Scholar Rock Reports First Quarter 2026 Financial Results and Recent Business Highlights

May 7, 2026

- *FDA accepted apitegromab Biologics License Application (BLA) for treatment of children and adults with spinal muscular atrophy (SMA) with September 30, 2026 Prescription Drug User Fee Act (PDUFA) action date*
- *Accepted apitegromab BLA includes two fill-finish facilities, Catalent Indiana LLC (part of Novo Nordisk), and a second U.S.-based facility*
- *FDA has completed reinspection of Catalent Indiana; classification of facility expected within 90 days following reinspection, in accordance with FDA guidelines*
- *Second fill-finish facility on track to have commercial apitegromab supply in early Q3 2026*
- *Scholar Rock is prepared for U.S. apitegromab launch immediately upon FDA approval, which may be granted at any time through September 30, 2026*
- *Cash, cash equivalents, and marketable securities of $480 million as of March 31, 2026; includes an additional $100 million in debt and $98 million in net cash proceeds from the Company's at-the-market (ATM) program*
- *Management to host a conference call today at 8:00 a.m. ET*

CAMBRIDGE, Mass.--(BUSINESS WIRE)--May 7, 2026-- Scholar Rock (NASDAQ: SRRK), a global biopharmaceutical company dedicated to 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 first quarter ended March 31, 2026, and provided an update on recent company developments.

"With the FDA's acceptance of our apitegromab BLA, we have achieved another critical milestone as we work with urgency to deliver on our mission to bring the world's first muscle-targeted treatment to the SMA community," said David L. Hallal, Chairman and Chief Executive Officer of Scholar Rock. "We are grateful for the FDA's continued high level of engagement, and we are pleased that important progress continues to be made at both of our fill-finish facilities. Our U.S. commercial team stands ready to launch apitegromab on or at any time prior to the September 30th PDUFA date."

Mr. Hallal continued, "Our balance sheet is strong, our clinical-stage pipeline continues to advance, and we are poised, now more than ever, to usher in the next phase of innovation for patients with SMA."

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 a statistically significant and clinically meaningful benefit in a pivotal Phase 3 clinical trial (SAPPHIRE).

SMA Program

- **Apitegromab BLA accepted by FDA with September 30, 2026 PDUFA action date.** The accepted BLA includes two fill-finish facilities, Catalent Indiana and a second U.S.-based fill-finish facility.
- **FDA completed reinspection of Catalent Indiana.** Following the FDA's acceptance of the apitegromab BLA, the Agency completed reinspection of Catalent Indiana. In accordance with FDA guidelines, classification of the facility is anticipated within 90 days following reinspection.
- **Significant progress continues at second fill-finish facility.** Apitegromab commercial supply from this facility is expected to be available early in the third quarter of 2026.
- **Preparations ongoing for U.S. commercial launch.** The Commercial team continues to expand its reach and engagement with key stakeholders, including a significant presence at the Muscular Dystrophy Association (MDA) Clinical & Scientific Conference, which was held March 8 – 11, 2026 in Orlando, FL and at the upcoming 2026 Annual SMA Conference, which is being held June 25 – 28, 2026 in Orlando, FL. The U.S. commercial team is prepared to launch apitegromab immediately upon FDA approval.
- **European Medicines Agency (EMA) regulatory review ongoing.** A Committee for Medicinal Products for Human Use (CHMP) opinion for the apitegromab Marketing Authorisation Application (MAA) is anticipated near mid-2026. The Scholar Rock team in Europe continues to engage with key stakeholders on SMA disease awareness and education initiatives, including at the 5th International Scientific Congress on SMA, which was held March 11 – 14, 2026 in Budapest, Hungary. The Company is planning for an apitegromab launch in Europe in the second half of 2026, beginning with Germany.

- **Enrollment progressing in Phase 2 OPAL clinical trial**. Patients continue to be enrolled and dosed in the Phase 2 OPAL study (NCT07047144). The trial is evaluating apitegromab in infants and toddlers with SMA under two years of age who have received an approved SMN1-targeted gene therapy or who are receiving ongoing treatment with an approved SMN2-targeted therapy.
- **Subcutaneous apitegromab development continues to progress.** Scholar Rock is advancing a subcutaneous formulation of apitegromab intended to provide optionality for patients as a small volume, self- or caregiver-administered anti-myostatin antibody suitable for an autoinjector. A Phase 1 study in healthy volunteers has been completed. Further development activities are ongoing, including anticipated FDA and EMA regulatory engagements following apitegromab approvals.

FSHD Program

- **Phase 2 FORGE trial on track for initiation in mid-2026.** Scholar Rock is developing apitegromab for the treatment of people with facioscapulohumeral muscular dystrophy (FSHD). FSHD is a rare, progressive neuromuscular disease characterized by muscle atrophy and functional decline, affecting approximately 30,000 individuals across the U.S. and Europe. Initiation of a Phase 2 randomized, double-blind, placebo-controlled trial, called FORGE, is expected in mid-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.

- **Phase 1 healthy volunteer study ongoing.** A Phase 1 study evaluating SRK-439 in healthy volunteers is underway, with topline data expected in the second half of 2026.

First Quarter 2026 Financial Results

Scholar Rock reported a net loss of $105.5 million, including stock-based compensation of $18.2 million, for the quarter ended March 31, 2026, compared to a net loss of $74.7 million, including stock-based compensation of $13.4 million, for the quarter ended March 31, 2025. Net loss per common share was $0.83 for the quarter ended March 31, 2026, compared to $0.67 per common share for the quarter ended March 31, 2025.

- The Company did not record any revenue for the quarters ended March 31, 2026 and 2025.
- Research and development expense was $51.8 million, including $6.5 million in stock-based compensation, for the quarter ended March 31, 2026, compared to $48.7 million, including $4.0 million in stock-based compensation, for the quarter ended March 31, 2025.
- General and administrative expense was $50.2 million, including $11.7 million in stock-based compensation, for the quarter ended March 31, 2026, compared to $28.4 million, including $9.4 million in stock-based compensation, for the quarter ended March 31, 2025.
- As of March 31, 2026, Scholar Rock had cash, cash equivalents, and marketable securities of $479.9 million. This reflects a drawdown of $100.0 million from the Company's debt facility and net cash proceeds of $98.0 million from the Company's at-the-market (ATM) program.

Conference Call Information

Scholar Rock will host a conference call and webcast today, Thursday, May 7, at 8:00 a.m. ET to review its first quarter 2026 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.

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 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 and development programs for apitegromab, including its subcutaneous formulation, SRK-439 and its preclinical programs, and indication selection and development timing, including the timing of any regulatory submissions, decisions 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, expectations regarding actions by the FDA after its reinspection of the Catalent Indiana facility; the expected timing and outcome of FDA review of the accepted BLA for apitegromab, including the September 30, 2026 PDUFA action date; expectations regarding the availability and timing of commercial supply of apitegromab from Catalent Indiana and a second U.S.-based fill-finish facility, including expected supply from the second fill-finish facility; 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, 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 and any results from ongoing or future clinical trials, including the Phase 2 OPAL clinical trial, the Phase 2 FORGE trial and the Phase 1 clinical trial of SRK-439, will be sufficient to support regulatory approval or further development; that preclinical and clinical data, including the results from the Phase 2 or Phase 3 clinical trial of apitegromab, data from any ongoing or future trials of apitegromab or 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 Catalent Indiana fill finish facility in response to the FDA Observations, 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 March 31, 2026, 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 March 31,	
	2026	**2025**
Operating expenses		
Research and development	$ 51,814	$ 48,678
General and administrative	50,202	28,412
Total operating expenses	102,016	77,090
Loss from operations	(102,016)	(77,090)
Other income (expense), net	(3,494)	2,367
Net loss	$ (105,510)	$ (74,723)

Net loss per share, basic and diluted	$ (0.83)	$ (0.67)
Weighted average common shares outstanding, basic and diluted	127,277,144	111,838,272

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

	March 31, 2026	December 31, 2025
Assets		
Cash, cash equivalents and marketable securities	$ 479,944	$ 367,563
Other current assets	35,340	17,584
Total current assets	515,284	385,147
Other assets	19,970	19,125
Total assets	$ 535,254	$ 404,272
Liabilities and Stockholders' Equity		
Current liabilities	$ 59,643	$ 55,419
Long-term liabilities	199,598	103,365
Total liabilities	259,241	158,784
Total stockholders' equity	276,013	245,488
Total liabilities and stockholders' equity	$ 535,254	$ 404,272

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