Table of Contents

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, 2025

OR

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

FOR THE TRANSITION PERIOD FROM _ TO _

COMMISSION FILE NUMBER 001-38501

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 5, 2025 was 102,007,457.

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 from the U.S. Food and Drug Administration ("FDA"), the European Commission ("EC") and other regulatory authorities for apitegromab, and any related restrictions, limitations or warnings in the label of any approval for apitegromab;
- our ability to obtain regulatory approval for apitegromab is dependent on the timely and satisfactory response to the Complete Response Letter ("CRL") received from the FDA in September 2025 and resolution of the observations identified during the FDA's routine general site inspection of our third-party fill-finish facility. This facility was issued a Form 483 by the FDA in July 2025 and the inspection classification of this facility is "official action indicated" (OAI);
- our plans to address the CRL;
- our ability to successfully build a commercial infrastructure to launch and market apitegromab, or otherwise provide access to apitegromab, if and when it is approved or receives pricing or reimbursement approval;
- our ability, through third party manufacturers, to successfully manufacture commercial supply of apitegromab;
- the rate and degree of market acceptance of apitegromab, if approved;
- 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 apitegromab and its potential advantages over other therapeutic options;
- the potential benefit of orphan drug exclusivity, Orphan Drug designation, Fast Track designation and Rare Pediatric Disease designation for apitegromab;
- our success in identifying and executing development programs for additional 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 study or the results of other ongoing or future studies;
- our success in identifying and executing a development program for our preclinical product candidates, including SRK-439, SRK-373, SRK-256 and identifying additional product candidates from our preclinical 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 complete further development and, upon successful development, if approved, commercialization of apitegromab, SRK-181, SRK-439, SRK-373, SRK-256 or any of our 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 United States 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;
- our expectations regarding the period during which we qualify as a "smaller reporting company" as defined by Rule 12b-2 of the Exchange Act; 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
TABLE OF CONTENTS

	Page
PART I – FINANCIAL INFORMATION	
Item 1. Financial Statements (Unaudited)	6
Condensed Consolidated Balance Sheets as of September 30, 2025 and December 31, 2024	6
Condensed Consolidated Statements of Operations and Comprehensive Loss for the Three and Nine Months Ended September 30, 2025 and 2024	7
Condensed Consolidated Statements of Stockholders' Equity for the Nine Months Ended September 30, 2025 and 2024	8
Condensed Consolidated Statements of Cash Flows for the Nine Months Ended September 30, 2025 and 2024	10
Notes to Condensed Consolidated Financial Statements	11
Item 2. Management's Discussion and Analysis of Financial Condition and Results of Operations	22
Item 3. Quantitative and Qualitative Disclosures About Market Risk	33
Item 4. Controls and Procedures	33
PART II. OTHER INFORMATION	
Item 1. Legal Proceedings	34
Item 1A. Risk Factors	35
Item 2. Unregistered Sales of Equity Securities	85
Item 3. Defaults Upon Senior Securities	86
Item 4. Mine Safety Disclosures	86
Item 5. Other Information	86
Item 6. Exhibits	87
SIGNATURES	88

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, 2025	December 31, 2024
Assets		
Current assets:		
Cash and cash equivalents	$ 286,770	$ 177,878
Marketable securities	82,860	259,400
Prepaid expenses and other current assets	21,081	13,887
Total current assets	390,711	451,165
Property and equipment, net	2,116	2,761
Operating lease right-of-use asset	11,767	15,644
Restricted cash	3,807	2,407
Other long-term assets	3,317	2,945
Total assets	$ 411,718	$ 474,922
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 6,765	$ 10,095
Accrued expenses	49,882	31,067
Operating lease liability	5,325	5,774
Total current liabilities	61,972	46,936
Long-term portion of operating lease liability	5,140	9,206
Long-term debt	99,611	50,146
Total liabilities	166,723	106,288
Commitments and contingencies (Note 10)		
Stockholders' equity:		
Preferred stock, $0.001 par value; 10,000,000 shares authorized; no shares issued and outstanding at September 30, 2025 and December 31, 2024	—	—
Common stock, $0.001 par value; 300,000,000 shares authorized; 99,171,486 and 93,823,678 shares issued and outstanding as of September 30, 2025 and December 31, 2024, respectively	99	94
Additional paid-in capital	1,454,468	1,291,095
Accumulated other comprehensive income	117	160
Accumulated deficit	(1,209,689)	(922,715)
Total stockholders' equity	244,995	368,634
Total liabilities and stockholders' equity	$ 411,718	$ 474,922

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,	
	2025	2024	2025	2024
Operating expenses:				
Research and development	$ 50,486	$ 48,719	$ 161,565	$ 134,185
General and administrative	53,064	16,061	131,184	48,512
Total operating expenses	103,550	64,780	292,749	182,697
Loss from operations	(103,550)	(64,780)	(292,749)	(182,697)
Other income (expense):				
Interest income	2,733	2,083	10,374	8,121
Interest expense	(1,334)	(1,744)	(4,359)	(5,191)
Other expense, net	(69)	(38)	(240)	(73)
Total other income (expense), net	1,330	301	5,775	2,857
Net loss	$ (102,220)	$ (64,479)	$ (286,974)	$ (179,840)
Net loss per share, basic and diluted	$ (0.90)	$ (0.66)	$ (2.54)	$ (1.86)
Weighted average common shares outstanding, basic and diluted	113,729,850	97,050,637	112,763,974	96,587,149
Comprehensive loss:				
Net loss	$ (102,220)	$ (64,479)	$ (286,974)	$ (179,840)
Other comprehensive income (loss):				
Unrealized gain (loss) on marketable securities	59	182	(43)	36
Total other comprehensive income (loss)	59	182	(43)	36
Comprehensive loss	$ (102,161)	$ (64,297)	$ (287,017)	$ (179,804)

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 (Loss)	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
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 restricted stock units ("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 STOCKHOLDERS' EQUITY (CONTINUED)

(Unaudited)
(In thousands, except share data)

	Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
Balance at December 31, 2023	**75,979,495**	**$ 76**	**$ 901,471**	**$ 92**	**$ (676,421)**	**$ 225,218**
Unrealized loss on marketable securities	—	—	—	(142)	—	(142)
Exercise of stock options	47,293	—	280	—	—	280
Issuance of common shares upon RSU vesting	360,373	—	—	—	—	—
Exercise of pre-funded and common warrants	3,357,493	4	6,102	—	—	6,106
Equity-based compensation expense	—	—	8,164	—	—	8,164
Net loss	—	—	—	—	(56,853)	(56,853)
Balance at March 31, 2024	**79,744,654**	**$ 80**	**$ 916,017**	**$ (50)**	**$ (733,274)**	**$ 182,773**
Unrealized loss on marketable securities	—	—	—	(4)	—	(4)
Exercise of stock options	6,617	—	47	—	—	47
Issuance of common shares upon RSU vesting	240,797	—	—	—	—	—
Equity-based compensation expense	—	—	9,312	—	—	9,312
Net loss	—	—	—	—	(58,508)	(58,508)
Balance at June 30, 2024	**79,992,068**	**$ 80**	**$ 925,376**	**$ (54)**	**$ (791,782)**	**$ 133,620**
Unrealized gain on marketable securities	—	—	—	182	—	182
Exercise of stock options	41,078	—	223	—	—	223
Issuance of common shares upon RSU vesting	48,389	—	—	—	—	—
Equity-based compensation expense	—	—	9,650	—	—	9,650
Net loss	—	—	—	—	(64,479)	(64,479)
Balance at September 30, 2024	**80,081,535**	**$ 80**	**$ 935,249**	**$ 128**	**$ (856,261)**	**$ 79,196**

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,	
	2025	2024
Cash flows from operating activities:		
Net loss	$ (286,974)	$ (179,840)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	1,233	1,542
Amortization of debt discount and debt issuance costs	325	206
Equity-based compensation	56,177	27,126
Amortization/accretion of investment securities	(3,791)	(3,945)
Non-cash operating lease expense	3,877	4,034
Change in operating assets and liabilities:		
Prepaid expenses and other current assets	(5,965)	(5,734)
Other assets	(372)	608
Accounts payable	(3,331)	606
Accrued expenses	18,797	8,705
Operating lease liabilities	(4,515)	(4,744)
Net cash used in operating activities	(224,539)	(151,436)
Cash flows from investing activities:		
Purchases of property and equipment	(570)	(76)
Purchases of marketable securities	(98,009)	(82,710)
Sales and maturities of marketable securities	278,298	202,100
Net cash provided by investing activities	179,719	119,314
Cash flows from financing activities:		
Proceeds from stock option exercises	10,945	677
Proceeds from pre-funded and common warrant exercises	3,367	6,106
Proceeds from the sale of common shares, net	91,658	—
Proceeds from debt facility	49,992	—
Debt modification payment	—	(125)
Proceeds from debt refinancing	24,668	—
Payment of long-term debt	(25,520)	—
Other	2	
Net cash provided by financing activities	155,112	6,658
Net increase (decrease) in cash, cash equivalents and restricted cash	110,292	(25,464)
Cash, cash equivalents and restricted cash, beginning of period	180,285	104,262
Cash, cash equivalents and restricted cash, end of period	$ 290,577	$ 78,798
Supplemental disclosure for non-cash items:		
Operating lease liability adjustment from rent modification	$ —	$ 9,451
Property and equipment purchases in accounts payable & accrued expenses	$ —	$ 22
Stock options exercised receivable	$ 1,229	$ —
Supplemental cash flow information:		
Cash paid for interest	$ 4,038	$ 5,003

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 dramatically improving the lives of children and adults with spinal muscular atrophy ("SMA") and additional rare, severe and debilitating neuromuscular diseases. As a global 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. By targeting the signaling proteins at the cellular level and acting in the disease microenvironment, the Company believes that it may avoid the historical dose-limiting safety challenges associated with inhibiting growth factors for therapeutic effect. The Company believes its focus on biologically validated growth factors may facilitate a more efficient development path.

The Company's first product candidate, apitegromab, is a highly selective, fully human, monoclonal antibody, with a unique mechanism of action that results in inhibition of the activation of the growth factor, myostatin, in skeletal muscle. Apitegromab is being developed as a potential first muscle-targeted treatment for SMA. In October 2024, the Company announced positive top-line results from SAPPHIRE, a pivotal Phase 3 clinical trial to evaluate the efficacy and safety of apitegromab in patients with non-ambulatory SMA. The study achieved its primary endpoint. The Company submitted a U.S. Biologics License Application ("BLA") to the FDA in January 2025 and the BLA was granted priority review designation. In September 2025, the Company received a Complete Response Letter ("CRL") from the FDA related to observations identified during a routine general site inspection of a third-party fill-finish facility. The facility was issued a Form 483 by the FDA in July 2025 and the inspection classification of this facility is "official action indicated" (OAI). The observations were site-related and not specific to apitegromab. The CRL did not cite any other approvability concerns, including apitegromab's efficacy and safety data or the third-party drug substance manufacturer. In November 2025, the Company completed a constructive in-person Type A meeting with the FDA that included participation of representatives from the third-party fill-finish facility. The Company plans to resubmit the apitegromab BLA at such time as the facility has been reclassified by the FDA. In March 2025, the Company submitted to the European Medicines Agency ("EMA") and received validation of its marketing authorisation application ("MAA") for apitegromab for the treatment of SMA. The FDA granted Fast Track designation, Rare Pediatric Disease designation and Orphan Drug designation to apitegromab for the treatment of SMA in May 2021, August 2020 and March 2018, respectively. The EMA granted Priority Medicines ("PRIME") designation in March 2021 and the European Commission ("EC") granted orphan medicinal product designation in December 2018 to apitegromab for the treatment of SMA. If apitegromab is approved by the FDA or EMA, the Company expects to initiate a commercial product launch in the applicable jurisdictions upon approval. The Company continues to develop apitegromab 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.

In October 2023, the Company announced the expansion of its anti-myostatin program with SRK-439, a novel, fully human anti-myostatin monoclonal antibody with high potency and subcutaneous formulation. The Company intends to explore SRK-439's potential in various, severe debilitating neuromuscular disorders and in September 2025, an investigational new drug application was cleared by the FDA. The Company plans to initiate dosing of SRK-439 in healthy volunteers in the fourth quarter of 2025.

The Company's product candidate, SRK-181, a highly selective inhibitor of the activation of latent TGFβ1, is being developed for the treatment of cancers that are resistant to checkpoint inhibitor therapies, such as anti-PD-1 or anti-PD-L1 antibody therapies (referred to together as anti-PD-(L)1 antibody therapies). SRK-181 has been evaluated in the Company's Phase 1 DRAGON proof-of-concept clinical trial in patients with locally advanced or metastatic solid tumors that exhibit resistance to anti-PD-(L)1 antibody therapies. The Company completed the Phase 1 DRAGON trial in June 2025. This two-part clinical trial consisted of a dose escalation portion (Part A) and a dose expansion portion

evaluating SRK-181 in combination with an approved anti-PD-(L)1 antibody therapy (Part B). Part B included the following cohorts: urothelial carcinoma, cutaneous melanoma, non-small cell lung cancer, clear cell renal cell carcinoma and head and neck squamous cell carcinoma.

Additionally, the Company continues to discover and develop a pipeline of product candidates to deliver life-changing therapies for people with serious diseases that have high unmet need, including neuromuscular disorders, cardiometabolic disorders, cancer, and other conditions where growth factor-targeted therapies can play a transformational role. The Company was originally formed in May 2012. Its principal offices are 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 and performing research and development activities. The Company has primarily financed its operations through various equity financings, as well as research and development collaboration agreements and the Company's debt facility (see Note 11).

Revenue generation activities have been limited to two collaborations, both containing research services and the issuance of a license. No revenues have been recorded from the sale of any commercial product.

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

The Company believes that its existing cash, cash equivalents and marketable securities at September 30, 2025 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, 2024, and the notes thereto, which are included in the Company's Annual Report on Form 10-K. 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, 2024.

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,	
	2025	2024
Cash and cash equivalents	$ 286,770	$ 76,391
Restricted cash	3,807	2,407
	$ 290,577	$ 78,798

The Company's restricted cash is associated with a letter of credit related to its lease at 301 Binney Street in Cambridge and a collateral account associated with a corporate credit card program.

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.

Recently Adopted Accounting Pronouncements

In November 2023, the Financial Accounting Standards Board ("FASB") issued Accounting Standards Update ("ASU") 2023-07, *Segment Reporting (Topic 280): Improvements to Reportable Segment Disclosures* ("ASU 2023-07"). The standard requires disclosure of incremental segment information on an annual and interim basis and allows for multiple measures of a segment's profit or loss provided that one of those measures is consistent with GAAP. The amendments in this update do not change how a public company identifies its operating segments, aggregates those operating segments, or applies the quantitative thresholds to determine its reportable segments, but rather requires public entities to disclose significant segment expenses and other segment items on an annual and interim basis and provide in interim periods all disclosures about a reporting segment's profit or loss and assets that are currently required annually. ASU 2023-07 became effective for the annual period starting on January 1, 2024, and for interim periods starting on January 1, 2025**.** The adoption of this standard did not have a material impact on the Company's condensed consolidated financial position and results of operations.

Recently Issued Accounting Pronouncements

In December 2023, the FASB issued ASU 2023-09, *Income Taxes (Topic 740): Improvements to Income Tax Disclosures* ("ASU 2023-09"), which enhances the transparency of income tax disclosures to provide information to investors to better assess how a company's operations and related tax risks, tax planning and operational opportunities affect its tax rate and prospects for future cash flows. This requires public entities to disclose additional categories in the rate reconciliation regarding federal and state income taxes, and provide more details surrounding reconciling items if a quantitative threshold is met. The effective date for public companies is for annual periods starting on January 1, 2025. Early adoption is permitted for annual financial statements that have not yet been issued or made available for issuance, however, the Company has decided not to early adopt, does not anticipate a material impact to its net financial position, and is still evaluating the impact on its disclosures will be as a result of the adoption of ASU 2023-09.

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, 2025 and December 31, 2024 (in thousands):

	Fair Value Measurements at September 30, 2025			
	Total	Level 1	Level 2	Level 3
Assets:				
Cash and cash equivalents:				
Money market funds	$ 253,151	$ 253,151	$ —	$ —
U.S. treasury obligations	19,984	19,984	—	—
Marketable securities:				
U.S. treasury obligations and government agency securities	82,860	82,860	—	—
Total assets	$ 355,995	$ 355,995	$ —	$ —

	Fair Value Measurements at December 31, 2024			
	Total	Level 1	Level 2	Level 3
Assets:				
Cash and cash equivalents:				
Money market funds	$ 97,290	$ 97,290	$ —	$ —
U.S. treasury obligations	63,171	63,171	—	—
Marketable securities:				
U.S. treasury obligations and government agency securities	259,400	259,400	—	—
Total assets	$ 419,861	$ 419,861	$ —	$ —

Level 1 assets include investments in money market funds, U.S. treasury obligations and government agency securities that are valued using quoted market prices. There were no transfers of assets between fair value measurement levels during the nine months ended September 30, 2025 or 2024.

The carrying amounts reflected in the balance sheets for prepaid expenses and other current assets, accounts payable, and accrued expenses approximate their fair values at September 30, 2025 and December 31, 2024, due to their short-term nature.

The Company believes the terms of its debt reflect current market conditions for an instrument with similar terms and maturity, therefore the carrying value of the Company's debt approximates its fair value based on Level 3 of the fair value hierarchy.

4. Marketable Securities

The following table summarizes the Company's investments as of September 30, 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	$ 82,743	$ 117	$ —	$ 82,860
Total available-for-sale securities	$ 82,743	$ 117	$ —	$ 82,860

The following table summarizes the Company's investments as of December 31, 2024 (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	$ 259,240	$ 180	$ (20)	$ 259,400
Total available-for-sale securities	$ 259,240	$ 180	$ (20)	$ 259,400

Amortized cost approximated fair value for money market funds and U.S. treasury obligations included in cash and cash equivalents.

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, 2025.

5. Prepaid Expenses and Other Assets

At September 30, 2025 and December 31, 2024, prepaid expenses and other current assets consist of the following (in thousands):

	As of September 30, 2025	As of December 31, 2024
Prepaid external research and development expenses	$ 10,405	$ 7,716
Prepaid other	3,845	2,584
Receivables	3,549	2,151
Prepaid compensation expense	1,470	377
Prepaid professional services expense	1,023	419
Prepaid insurance	789	640
	$ 21,081	$ 13,887

At September 30, 2025 and December 31, 2024, other long-term assets consist of the following (in thousands):

	As of September 30, 2025	As of December 31, 2024
Prepaid external research and development expenses	$ 2,590	$ 2,395
Prepaid other	689	536
Prepaid insurance	38	14
	$ 3,317	$ 2,945

6. Accrued Expenses

At September 30, 2025 and December 31, 2024, accrued expenses consist of the following (in thousands):

	As of	
	September 30, 2025	December 31, 2024
Accrued payroll and related expenses	$ 22,738	$ 14,776
Accrued external research and development expense	16,916	12,116
Accrued professional services expense	9,361	3,296
Accrued other	867	879
	$ 49,882	$ 31,067

7. Common Stock

In June 2024, the stockholders approved an amendment to the Company's amended and restated certificate of incorporation to increase the number of authorized shares of common stock from 150,000,000 to 300,000,000.

In October 2024, the Company entered into an underwriting agreement (the "Underwriting Agreement") with J.P. Morgan Securities LLC, Jefferies LLC and Piper Sandler & Co., as representatives of the several underwriters named therein (the "Underwriters"), relating to the issuance and sale of an aggregate of 10,265,488 shares of the Company's common stock at $28.25 per share and pre-funded warrants to purchase 353,983 shares of its common stock. The offering price per pre-funded warrant was $28.2499, which equals the per share public offering price for the common shares less the $0.0001 exercise price for each such pre-funded warrant. The pre-funded warrants are exercisable at any time and only expire when exercised in full. The offering closed on October 10, 2024. Pursuant to the Underwriting Agreement, the Underwriters were granted a 30-day option to purchase up to 1,592,920 additional shares (the "Option Shares") of common stock, which was exercised in full on October 16, 2024. Total proceeds of the transaction, including the Option Shares were approximately $324.4 million, net of underwriting discounts and estimated offering expenses.

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 ATM agreement with Jefferies, established 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. As of September 30, 2025, the Company has sold 3,386,290 shares, generating net proceeds of $96.9 million, under the ATM program. Of this amount, the Company sold 2,767,000 shares of its common stock under the ATM program during the nine months ended September 30, 2025, generating net proceeds of $91.7 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 pre-funded warrants do not expire and are exercisable at any time. In October 2024, June 2022 and November 2020, the Company issued 353,983, 25,510,205 and 2,179,487 pre-funded warrants, respectively. During the nine months ended September 30, 2025 and 2024, 0 and 2,526,833, respectively, of the Company's pre-funded warrants were exercised. As of September 30, 2025, the Company has 17,362,147 pre-funded warrants outstanding. In June 2022, the Company also issued 10,459,181 warrants with an exercise price of $7.35, which expire on December 31, 2025. During the nine months ended September 30, 2025 and 2024, 458,044 and 830,660, respectively, of the Company's warrants were exercised. As of September 30, 2025, the Company has 8,220,620 warrants outstanding.

Shares Reserved For Future Issuance

As of September 30, 2025, the Company had common shares reserved for issuance as follows:

	As of September 30, 2025
Common shares reserved for exercise of pre-funded warrants	17,362,147
Common shares reserved for issuance upon exercise of outstanding warrants	8,220,620
Common shares reserved for exercise of outstanding stock options and unvested restricted stock units under the 2017 and 2018 Plans	8,376,160
Common shares reserved for exercise of outstanding stock options and unvested restricted stock units under the 2022 Inducement Plan	5,170,742
Common shares reserved for future issuance under the 2018 Plan	3,816,104
Common shares reserved for future issuance under the 2022 Inducement Plan	1,155,782
Common shares reserved for future issuance under the 2018 ESPP	2,550,305
	46,651,860

8. 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, 2025 and 2024 (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Research and development expense	$ 5,497	$ 4,398	$ 15,375	$ 11,980
General and administrative expense	12,834	5,252	40,802	15,146
	$ 18,331	$ 9,650	$ 56,177	$ 27,126

During the nine months ended September 30, 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 during the three and nine months ended September 30, 2025 includes zero and $13.0 million, respectively, related to the modification of certain equity awards. No equity modifications occurred during the three months ended September 30, 2025.

The following table summarizes the Company's unrecognized equity-based compensation expense as of September 30, 2025:

	As of September 30, 2025	
	Unrecognized Expense (in thousands)	Weighted Average Remaining Period of Recognition (years)
RSUs	$ 73,419	2.8
Performance stock units ("PSUs")	22,338	3.6
Stock options	76,942	2.7
	$ 172,699	

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, 2024	3,486,668	$ 13.86
Granted	2,135,193	$ 34.15
Vested	(1,162,887)	$ 12.78
Forfeited	(923,054)	$ 19.55
RSUs as of September 30, 2025	3,535,920	$ 24.98

The total fair value of RSUs vested during the nine months ended September 30, 2025 and 2024 was $44.0 million and $8.6 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, 2024	—	$ —
Granted	1,100,000	$ 28.63
PSUs as of September 30, 2025	1,100,000	$ 28.63

In April 2025, the Company granted PSUs to certain employees. The number of PSUs granted represents the target number of units that are eligible to be earned based on certain market-based conditions, subject to continued employment through applicable service vesting dates. The Company concluded that issued PSUs are equity-based awards and include market and service-based vesting conditions. Participants may ultimately earn up to 250% of the target number of units granted based on the degree of actual performance metric achievement. The Company estimated the fair value of these PSUs using a Monte-Carlo simulation model at the grant date. The Monte Carlo simulation model requires key inputs including risk-free interest rate, dividend yield and historical volatility.

No PSUs vested during the three and 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, 2024	8,469,926	$ 15.19	7.83	$ 244,677
Granted	2,711,695	$ 34.24		
Exercised	(959,877)	$ 12.68		
Cancelled	(1,310,762)	$ 20.39		
Outstanding as of September 30, 2025	8,910,982	$ 20.49	6.59	$ 159,643
Options exercisable as of September 30, 2025	4,517,884	$ 16.86	4.95	$ 101,236

Using the Black-Scholes option pricing model, the weighted average grant date fair value of options granted during the nine months ended September 30, 2025 and 2024 was $28.73 and $10.38, respectively.

The following weighted average assumptions were used in determining the grant date fair value of options granted in the nine months ended September 30, 2025 and 2024:

	Nine Months Ended September 30,	
	2025	2024
Risk-free interest rate	4.07 %	4.09 %
Expected dividend yield	0.0 %	0.0 %
Expected term (in years)	6.07	6.00
Expected volatility	108.29 %	91.15 %

9. 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.

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Lease cost:				
Operating lease cost	$ 1,714	$ 1,714	$ 5,142	$ 5,290
Variable lease cost	381	363	1,153	1,086
Total lease cost	$ 2,095	$ 2,077	$ 6,295	$ 6,376

	Nine Months Ended September 30,	
	2025	2024
Other information:		
Operating cash flows used for operating leases	$ 5,779	$ 6,000
Weighted average remaining lease term	1.9	2.9
Weighted average incremental borrowing rate	13.1 %	13.1 %

10. Commitments and Contingencies

Legal Proceedings

The Company, from time to time, may be party to litigation arising in the ordinary course of its business. The Company was not subject to any material legal proceedings during the nine months ended September 30, 2025 and 2024.

11. Debt

On October 16, 2020 (the "Closing Date") 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. Pursuant to the Loan and Security Agreement, the Company was required to maintain cash equal to the lesser of 100% of the Company's consolidated cash or 105% of the dollar amount of the outstanding debt.

On February 10, 2025, the Company entered into an Amended and Restated Loan and Security Agreement (the "Amended and Restated Loan and Security Agreement") with Oxford. The Amended and Restated Loan and Security Agreement amends and restates in its entirety that certain Loan and Security Agreement, as amended.

The Amended and Restated Loan and Security Agreement provides for term loans in an aggregate principal amount of up to $200.0 million (each, a "Term Loan" and together, the "Term Loans") subject to funding in four tranches. The Amended and Restated Loan and Security Agreement consolidates the existing outstanding loan tranches solely with Oxford and also extends the interest-only payment period through March 2029, with principal payments to commence in April 2029. The maturity date of the loan was extended to February 1, 2030. If certain business and development milestones are achieved, the interest-only payment period may be extended by an additional twelve months, and the maturity date will be extended to February 1, 2031. In conjunction with the Amended and Restated Loan and Security 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.

The outstanding principal of each Term Loan has an annual interest rate of (a) the greater of (i) the 1-Month CME Term SOFR on the last business day of the month that immediately precedes the month in which the interest will accrue and (ii) 3%, plus (b) 5.5%. Interest is payable on a monthly basis based on the principal amount outstanding during the preceding month. In addition, the Company is required to pay Oxford a final payment fee equal to 2.00% of the original principal amount of each Term Loan advanced to the Company.

On September 19, 2025, the Company entered into a Third Amendment to the Amended and Restated Loan and Security Agreement (the "Third Amendment") with Oxford. Pursuant to the Third Amendment, the Company was required to maintain cash equal to the lesser of 100% of the Company's consolidated cash or 115% of the dollar amount of the outstanding debt in a U.S. controlled bank account.

On September 29, 2025, the Company received $50.0 million from the next available tranche under the Amended and Restated Loan and Security Agreement. To date, the Company has received $100.0 million of Term Loans. The remaining two tranches, each in an amount of $50.0 million, are available to be borrowed (a) in the case of the third tranche, after the achievement of certain development and business performance milestones until September 30, 2026 and (b) in the case of the fourth tranche, after the achievement of certain development and business performance milestones until December 31, 2027.

12. 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,	
	2025	2024
RSUs	3,535,920	3,386,857
PSUs	1,100,000	—
Stock options	8,910,982	9,670,164
Warrants	8,220,620	9,157,496
	21,767,522	22,214,517

13. Segment Reporting

The Company operates and manages its business as a single 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 and therefore we have presented segment information on the same basis. When evaluating the Company's financial performance, the CODM regularly reviews net loss, non-operating expenses and operating expenses with non-cash expenses such as depreciation and equity-based compensation expense removed. The CODM considers net loss in making decisions on how to allocate resources. The measure of segment assets is reported on the balance sheet as total consolidated assets. All of the Company's long-lived assets are held in the United States.

The following table presents significant expense information about the Company's operating segment:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Operating expenses:				
Employee related expense	$ 34,982	$ 16,876	$ 90,404	$ 49,076
External R&D expense - Apitegromab	21,361	22,523	68,618	57,852
External R&D expense - SRK-181	357	2,064	2,259	8,069
External R&D expense - SRK-439	1,608	2,787	8,462	6,321
External R&D expense - Early research and other	1,129	589	4,811	1,998
External expense - G&A	19,433	5,093	43,328	16,221
Other segment related expense*	5,926	4,752	17,457	14,492
Employee related equity-based compensation expense	18,331	9,650	56,177	27,126
Depreciation and amortization expense	423	446	1,233	1,542
Other non-operating expense/(income), net	(1,330)	(301)	(5,775)	(2,857)
Net loss	$ 102,220	$ 64,479	$ 286,974	$ 179,840

* Consists of other segment expenses related to supplies, corporate and facilities expenses.

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, 2024.

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 not to place 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 dramatically improving the lives of children and adults with SMA and additional rare, severe and debilitating neuromuscular diseases. As a global leader in the biology of TGFβ superfamily, our novel understanding of the molecular mechanisms of growth factor activation enabled us to develop a proprietary platform for the discovery and development of monoclonal antibodies that locally and selectively target the precursor, or latent, forms of growth factors. By targeting the signaling proteins at the cellular level and acting in the disease microenvironment, we believe we may avoid the historical dose-limiting safety challenges associated with inhibiting growth factors for therapeutic effect. We believe our focus on biologically validated growth factors may facilitate a more efficient development path.

Based on this proprietary and scalable technology platform, we are building a growing portfolio of novel product candidates to deliver life-changing therapies for people with serious diseases that have high unmet need, including neuromuscular disorders, cardiometabolic disorders, cancer, and other conditions where growth factor-targeted therapies can play a transformational role. We have discovered and progressed the development of:

- Apitegromab, an investigational, fully human monoclonal antibody that inhibits myostatin activation by selectively binding the pro- and latent forms of myostatin in skeletal muscle and is being developed for the treatment of SMA. We also believe apitegromab could have potential in the treatment of other neuromuscular disorders where the inhibition of myostatin may be beneficial.
- SRK-439, a novel, investigational myostatin inhibitor that has high in vitro affinity for pro- and latent myostatin and maintains myostatin specificity and is being developed for the treatment of neuromuscular diseases.
- SRK-181, an investigational inhibitor of the activation of latent TGFβ1, that is being developed for the treatment of cancers that are resistant to anti-PD-(L)1 antibody therapies.
- SRK-373, a novel, preclinical, investigational TGFβ inhibitor that selectively inhibits the activation of latent TGFβ1 isoform in the context of fibrotic extracellular matrix and that avoids perturbing TGFβ1 presented by cells of the immune system and is being developed for the treatment of fibrotic diseases.

- SRK-256, a novel, preclinical, investigational inhibitor that selectively inhibits RGMc or hemojuvelin, the co-receptor of bone morphogenic protein 6 (“BMP6”), and hence inhibits BMP6 signaling. BMP6 signaling is critical for iron homeostasis and SRK-256 has wide potential applicability in states of iron-restricted anemias.
- Additional discovery and early preclinical programs related to the selective modulation of growth factor signaling.

Our first product candidate, apitegromab, is a highly selective, fully human, monoclonal antibody, with a unique mechanism of action that results in inhibition of the activation of the growth factor, myostatin, in skeletal muscle. Apitegromab is being developed as a potential first muscle-targeted treatment for SMA. In October 2024, we announced positive top-line results in SAPPHIRE, a pivotal Phase 3 clinical trial to evaluate the efficacy and safety of apitegromab in patients with non-ambulatory Type 2 and Type 3 SMA (which is estimated to represent the majority of the current prevalent SMA patient population in the U.S. and Europe). The study achieved its primary endpoint. At the March 2025 Muscular Dystrophy Association Clinical & Scientific Conference, we presented additional data related to secondary endpoint analyses in which apitegromab demonstrated a clinically meaningful and consistent benefit in motor function across pre-specified patient subgroups. We submitted a U.S. Biologics License Application to the FDA in January 2025 and the BLA was granted priority review designation. Priority review designation conveys that the FDA has determined that if apitegromab is approved, it could offer significant improvement in the safety or effectiveness of treatment of the serious condition of SMA. In September 2025, we received a Complete Response Letter (“CRL”) from the FDA related to observations identified during a routine general site inspection of a third-party fill-finish facility. The facility was issued a Form 483 by the FDA in July 2025 and the inspection classification of this facility is “official action indicated” (OAI). The observations were site-related and not specific to apitegromab. The CRL did not cite any other approvability concerns, including apitegromab’s efficacy and safety data or the third-party drug substance manufacturer. In November 2025, we completed a constructive in-person Type A meeting with the FDA that included participation of representatives from the third-party fill-finish facility. We plan to resubmit the apitegromab BLA at such time as the facility has been reclassified by the FDA. In March 2025 we submitted to the European Medicines Agency (“EMA”) and received validation of our marketing authorisation application (“MAA”) for apitegromab for the treatment of SMA. Validation confirms that the application includes the essential regulatory elements required for scientific assessment of the MAA and the scientific evaluation process by the EMA’s Committee for Medicinal Products for Human Use can begin. If apitegromab is approved by the FDA or EMA, we expect to initiate a commercial product launch in the applicable jurisdictions upon approval.

Apitegromab was evaluated in our Phase 2 TOPAZ proof-of-concept clinical trial for the treatment of patients with Type 2 and Type 3 SMA. Positive 12-month top-line results were initially announced in April 2021. We have subsequently presented data from the TOPAZ trial over 24-months (2022), 36-months (2023) and 48-months (2024). At 48-months over 90% of TOPAZ patients with non-ambulatory Type 2 and 3 SMA receiving a survival motor neuron (“SMN”) targeted treatment remained on apitegromab and showed sustained clinical benefit, with a continued generally favorable safety profile and no new safety findings. Additionally, we are conducting a long-term extension study, ONYX, for patients from both the TOPAZ and SAPPHIRE studies, who are receiving apitegromab in conjunction with an approved SMN-targeted treatment. The FDA granted Fast Track designation, Rare Pediatric Disease designation and Orphan Drug designation to apitegromab for the treatment of SMA in May 2021, August 2020 and March 2018, respectively. The EMA granted Priority Medicines (“PRIME”) designation in March 2021 and the EC granted orphan medicinal product designation in December 2018 to apitegromab for the treatment of SMA.

We believe that apitegromab has the potential to be the first muscle-targeted treatment that is aimed at improving motor function in patients with SMA who are receiving an SMN-targeted therapy. We also have identified opportunities to potentially expand the therapeutic benefit of apitegromab, including additional patient populations in SMA (such as patients with SMA under two years of age) and multiple other diseases beyond SMA where selective inhibition of myostatin activation may offer therapeutic benefit. We initiated the Phase 2 OPAL trial in SMA patients under two years of age in the third quarter of 2025.

In addition to advancing development of apitegromab in neuromuscular diseases, we continue to advance our efforts for a second anti-myostatin product candidate and explore therapeutic benefit beyond neuromuscular disorders. In October 2023, we announced an expansion of our anti-myostatin program with SRK-439, a novel, fully human anti-myostatin

monoclonal antibody and in September 2025, an investigational new drug application ("IND") for SRK-439 was cleared by the FDA. The Company plans to initiate dosing of SRK-439 in healthy volunteers in the fourth quarter of 2025.

In 2024, we presented preclinical data at scientific conferences which support the potential of SRK-439 to increase lean mass and contribute to a favorable body composition in conjunction with GLP-1 receptor agonist ("GLP-1 RA") treatment. In June 2025, the Company announced positive top-line results from the Phase 2 EMBRAZE proof-of-concept trial, designed to assess the safety and efficacy of apitegromab to preserve lean mass in individuals living with obesity and on background therapy of a GLP-1 RA (tirzepatide). The trial demonstrated that patients receiving apitegromab with tirzepatide over 24 weeks showed a 54.9% preservation of lean mass (+4.2 lbs of lean mass) versus tirzepatide alone (p=0.001).

Our product candidate, SRK-181, a highly selective inhibitor of the activation of latent TGFβ1, is being developed for the treatment of cancers that are resistant to checkpoint inhibitor therapies, such as anti-PD-1 or anti-PD-L1 antibody therapies (referred to together as anti-PD-(L)1 antibody therapies). SRK-181 has been evaluated in our Phase 1 DRAGON proof-of-concept clinical trial in patients with locally advanced or metastatic solid tumors that exhibit resistance to anti-PD-(L)1 antibody therapies. We completed the DRAGON trial in June 2025. This two-part clinical trial consisted of a dose escalation portion (Part A) and a dose expansion portion evaluating SRK-181 in combination with an approved anti-PD-(L)1 antibody therapy (Part B). Part B included the following cohorts: urothelial carcinoma, cutaneous melanoma, non-small cell lung cancer, clear cell renal cell carcinoma ("ccRCC"), and head and neck squamous cell carcinoma. The data showed encouraging responses in heavily pretreated and anti-PD-(L)1 resistant patients across multiple tumor types. We believe that the DRAGON trial achieved its study objectives by showing objective, durable clinical responses in patients with ccRCC resistant to PD-1 therapy above what is expected from continuing PD-1 alone.

Using our innovative approach and proprietary platform, we are creating a pipeline of novel product candidates that selectively modulate the activation of growth factors implicated in a variety of serious diseases with high unmet need, including neuromuscular disorders, cardiometabolic disorders, cancer, and other serious diseases where growth factor-targeted therapies can play a transformational role. Our proprietary platform is designed to generate highly selective antibodies that target the growth factor's latent precursor form prior to its activation within the disease microenvironment, or tissue where it is localized. Our structural insights and unique antibody discovery capabilities can also be applied to other protein classes beyond growth factors, with an aim of generating differentiated candidates targeting cell surface receptors such as immune cell receptors or G-protein coupled receptors, where selectivity remains challenging.

We have incurred significant operating losses since inception. Our net losses were $287.0 million for the nine months ended September 30, 2025. As of September 30, 2025, we had an accumulated deficit of $1.2 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 apitegromab, including the close out of our Phase 3 SAPPHIRE pivotal clinical trial in SMA, the conduct of ONYX, the conduct of our Phase 2 OPAL study for SMA patients under two years of age and the associated drug supply;
- continue research and development activities for our anti-myostatin program, including our planned 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 infrastructure to support our operations as a public company.

To date, we have not generated any revenue from product sales. If we successfully obtain regulatory approval for apitegromab we may generate revenue in the future from product sales. In addition, if we obtain regulatory approval for apitegromab we have and expect to incur significant expenses related to developing our commercialization capabilities to support product sales, marketing and distribution activities.

Financial Operations Overview

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, preclinical 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 preclinical 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 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.

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, 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, 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 preclinical development activities, clinical trials and other research and development activities;
- 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 from applicable regulatory authorities, if any;
- 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;
- commercializing the product candidates, if and when approved, whether alone or in collaboration with others; and
- continued acceptable safety profile of the products following any regulatory approval.

A change in the outcome of any of these variables with respect to the development of apitegromab, 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.

General and Administrative

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 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 general and administrative expense to continue to be substantial as we continue to invest in building the infrastructure to support the commercialization of apitegromab.

Other Income (Expense), Net

Other income (expense), net consists primarily of interest income earned on our cash, cash equivalents and marketable securities, partially 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, 2025 and 2024

The following table summarizes our results of operations for the three months ended September 30, 2025 and 2024 (in thousands, except percentages):

	Three Months Ended September 30,		Change	
	2025	2024	$	%
Operating expenses:				
Research and development	$ 50,486	$ 48,719	$ 1,767	3.6 %
General and administrative	53,064	16,061	37,003	230.4 %
Total operating expenses	103,550	64,780	38,770	59.8 %
Loss from operations	(103,550)	(64,780)	(38,770)	59.8 %
Other income (expense), net	1,330	301	1,029	341.9 %
Net loss	$ (102,220)	$ (64,479)	$ (37,741)	58.5 %

Operating Expenses

Research and Development

Research and development expense was $50.5 million and $48.7 million for the three months ended September 30, 2025 and 2024, respectively, an increase of $1.8 million or 3.6%. The following table summarizes our research and development expense for the three months ended September 30, 2025 and 2024 (in thousands, except percentages):

	Three Months Ended September 30,		Change	
	2025	2024	$	%
External costs by program				
Apitegromab	$ 21,361	$ 22,523	$ (1,162)	(5.2)%
SRK-181	357	2,064	(1,707)	(82.7)%
SRK-439	1,608	2,787	(1,179)	(42.3)%
Other early programs and unallocated costs	1,129	589	540	91.7 %
Total external costs	24,455	27,963	(3,508)	(12.5)%
Internal costs:				
Employee compensation and benefits	21,423	16,552	4,871	29.4 %
Facility and other	4,608	4,204	404	9.6 %
Total internal costs	26,031	20,756	5,275	25.4 %
Total research and development expense	$ 50,486	$ 48,719	$ 1,767	3.6 %

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

- A decrease in our external research and development costs of $3.5 million, which primarily consisted of:
  - $1.2 million decrease in costs associated with apitegromab primarily due to a decrease in clinical trial costs as our Phase 2 TOPAZ trial extension period and Phase 3 SAPPHIRE clinical trial are completed, partially offset by an increase in drug supply manufacturing and the initiation of our Phase 2 OPAL trial in SMA patients under two years of age;
  - $1.7 million decrease in costs associated with SRK-181, as our Phase 1 DRAGON trial is completed;
  - $1.2 million decrease in preclinical costs and manufacturing development for SRK-439; and
  - $0.5 million increase in other early development candidates and unallocated costs.

- $5.3 million increase in internal research and development costs, which was primarily driven by an increase of $3.0 million in employee related costs, including salaries, bonus, benefits and payroll taxes related to increased headcount, an increase of $1.1 million in non-cash equity-based compensation expense related to increased headcount and severance related costs of $0.8 million.

Total research and development expenses are expected to continue to be substantial, driven by employee compensation costs and development costs associated with our manufacture of drug supply, as well as development of our clinical stage programs as we continue development activities for apitegromab in SMA, the conduct of ONYX, the conduct of our Phase 2 OPAL trial in SMA patients under the age of two, as well as costs associated with supporting our anti-myostatin program, including SRK-439. Additionally, we will continue to invest in our pipeline. We expect costs of our SRK-181 program to decrease, as we completed the Phase 1 DRAGON clinical trial in June 2025.

General and Administrative

General and administrative expense was $53.1 million and $16.1 million for the three months ended September 30, 2025 and 2024, respectively, an increase of $37.0 million or 230.4%. The total increase was primarily driven by investments in infrastructure to support launch readiness for apitegromab, including an increase of approximately $13.2 million in employee-related costs including salaries, bonus, benefits and payroll taxes related to increased headcount, an increase of $7.6 million in non-cash equity-based compensation expense related to increased headcount, severance related costs of $1.1 million and an increase of approximately $14.3 million in professional service fees. The increase in headcount is partially associated with the hiring of our commercial and field-facing teams. We expect general and administrative expenses to continue to be substantial as we continue to invest in building the infrastructure to support the commercialization of apitegromab.

Other Income (Expense), Net

The change in other income (expense), net was primarily attributable to an increase in interest income earned due to higher balances in our cash, cash equivalents and marketable securities.

Comparison of the Nine Months Ended September 30, 2025 and 2024

The following table summarizes our results of operations for the nine months ended September 30, 2025 and 2024 (in thousands, except percentages):

	Nine Months Ended September 30,		Change	
	2025	2024	$	%
Operating expenses:				
Research and development	$ 161,565	$ 134,185	$ 27,380	20.4 %
General and administrative	131,184	48,512	82,672	170.4 %
Total operating expenses	292,749	182,697	110,052	60.2 %
Loss from operations	(292,749)	(182,697)	(110,052)	60.2 %
Other income (expense), net	5,775	2,857	2,918	102.1 %
Net loss	$ (286,974)	$ (179,840)	$ (107,134)	59.6 %

Operating Expenses

Research and Development

Research and development expense was $161.6 million and $134.2 million for the nine months ended September 30, 2025 and 2024, respectively, an increase of $27.4 million or 20.4%. The following table summarizes our

research and development expense for the nine months ended September 30, 2025 and 2024 (in thousands, except percentages):

	Nine Months Ended September 30,		Change	
	2025	2024	$	%
External costs by program:				
Apitegromab	$ 68,618	$ 57,852	$ 10,766	18.6 %
SRK-181	2,259	8,069	(5,810)	(72.0)%
SRK-439	8,462	6,321	2,141	33.9 %
Other early programs and unallocated costs	4,811	1,998	2,813	140.8 %
Total external costs	84,150	74,240	9,910	13.3 %
Internal costs:				
Employee compensation and benefits	63,134	46,977	16,157	34.4 %
Facility and other	14,281	12,968	1,313	10.1 %
Total internal costs	77,415	59,945	17,470	29.1 %
Total research and development expense	$ 161,565	$ 134,185	$ 27,380	20.4 %

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

- An increase in our external research and development costs of $9.9 million, which primarily consisted of:
  - $10.8 million increase in costs associated with apitegromab primarily due to an increase in drug supply manufacturing and the initiation of our Phase 2 OPAL trial in SMA patients under two years of age, partially offset by decreases in clinical trial costs as our Phase 2 TOPAZ trial extension period, our Phase 3 SAPPHIRE clinical trial and our proof-of-concept Phase 2 EMBRAZE trial are completed;
  - $5.8 million decrease in costs associated with SRK-181, as our Phase 1 DRAGON trial is completed;
  - $2.1 million increase in preclinical costs and manufacturing development for SRK-439; and
  - $2.8 million increase in other early development candidates and unallocated costs.

- $17.5 million increase in internal research and development costs, which was primarily driven by an increase of $8.7 million in employee related costs, including salaries, bonus, benefits, payroll taxes related to increased headcount, an increase of $3.6 million in severance and other costs associated with our leadership change and an increase of $3.4 million in non-cash equity-based compensation expense related to increased headcount, including one-time charges of $0.4 million related to the modification of certain equity awards.

Total research and development expenses are expected to continue to be substantial, driven by employee compensation costs and development costs associated with manufacture of drug supply, as well as our clinical stage programs as we continue development activities for apitegromab in SMA, the conduct of ONYX, our initiation of our Phase 2 OPAL trial in SMA patients under the age of two, as well as costs associated with supporting our anti-myostatin program, including SRK-439. Additionally, we will continue to invest in our pipeline. We expect costs of our SRK-181 program to decrease, as we completed the Phase 1 DRAGON clinical trial in June 2025.

General and Administrative

General and administrative expense was $131.2 million and $48.5 million for the nine months ended September 30, 2025 and 2024, respectively, an increase of $82.7 million or 170.4%. The total increase was primarily driven by investments in infrastructure to support launch readiness for apitegromab, including an increase of approximately $22.8 million in employee-related costs including salaries, bonus, benefits and payroll taxes related to increased headcount, an increase of $5.8 million in severance and other costs associated with our leadership change, an increase of $13.1 million in non-cash equity-based compensation expense related to increased headcount, an increase in one-time charges in non-cash equity-based compensation expense of $12.6 million related to the modification of certain equity awards and an increase of approximately $27.1 million in professional service fees. The increase in headcount is partially associated with the hiring of our commercial and field-facing teams. We expect general and administrative expense, excluding the $18.4 million in charges associated with the leadership change, to continue to be substantial as we continue to invest in building the infrastructure to support the commercialization of apitegromab.

Other Income (Expense), Net

The change in other income (expense), net was primarily attributable to an increase in interest income earned due to higher balances in our cash, cash equivalents and marketable securities.

Liquidity and Capital Resources

Sources of Liquidity

Since our inception, we have not generated any product revenue and 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 ("ATM") sales, as well as payments from our research collaborations and the Loan and Security Agreement entered into in October 2020 and subsequently amended (see Note 11).

The following table provides information regarding our total cash, cash equivalents and marketable securities at September 30, 2025 and December 31, 2024 (in thousands):

	September 30, 2025	December 31, 2024
Cash and cash equivalents	$ 286,770	$ 177,878
Marketable securities	82,860	259,400
Total cash, cash equivalents and marketable securities	$ 369,630	$ 437,278

During the nine months ended September 30, 2025, our cash, cash equivalents and marketable securities balance decreased by $67.6 million. The change was primarily due to 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 and made interest payments on our debt, partially offset by proceeds from our debt facility, sales under our ATM program and the exercises of stock options.

Our current ATM program with Jefferies, established in November 2022, allows for the sale of shares of our common stock from time to time in "at-the-market" offerings through Jefferies as the Company's sales agent. As of September 30, 2025, the Company has sold 3,386,290 shares of our common stock, generating net proceeds of $96.9 million, under the ATM program. Of this amount, we sold 2,767,000 shares of our common stock under the ATM program during the nine months ended September 30, 2025, generating net proceeds of $91.7 million.

In February 2025, we entered into the Amended and Restated Loan and Security Agreement with Oxford 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 (see Note 11). The Amended and Restated Loan and Security Agreement consolidates the existing outstanding loan tranches solely with Oxford.

For additional information on certain prior liquidity sources see Note 7.

During the nine months ended September 30, 2025, none of the Company's pre-funded warrants were exercised. As of September 30, 2025, the Company had 17,362,147 pre-funded warrants outstanding.

During the nine months ended September 30, 2025, 458,044 of the Company's common warrants were exercised, generating proceeds of $3.4 million. As of September 30, 2025, the Company had 8,220,620 common warrants outstanding with an exercise price of $7.35, which expire on December 31, 2025.

Cash Flows

The following table provides information regarding our cash flows for the nine months ended September 30, 2025 and 2024 (in thousands):

	Nine Months Ended September 30,	
	2025	2024
Net cash used in operating activities	$ (224,539)	$ (151,436)
Net cash provided by investing activities	179,719	119,314
Net cash provided by financing activities	155,112	6,658
Net increase (decrease) in cash, cash equivalents and restricted cash	$ 110,292	$ (25,464)

Net Cash Used in Operating Activities

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 used in operating activities was $151.4 million for the nine months ended September 30, 2024, and consisted of our net loss of $179.8 million and changes in our assets and liabilities of $0.6 million, partially offset by non-cash adjustments of $29.0 million. The non-cash adjustments are primarily from equity-based compensation.

Net Cash Provided by Investing Activities

Net cash provided by investing activities was $179.7 million for the nine months ended September 30, 2025 compared to net cash provided by investing activities of $119.3 million for the nine months ended September 30, 2024. Net cash provided by 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 $155.1 million for the nine months ended September 30, 2025 compared to $6.7 million for the nine months ended September 30, 2024. 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. Net cash provided by financing activities for the nine months ended September 30, 2024 was primarily attributable to proceeds from the exercise of warrants.

Funding Requirements

We expect our expenses to be substantial as we continue the research and development of apitegromab in SMA. In addition, we are seeking marketing approval for apitegromab, and we expect to incur significant commercialization expenses related to product sales, marketing, manufacturing and distribution. 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. We expect to incur costs to support our anti-myostatin program, including the close out activities for our Phase 2 EMBRAZE proof-of-concept trial of apitegromab and our Phase 1 trial for SRK-439. Additionally, we will support the development of our pipeline and any other preclinical 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, marketable securities and cash available to us will enable us to fund our operating expenses and capital expenditure requirements into 2027. 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 product candidates and future product candidates, including costs associated with apitegromab 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 planned Phase 1 trial of SRK-439 in healthy volunteers and the costs and timing of conducting future preclinical studies and clinical trials for SRK-373, SRK-256 or any other product candidates;
- the costs of future manufacturing of apitegromab, SRK-181, SRK-439, SRK-373, SRK-256 and any other future product candidates;
- the scope, progress, results and costs of discovery, preclinical 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 of seeking marketing approvals for apitegromab;
- the costs and timing of future commercialization activities, including product sales, marketing, manufacturing and distribution for apitegromab, if approved;
- the amount of revenue, if any, received from commercial sales of apitegromab, if approved;
- 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 business operations and research and development activities;
- the costs of supporting our infrastructure and facilities, including equipment and physical infrastructure to support our research and development;
- the costs of operating as a 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 preclinical 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. In addition, our product candidates, if approved, 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, 2024.

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, 2024, we were considered a "smaller reporting company," effective as of December 31, 2024. Based on the market value of our common stock held by our non-affiliates as of June 30, 2025, we will no longer be a smaller reporting company. Accordingly, we will cease 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 will thus be subject to additional disclosure and compliance requirements. Because we remain eligible to use the requirements for smaller reporting companies through December 31, 2025, we will continue to be a non-accelerated filer as of December 31, 2025 and will remain a "non-accelerated filer" through December 31, 2026.

Item 3. Quantitative and Qualitative Disclosures About Market Risk

We are a smaller reporting company as defined by Rule 12b-2 of the Exchange Act, and are not required to provide the information required under this item.

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) has evaluated the effectiveness of our disclosure controls and procedures as of September 30, 2025, 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, 2025 that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

Part II. OTHER INFORMATION

Item 1. Legal Proceedings

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

- The regulatory approval process for our product candidates in the U.S., EU 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. The Complete Response Letter ("CRL") received from the FDA in September 2025 will require us to resubmit the apitegromab biologics license application ("BLA") and our progress toward potential commercialization of apitegromab has been delayed.
- We have never commercialized a product and are in the process of building and scaling our business for potential commercialization of apitegromab in the United States and Europe, including building our compliance, medical affairs and commercial organizations, which, if we are not able to do so successfully could negatively impact our business, including 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, including the timely review and potential approval of our biologics license application ("BLA") when resubmitted, and any such disruptions and changes could negatively impact our business.
- 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 apitegromab, 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 candidates.
- The results of preclinical studies and early-stage clinical trials may not be predictive of future results. Success of a product candidate in an early-stage clinical trial may not be replicated in later-stage clinical trials.
- 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 our clinical trials and to conduct certain aspects of our preclinical studies. 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 or commercialize apitegromab, SRK-181, SRK-439 or any future product candidates, and our business could be materially harmed.
- Preclinical development is uncertain. Our preclinical 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 research and development, preclinical and clinical development materials, and, if approved, commercial materials, 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 our drug 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 apitegromab, SRK-181, SRK-439 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 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 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 apitegromab, 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

The regulatory approval process for our product candidates in the U.S., EU 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. The Complete Response Letter ("CRL") received from the FDA in September 2025 will require us to resubmit the apitegromab biologics license application ("BLA") and our progress toward potential commercialization of apitegromab has been delayed.

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. We are not permitted to market any biological product in the U.S. until we receive a biologics license from the FDA. Prior to filing 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 have not submitted a BLA to the FDA or MAA to the EMA or similar marketing application to comparable foreign authorities. A BLA must include extensive preclinical and clinical data and supporting information to establish that the product candidate is safe, pure and potent for each desired indication. FDA approval of a new biologic or drug generally requires dispositive data from two (and in some cases, one) adequate and well-controlled pivotal Phase 3 clinical trials of the biologic or drug in the relevant patient population. The FDA, EMA or comparable foreign regulatory authorities may disagree with the design or implementation of our clinical trials or our analysis or interpretation of data from preclinical 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. A BLA 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 FDA, 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.

In September 2025, we received a CRL from the FDA related to observations identified during a routine general site inspection of a third-party fill-finish facility. This facility was issued a Form 483 by the FDA in July 2025 and the inspection classification of this facility is "official action indicated" (OAI). The observations were site-related and not specific to apitegromab. The CRL did not cite any other approvability concerns, including apitegromab's efficacy and safety data or the third-party drug substance manufacturer. In November 2025, we completed a constructive in-person Type A meeting with the FDA that included participation of representatives from the third-party fill-finish facility. We plan to resubmit the apitegromab BLA at such time as the facility has been reclassified by the FDA, however, there can be no guarantee the FDA will approve apitegromab upon our resubmission of the BLA. The CRL has delayed our progress toward potential commercialization of apitegromab and we may be subject to additional risks such as a loss of our competitive advantage, increased litigation and other regulatory issues.

The FDA may seek independent advice from a panel of experts, referred to as an Advisory Committee, on complex or novel issues that may be presented in an application, including issues related to the adequacy of the safety and efficacy data to support approval. The opinion of the Advisory Committee, although not binding, may have a significant impact on our ability to receive approval of any product candidates that we develop based on the completed clinical trials.

Further, a clinical trial may be suspended or terminated by us, the IRBs for the institutions at which such trials are being conducted, or the FDA, the competent authorities and/or ethics committees of the EU Member States or other regulatory authorities, or recommended for suspension or termination by the DSMB for such trial, due to a number of factors, including failure to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols, inspection of the clinical trial operations or trial site by the FDA, EMA, competent authorities of the EU Member States or other regulatory authorities resulting in the imposition of a clinical hold, unforeseen safety issues or adverse side effects, failure to demonstrate a benefit from using a product candidate, changes in governmental regulations or administrative actions or lack of adequate funding to continue the clinical trial. If we experience termination of, or delays in the completion of, any clinical trial of our product candidates, the prospects for regulatory approval and commercial prospects for our product candidates will be harmed, and our ability to generate product revenue will be

delayed. In addition, any delays in completing any clinical trials will increase our costs, slow down our product development and approval process and jeopardize our ability to commence product sales and generate revenue.

We have never commercialized a product and are in the process of building and scaling our business for potential commercialization of apitegromab in the United States and Europe, including building our compliance, medical affairs and commercial organizations, which, if we are not able to do so successfully could negatively impact our business, including the potential for a successful commercialization of apitegromab.

Although we are preparing our commercialization capabilities in anticipation of a potential approval and commercial launch of apitegromab, we have no prior sales or distribution experience and limited capabilities for marketing and market access. We expect to invest significant financial and management resources over time to establish compliance, medical affairs and commercial organizations for the marketing, sales and distribution of apitegromab in the United States as well as Europe, if approved in each jurisdiction, and other capabilities and infrastructure to support commercial operations. 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 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, including the timely review and potential approval of our BLA, when resubmitted, 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 as we navigate the resubmission of our BLA to the FDA and make other preparations for potential commercialization. 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.

We have received priority review for the BLA for apitegromab for the treatment of SMA, which we requested at the time of submission of our BLA to the FDA. Even though received, priority review designation may not result in a shorter timeline to approval, and such designation may be rescinded if a product no longer meets the qualifying criteria.

We requested priority review at the time of submitting our marketing application for apitegromab for the treatment of SMA and received priority review designation, which shortens the FDA's goal review time to six months from the date of filing acceptance. The FDA may designate a product for priority review if it is a product that treats a serious condition and, if approved, would provide a significant improvement in safety or effectiveness when compared with other available therapies. Significant improvement may be illustrated by evidence of increased effectiveness in the treatment of a condition, elimination or substantial reduction of a treatment-limiting adverse reaction, documented enhancement of patient compliance that may lead to improvement in serious outcomes, or evidence of safety and effectiveness in a new subpopulation. A priority review designation is intended to direct overall attention and resources to the evaluation of such applications, and to shorten the FDA's PDUFA goal date for taking action on a marketing application from ten months to six months from FDA's acceptance of the application for review. However, priority review does not guarantee a faster review or approval process or assure ultimate approval by the FDA. For example, in September 2025, we received a CRL from the FDA related to observations identified during a routine general site inspection of a third-party fill-finish facility. This facility was issued a Form 483 by the FDA in July 2025 and the inspection classification of this facility is "official action indicated" (OAI). The observations were site-related and not specific to apitegromab. The CRL did not cite any other approvability concerns, including apitegromab's efficacy and safety data or the third-party drug substance manufacturer. In addition, priority review designation may be rescinded if the FDA determines that a product no longer meets the qualifying criteria.

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 apitegromab, 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 candidates.

Before obtaining regulatory approvals for the commercial sale of any product candidates, we must demonstrate through extensive preclinical studies and clinical trials that our 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 marketing approval or commercialize apitegromab, 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;
- failure by our collaborators to provide us with an adequate and timely supply of product that complies with the applicable quality and regulatory requirements for a combination trial;
- collaborators may provide insufficient funding for a clinical trial program, delay or stop a clinical trial, abandon a product candidate or clinical trial program, repeat or conduct new clinical trials or require a new formulation of a drug candidate for clinical testing;
- clinical trials of any 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 preclinical studies or clinical trials or we may decide to abandon product development programs;
- the number of subjects required for clinical trials of any 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 or ethics committees may require that we or our investigators, suspend or terminate clinical research or trials for various reasons, including noncompliance with regulatory requirements or a finding that the participants are being exposed to unacceptable health risks;
- 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 candidates may have undesirable side effects or other unexpected characteristics, causing us or our investigators, regulators, IRBs or ethics committees to suspend or terminate the trials, or reports from clinical testing of other therapies may raise safety or efficacy concerns about our product candidates;
- our 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 preclinical 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;

- 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 preclinical studies or clinical trials; and
- lack of adequate funding to complete a clinical trial.

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 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. We, the FDA, the competent authorities and/or ethics committees of the EU Member States or other applicable regulatory authorities for their jurisdictions, or an IRB for their site(s) may suspend clinical trials of a product candidate at any time for various reasons, including a belief that subjects or patients in such trials are being exposed to unacceptable health risks or adverse side effects.

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 candidates and may allow our competitors to bring products to market before we do, potentially impairing our ability to successfully commercialize our product candidates and harming our business and results of operations. Any delays in our clinical development programs may harm our business, financial condition and results of operations significantly.

Our clinical development strategy 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, are receiving apitegromab 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 long-term extension trials, or the medical condition of patients may be affected which could negatively affect the efficacy and safety results for apitegromab 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 and delay our clinical development programs and harm our business, financial condition and results of operations.

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

The results or success of preclinical studies and early-stage clinical trials of our product candidates may not be predictive of future results or replicated in later preclinical studies or later-stage clinical trials. Preclinical 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 preclinical studies and early-stage clinical trials. Our Phase 3 SAPPHIRE clinical trial evaluated the safety and efficacy of apitegromab for the treatment of patients with Type 2 and Type 3 SMA and in October 2024, we announced positive top-line data from this clinical trial. We announced top-line results of our Phase 2 EMBRAZE proof-of-concept trial of apitegromab in combination with tirzepatide in obesity in June 2025. We cannot assure you that any future clinical trials of apitegromab, such as our Phase 2 OPAL clinical trial in patients with SMA less than 2 years of age, SRK-439, or any other anti-myostatin antibodies will show positive results. 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. There can be no assurance that any of our current or planned clinical trials will ultimately be successful or support further clinical development of any of our product candidates. There can also be no assurance that any of our future clinical trials will show similar results to our earlier clinical trials or support further development or registration of any of our product candidates.

Interim, initial, or 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.

From time to time, we may publish interim, initial, or preliminary data, including interim top-line results or initial or preliminary results from our clinical trials. Any interim, initial or preliminary data and other results 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 interim, initial or preliminary 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. For example, clinical data from our Phase 1 DRAGON trial in cancer immunotherapy, including preliminary safety, efficacy and biomarker data were presented in June 2024 at the ASCO 2024 annual meeting and in November 2024 at the SITC 39th Annual Meeting. Tumor response data from DRAGON was based on assessments by site investigators. Central reads for the tumor responses were also conducted in DRAGON, and a comprehensive review of the central reads was performed once completed within and/or across the cohorts. 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, preclinical and clinical data are often susceptible to varying interpretations and analyses, and many companies that believed their product candidates performed satisfactorily in preclinical studies and clinical trials nonetheless failed to obtain regulatory approval. If we fail to produce positive results in our ongoing and planned preclinical studies and clinical trials with apitegromab, SRK-181, 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 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 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 initially developing apitegromab for the treatment of SMA, a rare disease, affecting an estimated 20,000 patients in the U.S. and Europe. As a result, we may encounter difficulties enrolling patients in our clinical trials for apitegromab in SMA 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 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 candidates.

We rely on third parties to conduct our clinical trials and certain aspects of our preclinical studies. 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 or commercialize apitegromab, SRK-181, SRK-439, or any future product candidates, and our business could be materially harmed.

We depend upon third parties to conduct certain aspects of our preclinical 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 preclinical studies or clinical trials before approving our marketing applications. We cannot be certain that, upon inspection, such regulatory authorities will determine that any of our clinical trials comply with 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.

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 preclinical 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 preclinical studies and clinical trials. The third party CROs and clinical trial sites that conduct our clinical trials have experienced 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 preclinical or clinical data they obtain is compromised due to the failure to adhere to our protocols or regulatory requirements or for other reasons, our development timelines, including clinical development timelines, may be extended, delayed or terminated and we may not be able to complete development of, receive regulatory approval of or successfully commercialize our product candidates. As a result, our financial results and the commercial prospects for our product candidates would be harmed, our costs could increase and our ability to generate revenue could be delayed.

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 have received Orphan Drug designation from the FDA for apitegromab for the treatment of SMA 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 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, 2024 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 Rare Pediatric Disease designation for apitegromab for the treatment of SMA. However, a marketing application for apitegromab, if approved, may not meet the eligibility criteria for a rare pediatric disease priority review voucher.

We have received Rare Pediatric Disease designation for apitegromab for the treatment of SMA. Designation of a biologic as a product for a rare pediatric disease does not guarantee that a BLA for such biologic will meet the eligibility criteria for a rare pediatric disease priority review voucher at the time the application is approved. Pursuant to the Federal Food, Drug, and Cosmetic Act, in January 2025 we requested a rare pediatric disease priority review voucher in our BLA submission for apitegromab for the treatment of SMA. The FDA may determine that the BLA for apitegromab, if approved, does not meet the eligibility criteria for a rare pediatric disease priority review voucher, including for the following reasons:

- SMA no longer meets the definition of a rare pediatric disease;
- apitegromab contains an active ingredient (including any ester or salt of the active ingredient) that has been previously approved in an application;
- the BLA is not deemed eligible for priority review;
- the BLA does not rely on clinical data derived from studies examining a pediatric population and dosages of the drug intended for that population; or
- the BLA seeks approval for a different adult indication than the rare pediatric disease for which apitegromab is designated.

The FDA's authority to grant rare pediatric disease designations expired on December 20, 2024. Under the amended statutory sunset provisions, the FDA may award a priority review voucher for an approved rare pediatric disease product application only if the sponsor has rare pediatric disease designation for the drug and if that designation was granted by December 20, 2024. After September 30, 2026, the FDA may not award any rare pediatric disease priority review vouchers. If the BLA for apitegromab is not approved on or prior to September 30, 2026 for any reason, it will not be eligible for a priority review voucher. However, it is possible the authority for the FDA to award rare pediatric disease priority review vouchers will be further extended by Congress.

We have received Fast Track designation from the FDA 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 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 candidates.

In May 2021, the FDA granted Fast Track designation for apitegromab for the treatment of SMA. 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 SMA, 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, 2024 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, 2024 entitled, “Business – Government Regulation – European Union Drug Development — European Union Expedited Review and Development.”

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

Receiving and maintaining regulatory approval of our product candidates 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 if the FDA grants marketing approval of a product candidate, 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 the product candidate 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 preclinical 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., a product candidate 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 product candidates 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 our products in certain

countries. If we fail to comply with the regulatory requirements in international markets and/or receive applicable marketing approvals, our target market will be reduced and our ability to realize the full market potential of our product candidates will be harmed.

Even if we receive regulatory approval of any product candidates, 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 product candidates.

If any of our product candidates are approved, they 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 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 the safety and efficacy of the product candidate. The FDA may also require a REMS program as a condition of approval of our product candidates, which could entail requirements for long-term patient follow-up, a medication guide, physician communication plans or additional elements to ensure safe use, such as restricted distribution methods, patient registries and other risk minimization tools.

Later discovery of previously unknown problems with our product candidates, including adverse events of unanticipated severity or frequency, or with our third-party manufacturers or manufacturing processes, or failure to comply with regulatory requirements may result in revisions to the approved labeling to add new safety information; imposition of post-market studies or clinical trials to assess new safety risks; or imposition of distribution restrictions or other restrictions under a REMS program. Other potential consequences include, among other things:

- restrictions on the marketing or manufacturing of our products, withdrawal of the 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 candidates; 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.

Even if apitegromab receives marketing approval, it may fail to achieve the degree of market acceptance by physicians, patients, third-party payors and others in the medical community necessary for commercial success.

If apitegromab receives marketing approval it 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 apitegromab, 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 apitegromab is approved but 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 apitegromab in conjunction with such SMN-targeted treatment. The degree of market acceptance of apitegromab, if approved for commercial sale, will depend on a number of factors, including:

- the efficacy and safety of apitegromab as demonstrated in clinical trials;
- the indications for which apitegromab is 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 apitegromab, if approved, for sale at competitive prices;
- the timing of market introduction of apitegromab 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.

If we are unable to establish sales and marketing capabilities or enter into agreements with third parties to sell and market apitegromab, 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 and have limited experience in the sale, marketing or distribution of pharmaceutical products. To achieve commercial success for any approved product 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.

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 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 any future products; 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 any products that we develop ourselves. In addition, we may not be successful in entering into arrangements with third parties to sell and market our product candidates 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 our products 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 our product candidates.

Competing therapies may exist or could emerge that adversely affect the amount of revenue we are able to generate from the sale of apitegromab, if approved, 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 candidates or address similar markets. If we are successful in developing apitegromab, it is probable that the number of companies seeking to develop products and therapies similar to our products 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 apitegromab, if approved, 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 apitegromab. 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.

Preclinical development is uncertain. Our preclinical 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 preclinical 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 preclinical 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 preclinical 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 preclinical 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 preclinical 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 preclinical testing and studies may cause us to incur additional operating expenses. We also may be affected by delays associated with the preclinical 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 preclinical 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 preclinical study program and/or appropriate preclinical 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 research and development, preclinical and clinical development materials, and, if approved, commercial materials, may become limited or interrupted or may not be of satisfactory quantity or quality.

We have no experience manufacturing our 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, if approved, all of our commercial product supplies, including all of our drug substance, fill-finish, labeling, and packaging. We do not own our own manufacturing facilities for producing any clinical trial or commercial product supplies. There can be no assurance that our preclinical, 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 each the drug substance and fill-finish manufacture of apitegromab, SRK-181, and SRK-439. 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, 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 related to observations identified during a routine general site inspection of a third-party fill-finish facility. This facility was issued a Form 483 by the FDA in July 2025 and the inspection classification of this facility is OAI. The observations were site-related and not specific to apitegromab. In November 2025, we completed a constructive in-person Type A meeting with the FDA that included participation of representatives from the third-party fill-finish facility. We plan to resubmit the apitegromab BLA at such time as the facility has been reclassified by the FDA, however, there can be no guarantee the FDA will approve apitegromab upon our resubmission of the BLA. The CRL has delayed our progress toward potential commercialization of apitegromab and we may be subject to additional risks such as a loss of our competitive advantage, increased litigation and other regulatory issues. The manufacturing process for a 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 manufacture the materials ourselves, 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 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 candidates. If we must change manufacturers for any reason, we will be required to verify that the new manufacturer maintains facilities and procedures that comply with quality standards and with all applicable regulations and guidelines. We will also need to verify, such as through a manufacturing

comparability study, that any new manufacturing process will produce our product candidate according to the specifications previously submitted to the FDA or another regulatory authority. The delays associated with the verification of a new manufacturer could negatively affect our ability to develop and commercialize 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, fill-finish, and packaged and labeled product for apitegromab, if we receive regulatory approval. We do not have long-term supply agreements in place with many of our contract manufacturers, and each batch for our product candidates is individually contracted through a purchase order governed by master service and quality agreements. If our existing fill-finish contract manufacturers for our product candidates are not willing to enter into long-term supply agreements, or are not willing or are unable to supply product candidate supplies to us, we could be required to engage new contract manufacturers who would need to scale up the manufacturing process before we would be able to use the product candidate supplies they manufacture, which could result in delays to our clinical trials or future commercialization plans, if we are successful and gain approval.

To the extent that we have existing, or in the future enter into, manufacturing arrangements with third parties, we will depend on these third parties to perform their obligations in a timely manner consistent with contractual and regulatory requirements, including those related to quality control and assurance. If we are unable to obtain or maintain third-party manufacturing for product candidates, or to do so on commercially reasonable terms, we may not be able to develop and commercialize our product candidates successfully. Our or a third-party's failure to execute on our manufacturing requirements and comply with cGMP could adversely affect our business in a number of ways, including:

- an inability to initiate or continue clinical trials for apitegromab, SRK-181, SRK-439 or of future product candidates under development;
- delay in submitting regulatory applications, or receiving regulatory approvals, for apitegromab, SRK-181, SRK-439 or future product candidates;
- loss of the cooperation of an existing or future collaborator;
- subjecting third-party manufacturing facilities or our manufacturing facilities to additional inspections by regulatory authorities;
- requirements to cease distribution or to recall batches of apitegromab, SRK-181, SRK-439 or future product candidates; and
- in the event of approval to market and commercialize apitegromab, SRK-181, SRK-439 or a future product candidate, an inability to meet commercial demands for our products.

Failure to maintain compliance with cGMP can result in a contractor receiving FDA sanctions, which can impact our ability to operate or lead to delays in any clinical development programs or commercial supply. In addition, any delay in contracting for fill and finish services, or failure of the contract manufacturer to perform the services as needed, may delay any clinical trials, registration and 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.

In order to conduct later-stage clinical trials, or, if approved, produce commercial product, we will need to manufacture such product candidate in large quantities. In particular, we expect to rely on our contract manufacturers to scale our manufacturing processes for future clinical trials of apitegromab, and if our development efforts are successful and if apitegromab is approved, for commercial supply of apitegromab. We, or any manufacturing partners, may be unable to successfully increase the manufacturing capacity for apitegromab in a timely or cost-effective manner to meet our supply requirements. If we successfully commercialize any of our product candidates, we may be required to establish large-scale commercial manufacturing capabilities. As our drug development pipeline increases and matures, we will have a

greater need for clinical study and commercial manufacturing capacity. In addition, quality-control issues may arise during scale-up activities. If we, or any manufacturing partners, are unable to successfully scale-up the manufacture of our product candidates in sufficient quality and quantity, the development, testing, clinical trials, and if approved, commercial supply, of that product candidate may be delayed or infeasible, and regulatory approval, commercial launch or commercial supply of any resulting product may be delayed or not received, which could significantly harm our business. In September 2025, we received a CRL from the FDA for the apitegromab BLA related to observations identified during a routine general site inspection of a third-party fill-finish facility. This facility was issued a Form 483 by the FDA in July 2025 and the inspection classification of this facility is OAI. The observations were site-related and not specific to apitegromab. We plan to resubmit the apitegromab BLA at such time as the facility has been reclassified by the FDA.

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 apitegromab. If apitegromab is approved, we expect 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, quality-control issues, non-compliance with regulatory requirements, 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 apitegromab 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 apitegromab, SRK-181, 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 United States, 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 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 apitegromab approaches commercialization, we will also need to hire sales, marketing and other commercial personnel. Future growth would impose significant added responsibilities on members of management, including:

- identifying, recruiting, integrating, maintaining and motivating additional employees;
- managing our development efforts effectively, including the clinical and regulatory review process for apitegromab, SRK-181, 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 apitegromab, if approved, 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 preclinical 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 apitegromab, 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 apitegromab 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 potentially commercialize apitegromab, SRK-181 and SRK-439 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 preclinical 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 apitegromab, SRK-181 and SRK-439 and to conduct preclinical 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, 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). 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 and other similar foreign regulatory bodies; provide true, complete and accurate information to the FDA, EMA 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; or report financial information or data accurately or to disclose unauthorized activities to us. If we receive FDA approval, EC approval or approval from other foreign

regulatory bodies of apitegromab and begin commercializing that product in the U.S. or 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.

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, 2024 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 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 efforts 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 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.

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 April 15, 2025, the Trump Administration published Executive Order 14273, “Lowering Drug Prices by Once Again Putting Americans First,” which generally directs the federal government to take measures to reduce drug prices, including eliminating the so-called “pill penalty” under the Inflation Reduction Act that creates a distinction between small molecule and large molecule products for purposes of determining when a drug may be eligible for drug price negotiation. On May 12, 2025, the Trump Administration published Executive Order 14297, “Delivering Most-Favored-Nation Prescription Drug Pricing to American Patients” which generally, among other things, directs the federal government to establish and communicate most-favored-nation price targets to pharmaceutical manufacturers to bring prices for American patients in line with comparably developed nations. Further, the Executive Order directs the federal government to support regulatory paths to allow direct-to-patient sales for companies that meet these targets. It also states that the Administration will take additional aggressive action (for example, examining whether marketing approvals should be modified or rescinded or opening the door for individual drug importation waivers) should manufacturers fail to offer American consumers the

most-favored-nation lowest price. It also directs the Secretary of Commerce and the U.S. Trade Representative to "take all necessary and appropriate action to ensure foreign countries are not engaged in any act, policy, or practice that may be unreasonable or discriminatory or that may impair United States national security . . . including by suppressing the price of pharmaceutical products below fair market value in foreign countries." Notably, a similar "Most Favored Nation" pricing rule enacted under the first Trump Administration was subject to an injunction resulting from judicial challenges to the rule, which was formally rescinded by the former Biden Administration in August 2021.

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 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, 2024 entitled "Business – Government Regulation – Other Healthcare Laws."

It is possible that governmental and enforcement authorities will conclude that our business practices 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.

Failure to comply with health care privacy and data protection laws and regulations could lead to government enforcement actions (which could include civil or criminal penalties), private litigation, and/or adverse publicity and could negatively affect our operating 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 our Phase 2 TOPAZ clinical trial and Phase 3 SAPPHIRE clinical trial of apitegromab in the European Economic Area ("EEA") and the UK, and are currently conducting ONYX, our long-term extension clinical trial of apitegromab, in the EEA and the UK, and may conduct future clinical trials in the EEA or the UK and therefore may be subject to additional privacy laws. The EU General Data Protection Regulation (the "EU GDPR") imposes a broad range of strict requirements on companies subject to the EU GDPR, including requirements relating to having legal bases and conditions for processing personal information relating to 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, 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 EU GDPR imposes penalties in the event of non-compliance, including fines of up to 10,000,000 Euros or up to 2% of our total worldwide annual turnover for certain comparatively minor offenses, or up to 20,000,000 Euros or up to 4% of our total worldwide annual turnover for more serious offenses. The EU 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 EU GDPR.

The EU GDPR ceased to apply in the UK after the UK's exit from the EU on January 31, 2020, but the UK incorporated the EU GDPR (as it existed on December 31, 2020 but subject to certain UK specific amendments) into UK law ("the UK GDPR"). The UK GDPR and the UK Data Protection Act 2018 set out the UK's data protection regime, which is independent from but currently still aligned to the EU's data protection regime. Non-compliance with the UK GDPR may result in monetary penalties of up to £17.5 million or 4% of worldwide revenue, whichever is higher. Although the UK is regarded as a third country under the EU's GDPR, the UK is recognized as providing adequate protection under the EU GDPR ("UK Adequacy Decision") and, therefore, transfers of personal data originating in the EU to the UK remain unrestricted. Likewise, personal data transfers from the UK to the EEA remain free flowing. The UK Government introduced a Data Protection and Digital Information Bill which failed in the UK legislative process. A new Data (Use and Access) Bill ("UK Bill") has been introduced into parliament. If passed, the final version of the UK Bill may have the effect of further altering the similarities between the UK and EEA data protection regime and threaten the UK Adequacy Decision from the European Commission, or EC. Further, this may lead to additional compliance costs and could increase our overall risk. The respective provisions and enforcement of the EU GDPR and UK GDPR may further diverge in the future and create additional regulatory challenges and uncertainties.

Adequate safeguards must be implemented to enable the transfer of personal data outside of the EEA or the UK in compliance with European and UK data protection laws. The EC has issued forms of standard contractual clauses ("SCCs") for data transfers from controllers or processors in the EEA (or otherwise subject to the EU GDPR) to controllers or processors established outside the EEA (and not subject to the EU GDPR). The UK is not subject to the EC's SCCs but has published its own standard clauses, the International Data Transfer Agreement, which enables transfers from the UK. We will be required to implement these new safeguards when conducting restricted data transfers under the EU GDPR and UK GDPR and doing so will require significant effort and cost. 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.

In July 2023, the EC adopted its adequacy decision for the EU-U.S. Data Privacy Framework ("Framework"). On the basis of the new adequacy decision, personal data can flow safely from the EU to U.S. companies participating in the Framework, without having to put in place additional data protection safeguards. There has been an extension to the Framework to cover UK transfers to the United States. The long-term validity of the Framework remains uncertain as the Framework could be challenged like its predecessor frameworks. This complexity and the additional contractual burden increases our overall risk exposure. There may be further divergence in the future, including with regard to administrative burdens.

The EU GDPR and UK GDPR may increase our responsibility and liability in relation to personal data that we process where such processing is subject to the EU GDPR and UK GDPR, and we may be required to put in place additional mechanisms to ensure compliance with the EU GDPR and UK GDPR, including as implemented by individual countries. Given the new law, we face uncertainty as to the exact interpretation of the new 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 EU GDPR and UK GDPR will be 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.

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.

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, California's California Consumer Privacy Act ("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. We continue to monitor the impact that the state consumer privacy and protection laws, like the CCPA, may have on our business activities. See the section in our Annual Report on Form 10-K for the year ended December 31, 2024 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. 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 United States 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 candidates.

We face an inherent risk of product liability as a result of testing apitegromab, SRK-181, SRK-439 and any of our future product candidates in clinical trials and will face an even greater risk if we commercialize any products, if approved. For example, we may be sued if our 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, 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 candidates. Even successful defense would require significant financial and management resources. Regardless of the merits or eventual outcome, liability claims may result in:

- inability to bring a product candidate to the market;
- decreased demand for our products;
- injury to our reputation;

- 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;
- loss of revenue;
- exhaustion of any available insurance and our capital resources;
- the inability to commercialize any product candidate, if approved; and
- decline in our share price.

Our inability to obtain 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 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. For example, we are conducting ONYX, our long-term extension clinical trial of apitegromab in the EEA, and economic disruptions in Europe and the military conflict between Russia and Ukraine could adversely affect the conduct of our clinical trial. 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 preclinical 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 our product candidates, if approved, which could make it difficult for us to sell apitegromab profitably.

The success of apitegromab, if approved, depends on the availability of coverage and adequate reimbursement from third-party payors. We cannot be sure that coverage and reimbursement will be available for, or accurately estimate the potential revenue from, apitegromab. See the sections in our Annual Report on Form 10-K for the year ended December 31, 2024 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. Even if we obtain coverage for apitegromab, the resulting reimbursement payment rates might not be adequate for us to achieve or sustain profitability or may require co-payments that patients find unacceptably high. Additionally, third-party payors may not cover, or provide adequate reimbursement for, long-term follow-up evaluations required following the use of apitegromab. Patients are unlikely to use apitegromab unless coverage is provided and reimbursement is adequate to cover a significant portion of the cost of apitegromab. There is significant uncertainty related to insurance coverage and reimbursement of newly approved products. It is difficult to predict at this time what third-party payors will decide with respect to the coverage and reimbursement for apitegromab.

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 apitegromab. 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 apitegromab 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 European Member States.

We intend to seek approval to market apitegromab in both the U.S. and 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. 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.

In addition, in most foreign countries, including several EU Member States, 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, the EU provides options for its Member States to restrict the range of medicinal

products for which their national health insurance systems provide reimbursement and to control the prices of medicinal products for human use. 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. 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 some countries, we may be required to conduct a clinical study or other studies that compare the cost-effectiveness of any of our product candidates to other available therapies in order to obtain or maintain reimbursement or pricing approval. 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. 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. 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.

We may seek to enter into collaborations in the future with third parties, including for apitegromab, 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 preclinical 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 preclinical or clinical development or commercialization efforts;
- collaborators may not pursue development and commercialization of any product candidates that achieve regulatory approval or may elect not to continue or renew development or commercialization programs or license arrangements based on clinical trial results, changes in the collaborators' strategic focus or available funding, or external factors, such as a strategic transaction that may divert resources or create competing priorities;
- collaborators may delay clinical trials, provide insufficient funding for a clinical trial program, stop a clinical trial or abandon a product candidate, repeat or conduct new clinical trials or require a new formulation of a product candidate for clinical testing;
- collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our products and product candidates if the collaborators believe that the competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours;
- product candidates discovered in collaboration with us may be viewed by our collaborators as competitive with their own product candidates or products, which may cause collaborators to cease to devote resources to the commercialization of our product candidates;
- collaborators may fail to comply with applicable regulatory requirements regarding the development, manufacture, distribution or marketing of a product candidate or product;

- collaborators with marketing and distribution rights to one or more of our product candidates that achieve regulatory approval may not commit sufficient resources to the marketing and distribution of such product or products;
- disagreements with collaborators, including disagreements over proprietary rights, contract interpretation or the preferred course of development, might cause delays or terminations of the research, development or commercialization of product candidates, might lead to additional responsibilities for us with respect to product candidates, or might result in litigation or arbitration, any of which would be time-consuming and expensive;
- collaborators may not properly maintain or defend our intellectual property rights or may use our proprietary information in such a way as to invite litigation that could jeopardize or invalidate our intellectual property or proprietary information or expose us to potential litigation;
- collaborators may infringe the intellectual property rights of third parties, which may expose us to litigation and potential liability;
- if a collaborator of ours is involved in a business combination, the collaborator might 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 candidates.

If our future collaborations do not result in the successful discovery, development and commercialization of product candidates or if one of our collaborators terminates its agreement with us, we may not receive any future research funding or milestone or royalty payments under such collaboration. All of the risks relating to product development, regulatory approval and commercialization described in our Annual Report on Form 10-K for the year ended December 31, 2024 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 candidates, and the negotiation process is time-consuming and complex. In order for us to successfully partner our product candidates, potential partners must view these 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 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 candidates could delay the development and commercialization of our 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 candidates, their respective components, formulations, combination therapies, methods used to manufacture them and methods of treatment, as well as successfully defending these patents against third-party challenges. Our ability to stop unauthorized third parties from making, using, selling, offering to sell or importing our product candidates is dependent upon the extent to which we have rights under valid and enforceable patents that cover these activities. If we are unable to secure and maintain patent protection for any product or technology we develop, or if the scope of the patent protection secured is not sufficiently broad, our competitors could develop and commercialize products and technology similar or identical to ours, and our ability to commercialize any product candidates we may develop may be adversely affected.

The patenting process is expensive and time-consuming, and we may not be able to file 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 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 candidates is threatened, it could dissuade companies from collaborating with us to develop, and threaten our ability to commercialize, our product candidates. Further, if we encounter delays in our clinical trials, the period of time during which we could market our product candidates under patent protection would be reduced.

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 candidates, but our competitors may achieve issued claims, including in patents we consider to be unrelated, which block our efforts or may potentially result in our product candidates or our activities infringing such claims. The possibility exists that others will develop products which have the same effect as our products 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 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 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;
- 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 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 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 product candidates or diagnostic tests 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 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 candidates that if issued as patents are expected to expire from 2034 through 2046, 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 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 preclinical 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 candidates and use our proprietary technologies without infringing the proprietary rights of third parties. There is a substantial amount of litigation involving patents and other intellectual property rights in the biotechnology and pharmaceutical industries, as well as administrative proceedings for challenging patents, including interference, derivation, inter partes review, post-grant review, and reexamination proceedings before the USPTO or oppositions and other comparable proceedings in foreign jurisdictions. We may be exposed to, or threatened with, future litigation by third parties having patent or other intellectual property rights alleging that our product candidates and/or proprietary technologies infringe their intellectual property rights. Numerous U.S. and foreign issued patents and pending patent applications, which are owned by third parties, exist in the fields in which we are developing our product candidates. As the biotechnology and pharmaceutical industries expand and more patents are issued, the risk increases that our product candidates may give rise to claims of infringement of the patent rights of others. Moreover, it is not always clear to industry participants, including us, which patents cover various types of drugs, products or their methods of use or manufacture. Thus, because

of the large number of patents issued and patent applications filed in our fields, there may be a risk that third parties may allege they have patent rights encompassing our product candidates, technologies or methods.

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

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

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

Third parties may assert that we are employing their proprietary technology without authorization. Generally, conducting clinical trials and other development activities in the U.S. is protected under the Safe Harbor exemption as set forth in 35 U.S.C. § 271. If and when apitegromab, SRK-181, or another one of our product candidates is approved by the FDA, that certain third-party may then seek to enforce its patent by filing a patent infringement lawsuit against us. While we are not aware of any claims of such a patent that could otherwise materially adversely affect commercialization of our 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 candidates. Because patent applications can take many years to issue, there may be currently pending patent applications which may later result in issued patents that our product candidates may infringe. In addition, third parties may obtain patents in the future and claim that use of our technologies infringes upon these patents. If any third-party patents were held by a court of competent jurisdiction to cover the manufacturing process of our product candidates, constructs or molecules used in or formed during the manufacturing process, or any final product itself, the holders of any such patents may be able to block our ability to commercialize the product candidate unless we obtained a license under the applicable patents, or until such patents expire or they are finally determined to be held invalid or unenforceable. Similarly, if any third-party patent were held by a court of competent jurisdiction to cover aspects of our formulations, processes for manufacture or methods of use, the holders of any such patent may be able to block our ability to develop and commercialize the product candidate unless we obtained a license or until such patent expires or is finally determined to be held invalid or unenforceable. In either case, such a license may not be available on commercially reasonable terms, or at all. If we are unable to obtain a necessary license to a third-party patent on commercially reasonable terms, or at all, our ability to commercialize our product candidates may be impaired or delayed, which could in turn significantly harm our business. Even if we obtain a license, it may be non-exclusive, thereby giving our competitors access to the same technologies licensed to us. In addition, if the breadth or strength of protection provided by our patents and patent applications is threatened, it could dissuade companies from collaborating with us to license, develop or commercialize current or future product candidates.

Parties making claims against us may seek and obtain injunctive or other equitable relief, which could effectively block our ability to further develop and commercialize our product candidates. Defense of these claims, regardless of their merit, would involve substantial litigation expense and would be a substantial diversion of employee resources from our business. In the event of a successful claim of infringement against us, we may have to pay substantial damages, including treble damages and attorneys' fees for willful infringement, obtain one or more licenses from third parties, 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 candidates. We may fail to obtain any of these licenses at a reasonable cost or on reasonable terms, if at all. In that event, we would be unable to further develop and commercialize our product candidates, which could harm our business significantly.

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 candidates or proprietary technologies.

Additionally, the Unitary Patent/Unified Patent Court 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.

- The opt-out selection afforded during the transition may have a direct impact on future litigation and may result in loss of certain flexibility with regard to choice of forum and other litigation strategy considerations.

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 product candidates could be found invalid or unenforceable if challenged in court or the USPTO.

If we or one of our licensing partners initiate legal proceedings against a third-party to enforce a patent covering one of our product candidates, the defendant could counterclaim that the patent covering our product candidate, as applicable, is invalid and/or unenforceable. In patent litigation in the 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, EP3368069, EP2981822 and EP3365368 are currently subject to opposition proceedings. 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 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, European patents have the option of opting out of the jurisdiction of the UPC and remaining as national patents in the UPC countries. 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 candidates, and we may be unable to obtain patent term extensions and data exclusivity for our 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. We have not yet demonstrated the ability to progress any of our product candidates through clinical trials, we have no products approved for commercial sale and we have not generated any revenue from product sales to date. 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, 2025 and 2024, we reported a net loss of $287.0 million and $179.8 million, respectively. We have incurred losses since our inception, and as of September 30, 2025, we had an accumulated deficit of $1.2 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 candidates, apitegromab, SRK-181, SRK-439, and any future product candidates and prepare for the commercialization of apitegromab, if approved.

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 preclinical studies and clinical trials, receiving marketing approval for 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.

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

We will 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 apitegromab, 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 and preclinical studies and clinical trials for SRK-439 and any future product candidates, to seek regulatory approvals for our product candidates and to launch and commercialize any products for which we receive regulatory approval. As of September 30, 2025, we had approximately $369.6 million in cash, cash equivalents and marketable securities. Based on our current operating model, we believe that our existing cash, cash equivalents, marketable securities and cash available to us as of September 30, 2025, will be sufficient to fund our operating expenses and capital expenditure requirements into 2027. 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 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 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, 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 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 crisis or macroeconomic conditions; and
- the cost of establishing sales, marketing and distribution capabilities for any product candidates for which we may receive regulatory approval in regions where we choose to commercialize our products 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 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, SRK-181, SRK-439 or any future product candidate at an earlier stage than otherwise would be desirable or relinquish our rights to product candidates or technologies that we otherwise would seek to develop or commercialize ourselves. 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, 2024, we had net operating loss carryforwards for U.S. federal and state income tax purposes of $512.6 million and $498.8 million, respectively, which begin to expire in 2032, except for our post 2017 U.S. federal net operating loss carryforwards of $462.1 million, and $0.2 million of state net operating losses which do not expire. As of December 31, 2024, we also had available tax credit carryforwards for U.S. federal and state income tax purposes of $54.9 million and $7.7 million, respectively, which begin to expire in 2034 and 2025, respectively. Additionally, for taxable years beginning after December 31, 2017 the deductibility of the indefinite lived U.S. 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, 2025, the fair value of our cash, cash equivalents and investments in our marketable debt securities portfolio was approximately $369.6 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, 2025, we had $0.1 million in net unrealized gains 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 and security agreement 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 10, 2025, we entered into an Amended and Restated Loan and Security Agreement (the "Amended and Restated Loan and Security Agreement") with Oxford Finance LLC ("Oxford"). The Amended and Restated Loan and Security Agreement amends and restates in its entirety that certain Loan and Security Agreement dated as of October 16, 2020, as amended.

The Amended and Restated Loan and Security Agreement provides us with up to $200.0 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 Amended and Restated Loan and Security 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 Amended and Restated Loan and Security Agreement, Oxford may accelerate all of our repayment obligations and exercise all of their rights and remedies under the Amended and Restated Loan and Security 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. Oxford could declare a default upon the occurrence of customary events of default, including events that they interpret as a material adverse change as delineated in the Amended and Restated Loan and Security Agreement, payment defaults or breaches of certain affirmative or negative covenants,

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 Oxford, 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, 2025, our executive officers, directors and their affiliates beneficially hold, in the aggregate, approximately 9% 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.

Beginning January 1, 2026, we will no longer qualify as a smaller reporting company, 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 will no longer be a smaller reporting company as of January 1, 2026, and thus will be subject to additional disclosure and compliance requirements beginning with our Quarterly Report on Form 10-Q for the quarter ended March 31, 2026. Due to this

upcoming transition, we expect to devote significant time and effort to implement and comply with the additional standards, rules and regulations that will apply to us upon losing our smaller reporting company status. Compliance with the additional requirements will also increase our legal, accounting and financial compliance costs.

As a smaller reporting company, we have 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 will no longer be able to rely on the scaled disclosure requirements and other accommodations available to smaller reporting companies. This may require 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 will require significant resources, including additional personnel, enhanced systems and processes, and increased professional fees for accounting, legal, and compliance services. We will 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 new compliance initiatives.

As a public company, we incur significant legal, accounting and other expenses that we did not incur as a private company. 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, 2025, approximately 8,220,620 shares of our common stock were reserved for issuance upon exercise of outstanding common stock purchase warrants. As of September 30, 2025, we also have 17,362,147 shares of our common stock reserved for issuance upon exercise of pre-funded warrants, which are already included in our calculation of our weighted average common shares outstanding. Additionally, 13,546,902 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 LLC ("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, 2025 through September 30, 2025, we sold 2,767,000 shares of our common stock resulting in net proceeds to us of $91.7 million pursuant to the Jefferies sales agreement.

Item 2. Unregistered Sales of Equity Securities

Recent Sales of Unregistered Securities

None.

Issuer Purchases of Equity Securities

None.

Item 3. Defaults Upon Senior Securities

None.

Item 4. Mine Safety Disclosures

None.

Item 5. Other Information

Trading Plans

During the three months ended September 30, 2025, none of our directors or officers adopted contracts, instructions or written plans for the purchase or sale of our securities.

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
10.1*†	Third Amended and Restated Loan and Security Agreement, dated September 19, 2025, by and between the Registrant and Oxford Finance LLC				
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				
101.SCH	Inline XBRL Taxonomy Extension Schema Document				
101.CAL	Inline XBRL Taxonomy Extension Calculation Linkbase Document				
101.DEF	Inline XBRL Taxonomy Extension Definition Linkbase Document				
101.LAB	Inline XBRL Taxonomy Extension Label Linkbase Document				
101.PRE	Inline XBRL Taxonomy Extension Presentation Linkbase Document				
104	Cover Page Interactive Data File (formatted as inline XBRL with applicable taxonomy extension information contained in Exhibits 101.)				

* Filed herewith

** Furnished herewith

† Portions of this exhibit (indicated by asterisks) were omitted in accordance with the rules of the Securities and Exchange Commission.

\+ Indicates a management contract or compensatory plan.

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 14, 2025	By:	/s/ David Hallal
		David Hallal Chief Executive Officer (Principal Executive Officer)
Date: November 14, 2025	By:	/s/ Vikas Sinha
		Vikas Sinha Chief Financial Officer (Principal Financial and Accounting Officer)

CERTAIN IDENTIFIED INFORMATION HAS BEEN EXCLUDED FROM THIS EXHIBIT BECAUSE IT IS BOTH NOT MATERIAL AND IS THE TYPE THAT THE REGISTRANT TREATS AS PRIVATE OR CONFIDENTIAL. INFORMATION THAT WAS OMITTED HAS BEEN NOTED IN THIS DOCUMENT WITH A PLACEHOLDER IDENTIFIED BY THE MARK "[***]".

JOINDER AND THIRD AMENDMENT TO AMENDED AND RESTATED LOAN AND SECURITY AGREEMENT

September 19, 2025

THIS JOINDER AND THIRD AMENDMENT TO AMENDED AND RESTATED LOAN AND SECURITY AGREEMENT (this "**Amendment**") is entered into as of the date first written above, by and among OXFORD FINANCE LLC, a Delaware limited liability company with an office located at 115 South Union Street, Suite 300, Alexandria, VA 22314 ("**Oxford**"), as collateral agent (in such capacity, "**Collateral Agent**"), the Lenders listed on Schedule 1.1 of the Loan Agreement (as defined below) or otherwise party thereto from time to time including Oxford in its capacity as a Lender (each a "**Lender**" and collectively, the "**Lenders**"), SCHOLAR ROCK HOLDING CORPORATION, a Delaware corporation ("**Parent**"), SCHOLAR ROCK, INC., a Delaware corporation ("**SR OpCo**" and together with Parent, each an "**Existing Borrower**" and collectively, "**Existing Borrowers**"), SCHOLAR ROCK U.S. OPERATIONS, INC., a Delaware corporation ("**SR Distribution**"), and SCHOLAR ROCK FOREIGN HOLDINGS, INC., a Delaware corporation ("**SR Foreign Holdings**" and together with SR Distribution, each a "**New Borrower**" and collectively, "**New Borrowers**"; and New Borrowers together with Existing Borrowers, individually and collectively, jointly and severally, "**Borrower**"), each with an office located at 301 Binney Street, 3rd Floor, Cambridge, MA 02142.

A. Collateral Agent, Lenders and Borrower have entered into that certain Amended and Restated Loan and Security Agreement dated as of February 10, 2025, as amended by that certain First Amendment to Amended and Restated Loan and Security Agreement dated as of July 2, 2025 and as amended by that certain Second Amendment to Amended and Restated Loan and Security Agreement dated as of July 30, 2025 (as amended, supplemented or otherwise modified from time to time, the "**Loan Agreement**") pursuant to which Lenders have provided to Borrower certain loans in accordance with the terms and conditions thereof;

B. Borrower has requested that Collateral Agent and Required Lenders (i) join each New Borrower as Borrower under the Loan Agreement and all other Loan Documents, and (ii) modify certain provisions of the Loan Agreement; and

C. Collateral Agent and Required Lenders have agreed to join each New Borrower to the Loan Agreement and all other Loan Documents and amend certain provisions of the Loan Agreement, subject to, and in accordance with, the terms and conditions set forth herein, and in reliance upon the representations and warranties set forth herein.

AGREEMENT

NOW, THEREFORE, in consideration of the promises, covenants and agreements contained herein, and other good and valuable consideration, the receipt and adequacy of which are hereby acknowledged, Borrower, Collateral Agent and Required Lenders hereby agree as follows:

1. Definitions. Capitalized terms used but not defined in this Amendment shall have the meanings given to them in the Loan Agreement.

2. Joinder.

2.1 New Borrowers. Each New Borrower is hereby added as a "Borrower" under the Loan Agreement and all other Loan Documents. All references in the Loan Agreement and all other Loan Documents to "Borrower" shall hereafter mean and include the Existing Borrowers and each New Borrower, individually and collectively, jointly and severally, and each New Borrower shall hereafter have all rights, duties and obligations of "Borrower" thereunder.

2.2 Joinder to Loan Agreement. Each New Borrower hereby joins the Loan Agreement and each of the other Loan Documents, and agrees to comply with and be bound by all of the terms, conditions and covenants of the Loan Agreement and Loan Documents, as if it were originally named a "Borrower" therein. Without limiting the generality of the preceding sentence, each New Borrower agrees that it will be jointly and severally liable, together with Existing Borrowers, for the payment and performance of all obligations and liabilities of Borrower under the Loan Agreement, including, without limitation, the Obligations. Each Borrower may, acting singly, request Credit Extensions pursuant to the Loan Agreement. Each Borrower hereby appoints the other as agent for the other for all purposes under the Loan Documents, including with respect to requesting Credit Extensions pursuant to the Loan Agreement. Each Borrower shall be obligated to repay all outstanding Credit Extensions made pursuant to the Loan Agreement when due, regardless of which Borrower actually received such Credit Extensions, as if each Borrower directly received all Credit Extensions.

2.3 Grant of Security Interest. Each New Borrower hereby grants to Collateral Agent, for the ratable benefit of the Lenders, to secure the payment and performance in full of all of the Obligations, a continuing security interest in, and pledges to Collateral Agent, for the ratable benefit of the Lenders, the Collateral, wherever located, whether now owned or hereafter acquired or arising, and all proceeds and products thereof; provided, however, that solely with respect to such New Borrower's Intellectual Property, such security interest shall only become effective on the date on which the IP Trigger Event occurs and shall remain effective at all times thereafter. Each New Borrower represents, warrants, and covenants that the security interest granted herein is and shall at all times continue to be a first priority perfected security interest in the Collateral (other than with respect to perfection of the security interest on the Shares of any Foreign Subsidiary, which are governed by local law share pledges (if any)), subject only to Permitted Liens that are permitted by the terms of the Loan Agreement to have priority to Collateral Agent's Lien. Each New Borrower hereby authorizes Collateral Agent to file financing statements or take any other action required to perfect Collateral Agent's security interests in the Collateral, without notice to New Borrower, with all appropriate jurisdictions to perfect or protect Collateral Agent's interest or rights under the Loan Documents, including a notice that any disposition of the Collateral, except to the extent permitted by the terms of the Loan Agreement, by New Borrower, or any other Person, shall be deemed to violate the rights of Collateral Agent under the Code.

3. Amendments to Loan Agreement.

3.1 Section 2.5(i) (Third Amendment Fee). Section 2.5 of the Loan Agreement is hereby amended by inserting the following new clause (i) to the end of such Section:

> "(i) Third Amendment Fee. A fully-earned, non-refundable amendment fee in the aggregate amount of [***] Dollars ($[***]) to be shared between the Lenders in accordance with their respective pro Rata Shares due and payable on the Third Amendment Effective Date."

3.2 Section 6.6(a) (Operating Accounts). Section 6.6(a) of the Loan Agreement is hereby amended and restated in its entirety as follows:

> "(a) Maintain all of Borrower's and its Subsidiaries Collateral Accounts at the banks and financial institutions as disclosed in the Perfection Certificates delivered on the Third Amendment Effective Date, or, to the extent constituting Collateral, such other Collateral Accounts as to which Borrower provided notice pursuant to Section 6.6(b), which are subject to a Control Agreement in favor of Collateral Agent (other than any Collateral Account that is not required to constitute Collateral). Borrower shall at all times have on deposit in Collateral Accounts maintained in the United States that are subject to a Control Agreement governed by the law of any state in the United States in favor of Collateral Agent cash in an amount equal to the lesser of (i) one hundred percent (100.00%) of the Dollar value of all of Borrower's consolidated cash, including any Subsidiaries', in the aggregate, at all financial institutions, and (ii) one hundred fifteen percent (115.00%) of the Dollar amount of the then-outstanding Obligations. Clause (i) of the previous sentence shall not be applicable to (and no additional amounts need to be maintained to cover the amounts in) deposit accounts exclusively used for (x) payroll, payroll taxes and other employee wage and benefit payments to or for the benefit of Borrower's, or any of its Subsidiaries', employees identified to Collateral Agent by Borrower as such in the Perfection Certificates (as may be updated from time to time in accordance with the terms hereof) so long as the amounts in such deposit accounts do not exceed one month

of payroll at any time and (y) cash collateral purposes to secure obligations for the Indebtedness permitted by clauses (g) and (k) of the defined term "Permitted Indebtedness"."

3.3 Section 6.12 (Creation/Acquisition of Subsidiaries). Section 6.12 of the Loan Agreement is hereby amended and restated in its entirety as follows:

"In the event Borrower or any of its Subsidiaries creates or acquires any Subsidiary (including, without limitation, pursuant to a Division) after the Effective Date, Borrower shall provide prior written notice to Collateral Agent of the creation or acquisition of such new Subsidiary and take all such action as may be reasonably required by Collateral Agent or any Lender to cause each such Subsidiary to become a co-Borrower hereunder or to guarantee the Obligations of Borrower under the Loan Documents and, in each case, grant a continuing pledge and security interest in and to the assets of such Subsidiary (substantially as described on Exhibit A hereto); and Borrower (or its Subsidiary, as applicable) shall grant and pledge to Collateral Agent, for the ratable benefit of the Lenders, a perfected security interest in the Shares of each such newly created Subsidiary; provided, however, that solely in the circumstance in which Borrower or any Subsidiary creates or acquires a Foreign Subsidiary in an acquisition approved by the Required Lenders, (i) such Foreign Subsidiary shall not be required to guarantee the Obligations of Borrower under the Loan Documents and grant a continuing pledge and security interest in and to the assets of such Foreign Subsidiary, and (ii) Borrower shall not be required to grant and pledge to Collateral Agent, for the ratable benefit of Lenders, a perfected security interest in more than sixty-five percent (65%) of the Shares of such Foreign Subsidiary, if Borrower demonstrates to the reasonable satisfaction of Collateral Agent that a pledge or grant of a security interest in more than sixty-five percent (65%) of the Shares of such Foreign Subsidiary would create a present and existing adverse tax consequence to Borrower under the IRC. For the avoidance of doubt, neither Scholar Rock Netherlands, Scholar Rock Ireland nor any of their respective Foreign Subsidiaries shall be required to become a Loan Party."

3.4 Section 6.14 (Swiss Lien). New Section 6.14 is hereby added to the end of Section 6 of the Loan Agreement as follows:

3.5 "**6.14** [***]**Lien.** Within sixty (60) days after the Third Amendment Effective Date (or such later date as agreed to by Collateral Agent in its sole discretion), Borrower shall cause Scholar Rock [***]to grant a first priority lien to [***] in the [***] License Agreement, together with cash of Scholar Rock [***]to the extent any amounts are due and owing to [***]or its Affiliates thereunder (subject to priority liens of the bank holding Scholar Rock [***]'s bank accounts created under such bank's general terms and conditions), pursuant to security documents governed by the laws of [***]or such other appropriate jurisdiction (each in form and substance reasonably satisfactory to Collateral Agent, a "**[***] Security Document**" and collectively, the "**[***] Security Documents**"). [***]shall, subject to compliance with applicable [***]law and customary [***] (up-/cross-stream security-) limitation provisions to be included in the [***] Security Documents with respect to the protection of Scholar Rock [***]'s capital and Scholar Rock [***]s directors from personal liability , take all actions to ensure that the [***] Security Documents are effective and enforceable under Swiss law and shall take all other actions reasonably requested by Collateral Agent related thereto. Borrower shall provide Collateral Agent with a copy of any notice provided by Scholar Rock [***] to [***]pursuant to any [***] Security Document within two (2) Business Days of [***]receipt of such notice."

3.6 Section 7.13 ([***] IP License Agreement and [***] Security Documents). New Section 7.13 is hereby added to the end of Section 7 of the Loan Agreement as follows:

"**7.13 [***] IP License Agreement and [***] Security Documents.** Amend, modify or waive any provision of the [***] IP License Agreement or any [***] Security Document, if such amendment, modification or waiver is adverse to Collateral Agent in any material respect."

3.7 Section 8.2(a) (Covenant Default). Section 8.2(a) of the Loan Agreement is hereby amended and restated in its entirety as follows:

"(a) Borrower or any of its Subsidiaries fails or neglects to perform any obligation in Sections 6.2 (Financial Statements, Reports, Certificates), 6.4 (Taxes), 6.5 (Insurance), 6.6 (Operating Accounts), 6.7

(Protection of Intellectual Property Rights), 6.9 (Notice of Litigation and Default), 6.10 (Financial Covenant), 6.11 (Landlord Waivers; Bailee Waivers), 6.12 (Creation/Acquisition of Subsidiaries), 6.13 (Further Assurances), 6.14 (Swiss Lien) or Borrower violates any covenant in Section 7; or"

3.8 Section 8.14 ([***] IP License Agreement and [***] Security Document). New Section 8.14 is hereby added to the end of Section 8 of the Loan Agreement as follows:

"**8.14 [***] IP License Agreement and [***] Security Documents.** There is a breach or default under the [***] IP License Agreement or any [***] Security Document; provided, however, that the Event of Default under this Section 8.14 caused by the occurrence of a breach or default under such other agreement shall be cured or waived for purposes of this Agreement upon Collateral Agent receiving written notice from the party asserting such breach or default of such cure or waiver of the breach or default under such other agreement, if at the time of such cure or waiver under such other agreement (x) Collateral Agent or any Lender has not declared an Event of Default under this Agreement and/or exercised any rights with respect thereto; (y) any such cure or waiver does not result in an Event of Default under any other provision of this Agreement or any Loan Document; and (z) in connection with any such cure or waiver under such other agreement, the terms of such agreement are not modified or amended in any manner in breach of this Agreement."

3.9 Section 12.12 (Borrower Liability). The reference to "either Borrower" in the first sentence of Section 12.12 of the Loan Agreement is hereby replaced with "Any Borrower".

3.10 Section 13.1 (Definitions). The following defined terms are hereby added in appropriate alphabetical order to Section 13.1 of the Loan Agreement as follows:

"[***]" is [***], a Delaware corporation.

"[***]" is [***], a company organized under the laws of Ireland.

"[***]" is [***], a company organized under the laws of Switzerland.

"**[***] IP License Agreement**" is that certain IP License Agreement dated as of the Third Amendment Effective Date by and between [***], as licensor, and [***], as licensee, as amended, restated or otherwise modified from time to time after the Third Amendment Effective Date in compliance with the terms of this Agreement.

"**Third Amendment**" means that certain Third Amendment to Amended and Restated Loan and Security Agreement, dated as of the Third Amendment Effective Date, by and among Borrower, Parent, Oxford as a lender and Collateral Agent, and the other Lenders party thereto.

"**Third Amendment Effective Date**" is September 19, 2025.

3.11 Section 13.1 (Definitions). The defined term "**Foreign Subsidiary Trigger Event**" is hereby deleted from Section 13.1 of the Loan Agreement.

3.12 Section 13.1 (Definitions). Clause (l) of the defined term "**Permitted Investments**" in Section 13.1 of the Loan Agreement is hereby amended and restated as follows:

"(l) so long as Borrower is in compliance with Section 6.6(a), Investments (including, for the avoidance of doubt, transfer pricing, cost plus or similar arrangements with Foreign Subsidiaries in the ordinary course of business to the extent constituting Investments) by Borrower or its Subsidiaries in Foreign Subsidiaries; and"

3.13 Section 13.1 (Definitions). The defined term "**Permitted Licenses**" in Section 13.1 of the Loan Agreement is hereby amended and restated as follows:

"**Permitted Licenses**" are (A) licenses of over-the-counter software that is commercially available to the public, (B) non-exclusive and exclusive licenses for the use of the Intellectual Property of Borrower or any of its Subsidiaries entered into in the ordinary course of business, provided, that, with respect to each such license described in clause (B), (i) no Event of Default has occurred or is continuing at the time of such license; (ii) the license constitutes an arms-length transaction, the terms of which, on their face (in each case, other than non-exclusive licenses from or to contract manufacturers, clinical research organizations, clinical trial or research sites, service providers and other entities (A) entered into in the ordinary course of business, (B) the principal purpose of which does not include the acquisition of licensing rights, but which contain such non-exclusive licenses merely ancillary to, and for the limited purpose of, facilitating the principal purposes of the agreement and (C) so long as Borrower has complied with all other applicable terms of the Loan Documents applicable thereto), do not provide for a sale or assignment of any Intellectual Property and do not restrict the ability of Borrower or any of its Subsidiaries, as applicable, to pledge, grant a security interest in or lien on, or assign or otherwise Transfer any Intellectual Property; (iii) in the case of any exclusive license, (x) Borrower delivers ten (10) days' prior written notice and a brief summary of the terms of the proposed license to Collateral Agent and the Lenders and delivers copies of the final executed licensing documents in connection with the exclusive license promptly upon consummation thereof, and (y) any such license could not result in a legal transfer of title of the licensed property but may be exclusive in respects other than territory and may be exclusive as to territory only as to discrete geographical areas outside of the United States; and (iv) all upfront payments, royalties, milestone payments or other proceeds arising from the licensing agreement that are payable to Borrower are paid to a Deposit Account that is governed by a Control Agreement (except in connection with a Permitted Royalty Transaction that is not otherwise restricted by any applicable Acceptable Intercreditor Agreement), (D) any other ancillary license provided in connection with a Permitted Royalty Transaction structured as a synthetic royalty transaction that is in form and substance reasonably satisfactory to the Collateral Agent and otherwise satisfies the conditions of clauses (B)(i) through (iii) hereof (unless otherwise reasonably agreed by the Collateral Agent with respect to clause (B)(ii) in connection with any negative lien covenant provided in any such synthetic royalty transaction (subject to such covenant expressly permitting the Collateral Agent's Liens under the Loan Documents)) and (E) the [***] IP License Agreement.

3.14 Schedule 1.1 (Lenders and Commitments). Schedule 1.1 of the Loan Agreement is hereby amended and restated in its entirety with the Schedule 1.1 attached hereto as Exhibit A.

3.15 Exhibit C (Compliance Certificate). Exhibit C of the Loan Agreement is hereby amended and restated in its entirety with the Exhibit C attached hereto as Exhibit B.

4. Limitation of Joinder and Amendment.

4.1 The joinder and amendments set forth in Section 2 and Section 3 above are effective for the purposes set forth herein and shall be limited precisely as written and shall not be deemed to (a) be a consent to any amendment, waiver or modification of any other term or condition of any Loan Document, or (b) otherwise prejudice any right, remedy or obligation which Collateral Agent or any Lender may now have or may have in the future under or in connection with any Loan Document, as amended hereby.

4.2 This Amendment shall be construed in connection with and as part of the Loan Documents and all terms, conditions, representations, warranties, covenants and agreements set forth in the Loan Documents are hereby ratified and confirmed and shall remain in full force and effect.

5. Representations and Warranties. To induce Collateral Agent and Required Lenders to enter into this Amendment, each Existing Borrower and each New Borrower hereby represent and warrant to Collateral Agent and Required Lenders as follows:

5.1 Immediately after giving effect to this Amendment (a) the representations and warranties contained in the Loan Documents are true, accurate and complete in all material respects as of the date hereof (except to the extent such representations and warranties relate to an earlier date, in which case they are true and correct in all material respects as of such date) and (b) no Event of Default has occurred and is continuing;

5.2 Each Existing Borrower and each New Borrower have the corporate power and authority to execute and deliver this Amendment and to perform its obligations under the Loan Agreement, as amended by this Amendment;

5.3 The Operating Documents of each Existing Borrower and each New Borrower delivered to Collateral Agent (on the Effective Date in the case of Existing Borrowers and on the date hereof in the case of New Borrowers), and updated pursuant to subsequent deliveries by or on behalf of such Person to Collateral Agent, if applicable, remain true, accurate and complete and have not been amended, supplemented or restated and are and continue to be in full force and effect;

5.4 The execution and delivery by each Existing Borrower and each New Borrower of this Amendment and the performance by each Existing Borrower and each New Borrower of their respective obligations under the Loan Agreement, as amended by this Amendment, do not contravene (i) any material law or regulation binding on or affecting any Existing Borrower or New Borrower, (ii) any material contractual restriction with a Person binding on any Existing Borrower or New Borrower, (iii) any order, judgment or decree of any court or other governmental or public body or authority, or subdivision thereof, binding on any Existing Borrower or New Borrower, or (iv) the Operating Documents of any Existing Borrower or New Borrower;

5.5 The execution and delivery by each Existing Borrower and each New Borrower of this Amendment and the performance by each Existing Borrower and each New Borrower of its obligations under the Loan Agreement, as amended by this Amendment, do not require any order, consent, approval, license, authorization or validation of, or filing, recording or registration with, or exemption by any governmental or public body or authority, or subdivision thereof, binding on any Existing Borrower or New Borrower, except as already has been obtained or made; and

5.6 This Amendment has been duly executed and delivered by Borrower and is the binding obligation of each Existing Borrower and each New Borrower, enforceable against each Existing Borrower and each New Borrower in accordance with its terms, except as such enforceability may be limited by bankruptcy, insolvency, reorganization, liquidation, moratorium or other similar laws of general application and equitable principles relating to or affecting creditors' rights.

6. Loan Document. Each Existing Borrower, each New Borrower, Collateral Agent and Lenders agree that this Amendment shall be a Loan Document. Except as expressly set forth herein, the Loan Agreement and the other Loan Documents shall continue in full force and effect without alteration or amendment. This Amendment and the Loan Documents represent the entire agreement about this subject matter and supersede prior negotiations or agreements.

7. Effectiveness. This Amendment shall be deemed effective as of the date hereof upon Collateral Agent and the Lenders' receipt of each of the following, in form and substance acceptable to Collateral Agent and the Lenders:

7.1 this Amendment duly executed by each Existing Borrower, each New Borrower, Collateral Agent and each Lender;

7.2 second amended and restated Secured Promissory Notes with respect to the Term A-1 Loans and amended and restated Secured Promissory Notes with respect to the Term A-2 Loans, each duly executed by each Existing Borrower and each New Borrower;

7.3 updated Perfection Certificate duly executed by Parent, covering each Subsidiary of any Borrower;

7.4 a duly executed IRS Form W-9 for each New Borrower, respectively;

7.5 Corporate Borrowing Certificate duly executed by each New Borrower and a copy of the resolutions of the respective governing bodies of each New Borrower authorizing this Amendment;

7.6 copies of each New Borrower's organizational documents and such other documents and certifications as Collateral Agent may reasonably require to evidence that each New Borrower is duly organized or formed, and that each New Borrower is validly existing and in good standing in its jurisdiction of organization;

7.7 all documents and instruments, including Uniform Commercial Code financing statements, required by Collateral Agent to be filed, registered or recorded to create or perfect the first priority Liens intended to be created under the Loan Documents;

7.8 evidence that no Liens exist on the assets of any Borrower other than Permitted Liens;

7.9 a copy of the [***] IP License Agreement duly executed by the parties thereto;

7.10 a duly executed legal opinion of counsel to New Borrowers;

7.11 Borrower's payment of the amendment fee in the aggregate amount of [***] Dollars ($[***]) as set forth in Section 2.5(i) of the Loan Agreement; and

7.12 Borrower's payment of all Lenders' Expenses to the extent invoiced prior to the date hereof.

8. Post Closing. Borrower shall deliver to Collateral Agent (each, respectively, in form and substance satisfactory to Collateral Agent in is reasonable discretion), or otherwise satisfy the following obligations, in accordance with the applicable time periods (or such longer periods, respectively, as Collateral Agent may otherwise agree in its sole discretion), as follows:

8.1 On or prior to five (5) Business Days after the Third Amendment Effective Date, a duly executed IRS Form W8-BEN for each of [***], [***] and [***];

8.2 On or prior to five (5) Business Days after the Third Amendment Effective Date, with respect to the Shares of each New Borrower, the share certificates and assignments separate from certificate, duly executed in blank;

8.3 On or prior to thirty (30) days after the Third Amendment Effective, the organizational documents for (a) the entity currently contemplated to be named "[***]" ("[***]") and (b) each Foreign Subsidiary of Borrower to the extent not delivered to Collateral Agent on or prior to the Third Amendment Effective Date;

8.4 On or prior to the earlier of (a) thirty (30) days after the Third Amendment Effective Date and (b) any subsequent Term Loan, the insurance certificates and endorsements required by Section 6.5 of the Loan Agreement;

8.5 On or prior to sixty (60) days after the Third Amendment Effective Date, the [***] Security Documents; and

8.6 On or prior to April 30, 2026, Borrower shall cause [***] to (a) become a co-Borrower under the Loan Agreement and the other Loan Documents, all in accordance with Section 6.12 of the Loan Agreement or (b) be dissolved with all assets and liabilities of [***] transferred to Borrower.

9. Counterparts. This Amendment may be executed in any number of counterparts, each of which shall be deemed an original, and all of which, taken together, shall constitute one and the same instrument. Delivery by electronic transmission (e.g. ".pdf") of an executed counterpart of this Amendment shall be effective as a manually executed counterpart signature thereof.

10. Governing Law. This Amendment and the rights and obligations of the parties hereto shall be governed by and construed in accordance with the laws of the State of New York without regard to principles of conflicts of law.

[*Balance of Page Intentionally Left Blank*]

IN WITNESS WHEREOF, the parties hereto have caused this Joinder and Third Amendment to Amended and Restated Loan and Security Agreement to be duly executed and delivered as of the date first set forth above.

EXISTING BORROWERS:

SCHOLAR ROCK HOLDING CORPORATION

By /s/ Vikas Sinha
Name: Vikas Sinha
Title: Chief Financial Officer

SCHOLAR ROCK, INC.

By /s/ Vikas Sinha
Name: Vikas Sinha
Title: Chief Financial Officer

NEW BORROWERS:

SCHOLAR ROCK U.S. OPERATIONS, INC.

By /s/ Julin Ho
Name: Julin Ho
Title: Secretary

SCHOLAR ROCK FOREIGN HOLDINGS, INC.

By /s/ Vikas Sinha
Name: Vikas Sinha
Title: Treasurer

{*signature pages continue*}

COLLATERAL AGENT:

OXFORD FINANCE LLC, as Collateral Agent

By: /s/ Colette H. Featherly
Name: Colette H. Featherly
Its: Executive Vice President

LENDERS:

OXFORD FINANCE LLC, as a Lender

By: /s/ Colette H. Featherly
Name: Colette H. Featherly
Its: Executive Vice President

OXFORD FINANCE FUNDING I, LLC, as a Lender
OXFORD FINANCE FUNDING IX, LLC, as a Lender
OXFORD FINANCE FUNDING XIII, LLC, as a Lender
OXFORD FINANCE FUNDING TRUST 2023-1, as Lender
By: Oxford Finance LLC, as servicer

By /s/ Colette H. Featherly
Name: Colette H. Featherly
Title: Executive Vice President

OXFORD FINANCE CREDIT FUND FUNDING TRUST II, as a Lender
By: Oxford Finance Credit Fund II LP, as servicer
By: Oxford Finance Advisors, LLC, as manager

By: /s/ Colette H. Featherly
Name: Colette H. Featherly
Its: Executive Vice President

OXFORD FINANCE CREDIT FUND III 2025-A, LP, as a Lender
By: Oxford Finance Advisors, LLC, as servicer

By: /s/ Colette H. Featherly
Name: Colette H. Featherly
Its: Executive Vice President

OXFORD FINANCE CREDIT FUND IV LP, as a Lender
By: Oxford Finance Advisors, LLC, as manager

By: /s/ Colette H. Featherly
Name: Colette H. Featherly
Its: Executive Vice President

Exhibit A

SCHEDULE 1.1

Lenders and Commitments

[***]

Exhibit B

EXHIBIT C

Compliance Certificate

[***]

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 14, 2025

/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 14, 2025

/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, 2025 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 14, 2025

/s/ David Hallal
David Hallal
Chief Executive Officer

Date: November 14, 2025

/s/ Vikas Sinha
Vikas Sinha
Chief Financial Officer