

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): August 6, 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 August 6, 2025, Scholar Rock Holding Corporation (the “Company”) issued a press release announcing its financial and operating results for the fiscal quarter ended June 30, 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 (the “Securities Act”), 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 8:00 am ET on August 6, 2025 to announce its financial and operating results for the fiscal quarter ended June 30, 2025 and to provide a business update on 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 Current 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 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 or the Exchange Act, except as expressly set forth by specific reference in such filing.

**Item 9.01. Financial Statements and Exhibits.**

(d) Exhibits

<b>Exhibit No.</b>	<b>Description</b>
99.1	<a href="#">Press Release issued by the Company on August 6, 2025, furnished hereto.</a>
99.2	<a href="#">Presentation Slides for August 6, 2025 Conference Call, furnished hereto.</a>
104	Cover Page Interactive Data File (embedded within the Inline XBRL document)

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**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: August 6, 2025

By: /s/ Junlin Ho

Junlin Ho  
General Counsel & Corporate Secretary

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### Scholar Rock Reports Second Quarter 2025 Financial Results and Highlights Business Progress

- *FDA accepted the apitegromab BLA under priority review with a PDUFA target action of September 22, 2025; finalizing U.S. commercial launch preparations*
- *European Medicines Agency validated Marketing Authorisation Application (MAA), and regulatory process continues to progress; European launch anticipated in 2026*
- *Positive topline results from Phase 2 EMBRAZE proof-of-concept trial in adult patients with obesity showed statistically significant preservation of lean mass with apitegromab during tirzepatide-induced weight loss*
- *Cash, cash equivalents and marketable securities of \$295 million as of June 30, 2025; expected to support commercial and development programs into 2027*
- *Management to host update call today at 8:00 a.m. ET*

CAMBRIDGE, Mass.--(BUSINESS WIRE)— August 6, 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 second quarter ended June 30, 2025.

“Our BLA is progressing under priority review towards our September 22 PDUFA date, and our team is working with urgency to prepare to serve children and adults living with spinal muscular atrophy,” said David L. Hallal, Chief Executive Officer of Scholar Rock. “If approved, apitegromab will be a first-in-class muscle-targeted therapy with the potential to establish a new standard of care in the treatment for SMA. As we prepare for a successful commercial launch in the U.S., we are continuing to advance our MAA in the EU in parallel, while also planning for extensive global expansion to serve the SMA community with apitegromab worldwide.”

Mr. Hallal added, “In addition, we were pleased to share positive EMBRAZE topline results highlighting the broader potential of our highly selective myostatin inhibition approach to support healthier weight loss by safely preserving lean mass. These results demonstrate the promise of our highly innovative myostatin platform to deliver potentially life-transforming benefits.”

#### Company Highlights and Upcoming Milestones

##### Spinal Muscular Atrophy (SMA) Program

**Apitegromab** is an investigational fully human monoclonal antibody inhibiting myostatin activation by selectively binding the pro- and latent forms of myostatin in skeletal muscle. It is the first muscle-targeted therapeutic candidate in spinal muscular atrophy (SMA) to demonstrate clinical success in a pivotal phase 3 (SAPPHIRE) clinical trial.

- **September 22 PDUFA date.** The FDA has accepted the Biologics License Application (BLA) for apitegromab under priority review and has assigned a Prescription Drug User Fee Act (PDUFA)

target action date for apitegromab of September 22, 2025. In anticipation of potential regulatory approvals, Scholar Rock is planning for a U.S. commercial launch upon approval in 2025.

- **Continuing to finalize key launch preparations.** U.S. customer-facing teams have been hired and deployed in the field and U.S. commercial launch preparation is progressing across the country.
- **Received validation for the Marketing Authorisation Application (MAA) from the European Medicines Agency (EMA).** European launch of apitegromab is anticipated in 2026 upon approval. Scholar Rock is actively progressing launch preparedness in Germany for its first European market. Disease awareness and market access initiatives are underway across additional key European markets.
- **Presented positive Phase 3 SAPPHIRE clinical trial data at the 2025 Annual Cure SMA Research and Clinical Care Meeting in June.**
- **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-targeted 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, activities of daily living, and independence, as part of its efforts to be a leading neuromuscular disease company. These disorders include Duchenne muscular dystrophy (DMD) and Facioscapulohumeral muscular dystrophy (FSHD), and others.

#### Cardiometabolic Program

- **Reported positive topline data from the Phase 2 EMBRAZE proof-of-concept trial in obesity demonstrating statistically significant preservation of lean mass with apitegromab during tirzepatide-induced weight loss.** The trial demonstrated that 30% of total weight loss with tirzepatide alone was due to lean mass loss. Patients receiving apitegromab dosed at 10 mg/kg with tirzepatide over 24 weeks preserved an additional 4.2 pounds (1.9 kilograms) or 54.9% ( $p=0.001$ ) of lean mass compared to tirzepatide alone, leading to higher quality weight loss. Apitegromab with tirzepatide was generally well tolerated by participants. The EMBRAZE results highlight the potential for the Company's highly innovative approach to myostatin inhibition to be important in the development of therapies for patients with obesity and support the opportunity to advance earlier-stage anti-myostatin research assets through collaboration with leading cardiometabolic-focused partners.

#### Advancing Our Portfolio of Highly Innovative and Selective Latent Myostatin Inhibitors

**SRK-439** is a novel, investigational, preclinical myostatin inhibitor for subcutaneous administration 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 potently inhibit myostatin and increase muscle mass and is being developed for the treatment of rare, severe neuromuscular diseases.

- **Scholar Rock remains on track to file an IND application for SRK-439 to support the first in human study in the second half of 2025.**
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**Second Quarter 2025 Financial Results**

For the quarter ended June 30, 2025, net loss was \$110 million or \$0.98 per share compared to a net loss of \$58.5 million or \$0.60 per share for the quarter ended June 30, 2024.

- The Company did not record any revenue for the quarter ended June 30, 2025 or for the quarter ended June 30, 2024.
- Research and development expense was \$62.4 million for the quarter ended June 30, 2025, compared to \$42.4 million for the quarter ended June 30, 2024. The increase of \$20.0 million was primarily due to an increase in external research and development costs of \$11.0 million largely due to drug supply manufacturing costs, an increase in employee related expenses of \$6.3 million, of which \$2.7 million are related to one-time leadership transition costs, and an increase in stock-based compensation expense of \$1.8 million.
- General and administrative expense was \$49.7 million for the quarter ended June 30, 2025, compared to \$17.1 million for the quarter ended June 30, 2024. The increase of \$32.6 million was primarily due to an increase in stock-based compensation expense of \$13.3 million, of which \$8.6 million is related to one-time leadership transition costs, and an increase in employee related expenses of \$10.0 million, of which \$4.4 million are one-time leadership transition costs. In addition, professional services fees increased by \$8.8 million as we continue to build the infrastructure for launch readiness.
- As of June 30, 2025, Scholar Rock had cash, cash equivalents, and marketable securities of approximately \$295 million, which along with cash available to the Company and planned revenues, 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 second quarter 2025 results via conference call on Wednesday, August 6 at 8:00 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 join by dialing 800-715-9871 (domestic) or 646-307-1963 (international) and referencing the conference ID 3205013. An archived replay of the webcast will be available on the Company’s website for approximately 90 days.

**About Scholar Rock**

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. As a global leader in the biology of the transforming growth factor beta (TGFβ) superfamily, the company is named for the visual resemblance of protein structures to scholar rocks. Over the past decade, Scholar Rock has created a pipeline with the potential to advance

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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 on LinkedIn.

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](https://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 and anticipated approvals, the therapeutic potential, clinical benefits and safety of any product candidates, its cash runway, expectations regarding commercial launch timing and 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, whether preclinical and clinical data, including the results from the Phase 3 SAPPHERE trial, will be sufficient to support regulatory approval, that the full results from the Phase 3 SAPPHERE 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

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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 June 30, 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.

<b>Scholar Rock Holding Corporation</b>				
<b>Condensed Consolidated Statements of Operations</b>				
(unaudited)				
(in thousands, except share and per share data)				
	<b>Three Months Ended June 30,</b>		<b>Six Months Ended June 30,</b>	
	<b>2025</b>	<b>2024</b>	<b>2025</b>	<b>2024</b>
Operating expenses				
Research and development	\$ 62,401	\$ 42,373	\$ 111,079	\$ 85,466
General and administrative	49,708	17,125	78,120	32,451
Total operating expenses	<u>112,109</u>	<u>59,498</u>	<u>189,199</u>	<u>117,917</u>
Loss from operations	(112,109)	(59,498)	(189,199)	(117,917)
Other income (expense), net	2,078	990	4,445	2,556
Net loss	<u>\$ (110,031)</u>	<u>\$ (58,508)</u>	<u>\$ (184,754)</u>	<u>\$ (115,361)</u>
Net loss per share, basic and diluted	<u>\$ (0.98)</u>	<u>\$ (0.60)</u>	<u>\$ (1.65)</u>	<u>\$ (1.20)</u>
Weighted average common shares outstanding, basic and diluted	<u>112,703,014</u>	<u>96,813,116</u>	<u>112,273,032</u>	<u>96,352,858</u>

**Scholar Rock Holding Corporation**  
**Condensed Consolidated Balance Sheets**  
(unaudited)  
(in thousands)

	<u>June 30, 2025</u>	<u>December 31, 2024</u>
<b>Assets</b>		
Cash, cash equivalents and marketable securities	\$ 295,013	\$ 437,278

Other current assets	24,113	13,887
Total current assets	319,126	451,165
Other assets	20,919	23,757
Total assets	<u>\$ 340,045</u>	<u>\$ 474,922</u>
<b>Liabilities and Stockholders' Equity</b>		
Current liabilities	\$ 50,435	\$ 46,936
Long-term liabilities	56,317	59,352
Total liabilities	<u>106,752</u>	<u>106,288</u>
Total stockholders' equity	233,293	368,634
Total liabilities and stockholders' equity	<u>\$ 340,045</u>	<u>\$ 474,922</u>

**Scholar Rock:****Investors**

Rushmie Nofsinger  
 Scholar Rock  
[ir@scholarrock.com](mailto:ir@scholarrock.com)  
 857-259-5573

**Media**

Molly MacLeod  
[media@scholarrock.com](mailto:media@scholarrock.com)  
 802-579-5995

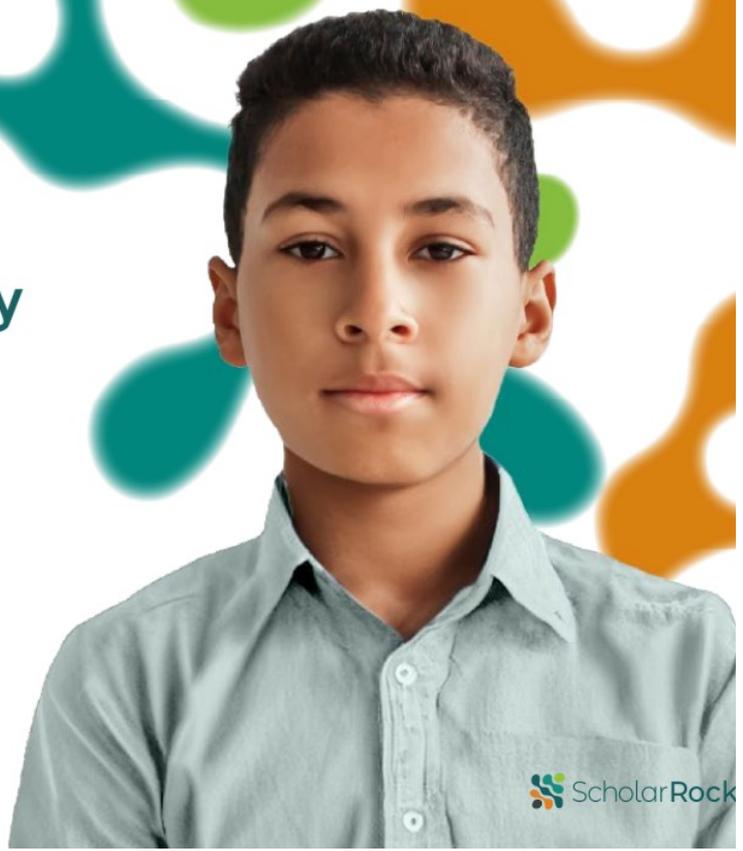
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# Dedicated to Dramatically Improving the Lives of Children and Adults with SMA

**Q2 2025 BUSINESS UPDATE**  
August 6, 2025

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**Q2  
EARNINGS  
CALL**

TOPIC	SPEAKER
<b>SCHOLAR ROCK NEXT PHASE OF GROWTH</b>	<b>David L. Hallal</b> Chairman and Chief Executive Officer
<b>R&amp;D PROGRESS</b>	<b>Akshay Vaishnav, M.D., Ph.D</b> President of R&D
<b>COMMERCIAL READINESS</b>	<b>Keith Woods</b> Chief Operating Officer
<b>COMPANY FINANCIALS</b>	<b>Vikas Sinha</b> Chief Financial Officer
<b>Q&amp;A SESSION</b>	

# Forward-Looking Statements

© 2025 Scholar Rock, Inc. All rights reserved. This presentation 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 apitegror and its preclinical programs, including SRK- 439, and indication selection and development timing, including the timing of any regulatory submissions and anticipated approvals, the therapeutic potential, clinical benefits and safety of any product candidatesits cash runv expectations regarding commercial launch timing in the US and in Europe, expectations regarding the achievement of important milestones, ability of any product candidate to perform in humans in a manner consistent with earlier nonclinical, preclinical or clinical trial data, and 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 intended to identify such forward-looking statements. All such forward-looking statements are based on management's current expectation: future events and are subject to a number of risks and uncertainties that could cause actual results to differ materially and adversely from th set forth in or implied by such forward-looking statements. These risks and uncertainties include, without limitation, whether preclinical clinical data, including the results from the Phase 3 SAPPHIRE trial will be sufficient to support regulatory approval, that the full results from 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, 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 intellectual property; and Scholar Rock's dependence on third parties for development and manufacture of product candidates including, with 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 June 30, 2025, as well as discussions of potential risks, uncertainties, and other important factors. Scholar Rock's subsequent filings with the Securities and Exchange Commission. Any forward-looking statements represent Scholar Rock's view 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.

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



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# SCHOLAR ROCK NEXT PHASE OF GROWTH

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**David L. Hallal**

**Chairman and  
Chief Executive Officer**

## 2025 Priorities Support Long-Term Growth

### ➤ **Apitegromab in SMA Regulatory Approvals & Commercialization**

- Execute Successful US Commercial Launch\*
- Advance EU Launch Preparedness

### ➤ **Expand into Additional Rare, Severe & Debilitating Neuromuscular Diseases**

- **Apitegromab Development Program:** Building a Pipeline in a Product
- Leverage Highly Innovative Anti-myostatin Platform

### ➤ **Disciplined Capital Allocation**

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

\*Pending regulatory approval

# Progress at a Glance: Our Key Milestones

## SPINAL MUSCULAR ATROPHY

✓ **FDA Target Action Date**  
Sept 22, 2025

✓ **MAA Validated**  
Anticipated 2026 approval

✓ **Positive SAPPHIRE Data** Presented at Cure SMA Research Meeting

## PIPELINE

✓ **EMBRAZE Achieved Primary Endpoint**  
Positive Ph 2 proof-of-concept trial in obesity

➡ **File IND for SRK-439**  
in 2H 2025

➡ **Initiate OPAL Trial in Q3**  
In infants & toddlers with SMA

## CORPORATE

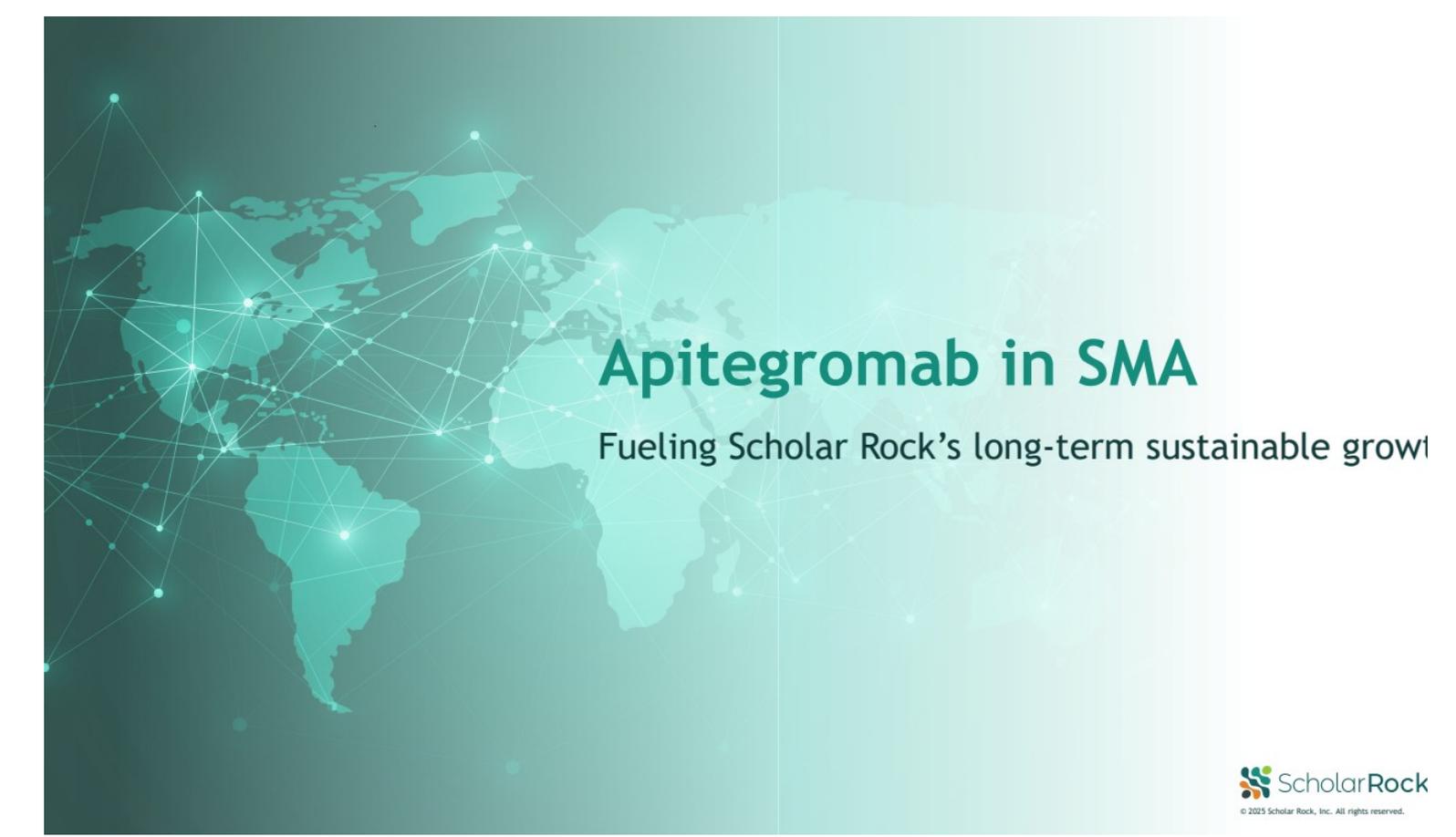
✓ **Experienced Team**  
Powering growth & accelerating transformation

✓ **Commercial Buildout**  
Customer-facing team deployed in the U.S.

✓ **\$295 Million Cash**  
as of June 30, 2025

Pending regulatory approval

 **ScholarRock**  
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A world map in shades of teal and green, overlaid with a network of white lines and dots representing global connectivity. The map is centered on the Atlantic Ocean, showing the Americas on the left and Europe and Africa on the right.

# Apitegromab in SMA

Fueling Scholar Rock's long-term sustainable growth



# R&D PROGRESS

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

# Positive Phase 3 Trial with Gold Standard Hammersmith Functional Motor Scale-Expanded in SMA

**1.8**

**POINT HFMSSE  
IMPROVEMENT**  
vs placebo\*; (n=156)

**30% vs 12.5%**

of apitegromab patients  
**ACHIEVED  $\geq$ 3PT  
IMPROVEMENT IN HFMSSE†**  
compared to placebo

**CONSISTENT**

clinically meaningful  
benefit (1.8 points)  
observed across **ALL AGE  
GROUPS, 2-21; (n=188)**

**FAVORABLE SAFE**

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; n=106) + SOC versus placebo + SOC (n=50)  
(Hochberg multiplicity adjustment).  
†12.5% of patients on placebo + SOC achieved a  $\geq$ 3-point improvement in HFMSSE.  
SOC=Standard of care (i.e., nusinersen or risdiplam); HFMSSE=Hammersmith Functional Motor Scale-Expanded.

# FDA Accepted BLA Under Priority Review - Target Action 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:



Being a treatment for a serious or life-threatening condition.



Providing a significant improvement in safety or effectiveness over existing treatments.

# Expanding Our Impact: Initiating Phase 2 OPAL Trial in Q3

Studying apitegromab in patients under 2 years old



## ADDRESSING THE NEEDS OF INFANTS & TODDLERS WITH SMA

Potential to alter the course of SMA in children under 2



## EXPANDING OUR IMPACT

Including evaluation of apitegromab in patients who received gene therapy



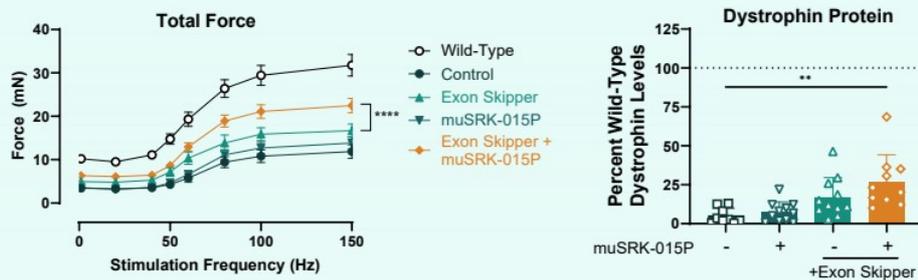
## TIME IS MUSCLE

Reaching patients earlier

# Apitegromab: Pipeline in a Product

Potential to address additional rare, severe and debilitating neuromuscular disorders

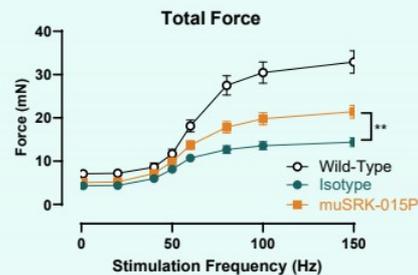
## MuSRK-015P Increased Muscle Force and Dystrophin Protein in Combination With an Exon Skipper in a Mouse Model of DMD<sup>1</sup>



Preclinical data presented at Muscular Dystrophy Association (MDA) Clinical and Scientific Conference 2025

- muSRK-015P treatment increased muscle size as a single agent and in combination with exon skipper (data not shown)
- Combined with an exon skipper, muSRK-015P further enhanced total muscle force and augmented dystrophin expression induced by the exon skipper

## MuSRK-015P Increased Muscle Force in a Model of FSHD as Stand-Alone Therapy



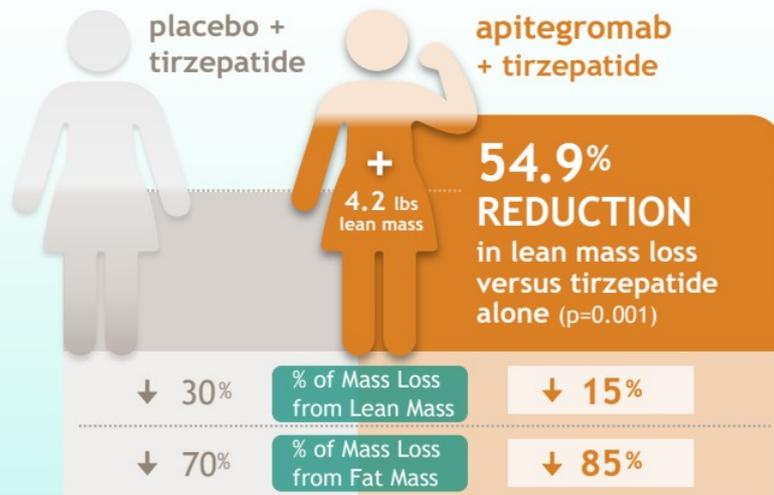
Preclinical data presented at FSHD Society International Research Congress 2025

- muSRK-015P treatment increased muscle size (data not shown)
- Treatment led to increase in total force and enhanced endurance (data not shown)

muSRK-015P used in these studies is a murine version of apitegromab.

1. Fogel, A., et al. "muSRK-015 builds muscle mass and strength in combination with dystrophin upregulation in a mouse model of DMD." Presented at Muscular Dystrophy Association (MDA) Clinical & Scientific Conference, March 2025. 2. Fogel, A., et al. "SRK-015 improves muscle mass, strength, and endurance in the FLEXDUX4.Cre mouse model of FSHD." Presented at FSHD Society International Research Congress, June 2025.

## EMBRAZE Proof-of-Concept Study Achieved Goals



Combining  
apitegromab 10 mg/kg  
with tirzepatide  
over 24 weeks



HIGHER QUALITY  
OF WEIGHT LOSS  
OBSERVED

Apitegromab was generally well tolerated and consistent with safety profile observed in other clinical trials

# Growing Our Innovative Anti-myostatin Platform: SRK-439



## OPTIMIZED FOR SUBCUTANEOUS ADMINISTRATION

Novel, highly selective myostatin inhibitor



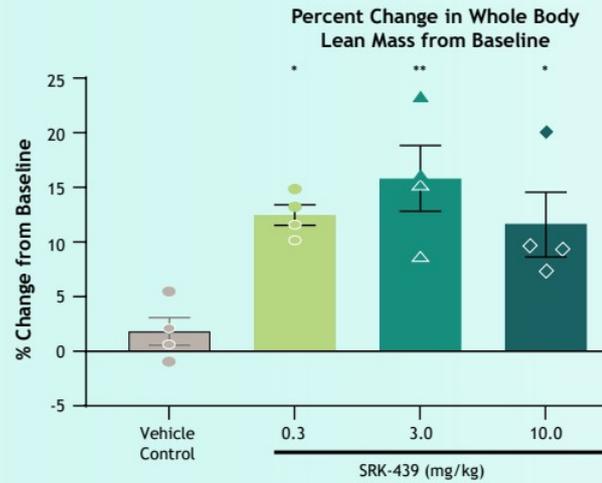
## STRONG SCIENTIFIC VALIDATION

Preclinical data demonstrated favorable muscle mass preservation



## ADVANCING TO CLINIC

On track to file the IND application for SRK-439 in 2H 2025



## Remain Focused On Delivering R&D Priorities

- 1** Advance toward anticipated US approval of apitegromab in Q3 2025; anticipated EU approval in 2026
- 2** Initiate Ph 2 OPAL Trial in Q3 2025
- 3** File IND for SRK-439 in 2H 2025
- 4** Complete clinical development plans: apitegromab in additional rare, severe, and debilitating neuromuscular disorders





# COMMERCIAL READINESS

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**Keith Woods**

**Chief Operating Officer**

# Ushering in a New Treatment Era for SMA

## Preparing for a global launch of apitegromab in SMA\*

➤ SMA is a progressive and devastating disease that leads to loss of mobility, limited activities of daily living, and lack of independence <sup>1</sup>

➤ Apitegromab is the first and only muscle-targeted treatment to show clinically meaningful and statistically significant motor function improvement in SMA.

\*Pending regulatory approvals <sup>1</sup>. Cure SMA State of SMA 2024 Report. [https://www.curesma.org/wp-content/uploads/2025/04/State-of-SMA-Report2024\\_vWeb.pdf](https://www.curesma.org/wp-content/uploads/2025/04/State-of-SMA-Report2024_vWeb.pdf)  
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 SMA<sup>1</sup>

**>80%** OF NEUROLOGISTS AGREE efforts to preserve muscle should start as early as possible in SMA<sup>2</sup>

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

“

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 patient's HFMSE score is really important. It's measurable and meaningful.

- PEDIATRIC NEUROLOGIST (UK)

1. Cure SMA State of SMA 2024 Report. [https://www.curesma.org/wp-content/uploads/2025/04/State-of-SMA-Report2024\\_vWeb.pdf](https://www.curesma.org/wp-content/uploads/2025/04/State-of-SMA-Report2024_vWeb.pdf); 2. Scholar Rock Internal Market Research (US Neurologists), 2024.

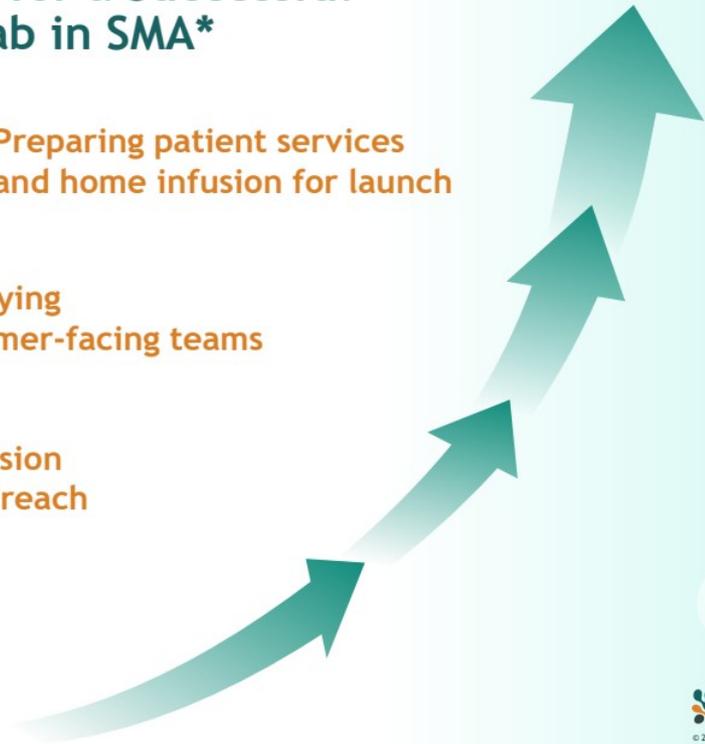
# Scholar Rock is Positioned for a Successful U.S. Launch of Apitegromab in SMA\*

Preparing patient services and home infusion for launch

Deploying customer-facing teams

Continued expansion of U.S. payer outreach in Q2/Q3

Ongoing disease education and SMA stakeholder engagement



\* Pending regulatory approval.

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

## SMA TODAY



IN THE US

**~10,000**Living with SMA<sup>1</sup>**2/3**SMA patients received an SMN therapy<sup>1</sup>

WORLDWIDE

**~35,000**patients have received an approved SMN-targeted therapy<sup>2,3,4</sup>

**74% of neurologists agree that in the future, multiple modalities are necessary to treat SMA<sup>5</sup>**

“

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 SMA<sup>6</sup>

1. Cure SMA State of SMA 2023 Report. [https://www.curesma.org/wp-content/uploads/2024/06/9042024\\_State-of-SMA\\_vWeb.pdf](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 FINANCIALS

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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
- Expand in additional rare, severe, and debilitating neuromuscular indications with apitegromab
- SRK-439 IND filing planned for 2H 2025

## 2025 Priorities Support Long-Term Growth

### ➤ **Apitegromab in SMA Regulatory Approvals & Commercialization**

- Execute Successful US Commercial Launch\*
- Advance EU Launch Preparedness

### ➤ **Expand into Additional Rare, Severe & Debilitating Neuromuscular Diseases**

- **Apitegromab Development Program:** Building a Pipeline in a Product
- Leverage Highly Innovative Anti-myostatin Platform

### ➤ **Disciplined Capital Allocation**

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

\*Pending regulatory approval



# Q&A

