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

August 6, 2025

- *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)--Aug. 6, 2025-- 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, Chairman and 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.**

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 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 on LinkedIn.

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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 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 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 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.

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2025	2024	2025	2024
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	112,109	59,498	189,199	117,917
Loss from operations	(112,109)	(59,498)	(189,199)	(117,917)
Other income (expense), net	2,078	990	4,445	2,556

Net loss	$ (110,031)	$ (58,508)	$ (184,754)	$ (115,361)
Net loss per share, basic and diluted	$ (0.98)	$ (0.60)	$ (1.65)	$ (1.20)
Weighted average common shares outstanding, basic and diluted	112,703,014	96,813,116	112,273,032	96,352,858

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

	June 30, 2025	December 31, 2024
Assets		
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	$ 340,045	$ 474,922
Liabilities and Stockholders' Equity		
Current liabilities	$ 50,435	$ 46,936
Long-term liabilities	56,317	59,352
Total liabilities	106,752	106,288
Total stockholders' equity	233,293	368,634
Total liabilities and stockholders' equity	$ 340,045	$ 474,922

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