

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

FORM 8-K

CURRENT REPORT

Pursuant to Section 13 or 15(d) of the Securities Exchange Act of 1934

Date of Report (Date of earliest event Reported): May 14, 2025

Scholar Rock Holding Corporation

(Exact Name of Registrant as Specified in Charter)

Delaware
(State or Other Jurisdiction of
Incorporation)

001-38501
(Commission File Number)

82-3750435
(I.R.S. Employer Identification Number)

301 Binney Street, 3rd Floor, Cambridge, MA 02142
(Address of Principal Executive Offices) (Zip Code)

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

(Former name or former address, if changed since last report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

- Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
- Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
- Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
- Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

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

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

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (17 CFR §230.405) or Rule 12b-2 of the Securities Exchange Act of 1934 (17 CFR §240.12b-2). 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.

Item 2.02. Results of Operations and Financial Condition.

On May 14, 2025, Scholar Rock Holding Corporation (the “Company”) issued a press release announcing its financial and operating results for the quarter ended March 31, 2025. A copy of the press release is being furnished as Exhibit 99.1 to this Report on Form 8-K.

The information in this Report on Form 8-K and Exhibit 99.1 attached hereto is intended to be furnished and shall not be deemed “filed” for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the “Exchange Act”), or otherwise subject to the liabilities of that section, nor shall it be deemed incorporated by reference in any filing under the Securities Act of 1933, as amended, or the Exchange Act, except as expressly set forth by specific reference in such filing.

Item 7.01. Regulation FD Disclosure.

The Company will utilize slides during its conference call scheduled for 8:15 am ET on May 14, 2025 to announce its financial and operating results for the fiscal quarter ended March 31, 2025 and to provide a business update for the Company. A copy of the slides is furnished as Exhibit 99.2 to this Current Report on Form 8-K and is incorporated by reference into this Item 7.01 as if fully set forth herein.

The information in this Report on Form 8-K and Exhibits 99.1 and 99.2 attached hereto are intended to be furnished and shall not be deemed “filed” for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the “Exchange Act”), or otherwise subject to the liabilities of that section, nor shall it be deemed incorporated by reference in any filing under the Securities Act of 1933, as amended, or the Exchange Act, except as expressly set forth by specific reference in such filing.

Item 9.01. Financial Statements and Exhibits.

(d) Exhibits

**Exhibit
No.**

Description

99.1	Press Release issued by the Company on May 14, 2025, furnished hereto.
99.2	Presentation Slides for May 14, 2025 Conference Call, furnished hereto.
104	Cover Page Interactive Data File (embedded within the Inline XBRL document)

SIGNATURE

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 hereunto duly authorized.

Scholar Rock Holding Corporation

Date: May 14, 2025

By: /s/ Junlin Ho

Junlin Ho
General Counsel & Corporate Secretary

Scholar Rock Reports First Quarter 2025 Financial Results and Highlights Business Progress

- *Apitegromab BLA accepted under priority review for patients with Spinal Muscular Atrophy (SMA) and FDA's PDUFA date set for September 22, 2025; commercial readiness progresses*
- *European Medicines Agency validated Marketing Authorisation Application (MAA) for apitegromab for patients with SMA*
- *Topline results from Phase 2 EMBRAZE proof-of-concept trial in adult patients with obesity on track for June 2025*
- *Cash, cash equivalents and marketable securities of \$364.4 million as of March 31, 2025; expected to support commercial and development programs into 2027*
- *Management to host update call today at 8:15 a.m. ET*

CAMBRIDGE, Mass.--(BUSINESS WIRE)— May 14, 2025-- Scholar Rock (NASDAQ: SRRK), a late-stage biopharmaceutical company focused on developing and commercializing apitegromab for patients with spinal muscular atrophy (SMA) and other severe and debilitating neuromuscular diseases, today reported financial results and updates for the first quarter ended March 31, 2025.

"Scholar Rock is at an inflection point as we evolve to a commercial-stage biopharmaceutical company, and our team remains focused on key priorities in preparation for the potential U.S. launch of apitegromab in Q3 2025," said David L. Hallal, Chief Executive Officer of Scholar Rock. "With the FDA granting our BLA priority review and review of the MAA underway in Europe, I am delighted we have bolstered our leadership with the appointments of Akshay Vaishnav, Keith Woods and Vikas Sinha as we scale our operations to serve patients with SMA in the U.S., Europe and in many additional countries around the world."

Company Highlights and Upcoming Milestones

Spinal Muscular Atrophy (SMA) Program

Apitegromab is an investigational monoclonal antibody that inhibits myostatin activation by selectively binding the pro- and latent forms of myostatin in skeletal muscle. Apitegromab is the only muscle-targeted therapeutic candidate for patients with SMA to demonstrate statistically significant improvement and clinically meaningful benefit in a pivotal Phase 3 (SAPPHIRE) clinical trial.

- **Granted priority review for BLA.** In March 2025, the FDA accepted the Company's BLA for apitegromab for people with SMA and granted the application Priority Review with a Prescription Drug User Fee Act (PDUFA) target action date of September 22, 2025. In anticipation of potential regulatory approvals, Scholar Rock is planning for a U.S. commercial launch upon approval in 2025.
-

- **Received validation for the Marketing Authorisation Application (MAA) from the European Medicines Agency (EMA).** European launch of apitegromab is anticipated in 2026 upon approval.
- **Advancing U.S. commercial launch readiness,** including hiring of customer-facing teams mid-year.
- **Progressing launch preparedness in Germany, the first European market targeted in 2026.** Disease awareness and market access initiatives are underway across key European markets.
- **Presented new clinical data from the Phase 3 SAPPHIRE clinical trial at the 2025 MDA Clinical & Scientific Conference.** In addition to treatment with apitegromab achieving statistical significance and clinically meaningful motor function improvement on the SAPPHIRE trial's primary endpoint (the gold standard Hammersmith Functional Motor Scale Expanded, or HFMSSE), additional data demonstrated clinically meaningful and consistent benefit in motor function across pre-specified SMA patient subgroups (type of SMN-targeted therapy, age, age of initiation of SMN-therapy, and geography). Efficacy was also supported by additional pre-specified patient outcome measures of motor function, including Revised Upper Limb Module (RULM) and World Health Organization (WHO) motor development milestones.
- **Expect to initiate the Phase 2 OPAL clinical trial in SMA in Q3 2025.** The trial will evaluate apitegromab in infants and toddlers with SMA under two years of age who have been or are continuing to be treated with any currently approved SMN therapy.

Apitegromab in Additional Rare, Severe and Debilitating Neuromuscular Disorders

- **Expanding development of apitegromab in additional rare, severe and debilitating neuromuscular disorders.** Building on the positive Phase 3 SAPPHIRE trial in SMA, the Company is exploring the development of apitegromab in neuromuscular conditions characterized by progressive muscle degeneration leading to loss of mobility, reduction in activities of daily living, and lack of independence. These disorders include Duchenne muscular dystrophy (DMD) and Facioscapulohumeral muscular dystrophy (FSHD), and the Company continues to evaluate its clinical development programs in these areas and others as part of its efforts to be a leading neuromuscular disease company.
- **Presented preclinical data demonstrating the impact of murine equivalent of apitegromab in a DMD mouse model at the MDA Clinical and Scientific Conference in March 2025.** The poster presentation included preclinical data combining the murine equivalent of apitegromab and a dystrophin corrector for addressing muscle defects in a DMD mouse model. Treatment resulted in significant additive increases in muscle size, dystrophin levels, and strength. These preclinical results support the potential of apitegromab in DMD.

Cardiometabolic Program

- **Topline data to be reported in June 2025 from the Phase 2 EMBRAZE proof-of-concept trial.** The trial is designed to evaluate the safety and efficacy of apitegromab in preserving muscle mass in adults who are overweight or obese and receiving tirzepatide. This exploratory trial will be used to guide Scholar Rock's future plans for potential development in obesity.

Advancing Our Portfolio of Highly Innovative and Selective Latent Myostatin Inhibitors

SRK-439 is a novel, investigational, preclinical, subcutaneous myostatin inhibitor that binds to pro- and latent myostatin with high affinity and is selective for myostatin (i.e., no GDF11 or Activin A binding).

Based on preclinical data, SRK-439 has the potential to potentially inhibit myostatin and increase muscle mass.

- **IND application for SRK-439 to support the first in human study remains on track and is expected to be filed in Q3 2025.**

Corporate

- **David L. Hallal was appointed Chief Executive Officer as part of a planned transition ahead of global launch of apitegromab for SMA.** He has served as Chairman of Scholar Rock's Board of Directors since 2017. Jay Backstrom, M.D. MPH, to serve as a strategic advisor and will continue to work closely with the executive team and Board of Directors.
- **Akshay Vaishnav, M.D., Ph.D., was appointed to newly created role of President of R&D.** He has served as a Scholar Rock Board member since 2019 and was formerly President of Alnylam.
- **R. Keith Woods was appointed Chief Operating Officer to focus on evolving the organization into a fully integrated global enterprise.** He was formerly COO of argenx.
- **Vikas Sinha was appointed Chief Financial Officer.** He was formerly CFO of Alexion and ElevateBio.

First Quarter 2025 Financial Results

For the quarter ended March 31, 2025, net loss was \$74.7 million or \$0.67 per share compared to a net loss of \$56.9 million or \$0.59 per share for the quarter ended March 31, 2024.

- The Company did not record any revenue for the quarter ended March 31, 2025 or for the quarter ended March 31, 2024.
- Research and development expense was \$48.7 million for the quarter ended March 31, 2025, compared to \$43.1 million for the quarter ended March 31, 2024. The increase was primarily driven by investment in commercial manufacturing and launch readiness activities for apitegromab, continued development of SRK-439, and employee-related costs.
- General and administrative expense was \$28.4 million for the quarter ended March 31, 2025, compared to \$15.3 million for the quarter ended March 31, 2024. The increase was due to employee-related costs and investment into the infrastructure to support the commercialization of apitegromab.
- As of March 31, 2025, Scholar Rock had cash, cash equivalents, and marketable securities of approximately \$364.4 million, which along with cash available to the Company, is expected to fund the anticipated operating and capital expenditure requirements into 2027.

Conference Call Information

Management will provide an update on the Company and discuss first quarter 2025 results via conference call on Wednesday, May 14 at 8:15 am ET. To access the live conference call, participants

may register [here](#). The live audio webcast of the call will be available under "Events and Presentations" in the Investor Relations section of the Scholar Rock website at <http://investors.scholarrock.com>. To participate via telephone, please register in advance [here](#). Upon registration, all telephone participants will receive a confirmation email detailing how to join the conference call, including the dial-in number along with a unique passcode and registrant ID that can be used to access the call. An archived replay of the webcast will be available on the Company's website for approximately 90 days.

About Scholar Rock

Scholar Rock is a biopharmaceutical company that discovers, develops, and delivers life-changing therapies for people with serious diseases that have high unmet need. As a global leader in the biology of the transforming growth factor beta (TGF β) superfamily, the company is named for the visual resemblance of a scholar rock to protein structures. Over the past decade, Scholar Rock has created a pipeline with the potential to advance the standard of care for neuromuscular disease, cardiometabolic disorders, cancer, and other conditions where growth factor-targeted drugs can play a transformational role.

This commitment to unlocking fundamentally different therapeutic approaches is powered by broad application of a proprietary platform, which has developed novel monoclonal antibodies to modulate protein growth factors with extraordinary selectivity. By harnessing cutting-edge science in disease spaces that are historically under-addressed through traditional therapies, Scholar Rock works every day to create new possibilities for patients. Learn more about our approach at [ScholarRock.com](https://www.scholarrock.com) and follow @ScholarRock and on LinkedIn.

The efficacy and safety of apitegromab, SRK-181, and SRK-439 have not been established and apitegromab, SRK-181, and SRK-439 have not been approved for any use by the FDA or any other regulatory agency.

Scholar Rock® is a registered trademark of Scholar Rock, Inc.

Availability of Other Information About Scholar Rock

Investors and others should note that we communicate with our investors and the public using our company website www.scholarrock.com, including, but not limited to, company disclosures, investor presentations and FAQs, Securities and Exchange Commission filings, press releases, public conference call transcripts and webcast transcripts, as well as on X (formerly known as Twitter) and LinkedIn. The information that we post on our website or on X (formerly known as Twitter) or LinkedIn could be deemed to be material information. As a result, we encourage investors, the media and others interested to review the information that we post there on a regular basis. The contents of our website or social media shall not be deemed incorporated by reference in any filing under the Securities Act of 1933, as amended.

Forward-Looking Statements

This press release contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995, including, but not limited to, statements regarding Scholar Rock's future expectations, plans and prospects, including without limitation, Scholar Rock's expectations regarding its

growth, strategy, progress and timing of its clinical trials for apitegromab and its preclinical programs, including SRK-439, and indication selection and development timing, including the timing of any regulatory submissions, the therapeutic potential, clinical benefits and safety of any product candidates, expectations regarding timing, success and data announcements of current ongoing preclinical and clinical trials, its cash runway, expectations relating to commercial launch timing in the US and in Europe, expectations regarding the achievement of important milestones, the ability of any product candidate to perform in humans in a manner consistent with earlier nonclinical, preclinical or clinical trial data, and the potential of its product candidates and proprietary platform. The use of words such as "may," "might," "could," "will," "should," "expect," "plan," "anticipate," "believe," "estimate," "project," "intend," "future," "potential," or "continue," and other similar expressions are intended to identify such forward-looking statements. All such forward-looking statements are based on management's current expectations of future events and are subject to a number of risks and uncertainties that could cause actual results to differ materially and adversely from those set forth in or implied by such forward-looking statements. These risks and uncertainties include, without limitation, that preclinical and clinical data, including whether the results from the Phase 3 SAPPHIRE trial will be sufficient to support regulatory approval, that the full results from the Phase 3 SAPPHIRE trial may differ from the topline data, that preclinical and clinical data, including the results from the Phase 2 or Phase 3 clinical trial of apitegromab, or Part A or Part B of the Phase 1 clinical trial of SRK-181, are not predictive of, may be inconsistent with, or more favorable than, data generated from future or ongoing clinical trials of the same product candidates; Scholar Rock's ability to manage expenses or provide the financial support, resources and expertise necessary to identify and develop product candidates on the expected timeline; information provided or decisions made by regulatory authorities; competition from third parties that are developing products for similar uses; Scholar Rock's ability to obtain, maintain and protect its intellectual property; and Scholar Rock's dependence on third parties for development and manufacture of product candidates including, without limitation, to supply any clinical trials as well as those risks more fully discussed in the section entitled "Risk Factors" in Scholar Rock's Quarterly Report on Form 10-Q for the quarter ended March 31, 2025, as well as discussions of potential risks, uncertainties, and other important factors in Scholar Rock's subsequent filings with the Securities and Exchange Commission. Any forward-looking statements represent Scholar Rock's views only as of today and should not be relied upon as representing its views as of any subsequent date. All information in this press release is as of the date of the release, and Scholar Rock undertakes no duty to update this information unless required by law.

Financial Statements

Scholar Rock Holding Corporation
Condensed Consolidated Statements of Operations
(unaudited)
(in thousands, except share and per share data)

	Three Months Ended March 31,	
	2025	2024
Operating expenses		
Research and development	\$ 48,678	\$ 43,094
General and administrative	28,412	15,325
Total operating expenses	<u>77,090</u>	<u>58,419</u>
Loss from operations	(77,090)	(58,419)
Other income (expense), net	2,367	1,566
Net loss	<u>\$ (74,723)</u>	<u>\$ (56,853)</u>
Net loss per share, basic and diluted	<u>\$ (0.67)</u>	<u>\$ (0.59)</u>
Weighted average common shares outstanding, basic and diluted	<u>111,838,272</u>	<u>95,892,601</u>

Scholar Rock Holding Corporation
Condensed Consolidated Balance Sheets

(unaudited)
(in thousands)

	March 31, 2025	December 31, 2024
Assets		
Cash, cash equivalents and marketable securities	\$ 364,375	\$ 437,278
Other current assets	20,872	13,887
Total current assets	<u>385,247</u>	<u>451,165</u>
Other assets	22,306	23,757
Total assets	<u>\$ 407,553</u>	<u>\$ 474,922</u>
Liabilities and Stockholders' Equity		
Current liabilities	\$ 37,574	\$ 46,936
Long-term liabilities	57,646	59,352
Total liabilities	<u>95,220</u>	<u>106,288</u>
Total stockholders' equity	<u>312,333</u>	<u>368,634</u>
Total liabilities and stockholders' equity	<u>\$ 407,553</u>	<u>\$ 474,922</u>

Scholar Rock:

Investors & Media

Rushmie Nofsinger

Scholar Rock

rnofsinger@scholarrock.com

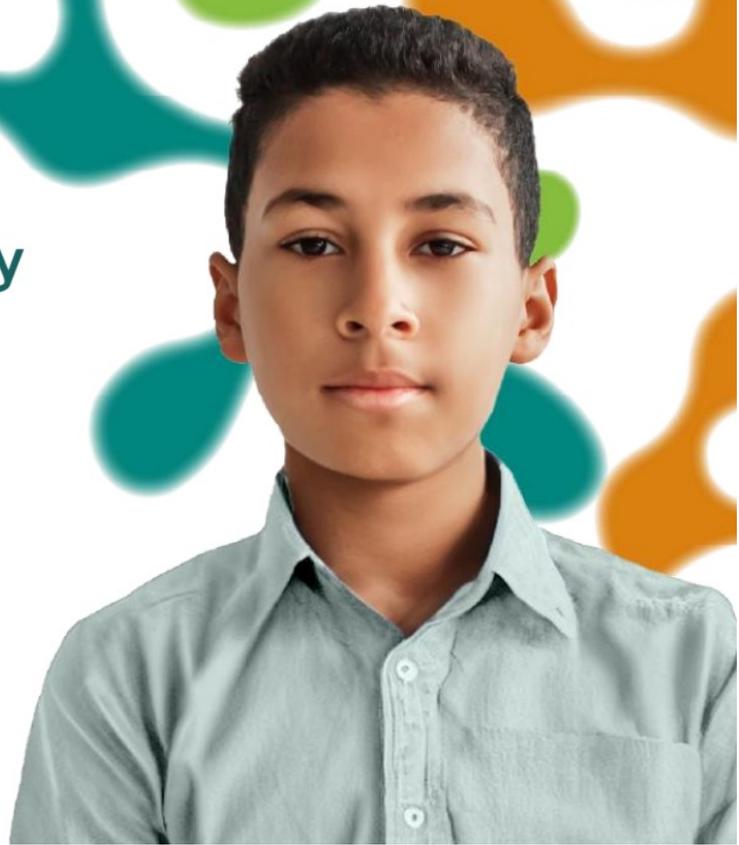
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Dedicated to Dramatically Improving the Lives of Patients with SMA

Q1 2025 Business Update
May 14, 2025



Q1 Earnings Call



TOPIC

SPEAKER

Scholar Rock
Next Phase of Growth

David L. Hallal
Chief Executive Officer

R&D Progress

Akshay Vaishnav, M.D., Ph.D
President of R&D

Commercial Readiness

Keith Woods
Chief Operating Officer

Company Financials

Vikas Sinha
Chief Financial Officer

Q&A Session

Forward-Looking Statements

This presentations contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995, include but not limited to, statements regarding Scholar Rock's future expectations, plans and prospects, including without limitation, Scholar Rock's expectations regarding its growth, strategy, progress and timing of its clinical trials for apitegromab and its preclinical programs, including SRK-439, and indication selection and development timing, including the timing of any regulatory submissions, the therapeutic potential, clinical benefits and safety of any product candidates, expectations regarding timing, success and data announcements of current ongoing preclinical clinical trials, its cash runway, expectations relating to commercial launch timing in the US and in Europe, expectations regarding achievement of important milestones, the ability of any product candidate to perform in humans in a manner consistent with earlier nonclinical preclinical or clinical trial data, and the potential of its product candidates and proprietary platform. The use of words such as "may," "might," "could," "will," "should," "expect," "plan," "anticipate," "believe," "estimate," "project," "intend," "future," "potential," or "continue," other similar expressions are intended to identify such forward-looking statements. All such forward-looking statements are based on management's current expectations of future events and are subject to a number of risks and uncertainties that could cause actual results to differ materially and adversely from those set forth in or implied by such forward-looking statements. These risks and uncertainties include, without limitation, that preclinical and clinical data, including whether the results from the Phase 3 SAPPHIRE trial will be sufficient to support regulatory approval, that the full results from the Phase 3 SAPPHIRE trial may differ from the topline data, that preclinical and clinical data, including the results from the Phase 2 or Phase 3 clinical trial of apitegromab, or Part A or Part B of the Phase 1 clinical trial of SRK-181, are predictive of, may be inconsistent with, or more favorable than, data generated from future or ongoing clinical trials of the same product candidates; Scholar Rock's ability to manage expenses or provide the financial support, resources and expertise necessary to identify and develop product candidates on the expected timeline; information provided or decisions made by regulatory authorities; competition from third parties that are developing products for similar uses; Scholar Rock's ability to obtain, maintain and protect its intellectual property; and Scholar Rock's dependence on third parties for development and manufacture of product candidates including, without limitation, to supply any clinical trial material as well as those risks more fully discussed in the section entitled "Risk Factors" in Scholar Rock's Quarterly Report on Form 10-Q for the quarter ended March 31, 2025, as well as discussions of potential risks, uncertainties, and other important factors in Scholar Rock's subsequent filings with the Securities and Exchange Commission. Any forward-looking statements represent Scholar Rock's views only as of today and should not be relied upon as representing its views as of any subsequent date. All information in this press release is as of the date of the release, and Scholar Rock undertakes no duty to update this information unless required by law.

Scholar Rock Next Phase of Growth

David L. Hallal
Chief Executive Officer



Strong Q1: Advances Scholar Rock Toward Apitegromab US Commercial Launch & Series of Country Launches in Years Ahead

SPINAL MUSCULAR ATROPHY



✓ Detailed Positive Phase 3 SAPHIRE data Presented at MDA

✓ BLA Granted Priority Review PDUFA date September 22

✓ MAA Validated anticipated 2026 approval

PIPELINE



✓ Apitegromab in DMD Preclinical data presented at MDA

✓ Exploratory Ph 2 EMBRAZE POC Study in Obesity Readout on track – June

CORPORATE



✓ Seasoned leadership team for next phase of growth

✓ Commercial buildout continues to progress

✓ \$364.4 million cash as of March 31, 2025

Pending regulatory approval



Advancing Apitegromab for Patients and Families with SMA

Apitegromab in SMA alone is opportunity

to build sustainable growth through the end of this decade and into the next



Commercial launch anticipated in SMA



Global expansion, starting w/ Europe, LATAM, APAC & beyond



SMA expansion with Ph 2 OPAL trial for patients <2 years old

R&D Progress

Akshay Vaishnaw, M.D., Ph.D
President of R&D



Positive Phase 3 Trial with Gold Standard Hammersmith Motor Function Scale in SMA

1.8 

POINT IMPROVEMENT
($p=0.0192$)

in HFMSE* vs. placebo

CONSISTENT

clinically meaningful
benefit observed
across **all age groups**
(2-21)

30% vs 12.5%

of apitegromab patients
ACHIEVED ≥ 3 PT
IMPROVEMENT IN
HFMSE[†] compared to
placebo

**FAVORABLE
SAFETY**

profile consistent with
>48 months experience
in Phase 2 TOPAZ trial

Apitegromab has the potential to transform the lives of children and adults with SMA

*Based on apitegromab combined dose (10 mg/kg and 20 mg/kg) + SOC versus placebo + SOC (Hochberg multiplicity adjustment).

†12.5% of patients on placebo + SOC achieved a ≥ 3 -point improvement in HFMSE

SOC=Standard of care (i.e., nusinersen or risdiplam); HFMSE=Hammersmith Functional Motor Scale-Expanded.

FDA Accepted BLA Under Priority Review (PDUFA Date September 22)

Potential clinical benefits of apitegromab, as demonstrated by our Phase 3 trial, are underscored by the FDA's priority review designation

By definition, a priority review designation by the FDA conveys the capacity of apitegromab to potentially impact unmet need in SMA by either:

- ✓ Being a treatment for a serious or life-threatening condition.
- ✓ Providing a significant improvement in safety or effectiveness over existing treatments.

Growing Our Innovative Anti-myostatin Platform: SRK-439



1 | Optimized for Subcutaneous Formulation

Novel, highly selective myostatin inhibitor



2 | Strong Scientific Validation

Preclinical data demonstrated favorable muscle mass preservation



3 | Advancing to Clinic

On track to file the IND application for SRK-439 in Q3

Goals of the Exploratory EMBRAZE Proof-of-Concept Study



PRESERVE LEAN MASS



COMPARABLE WEIGHT LOSS
REDUCE FAT REGAIN



COMPARABLE SAFETY
(no added toxicities)



EMBRAZE

**Exploratory
program to
generate insights**

Remain Focused & On Track to Deliver R&D Priorities

1

Advance toward anticipated US approval of apitegromab in Q3 2025; EU approval in 2026

2

Initiate Ph 2 OPAL Trial in Q3 2025

3

File IND for SRK-439 in Q3 2025

4

Complete clinical development plans: apitegromab in additional neuromuscular disorders

Commercial Readiness

Keith Woods
Chief Operating Officer



Compelling Phase 3 Results in Patients with SMA

- ✓ **Apitegromab** is the first and only muscle-targeted treatment to show clinically meaningful and statistically significant motor function improvement in SMA.
- ✓ **Apitegromab** has the potential to establish a new standard of care as a first-in-class, best-in-class SMA therapy.



Preparing for
commercial
launch of
apitegromab
in Q3¹

¹ Pending regulatory approval

Apitegromab is an investigational drug candidate under evaluation and has not been approved by any regulatory agency.

Progressive, Debilitating Muscle Degeneration Remains a Critical Unmet Need in SMA Despite SMN-Targeted Therapies



90%
of patients

seek improving muscle strength from a new treatment in SMA¹



>80% of
neurologists agree

efforts to preserve muscle should start as early as possible in SMA²



*Personal hygiene, using the toilet and the shower on my own would be huge. My four-year-old can do it on her own. **It's degrading.***

US Patient

***Muscle atrophy and loss of strength is a key issue in these patients.** Increasing a patients' HFMSE score is really important. It's measurable and meaningful.*

Pediatric Neurologist (UK)

**The SMA community is calling for
a treatment to address progressive muscle degeneration and motor function loss**

1. Cure SMA State of SMA 2024 Report https://www.curesma.org/wp-content/uploads/2025/04/State-of-SMA-Report2024_vWeb.pdf; 2. Scholar Rock Internal Market Research (US Neurologists), 2024.

The Future of SMA is in Treating the Muscle and the Motor Neuron

SMA Today



IN THE US



~10,000
living
with SMA¹



2/3
SMA patients
received an
SMN therapy¹



WORLDWIDE



~35,000
patients have received an
**approved SMN-targeted
therapy^{2,3,4}**

**74% of
neurologists**
agree that in the future,
**multiple modalities
are necessary**
to treat SMA⁵

“ Many researchers believe that it will take a combination of SMN-dependent and SMN-independent treatments to provide the most benefit for those with SMA ”
- Cure SMA⁶

1. Cure SMA State of SMA 2023 Report. https://www.curesma.org/wp-content/uploads/2024/06/9042024_State-of-SMA_vWeb.pdf; 2. Biogen Q4 2023 Report; 3. Roche Q3 2024 report; 4. Novartis Q4 2024 Report; 5. Scholar Rock internal market research (US Neurologists), 2024; 6. Cure SMA, 2025. Approaches to Drug Development. <https://www.curesma.org/approaches-to-drug-development/>

Scholar Rock is Positioned for a Successful Launch in SMA



* Pending regulatory approval.

Scholar Rock Financials

Vikas Sinha
Chief Financial Officer



Strong Cash Position Supports Launch & Additional Priorities

1 Financial Discipline

2 Capital Efficient Commercial Build

3 Disciplined Allocation to Advance Pipeline

2025 Priorities

- US launch expected in 2025 and EU launch in 2026
- SMA: <2 years old study initiation planned for Q3 2025
- Exploring additional neuromuscular indications with apitegromab
- SRK-439 IND filing planned for Q3 2025

Delivering on 2025 Priorities Supports Long-Term Growth

1

Regulatory Approvals & Commercialization

Execute Successful US Commercial Launch*

Advance EU Launch Preparedness

*Pending regulatory approval

2

Expand in Additional Rare, Severe & Debilitating Neuromuscular Diseases

Apitegromab Development Program: Building a Pipeline in a Product

Leverage Highly Innovative Anti-myostatin Platform

3

Disciplined Capital Allocation

Efficient Commercial Build

Phase Investments to Support Future High-value Commercial & Pipeline Initiatives

Q&A