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

May 14, 2025

- *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 Vaishnaw, 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 HFMSE), 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 potently 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 Vaishnaw, 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 (TGFβ) 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 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.

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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	77,090	58,419
Loss from operations	(77,090)	(58,419)
Other income (expense), net	2,367	1,566
Net loss	$ (74,723)	$ (56,853)
Net loss per share, basic and diluted	$ (0.67)	$ (0.59)
Weighted average common shares outstanding, basic and diluted	111,838,272	95,892,601

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	385,247	451,165
Other assets	22,306	23,757
Total assets	$ 407,553	$ 474,922
Liabilities and Stockholders' Equity		
Current liabilities	$ 37,574	$ 46,936
Long-term liabilities	57,646	59,352
Total liabilities	95,220	106,288
Total stockholders' equity	312,333	368,634
Total liabilities and stockholders' equity	$ 407,553	$ 474,922

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