Scholar Rock Reports Third Quarter 2025 Financial Results and Recent Business Highlights

November 14, 2025

- *Completed constructive and collaborative in-person Type A meeting with U.S. Food and Drug Administration (FDA) on November 12th for apitegromab biologics license application (BLA) for the treatment of children and adults with spinal muscular atrophy (SMA)*
- *Catalent Indiana, LLC (part of Novo Nordisk) participated in the Type A meeting in person and presented the progress made in implementing the remediation plan; confirmed to FDA that the site is on-track to be reinspection ready by the end of this year*
- *Resubmission of BLA and U.S. launch following approval of apitegromab for children and adults with SMA anticipated in 2026*
- *Timelines accelerated with additional apitegromab U.S. fill-finish facility; tech transfer underway with commercial capacity reserved beginning in Q1 2026*
- *Dosing underway in Phase 2 OPAL study evaluating apitegromab in infants and toddlers <2 years of age with SMA*
- *FDA cleared SRK-439 Investigational New Drug (IND) application; dosing in healthy volunteers to commence in Q4 2025*
- *Cash, cash equivalents and marketable securities of $369.6 million as of September 30, 2025; expected to fund operations into 2027*
- *Company to host conference call today at 8:00 a.m. ET*

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Nov. 14, 2025-- Scholar Rock (NASDAQ: SRRK), a global biopharmaceutical company dedicated to dramatically improving the lives of children and adults with spinal muscular atrophy (SMA) and additional rare, severe, and debilitating neuromuscular diseases by applying its leading platform in myostatin biology to advance musculoskeletal health, today reported financial results for the third quarter ended September 30, 2025, and provided an update on recent company developments.

“We are unwavering in our commitment to bring apitegromab, the world’s first and only muscle-targeted treatment to improve motor function, to children and adults living with SMA,” said David L. Hallal, Chairman and Chief Executive of Scholar Rock. “We are grateful to the FDA, Cure SMA, and our colleagues at Novo Nordisk for the positive engagement at our in-person Type A meeting earlier this week. We are encouraged by the discussion and by our shared understanding of the urgency to bring this important treatment to the SMA community as rapidly as possible.”

Mr. Hallal continued, “In parallel, we are equally focused on establishing Scholar Rock as the global leader in myostatin biology and muscle-targeted therapeutics, and we will continue to advance key strategic programs with tight financial discipline.”

Business Highlights and Upcoming Milestones

Apitegromab

Apitegromab is an investigational fully human monoclonal antibody designed to inhibit myostatin activation by selectively binding the pro- and latent forms of myostatin in skeletal muscle. It is the first and only muscle-targeted therapeutic candidate in SMA to demonstrate statistically significant and clinically meaningful benefit in a pivotal Phase 3 clinical trial (SAPPHIRE).

SMA Program

- **Completed Type A meeting with FDA.** On November 12, 2025, Scholar Rock had a Type A meeting with the U.S. Food and Drug Administration (FDA). The meeting was held in person and was constructive. Catalent Indiana, LLC (part of Novo Nordisk) joined Scholar Rock at the meeting and detailed the progress it has made in implementing the remediation plan, and affirmed that it expects the facility to be ready for reinspection by the end of this year. Resubmission of the BLA and U.S. launch following approval of apitegromab for children and adults with SMA is anticipated in 2026.
- **Accelerated timelines with second fill-finish facility.** Scholar Rock has accelerated timelines with an additional fill-finish facility for apitegromab. The facility is a commercially approved facility that is based in the U.S. and has a proven track record of successful site inspections. Tech transfer is underway, and commercial capacity has been reserved beginning in the first quarter of 2026.
- **European Medicines Agency (EMA) regulatory review ongoing.** Scholar Rock continues to expect a decision on its apitegromab Marketing Authorisation Application (MAA) by mid-2026.
- **Disease awareness and education activities remain underway.** U.S. customer-facing teams are in the field and continue to engage with key stakeholders on SMA disease awareness initiatives as well as on the unmet need and the

importance of targeting muscle. In Europe, disease awareness and market access initiatives continue in key markets.
- **Initiated dosing in Phase 2 OPAL clinical trial.** This trial is designed to evaluate apitegromab in infants and toddlers with SMA under two years of age who have been treated with an SMN1-targeted gene therapy or who are receiving treatment with an approved SMN2-targeted therapy.

Additional Indications

- **Clinical development activities in second indication to initiate by YE 2025.** Scholar Rock expects to initiate clinical development activities in a second neuromuscular disorder by year-end 2025. The Company plans to provide additional information on the disease and the clinical development strategy in early 2026.

SRK-439

SRK-439 is a novel, investigational, subcutaneously administered myostatin inhibitor that binds to pro- and latent myostatin with high affinity and selectivity (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.

- **FDA cleared IND in Q4 2025.** Scholar Rock expects to initiate dosing of SRK-439 in healthy volunteers in the fourth quarter of 2025.

Third Quarter 2025 Financial Results

Scholar Rock reported a net loss of $102.2 million, including stock-based compensation of $18.3 million, for the quarter ended September 30, 2025, compared to a net loss of $64.5 million, including stock-based compensation of $9.7 million, for the quarter ended September 30, 2024. Net loss per common share was $0.90 for the quarter ended September 30, 2025 compared to $0.66 per common share for the quarter ended September 30, 2024.

- The Company did not record any revenue for the quarter ended September 30, 2025 or for the quarter ended September 30, 2024.
- Research and development expense was $50.5 million, including $5.5 million in stock-based compensation, for the quarter ended September 30, 2025, compared to $48.7 million, including $4.4 million in stock-based compensation, for the quarter ended September 30, 2024. The increase of $1.8 million was primarily driven by investment in commercial manufacturing and launch readiness for apitegromab as well as employee related costs.
- General and administrative expense was $53.1 million, including $12.8 million in stock-based compensation, for the quarter ended September 30, 2025, compared to $16.1 million, including $5.2 million in stock-based compensation, for the quarter ended September 30, 2024. The increase of $37.0 million was primarily driven by investments in infrastructure to support launch readiness for apitegromab, including an increase in employee related costs of $13.2 million, an increase in non-cash stock-based compensation expense of $7.6 million related to increased headcount, and an increase in professional services fees of $14.3 million. Additionally, there were one-time severance-related costs of $1.1 million.
- As of September 30, 2025, Scholar Rock had cash, cash equivalents, and marketable securities of approximately $369.6 million. This reflects net proceeds of $91.7 million from the sale of 2.77 million common shares and a drawdown of $50.0 million from the Company's debt facility. This cash, in addition to approximately $60 million anticipated to be available to Scholar Rock from common warrants that expire on December 31, 2025, is expected to be sufficient to fund operations into 2027.

Conference Call Information

Scholar Rock will host a conference call and webcast today, Friday, November 14, at 8:00 a.m. ET to review its third quarter 2025 financial results and discuss recent business updates. To access the live audio webcast, please go to "Events and Presentations" in the Investors 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. A replay of the webcast will be available on the Company's website for approximately 90 days.

About Scholar Rock

Scholar Rock is a late-stage biopharmaceutical company focused on developing and commercializing apitegromab for children and adults with spinal muscular atrophy (SMA) and other rare, severe and debilitating neuromuscular diseases. As a global leader in myostatin biology, a field focused on proteins that regulate muscle mass, the biopharmaceutical company is named for the visual resemblance of a scholar rock to protein structures. Our commitment to unlock fundamentally different treatment approaches is powered by broad application of a proprietary platform, which has developed novel monoclonal antibodies to modulate protein growth factors with extraordinary selectivity. Scholar Rock works every day to create new possibilities for patients through its highly innovative anti-myostatin program, including opportunities in additional rare neuromuscular diseases. Learn more at ScholarRock.com and follow @ScholarRock on X and 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, 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, SRK-439 and its preclinical programs, 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 ability to address the observations identified in the complete response letter, its cash runway into 2027, expectations regarding commercial launch timing in the US and in Europe, expectations regarding a new fill finish facility 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 preclinical and clinical data, including the results from the Phase 2 or Phase 3 clinical trial of apitegromab, or the preclinical data for SRK-439, 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; whether the FDA will accept the remediations to the Novo Nordisk Bloomington Indiana fill finish facility in response to the FDA Observations, whether Scholar Rock will be able to resubmit its BLA in a timely manner and whether the updated BLA will be sufficient to support regulatory approval, 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 September 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 September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Operating expenses				
Research and development	$ 50,486	$ 48,719	$ 161,565	$ 134,185
General and administrative	53,064	16,061	131,184	48,512
Total operating expenses	103,550	64,780	292,749	182,697
Loss from operations	(103,550)	(64,780)	(292,749)	(182,697)
Other income (expense), net	1,330	301	5,775	2,857
Net loss	$ (102,220)	$ (64,479)	$ (286,974)	$ (179,840)
Net loss per share, basic and diluted	$ (0.90)	$ (0.66)	$ (2.54)	$ (1.86)
Weighted average common shares outstanding, basic and diluted	113,729,850	97,050,637	112,763,974	96,587,149

Condensed Consolidated Balance Sheets
(unaudited)
(in thousands)

	September 30, 2025	December 31, 2024
Assets		
Cash, cash equivalents and marketable securities	$ 369,630	$ 437,278
Other current assets	21,081	13,887
Total current assets	390,711	451,165
Other assets	21,007	23,757
Total assets	$ 411,718	$ 474,922
Liabilities and Stockholders' Equity		
Current liabilities	$ 61,972	$ 46,936
Long-term liabilities	104,751	59,352
Total liabilities	166,723	106,288
Total stockholders' equity	244,995	368,634
Total liabilities and stockholders' equity	$ 411,718	$ 474,922

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Investor Contact
Laura Ekas, Ph.D.
Scholar Rock
ir@scholarrock.com
917-439-0374

Media Contact
Molly MacLeod
media@scholarrock.com
802-579-5995

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