classified as inventory until the material is consumed or otherwise allocated for research and development. If the material is used or otherwise allocated for research and development, it is expensed as research and development in the period that determination is made.

Prior to regulatory approval of its product candidates, the Company expenses costs associated with the manufacturing of its product candidates to research and development expense unless the Company is reasonably certain such costs have future commercial use and net realizable value. When the Company believes regulatory approval and subsequent commercialization of its product candidates is probable, and the Company also expects future economic benefit from the sales of the product candidates to be realized, the Company will then capitalize the costs of production as inventory.

The Company performs an assessment of the recoverability of capitalized inventory during each reporting period, and it writes down any excess and obsolete inventories to their estimated realizable value in the period in which the impairment is first identified. Such impairment charges, should they occur, are recorded within cost of product revenue. The determination of whether inventory costs will be realizable requires estimates by management. If actual market conditions are less favorable than projected by management, additional write-downs of inventory may be required, which would be recorded as a cost of product revenue in the statements of operations. There were no expenses recorded for excess inventory or other impairments during the three and nine months ended September 30, 2026.

Product Revenue, Net

The Company recognizes net product revenue in accordance with ASC Topic 606, *Revenue from Contracts with Customers*, or ASC 606, which outlines a five-step process for recognizing revenue from contracts with customers: (i) identify the contract with the customer, (ii) identify the performance obligations in the contract, (iii) determine the transaction price, (iv) allocate the transaction price to the separate performance obligations in the contract, and (v) recognize revenue associated with the performance obligations as they are satisfied. The Company only applies the five-step model to contracts when it is probable that the Company will collect the consideration it is entitled to in exchange for the goods or services it transfers to the customer. The Company sells ISEMBYLD™ (apitegromab-mstn) (the "Product") in the U.S. to a specialty distributor, which resells the Product to qualified healthcare providers and to specialty pharmacies, which dispense the Product for administration to patients. The Company has determined that the delivery of the Product to a Customer constitutes a single performance obligation that is satisfied at a point in time, when control of the Product transfers upon delivery to the Customer's designated facility. Net product revenue is recognized at that time, measured at the transaction price.

The trade payment terms with the Company's customers do not exceed one year and therefore the Company has elected to apply the practical expedient and no amount of consideration has been allocated as a financing component.

Net product revenue is recorded at the transaction price, which reflects adjustments for chargebacks, government and commercial rebates, product and prompt pay discounts, and distributor and specialty pharmacy service fees, co-pay assistance and product returns (collectively, "gross-to-net adjustments"). These adjustments represent variable consideration under ASC 606 and are estimated using the expected value method or most likely amount method and are recorded when revenue is recognized on the sale of the Product. Gross-to-net adjustments are presented as reductions of accounts receivable when settlement is expected to occur through a reduction of the amount paid by the Customer, and as accrued liabilities when settlement is expected to occur through a payment from the Company. The gross-to-net adjustments are estimates based upon information received from external sources, such as written or oral information obtained from customers with respect to their period-end inventory levels and sales to end-users during the period, in combination with management's informed judgments. Due to the inherent uncertainty of these estimates, the actual results in the future may vary from the Company's original estimates. The Company will adjust these estimates in the period such variances become known, which would affect net product revenue and earnings.

Each of the gross-to-net adjustments is discussed below:

Chargebacks — Chargebacks represent the estimated obligations to Customers resulting from the difference in contractual and statutory commitments to make the Product available to certain downstream purchasers, including 340B covered entities and federal agencies, at prices lower than the list price charged to Customers who directly purchase the Product from the Company. These reserves are established in the same period that the related revenue is recognized, resulting in a reduction of product revenue and accounts receivable, and consist of amounts that Customers have claimed but for which the Company has not yet issued a credit, together with the credits the Company expects to issue for Product remaining in the distribution channel at the end of each reporting period.

Government and Commercial Rebates —The Company is subject to rebate obligations under government programs, including the Medicaid Drug Rebate Program and the Medicare Part D Manufacturer Discount Program, and expects to enter into rebate arrangements with commercial payers and pharmacy benefit managers in exchange for coverage of, and formulary position for, the Product. These reserves are recorded in the same period the related revenue is recognized, resulting in a reduction of product revenue and the establishment of a current liability which is included in accrued expenses and other current liabilities. The Company's liability for these rebates consists of invoices received for claims that have not been paid or for which an invoice has not yet been received, estimates of claims for the current period, and estimated future claims for Product that has been recognized as revenue, but remains in the distribution channel.

Discounts — Certain Customer agreements provide for a contractual discount from the Company's wholesale acquisition cost that is reflected directly on the Customer's invoice; product revenue and accounts receivable are recorded as the net invoiced amount. The Company estimates prompt pay discounts based on contractual terms and expectations regarding future customer payment patterns. The adjustments are recorded in the same period the related revenue is recognized, resulting in a reduction of product revenue and accounts receivable.

Distributor and Specialty Pharmacy Service Fees — The Company pays fees to its specialty distributor and specialty pharmacy customers for distribution and other services, including a specialty distributor fee based on a contractually determined fixed percentage of sales and specialty pharmacy fees for program and account management, inventory management, enhanced nursing services, data reporting and shipping. To the extent that the Company has determined that these services are not distinct from the Customer's purchase of the Product, the adjustments are recorded in the same period the related revenue is recognized, resulting in a reduction of product revenue and the establishment of a current liability which is included in accrued expenses and other current liabilities. Where the Company has determined these services are distinct and at fair value, the fees for such services are recorded as operating expenses as the services are provided.

Copay Assistance — The Company offers a co-pay assistance program, which is intended to provide financial assistance to qualified commercially-insured patients with prescription drug co-payments required by payers. Patients whose Product is reimbursed by a federal or state health care program are not eligible for the program. The Company estimates co-pay assistance using program participation and redemption data provided by the third-party administrator of the program, including amounts expected to be redeemed on Product remaining in the distribution channel, and records the estimate as a reduction of product revenue.

Product Returns — The Company's contracts with its Customers generally provide for returns only if the Product is damaged or defective upon delivery, if there is a shipment error, or if the product is within an eligible expiry window. The Company estimates product return reserves using contractual terms, Product shelf life, contracted days-on-hand limits, available industry data and its own sales information, including its visibility into the inventory remaining in the distribution channel. These reserves are established in the same period that the related revenue is recognized, resulting in a reduction of product revenue and a refund liability, which is included in accrued expenses and other current liabilities in the Company's financial statements.

Cash, Cash Equivalents and Restricted Cash

The following table reconciles cash, cash equivalents and restricted cash per the balance sheet to the statement of cash flows (in thousands):

	As of September 30,	
	2026	2025
Cash and cash equivalents	$ 366,269	$ 286,770
Restricted cash	3,807	3,807
	$ 370,076	$ 290,577

The Company maintains restricted cash associated with a letter of credit related to its lease at 301 Binney Street in Cambridge, Massachusetts and an account associated with its corporate credit card program. The portion of the restricted cash that is associated with its letter of credit is recorded in other current assets on the Company's balance sheet and the portion associated with the collateral account is recorded as a long-term asset on the Company's balance sheet.

Recently Issued Accounting Pronouncements

In November 2024, the FASB issued ASU No. 2024-03, *Income Statement - Reporting Comprehensive Income - Expense Disaggregation Disclosures (Subtopic 220-40)*: *Disaggregation of Income Statement Expenses* ("ASU 2024-03"), and in January 2025, the FASB issued ASU No. 2025-01, *Income Statement - Reporting Comprehensive Income - Expense Disaggregation Disclosures (Subtopic 220-40): Clarifying the Effective Date*. ASU 2024-03 requires additional disclosure of the nature of expenses included in the income statement as well as disclosures about specific types of expenses included in the expense captions presented in the income statement. ASU 2024-03, as clarified by ASU 2025-01, is effective for public companies for annual periods beginning after December 15, 2026, and interim reporting periods beginning after December 15, 2027. Early adoption is permitted. The Company is still evaluating the impact on its disclosures in future years as a result of the adoption of ASU 2024-03.

3. Fair Value of Financial Assets and Liabilities

The following tables summarize the assets and liabilities measured at fair value on a recurring basis at September 30, 2026 and December 31, 2025 (in thousands):

	Fair Value Measurements at September 30, 2026			
	Total	Level 1	Level 2	Level 3
Assets:				
Cash and cash equivalents:				
Money market funds	$ 200,606	$ 200,606	$ —	$ —
U.S. treasury obligations	158,163	158,163	—	—
Marketable securities:				
U.S. treasury obligations and government agency securities	47,330	47,330	—	—
Total assets	$ 406,099	$ 406,099	$ —	$ —

	Fair Value Measurements at December 31, 2025			
	Total	Level 1	Level 2	Level 3
Assets:				
Cash and cash equivalents:				
Money market funds	$ 309,089	$ 309,089	$ —	$ —
Marketable securities:				
U.S. treasury obligations and government agency securities	44,036	44,036	—	—
Total assets	$ 353,125	$ 353,125	$ —	$ —

Level 1 assets include investments in money market funds, U.S. treasury obligations and government agency securities that are valued using quoted market prices in active markets. There were no transfers of assets between fair value measurement levels during the nine months ended September 30, 2026 and 2025.

The carrying amounts reflected in the balance sheets for accounts receivable, net, prepaid expenses and other current assets, accounts payable, and accrued expenses approximate their fair values at September 30, 2026 and December 31, 2025, due to their short-term nature.

4. Marketable Securities

The following table summarizes the Company's investments as of September 30, 2026 (in thousands):

	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Estimated Fair Value
Marketable securities available-for-sale:				
U.S. treasury obligations and government agency securities	$ 47,350	$ —	$ (20)	$ 47,330
Total available-for-sale securities	$ 47,350	$ —	$ (20)	$ 47,330

The following table summarizes the Company's investments as of December 31, 2025 (in thousands):

	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Estimated Fair Value
Marketable securities available-for-sale:				
U.S. treasury obligations and government agency securities	$ 43,940	$ 96	$ —	$ 44,036
Total available-for-sale securities	$ 43,940	$ 96	$ —	$ 44,036

The Company believes that U.S. treasury obligations and government agency securities are subject to minimal credit risk. As a result, the Company did not record any charges for credit-related impairments for its available-for-sale securities for the nine months ended September 30, 2026.

5. Prepaid Expenses and Other Current Assets

At September 30, 2026 and December 31, 2025, prepaid expenses and other current assets consist of the following (in thousands):

	As of September 30, 2026	As of December 31, 2025
Prepaid external research and development expenses	$ 7,166	$ 6,509
Prepaid software	3,054	3,905
Short -term restricted cash	2,407	—
Prepaid other	2,892	2,145
Other receivables	1,691	2,799
Prepaid compensation expense	758	950
Prepaid professional services expense	412	1,276
	$ 18,380	$ 17,584

At September 30, 2026 and December 31, 2025, other long-term assets consist of the following (in thousands):

	As of	
	September 30, 2026	December 31, 2025
Deferred financing costs related to undrawn debt	$ 1,321	$ —
Prepaid software	1,069	529
Prepaid insurance	35	39
Prepaid external research and development expenses	12	2,588
Prepaid other	10	75
	$ 2,447	$ 3,231

6. Accrued Expenses

At September 30, 2026 and December 31, 2025, accrued expenses consist of the following (in thousands):

	As of	
	September 30, 2026	December 31, 2025
Accrued external research and development expense	$ 28,695	$ 14,205
Accrued payroll and related expenses	26,717	20,114
Accrued professional services expense	11,040	3,829
Accrued other	1,689	1,418
	$ 68,141	$ 39,566

7. Product Revenues, Net, Accounts Receivable, Net and Reserves for Product Revenues

On September 11, 2026, the U.S. Food and Drug Administration ("FDA") approved ISEMBYLD for the treatment of SMA in adults and children two years of age and older who are currently receiving a survival motor neuron 2 ("SMN2")-targeted treatment.

During the three and nine months ended September 30, 2026 we recorded net product revenue of $0.6 million from sales of ISEMBYLD in the U.S.

The following table summarizes the Company's product revenues, by customer, for those customers that exceeded 10% of gross revenue for the periods indicated:

	Three and Nine Months Ended September 30,	
	2026	2025
Customer 1	50%	—%
Customer 2	25%	—%
Customer 3	16%	—%

As of September 30, 2026, the Company's accounts receivable, net were $0.7 million and related to product sales in the U.S., net of discounts and allowances. All customers in the U.S. have standard payment terms that generally require payment within 65 to 90 days. Three customers accounted for 50%, 25% and 16% of the total accounts receivable balance as of September 30, 2026.

The following tables summarize an analysis of the change in reserves for discounts and allowances for each of the periods indicated:

	Chargebacks	Rebates	Co-Pay	Other Accruals	Total
			(in thousands)		
Balance, as of December 31, 2025	$ —	$ —	$ —	$ —	$ —
Provision	60	46	37	98	241
Adjustments relating to prior periods	—	—	—	—	—
Payments/credits	—	—	—	—	—
Balance, as of September 30, 2026	$ 60	$ 46	$ 37	$ 98	$ 241

8. Inventory

The following table summarizes the components of the Company's inventory for each of the periods indicated:

	As of	
	September 30, 2026	December 31, 2025
Raw Materials	$ —	$ —
Work In Process	312	—
Finished Goods	461	—
Total inventory	$ 773	$ —

Prior to regulatory approval of ISEMBYLD, the Company expensed costs associated with the manufacturing of apitegromab to research and development expense.

There were no inventory losses for write-downs of excess and obsolete inventory for the three and nine months ended September 30, 2026.

9. Common Stock

The Company's amended and restated certificate of incorporation provides for 300,000,000 authorized shares of common stock.

The Company has had a sales agreement in place during various time periods with Jefferies LLC ("Jefferies") with respect to an at-the-market ("ATM") offering program. Under this program, the Company is able to offer and sell, from time to time at its sole discretion, shares of its common stock through Jefferies as its sales agent. In an ATM offering, exchange-listed companies incrementally sell newly issued shares into the secondary trading market through a designated broker-dealer at prevailing market prices. The current sales agreement with Jefferies, entered into in November 2022, allows for the sale of shares of common stock from time to time in "at the market" offerings through Jefferies as the Company's sales agent. The Company sold 3,543,692 shares of its common stock under the ATM program during the nine months ended September 30, 2026, generating net proceeds of $160.8 million.

The Company has issued pre-funded warrants to purchase common stock, as well as warrants to purchase common stock as part of its financing activities. Both the pre-funded warrants and warrants meet the conditions for equity classification and are recorded as a component of stockholders' equity within additional paid-in capital. The table below summarizes the pre-funded warrants issued by the Company.

	Date Issued	Number Issued	Exercise Price	Expiry Date	Number Outstanding as of September 30, 2026
Pre-funded warrants	October 2024	353,983	$ 0.0001	—	353,983
Pre-funded warrants	June 2022	25,510,205	$ 0.0001	—	10,204,082
Pre-funded warrants	November 2020	2,179,487	$ 0.0001	—	—
		28,043,675			10,558,065

During the nine months ended September 30, 2026 and 2025, 6,804,082 and 0, respectively, of the Company's pre-funded warrants were exercised.

In association with the June 2022 financing, the Company also issued 10,459,181 warrants with an exercise price of $7.35, which had an expiry date of December 31, 2025. All of the common warrants outstanding associated with the June 2022 financing were exercised prior to their expiry.

In October 2025, the Company issued 250,000 warrants with an exercise price of $28.59 for non-employee services. Warrants issued to non-employees in connection with providing services meet the conditions for equity classification and are recorded as a component of stockholders' equity within additional paid in capital. All of the warrants issued for non-employee services were exercised during the nine months ended September 30, 2026.

Shares Reserved For Future Issuance

As of September 30, 2026, the Company had common shares reserved for issuance as follows:

	As of September 30, 2026
Common shares reserved for exercise of pre-funded warrants	10,558,065
Common shares reserved for exercise of outstanding stock options, unvested RSUs and unvested PSUs under the 2017 and 2018 Plans	7,217,741
Common shares reserved for exercise of outstanding stock options, unvested RSUs and unvested PSUs under the 2022 Inducement Plan	3,906,469
Common shares reserved for future issuance under the 2018 Plan	7,075,530
Common shares reserved for future issuance under the 2022 Inducement Plan	2,212,442
Common shares reserved for future issuance under the 2018 ESPP	2,903,919
	33,874,166

10. Equity-Based Compensation

The Company recorded equity-based compensation expense related to all equity-based awards, which was allocated as follows in the condensed consolidated statements of operations and comprehensive loss for the three and nine months ended September 30, 2026 and 2025 (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Research and development expense	$ 6,368	$ 5,497	$ 20,403	$ 15,375
Selling, general and administrative expense	11,543	12,834	35,415	40,802
	$ 17,911	$ 18,331	$ 55,818	$ 56,177

During the nine months ended September 30, 2026 and 2025, the Company entered into separation agreements with certain employees. Per the separation terms, certain stock option awards and RSUs were amended and may provide for continued vesting, accelerated vesting, and extended time to exercise vested options. As a result, equity-based compensation expense during the nine months ended September 30, 2026 and 2025 includes $0.3 million and $13.0 million, respectively, related to the modification of certain equity awards.

The following table summarizes the Company's unrecognized equity-based compensation expense as of September 30, 2026:

	As of September 30, 2026	
	Unrecognized Expense (in thousands)	Weighted Average Remaining Period of Recognition (years)
RSUs	$ 105,734	2.7
PSUs	8,555	2.1
Stock options	62,742	2.3
	$ 177,031	

Restricted Stock Units

The following table summarizes the Company's RSU activity for the current year:

	Number of Units	Weighted Average Grant Date Fair Value
RSUs as of December 31, 2025	3,283,939	$ 25.50
Granted	1,668,580	$ 48.05
Vested	(1,033,618)	$ 23.06
Forfeited	(509,369)	$ 29.10
RSUs as of September 30, 2026	3,409,532	$ 36.74

The total fair value of RSUs vested during the nine months ended September 30, 2026 and 2025 was $49.0 million and $44.0 million, respectively.

Performance Stock Units

The following table summarizes the Company's PSU activity for the current year:

	Number of Units	Weighted Average Grant Date Fair Value
PSUs as of December 31, 2025	1,100,000	$ 28.63
Granted	625,809	$ 43.39
Vested	(275,000)	$ 30.94
Forfeited	(29,258)	$ 47.85
PSUs as of September 30, 2026	1,421,551	$ 34.29

The Company has granted various PSUs to certain employees. Depending on the terms of the PSUs and the outcome of the pre-established performance criteria, which may include a market condition, performance condition and/or time-based vesting, the recipient may ultimately earn the target number of PSUs granted or a specified multiple thereof at the end of the vesting period.

The total fair value of PSUs vested during the nine months ended September 30, 2026 was $12.0 million. No PSUs vested during the nine months ended September 30, 2025.

Stock Options

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

	Number of Shares	Weighted Average Exercise Price	Weighted Average Remaining Contractual Term (in years)	Aggregate Intrinsic Value (in thousands)
Outstanding as of December 31, 2025	7,845,460	$ 21.06	6.72	$ 185,525
Granted	602,980	$ 47.80		
Exercised	(1,567,194)	$ 13.12		
Cancelled	(588,119)	$ 31.98		
Outstanding as of September 30, 2026	6,293,127	$ 24.58	7.03	$ 152,296
Options exercisable as of September 30, 2026	3,718,083	$ 19.64	6.04	$ 109,129

Using the Black-Scholes option pricing model, the weighted average grant date fair value of options granted during the nine months ended September 30, 2026 and 2025 was $39.79 and $28.73, respectively.

The following weighted average assumptions were used in determining the fair value of options granted in the nine months ended September 30, 2026 and 2025:

	Nine Months Ended September 30,	
	2026	2025
Risk-free interest rate	4.02%	4.07%
Expected dividend yield	0.0%	0.0%
Expected term (in years)	6.05	6.07
Expected volatility	106.81%	108.29%

11. Leases

Operating Lease

In November 2019, the Company entered into a lease of office and laboratory space at 301 Binney Street in Cambridge, Massachusetts to be used as its new corporate headquarters (the "Lease"). The expiration date of the Lease was originally in August 2025 and included an option to extend the term by two years. The base rent under the original Lease was $6.9 million per year, subject to an annual increase of 3.5%. Variable lease payments include the Company's allocated share of costs incurred and expenditures made by the landlord in the operation and management of the building. The Lease included incentives of $14.1 million in the form of an allowance for tenant improvements related to the design and build out of the space. In connection with the Lease, the Company has secured a letter of credit for $2.3 million which renews automatically each year.

In May 2024, the Company entered into the First Amendment (the "Lease Amendment") to the Lease to extend the term for approximately two years, commencing on August 19, 2025 (the "First Extension Term") with the base rent to start at approximately $6.2 million per year, followed by a 3% annual increase. Pursuant to the Lease Amendment, the Company also has an option to extend the term of the Lease by five years, upon the expiration of the First Extension Term.

In October 2025, the Company entered into a lease of office space in Zug, Switzerland (the "2025 Switzerland Lease"). The 2025 Switzerland Lease commencement date was January 1, 2026, which is when the Company gained access to the space under the terms of the lease. The 2025 Switzerland Lease had an expiration date of December 31, 2029, with a minimum term of twelve months. The base rent under the 2025 Switzerland Lease was CHF 276,000 per year.

In July 2026, the Company entered into a new lease agreement for office space in Zug, Switzerland (the "2026 Switzerland Lease"), which replaces the 2025 Switzerland Lease. The 2026 Switzerland Lease commenced on September 1, 2026, which is when the Company gained access to the space under the terms of the lease. Upon commencement of the 2026 Switzerland Lease, the 2025 Switzerland Lease was terminated. The 2026 Switzerland Lease has an indefinite term with a minimum non-cancelable period through February 28, 2029. Thereafter, the lease may be terminated by either party upon six months' written notice. The base rent under the 2026 Switzerland Lease is CHF 621,000 per year.

Other information related to the leases are as follows (in thousands, except lease term and discount rate):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Lease cost:				
Operating lease cost	$ 1,844	$ 1,714	$ 5,461	$ 5,142
Variable lease cost	407	381	1,262	1,153
Total lease cost	$ 2,251	$ 2,095	$ 6,723	$ 6,295

	Nine Months Ended September 30,	
	2026	2025
Other information:		
Operating cash flows used for operating leases	$ 5,116	$ 5,779
Weighted average remaining lease term	1.3	1.9
Weighted average incremental borrowing rate	10.9%	13.1%

12. Commitments and Contingencies

Legal Proceedings

The Company, from time to time, may be party to litigation arising in the ordinary course of its business. Although the results of litigation and claims cannot be predicted with certainty, the Company does not believe they are party to any claim or litigation the outcome of which, if determined adversely to the Company, would individually or in the aggregate be reasonably expected to have a material adverse effect on the Company's business.

13. Debt

On October 16, 2020, the Company entered into a Loan and Security Agreement with Oxford Finance LLC ("Oxford") and Silicon Valley Bank ("SVB") for $50.0 million (the "Loan and Security Agreement"), which was amended in 2022 to increase the Company's borrowing capacity.

On February 10, 2025, the Company entered into an Amended and Restated Loan and Security Agreement with Oxford (such agreement, as amended, the "Oxford Loan Agreement"). The Oxford Loan Agreement consolidated the existing outstanding loan tranches solely with Oxford and amends and restates in its entirety that certain Loan and Security Agreement, as amended. In conjunction with the Oxford Loan Agreement, the Company was required to pay $0.9 million, which included $0.5 million to SVB for the final payment on the outstanding tranche.

On September 29, 2025, the Company received $50.0 million from the next available tranche under the Oxford Loan Agreement, bringing the total borrowings to $100.0 million.

On February 27, 2026, the Company terminated the Oxford Loan Agreement and entered into a Financing Agreement (the "Financing Agreement") with certain funds managed by Blue Owl Capital Corporation ("Blue Owl"). Concurrently with the entry into the Financing Agreement, the Company also entered into a Pledge and Security Agreement (the "Security Agreement," together with the Financing Agreement, the "2026 Loan Agreement") with Blue Owl. The Financing Agreement contains customary representations, warranties and covenants. The Company is also required to maintain a minimum unrestricted cash balance of at least 60% of the aggregate outstanding principal amount, unless certain milestones are met and maintained. The obligations under the 2026 Loan Agreement are guaranteed by certain of the Company's subsidiaries and secured by a first-priority lien on substantially all of the assets of the Company and the guarantors, subject to customary exclusions and exceptions.

The 2026 Loan Agreement provides for up to $350.0 million (each, a "Term Loan" and together, the "Term Loans"), of which $100.0 million of the initial term loan (the "Initial Term Loan") was received in February 2026. In addition, the Financing Agreement includes an uncommitted incremental term loan facility that requires the consent of Blue Owl in an aggregate principal amount not to exceed $200.0 million. Deferred financing costs related to undrawn debt are included in "Other long-term assets" in the consolidated balance sheets. The Company used the proceeds of the Initial Term Loan upon the closing under the 2026 Loan Agreement to repay all outstanding obligations, totaling $103.7 million, which included $2.0 million related to the final payment and $1.0 million related to the prepayment fee, under the Oxford Loan Agreement with Oxford and upon such repayment, terminated the Oxford Loan Agreement. The amount repaid by the Company included $100.0 million of outstanding indebtedness plus accrued and unpaid interest as of February 27, 2026 and fees. The Company recognized a loss on extinguishment of debt of $3.3 million in connection with the termination of the Oxford Loan Agreement. As a result of the termination, all credit commitments under the Oxford Loan Agreement were terminated and all security interests and guarantees executed in connection with the Oxford Loan Agreement were released.

On March 31, 2026, the Company received $100.0 million from the next available tranche under the 2026 Loan Agreement. To date, the Company has received $200.0 million of Term Loans. The additional delayed draw term loan commitment in an aggregate principal amount not to exceed $150.0 million became available on September 11, 2026, on the date of the FDA's approval for apitegromab in SMA. The additional loan will be available until the date that is the earliest of the full usage thereof, termination thereof and September 30, 2027, which may be borrowed by the Company at its sole option upon satisfying certain customary conditions, including satisfaction of the minimum cash covenant (if applicable).

The outstanding principal of the Term Loan bears interest at a rate per annum on the basis of a 360 day year equal to (a) in the case of Term Loans bearing interest based on the base rate defined in the Financing Agreement, the sum of (i) the base rate (and which base rate will not be less than 2.00%) plus (ii) 4.00% and (b) in the case of Term Loans bearing interest based on the three-month forward-looking term secured overnight financing rate as published by CME Group Benchmark Administration Limited (the "Term SOFR"), the sum of (i) three-month Term SOFR (subject to 1.00% per annum floor), plus (ii) 5.00%. Accrued interest is payable quarterly, on any date of prepayment of the Term Loans and at maturity.

The maturity date of the Term Loan is the earlier of February 26, 2032 and the date the Term Loans shall otherwise become due and payable in full under the Financing Agreement. The Company may, at its option, prepay at any time the Term Loans on any Business Day in whole or in part. In certain instances and during certain time periods, these prepayments, together with certain mandatory prepayments and payments of the Term Loans resulting from the exercise of remedies under the Financing Agreement, will be subject to customary prepayment fees.

14. Net Loss per Share

The Company calculates basic net loss per share by dividing net loss by the weighted average number of common shares outstanding. The weighted average number of common shares used in the basic and diluted net loss per share calculation includes the pre-funded warrants issued in connection with the Company's November 2020, June 2022 and October 2024 follow-on offerings as the pre-funded warrants are exercisable at any time for nominal cash consideration. The Company has generated a net loss in all periods presented, so the basic and diluted net loss per share are the same, as the inclusion of the potentially dilutive securities would be anti-dilutive.

The following table sets forth the outstanding common stock equivalents, presented based on amounts outstanding at each period end, that have been excluded from the calculation of diluted net loss per share for the periods indicated because their inclusion would have been anti-dilutive:

	As of September 30,	
	2026	2025
RSUs	3,409,532	3,535,920
PSUs	1,421,551	1,100,000
Stock options	6,293,127	8,910,982
Warrants	—	8,220,620
	11,124,210	21,767,522

15. Segment Reporting

The Company operates and manages its business on a consolidated basis as a single reportable and single operating segment for the purposes of assessing performance and making operating decisions. The Company's chief executive officer, who is the chief operating decision maker ("CODM"), reviews the Company's financial information on a consolidated basis for purposes of evaluating financial performance and allocating resources. When evaluating the Company's financial performance, the CODM regularly reviews net loss. The CODM considers net loss in making decisions on how to allocate resources. Net loss is used to monitor budget versus actual results in an effort to refine forecasts and control costs. The measure of segment assets is reported on the balance sheet as total consolidated assets.

All of the Company's material long-lived assets are held in the U.S., Ireland and Switzerland. Long-lived assets are comprised of property and equipment and operating lease right-of-use assets. As of September 30, 2026, long-lived assets based in the U.S. were $6.9 million and long-lived assets based in Ireland and Switzerland were $1.8 million. As of December 31, 2025, all of the Company's material long-lived assets were held in the U.S.

The following table presents significant expense information about the Company's operating segment (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Product revenue, net	$ 570	$ —	$ 570	$ —
Cost of revenue	8	—	8	—
Employee related expense[1]	40,961	34,982	115,932	90,404
External R&D expense - apitegromab	19,982	21,361	63,191	68,618
External R&D expense - SRK-181	17	357	62	2,259
External R&D expense - SRK-439	2,852	1,608	8,371	8,462
External R&D expense - Early research and other	1,459	1,129	4,279	4,811
External expense - G&A	16,148	19,433	48,877	43,328
Other segment items[2]	7,035	5,926	20,117	17,457
Equity-based compensation expense	17,911	18,331	55,818	56,177
Depreciation and amortization expense	258	423	890	1,233
Loss on disposal of property and equipment	-	-	3	-
Other non-operating income (expense), net	(1,178)	1,330	(5,667)	5,775
Net loss	$ (107,239)	(102,220)	$ (322,645)	$ (286,974)

(1) Excludes equity-based compensation expense.
(2) Consists of other segment expenses related to supplies, corporate and facilities expenses.

16. Subsequent Events

On November 9, 2026, the Company entered into an asset purchase agreement (the "PRV Asset Purchase Agreement") with BioNTech US Inc. (the "Buyer"), pursuant to which the Company agreed to sell its Rare Pediatric Disease Priority Review Voucher ("PRV") to Buyer for $205.0 million in cash upon the closing of the sale. The Company received the PRV in connection with the FDA approval of ISEMBYLD in September 2026. The transaction remains subject to customary closing conditions, including the expiration or termination of the applicable waiting period under the Hart-Scott-Rodino Antitrust Improvements Act of 1976.

Item 2. Management's Discussion and Analysis of Financial Condition and Results of Operations

The following discussion and analysis of our financial condition and results of operations should be read in conjunction with our unaudited condensed consolidated financial statements and related notes appearing elsewhere in this Quarterly Report, and the audited financial information and the notes thereto included in our Annual Report on Form 10-K for the year ended December 31, 2025.

Our actual results and timing of certain events may differ materially from the results discussed, projected, anticipated, or indicated in any forward-looking statements. We caution you that forward-looking statements are not guarantees of future performance and that our actual results of operations, financial condition and liquidity, and the development of the industry in which we operate may differ materially from the forward-looking statements contained in this Quarterly Report. In addition, even if our results of operations, financial condition and liquidity, and the development of the industry in which we operate are consistent with the forward-looking statements contained in this Quarterly Report, they may not be predictive of results or developments in future periods.

The following information and any forward-looking statements should be considered in light of factors discussed elsewhere in this Quarterly Report, including those risks identified under Part II, Item 1A. Risk Factors.

We caution readers against placing undue reliance upon any such forward-looking statements, which speak only as of the date they are made. We disclaim any obligation, except as specifically required by law and the rules of the SEC, to publicly update or revise any such statements to reflect any change in our expectations or in events, conditions or circumstances on which any such statements may be based, or that may affect the likelihood that actual results will differ from those set forth in the forward-looking statements.

Overview

We are a global biopharmaceutical company dedicated to improving the lives of children and adults with spinal muscular atrophy ("SMA") and additional rare, severe and debilitating neuromuscular diseases. As a leader in the biology of transforming growth factor beta ("TGFβ") superfamily, our novel understanding of the molecular mechanisms of growth factor activation enabled the development of a proprietary platform for the discovery and development of monoclonal antibodies that locally and selectively target the precursor, or latent, forms of growth factors. Based on our innovative, proprietary, and scalable technology platform, we are building a world-leading anti-myostatin pipeline.

We have one product approved in the U.S., ISEMBYLD™ (apitegromab-mstn), for the treatment of SMA, and our lead product candidates include a subcutaneous formulation of apitegromab, and SRK-439.

Apitegromab is a fully human monoclonal antibody that inhibits myostatin activation by selectively binding the pro- and latent forms of myostatin in skeletal muscle. Myostatin is a catabolic agent that functions as a negative regulator of muscle mass; therefore, inhibition of myostatin results in increased muscle mass and strength. On September 11, 2026, the U.S. Food and Drug Administration (the "FDA") approved ISEMBYLD for the treatment of SMA in adults and children two years of age and older who are currently receiving a survival motor neuron 2 ("SMN2")-targeted treatment. ISEMBYLD is the first and only muscle-targeted treatment approved for patients with SMA currently receiving SMN2-targeted therapy, and we have initiated the U.S. commercial launch of ISEMBYLD. We plan to seek marketing authorizations for apitegromab for use in SMA in other jurisdictions. In October 2026, we resubmitted our Marketing Authorisation Application ("MAA") for apitegromab for the treatment of children and adults with SMA to the European Medicines Agency ("EMA"). In November 2026, we submitted our Japanese New Drug Application ("JNDA") for apitegromab for the treatment of children and adults with SMA to Japan's Pharmaceuticals and Medicines Devices Agency ("PMDA").

We continue to evaluate ISEMBYLD in SMA with its ongoing long-term extension ONYX trial and its Phase 2 OPAL trial in SMA patients under two years of age, which was initiated in the third quarter of 2025. Additionally, we are evaluating apitegromab for the treatment of patients with facioscapulohumeral muscular dystrophy ("FSHD") with our Phase 2 FORGE trial, which was initiated in the second quarter of 2026. We see potential for apitegromab broadly in additional rare, severe, and debilitating neuromuscular diseases where muscle atrophy is a key component of disease pathogenesis, and we are actively exploring indications beyond SMA and FSHD.

In addition to the current intravenous ("IV") formulation, we are developing a subcutaneous ("SC") formulation of apitegromab. A Phase 1 study in healthy volunteers has been completed and demonstrated that SC apitegromab has favorable bioavailability and a comparable pharmacodynamic profile relative to IV apitegromab. Further development activities are ongoing, including planned FDA and EMA regulatory engagements.

Our clinical-stage pipeline also includes SRK-439, a novel, investigational, subcutaneously administered fully human anti-pro/latent myostatin antibody that has high inhibitory potency while maintaining selectivity towards myostatin. SRK-439 is being developed for the treatment of patients with rare, severe, and debilitating neuromuscular diseases. A Phase 1 study of SRK-439 in healthy volunteers

is currently underway. The company expects to have topline data by year end 2026 and plans to share data from this study in the first quarter of 2027.

Using our innovative approach and proprietary platform, we are creating a pipeline of novel product candidates that selectively modulate growth factor activation implicated in rare, severe, and debilitating neuromuscular diseases.

As we focus our strategy on rare neuromuscular diseases, we are currently seeking partnerships for our non-neuromuscular disease programs. These programs include: SRK-181, a Phase 2-ready investigational inhibitor of latent TGFβ1 in development for the treatment of patients with solid tumors that are resistant to anti-PD-(L)1 antibody therapies; SRK-373, an investigational, highly selective inhibitor of the latent TGFβ1 isoform with selective activity in the fibrotic extracellular matrix, in nonclinical development for the treatment of fibrotic diseases; and SRK-256, an investigational inhibitor of RGMc, or hemojuvelin, in nonclinical development for the treatment of iron-restricted anemias. We are also seeking partners to further evaluate the potential for myostatin inhibition in combination with GLP-1 weight loss approaches following our positive Phase 2 EMBRAZE study, demonstrating proof-of-concept in the ability of apitegromab to drive statistically significant preservation of lean mass during tirzepatide-induced weight loss.

We have incurred significant operating losses since inception. Our net losses were $322.6 million for the nine months ended September 30, 2026. As of September 30, 2026, we had an accumulated deficit of $1.6 billion. We expect to continue to incur significant expenses and operating losses for the foreseeable future in performing our ongoing activities, as we:

- develop our commercialization capabilities to support product sales, marketing and distribution activities;
- continue development activities for ISEMBYLD in SMA, including the ONYX study, our Phase 2 OPAL study for SMA patients under two years of age and the associated drug supply;
- explore and continue development activities for apitegromab in other neuromuscular disorders, including our Phase 2 FORGE clinical trial for apitegromab in FSHD;
- continue research and development activities for subcutaneous apitegromab and for our novel anti-myostatin program, including our Phase 1 clinical trial for SRK-439;
- continue to discover, validate and develop additional product candidates through the use of our proprietary platform;
- maintain, expand and protect our intellectual property portfolio;
- hire additional research, development, commercial and other business personnel; and
- continue to build the global infrastructure to support our operations as a global public company.

In September 2026, we commenced commercialization of ISEMBYLD in the U.S. following FDA approval. As a result, we expect to generate product revenue from sales of ISEMBYLD while continuing to incur substantial expenses associated with commercialization activities, including sales, marketing, medical affairs, manufacturing, distribution and post-approval development activities. Although we have begun generating product revenue, we expect to continue to incur significant operating losses for the foreseeable future as we commercialize ISEMBYLD and advance our development pipeline.

Financial Operations Overview

Revenue

Product revenue, net

On September 11, 2026, the FDA approved ISEMBYLD for the treatment of SMA in adults and children two years of age and older who are currently receiving SMN2-targeted treatment. We began commercial shipments of ISEMBYLD in the U.S. during the third quarter of 2026 and therefore began recognizing product revenue during the quarter. We sell ISEMBYLD in the U.S. to a specialty distributor, which resells the ISEMBYLD to qualified health care providers, and to specialty pharmacies, which dispenses ISEMBYLD for administration to patients. We record product revenue net of estimated variable consideration, including chargebacks, government and commercial rebates, discounts, distributor and specialty pharmacy service fees, copay assistance and estimated product returns. Product revenue during the launch period reflects sales to our customers, including inventory build within the channel, and may not directly correspond to patient starts, infusions or underlying patient demand during the period.

Cost of product revenue

Cost of product revenue includes the cost of producing and distributing inventories that are related to product revenue and may also include costs related to excess or obsolete inventory adjustment charges, abnormal costs, unabsorbed manufacturing and overhead costs, or other reasons. Based on our policy to expense costs associated with the manufacturing of our products prior to regulatory approval, certain of the manufacturing costs associated with product shipments of ISEMBYLD recorded during the three months ended September 30, 2026 were expensed prior to FDA approval of ISEMBYLD. This inventory carries a lower cost basis, resulting in lower cost of product revenue and higher gross margin during the initial commercialization period. We had expensed $25.6 million for this inventory prior to receiving approval on September 11, 2026.

Operating Expenses

Research and Development

Research and development expenses consist primarily of costs incurred for our research and development activities, including our product candidate discovery efforts, nonclinical studies, manufacturing, and clinical trials under our research programs, which include:

- employee-related expenses, including salaries, benefits and equity-based compensation expense for our research and development personnel;
- expenses incurred under agreements with third parties that conduct research and development and nonclinical activities on our behalf;
- expenses incurred under agreements related to our clinical trials, including the costs for investigative sites and contract research organizations ("CROs"), that conduct our clinical trials;
- manufacturing process development, manufacturing of clinical supplies, commercial drug supply prior to FDA approval and technology transfer expenses;
- consulting and professional fees related to research and development activities;
- costs of purchasing laboratory supplies and non-capital equipment used in our internal research and development activities;
- costs related to compliance with clinical regulatory requirements; and
- facility costs and other allocated expenses, which include expenses for rent and maintenance of facilities, insurance, depreciation and other supplies.

Research and development costs are expensed as incurred. Costs for certain activities are recognized based on an evaluation of the progress to completion of specific tasks. Nonrefundable advance payments for research and development goods and services to be received in the future from third parties are deferred and capitalized. The capitalized amounts are expensed as the related services are performed.

Prior to the regulatory approval of our drug candidates, we incurred expenses for the manufacture of drug product supplies to support clinical development that could potentially be available to support the commercial launch of those drugs. Until the date at which regulatory approval has been received or is otherwise considered probable, we record all such costs as research and development expenses.

A significant portion of our research and development costs have been external costs, which we track on a program-by-program basis after a clinical product candidate has been identified. However, we do not allocate our internal research and development expenses, consisting primarily of employee-related costs, depreciation and other indirect costs, on a program-by-program basis as they are deployed across multiple projects.

Research and development activities are central to our business model. Product candidates in later stages of clinical development 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, as well as the associated clinical trial material requirements. We expect research and development costs for our product candidates to continue to be substantial for the foreseeable future as the development programs progress. However, we do not believe that it is possible at this time to accurately project total program-specific expenses through

commercialization. There are numerous factors associated with the successful commercialization of any of our product candidates, 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 programs and plans.

The successful development of apitegromab for indications other than SMA, SRK-181, SRK-439, SRK-373, SRK-256 and any future product candidates is uncertain. Accordingly, at this time, we cannot reasonably estimate or know the nature, timing and estimated costs of the efforts that will be necessary to complete the remainder of the development of apitegromab for indications other than SMA, SRK-181, SRK-439, SRK-373, SRK-256 and any future product candidates. We are also unable to predict when, if ever, material net cash inflows will commence from the sale of our product candidates, if approved. This is due to the numerous risks and uncertainties associated with developing product candidates, including the uncertainty of:

- the scope, progress, outcome and costs of our nonclinical development activities, clinical trials and other research and development activities;
- developing product candidates, whether alone or in collaboration with others;
- establishing an appropriate safety profile;
- successful enrollment in and completion of clinical trials;
- whether our product candidates show safety and efficacy in our clinical trials;
- receipt of marketing approvals, if any, from applicable regulatory authorities;
- establishing commercial manufacturing capabilities or making arrangements with third-party manufacturers;
- obtaining and maintaining patent and trade secret protection and regulatory exclusivity for our product candidates;
- significant and changing government regulation; and
- continued acceptable safety profile of the products following any regulatory approval.

Any of these variables, or other factors, with respect to the development of apitegromab in indications other than SMA, SRK-181, SRK-439, SRK-373, SRK-256 or any of our future product candidates could significantly change the costs and timing associated with the development of that product candidate.

Selling, General and Administrative

Selling, general and administrative expenses consist primarily of employee-related expenses, including salaries, benefits and equity-based compensation expenses for personnel in executive, finance, business development, investor relations, legal, information technology, human resources and commercial functions. Other significant selling, general and administrative expenses include facility costs not otherwise included in research and development expenses, legal fees relating to patent and corporate matters and fees for accounting, consulting services, professional services and corporate expenses. We expect selling, general and administrative expense to continue to increase as we support the commercial launch and ongoing commercialization of ISEMBYLD in the U.S. and continue to build infrastructure to support potential future launches in other jurisdictions, as well as in support of our research and development activities.

Other Income (Expense), Net

Other income (expense), net consists primarily of interest income earned on our cash, cash equivalents and marketable securities, offset by interest expense incurred on our debt facility, including amortization of debt discount and debt issuance costs.

Results of Operations

Comparison of 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, except percentages):

	Three Months Ended September 30,		Change	
	2026	2025	$	%
Product revenue, net	$ 570	$ —	$ 570	NM
Operating expenses:				
Cost of revenue	8	—	8	NM
Research and development	54,128	50,486	3,642	7.2%
Selling, general and administrative	52,495	53,064	(569)	(1.1)%
Total operating expenses	106,631	103,550	3,081	3.0%
Loss from operations	(106,061)	(103,550)	(2,511)	2.4%
Other income (expense), net	(1,178)	1,330	(2,508)	(188.6)%
Net loss	$ (107,239)	$ (102,220)	$ (5,019)	4.9%

NM - not meaningful

Product revenue, net

For the three months ended September 30, 2026, we recorded net product revenue of $0.6 million from sales of ISEMBYLD in the U.S. There was no net product revenue for the three months ended September 30, 2025.

Cost of product revenue

For the three months ended September 30, 2026, we recorded cost of product revenue of $8,000 related to sales of ISEMBYLD in the United States. Prior to the FDA approval of ISEMBYLD, product costs were expensed as research and development expense.

Operating Expenses

Research and Development

Research and development expense was $54.1 million and $50.5 million for the three months ended September 30, 2026 and 2025, respectively, an increase of $3.6 million or 7.2%. The following table summarizes our research and development expense for the three months ended September 30, 2026 and 2025 (in thousands, except percentages):

	Three Months Ended September 30,		Change	
	2026	2025	$	%
External costs by program				
apitegromab	$ 19,982	$ 21,361	$ (1,379)	(6.5)%
SRK-181	17	357	(340)	(95.2)%
SRK-439	2,852	1,608	1,244	77.4%
Other early programs and unallocated costs	1,459	1,129	330	29.2%
Total external costs	24,310	24,455	(145)	(0.6)%
Internal costs:				
Employee compensation and benefits	24,413	21,423	2,990	14.0%
Facility and other	5,405	4,608	797	17.3%
Total internal costs	29,818	26,031	3,787	14.5%
Total research and development expense	$ 54,128	$ 50,486	$ 3,642	7.2%

The increase in research and development expense was primarily attributable to the following:

- A decrease in our external research and development costs of $0.1 million, which primarily consisted of:

- $1.4 million decrease in costs associated with apitegromab primarily due to a decrease in drug supply manufacturing and a decrease in clinical trial costs as our Phase 3 SAPPHIRE and our Phase 2 EMBRAZE trials are completed, partially offset by an increase in costs related to our Phase 2 OPAL trial in SMA patients under two years of age and our Phase 2 FORGE trial in FSHD patients, which was initiated during the second quarter of 2026;
- $0.3 million decrease in costs associated with SRK-181, as our Phase 1 DRAGON trial is completed;
- $1.2 million increase in costs associated with SRK-439 including costs associated with our Phase 1 study in healthy volunteers, partially offset by decreases in nonclinical costs and manufacturing development; and
- $0.3 million increase in costs associated with other early development candidates and unallocated costs;

- A $3.8 million increase in internal research and development costs, which was primarily driven by an increase of $2.1 million in employee related costs, including salaries, bonus, benefits and payroll taxes related to increased headcount, an increase in non-cash equity-based compensation expense of $0.9 million related to increased headcount, as well as an increase of $0.8 million in facilities and other costs to support our internal research and development team.

Total research and development expenses are expected to continue to be substantial as we support post-approval development activities for ISEMBYLD, including post-marketing requirements from the FDA by ISEMBYLD, ONYX, and OPAL, continue development activities in FSHD through the FORGE trial, advance the development of a subcutaneous formulation of apitegromab and continue development of SRK-439 and our broader pipeline. We expect costs of our SRK-181 program to continue to decrease, as compared to the prior year, as we completed the Phase 1 DRAGON clinical trial in June 2025.

Selling, General and Administrative

Selling, general and administrative expense was $52.5 million and $53.1 million for the three months ended September 30, 2026 and 2025, respectively, a decrease of $0.6 million or 1.1%. The total decrease was primarily driven by a decrease in professional fees of $3.2 million, partially offset by an increase of approximately $2.6 million in employee-related costs including salaries, bonus, benefits and payroll taxes related to increased headcount. We expect selling, general and administrative expenses to continue to increase as we continue to invest in building the global infrastructure to support the commercialization of apitegromab.

Other Income (Expense), Net

The change in other income (expense), net was primarily attributable to interest expense incurred on our debt facility, including amortization of debt discount and debt issuance costs, partially offset by an increase in interest income earned due to higher average balances in our cash, cash equivalents and marketable securities.

Comparison of 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, except percentages):

	Nine Months Ended September 30,		Change	
	2026	2025	$	%
Product revenue, net	$ 570	$ —	$ 570	NM
Operating expenses:				
Cost of revenue	8	—	8	NM
Research and development	164,176	161,565	2,611	1.6%
Selling, general and administrative	153,364	131,184	22,180	16.9%
Total operating expenses	317,548	292,749	24,799	8.5%
Loss from operations	(316,978)	(292,749)	(24,229)	8.3%
Other income (expense), net	(5,667)	5,775	(11,442)	(198.1)%
Net loss	$ (322,645)	$ (286,974)	$ (35,671)	12.4%

NM - not meaningful

Product revenue, net

In September 2026, the U.S. Food and Drug Administration (“FDA”) approved ISEMBYLD for the treatment of SMA in adults and

children two years of age and older who are currently receiving a survival motor neuron 2 ("SMN2")-targeted treatment. For the nine months ended September 30, 2026, we recorded net product revenue of $0.6 million from sales of ISEMBYLD in the U.S. There was no net product revenue for the nine months ended September 30, 2025.

Cost of product revenue

For the nine months ended September 30, 2026, we recorded cost of product revenue of $8,000 related to sales of ISEMBYLD in the U.S. Prior to the FDA approval of ISEMBYLD, product costs were expensed as research and development expense.

Operating Expenses

Research and Development

Research and development expense was $164.2 million and $161.6 million for the nine months ended September 30, 2026 and 2025, respectively, an increase of $2.6 million or 1.6%. The following table summarizes our research and development expense for the nine months ended September 30, 2026 and 2025 (in thousands, except percentages):

	Nine Months Ended September 30,		Change	
	2026	2025	$	%
External costs by program:				
apitegromab	$ 63,191	$ 68,618	$ (5,427)	(7.9)%
SRK-181	62	2,259	(2,197)	(97.3)%
SRK-439	8,371	8,462	(91)	(1.1)%
Other early programs and unallocated costs	4,279	4,811	(532)	(11.1)%
Total external costs	75,903	84,150	(8,247)	(9.8)%
Internal costs:				
Employee compensation and benefits	72,962	63,134	9,828	15.6%
Facility and other	15,311	14,281	1,030	7.2%
Total internal costs	88,273	77,415	10,858	14.0%
Total research and development expense	$ 164,176	$ 161,565	$ 2,611	1.6%

The increase in research and development expense was primarily attributable to the following:

- A decrease in our external research and development costs of $8.2 million, which primarily consisted of:
    - $5.4 million decrease in costs associated with apitegromab primarily due to a decrease in drug supply manufacturing and a decrease in clinical trial costs as our Phase 3 SAPPHIRE and our Phase 2 EMBRAZE trials are completed, partially offset by costs associated with our Phase 2 OPAL trial in SMA patients under two years of age, our early access program for ISEMBYLD and our Phase 2 FORGE trial in FSHD patients, which was initiated during the second quarter of 2026;
    - $2.2 million decrease in costs associated with SRK-181, as our Phase 1 DRAGON trial is completed;
    - $0.1 million decrease in nonclinical costs and manufacturing development for SRK-439, partially offset by increases in clinical trial costs as we conduct our Phase 1 study in healthy volunteers; and
    - 0.5 million decrease in costs associated with other early development candidates and unallocated costs;
- A $10.9 million increase in internal research and development costs, which was primarily driven by an increase of $5.8 million in employee related costs, including salaries, bonus, benefits and payroll taxes, an increase of $5.1 million in non-cash equity-based compensation expense related to increased headcount, an increase in severance costs of $1.0 million, as well as an increase of $1.0 million in facilities and other infrastructure costs, partially offset by a decrease in costs associated with our leadership transition in 2025, including $2.0 million in bonuses.

Total research and development expenses are expected to continue to be substantial as we support post-approval development activities for ISEMBYLD, including ONYX and OPAL, continue development activities in FSHD through the FORGE trial, advance the development of a subcutaneous formulation of apitegromab and continue development of SRK-439 and our broader pipeline. We expect costs of our SRK-181 program to continue to decrease, as compared to the prior year, as we completed the Phase 1 DRAGON clinical trial in June 2025.

Selling, General and Administrative

Selling, general and administrative expense was $153.4 million and $131.2 million for the nine months ended September 30, 2026 and 2025, respectively, an increase of $22.2 million or 16.9%. The total increase was primarily driven by investments in infrastructure to support commercial launch readiness for apitegromab, including an increase of approximately $24.7 million in employee-related costs including salaries, bonus, benefits and payroll taxes related to increased headcount, an increase of $7.2 million in equity-based compensation expense, an increase of approximately $5.7 million in professional service fees and an increase of approximately $1.1 million in facility and other costs. The increase in headcount is partially associated with the hiring of our commercial and field-facing teams. These increases were partially offset by a decrease in costs associated with our leadership transition in 2025, including $12.6 million in non-cash equity-based compensation expense, a decrease of $2.0 million in bonuses, as well as an overall decrease of $1.9 million in severance related costs. We expect general and administrative expenses to continue to be substantial as we continue to invest in building the global infrastructure to support the commercialization of apitegromab.

Other Income (Expense), Net

The change in other income (expense), net was primarily attributable to our loss on extinguishment of debt and interest expense incurred on our debt facility, including amortization of debt discount and debt issuance costs.

Liquidity and Capital Resources

Sources of Liquidity

Since our inception, our funding primarily derived from equity financings, debt financings and collaboration arrangements. Following the FDA approval of ISEMBYLD in September 2026, we began generating product revenue in the U.S.; however, product revenue has not yet been sufficient to fund our operations and we have incurred significant operating losses and negative cash flows from our operations. We have funded our operations to date primarily with proceeds from the sale of our convertible preferred stock and units in private placements before our initial public offering (“IPO”), and issuance of our common stock through our IPO in 2018, to Gilead in an exempt private placement, through multiple secondary public offerings and through “at-the-market offerings” (“ATM”) sales, as well as payments from our prior research collaborations, prior issuances of debt and the 2026 Loan Agreement (as defined below) entered into in February 2026 (see Note 13).

The following table provides information regarding our total cash, cash equivalents and marketable securities at September 30, 2026 and December 31, 2025 (in thousands):

	September 30, 2026	December 31, 2025
Cash and cash equivalents	$ 366,269	$ 323,527
Marketable securities	47,330	44,036
Total cash, cash equivalents and marketable securities	$ 413,599	$ 367,563

During the nine months ended September 30, 2026, our cash, cash equivalents and marketable securities balance increased by $46.0 million. The change was primarily due to proceeds from our debt facility, sales under our ATM program and the exercises of stock options and warrants, partially offset by cash used to operate our business, including payments related to, among other things, research and development and general and administrative expenses as we continued to invest in our product candidates and supported our internal research and development efforts, invested in building the infrastructure to support the commercialization of apitegromab and made interest payments on our debt.

Our current sales agreement with Jefferies, entered into in November 2022, allows for the sale of shares of our common stock from time to time in ATM offerings through Jefferies as the Company’s sales agent. During the nine months ended September 30, 2026, we sold 3,543,692 shares of our common stock under the ATM program, generating net proceeds of $160.8 million.

In February 2025, we entered into an Amended and Restated Loan and Security Agreement with Oxford Finance LLC (“Oxford”) and Silicon Valley Bank (“SVB”) (such agreement, as amended, the “Oxford Loan Agreement”) for up to $200 million of which $25.0 million from Tranche 1 was received in October 2020, $25.0 million from Tranche 2 was received in December 2021 and $50.0

million from Tranche 3 was received in September 2025, bringing the total outstanding balance under the Term Loans to $100.0 million. This debt facility was paid off in February 2026 (see Note 13).

In February 2026, we entered into a Financing Agreement (the "Financing Agreement") and a Pledge and Security Agreement (the "Security Agreement," together with the Financing Agreement, the "2026 Loan Agreement") with certain funds managed by Blue Owl Capital Corporation ("Blue Owl") for up to $350.0 million, of which $100.0 million of the initial term loan (the "Initial Term Loan") was received in February 2026. In addition, the Financing Agreement includes an uncommitted incremental term loan facility that requires the consent of Blue Owl in an aggregate principal amount not to exceed $200.0 million. We used the proceeds of the Initial Term Loan upon the closing under the 2026 Loan Agreement to repay all outstanding obligations, totaling $103.7 million, under the Oxford Loan Agreement with Oxford and upon such repayment, terminated the Oxford Loan Agreement. The amount repaid by the Company included $100.0 million of outstanding indebtedness plus accrued and unpaid interest as of February 27, 2026 and fees. We recognized a loss on extinguishment of debt of $3.3 million in connection with the termination of the Oxford Loan Agreement. As a result of the termination, all credit commitments under the Oxford Loan Agreement were terminated and all security interests and guarantees executed in connection with the Oxford Loan Agreement were released. On March 31, 2026, the Company received $100.0 million from the next available tranche under the 2026 Loan Agreement, bringing the outstanding balance to $200.0 million (see Note 13). The additional delayed draw term loan commitment in an aggregate principal amount not to exceed $150.0 million became available on September 11, 2026, on the date of the FDA's approval for ISEMBYLD in SMA. The additional loan will be available until the date that is the earliest of the full usage thereof, termination thereof and September 30, 2027, which may be borrowed by the Company at its sole option upon satisfying certain customary conditions, including satisfaction of the minimum cash covenant (if applicable).

During the nine months ended September 30, 2026, 6,804,082 of the Company's pre-funded warrants were exercised. As of September 30, 2026, the Company had 10,558,065 pre-funded warrants outstanding.

During the nine months ended September 30, 2026, 250,000 of the Company's common warrants were exercised. As of September 30, 2026, the Company did not have any common warrants outstanding.

In connection with the approval of ISEMBYLD, we were issued a Rare Pediatric Disease priority review voucher ("PRV") by the FDA. The PRV can be used to obtain priority review of a subsequent marketing application or sold or transferred to another sponsor. In November 2026, we entered into an asset purchase agreement with BioNTech US Inc., pursuant to which we agreed to sell our PRV for $205.0 million in cash upon the closing of the sale. The transaction remains subject to customary closing conditions. See Note 16, "Subsequent Events," to our condensed consolidated financial statements.

Cash Flows

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

	Nine Months Ended September 30,	
	2026	2025
Net cash used in operating activities	$ (234,215)	$ (224,539)
Net cash provided by (used in) investing activities	(2,891)	179,719
Net cash provided by financing activities	280,031	155,112
Net increase (decrease) in cash, cash equivalents and restricted cash	$ 42,925	$ 110,292

Net Cash Used in Operating Activities

Net cash used in operating activities was $234.2 million for the nine months ended September 30, 2026, and consisted of our net loss of $322.6 million, partially offset by changes in our assets and liabilities of $22.5 million and non-cash adjustments of $65.9 million. The non-cash adjustments are primarily from equity-based compensation.

Net cash used in operating activities was $224.5 million for the nine months ended September 30, 2025, and consisted of our net loss of $287.0 million, partially offset by changes in our assets and liabilities of $4.6 million and non-cash adjustments of $57.8 million. The non-cash adjustments are primarily from equity-based compensation.

Net Cash Provided by Investing Activities

Net cash used in investing activities was $2.9 million for the nine months ended September 30, 2026 compared to net cash provided by investing activities of $179.7 million for the nine months ended September 30, 2025.

Net cash provided by and used in investing activities for both periods was primarily associated with transactions involving our marketable securities.

Net Cash Provided by Financing Activities

Net cash provided by financing activities was $280.0 million for the nine months ended September 30, 2026 compared to $155.1 million for the nine months ended September 30, 2025. Net cash provided by financing activities for the nine months ended September 30, 2026 was primarily attributable to $160.8 million in proceeds from the sale of common shares under our ATM program, $197.7 million in proceeds from our debt facility and $20.6 million from the exercise of stock options, $7.1 million from the exercise of warrants, partially offset by $103.1 million from the repayment of our Oxford Loan Agreement with Oxford and $3.1 million of debt issuance costs related to our Financing Agreement with Blue Owl.

Net cash provided by financing activities for the nine months ended September 30, 2025 was primarily attributable to $91.7 million in proceeds from the sale of common shares under our ATM program, $50.0 million in proceeds from our debt facility and $3.4 million from the exercise of common warrants and $10.9 million from the exercise of stock options, partially offset by the net impact of our debt refinancing.

Funding Requirements

We expect our expenses to be substantial as we continue the research and development of apitegromab in indications, other than SMA and invest in our pipeline, as well as funding our ongoing commercialization of ISEMBYLD in the United States. We continue to pursue regulatory approvals for apitegromab in jurisdictions outside the U.S., including Europe. We expect to incur significant expenses related to commercial operations, manufacturing, supply chain, medical affairs, distribution and potential future launches outside the U.S. We expect to continue to incur apitegromab development costs as we invest in trials to support other SMA patient populations, such as our Phase 2 OPAL clinical trial, and for programs in multiple other diseases beyond SMA where selective inhibition of myostatin activation may offer therapeutic benefit, such as our Phase 2 FORGE trial. We expect to incur costs to support our anti-myostatin program, and our Phase 1 trial for SRK-439. Additionally, we will support the development of our pipeline and any other nonclinical programs. Furthermore, we expect to continue to incur costs associated with operating as a public company.

Based on our current operating model, we expect that our existing cash, cash equivalents and marketable securities, together with the cash available under our debt facility and our anticipated product revenues will enable us to fund our operating expenses and capital expenditure requirements into 2028. However, we will require additional capital in order to complete clinical development and commercialization for each of our current programs. We have based this estimate on assumptions that may prove to be wrong, and we may use our available capital resources sooner than we currently expect. Our future capital requirements will depend on many factors, including:

- the costs and timing of developing our products, product candidates and future product candidates, including costs associated with ISEMBYLD in ONYX, our long-term extension study in SMA for patients from both the TOPAZ and SAPPHIRE studies, our Phase 2 OPAL trial in SMA patients under the age of two, our Phase 2 FORGE trial of apitegromab in FSHD patients, our Phase 1 trial of SRK-439 in healthy volunteers and the costs and timing of conducting future nonclinical studies and clinical trials for SRK-373, SRK-256 or any other product candidates;
- the costs of future manufacturing of apitegromab indications, SRK-181, SRK-439, SRK-373, SRK-256 and any other future product candidates;
- the scope, progress, results and costs of discovery, nonclinical development, laboratory testing and clinical trials for other potential product candidates we may develop, if any;
- the costs of identifying and developing, or in-licensing or acquiring, additional product candidates and technologies;
- the costs, timing and outcome of regulatory review of our product candidates;
- our ability to establish and maintain collaborations on favorable terms, if at all;
- the achievement of milestones or occurrence of other developments that trigger payments under any collaboration agreements, license agreements, or other agreements we might have at such time;
- the costs associated with maintaining regulatory approvals for ISEMBYLD, obtaining approvals in additional jurisdictions and pursuing approvals for additional indications;

- the costs and timing of ongoing commercialization activities for ISEMBYLD, including product sales, marketing, manufacturing, distribution, market access and patient support activities;
- the amount of revenue generated from commercial sales of ISEMBYLD and the market acceptance of the product;
- the costs of preparing, filing and prosecuting patent applications, obtaining, maintaining and enforcing our intellectual property rights and defending intellectual property-related claims;
- our headcount growth and associated costs as we expand our global business operations and research and development activities;
- the costs of supporting our global infrastructure and facilities, including equipment and physical infrastructure to support our research and development;
- the costs of operating as a global public company; and
- the impact of adverse global economic conditions on our business, including increased costs associated with global tariff policies, which may exacerbate the magnitude of the factors discussed above.

Identifying potential product candidates and conducting nonclinical studies and clinical trials is a time-consuming, expensive and uncertain process that takes years to complete, and we may never generate the necessary data or results required to obtain marketing approval and achieve product sales for additional product candidates. In addition, commercialization of ISEMBYLD may not be successful, and any future approved products may not achieve commercial success. Accordingly, we will need to continue to rely on additional financing to achieve our business objectives. Adequate additional financing may not be available to us on acceptable terms, or at all.

Until such time, if ever, as we can generate substantial product revenues, we expect to finance our cash needs through a combination of equity offerings, debt financings, collaborations, strategic alliances and licensing arrangements. To the extent that we raise additional capital through the sale of equity or convertible debt securities, common stockholder ownership interests may be diluted, and the terms of these securities may include liquidation or other preferences that could adversely affect the rights of a common stockholder. Additional debt financing, if available, may involve agreements that include restrictive covenants that limit our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends, that could adversely impact our ability to conduct our business.

If we raise funds through collaborations, strategic alliances or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or to grant licenses on terms that may not be favorable to us. Market volatility or other factors could also adversely impact our ability to access capital as and when needed. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

Critical Accounting Estimates

This management's discussion and analysis is based on our condensed consolidated financial statements, which have been prepared in accordance with U.S. generally accepted accounting principles. The preparation of these condensed consolidated financial statements requires us to make judgments and estimates that affect the reported amounts of assets, liabilities, revenues and expenses and the disclosure of contingent assets and liabilities in our condensed consolidated financial statements. We base our estimates on historical experience, known trends and events and various other factors that we believe to be reasonable under the circumstances, the results of which form the basis for making judgements about the carrying values of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions. On an ongoing basis, we evaluate our judgments and estimates in light of changes in circumstances, facts and experience. The effects of material revisions in estimates, if any, will be reflected in the condensed consolidated financial statements prospectively from the date of change in estimates. Our actual results may differ from these estimates under different assumptions or conditions.

There have been no material changes to our critical accounting estimates from those described in Part II, Item 7. "*Management's Discussion and Analysis of Financial Condition and Results of Operations*" included in our Annual Report on Form 10-K for the year ended December 31, 2025.

Off-Balance Sheet Arrangements

We did not have during the periods presented, and we do not currently have, any off-balance sheet arrangements, as defined under applicable SEC rules.

Recent Accounting Pronouncements

We have reviewed all recently issued standards and have determined that, other than as disclosed in Note 2 to our condensed consolidated financial statements appearing elsewhere in this Quarterly Report, they will not have a material impact on our financial statements or do not otherwise apply to our operations.

Smaller Reporting Company and Non-Accelerated Filer Status

Based on the market value of our common stock held by our non-affiliates as of June 30, 2025, we are no longer a smaller reporting company. Accordingly, we ceased to be eligible to use the requirements for a smaller reporting company beginning with our Quarterly Report on Form 10-Q for the quarter ended March 31, 2026, and are thus subject to additional disclosure and compliance requirements. Because we remained eligible to use the requirements for smaller reporting companies through December 31, 2025, we will remain a "non-accelerated filer" through December 31, 2026.

Item 3. Quantitative and Qualitative Disclosures About Market Risk

Interest Rate Risk

As of September 30, 2026 and December 31, 2025, we had cash, cash equivalents, and marketable securities totaling $413.6 million and $367.6 million, respectively, which included bank deposits, money market funds, U.S. treasury obligations and government agency securities. Our primary exposure to market risk is interest rate sensitivity, which is affected by changes in the general level of U.S. interest rates, particularly because our investments are in short-term marketable securities. Such interest-earning instruments carry a degree of interest rate risk; however, historical fluctuations in interest income have not been significant for us. Due to the short-term duration of our cash equivalents and marketable securities, we do not believe an immediate one percent change in interest rates would have a material effect on the fair value of our cash equivalents and marketable securities.

Foreign Currency Risk

Our operations include activities in countries outside the U.S. As a result, our financial results are impacted by factors such as changes in foreign currency exchange rates or weak economic conditions in the foreign markets where we operate. We do not currently hedge our foreign currency exchange rate risk. Our monetary exposures on our condensed consolidated balance sheet were immaterial to our financial position as of September 30, 2026 and December 31, 2025.

Inflation

Inflation generally affects us by increasing our cost of labor, research, manufacturing and development costs, and clinical trial costs. We have not seen a significant impact from inflation on our business, financial condition or results of operations as of September 30, 2026. However, a significant or prolonged period of high inflation could adversely impact our results if the increase in costs outpaces the growth in our revenues.

Item 4. Controls and Procedures

Management's Evaluation of our Disclosure Controls and Procedures

We maintain "disclosure controls and procedures," as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act, that are designed to ensure that information required to be disclosed in the reports that we file or submit under the Exchange Act is (1) recorded, processed, summarized and reported within the time periods specified in the Securities and Exchange Commission's rules and forms and (2) accumulated and communicated to our management, including our principal executive officer and principal financial and accounting officer, as appropriate to allow timely decisions regarding required disclosure. Our management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives and our management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Our disclosure controls and procedures are designed to provide reasonable assurance of achieving their control objectives.

Our management, with the participation of our chief executive officer (principal executive officer) and chief financial officer (principal financial and accounting officer) have evaluated the effectiveness of our disclosure controls and procedures as of September 30, 2026, the end of the period covered by this Quarterly Report on Form 10-Q. Based upon such evaluation, our chief

executive officer and chief financial officer have concluded that our disclosure controls and procedures were effective at the reasonable assurance level as of such date. We continue to review and document our disclosure controls and procedures, including our internal controls and procedures for financial reporting, and may from time to time make changes aimed at enhancing their effectiveness and to ensure that our systems evolve with our business.

Changes in Internal Controls Over Financial Reporting

No change in our internal controls over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) occurred during the nine months ended September 30, 2026 that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

Part II. OTHER INFORMATION

Item 1. Legal Proceedings

From time to time, we are subject to various legal proceedings and claims that arise in the ordinary course of our business activities. Although the results of litigation and claims cannot be predicted with certainty, as of the date of this Quarterly Report, we do not believe we are party to any claim or litigation the outcome of which, if determined adversely to us, would individually or in the aggregate be reasonably expected to have a material adverse effect on our business. Regardless of the outcome, litigation can have an adverse impact on us because of defense and settlement costs, diversion of management resources and other factors.

Item 1A. Risk Factors

Careful consideration should be given to the following risk factors, together with all other information set forth in this Quarterly Report, including our condensed consolidated financial statements and related notes, and "Management's Discussion and Analysis of Financial Condition and Results of Operations," and in other documents that we file with the SEC, in evaluating Scholar Rock Holding Corporation and our subsidiaries (collectively, the "Company," "we," or "our") and our business, before investing in our common stock. Investing in our common stock involves a high degree of risk. If any of the following risks and uncertainties actually occurs, our business, prospects, financial condition and results of operations could be materially and adversely affected. The market price of our common stock could decline if one or more of these risks or uncertainties were to occur, which may cause you to lose all or part of the money you paid to buy our common stock. The risk factors described below disclose both material and other risks, and are not intended to be exhaustive and are not the only risks facing the Company. New risk factors can emerge from time to time, and it is not possible to predict the impact that any factor or combination of factors may have on our business, prospects, financial condition and results of operations. Certain statements below are forward-looking statements. See "Special Note Regarding Forward-Looking Statements" in this Quarterly Report.

Summary of the Material Risks Associated with Our Business

Our business is subject to numerous risks and uncertainties that you should be aware of before making an investment decision, including those highlighted in the section entitled "Risk Factors." These risks include, but are not limited to, the following:

Risks Related to Product Development, Regulatory Approval and Commercialization

- Our near-term prospects are wholly dependent on the successful commercialization of ISEMBYLD. We have limited experience commercializing an approved product, and if we are unable to successfully commercialize ISEMBYLD in the U.S. for its approved indication, our ability to generate meaningful revenue or achieve profitability will be materially and adversely affected.
- The regulatory approval process for apitegromab in the EU, Japan and other jurisdictions will be lengthy, time-consuming and inherently unpredictable and we may fail to receive or be delayed in receiving regulatory approval of apitegromab, or we may receive regulatory approval for only a subset of SMA patients. Our progress toward potential commercialization of apitegromab in the EU has been delayed. We recently resubmitted our MAA to the EMA and submitted our JNDA for apitegromab to the PMDA. The review and approval of our applications in these jurisdictions may be delayed or unsuccessful.
- Even though ISEMBYLD has been approved by the FDA, if and when other product candidates receive marketing approval, they 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.
- We have recently launched ISEMBYLD in the U.S. and are continuing to build and scale the infrastructure to support its successful commercialization in the U.S. and the potential future commercialization of apitegromab in additional jurisdictions, including Europe and Japan. If we are unable to successfully execute our commercial strategy, expand and maintain our compliance, medical affairs and commercial capabilities, or effectively support the launch and ongoing commercialization of ISEMBYLD in the U.S., our business could be negatively impacted, including a negative impact on the potential for a successful commercialization of apitegromab.
- Changes or disruptions at the FDA and other government agencies caused by funding cuts, government shutdowns, personnel reductions, substantial changes in leadership and policy, or other changes or disruptions to these agencies' operations could prevent these agencies from performing functions on which the operation of our business relies, and any such disruptions and changes could negatively impact our business.
- Unfavorable pricing and reimbursement decisions in the EU could significantly delay or limit patient access to apitegromab, if approved, and materially reduce our anticipated revenues. Even if we obtain marketing authorization, the EU health

technology assessment bodies and national payors may impose price reductions, require additional clinical or real world evidence, restrict reimbursement, or deny coverage altogether. Pricing and reimbursement negotiations in the EU are often lengthy, highly uncertain, and subject to country by country requirements, reference pricing, budget impact assessments, and mandatory discounts. As a result, the commercial viability of apitegromab in the EU may be materially impacted.

- Product development involves a lengthy and expensive process, with an uncertain outcome. We may incur additional costs or experience delays in completing, or ultimately be unable to complete, the development and commercialization of ISEMBYLD, SRK-181, SRK-439, or any future product candidates. Many of the factors that cause, or lead to, a delay in the initiation or completion of clinical trials may also ultimately lead to the denial of regulatory approval or limit market acceptance of our product and product candidates.
- The results or success of nonclinical studies and early-stage clinical trials of our product candidates may not be predictive of future results or replicated in later nonclinical studies or clinical trials of our product candidates in the same indications or other indications.
- Interim, initial and preliminary results from our clinical trials that we announce or publish from time to time may change (e.g., from positive safety or efficacy results to poor or negative safety or efficacy results) as more patient data become available and are subject to additional audit, validation and verification procedures that could result in material changes in the final data.
- We rely on third parties to conduct certain aspects of our clinical trials and to conduct certain aspects of our nonclinical studies and on commercial distributors to distribute our product. If these third parties do not successfully carry out their contractual duties or meet expected deadlines or comply with legal and regulatory requirements, we may be delayed or unable to receive regulatory approval of our product candidates or commercialize ISEMBYLD or any future product candidates, and our business could be materially harmed.
- Nonclinical development is uncertain. Our nonclinical programs may experience delays or may never advance to clinical trials, which would adversely affect our ability to develop our product pipeline and receive regulatory approvals or commercialize these programs on a timely basis or at all, which would have an adverse effect on our business.

Risks Related to Our Business and Operations

- Because we rely on a limited number of third-party manufacturing and supply partners, our supply of commercial product for ISEMBYLD and materials for our research and development, nonclinical and clinical development programs may become limited or interrupted or may not be of satisfactory quantity or quality.
- Our reliance on third parties, such as manufacturers, third-party logistics providers, specialty distributors, specialty pharmacies, and patient service providers, may subject us to risks and may cause us to undertake substantial obligations, including financial obligations.
- We will need to continue to grow our organization in certain areas, including our personnel, systems and relationships with third parties, in order to develop and potentially commercialize our product and product candidates and we may experience difficulties in managing this growth.
- Our executives and highly skilled technical and managerial personnel are critical to our business. If we have transition in management, lose key personnel, or if we fail to recruit additional highly skilled personnel, our ability to further develop and commercialize ISEMBYLD and any of our other product candidates, if approved, and identify and develop new or next generation product candidates may be impaired.
- Failure to comply with health care, privacy and data protection laws and regulations could lead to government or competent authority enforcement actions (which could include civil or criminal penalties), private litigation, and/or adverse publicity and could negatively affect our operation results and business.

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

Risks Related to Our Financial Condition and Capital Requirements

- We have incurred net losses in every year since our inception and anticipate that we will continue to incur net losses in the future.
- We will require additional capital to fund our operations and if we fail to obtain necessary capital, we will not be able to complete the development and commercialization of ISEMBYLD, SRK-181, SRK-439 and any future product candidates.

Risks Related to Our Common Stock

- The price of our stock is volatile, and you could lose all or part of your investment.

Risks Related to Product Development, Regulatory Approval and Commercialization

Our near-term prospects are wholly dependent on the successful commercialization of ISEMBYLD. We have limited experience commercializing an approved product, and if we are unable to successfully commercialize ISEMBYLD in the U.S. for its approved indication, our ability to generate meaningful revenue or achieve profitability will be materially and adversely affected.

On September 11, 2026, the FDA approved ISEMBYLD for the treatment of SMA in adults and children two years of age and older who are currently receiving SMN2-targeted treatment. We have recently launched ISEMBYLD in the U.S. for that indication.

ISEMBYLD is our only product approved for marketing by the FDA, and our ability to generate revenue from product sales and achieve profitability is wholly dependent on our ability to successfully commercialize ISEMBYLD in the U.S. for its approved indication. We may not be able to successfully commercialize ISEMBYLD for a number of reasons, including:

- we may not be able to establish or demonstrate in the medical community the safety and efficacy of ISEMBYLD;
- physicians may be reluctant to prescribe ISEMBYLD until longer-term efficacy and safety data exists;
- we have limited experience in marketing, selling and distributing ISEMBYLD or any other product;
- we are dependent on the reimbursement and coverage policies of government and private payors such as Medicare, Medicaid, insurance companies, health maintenance organizations and other plan administrators and may not be able to obtain adequate reimbursement and coverage for ISEMBYLD;
- we may be unable to secure or maintain favorable coverage, reimbursement and formulary access for ISEMBYLD on acceptable terms or within expected timeframes;
- sales may be affected by the relative price of ISEMBYLD as compared to alternative treatment options;
- sales volumes will depend on the incidence and prevalence of patients in ISEMBYLD's approved indication, and our market and sales estimates may be incorrect;
- physicians, patients and payors may have concerns regarding the safety profile of ISEMBYLD, which could limit prescribing, slow patient adoption, result in additional monitoring requirements or adversely affect reimbursement, market access and the commercial success of ISEMBYLD;
- future competitive or other market factors may adversely affect the commercial potential of ISEMBYLD;
- we may not be able to obtain and maintain regulatory approvals for ISEMBYLD in any other jurisdictions or for any other apitegromab indications;
- changes could be required to the label for ISEMBYLD that restrict how we market and sell ISEMBYLD, including as a result of adverse events observed following commercialization or in ongoing and future studies of ISEMBYLD;
- we may encounter manufacturing challenges that may impact product supply or may need to conduct product recalls;
- we might not be able to maintain compliance with the legal and regulatory requirements for the manufacturing, marketing, promotion, sale, distribution and commercialization of ISEMBYLD and may face enforcement actions;

- we may not be able to maintain adequate commercial supplies of ISEMBYLD, or there may not be adequate supplies of apitegromab to meet demand at an acceptable cost or at all; and
- we may need additional financial or other resources to successfully commercialize ISEMBYLD.

Moreover, successful commercialization of ISEMBYLD may not generate sufficient revenue from product sales, and we may not become profitable in the near term, or at all. If we are unable to successfully commercialize ISEMBYLD in the U.S. for its approved indication, our ability to generate meaningful revenue from product sales and achieve profitability will be materially and adversely affected, which in turn could have a material adverse effect on our business and business prospects.

The regulatory approval process for apitegromab in the EU, Japan and other jurisdictions will be lengthy, time-consuming and inherently unpredictable and we may fail to receive or be delayed in receiving regulatory approval of apitegromab, or we may receive regulatory approval for only a subset of SMA patients. Our progress toward potential commercialization of apitegromab in the EU has been delayed. We recently resubmitted our MAA to the EMA and submitted our JNDA for apitegromab to the PMDA. The review and approval of our applications in these jurisdictions may be delayed or unsuccessful.

The research, testing, manufacturing, labeling, approval, sale, import, export, marketing, promotion and distribution of drug products, including biologics, are subject to extensive regulation by the FDA in the U.S. and other regulatory authorities outside the U.S. On September 11, 2026, the FDA approved ISEMBYLD for the treatment of SMA in adults and children two years of age and older who are currently receiving SMN2-targeted treatment. We are not permitted to market apitegromab outside the U.S. until we receive marketing authorizations from applicable regulatory authorities. Prior to submitting the BLA to the U.S. FDA in January 2025 and submitting the MAA to the EMA in March 2025 for apitegromab as a treatment for patients with SMA, we had not submitted a BLA to the FDA or MAA to the EMA or similar marketing application to comparable foreign authorities. In November 2026 we submitted our JNDA for apitegromab to the PMDA. An MAA or comparable marketing authorization application, including a JNDA, must include extensive nonclinical and clinical data and supporting information to establish that the product candidate is safe, pure and potent or otherwise satisfies applicable standards for approval for each desired indication. EC or comparable foreign regulatory authorities' approval of a new biologic or drug generally requires data from one or more adequate and well-controlled pivotal Phase 3 clinical trials of the biologic or drug in the relevant patient population. The EMA, PMDA or comparable foreign regulatory authorities may disagree with the design or implementation of our clinical trials or our analysis or interpretation of data from nonclinical studies or clinical trials, the results of our clinical trials may not meet the level of statistical significance or amount of data required for approval, regulatory authorities may not agree with the statistical methods we used to evaluate our clinical data, or we may be unable to demonstrate that apitegromab's clinical and other benefits outweigh its safety risks. Although we have recently received approval from the FDA for ISEMBYLD, for the treatment of SMA in adults and children two years of age and older who are currently receiving SMN2-targeted treatment, the EMA, PMDA or other regulatory authorities may determine that the approval should be further limited to a subset of SMA patients, which could restrict the commercial potential of ISEMBYLD in SMA. An MAA to the EMA or JNDA to the PMDA must also include significant information regarding the chemistry, manufacturing, and controls for the product, and the manufacturing facilities must complete a successful pre-license inspection as well as certain key clinical sites conducting our clinical trials. The EMA or comparable foreign regulatory authorities may fail to approve the manufacturing processes or facilities of third-party manufacturers with which we contract for clinical and commercial supplies.

The third-party fill-finish facility included in our original MAA received an OAI inspection classification from the FDA following inspections in 2025 and again in 2026. As a result, and following discussions with the EMA, we withdrew our original MAA and resubmitted the application in October 2026 to incorporate an alternate fill-finish facility. The EMA will require time to review the resubmitted MAA and there is no guarantee that the EMA will give a positive opinion on the grant of a marketing authorisation for apitegromab on the basis of the resubmitted MAA. In addition, in November 2026 we submitted our JNDA for apitegromab to the PMDA, and there can be no assurance that the PMDA will complete its review within our anticipated timeframe or approve apitegromab for the indication or patient population sought, if at all. The PMDA may request additional data or analyses or impose other requirements in connection with its review of our application, which could delay or prevent regulatory approval of apitegromab in Japan.

Even though ISEMBYLD has been approved by the FDA, if and when other product candidates receive marketing approval, they 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.

ISEMBYLD, or other product candidates that in the future receive marketing approval, may nonetheless fail to gain sufficient market acceptance by physicians, patients, third-party payors, and others in the medical community. There may be delays in getting ISEMBYLD, or other product candidates if approved, on hospital or insurance formularies, or there may be limitations on coverage in the early stages of commercialization for newly approved drugs. If ISEMBYLD, or other product candidates if approved, fails to achieve market acceptance among hospitals, physicians, patients or health care payors, we will not be able to generate significant

revenues and we may not become profitable, which would have a material adverse effect on our business, prospects, financial condition and results of operations. For example, doctors may deem it sufficient to treat patients with SMA with an SMN-targeted treatment such as nusinersen or risdiplam, and therefore will not be willing to utilize ISEMBYLD in conjunction with such SMN-targeted treatment. The degree of market acceptance of ISEMBYLD or other product candidates if approved for commercial sale, will depend on a number of factors, including:

- the efficacy and safety of ISEMBYLD or other product candidates as demonstrated in clinical trials;
- the indications for which other apitegromab indications or product candidates are approved;
- efficacy and potential advantages compared to alternative treatments;
- the ability to obtain sufficient third-party coverage and adequate reimbursement;
- the amount, scope and nature of the clinical data (and other forms of data) available;
- the ability to offer ISEMBYLD for sale at competitive prices;
- the timing of market introduction of ISEMBYLD as well as competitive products;
- convenience and ease of administration compared to alternative treatments;
- the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies;
- the strength of marketing and distribution support; and
- the prevalence and severity of any side effects.

Market acceptance of our product candidates is heavily dependent on patients' and physicians' perceptions that our products and product candidates are safe and effective treatments for their targeted indications and willingness to use screening and/or diagnostic tests to identify at-risk target populations for our products and product candidates, if approved. The perceptions of any product are also influenced by perceptions of competitors' products that are in the same class or that have a similar mechanism of action. Because we expect sales of our ISEMBYLD to generate substantially all our revenues in the foreseeable future, the failure of ISEMBYLD to find market acceptance would harm our business.

We have recently launched ISEMBYLD in the U.S. and are continuing to build and scale the infrastructure necessary to support its successful commercialization in the U.S. and the potential future commercialization of apitegromab in additional jurisdictions, including Europe and Japan. If we are unable to successfully execute our commercial strategy, expand and maintain our compliance, medical affairs and commercial capabilities, or effectively support the launch and ongoing commercialization of ISEMBYLD in the U.S., our business could be negatively impacted, including a negative impact on the potential for a successful commercialization of apitegromab.

Although we have recently launched ISEMBYLD in the U.S. and are preparing our commercialization capabilities in anticipation of a potential approval and commercial launch of apitegromab in Europe, Japan and other jurisdictions around the globe, we have no prior sales or distribution experience and limited capabilities for marketing and market access. We expect to continue to invest significant financial and management resources over time in our compliance, medical affairs and commercial organizations to support the ongoing commercialization of ISEMBYLD in the U.S. and potential launches in Europe, Japan and other jurisdictions, if approved. If we are unable to establish these commercial capabilities and infrastructure in a timely manner or to enter into agreements with third parties to market, sell, and/or distribute apitegromab, if approved, we may be unable to complete a successful commercial launch. To the extent we enter into agreements with third parties, the revenue we receive may depend upon the efforts of such third parties, over which we may have limited or no control, and our revenue from product sales may be lower than if we had commercialized the products ourselves. We also face competition in our search for third parties to assist us with the distribution, sales and marketing of our products.

Furthermore, we intend to commercialize apitegromab in Europe, Japan and other jurisdictions globally, if approved. In order to do so, we must build, on a territory-by-territory basis, marketing, sales, distribution, managerial and other capabilities or make arrangements with third parties to perform these services, and we may not be successful in doing so.

Changes or disruptions at the FDA and other government agencies caused by funding cuts, government shutdowns, personnel reductions, substantial changes in leadership and policy, or other changes or disruptions to these agencies' operations could prevent these agencies from performing functions on which the operation of our business relies and any such disruptions and changes could negatively impact our business.

The ability of the FDA and foreign regulatory authorities to review and or approve new products can be affected by a variety of factors, including government budget and funding levels, staffing levels, and statutory, regulatory, and policy changes, the FDA's and

foreign regulatory authorities' ability to hire and retain key personnel and accept the payment of user fees, and other events that may otherwise affect the FDA's ability to perform routine functions. Average review times at the FDA and foreign regulatory authorities have fluctuated in recent years as a result. Disruptions at the FDA and other agencies, including substantial leadership, personnel, and policy changes, may also slow the time necessary for new drugs to be reviewed and/or approved by necessary government agencies, which would adversely affect our business. In 2025, the U.S. government has, among other measures, issued executive orders and undertaken reductions in force that have adversely impacted FDA staffing and resources. Such changes could significantly impact the ability of the FDA to timely review and take action on our regulatory submissions, which could have a material adverse effect on our business.

In addition, changes in the requirements and policies of the FDA and other regulatory agencies with jurisdiction over our products, including applicable pricing and reimbursement frameworks under federal healthcare programs, could affect the commercial viability of our products, create revenue uncertainty, and impact our ability to achieve profitability. Additionally, regulatory changes may introduce new challenges in obtaining FDA approval or navigating commercialization, and any delay in securing applicable regulatory approvals would adversely affect our business and prospects. These uncertainties could also present new challenges and/or opportunities. Any delay in obtaining, or our inability to obtain, applicable regulatory approvals would delay or prevent commercialization of apitegromab and could materially adversely impact our business and prospects.

Product development involves a lengthy and expensive process, with an uncertain outcome. We may incur additional costs or experience delays in completing, or ultimately be unable to complete, the development and commercialization of ISEMBYLD, SRK-181, SRK-439, or any future product candidates. Many of the factors that cause, or lead to, a delay in the initiation or completion of clinical trials may also ultimately lead to the denial of regulatory approval or limit market acceptance of our product and product candidates.

Before obtaining regulatory approvals for the commercial sale of any product and product candidates, we must demonstrate through extensive nonclinical studies and clinical trials that our product and product candidates are safe and effective in humans. Clinical development is expensive and can take many years to complete, and its outcome is inherently uncertain, a clinical trial can fail at any stage of development. We may experience delays in initiating, progressing or completing our clinical trials. We may be unable to establish clinical endpoints that applicable regulatory authorities would consider clinically meaningful. Clinical trials may fail to meet their primary or secondary endpoints, raise safety concerns or generate mixed results. Differences in trial design between early-stage clinical trials and later-stage clinical trials make it difficult to extrapolate the results of earlier clinical trials to later clinical trials. Clinical data may not be sufficient to apply for and obtain regulatory approval on the timelines we expect or at all. Other decisions or actions of regulatory agencies may affect our plans, progress or results.

We also may experience numerous unforeseen events during, or as a result of, any clinical trials in process or any future clinical trials that we conduct that could delay or prevent our ability to receive and retain marketing approval or commercialize ISEMBYLD, SRK-181, SRK-439, or any future product candidates, including:

- delay or inability to reach agreement with the FDA or comparable foreign regulatory authorities on acceptable clinical trial design, conduct or statistical analysis plan;
- regulators, Institutional Review Boards ("IRBs") or ethics committees may not authorize us or our investigators to commence a clinical trial or conduct a clinical trial at a prospective trial site;
- we may experience delays in reaching, or fail to reach, agreement on acceptable terms with prospective trial sites and prospective CROs, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and trial sites;
- clinical trials of any product and product candidates may fail to show safety and effectiveness, or produce negative or inconclusive results and we may decide, or regulators may require us, to conduct additional nonclinical studies or clinical trials or we may decide to abandon product development programs;
- the number of subjects required for clinical trials of any product and product candidates may be larger than we anticipate, enrollment in these clinical trials may be slower or more challenging than we anticipate or subjects may drop out of these clinical trials or fail to return for post treatment follow-up at a higher rate than we anticipate;
- challenges in identifying or recruiting sufficient study sites or investigators for clinical trials;
- our third-party contractors may fail to comply with regulatory requirements or meet their contractual obligations to us in a timely manner, or at all;

- clinical study sites or clinical investigators may deviate from the clinical trial protocol or drop out of the trial, which may require that we add new clinical trial sites or investigators;
- we may elect to, or regulators, IRBs, data safety monitoring boards, or ethics committees may recommend or require that we or our investigators, suspend or terminate clinical research or trials for various reasons, including noncompliance with regulatory requirements, a finding that the participants are being exposed to unacceptable health risks, undesirable side effects or unexpected characteristics, or reports from clinical testing of other therapies that raise safety or efficacy concerns about our product candidates;
- limitations on our or our CROs' ability to access and verify clinical trial data captured at clinical study sites through monitoring and source document verification;
- the cost of clinical trials of a product candidate may be greater than we anticipate;
- the supply or quality of our product candidates or other materials necessary to conduct clinical trials of our product candidates may be insufficient or inadequate to initiate or complete a given clinical trial;
- our product and product candidates may have undesirable side effects or other unexpected characteristics when used in a new disease indication or with products in a different class which may raise safety, efficacy or other concerns about our product candidate as a potential therapy in that new disease indication or other indications or its use with products in a different class;
- our failure to establish an appropriate safety profile for a product candidate based on clinical or nonclinical data for such product candidate and/or data emerging from other molecules in the same class as our product candidate;
- the FDA, EMA or other regulatory authorities may require us to submit additional data, such as long-term toxicology studies, or change or impose other requirements before permitting us to initiate a clinical trial; and
- evolution in the standard of care or changes in applicable governmental regulations or policies during the development of a product candidate that require amendments to ongoing clinical trials and/or the conduct of additional nonclinical studies or clinical trials.

Many of the factors that cause, or lead to, a delay in the initiation or completion of clinical trials may also ultimately lead to the denial of regulatory approval or limit market acceptance of our product and product candidates. For example, we anticipate some of our future trials to, in part, utilize an open-label trial design, and our ongoing ONYX long-term extension study for apitegromab in patients from both the TOPAZ and SAPPHIRE trials utilizes an open-label trial design. An open-label trial is one where both the patient and investigator know whether the patient is receiving the test article or either an existing approved drug or placebo. Open-label trials are subject to various limitations that may exaggerate any therapeutic effect as patients in open-label studies are aware that they are receiving treatment. Open-label trials may be subject to a patient bias, for example, if patients perceive their symptoms to have improved merely due to their awareness of receiving an experimental treatment. Open-label trials also 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. The potential sources of bias in clinical trials as a result of open-label design may not be adequately mitigated and may cause any of our trials that utilize such design to fail and additional trials may be necessary to support future marketing applications. In addition, other types of trials (including randomized, double-blind, parallel arm studies), particularly if smaller in size or if limited to one study, are also subject to potential sources of bias and limitations that may exaggerate any therapeutic effect or falsely identify a positive efficacy signal**,** or conversely, fail to detect an efficacy signal when in fact there may actually be a positive therapeutic effect.

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 could also shorten any periods during which we may have the exclusive right to commercialize our product and product candidates and may allow our competitors to bring products to market before we do, potentially impairing our ability to successfully commercialize our product and product candidates. Any delays in our clinical development programs may harm our business, financial condition and results of operations significantly.

Our clinical development strategy and commercialization of ISEMBYLD depends on the continued use and availability of certain third-party approved drug therapies.

Patients in ONYX, our long-term extension study for patients from both the TOPAZ and SAPPHIRE studies, and OPAL, our Phase 2 clinical trial of ISEMBYLD in children under the age of two living with SMA, are receiving ISEMBYLD in conjunction with an approved SMN targeted treatment. These patients are reliant on the continued use and availability of such treatments. If access to an approved SMN targeted treatment such as nusinersen or risdiplam becomes limited or is unavailable, we may be forced to pause or stop our ONYX or OPAL clinical trials, or the medical condition of patients may be affected which could negatively affect the efficacy and safety results for ISEMBYLD in the trial or reduce the amount of data or confound the data from this trial. Any delay or suspension of our clinical trials would significantly delay our clinical development programs.

Commercial use of ISEMBYLD also requires patients to receive an SMN2-targeted treatment. Accordingly, limitations on the availability of, or our patients' access to, these third-party therapies, including due to supply disruptions, safety concerns, regulatory restrictions or inadequate insurance coverage or reimbursement, could limit patients' ability to initiate or continue treatment with ISEMBYLD. We do not control the manufacture, supply, pricing or reimbursement of these therapies. Any resulting reduction in the number of patients able to receive ISEMBYLD could adversely affect its commercial adoption and sales. These clinical and commercial risks could harm our business, financial condition and results of operations.

The results or success of nonclinical studies and early-stage clinical trials of our product candidates may not be predictive of future results or replicated in later nonclinical studies or clinical trials of our product candidates in the same indications or other indications.

The results or success of nonclinical studies and early-stage clinical trials of our product candidates may not be predictive of future results or replicated in later nonclinical studies or later-stage clinical trials. Nonclinical studies and early-stage clinical trials are primarily designed to study PK and PD, understand the side effects of product candidates, and evaluate various doses and dosing schedules. Our current or future product candidates may demonstrate different chemical, biological and pharmacological properties in patients than they do in laboratory studies or may interact with human biological systems in unforeseen or harmful ways. Product candidates in later-stages of clinical trials may fail to show desired pharmacological properties or produce positive safety and efficacy results despite having progressed through nonclinical studies and early-stage clinical trials. Additionally, product candidates evaluated in one disease indication may interact in unforeseen or harmful ways in a patient population with a different disease indication than was previously studied. For example, apitegromab was evaluated in our Phase 3 SAPPHIRE clinical trial for the treatment of patients with Type 2 and Type 3 SMA and apitegromab was evaluated in our Phase 2 EMBRAZE proof-of-concept trial in combination with tirzepatide in obesity. Top-line data from each clinical trial have been released and apitegromab did not interact in any new ways despite the different patient population and disease indications studied in each trial. We cannot assure you that any future clinical trials of apitegromab, such as our Phase 2 FORGE clinical trial in patients with FSHD, or any of our other anti-myostatin antibodies will show positive results or interact in ways similar to when those antibodies were previously studied in different indications. There can be no assurance that any of our current or planned clinical trials will ultimately be successful or support further clinical development or registration of any of our product candidates.

Interim, initial, and preliminary results from our clinical trials that we announce or publish from time to time may change (e.g., from positive safety or efficacy results to poor or negative safety or efficacy results) as more patient data become available and are subject to additional audit, validation and verification procedures that could result in material changes in the final data.

Any interim, initial or preliminary data or results from clinical trials, including interim top-line results and other results published from our clinical trials may materially change as more patient data become available. Preliminary, initial, interim or top-line results also remain subject to audit, validation and verification procedures that may result in the final data being materially different from the data we previously published. As a result, interim, initial or preliminary data may not be predictive of final results and should be viewed with caution until the final data are available. We may also arrive at different conclusions, or considerations may qualify such results, once we have received and fully evaluated additional data. Differences between preliminary, initial or interim data and final data could adversely affect our business.

There is a high failure rate for drugs and biologics proceeding through clinical trials. A number of companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in late-stage clinical development even after achieving promising

results in earlier studies, and we cannot be certain that we will not face similar setbacks. Many drugs have failed to replicate efficacy and safety results in larger or more complex later stage trials. 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 regulatory approval. If we fail to produce positive results in our ongoing and planned nonclinical studies and clinical trials with apitegromab indications or SRK-439 or if a regulatory authority interprets and analyzes the results as not positive, the development timeline and regulatory approval and commercialization prospects for our product and product candidates, and, correspondingly, our business and financial prospects, may be materially adversely affected.

If we encounter difficulties enrolling patients in our clinical trials, our clinical development activities could be delayed or otherwise adversely affected.

We may experience difficulties in patient enrollment in our clinical trials for a variety of reasons. The timely completion of clinical trials in accordance with their protocols depends, among other things, on our ability to enroll a sufficient number of patients who remain in the trial until its conclusion. The enrollment of patients depends on many factors, including:

- the patient eligibility and exclusion criteria defined in the protocol;
- the size of the patient population required for analysis of the trial's primary endpoints;
- the willingness or availability of patients to participate in our trials;
- the number and location of participating trial sites;
- the proximity of patients to trial sites and any limitations on travel or access to trial sites;
- the design of the trial;
- our ability to recruit clinical trial investigators with the appropriate competencies and experience;
- clinicians' and patients' perceptions as to the potential advantages and risks of the product and product candidate being studied in relation to other therapies;
- our ability to obtain and maintain patient consents; and
- the risk that patients enrolled in clinical trials will drop-out of the trials before completion of their involvement in the study.

For example, we are developing apitegromab for the treatment of people with FSHD, a rare disease, affecting an estimated 30,000 people in the U.S. and Europe. As a result, we may encounter difficulties enrolling patients in our clinical trials for apitegromab in FSHD due, in part, to the small size of this patient population. In addition, our clinical trials will compete with other clinical trials for product candidates that are in the same therapeutic areas as our product and 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. Additionally, patients may opt out of participation in clinical trials in favor of treatment with FDA approved therapies, or therapies approved in the EU or other foreign jurisdictions.

Delays in patient enrollment 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 and product candidates.

We rely on third parties to conduct certain aspects of our clinical trials and certain aspects of our nonclinical studies and on commercial distributors to distribute our product. If these third parties do not successfully carry out their contractual duties or meet expected deadlines or comply with legal and regulatory requirements, we may be delayed or unable to receive regulatory approval of our product candidates or commercialize ISEMBYLD or any future product candidates, and our business could be materially harmed.

We depend upon third parties to conduct certain aspects of our nonclinical studies and to conduct our clinical trials, under agreements with universities, medical institutions, CROs, strategic partners and others. We often have to negotiate budgets and contracts with such third parties, and if we are unsuccessful or if the negotiations take longer than anticipated, this could result in delays to our development timelines and increased costs.

We rely especially heavily on third parties over the course of our clinical trials, and, as a result, have limited control over the clinical investigators and limited visibility into their day-to-day activities, including with respect to their individual employment policies or compliance with the approved clinical protocol. Nevertheless, we are responsible for ensuring that each of our trials is conducted in accordance with the applicable protocol, legal and regulatory requirements and scientific standards, and our reliance on third parties does not relieve us of our regulatory responsibilities. We and these third parties are required to comply with Good Clinical Practice ("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 and accurate and that the rights, integrity and confidentiality of trial participants are protected. Regulatory authorities enforce these GCP requirements through periodic inspections of trial sponsors, clinical investigators and trial sites. If we or any of these third parties fail to comply with applicable GCP requirements, the clinical data generated in our clinical trials may be deemed unreliable and the FDA or comparable foreign regulatory authorities may require us to suspend or terminate these trials or perform additional nonclinical studies or clinical trials before approving our marketing applications. We cannot be certain that, upon inspection, such regulatory authorities will determine that any of our clinical trials comply with the GCP requirements. We also are required to register certain ongoing clinical trials and post the results of completed clinical trials on a government-sponsored database, ClinicalTrials.gov, within specified timeframes. Failure to do so can result in civil monetary penalties, adverse publicity and civil and criminal sanctions. The FDA and National Institutes of Health have signaled the government's willingness to begin enforcing these registration and reporting requirements against non-compliant clinical trial sponsors. As a result, our financial results and the commercial prospects for our product and product candidates would be harmed, our costs could increase and our ability to generate revenue could be delayed.

Our failure or any failure by these third parties to comply with these regulations would delay the regulatory approval process. Moreover, our business may be implicated if any of these third parties violate federal or state fraud and abuse or false claims laws and regulations or healthcare privacy and security laws.

Any third parties conducting aspects of our nonclinical studies or clinical trials will not be our employees and, except for remedies that may be available to us under our agreements with such third parties, we cannot control whether they devote sufficient time and resources to our nonclinical studies and clinical trials. The third party CROs and clinical trial sites that conduct our clinical trials may experience staffing shortages and the inability of a CRO or clinical trial site to maintain appropriate levels of competent staffing to support the demands of our clinical trials could negatively impact the execution of our clinical trials. These third parties may also have relationships with other commercial entities, including our competitors, for whom they may also be conducting clinical trials or other product development activities, which could affect their performance on our behalf. If these third parties do not successfully carry out their contractual duties or obligations or meet expected deadlines if they need to be replaced or if the quality or accuracy of the nonclinical or clinical data they obtain is compromised due to the failure to adhere to our protocols or regulatory requirements or for other reasons, our development timelines, including clinical development timelines, may be extended, delayed or terminated and we may not be able to complete development of, receive regulatory approval of or successfully commercialize our product and product candidates.

If any of our relationships with these third-party CROs or others terminate, we may not be able to enter into arrangements with alternative CROs or other third parties or to do so on commercially reasonable terms. Switching or adding additional CROs involves additional cost and requires management time and focus. In addition, there is a natural transition period when a new CRO begins work. As a result, delays may occur, which can materially impact our ability to meet our desired development timelines. Though we carefully manage our relationships with our 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 prospects.

We also rely on third-party commercial distributors to distribute ISEMBYLD and may rely on these or other distributors for any products approved in the future. Our ability to successfully commercialize our product depend in part on these distributors' ability to fulfill orders accurately and on a timely basis, maintain appropriate inventory levels, comply with applicable storage, handling and distribution requirements, and provide timely and accurate inventory and sales information. We have limited control over these third parties, and their failure to perform their contractual obligations or comply with applicable legal and regulatory requirements, or disruptions to their operations, could interrupt product distribution, delay patient access, result in product loss or additional costs, and adversely affect our sales and revenue. If any of these relationships terminate or a distributor is unable or unwilling to continue distributing our product, we may not be able to establish alternative arrangements in a timely manner or on commercially reasonable terms, which could materially adversely affect our business, financial condition and results of operations.

We have received Orphan Drug designation from the FDA for apitegromab for the treatment of SMA and FSHD and the EC granted Orphan Medicinal Product designation to apitegromab for the treatment of SMA. We may seek Orphan Drug designation from regulatory authorities in other jurisdictions for apitegromab. We may not receive the requested designation or we may be unable to realize the benefits associated with Orphan Drug designation, including the potential for market exclusivity.

We have received Orphan Drug designation from the FDA for apitegromab for the treatment of SMA and FSHD, and following the EMA's Committee for Orphan Medicinal Products' positive opinion, the EC designated apitegromab as an orphan medicinal product for the treatment of SMA. Even if we receive orphan drug exclusivity, the benefit of that exclusivity may be limited if we seek approval for an indication broader than the orphan-designated indication or could be revoked under certain circumstances, for example if the FDA later determines that the request for designation was materially defective or that we are unable to assure sufficient quantities of the product to meet the needs of patients with the rare disease or condition. Further, even if we receive orphan drug exclusivity for a product, that exclusivity may not effectively protect the product from competition during the exclusivity period because different drugs with different active moieties can be approved for the same condition, and the same product can be approved for different uses. Also, in the U.S., even after an orphan drug is approved and receives orphan drug exclusivity, the FDA may subsequently approve another drug for the same condition if the FDA concludes that the latter drug is not the same drug, including because it has been shown to be clinically superior to the drug with exclusivity because it is safer, more effective or makes a major contribution to patient care. In the EU, a marketing authorization may be granted to a similar medicinal product to an authorized orphan product for the same orphan indication if:

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

See the sections of our Annual Report on Form 10-K for the year ended December 31, 2025 entitled, "Business — Government Regulation — US Biological Product Development — Orphan Drug Designation" and "Business – Government Regulation – European Union Drug Development — European Union Orphan Designation and Exclusivity."

The FDA may reevaluate the Orphan Drug Act and its regulations and policies. We do not know if, when, or how the FDA may change the orphan drug regulations and policies in the future, and it is uncertain how any changes might affect our business. Depending on what changes the FDA may make to its orphan drug regulations and policies, our business could be adversely impacted.

We have received Fast Track designation from the FDA for the treatment of FSHD, and PRIME designation from the EMA for apitegromab for the treatment of SMA. We may seek Fast Track designation or Breakthrough Therapy designation from the FDA or PRIME designation from the EMA for certain of our current and future products and product candidates, and we may not be successful in receiving such designations, or if received, such designation may not actually lead to a faster development or regulatory review or approval process.

We may seek Fast Track designation, Breakthrough Therapy designation or PRIME designation for certain of our product and product candidates.

In September 2026 the FDA granted Fast Track designation to apitegromab for the treatment of FSHD. 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 that the FDA would decide to grant it. Although the FDA has granted Fast Track designation for apitegromab in FSHD, we may not experience a faster development process, review or approval compared to conventional FDA procedures. The FDA may withdraw Fast Track designation if it believes that the designation is no longer supported by data from our clinical development program.

Designation as a breakthrough therapy is within the discretion of the FDA. Accordingly, even if we believe one of our product candidates meets the criteria for designation as a breakthrough therapy, the FDA may disagree and instead determine not to make such designation. In any event, the receipt of a Breakthrough Therapy designation for a product candidate may not result in a faster development process, review or approval compared to products considered for approval under conventional FDA procedures and does not assure ultimate approval by the FDA. In addition, even if one or more of our product candidates qualify as breakthrough therapies, the FDA may later decide that the products no longer meet the conditions for qualification and rescind the breakthrough designation. See the sections of our Annual Report on Form 10-K for the year ended December 31, 2025 entitled, "*Business — Government Regulation — US Biological Product Development — Expedited Development and Review Programs.*"

In March 2021, the EMA granted PRIME designation to apitegromab for the treatment of SMA. PRIME is a scheme provided by the EMA to enhance support for the development of medicines that target an unmet medical need. The receipt of PRIME designation for apitegromab for the treatment of SMA may not result in a faster development process, review or approval compared to products considered for approval under conventional regulatory agency procedures and does not assure ultimate approval by the EMA.

See the section of our Annual Report on Form 10-K for the year ended December 31, 2025 entitled, "*Business – Government Regulation – European Union Drug Development — European Union Expedited Review and Development.*"

Receiving and maintaining regulatory approval of apitegromab in one jurisdiction does not mean that we will be successful in receiving or maintaining regulatory approval of apitegromab in other jurisdictions.

Receiving and maintaining regulatory approval of apitegromab in one jurisdiction does not guarantee that we will be able to receive or maintain regulatory approval in any other jurisdiction, but a failure or delay in receiving regulatory approval in one jurisdiction may have a negative effect on the regulatory approval process in other jurisdictions. Even though the FDA has granted marketing approval of ISEMBYLD, or if it grants approval for apitegromab in any other indication, the EC, the competent authorities of EU Member States or comparable regulatory authorities in foreign jurisdictions may not approve the manufacturing, marketing and promotion of apitegromab in other countries. Approval procedures vary among jurisdictions and can involve requirements and administrative review periods different from, and greater than, those in the U.S., including additional nonclinical studies or clinical trials, as clinical trials conducted in one jurisdiction may not be accepted by regulatory authorities in other jurisdictions. In many jurisdictions outside the U.S., apitegromab must be approved for reimbursement before it can be approved for sale in that jurisdiction. In some cases, the price that we intend to charge for our products is also subject to approval.

We may also submit marketing applications in other countries. Regulatory authorities in jurisdictions outside of the U.S. have requirements for approval of apitegromab with which we must comply prior to marketing in those jurisdictions. Receiving foreign regulatory approvals and compliance with foreign regulatory requirements could result in significant delays, difficulties and costs for us and could delay or prevent the introduction of apitegromab in certain countries. If we fail to comply with the regulatory requirements in international markets and/or receive and retain applicable marketing approvals, our target market will be reduced and our ability to realize the full market potential of apitegromab will be harmed.

Even though we have received FDA approval for ISEMBYLD and even if our product candidates receive regulatory approval in other jurisdictions, 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 our products and product candidates.

Any product candidate for which we obtain marketing approval will be subject to ongoing regulatory requirements, including requirements related to manufacturing, labeling, packaging, storage, advertising, promotion, sampling, record-keeping, import, export, conduct of post-marketing studies and submission of safety, efficacy and other post-marketing information. The safety and efficacy profile of any product that receives approval will continue to be closely monitored by the FDA and comparable foreign regulatory authorities. In addition, we will be subject to continued compliance with current Good Manufacturing Practice ("cGMP") and GCP requirements for any clinical trials that we conduct post-approval.

Manufacturers and manufacturers' facilities are required to comply with extensive FDA, EU 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 periodic review and inspections to assess compliance with cGMP and adherence to commitments made in any BLA or other marketing 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 uses for which the product may be marketed or contain requirements for potentially costly post-market testing, including Phase 4 clinical trials and surveillance to monitor safety and efficacy. For example, as specified in the ISEMBYLD approval letter, we are required to establish a pregnancy safety study to collect and analyze information on pregnancy complications and birth outcomes for exposed pregnancies.

Later discovery of previously unknown problems with our products that receive approval, 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. Other potential consequences include, among other things:

- restrictions on the marketing or manufacturing, withdrawal of our product from the market or voluntary or mandatory product recalls;
- fines, warning letters, untitled letters or holds on clinical trials;
- refusal by the FDA 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; and
- permanent injunctions and consent decrees, including the imposition of civil or criminal penalties.

The FDA strictly regulates marketing, labeling, advertising, and promotion of products that are placed on the market. Products may be promoted only for their approved indications and in a manner consistent with their FDA-approved labeling. The FDA and other agencies actively enforce the laws and regulations prohibiting the promotion of unapproved uses and a company that is found to have improperly promoted unapproved uses may be subject to significant liability.

We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action, either in the U.S. or abroad. In addition, the U.S. Supreme Court's July 2024 decision to overturn established case law giving deference to regulatory agencies' interpretations of ambiguous statutory language has introduced uncertainty regarding the extent to which the FDA's regulations, policies and decisions may become subject to increasing legal challenges, delays, and/or changes. 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 face enforcement action and our business may be harmed.

If we are unable to establish sales and marketing capabilities or enter into agreements with third parties to sell and market apitegromab in the EU or other jurisdictions, we may not be successful in commercializing apitegromab if and when it is approved.

We have recently begun to build our sales or marketing infrastructure in Europe and we have limited experience in the sale, marketing or distribution of pharmaceutical products. To achieve commercial success for apitegromab, if approved, in markets for which we

retain sales and marketing responsibilities, we must continue to develop a sales and marketing organization and/or outsource certain functions to third parties, including third party distributors.

There are risks involved both with establishing our own sales and marketing capabilities and with entering into arrangements with third parties to perform these services. If the commercial launch of a product candidate for which we recruit a sales force and establish marketing capabilities is delayed or does not occur for any reason, we would have prematurely or unnecessarily incurred these commercialization expenses. This may be costly, 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 apitegromab on our own in Europe include:

- our ability to recruit and retain adequate numbers of effective sales and marketing personnel;
- the ability of sales personnel to obtain access to physicians or educate adequate numbers of physicians on the benefits of prescribing apitegromab; and
- unforeseen costs and expenses associated with creating an independent sales and marketing organization.

If we enter into arrangements with third parties to perform sales and marketing services, our product revenue or the profitability of these product revenue to us may be lower than if we were to market and sell apitegromab ourselves. In addition, we may not be successful in entering into arrangements with third parties to sell and market apitegromab or may be unable to do so on terms that are favorable to us. We may have little control over such third parties, and any of them may fail to devote the necessary resources and attention to sell and market apitegromab effectively. If we do not establish sales and marketing capabilities successfully, either on our own or in collaboration with third parties, we will not be successful in commercializing apitegromab.

Competing therapies may exist or could emerge that adversely affect the amount of revenue we are able to generate from the sale of ISEMBYLD or any of our future product candidates, if successfully developed and approved.

The biopharmaceutical industry is highly competitive. There are many public and private companies, universities, governmental agencies and other research organizations actively engaged in the research and development of products that may be similar to our product and product candidates or address similar markets. If we are successful in evaluating ISEMBYLD or developing other apitegromab indications, it is probable that the number of companies seeking to develop products and therapies similar to our product and product candidates or targeting similar indications will increase. Many of our potential competitors, alone or with their strategic partners, have substantially greater financial, technical and human resources than we do, and significantly greater experience in the discovery and development of product candidates, obtaining FDA and other regulatory approvals of treatments and the commercialization of those treatments. Mergers and acquisitions in the biotechnology and pharmaceutical industries may result in even more resources being concentrated among a smaller number of our competitors. We expect competition in the indications we are pursuing will focus on efficacy, safety, convenience, availability, and price. The commercial opportunity for ISEMBYLD could be reduced or eliminated if our competitors develop and commercialize products that are perceived to be safer, more effective, have fewer or less severe side effects, are more convenient or are less expensive than ISEMBYLD. Our competitors also may obtain FDA or other regulatory approval for their products more rapidly than we may obtain approval for ours, which could result in our competitors establishing a strong market position before we are able to enter the market.

Nonclinical development is uncertain. Our nonclinical programs may experience delays or may never advance to clinical trials, which would adversely affect our ability to develop our product pipeline and receive 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 our planned INDs in the U.S., or similar applications in other jurisdictions. We cannot be certain of the timely completion or outcome of our nonclinical studies or of the timing of any planned IND submission to the FDA or similar applications in other jurisdictions, and cannot predict if the FDA, EMA or other regulatory authorities will accept our proposed clinical programs or if the outcome of our nonclinical studies will ultimately support the further development of our programs. As a result, we cannot be sure that we will be able to submit INDs or similar applications for the clinical development of 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, the competent authorities and/or ethics committees in the EU Member States or other regulatory authorities allowing clinical trials to begin.

Conducting nonclinical testing can be a lengthy, time-consuming and expensive process. The time required for such testing may vary substantially according to the type, complexity and novelty of the program, and can be several years or more per program. Delays associated with programs for which we are conducting nonclinical testing and studies may cause us to incur additional operating expenses. We also may be affected by delays associated with the nonclinical testing and studies of certain programs that are the

responsibility of our collaborators or our potential future collaborators over which we have limited or no control. The commencement and rate of completion of nonclinical studies for a product candidate may be delayed by many factors, including, for example, challenges in reaching consensus with regulatory agencies regarding the scope of the necessary nonclinical study program and/or appropriate nonclinical study designs.

Risks Related to Our Business and Operations

Because we rely on a limited number of third-party manufacturing and supply partners, our supply of commercial product for ISEMBYLD and materials for our research and development, nonclinical and clinical development programs may become limited or interrupted or may not be of satisfactory quantity or quality.

We have limited experience manufacturing our product and product candidates on a commercial scale. We rely on a limited number of third-party contract manufacturers to manufacture all of our clinical trial product supplies and commercial product supplies, including drug substance, fill-finish, labeling, and packaging for ISEMBYLD and our product candidates. We do not own our own manufacturing facilities for producing any clinical trial or commercial product supplies. Our supply chain also depends on a limited number of suppliers for critical raw materials and components (including container closure systems), and any disruption, quality issue, or shortage could delay manufacturing or regulatory approvals. There can be no assurance that our nonclinical, clinical development, and, if approved, commercial product supplies will not be limited or interrupted due to impacts to our third-party contract manufacturers. For example, we rely on a single source supplier for the drug substance and fill-finish manufacture of apitegromab indications, SRK-181 and SRK-439. Our third-party manufacturing and supply partners may experience batch failures, lower-than-expected yields or other manufacturing inefficiencies, which could increase manufacturing costs, adversely affect gross margins, reduce available production capacity and, if persistent, impair our ability to maintain adequate commercial supply of ISEMBYLD. Any replacement of our current drug substance contract manufacturer or fill-finish contract manufacturer would require significant resources, lead time and expertise because there may be a limited number of qualified replacements. In addition, our ability to procure sufficient supplies for the development of apitegromab indications, SRK-181, SRK-439 or future product candidates could be impacted by factors outside of our control such as current macroeconomic and geopolitical events, the changing rates of inflation and interest rates and tariff policies. We have no direct control over our contract manufacturers' ability to maintain adequate quality control, quality assurance and qualified personnel. Furthermore, all of our third-party contract manufacturers supply and/or manufacture materials or products for other companies, which exposes our third-party contract manufacturers to regulatory risks for the production of such materials and products. As a result, failure to satisfy the regulatory requirements for the production of those materials and products may affect the regulatory clearance of our contract manufacturers' facilities generally.

For example, in September 2025, we received a CRL from the FDA citing observations identified during an FDA inspection of a third-party fill-finish facility used in our BLA and MAA for apitegromab. Although the observations were related to the facility and were not specific to apitegromab, we were required to identify and qualify an alternate fill finish facility and amend our regulatory submissions. As a result, we resubmitted our BLA and MAA to incorporate the alternate facility, which has caused delays in the regulatory review process.

The manufacturing process for a product or product candidate is subject to FDA and foreign regulatory authority review. Suppliers and manufacturers must meet applicable manufacturing requirements and undergo rigorous facility and process validation tests required by regulatory authorities in order to comply with regulatory standards, such as cGMP. In the event that any of our manufacturers fails to comply with such requirements or to perform its obligations to us in relation to quality, timing or otherwise, or if our supply of components or other materials becomes limited or interrupted for other reasons, we may be forced to internalize certain activities or rapidly onboard alternative third-party manufacturing capacity, for which we currently do not have the capabilities or resources, or enter into an agreement with another third-party, which we may not be able to do on reasonable terms, if at all. In some cases, the technical skills or technology required to manufacture our product and product candidates may be unique or proprietary to the original manufacturer and we may have difficulty transferring such skills or technology to another third-party and a feasible alternative may not exist. These factors would increase our reliance on the original manufacturer or require us to obtain a license from such manufacturer in order to have another third-party manufacture our product and product candidates. If we must change manufacturers for any reason, we may be required to qualify and validate a new manufacturer's facilities, processes and quality systems for compliance with applicable regulatory requirements, including cGMP. We may also need to conduct manufacturing comparability studies to demonstrate that any new manufacturing process produces product consistent with specifications previously submitted to the FDA or other regulatory authorities. These activities could result in delays and increased costs and could negatively affect our ability to develop and commercialize our product and product candidates in a timely manner or within budget.

We expect to continue to rely on third-party manufacturers for commercial supplies of drug substance, drug product, fill-finish, and packaged and labeled product for ISEMBYLD. We do not have long-term supply agreements in place with many of our contract manufacturers, and each batch for our product and product candidates is individually contracted through a purchase order governed by master service and quality agreements. If any of these manufacturers are unwilling to or unable to supply adequate quantities of

product on acceptable terms or timelines, or are unable to comply with cGMP or other applicable requirements, we may be required to qualify and onboard alternative suppliers, which could result in delays to clinical development, regulatory submissions or approvals, or commercialization.

Failure to maintain compliance with cGMP can result in regulatory action against our third-party manufacturers, including FDA sanctions, which could disrupt our manufacturing and supply chain and lead to delays in our clinical development programs, regulatory submissions or approvals, or commercial supply, if approved. In addition, delays in securing or maintaining manufacturing capacity for drug substance, fill-finish, labeling and packaging, or failures by our contract manufacturers or suppliers to perform as required, could delay clinical trials, regulatory filings, or commercial launches, which could negatively affect our business.

Our reliance on third parties, such as manufacturers, third-party logistics providers, specialty distributors, specialty pharmacies, and patient service providers, may subject us to risks and may cause us to undertake substantial obligations, including financial obligations.

To conduct later-stage clinical trials and support commercial supply of ISEMBYLD, we will need to manufacture our product and product candidates in larger quantities and at appropriate quality standards. We rely on third-party contract manufacturers to manufacture commercial supply of ISEMBYLD and our supply for our clinical programs. We and our manufacturing partners may be unable to maintain or increase manufacturing capacity in a timely or cost-effective manner, and quality control issues may arise during process scale-up, technology transfer, or commercial production. If we, or any manufacturing partners, are unable to manufacture our product and product candidates in sufficient quantity or quality, development activities, clinical trials, regulatory submissions and approvals, or commercialization and commercial supply could be delayed, disrupted, or become infeasible, which could materially affect our business.

Our reliance on third-party logistics providers, specialty distributors, specialty pharmacies, and patient services providers may subject us to risks that could impact the commercialization and patient access to ISEMBYLD. We expect to continue to use limited distribution agreements, which could concentrate supply with a small number of specialty pharmacies, increasing the risk of distribution disruptions, capacity constraints, and gaps in patient access if these partners fail to perform. Additionally, third parties manage high-touch patient support, reimbursement processing, and copay assistance, and any failures in these areas could lead to delays in initiation of treatment, coverage denials, and financial barriers for patients. Additionally, challenges such as disruptions in logistics, product-handling or quality-control issues, cybersecurity or privacy incidents, licensing or other regulatory non-compliance, or delays in onboarding key distribution partners may arise and may cause us to undertake substantial obligations, including financial obligations. If we or our third-party distribution service providers fail to scale and manage commercial distribution operations efficiently, the launch, commercialization, or continued supply of ISEMBYLD may be delayed or compromised, which could significantly harm our business, reputation, and financial results.

We are currently operating in a period of economic uncertainty and capital markets disruption, which has been significantly impacted by geopolitical instability, ongoing military conflicts, new regulatory activities and economic policies, high inflation and interest rates and the imposition of tariffs, other trade barriers and retaliatory countermeasures, any of which could have a material adverse effect on our business, financial condition and results of operations.

U.S. and global markets have recently been experiencing volatility and disruption caused by economic uncertainty, including as a result of geopolitical instability, ongoing military conflicts, new regulatory activities and economic policies, and high inflation and interest rates. We are continuing to monitor global capital markets and assessing the potential impact of these factors on our business, including the impact on the supply chains we rely on for the manufacture of ISEMBYLD, SRK-439, and any other current or future product candidates.

Although, to date, our business has not been materially impacted by the events described above, geopolitical tensions, or record inflation, it is impossible to predict the extent to which our operations will be impacted in the short and long term, or the ways in which such matters may impact our business. The extent and duration of such ongoing military conflicts, geopolitical tensions, record inflation and resulting market disruptions are impossible to predict but could be substantial. Any such disruptions may also magnify the impact of other risks we face.

Further, there have been, and may continue to be, significant changes to U.S. trade policies, sanctions, legislation, treaties and tariffs, including, but not limited to, trade policies and tariffs affecting products from outside of the U.S. The extent and duration of increased tariffs and the resulting impact on general economic conditions and on our business are uncertain and depend on various factors, such as negotiations between the U.S. and affected countries, the responses of other countries or regions, exemptions or exclusions that may be granted, availability and cost of alternative sources of supply, and demand in affected markets. While many of our supply chain and manufacturing activities occur within the U.S., we rely on globally sourced raw materials, components, and consumables. As a result, changes in trade policies, including the imposition of tariffs, export restrictions, or other geopolitical restrictions could result in increased costs or cause delays in the procurement of necessary materials or services. Any such disruptions in the global supply chain

caused by macroeconomic events and conditions could adversely affect the development, testing and clinical trials of apitegromab, SRK-181, SRK-439, and any future product candidates, or it may be delayed or infeasible, and regulatory approval or commercial launch of any resulting product may be delayed or not obtained, which could significantly harm our business.

We will need to continue to grow our organization in certain areas, including our personnel, systems and relationships with third parties, in order to develop and potentially commercialize our product and product candidates, and we may experience difficulties in managing this growth.

As our clinical development plans and commercialization strategies continue to develop and expand, we expect we will need to hire additional managerial, clinical development, scientific, regulatory, commercial, and administrative personnel. Our ability to compete in the highly competitive biotechnology industry depends upon our ability to attract and retain highly qualified specialized personnel. As ISEMBYLD approaches commercialization globally, we will also need to hire sales, marketing and other commercial personnel in the new markets we plan to enter. 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 regulatory review process for apitegromab indications, SRK-439, and any future product candidates, while complying with our contractual obligations to 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 ISEMBYLD, if approved in jurisdictions outside the U.S., 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 third parties, advisors and consultants to provide certain services, including CROs, contract manufacturers and companies focused on antibody development and discovery activities. There can be no assurance that the services of third parties, 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, accuracy or quantity of the services provided is compromised for any reason, our nonclinical studies and clinical trials may be extended, delayed or terminated, and we may not be able to receive, or may be substantially delayed in receiving, regulatory approval of additional apitegromab indications, SRK-181, SRK-439 or future product candidates or otherwise advance our business. There can be no assurance 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.

We may not be able to attract or retain qualified management and scientific personnel in the future due to the intense competition for a limited number of qualified personnel in the biopharmaceutical space. In this highly competitive market, there may be increased costs to attract and retain qualified personnel. Many of the other pharmaceutical companies that we compete against for qualified personnel have greater financial resources, different risk profiles and a longer history in the industry than we do. They also may provide more diverse opportunities and better chances for career advancement. Some of these characteristics may be more appealing to high quality candidates than what we have to offer. If we are not able to offer competitive compensation or appealing opportunities for high quality candidates, we may not be able to attract or retain qualified candidates and personnel. If we are not able to effectively expand our organization by hiring 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 ISEMBYLD and apitegromab in other indications and, accordingly, may not achieve our research, development and commercialization goals.

In addition, from time to time, there may be changes in our executive or senior management team that may be disruptive to our business. If our teams fail to execute our plans and strategies, including as a result of executive transitions, our business, financial condition, and results of operations could be adversely affected.

Our executives and highly skilled technical and managerial personnel are critical to our business. If we have transition in management, lose key personnel, or if we fail to recruit additional highly skilled personnel, our ability to further develop and commercialize ISEMBYLD and any of our other product candidates, if approved, and identify and develop new or next generation product candidates may be impaired.

Our performance substantially depends on the performance of our management team. Any transition or loss of the services of any of our executives or highly skilled technical and managerial personnel could have a disruptive impact on our ability to implement our

strategy and impede the achievement of our research, development and commercialization objectives. In addition, these transitions or departures could cause us to incur increased operating expenses, divert senior management resources in searching for replacements, or otherwise have a material adverse effect on our business, internal controls, financial condition and results of operations. Management transition inherently causes some loss of institutional knowledge, which can negatively affect strategy and operational execution during this phase. If we have additional changes to our executives or highly skilled technical and managerial personnel, we may be unable to successfully manage and grow our business, and our results of operations, execution of corporate goals, internal controls and financial condition could suffer as a result. The unplanned loss of the services of our executives or other personnel also could harm our reputation.

Our internal computer systems, or those used by our contract research organizations, or other contractors or consultants, may fail or suffer security breaches, incidents or compromises.

We have outsourced significant parts of our IT and business infrastructure to third-party providers, and we currently use these providers to perform business critical IT and business services for us. Despite the implementation of security measures, our computer systems, whether they are managed by us directly or by the third parties with whom we contract, and those of our existing and future CROs, and other contractors and consultants are vulnerable to damage from computer viruses and unauthorized access. While we have not experienced any such material system failure or security breach to date, if such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our development programs and our business operations. Our increased reliance on personnel working from home may increase our cyber security risk, create data accessibility concerns, and make us more susceptible to workforce and communication disruptions, any of which could adversely impact our business operations or delay necessary interactions with local and federal regulators, ethics committees, manufacturing sites, research or clinical trial sites and other agencies and contractors. 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. Likewise, we rely on third parties for the manufacture of ISEMBYLD, SRK-181 and SRK-439 and to conduct nonclinical studies and 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 breach were to result in a loss of, or damage to, our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability and the further development and commercialization of apitegromab in other indications, SRK-181, SRK-439 and future product candidates could be delayed.

As a company that uses IT systems, our systems may be subject to cyber-attacks, incidents or compromises. Due to the nature of some of these attacks, there is a risk that they may remain undetected for a period of time. While we have invested in the protection of data and information technology, our efforts may not prevent service interruptions or security breaches (e.g., ransomware attacks and social engineering attacks (phishing)). Like other companies in our industry, we, and our third-party vendors, have experienced threats and cybersecurity incidents relating to our information technology systems and infrastructure. We maintain cyber liability insurance; however, this insurance may not be sufficient to cover the financial, legal, business, or reputational losses, including regulatory fines, that may result from an interruption or breach of our systems.

Our employees, independent contractors, consultants, commercial partners, vendors and distributors may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements.

We are exposed to the risk of employee fraud or other illegal activity by our employees, independent contractors, consultants, commercial partners and vendors. Misconduct by these parties could include intentional, reckless and/or negligent conduct that fails to comply with the laws and regulations of the FDA, EU Member States, EMA, MHRA, PMDA and other similar foreign regulatory bodies; provide true, complete and accurate information to the FDA, EMA, PMDA and other similar foreign regulatory bodies; comply with manufacturing standards we have established; comply with healthcare fraud and abuse laws in the U.S. and similar foreign fraudulent misconduct laws; engage in appropriate sales, marketing, pricing, reimbursement, patient support, product distribution, import/export, anti-corruption or other commercial activities in connection with ISEMBYLD or apitegromab or report financial information or data accurately or to disclose unauthorized activities to us. Now that we have received FDA approval for ISEMBYLD, and if and when we receive EC approval or approval from other foreign regulatory authorities of apitegromab and begin commercializing in such other jurisdictions, our potential exposure under such laws will increase significantly, and our costs associated with compliance with such laws are also likely to increase. These laws may impact, among other things, our current activities with principal investigators and research patients, as well as proposed and future sales, marketing and education programs. We have adopted a code of business conduct and ethics, but it is not always possible to identify and deter misconduct by our employees, independent contractors, consultants, commercial partners and vendors, 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. If any actions are instituted against us and we are not successful in defending ourselves or asserting our rights, those actions could result in the imposition of civil, criminal and administrative penalties, damages, monetary fines, individual imprisonment, disgorgement, possible exclusion from participation in government healthcare programs, additional reporting obligations and oversight if we become subject to a corporate integrity agreement or other agreement to resolve allegations of non-compliance with these laws, contractual damages,

reputational harm, diminished profits and future earnings and the curtailment of our operations, any of which could adversely affect our ability to operate our business, financial condition and results of operations.

As part of our commercialization strategy, particularly in smaller jurisdictions outside the U.S., we may engage third-party distributors, commercialization partners, service providers and other agents to market, sell, distribute or otherwise support access to our products. Although we intend to conduct appropriate diligence and maintain contractual and compliance controls over such third parties, we may not be able to effectively monitor or control all of their activities. If any such third party engages in conduct that violates applicable healthcare, anti-corruption, anti-kickback, false claims, privacy, trade, sanctions, competition, pricing and reimbursement or other laws and regulations, or otherwise fails to comply with applicable regulatory requirements, we could be subject to investigations, enforcement actions, fines, penalties, reputational harm, contractual liability, restrictions on commercialization activities or other adverse consequences, even if we were not aware of, did not authorize or did not directly participate in such conduct.

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

Changes in statutes, regulations or the interpretation of existing statutes or regulations could impact our business in the future by requiring, for example: (i) changes to our manufacturing arrangements; (ii) additions or modifications to product labeling; (iii) the recall or discontinuation of our products; (iv) additional record-keeping requirements; or (v) changes to our pricing arrangements, or coverage of or reimbursement for our products. If any such changes were to be imposed, they could adversely affect the profitability and operation of our business. See the sections of our Annual Report on Form 10-K for the year ended December 31, 2025 entitled, "*Business - Government Regulation - Current and Future Healthcare Reform Legislation" and "Business - Government Regulation - Coverage and Reimbursement.*"

It is possible healthcare reform measures may be adopted in the future that may result in additional reductions in Medicare or other healthcare funding, more rigorous coverage criteria, or new payment methodologies or otherwise affect the prices we may obtain for any of our product and product candidates for which we may receive regulatory approval. Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from commercial payors. We cannot predict the reform initiatives that may be adopted in the future or whether initiatives that have been adopted will be modified or invalidated. The continuing health care reform initiatives of the government, insurance companies, managed care organizations and other payers of health care services to contain or reduce costs of health care may adversely affect the demand for any product and product candidates for which we may obtain regulatory approval, our ability to set a price that we believe is fair for our products, our ability to obtain coverage and reimbursement approval for a product, our ability to generate revenues and achieve or maintain profitability; and the level of taxes that we are required to pay.

In addition, federal and state efforts to impose new restrictions on manufacturer and third-party patient assistance programs could limit the availability or scope of these support services. Any such restrictions could reduce patient access to our products, which may materially and adversely affect our sales, business, and financial condition. Furthermore, patient support programs continue to face increased scrutiny from enforcement authorities. It is possible that regulators could determine that certain aspects of our programs do not comply with applicable healthcare fraud and abuse laws, including by viewing certain support services as improper inducements. Any such determination could have a significant adverse impact on our business, including the imposition of civil, criminal, or administrative penalties, and, in severe cases, exclusion from participation in federal and state healthcare programs.

On April 1, 2025, the Bureau of Industry and Security of the U.S. Department of Commerce initiated a Section 232 investigation on Pharmaceuticals and Pharmaceutical Ingredients, whose scope includes, but is not limited to, finished drug products, medical countermeasures, active pharmaceutical ingredients, starting materials, and derivative products of those items. This Section 232 investigation could result in substantial tariffs on these and related items, which could negatively impact our costs of production and operating margins. On October 31, 2025, the Centers for Medicare and Medicaid Services ("CMS") issued its final rule for the calendar-year Physician Fee Schedule ("BFSF Certification Final Rule"), which requires manufacturers, as of January 1, 2026, to obtain certifications from their third-party vendors confirming that Bona Fide Service Fees ("BFSFs") associated with Part B drug sales are not passed through, in whole or in part, to any client or customer, regardless of whether that entity takes title to the drug. In addition to submitting these certifications to CMS, manufacturers must maintain detailed documentation supporting the reasonable assumptions used to calculate Average Sales Price ("ASP"), including the methodologies used to classify BFSFs for each contract. These new requirements may increase the risk that certain fees could be reclassified as price concessions, which would negatively affect a product's ASP. Any such reclassification could reduce future reimbursement for our product(s) under Medicare Part B, which may materially and adversely impact our revenue.

Federal legislative and regulatory efforts to implement reference pricing or most-favored-nation pricing models could impact our product revenues and materially harm our business.

On May 12, 2025, President Trump issued an executive order calling on pharmaceutical manufacturers to voluntarily reduce the prices of medicines in the U.S. and directing the Secretary of the Department of Health and Human Services ("HHS") to communicate Most Favored Nation ("MFN") price targets to pharmaceutical manufacturers to align prices with those in comparably developed nations and, in the event significant progress towards MFN pricing is not delivered, to propose rulemaking to impose MFN pricing.

Since the May 12, 2025 order, the Trump administration has continued to exert pressure on drug manufacturers to implement MFN pricing, including by suggesting that the administration may impose significant tariffs on pharmaceuticals if such manufacturers do not reach agreements to implement MFN pricing. In December 2025, the CMS announced proposals for new mandatory demonstration payment models through two proposed rules under its CMS Innovation Center ("CMMI") authority: the Global Benchmark for Efficient Drug Pricing Model ("GLOBE") for Medicare Part B and the Guarding U.S. Medicare Against Rising Drug Costs ("GUARD") for Medicare Part D. On October 2, 2026, CMS finalized the GLOBE regulation, effective November 30, 2026, as a mandatory CMMI payment model under Section 1115A of the Social Security Act. As finalized, GLOBE will require manufacturers of specified single source drugs and sole source biologics, within designated therapeutic classes and that have incurred more than $100 million of Medicare Part B spending over a 12-month period to pay incremental rebates based on an alternative, internationally benchmarked calculation of the existing Medicare Part B drug inflation rebate. The GLOBE Model will begin on January 1, 2027 with the collection of voluntary manufacturer-submitted international drug net pricing data, and will be tested over five performance years, running from April 1, 2027 through March 31, 2032. The associated payment period will run from April 1, 2027 through March 31, 2034. GLOBE excludes orphan-only drugs, cell and gene therapy products, plasma-derived products, and drugs with an effective Medicare-negotiated maximum fair price.

GUARD remains a proposed regulation that has not been finalized and, as proposed, would similarly mandate manufacturer rebates for qualifying sole source drugs where the Medicare net price exceeds an MFN benchmark derived from international reference pricing methodologies, with its performance period proposed to begin in 2027. The GLOBE model and the proposed GUARD model will likely be subject to legal challenges that could delay implementation or modify their impact on manufacturer pricing and revenue. Additionally, in November 2025, CMS introduced the GENErating cost Reductions for U.S. Medicaid ("GENEROUS") Model, a voluntary MFN framework for manufacturers participating in the Medicaid Drug Rebate Program. Although it is voluntary, the GENEROUS Model could also impact the drug pricing landscape for manufacturers.

These reforms remain subject to change, potential legal challenges, or expansion through additional rulemaking or sub-regulatory guidance, creating uncertainty for our overall pricing strategy. It remains to be seen whether and how these drug pricing initiatives will apply to our product and product candidates, how they will affect the broader pharmaceutical industry, and whether similar reform measures may be adopted in the future.

Our relationships with healthcare providers and physicians and third-party payors will be subject to applicable anti-kickback, fraud and abuse and other healthcare laws and regulations, which could expose us to criminal sanctions, civil penalties, contractual damages, reputational harm and diminished profits and future earnings.

Healthcare providers, physicians and third-party payors in the U.S. and elsewhere play a primary role in the recommendation and prescription of pharmaceutical products. Arrangements with third-party payors and customers can expose pharmaceutical manufacturers to broadly applicable fraud and abuse and other healthcare laws and regulations, including, without limitation, the federal Anti-Kickback Statute and the federal False Claims Act, which may constrain the business or financial arrangements and relationships through which such companies sell, market and distribute pharmaceutical products. In particular, the research of our product and product candidates, as well as the promotion, sales and marketing of healthcare items and services, as well as certain business arrangements in the healthcare industry, are subject to extensive laws designed to prevent fraud, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, structuring and commission(s), certain customer incentive programs and other business arrangements generally. Activities subject to these laws also involve the improper use of information received in the course of patient recruitment for clinical trials. See the section in our Annual Report on Form 10-K for the year ended December 31, 2025 entitled "*Business – Government Regulation – Other Healthcare Laws.*"

It is possible that governmental and enforcement authorities will conclude that our business practices, or those of third parties acting on our behalf, may not comply with current or future statutes, regulations or case law interpreting applicable fraud and abuse or other healthcare laws and regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, including the imposition of civil, criminal and administrative penalties, damages, fines, disgorgement, individual imprisonment, possible exclusion from participation in federal and state funded healthcare programs, contractual damages and the curtailment or restricting of our operations, as well as additional reporting obligations and oversight if we become subject to a corporate integrity agreement or other agreement to resolve allegations of non-compliance with these laws. Any action for violation of these laws, even if successfully defended, could cause a

pharmaceutical manufacturer to incur significant legal expenses and divert management's attention from the operation of the business. Prohibitions or restrictions on sales or withdrawal of future marketed products could materially affect business in an adverse way.

If we fail to comply with our reporting and payment obligations under the Medicaid Drug Rebate program or other governmental pricing programs, we could be subject to additional reimbursement requirements, penalties, sanctions and fines, which could have a material adverse effect on our business, financial condition, results of operations and growth prospects.

We participate in the Medicaid Drug Rebate Program, the 340B program, the U.S. Department of Veterans Affairs, Federal Supply Schedule ("FSS") pricing program, and the Tricare Retail Pharmacy program, which require us to disclose average manufacturer pricing, and, in the future may require us to report the average sales price for certain of our drugs to the Medicare program. Pricing and rebate calculations vary across products and programs, are complex, and are often subject to interpretation by us, governmental or regulatory agencies and the courts. Furthermore, regulatory and legislative changes, and judicial rulings relating to these programs and policies (including coverage expansion), have increased and will continue to increase our costs and the complexity of compliance, have been and will continue to be time-consuming to implement, and could have a material adverse effect on our results of operations, particularly if CMS or another agency challenges the approach we take in our implementation. For example, in the case of our Medicaid pricing data, if we become aware that our reporting for a prior quarter was incorrect or has changed as a result of recalculation of the pricing data, we are generally obligated to resubmit the corrected data for up to three years after those data originally were due. Such restatements increase our costs and could result in an overage or underage in our rebate liability for past quarters. Price recalculations also may affect the ceiling price at which we are required to offer our products under the 340B program and give rise to an obligation to refund entities participating in the 340B program for overcharges during past quarters impacted by a price recalculation.

Civil monetary penalties can be applied if we are found to have knowingly submitted any false price or product information to the government, if we are found to have made a misrepresentation in the reporting of our average sales price, if we fail to submit the required price data on a timely basis, or if we are found to have charged 340B covered entities more than the statutorily mandated ceiling price. Additionally, our agreement to participate in the 340B program or our Medicaid drug rebate agreement could be terminated, in which case federal payments may not be available under Medicaid or Medicare Part D for our covered outpatient drugs. Additionally, if we overcharge the government in connection with our arrangements with FSS or Tricare Retail Pharmacy, we are required to refund the difference to the government. Failure to make necessary disclosures and/or to identify contract overcharges can result in allegations against us under the False Claims Act ("FCA") and other laws and regulations. Unexpected refunds to the government, and responding to a government investigation or enforcement action, would be expensive and time-consuming, and could have a material adverse effect on our business, financial condition, results of operations and growth prospects.

Further, legislation may be introduced that, if passed, would, among other things, further expand the 340B program to additional covered entities or would require participating manufacturers to agree to provide 340B discounted pricing on drugs used in an inpatient setting, and any additional future changes to the definition of average manufacturer price or the Medicaid rebate amount could affect our 340B ceiling price calculations and negatively impact our results of operations. Additionally, certain pharmaceutical manufacturers are involved in ongoing litigation regarding contract pharmacy arrangements under the 340B program. The outcome of those judicial proceedings and the potential impact on the way in which manufacturers extend discounts to covered entities through contract pharmacies remain uncertain.

Failure to comply with health care privacy and data protection laws and regulations could lead to government or competent authority enforcement actions (which could include civil or criminal penalties), private litigation, and/or adverse publicity and could negatively affect our operation results and business.

We, our CROs, and any potential collaborators may be subject to strict and changing federal, state, and foreign data protection laws and regulations (i.e., laws and regulations that address privacy and data security) and policies and contractual obligations related to data privacy and security. In the U.S., numerous federal and state laws and regulations, including federal health information privacy laws, state data breach notification laws, state health information privacy laws, and federal and state consumer protection laws (e.g., Section 5 of the Federal Trade Commission Act), that govern the collection, use, disclosure and protection of health-related and other personal information could apply to our operations or the operations of our CROs and collaborators. In addition, we may 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 the Health Insurance Portability and Accountability Act of 1996 ("HIPAA"), as amended by the Health Information Technology for Economic and Clinical Health Act of 2009. Depending on the facts and circumstances, we could be subject to civil, criminal, and administrative penalties if we knowingly obtain, use, or disclose individually identifiable health information maintained by a HIPAA-covered entity in a manner that is not authorized or permitted by HIPAA.

Compliance with U.S. and international data protection laws and regulations could require us to take on more onerous obligations in our contracts, restrict our ability to collect, use and disclose data, or in some cases, impact our ability to operate in certain jurisdictions. Failure to comply with these laws and regulations could result in government 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.

We have conducted clinical trials and are currently conducting ONYX, our long-term extension clinical trial of apitegromab and OPAL, our Phase 2 clinical trial of apitegromab in children under the age of two living with SMA, in the EEA and the UK, and may conduct future clinical trials in the EEA or the UK. We are therefore subject to European privacy laws, where we collect and use personal data including health related data, in connection with our clinical trials in the EEA, or the UK, including the EU General Data Protection Regulation (the "EU GDPR"), the UK General Data Protection Regulation (the "UK GDPR") (collectively referred to as the "GDPR"), as well as other national data protection legislation in force in the relevant EEA Member States and the UK (including the UK Data Protection Act of 2018), which govern the collection, use, storage, disclosure, transfer, or other processing of personal data (including health data processed in the context of clinical trials) regarding individuals in the EEA and UK. The GDPR imposes a broad range of strict requirements on companies that process personal data, including requirements relating to having legal bases and conditions for processing personal data and transferring such personal data outside the EEA or the UK, including to the U.S., providing details to those individuals regarding the processing of their personal information and, when relevant, data transfer agreement or other transfer mechanism in place for transfer of personal data from Europe to the US, keeping personal information secure, having data processing agreements with third parties who process personal information, responding to individuals' requests to exercise their rights in respect of their personal information, where required reporting security breaches involving personal data to the competent national data protection authority and affected individuals, where required, appointing data protection officers, where required conducting data protection impact assessments for high risk processing, and record-keeping. The GDPR imposes penalties in the event of non-compliance, including fines of up to 20.0 million Euros (17.5 million GBP for the UK) or up to 4% of our total worldwide annual turnover for more serious offenses. The GDPR also confers a private right of action on data subjects and consumer associations to lodge complaints with supervisory authorities, seek judicial remedies, and obtain compensation for damages resulting from violations of the GDPR.

The GDPR also imposes strict rules on the transfers of personal data outside of the EEA or the UK to third countries, such as the US in certain circumstances, unless a derogation exists or a valid GDPR transfer mechanism (for example, the EC approved standard contractual clauses ("SCCs") and the UK International Data Transfer Agreement/Addendum ("UK IDTA")). Where relying on the SCCs or UK IDTA for data transfers, we may also be required to carry out transfer impact assessments to assess whether the recipient is subject to local laws which allow public authority access to personal data. The complexity and the additional contractual burden increases our overall risk exposure. There may be further divergence on international transfer safeguards in the future, including with regard to administrative burdens. Any inability to transfer personal data from the UK and EEA to the US (and other third countries) in compliance with data protection laws may adversely affect our operations and our business and financial position. The UK data protection regime is independent from but currently still aligned with the EEA's data protection regime. However, going forward, there will be increasing scope for divergence in application, interpretation and enforcement of the data protection law as between the UK and EEA. Although the UK is regarded as a third country under the EU GDPR, the EC has issued a decision recognizing the UK as providing adequate protection under the EU GDPR and, therefore, transfers of personal data originating in the EEA to the UK remain unrestricted. Similarly, the UK government has confirmed that personal data transfers from the UK to the EEA remain free flowing. However, the UK Data (Use and Access) Act 2025 now in force, may further differentiate the UK's data protection regimes and could potentially impact the UK's adequacy decision granted by the EC. The respective provisions and enforcement of the EU GDPR and UK GDPR may continue to diverge, creating additional regulatory challenges and uncertainty. This evolving regulatory landscape could increase legal risk, complexity and cost to our handling of personal data, and may require us to adapt our privacy and data security compliance programs to account for increasing legal and regulatory divergence between the UK and the EEA.

Further, EU Member States have adopted implementing national laws to implement the EU GDPR which may partially deviate from the EU GDPR and the competent authorities in the EU Member States may interpret EU GDPR obligations slightly differently from country to country, so that we do not expect to operate in a uniform legal landscape in the EU. Also, as it relates to processing and transfer of genetic data, the EU GDPR specifically allows national laws to impose additional and more specific requirements or restrictions, and European laws have historically differed quite substantially in this field, leading to additional uncertainty.

The GDPR increases our responsibility and liability in relation to personal data that we process where such processing is subject to the GDPR. While we've taken steps to comply with the GDPR, including as implemented by individual countries, we face uncertainty as to the exact interpretation of these requirements and we may be unsuccessful in implementing all measures required by data protection authorities or courts in interpretation of the law. Compliance with the GDPR is a rigorous and time-intensive process that may increase our cost of doing business or require us to change our business practices, and despite those efforts, there is a risk that we may be subject to fines and penalties, litigation, and reputational harm in connection with our European activities.

In addition, in the United States, many states in which we operate have laws that protect the privacy and security of sensitive and personal information. Certain state laws may be more stringent or broader in scope, or offer greater individual rights, with respect to sensitive and personal information than federal, international or other state laws, and such laws may differ from each other, which may

complicate compliance efforts. Where state laws are more protective than HIPAA, we must comply with the state laws we are subject to, in addition to HIPAA. In certain cases, it may be necessary to modify our planned operations and procedures to comply with these more stringent state laws. Further, in some cases where we process sensitive and personal information of individuals from numerous states, we may find it necessary to comply with the most stringent state laws applicable to any of the information. For example, the CCPA creates comprehensive individual privacy rights for California consumers (as defined in the law) and places increased privacy and security obligations on entities handling personal data of consumers or households. While there are currently exceptions for protected health information that is subject to HIPAA and clinical trial regulations, as currently written, the CCPA, as amended by the California Privacy Rights Act, and other enacted or proposed comprehensive state consumer privacy legislation may impact our business activities. Furthermore, certain states have passed or are considering laws that are specifically focused upon health privacy, such as Washington's My Health My Data Act. The My Health My Data Act imposes new state restrictions and requirements on the processing and sale of consumer health data and creates a private right of action, which further increases the relevant compliance risk. Connecticut and Nevada have also passed similar laws regulating consumer health data. The effects of state and federal privacy laws are potentially significant and may require us to modify our data processing practices and policies and to incur substantial costs and potential liability in an effort to comply with such legislation. We continue to monitor the impact that the state consumer privacy and protection laws, like the CCPA and the My Health My Data Act, may have on our business activities. See the section in our Annual Report on Form 10-K for the year ended December 31, 2025 entitled "Business – Government Regulation – European General Data Protection Regulation" and "*Business – Government Regulation – Other Healthcare and Privacy Laws.*"

Artificial intelligence presents risks and challenges that can impact our business including by posing security risks to our confidential information, proprietary information, and personal data.

The potential use of new and evolving technologies, such as artificial intelligence, in our offerings to employees may result in additional spending and present risks and challenges that can impact our business including by posing security and other risks to our confidential information, proprietary information and personal information, and as a result we may be exposed to reputational harm, legal liability, and regulatory investigations and fines.

We may build and integrate artificial intelligence into our offerings, and this innovation may present risks and challenges that could affect its adoption, and therefore our business. If we enable or offer solutions that draw controversy due to perceived or actual negative societal impact, we may experience brand or reputational harm, competitive harm or legal liability. The use of certain artificial intelligence technology can give rise to intellectual property risks, including compromises to proprietary intellectual property and intellectual property infringement, for example where third-party data sources are used to train artificial intelligence models, or the output of artificial intelligence systems reproduce or incorporate third party intellectual property rights, in each case without the right to do so.

Additionally, we expect to see increasing government and supranational regulation related to artificial intelligence use and ethics, which may also significantly increase the burden and cost of research, development and compliance in this area. For example, the EU's Artificial Intelligence Act ("AI Act"), the world's first comprehensive AI law, entered into force on August 1, 2024, and most provisions of which will become effective on August 2, 2026 although the application of certain requirements for high-risk AI systems has been deferred until December 2, 2027 (or for certain product-embedded AI systems, August 2, 2028). This legislation imposes tiered obligations on providers and deployers of artificial intelligence systems which are put onto the EU market, or where the output is intended for use in the EU market, depending on the risk classification of the AI system, and encourages providers and deployers of artificial intelligence systems to account for EU fundamental rights in their development and use of these systems. If we develop or use AI systems that are governed by the AI Act, it may necessitate ensuring higher standards of data quality, transparency, and human oversight, and if the AI systems are considered high risk, we would be required to implement substantive risk and quality management systems and post-market monitoring systems and adhere to specific and burdensome and costly ethical, accountability, and administrative requirements. Other jurisdictions, including the U.S. and UK, are also taking steps to regulate AI systems. The rapid evolution of artificial intelligence will require the application of significant resources to design, develop, test and maintain our service offerings to help ensure that artificial intelligence is implemented in accordance with applicable law and regulation and in a socially responsible, safe and ethical manner and to minimize any real or perceived unintended harmful impacts.

Our vendors may in turn incorporate artificial intelligence tools into their own offerings, and the providers of these artificial intelligence tools may not meet existing or rapidly evolving regulatory or industry standards, including with respect to privacy and data security. Further, bad actors around the world use increasingly sophisticated methods, including the use of artificial intelligence, to engage in illegal activities involving the theft and misuse of personal information, confidential information and intellectual property. Any of these effects could damage our reputation, result in the loss of valuable property and information, cause us to breach applicable laws and regulations, and adversely impact our business. A risk of our proprietary intellectual property rights being compromised through the use of artificial intelligence could arise through third party vendors using our data to train their models and/or to generate output for other users of their systems. There is also a risk (as with any hosted service) of security incidents occurring that could lead to unauthorized access to our data. In the event that personal data (including special category data relating to patients) were to be compromised, we may also face action from regulators and affected data subjects, and damage to our reputation.

Additional laws and regulations governing international operations, including certain U.S. and foreign anti-corruption, anti-money laundering, export control, sanctions, and other trade laws and regulations, could negatively impact or restrict our operations.

If we further expand our operations outside of the U.S., we must dedicate additional resources to comply with numerous laws and regulations in each jurisdiction in which we plan to operate. The Foreign Corrupt Practices Act ("FCPA") prohibits any U.S. individual or business from paying, offering, authorizing payment or offering of 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 U.S. 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 for international operations.

Compliance with the FCPA is expensive and difficult, particularly in countries in which corruption is a recognized problem. In addition, the FCPA presents particular challenges in the pharmaceutical industry, because, in many countries, hospitals are operated by the government, and doctors and other hospital employees are considered foreign officials. Certain payments to hospitals in connection with clinical trials and other work have been deemed to be improper payments to government officials and have led to FCPA enforcement actions.

Various laws, regulations and executive orders also restrict the use and dissemination outside of the U.S., or the sharing with certain non-U.S. nationals, of information classified for national security purposes, as well as certain products and technical data relating to those products. If we expand our presence outside of the U.S., it will require us to dedicate additional resources to comply with these laws, and these laws may preclude us from developing, manufacturing, or selling certain products and product candidates outside of the U.S., which could limit our growth potential and increase our development costs.

The failure to comply with laws governing international business practices may result in substantial civil and criminal penalties and suspension or debarment from government contracting. The SEC also may suspend or bar issuers from trading securities on U.S. exchanges for violations of the FCPA's accounting provisions.

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, and as a result, may be subject to lengthy and expensive litigation and excessive damages and we may not have, or be able to obtain, sufficient capital to pay such amounts. 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.

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

We face an inherent risk of product liability as a result of the use of our product commercially, and the clinical testing of our product candidates and will face an even greater risk as we commercialize any additional products in the future. For example, we may be sued if our product or product candidates cause or are perceived to cause injury or are found to be otherwise unsuitable during clinical trials, manufacturing, marketing or sale. Any such product liability claims may include allegations of defects in manufacturing, defects in design, a failure to warn of dangers inherent in the product or product candidate, negligence, strict liability or a breach of warranties. Claims could also be asserted under state consumer protection acts. If we cannot successfully defend ourselves against product liability claims, we may incur substantial liabilities or be required to limit commercialization of our product and product candidates. Even a successful defense would require significant financial and management resources. Regardless of the merits or eventual outcome, product liability claims may result in:

- inability to bring a product or product candidate to the market;
- decreased demand for any product or product candidates;
- injury to our reputation and significant negative media attention;
- withdrawal of clinical trial participants and inability to continue clinical trials;
- initiation of investigations by regulators;
- costs to defend the related litigation;
- diversion of management's time and our resources;
- substantial monetary awards to trial participants;
- product recalls, withdrawals or labeling, marketing or promotional restrictions;
- decreased demand for a product;
- loss of revenue;
- exhaustion of any available insurance and our capital resources;
- the inability to commercialize any product or product candidate; and
- decline in our stock price.

Our inability to obtain and retain sufficient product liability insurance at an acceptable cost to protect against potential product liability claims could prevent or inhibit the commercialization of products we develop, alone or with collaborators. We may be unable to obtain, or may obtain on unfavorable terms, additional clinical trial insurance in amounts adequate to cover any liabilities from any of our clinical trials. Our insurance policies may also have various exclusions, and we may be subject to a product liability claim for which we have insufficient or no coverage. We may have to pay any amounts awarded by a court or negotiated in a settlement that exceed our coverage limitations or that are not covered by our insurance, and we may not have, or be able to obtain, sufficient capital to pay such amounts. Even if our agreements with any future corporate collaborators entitle us to indemnification against losses, such indemnification may not be available or adequate should any claim arise.

Our current laboratory operations are concentrated in one location, and we or the third parties upon whom we depend, including our clinical trial sites and the manufacturing facilities of our third-party contract manufacturers, may experience business interruptions and our business continuity and disaster recovery plans may not adequately protect us from a serious disaster, including earthquakes, outbreak of disease or other natural disasters.

Our office and laboratory facilities are located in Cambridge, Massachusetts. Any unplanned event, such as flood, fire, explosion, earthquake, extreme weather condition, medical epidemics, power shortage, telecommunication failure or other natural or manmade accidents or incidents that result in us being unable to fully utilize our facilities, the facilities at any clinical trial site, or the manufacturing facilities of our third-party contract manufacturers, may have a material and adverse effect on our ability to operate our business, particularly on a daily basis, and have significant negative consequences on our financial and operating conditions. Loss of access to these facilities may result in increased costs, delays in the development of apitegromab, SRK-181, SRK-439 and future

product candidates or interruption of our business operations. If a natural disaster, outbreak of disease, power outage or other event occurred that prevented us from using all or a significant portion of our headquarters, that damaged critical infrastructure, such as our research facilities, our clinical trial sites or the manufacturing facilities of our third-party contract manufacturers, or that otherwise disrupted operations, it may be difficult or, in certain cases, impossible, for us to continue our business for a substantial period of time.

Global events, including global health concerns could also result in social, economic, and labor instability in the countries in which we operate or where the third parties with whom we engage, including our clinical trial sites and manufacturing facilities of our third-party contract manufacturers, operate. Unforeseen global events, such as increasing rates of inflation and interest and tariff policies, could adversely impact our business. Such conflicts could lead to sanctions, embargoes, supply shortages, regional instability, geopolitical shifts, cyberattacks, other retaliatory actions, and adverse effects on macroeconomic conditions, currency exchange rates, and financial markets, which could adversely impact our operations and financial results, as well as those of third parties with whom we conduct business.

The disaster recovery and business continuity plans we have in place may prove inadequate in the event of a serious disaster or similar event. We may incur substantial expenses as a result of the limited nature of our disaster recovery and business continuity plans, which could have a material adverse effect on our business. As part of our risk management policy, we maintain insurance coverage at levels that we believe are appropriate for our business. However, in the event of an accident or incident at our facilities, we cannot assure you that the amounts of insurance will be sufficient to satisfy any damages and losses. If our facilities, the manufacturing facilities of our third-party contract manufacturers, or the sites where we conduct clinical trials or nonclinical studies, are unable to operate because of an accident or incident or for any other reason, even for a short period of time, our research and development programs may be harmed. Any business interruption may have a material and adverse effect on our business, financial condition, results of operations and prospects.

Coverage and reimbursement may be limited or unavailable in certain market segments for ISEMBYLD, which could make it difficult for us to sell ISEMBYLD profitably.

The success of ***ISEMBYLD*** depends on the availability of coverage and adequate reimbursement from third-party payors. We cannot be sure of the extent to which coverage and adequate reimbursement for ISEMBYLD will be available or maintained and any limitations, delays, or restrictions on coverage or reimbursement could adversely affect patient access to, utilization of and revenue from ISEMBYLD. See the sections in our Annual Report on Form 10-K for the year ended December 31, 2025 entitled "*Business– Government Regulation – Coverage and Reimbursement" and "Business–Government Regulation–Current and Future Healthcare Reform Legislation.*"

Patients who are provided medical treatment for their conditions generally rely on third-party payors to reimburse all or part of the costs associated with their treatment. Coverage and adequate reimbursement from governmental healthcare programs, such as Medicare and Medicaid or national payor bodies (such as in European countries), and commercial payors is critical to new product acceptance.

Government authorities and other third-party payors, such as private health insurers and health maintenance organizations, decide which drugs and treatments they will cover and the amount of reimbursement. Coverage and reimbursement by a third-party payor may depend upon a number of factors, including the third-party payor's determination that use of a product is:

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

In the U.S., no uniform policy of coverage and reimbursement for products exists among third party payors, Coverage and reimbursement for products can differ significantly from payor to payor. One payor's decision to cover a particular medical product or service does not ensure that other payors will also provide coverage for the medical product or service, or will provide coverage at an adequate reimbursement rate. Coverage and reimbursement for products may vary widely from payor to payor, state-to-state (for example, state Medicaid coverage and reimbursement for products may be subject to varying degrees of coverage restrictions or delays) or across national payors from country to country.

Payors are increasingly challenging the price and examining the medical necessity and cost-effectiveness of medical products and services, in addition to their safety and efficacy. In order to obtain and maintain coverage and reimbursement for any product, we may need to conduct expensive evidence generation studies in order to demonstrate the medical necessity and cost-effectiveness of such a product, in addition to the costs required to obtain regulatory approvals. If payors do not consider a product to be cost-effective compared to current standards of care, they may not cover the product as a benefit under their plans or, if they do, the level of payment may not be sufficient to allow a company to cover its costs or make a profit. Although ISEMBYLD has obtained coverage and reimbursement from a number of third-party payors, we cannot assure you that favorable coverage, reimbursement levels or patient access will be maintained or expanded. The reimbursement payment rates established by third-party payors may not be adequate for us to achieve or sustain profitability and may be reduced over time. Additionally, third-party payors may not cover, or provide adequate reimbursement for, long-term follow-up evaluations required following the use of ISEMBYLD. Patients are unlikely to use ISEMBYLD unless coverage is provided and reimbursement is adequate to cover a significant portion of the cost of ISEMBYLD. There is significant uncertainty related to insurance coverage and reimbursement of newly approved products. As a result, it remains difficult to predict the extent to which third-party payors will provide and maintain favorable coverage and reimbursement for ISEMBYLD over time.

Payment methodologies may be subject to changes in healthcare legislation and regulatory initiatives. Additional state and federal healthcare reform measures are expected to be adopted in the future, any of which could limit the amounts that federal and state governments will pay for healthcare products and services, which could result in reduced demand for certain pharmaceutical products or additional pricing pressures.

Moreover, increasing efforts by governmental and third-party payors in the U.S. and abroad to cap or reduce healthcare costs may cause such organizations to limit both coverage and the level of reimbursement for newly approved products and, as a result, they may not cover or provide adequate payment for ISEMBYLD. There has been increasing legislative and enforcement interest in the U.S. with respect to specialty drug pricing practices. Specifically, there have been several recent U.S. Congressional inquiries and proposed and enacted federal and state legislation designed to, among other things, bring more transparency to drug pricing, reduce the cost of prescription drugs under Medicare, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for drugs. We expect to experience pricing pressures in connection with the sale of ISEMBYLD due to the trend toward managed healthcare, the increasing influence of health maintenance organizations, cost containment initiatives and additional legislative changes.

EU drug marketing and reimbursement regulations may materially affect our ability to market and receive coverage for apitegromab in the EU Member States. Also, unfavorable pricing and reimbursement decisions in the EU could delay or limit commercialization of apitegromab, if approved, and materially adversely affect our business, financial condition and results of operations.

In addition to the FDA approval we received, we intend to seek approval to market apitegromab in selected foreign jurisdictions. If we receive approval in one or more foreign jurisdictions for apitegromab we will be subject to rules and regulations in those jurisdictions. In some foreign countries, particularly those in the EU, the pricing of medicinal products is subject to governmental control and other market regulations which could put pressure on the pricing and usage of apitegromab. In these countries, pricing negotiations with governmental authorities can take considerable time after receiving marketing approval. In addition, market acceptance and sales of apitegromab will depend significantly on the availability of adequate coverage and reimbursement from third-party payors for apitegromab and may be affected by existing and future health care reform measures.

Much like the federal Anti-Kickback Statute prohibition in the U.S., the provision of benefits or advantages to physicians to induce or encourage the prescription, recommendation, endorsement, purchase, supply, order or use of medicinal products is also prohibited in the EU. The provision of benefits or advantages to physicians is governed by the national anti-inducement, advertising and anti-bribery laws of EU Member States, and in respect of the UK (which is no longer a member of the EU), the Bribery Act 2010. Infringement of these laws could result in substantial fines and imprisonment.

Payments made to physicians in certain EU Member States must be disclosed publicly. Moreover, agreements with physicians often must be the subject of prior notification and approval by the physician's employer, his or her competent professional organization and/or the regulatory authorities of the individual EU Member States. These requirements are provided in the national laws, industry codes or professional codes of conduct, applicable in the EU Member States. EU Directive 2001/83/EC, which is the EU Directive governing medicinal products for human use, further provides that where medicinal products are being promoted to persons qualified to prescribe or supply them, no gifts, pecuniary advantages or benefits in kind may be supplied, offered or promised to such persons unless they are inexpensive and relevant to the practice of medicine or pharmacy. Failure to comply with these requirements could result in reputational risk, public reprimands, administrative penalties, fines or imprisonment.

Even if apitegromab or any of our future product candidates receive marketing authorization in the EU or UK, we may be unable to successfully commercialize such products in those markets if we are unable to secure pricing and reimbursement on terms that are

commercially acceptable. In most foreign countries, including several EU Member States, marketing authorization is only the first step toward patient access and the proposed pricing for a drug must be approved before it may be lawfully marketed. The requirements governing drug pricing and reimbursement vary widely from country to country. For example, each EU Member State conducts its own health technology assessment ("HTA"), pricing negotiations, and reimbursement decision-making, potentially resulting in delays of 12-24 months or more (on average) between EC approval and commercial availability. EU Member States can also 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. Payers in many European countries impose increasingly stringent clinical, pharmacoeconomic, and budget-impact requirements that may differ significantly from those considered by regulatory authorities during the marketing authorization process. As a result, European authorities may conclude that the benefit–risk profile, real-world evidence, or incremental clinical value of apitegromab, when used as an adjunct to SMN-targeted therapies, does not justify the price we seek.

In December 2021, the Health Technology Assessment Regulation, Regulation (EU) 2021/2282, ("HTAR") was adopted. The HTAR entered into force on January 11, 2022 and started to apply from January 12, 2025, with phased application depending on the category of health technology. Under the HTAR, EU Member States are required to use common HTA tools, methodologies, and procedures across the EU. Among others, the HTAR establishes an HTA coordination group, composed of national HTA bodies, which jointly conduct Joint Clinical Assessments ("JCAs") of certain new medicines (including, in the initial phase, oncology medicines and advanced therapy medicinal products) and certain high-risk medical devices and provides for a single EU-level dossier submission for the purposes of JCAs. However, the HTAR focuses on the clinical aspects of HTA, i.e. the relative clinical effectiveness and relative clinical safety of a new health technology compared with relevant existing health technologies, and, as such, individual EU Member States remain responsible for determining the overall value of a new health technology within their healthcare systems, as well as making pricing and reimbursement decisions.

Reference pricing used by various EU Member States and parallel distribution, or arbitrage between low-priced and high-priced Member States, can further reduce prices. Pricing negotiations in the EU are highly unpredictable and a Member State may approve a specific price for the medicinal product 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. In addition, regional decision-making, such as in Italy, Spain, and Germany, may lead to fragmented sub-national reimbursement requirements, duplicative reviews, or further restrictions on access. In some countries, we may be required to conduct a clinical study or other studies that compare the cost-effectiveness of any of our product and product candidates to other available therapies in order to obtain or maintain reimbursement or pricing approval. Generating such data may require substantial investment and time and may delay reimbursement approval or lead to conditional or temporary reimbursement that could be revoked if post-authorization evidence does not meet the expectations of payers.

There can be no assurance that any country that has price controls or reimbursement limitations for pharmaceutical products will allow favorable reimbursement and pricing arrangements for apitegromab. Historically, products launched in the EU do not follow price structures of the U.S. and generally prices tend to be significantly lower and, in certain countries, below levels required for commercial viability. Publication of discounts by third-party payors or authorities may lead to further pressure on the prices or reimbursement levels within the country of publication and other countries. Moreover, any delays or negative outcomes in pricing or reimbursement negotiations in major markets such as Germany, France, Italy, Spain, or the UK could adversely influence decisions in other EU Member States due to cross-border reference pricing, or coordinated negotiation mechanisms that are increasingly being adopted across the EU. A low price, stringent reimbursement limitation, or reimbursement denial in one influential country may therefore have a cascading effect across multiple markets. In addition, if negotiated EU list prices become public, an unfavorable price published in one EU Member State could place pressure on pricing negotiations in other jurisdictions.

If pricing is set at unsatisfactory levels or if reimbursement of apitegromab is unavailable or limited in scope or amount, our revenues from sales by us or our strategic partners and the potential profitability of apitegromab in those countries would be negatively affected. If we are unable to secure satisfactory pricing and reimbursement in the EU, or if the reimbursement landscape evolves in ways that impose additional uncertainty, delay, or financial burden, we may be unable to realize the full commercial potential of apitegromab or any future product candidate in these markets. Any of these events could materially adversely affect our business, prospects, financial condition and results of operations.

We may seek to enter into collaborations in the future with third parties, including for apitegromab in other indications, SRK-181, SRK-439 or potential product candidates. If we are unable to enter into such collaborations, or if these collaborations are not successful, our business could be adversely affected.

A part of our strategy is to evaluate and, as deemed appropriate, enter into additional collaborations or partnerships in the future when strategically attractive, including potentially with biotechnology or pharmaceutical companies. We have limited capabilities for product development and only recently have begun to build our capabilities to prepare for potential commercialization. Accordingly, we may enter into collaborations with other companies to provide us with important technologies, capabilities and funding for our programs and underlying technology.

Any future collaboration we enter into may pose a number of risks, including the following:

- collaborators may have significant discretion or decision-making authority in determining the efforts and resources that they will apply to the collaboration or that we are required to apply to the collaboration;
- collaborators may not perform their obligations as expected or in a manner satisfactory to us;
- we may commit to certain nonclinical or clinical development or commercialization efforts as part of the collaboration that we are unable to meet or our collaborators may not be satisfied with our nonclinical or clinical development or commercialization efforts;
- collaborators may not pursue development and commercialization of any product and 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 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 and 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 and product candidates, might lead to additional responsibilities for us with respect to product and 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 deemphasize or terminate the development or commercialization of any product candidate licensed to it by us;
- collaborations may be terminated by the collaborator, and, if terminated, we may be blocked to advance the program due to collaborator patents that are not licensed to 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 and product candidates.

If our future collaborations do not result in the successful discovery, development and commercialization of product and 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 our Annual Report on Form 10-K for the year ended December 31, 2025 also apply to the activities of potential 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 biotechnology or pharmaceutical industry, including within the business and financial communities, could be adversely affected.

We face significant competition in seeking appropriate partners for our product and product candidates, and the negotiation process is time-consuming and complex. In order for us to successfully partner our product and product candidates, potential partners must view these product and product candidates as economically valuable in markets they determine to be attractive in light of the terms that we are seeking and other available products for licensing by other companies. Collaborations are complex and time-consuming to negotiate and document. In addition, there have been a significant number of recent business combinations among large pharmaceutical companies that have resulted in a reduced number of potential future collaborators. Our ability to reach a definitive agreement for a collaboration will depend, among other things, upon our 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. If we are unable to reach agreements with suitable collaborators on a timely basis, on acceptable terms, or at all, we may have to curtail the development of a product candidate, reduce or delay its development program or one or more of our other development programs, delay its potential commercialization or 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 expertise and additional capital, which may not be available to us on acceptable terms, or at all. If we fail to enter into collaborations or do not have sufficient funds or expertise to undertake the necessary development and commercialization activities, we may not be able to further develop our product and product candidates, bring them to market and generate revenue from sales of drugs or continue to develop our technology, and our business may be materially and adversely affected. Even if we are successful in our efforts to establish new strategic collaborations, the terms that we agree upon may not be favorable to us, and we may not be able to maintain such strategic collaborations if, for example, development or approval of a product candidate is delayed or sales of an approved product are disappointing. Any delay in entering into new strategic collaboration agreements related to our product and product candidates could delay the development and commercialization of our product and product candidates and reduce their competitiveness even if they reach the market.

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 and 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 and 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 products or 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 and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner. In addition, we may not pursue or obtain patent protection in all relevant markets. Unforeseen global events, and sanctions or actions relating to such events, could affect our ability to file, prosecute, maintain, and/or defend patents and applications in those 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.

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 products and product candidates or uses thereof in the U.S. and/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. For example, Russia issued a decree in March of 2022, stating that patent owners who reside in a country "unfriendly" to Russia are not entitled to compensation in the event of patent infringement. Furthermore, even if they are unchallenged, our patents and patent applications may not adequately protect our intellectual property and/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 and product candidates is threatened, it could dissuade companies from collaborating with us to develop, and threaten our ability to commercialize, our product and product candidates. Further, if we encounter delays in our clinical trials, the period of time during which we could market our products and product candidates under patent protection would be reduced.

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. 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 and 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 or product candidates or our activities infringing such claims. The possibility exists that others will develop products which have the same effect as our products 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.

In addition, periodic maintenance fees on any issued patent are due to be paid to the U.S. Patent Office ("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. Moreover, complications due to public health crises such as global pandemics may result in inadvertent lapse due to, for example, unexpected closures of the USPTO or foreign patent offices, delays in delivery of notifications relating to deadlines, or failure to timely and/or properly obtain signatures on necessary documents. Additionally, due to the ongoing conflict in Ukraine, there remain uncertainties as to any potential impact on patent protection and/or enforcement in the region, including, for example, payments to the Russian Patent Office and other entities. In such an event, our competitors might be able to enter the market, which would have a material adverse effect on our business.

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:

- it is possible that our pending patent applications will not result in issued patents;
- we or our licensors, as the case may be, might not have been the first to file patent applications for these inventions;
- the claims of our owned or in-licensed issued patents or patent applications, if and when issued, may not cover our product and product candidates;
- 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;
- 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;
- 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;
- 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 U.S.;
- 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 or raise disputes with regard to ownership of our owned or in-licensed patents or patent applications;
- 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;
- others may be able to make or use compounds or cells that are similar to the biological compositions of our product and product candidates but that are not covered by the claims of our patents;

- others may independently develop similar or alternative technologies or duplicate any of our technologies;
- it is possible that others may circumvent our owned or in-licensed patents;
- the active biological ingredients in our current product and 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 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 products, product candidates, diagnostic tests, and manufacturing methods we develop may be covered by third parties' patents or other exclusive rights;
- it is possible that there are unpublished applications or patent applications maintained in secrecy that may later issue with claims covering our products or technology similar to ours; and/or
- the patents of others may have an adverse effect on our business.

Our current patents covering our proprietary technologies and our product and product candidates are expected to expire beginning in 2034, without taking into account any possible patent term adjustments or extensions. Our earliest patents may expire before, or soon after, our first product achieves marketing approval in the U.S. or foreign jurisdictions. Upon the expiration of our current patents, we may lose the right to exclude others from practicing these inventions. The expiration of these patents could also have a 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 and product candidates that if issued as patents are expected to expire from 2034 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.

We depend on intellectual property licensed from third parties. Failure to comply with our obligations under any of these licenses or 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, including intellectual property rights licensed from others. We may be a party to license agreements pursuant to which we in-license key patents and patent applications for our product candidates. These licenses impose various diligence, milestone payment, royalty, insurance and other obligations on us. If we fail to comply with these obligations, our licensors may have the right to terminate the license. Any termination of licenses by third parties could result in our loss of significant intellectual property rights and could harm our ability to commercialize our product candidates.

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 these licensors 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. We may have limited control over the manner in which our licensors initiate an infringement proceeding against a third-party infringer of the intellectual property rights, or defend certain of the intellectual property that is licensed to us. It is possible that the licensors' infringement proceeding or defense activities may be less vigorous than had we conducted them ourselves.

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 patent and other rights to third parties under collaborative development relationships; and
- the ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and us and our partners.

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 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 additional 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 or manufacturing processes 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 or 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.

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.

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.

In addition, 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 (the "America Invents Act"), enacted in 2013, the U.S. 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.

Recent U.S. Supreme Court rulings have also 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. As a consequence, issued patents may be found to contain invalid claims according to the newly revised eligibility and validity standards. Additionally, 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.

Depending on decisions by the U.S. Congress, the federal courts, and the USPTO, the laws and regulations governing patents, or interpretation thereof, could change in unpredictable ways that would weaken our ability to obtain new patents, to maintain, 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. While the validity of a subset of patents at issue was subsequently upheld by a district court jury, uncertainty remains as to the legal question pertaining to the written description requirement under 35 USC §112 as it relates to functional antibodies. 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. For example, Russia issued a decree in March of 2022, stating that patent owners who reside in a country "unfriendly" to Russia are not entitled to compensation in the event of patent infringement.

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 and 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 and 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. As the biotechnology and pharmaceutical industries expand and more patents are issued, the risk increases that our product and 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 or product candidates, technologies or methods.

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, 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 or 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; and
- redesigning our product or 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 U.S. is protected under the Safe Harbor exemption as set forth in 35 U.S.C. § 271. With respect to our product, or if 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 are not aware of any claims of such a patent that could otherwise materially adversely affect commercialization of our product or product candidates, 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 U.S. 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 or 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 or 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 or 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 or 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 or 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 or 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 our current product 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 or 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, and/or 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. 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 or 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 or product candidates, which could harm our business significantly.

We may also 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 the grant of a third-party's patent in opposition proceedings in the European Patent Office ("EPO") or other foreign patent office. 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, EPO 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, product candidates or proprietary technologies.

Additionally, the Unitary Patent/Unified Patent Court ("UPC") system in Europe became fully operational in June 2023.

- The new court may be associated with greater degrees of uncertainty in litigation, with respect to both planning and outcome.
- Subject to current transitional provisions, which are currently scheduled to expire in May 2030, published European patent applications and European patents can be opted out of the jurisdiction of the UPC. The timing and form of an opt-out can be important to its effectiveness and the opt-out status may affect the litigation strategy options available, including choice of forum.

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

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 against a third party(ies), 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. There is also the risk that, even if the validity of our patents or the patents of our licensors 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. 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.

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.

In some situations, we or our licensor, 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 licensors detect infringement by a third-party of our owned or in-licensed patents, we or our licensors, as the case may be, may choose not to pursue litigation against or settlement with the third-party. If we, or our licensors, 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 licensors to enforce our owned or in-licensed patents, as the case may be, against such third-party.

Issued patents covering our products or 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 products or product candidates, the defendant could counterclaim that the patent covering our product or product candidate, as applicable, is invalid and/or unenforceable. In patent litigation in the U.S., 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 U.S. or abroad, even outside the context of litigation. Such mechanisms include inter parties review, ex parte re-examination, post-grant review, and equivalent proceedings in foreign jurisdictions (e.g., opposition proceedings). For example, EP3816625, EP4218804, and EP4358995 are currently subject to opposition proceedings in Europe, while JP6823167 and JP7344337 are the subject of invalidation trials in Japan. Such proceedings are expensive and 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, products, and product candidates.

In addition, because some patent applications in the U.S. may be maintained in secrecy until the patents are issued, because patent applications in PCT member jurisdictions are typically not published until 18 months after the earliest filing, and because 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, compositions, methods of use, 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 U.S. 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.

For applications filed under pre-AIA, interference proceedings declared 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. 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 U.S.

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 U.S. 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 U.S. can be less extensive than those in the U.S. In addition, the laws of some foreign countries do not protect intellectual property rights to the same extent as federal and state laws in the U.S. Consequently, we may not be able to prevent third parties from practicing our inventions in all countries outside the U.S., or from selling or importing products made using our inventions in and into

the U.S. or other jurisdictions. Indeed, Russia issued a decree in March of 2022, stating that patent owners who reside in a country "unfriendly" to Russia are not entitled to compensation in the event of patent infringement. 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 U.S. These products may compete with our products 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 and/or methods of medical treatment, 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.

As another example, in Europe, a new unitary patent system became effective in June 2023, which may significantly impact European patents, including those granted before the introduction of such a system. Under the unitary patent system, European applications have the option, upon grant of a patent, of becoming a Unitary Patent which will be subject to the jurisdiction of the Unified Patent Court ("UPC"). As the UPC is a new court system, there is little precedent for the court, increasing the uncertainty of any litigation. Subject to current transitional provisions, which are currently scheduled to expire in May 2030, published European patent applications and European patents can be opted out of the jurisdiction of the UPC and remain or be validated as national patents in the UPC countries. However, the timing and form of an opt-out can be important to its effectiveness and the opt-out status may affect the litigation strategy options available, including choice of forum. Patents that remain under the jurisdiction of the UPC are potentially vulnerable to a single UPC-based revocation challenge that, if successful, could invalidate the patent in all countries who are signatories to the UPC. We cannot predict with certainty the long-term effects of any potential changes.

Patent terms may result in inadequate protection for our product or product candidates, and we may be unable to obtain patent term extensions and data exclusivity for our product or product candidates, resulting in material harm to our business.

Patents have a limited lifespan. In the U.S., 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.

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, also known as the 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. The patent term restoration period is generally one-half of the time between the effective date of the IND or the date of patent grant (whichever is later) and the date of submission of the BLA, plus the time between the date of submission of the BLA and the date of FDA approval of the product. The patent holder must apply for restoration within 60 days of approval. 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. 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.

Given the amount of time required for the development, testing and regulatory review of new product candidates, the patents protecting our product candidates might expire before or shortly after such candidates are commercialized. If we are unable to obtain patent term extension or the term of any such extension is less than we request, our competitors may obtain approval of competing products following our patent expiration. 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, which could materially harm our business, financial condition, results of operations, and prospects.

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 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 U.S. 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 parties may assert that our employees or consultants have wrongfully used, disclosed, or misappropriated their confidential information or 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.

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. 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 Financial Condition and Capital Requirements

We have incurred net losses in every year since our inception and anticipate that we will continue to incur net losses in the future.

We are a biopharmaceutical company formed in 2012 and our operations to date have been focused on research and development of monoclonal antibodies that selectively inhibit activation of growth factors for therapeutic effect. Although we have recently received approval from the FDA for ISEMBYLD, we have previously had no products approved for commercial sale. We have generated limited revenue from product sales to date, but we continue to incur significant research and development and other expenses related to our ongoing operations. As a result, we are not profitable and have incurred losses in each period since our inception. For the nine months ended September 30, 2026 and 2025, we reported a net loss of $322.6 million and $287.0 million, respectively. We have incurred losses since our inception, and as of September 30, 2026, we had an accumulated deficit of $1.6 billion. 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 product and product candidates, apitegromab in other indications, SRK-181, SRK-439, and any future product candidates and prepare for the commercialization of ISEMBYLD.

To become and remain profitable, we or any current or potential future collaborators must develop and eventually commercialize products with significant market potential and favorable pricing. This will require us to be successful in a range of challenging activities, including completing nonclinical studies and clinical trials, receiving and retaining marketing approval for product and product candidates, manufacturing, marketing and selling products for which we may receive marketing approval and satisfying any post-marketing requirements. We 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. 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 could 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.

We will continue to incur substantial research and development and other expenditures to evaluate ISEMBYLD and 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 require additional capital to fund our operations and if we fail to obtain necessary capital, we will not be able to complete the development and commercialization of ISEMBYLD, SRK-181, SRK-439 and any future product candidates.

Our operations have consumed substantial amounts of cash since inception. We expect to continue to spend substantial amounts of cash to conduct further research and development, including clinical trials for apitegromab indications and nonclinical studies and clinical trials for SRK-439 and any future product candidates, to seek regulatory approvals for our product and product candidates and to launch and commercialize ISEMBYLD and any other products for which we receive regulatory approval. As of September 30, 2026, we had approximately $413.6 million in cash, cash equivalents and marketable securities. Based on our current operating model, we believe that our existing cash, cash equivalents, and marketable securities as of September 30, 2026, together with the cash available under our debt facility and our anticipated product revenues, will be sufficient to fund our operating expenses and capital expenditure requirements into 2028. However, our future capital requirements and the period for which our existing resources will support our operations may vary significantly from what we expect, and we will in any event require additional capital in order to complete clinical development of any of our current programs. Our monthly spending levels will vary based on new and ongoing development and corporate activities. Because the length of time and activities associated with development of our product and product candidates is highly uncertain, we are unable to estimate the actual funds we will require for development and any approved marketing and commercialization activities. Additionally, any program setbacks or delays due to changes in federal or state laws or clinical site or clinical vendor policies as a result of the impacts of current macroeconomic and geopolitical events, increasing rates of inflation, tariff policies and rising interest rates could impact our programs and increase our expenditures. Our future funding requirements, both near and long-term, will depend on many factors, including, but not limited to:

- the initiation, progress, timing, completion, costs and results of clinical trials for our current and any future product and product candidates;
- the clinical development plans we establish for our product candidates;
- the number and characteristics of product candidates that we identify and develop;

- the terms of any collaboration, strategic alliance, or licensing agreements we are currently party to or may choose to enter into in the future;
- the outcome, timing and cost of meeting regulatory requirements established by the FDA, the EMA, the PMDA, and other comparable foreign regulatory authorities;
- the cost of filing, prosecuting, defending and enforcing our patent claims and other intellectual property rights;
- the cost of defending intellectual property disputes, including patent infringement actions brought by third parties against us or our product and product candidates;
- the effect of competing technological and market developments;
- the cost and timing of developing research cell lines and development and completion of commercial scale outsourced manufacturing activities;
- the impact of any business interruptions to our operations, including the timing and enrollment of patients in our planned clinical trials, or to those of our manufacturers, suppliers, or other vendors resulting from pandemics or similar public health crises or macroeconomic conditions; and
- the cost of establishing sales, marketing and distribution capabilities for any product and product candidates for which we may receive regulatory approval in regions where we choose to commercialize our products on our own.

We do not have any committed external source of funds or other support for our development efforts. 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. If we raise additional funds through public or private equity offerings, the terms of these securities may include liquidation or other preferences that adversely affect our stockholders' rights. Further, to the extent that we raise additional capital through the sale of common stock or securities convertible or exchangeable into common stock, your ownership interest will be diluted. In addition, any debt financing may subject us to fixed payment obligations and covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends. 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 and 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 apitegromab in other indications, SRK-181, SRK-439 or any future product candidate at an earlier stage than otherwise would be desirable or relinquish our rights to product and product candidates or technologies that we otherwise would seek to develop or commercialize ourselves. 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 apitegromab, SRK-181, SRK-439 or one or more of our future product candidates or 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.

Changes in tax law could adversely affect our business and financial condition.

The rules dealing with U.S. federal, state, and local income taxation are constantly under review by persons involved in the legislative process and by the Internal Revenue Service and the U.S. Treasury Department. For example, the One Big Beautiful Bill Act ("OBBBA") was signed into law on July 4, 2025 and made significant changes to U.S. federal tax law. Changes to tax laws (which changes may have retroactive application) could adversely affect us or holders of our common stock. For example, under Section 174 of the Internal Revenue Code of 1986, as amended (the "Code"), in taxable years beginning after December 31, 2021, expenses that are incurred for research and development outside the U.S. will be capitalized and amortized, which may have an adverse effect on our cash flow. The OBBBA provides that for taxable years beginning after December 31, 2024, expenses that are incurred for research and development performed in the U.S. may, at the taxpayer's election, be immediately deducted or capitalized and amortized. In addition, the OBBBA provides that for taxable years beginning after December 31, 2021 and before January 1, 2025, certain eligible taxpayers generally may elect to retroactively deduct expenses for research and development performed in the U.S. in such taxable years by filing amended tax returns for such taxable years, and all other taxpayers that are not eligible to make such an election and that amortized expenses for research and development performed in the U.S. in such taxable years generally may elect to accelerate and deduct the remaining unamortized amounts of such research and development expenses (i) in the first taxable year beginning after December 31, 2024, or (ii) ratably over the two-taxable year period beginning with the first taxable year beginning after December 31, 2024. In recent years, many changes to tax laws have been made and changes are likely to continue to occur in the future. Future changes in tax laws could have a material adverse effect on our business, cash flow, financial condition or results of operations. It

cannot be predicted whether, when, in what form or with what effective dates tax laws, regulations and rulings may be enacted, promulgated or issued, which could result in an increase in our or our shareholders' tax liability or require changes in the manner in which we operate in order to minimize or mitigate any adverse effects of changes in tax law. We urge investors to consult with their legal and tax advisers regarding the implications of potential changes in tax laws on an investment in our common stock.

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

As of December 31, 2025, we had net operating loss carryforwards for U.S. federal and state income tax purposes of $921.9 million and $861.4 million, respectively, which begin to expire in 2032, except for our post 2017 U.S. federal net operating loss carryforwards of $871.4 million and $3.2 million of state net operating losses which do not expire. As of December 31, 2025, we also had available tax credit carryforwards for U.S. federal and state income tax purposes of $74.1 million and $10.4 million, respectively, which begin to expire in 2034 and 2032, respectively. Additionally, for taxable years beginning after December 31, 2017 the deductibility of the indefinite lived federal and state net operating losses is limited to 80% of our taxable income in any future taxable year. Under Section 382 of the Code, changes in our ownership may limit the amount of our net operating loss carryforwards and tax credit carryforwards that could be utilized annually to offset our future taxable income, if any. This limitation would generally apply in the event of a cumulative change in ownership of our company of more than 50% within a three-year period. Any such limitation may significantly reduce our ability to utilize our net operating loss carryforwards and tax credit carryforwards before they expire. Private placements and other transactions that have occurred since our inception, as well as our IPO, may trigger such an ownership change pursuant to Section 382 of the Code. Any such limitation, whether as the result of our IPO, prior private placements, sales of our common stock by our existing stockholders or additional sales of our common stock by us, could have a material adverse effect on our results of operations in future years.

Adverse developments affecting the financial services industry, such as actual events or concerns involving liquidity, defaults, or non-performance by financial institutions or transactional counterparties, could adversely affect our current and projected business operations and our financial condition and results of operations.

Actual events involving limited liquidity, defaults, non-performance or other adverse developments that affect financial institutions, transactional counterparties or other companies in the financial services industry or the financial services industry generally, or concerns or rumors about any events of these kinds or other similar risks, have in the past and may in the future lead to market-wide liquidity problems. For example, on March 10, 2023, SVB was closed by the California Department of Financial Protection and Innovation, which appointed the FDIC as receiver. Similarly, on March 12, 2023, Signature Bank and Silvergate Capital Corp. were each swept into receivership. Since then, additional financial institutions have experienced similar failures and have been placed into receivership.

Inflation and rapid increases in interest rates have led to a decline in the trading value of previously issued government securities with interest rates below current market interest rates. Although the U.S. Department of Treasury, FDIC and Federal Reserve Board have announced a program to provide up to $25 billion of loans to financial institutions secured by certain of such government securities held by financial institutions to mitigate the risk of potential losses on the sale of such instruments, widespread demands for customer withdrawals or other liquidity needs of financial institutions for immediate liquidity may exceed the capacity of such program. Additionally, there is no guarantee that the U.S. Department of Treasury, FDIC and Federal Reserve Board will provide access to uninsured funds in the future in the event of the closure of other banks or financial institutions, or that they would do so in a timely fashion.

Although we assess our banking relationships as we believe necessary or appropriate, our access to funding sources and other credit arrangements in amounts adequate to finance or capitalize our current and projected future business operations could be significantly impaired by factors that affect the Company, the financial institutions with which the Company has credit agreements or arrangements directly, or the financial services industry or economy in general. These factors could include, among others, events such as liquidity constraints or failures, the ability to perform obligations under various types of financial, credit or liquidity agreements or arrangements, disruptions or instability in the financial services industry or financial markets, or concerns or negative expectations about the prospects for companies in the financial services industry. These factors could involve financial institutions or financial services industry companies with which the Company has financial or business relationships, but could also include factors involving financial markets or the financial services industry generally.

The results of events or concerns that involve one or more of these factors could include a variety of material and adverse impacts on our current and projected business operations and our financial condition and results of operations. These could include, but may not be limited to, the following:

- delayed access to deposits or other financial assets or the uninsured loss of deposits or other financial assets;
- delayed or lost access to, or reductions in borrowings available under our existing debt facility; or

- potential or actual breach of contractual obligations that require the Company to maintain certain financial accounts at specific financial institutions.

In addition, investor concerns regarding the U.S. or international financial systems could result in less favorable commercial financing terms, including higher interest rates or costs and tighter financial and operating covenants, or systemic limitations on access to credit and liquidity sources, thereby making it more difficult for us to acquire financing on acceptable terms or at all. Any decline in available funding or access to our cash and liquidity resources could, among other risks, adversely impact our ability to meet our operating expenses, financial obligations or fulfill our other obligations, result in breaches of our financial and/or contractual obligations or result in violations of federal or state wage and hour laws. Any of these impacts, or any other impacts resulting from the factors described above or other related or similar factors not described above, could have material adverse impacts on our liquidity and our current and/or projected business operations and financial condition and results of operations.

In addition, any further deterioration in the macroeconomic economy or financial services industry could lead to losses or defaults by our suppliers, which in turn, could have a material adverse effect on our current and/or projected business operations and results of operations and financial condition. For example, a supplier could be adversely affected by any of the liquidity or other risks that are described above as factors that could result in material adverse impacts on us, including but not limited to delayed access or loss of access to uninsured deposits or loss of the ability to draw on existing credit facilities involving a troubled or failed financial institution. Any supplier bankruptcy or insolvency, or any breach or default by a supplier, or the loss of any significant supplier relationships, could result in material losses to us and may have a material adverse impact on our business.

Our current investment policy focuses on preservation of capital. However, we could recognize losses on securities held in our investment portfolio, particularly if interest rates increase or economic and market conditions deteriorate.

As of September 30, 2026, the fair value of our cash, cash equivalents and investments in our marketable debt securities portfolio was approximately $413.6 million and consisted primarily of investments in money market funds and U.S. treasury obligations and government agency securities. Factors beyond our control can significantly influence the fair value of securities in our portfolio and can cause potential adverse changes to the fair value of these securities. For example, fixed-rate securities acquired by us are generally subject to decreases in market value when interest rates rise. Additional factors include, but are not limited to, rating agency downgrades of the securities or our own analysis of the value of the security, defaults by the issuer with respect to the underlying securities, and continued instability in the credit markets. Any of the foregoing factors could cause other-than-temporary impairment in future periods and result in realized losses. The process for determining whether impairment is other-than-temporary usually requires difficult, subjective judgments about the future financial performance of the issuer and any collateral underlying the security in order to assess the probability of receiving all contractual principal and interest payments on the security.

At September 30, 2026, we had $20,000 in net unrealized losses in our marketable securities available-for-sale portfolio, and unrealized losses in our securities portfolio may increase in the future due to the aforementioned economic factors. While our goal is to hold each security until maturity, that may not be possible in light of our policy to preserve capital and liquidity and because investment in securities with unrealized losses has a diminished utility as a source of liquidity prior to maturity. Selling securities with an unrealized loss would result in the realization of such losses, which could have an adverse effect on our financial condition and results of operations.

The terms of our loan agreements place restrictions on our operating and financial flexibility. If we raise additional capital through debt financing, the terms of any new debt could further restrict our ability to operate our business.

On February 27, 2026, we terminated the Oxford Loan Agreement and entered into the 2026 Loan Agreement with certain funds managed by Blue Owl.

The 2026 Loan Agreement currently provides us with up to $350.0 million of borrowing capacity. Our overall leverage and certain obligations and affirmative and negative covenants contained in the related documentation could adversely affect our financial health and business and future operations by limiting our ability to, among other things, satisfy our obligations under the 2026 Loan Agreement, refinance our debt on terms acceptable to us or at all, plan for and adjust to changing business, industry and market conditions, use our available cash flow to fund future acquisitions and make dividend payments, and obtain additional financing for working capital, to fund growth or for general corporate purposes, even when necessary to maintain adequate liquidity.

If we default under the 2026 Loan Agreement, Blue Owl may accelerate all of our repayment obligations and exercise all of their rights and remedies under the 2026 Loan Agreement and applicable law, potentially requiring us to renegotiate our agreement on terms less favorable to us. Further, if we are liquidated, the lenders' right to repayment would be senior to the rights of the holders of our common stock to receive any proceeds from the liquidation. Blue Owl could declare a default upon the occurrence of customary events of default, including payment defaults, breaches of certain affirmative or negative covenants, material contract terminations, and withdrawal events, subject in certain cases to grace periods, cure mechanics, exceptions and thresholds, thereby requiring us to

repay the loan immediately. Any declaration by the lender of an event of default could significantly harm our business and prospects and could cause the price of our common stock to decline. Additionally, if we raise any additional debt financing, the terms of such additional debt could further restrict our operating and financial flexibility.

Risks Related to Our Common Stock

The price of our stock is volatile, and you could lose all or part of your investment.

Similar to the trading prices of the common stock of other biopharmaceutical companies, the trading price of our common stock is subject to wide fluctuations in response to various factors, some of which are beyond our control, including limited trading volume. In addition to the factors discussed in this "Risk Factors" section and elsewhere in this Quarterly Report on Form 10-Q, these factors include:

- announcements of significant acquisitions, strategic collaborations or partnerships, joint ventures or capital commitments by us, our collaborators or our competitors;
- actual or anticipated variations in quarterly operating results or our cash position;
- our failure to meet the estimates and projections of the investment community or that we may otherwise provide to the public;
- changes in accounting practices; and
- significant lawsuits, including patent or stockholder litigation.

In addition, the stock market in general, and the market for biopharmaceutical companies in particular, have experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to the operating performance of these companies. Broad market and industry factors may negatively affect the market price of our common stock, regardless of our actual operating performance. In the past, securities class action litigation has often been instituted against companies following periods of volatility in the market price of a company's securities. This type of litigation, if instituted, could result in substantial costs and a diversion of management's attention and resources, which would harm our business, operating results or financial condition.

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

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. Furthermore, our ability to pay cash dividends is currently restricted by the terms of our debt facility with Blue Owl, and future debt or other financing arrangements may contain terms prohibiting or limiting the amount of dividends that may be declared or paid on our common stock. Any return to stockholders will therefore be limited to the appreciation of their stock.

The members of our board of directors, management, and their affiliates, own a significant percentage of our stock and will be able to exert significant control over matters subject to stockholder approval.

As of September 30, 2026, our executive officers, directors and their affiliates beneficially hold, in the aggregate, approximately 8% of our outstanding voting stock. These stockholders, acting together, are able to significantly influence all matters requiring stockholder approval. For example, these stockholders are able to significantly influence elections of directors, amendments of our organizational documents, or approval of any merger, sale of assets, or other major corporate transaction. This may prevent or discourage unsolicited acquisition proposals or offers for our common stock that you may feel are in your best interest as one of our stockholders.

We no longer qualify as a smaller reporting company as of January 1, 2026, which will increase our costs and demands on management.

Based on the market value of our common stock held by our non-affiliates as of June 30, 2025, we are no longer a smaller reporting company as of January 1, 2026, and are subject to additional disclosure and compliance requirements. Due to this transition, we have and expect to continue to devote significant time and effort to implement and comply with the additional standards, rules and regulations that apply to us upon losing our smaller reporting company status. Compliance with the additional requirements also increases our legal, accounting and financial compliance costs.

As a smaller reporting company, we availed ourselves of the exemption from the requirement that our independent registered public accounting firm attest to the effectiveness of our internal control over financial reporting under Section 404 of the Sarbanes-Oxley Act

of 2002. Upon losing our smaller reporting company status beginning with our Quarterly Report on Form 10-Q for the quarter ended March 31, 2026, we are no longer able to rely on the scaled disclosure requirements and other accommodations available to smaller reporting companies. This requires us to provide additional disclosures in our periodic reports, including more detailed executive compensation disclosures and additional financial statement information. The transition from smaller reporting company status requires significant resources, including additional personnel, enhanced systems and processes, and increased professional fees for accounting, legal, and compliance services. We need to implement more comprehensive internal controls and procedures to meet the heightened requirements applicable to larger public companies. Due to the complexity and logistical difficulty of implementing the standards, rules and regulations that apply to non-smaller reporting companies, there is an increased risk that we may be found to be in non-compliance with such standards, rules and regulations or to have significant deficiencies or material weaknesses in our internal controls over financial reporting. Any failure to maintain effective disclosure controls and internal control over financial reporting could materially and adversely affect our business, results of operations and financial condition, and could cause a decline in the trading price of our common stock.

We expect to continue to incur increased costs as a result of operating as a public company, and our management is required to devote substantial time to compliance initiatives.

As a public company, we incur significant legal, accounting and other expenses. In addition, the Sarbanes-Oxley Act and rules subsequently implemented by the SEC and Nasdaq have imposed various requirements on public companies, including establishment and maintenance of effective disclosure and financial controls and corporate governance practices. Our management and other personnel devote a substantial amount of time to these compliance initiatives. These rules and regulations have significantly increased our legal and financial compliance costs and we anticipate that these activities will become more time-consuming and costly over time now that we no longer qualify as an emerging growth company.

Pursuant to Section 404 of the Sarbanes-Oxley Act, we will be required to furnish a report by our management on our internal control over financial reporting. Our independent registered public accounting firm will not be required to formally attest to the effectiveness of our internal control over financial reporting pursuant to Section 404 of the Sarbanes-Oxley Act until the date we report at least $100 million in annual revenues and have a public float of at least $75 million for the most recent fiscal year or have a public float of at least $700 million for the most recent fiscal year. To achieve compliance with Section 404 within the prescribed period, we are engaged in a process to document and evaluate our internal control over financial reporting, which is both costly and challenging. In this regard, we will need to continue to dedicate internal resources, engage outside consultants and adopt a detailed work plan to assess and document the adequacy of internal control over financial reporting, continue steps to improve control processes as appropriate, validate through testing that controls are functioning as documented and implement a continuous reporting and improvement process for internal control over financial reporting. Despite our efforts, there is a risk that neither we nor our independent registered public accounting firm will be able to conclude within the prescribed timeframe that our internal control over financial reporting is effective as required by Section 404. This could result in an adverse reaction to the trading price of our common stock in the financial markets due to a loss of confidence in the reliability of our financial statements.

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.

Effective internal controls over financial reporting are necessary for us to provide reliable financial reports and, together with adequate disclosure controls and procedures, are designed to prevent fraud. Any failure to implement required new or improved controls, or difficulties encountered in their implementation could cause us to fail to meet our reporting obligations. In addition, any testing by us conducted in connection with Section 404, or any subsequent testing by our independent registered public accounting firm, may reveal deficiencies in our internal controls over financial reporting that are deemed to be material weaknesses or that may require prospective or retroactive changes to our financial statements or identify other areas for further attention or improvement. Inferior internal controls could also cause investors to lose confidence in our reported financial information, which could have a negative effect on the trading price of our stock.

We are required to disclose changes made in our internal controls and procedures on a quarterly basis and our management will be required to assess the effectiveness of these controls annually. However, if we were to ever regain status as a “smaller reporting company,” our independent registered public accounting firm would not be required to attest to the effectiveness of our internal controls over financial reporting pursuant to Section 404. We will qualify as a “smaller reporting company” if the market value of our common stock held by non-affiliates is below $250 million (or $700 million if our annual revenue is less than $100 million) as of June 30 in any given year. An independent assessment of the effectiveness of our internal controls over financial reporting could detect problems that our management’s assessment might not. Undetected material weaknesses in our internal controls over financial reporting could lead to financial statement restatements and require us to incur the expense of remediation.

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

Our management has broad discretion in the application of our existing cash, cash equivalents and marketable securities. Because of the number and variability of factors that will determine our use of our existing cash and cash equivalents, their ultimate use may vary substantially from their currently intended use. Our management might not apply our existing cash and cash equivalents in ways that ultimately increase the value of your investment. The failure by our management to apply these funds effectively could harm our business.

Anti-takeover provisions under our charter documents and Delaware law could delay or prevent a change of control which could limit the market price of our common stock and may prevent or frustrate attempts by our stockholders to replace or remove our current management.

Our amended and restated certificate of incorporation and amended and restated bylaws contain provisions that could delay or prevent a change of control of our company or changes in our board of directors that our stockholders might consider favorable. Some of these provisions include:

- a board of directors divided into three classes serving staggered three-year terms, such that not all members of the board will be elected at one time;
- a prohibition on stockholder action through written consent, which requires that all stockholder actions be taken at a meeting of our stockholders;
- a requirement that special meetings of stockholders be called only by the chairman of the board of directors, the chief executive officer, or by a majority of the total number of authorized directors;
- advance notice requirements for stockholder proposals and nominations for election to our board of directors;
- a requirement that no member of our board of directors may be removed from office by our stockholders except for cause and, in addition to any other vote required by law, upon the approval of not less than two thirds of all outstanding shares of our voting stock then entitled to vote in the election of directors;
- a requirement of approval of not less than two thirds of all outstanding shares of our voting stock to amend any bylaws by stockholder action or to amend specific provisions of our amended and restated certificate of incorporation; and
- the authority of the board of directors to issue convertible preferred stock on terms determined by the board of directors without stockholder approval and which convertible preferred stock may include rights superior to the rights of the holders of common stock.

In addition, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporation Law, which may prohibit certain business combinations with stockholders owning 15% or more of our outstanding voting stock. These anti-takeover provisions and other provisions in our amended and restated certificate of incorporation and amended and restated bylaws could make it more difficult for stockholders or potential acquirers to obtain control of our board of directors or initiate actions that are opposed by the then current board of directors and could also delay or impede a merger, tender offer or proxy contest involving our company. These provisions could also discourage proxy contests and make it more difficult for you and other stockholders to elect directors of your choosing or cause us to take other corporate actions you desire. Any delay or prevention of a change of control transaction or changes in our board of directors could cause the market price of our common stock to 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 depends in part on the research and reports that securities or industry analysts publish about us or our business. If one or more of the analysts who covers us downgrades our stock or publishes inaccurate or unfavorable research about our business, our stock price may decline. If one or more of these analysts ceases coverage of our company or fails to publish reports on us regularly, demand for our stock could decrease, which might cause our stock price and trading volume to decline.

We may be subject to securities litigation, which is expensive and could divert management attention.

The market price of our common stock may be volatile. The stock market in general, and Nasdaq and biopharmaceutical companies in particular, have experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to the operating performance of these companies. In the past, companies that have experienced volatility in the market price of their stock have been

subject to securities class action litigation. We may be the target of this type of litigation in the future. Securities litigation against us could result in substantial costs and divert our management's attention from other business concerns, which could seriously harm our business.

Our amended and restated bylaws contain certain exclusive forum provisions requiring that substantially all disputes between us and our stockholders be resolved in certain judicial forums, 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 bylaws provide that the Court of Chancery of the State of Delaware will be the exclusive forum for any derivative action or proceeding brought on our behalf, any action asserting a breach of fiduciary duty, any action asserting a claim against us arising pursuant to the Delaware General Corporation Law, our amended and restated certificate of incorporation or our bylaws, any action to interpret, apply, enforce, or determine the validity of our amended and restated certificate of incorporation or bylaws, or any action asserting a claim against us that is governed by the internal affairs doctrine. In addition, our amended and restated bylaws contain a provision by virtue of which, unless we consent in writing to the selection of an alternative forum, the U.S. District Court for the District of Massachusetts will be the exclusive forum for any complaint asserting a cause of action arising under the Securities Act. In addition, our amended and restated bylaws provide that any person or entity purchasing or otherwise acquiring any interest in shares of our common stock is deemed to have notice of and consented to the foregoing provisions, however, stockholders cannot and will not be deemed to have waived compliance with federal securities laws and the rules and regulations thereunder. We have chosen the U.S. District Court for the District of Massachusetts as the exclusive forum for such causes of action because our principal executive offices are located in Cambridge, Massachusetts. Some companies that have adopted similar federal district court forum selection provisions have had such provisions challenged in legal proceedings by stockholders. While the Delaware Supreme Court ruled in March 2020 in *Salzburg et al. v. Sciabacucchi* that federal forum selection provisions purporting to require claims under the Securities Act be brought in federal court are "facially valid" under Delaware law, there is uncertainty as to whether other courts will enforce our federal forum selection provision, and we may incur additional costs of litigation should such enforceability be challenged. If the federal forum selection provision is otherwise found inapplicable to, or unenforceable in respect of, one or more of the specified actions or proceedings, we may incur additional costs, which could have an adverse effect on our business, financial condition or results of operations. We recognize that the federal district court forum selection clause may impose additional litigation costs on stockholders who assert the provision is not enforceable and may impose more general additional litigation costs in pursuing any such claims, particularly if the stockholders do not reside in or near the Commonwealth of Massachusetts. Additionally, the choice of forum provision 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 such lawsuits against us and our directors, officers and other employees. Alternatively, if a court were to find the choice of forum provision contained in our amended and restated bylaws to be inapplicable or unenforceable in an action, we may incur additional costs associated with resolving such action in other jurisdictions, which could adversely affect our business and financial condition.

We have issued a substantial number of warrants and equity awards from our equity plans which are exercisable into shares of our common stock which could result in substantial dilution to the ownership interests of our existing stockholders.

As of September 30, 2026, approximately 10,558,065 shares of our common stock were reserved for issuance upon exercise of pre-funded warrants, which are already included in our calculation of our weighted average common shares outstanding. Additionally, 11,124,210 shares of our common stock were reserved for issuance upon exercise of outstanding stock options, vested restricted stock units and vested performance stock units. The exercise of these securities will result in a significant increase in the number of outstanding shares and substantially dilute the ownership interests of our existing stockholders. The shares underlying the equity awards from our equity plans are registered on a Form S-8 registration statement. As a result, upon vesting these shares can be freely exercised and sold in the public market upon issuance, subject to volume limitations applicable to affiliates. The exercise of options and the subsequent sale of the underlying common stock could cause a decline in our stock price.

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

The sales of a substantial number of the shares and/or the exercise and sale of a substantial number of the pre-funded warrants and common stock purchase warrants in the public market or the perception that these sales might occur could depress the market price of our common stock and could impair our ability to raise capital through the sale of additional equity securities. We are unable to predict the effect that sales may have on the prevailing market price of our common stock. In addition, the sale of substantial amounts of our common stock could adversely impact the price of our common stock. The sale, or the availability for sale, of a large number of shares of our common stock in the public market could cause the price of our common stock to decline.

The sale or issuance of our common stock to, or through, Jefferies may cause significant dilution and the sale of the shares of common stock acquired by Jefferies, or the perception that such sales may occur, could cause the price of our common stock to fall.

On November 14, 2022, we entered into a sales agreement with Jefferies, pursuant to which we may offer and sell our common stock, subject to certain limitations in the sales agreement and compliance with applicable law, at any time throughout the term of the sales agreement. The number of shares that are sold by Jefferies after delivering a placement notice will fluctuate based on the market price of the common stock during the sales period and limits we set with Jefferies. Because the price per share of each share sold will fluctuate based on the market price of our common stock during the sales period, it is not possible at this stage to predict the number of shares that will be ultimately issued. Sales to, or through, Jefferies by us could result in substantial dilution to the interests of other holders of our common stock. Additionally, the sale of a substantial number of shares of our common stock, or the anticipation of such sales, could make it more difficult for us to sell equity or equity-related securities in the future at a time and at a price that we might otherwise wish to effect sales.

From January 1, 2026 through September 30, 2026, we sold 3,543,692 shares of our common stock resulting in net proceeds to us of $160.8 million pursuant to the Jefferies sales agreement.

Item 2. Unregistered Sales of Equity Securities and Use of Proceeds

None.

Item 3. Defaults Upon Senior Securities

None.

Item 4. Mine Safety Disclosures

None.

Item 5. Other Information

(c)

Trading Plans

During the three months ended September 30, 2026, none of our directors or officers (as defined in Rule 16a-1 (f)) adopted, modified, or terminated a "Rule 10b5-1 trading arrangement" or a "non-Rule 10b5-1 trading arrangement," as each term is defined in Item 408(c) of Regulation S-K of the Exchange Act.

Item 6. Exhibits

EXHIBIT INDEX

Exhibit Number	Description	Incorporated by Reference to: Form	File No.	Exhibit No.	Filing Date
3.1	Amended and Restated Certificate of Incorporation of the Registrant	S-1/A	333-224493	3.2	May 8, 2018
3.2	Amendment to Amended and Restated Certificate of Incorporation of the Registrant	S-1/A	333-224493	3.1.1	May 14, 2018
3.3	Amendment to Amended and Restated Certificate of Incorporation of the Registrant	8-K	001-38501	3.1	June 28, 2024
3.4	Amended and Restated By-laws of the Registrant	S-1/A	333-224493	3.4	May 8, 2018
31.1*	Certification of Principal Executive Officer Pursuant to Rule 13a-14(a) or Rule 15d-14(a) of the Securities Exchange Act of 1934				
31.2*	Certification of Principal Financial Officer Pursuant to Rule 13a-14(a) or Rule 15d-14(a) of the Securities Exchange Act of 1934				
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 as its XBRL tags are embedded within the Inline XBRL document				
101.SCH	Inline XBRL Taxonomy Extension Schema Document With Embedded Linkbase Documents				
104	Cover Page Interactive Data File (formatted as inline XBRL with applicable taxonomy extension information contained in Exhibits 101.)				

* Filed herewith
**Furnished herewith

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.

SCHOLAR ROCK HOLDING CORPORATION

Date: November 10, 2026

By: /s/ David Hallal
David Hallal
Chief Executive Officer
(Principal Executive Officer)

Date: November 10, 2026

By: /s/ Vikas Sinha
Vikas Sinha
Chief Financial Officer
(Principal Financial and Accounting Officer)

Exhibit 31.1

Certifications

I, David Hallal, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Scholar Rock Holding Corporation;

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 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 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 10, 2026

/s/ David Hallal
David Hallal
Chief Executive Officer
(Principal Executive Officer)

Exhibit 31.2

Certifications

I, Vikas Sinha, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Scholar Rock Holding Corporation;

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 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 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 10, 2026

/s/ Vikas Sinha
Vikas Sinha
Chief Financial Officer
(Principal Financial and 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

In connection with the Quarterly Report on Form 10-Q of Scholar Rock Holding Corporation (the "Company") for the period ended September 30, 2026 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), each of the undersigned officers of the Company certifies, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, to his or her knowledge, that:

(1) the Report fully complies with the requirements of Section 13(a) or 15(d), as applicable, of the Securities Exchange Act of 1934, as amended; and

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

This certification is being provided pursuant to 18 U.S.C. 1350 and is not to be deemed a part of the Report, nor is it to be deemed to be "filed" for any purpose whatsoever.

Date: November 10, 2026

/s/ David Hallal
David Hallal
Chief Executive Officer

Date: November 10, 2026

/s/ Vikas Sinha
Vikas Sinha
Chief Financial Officer