



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

August 6, 2026

- *Apitegromab Biologics License Application (BLA) for spinal muscular atrophy (SMA) review by FDA continues to advance with two fill-finish facilities, representing two independent paths to an FDA approval decision by September 30, 2026 Prescription Drug User Fee Act (PDUFA) date*
- *FDA review of second fill-finish facility progressing; Data package for FDA review of second fill-finish facility has been submitted; Ample supply available for commercialization upon FDA approval*
- *Scholar Rock is prepared for U.S. apitegromab launch immediately upon FDA approval, which may be granted at any time through September 30, 2026*
- *Company engaging with European Medicines Agency (EMA) on next steps for apitegromab Marketing Authorisation Application (MAA); FDA inspection classification of Catalent Indiana LLC (part of Novo Nordisk) is pending*
- *Initiated Phase 2 FORGE study evaluating apitegromab in patients with facioscapulohumeral muscular dystrophy (FSHD)*
- *Cash, cash equivalents, and marketable securities of \$492 million as of June 30, 2026; Includes \$63 million in net cash proceeds from Company's at-the-market (ATM) program*
- *Management to host a conference call today at 8:00 a.m. ET*

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Aug. 6, 2026-- Scholar Rock (NASDAQ: SRRK), a global biopharmaceutical company dedicated to improving the lives of patients with rare, severe, and debilitating neuromuscular diseases by applying its leading platform in myostatin biology, today reported financial results for the second quarter ended June 30, 2026, and provided an update on recent company developments.

"We are on the threshold of securing the world's first ever regulatory approval of a myostatin inhibitor, which will also be the first ever muscle-targeted treatment for children and adults living with SMA," said David L. Hallal, Board Chair and Chief Executive Officer of Scholar Rock. "Backed by a strong balance sheet, our Scholar Rock team is ready to usher in the next phase of innovation for the SMA community in the U.S. immediately upon apitegromab approval."

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 Biologics License Application (BLA) on track for potential FDA approval by September 30, 2026 Prescription Drug User Fee Act (PDUFA) date.** The FDA review of the apitegromab BLA is advancing with both the Catalent Indiana fill-finish facility and a second fill-finish facility, representing two independent paths to an FDA approval decision. At the March 2026 Type C meeting, FDA and Scholar Rock agreed to the data package required for FDA review of the second fill-finish facility. That data package has been submitted, and Agency review of the data is progressing well. Ample supply from the second fill-finish facility is available for commercialization upon FDA approval. The FDA inspection classification of Catalent Indiana following an April 2026 general site inspection is pending.
- **U.S. Commercial team prepared to launch apitegromab upon FDA approval.** The U.S. Commercial team remains active in the field with SMA prescribers and centers of excellence nationwide, furthering disease education and awareness initiatives. The Company had a significant presence at Cure SMA's Annual SMA Conference and Annual SMA Research & Clinical Care Meeting, which was held June 23 – 28, 2026 in Orlando, FL.
- **Scholar Rock engaging with European Medicines Agency (EMA) on next steps for apitegromab Marketing Authorisation Application (MAA).** The EMA is reviewing the apitegromab MAA, which includes the Catalent Indiana fill-finish facility, and is awaiting the FDA inspection classification of Catalent Indiana. In parallel, Scholar Rock is engaging with the EMA on next steps, including the potential to add the Company's second fill-finish facility to the MAA. The Company plans to provide updated guidance on timelines for a Committee for Medicinal Products for Human Use (CHMP) opinion upon alignment with the EMA.
- **Continued robust enrollment in Phase 2 OPAL trial.** Enrollment and dosing in the Phase 2 OPAL study is ongoing (NCT07047144). The OPAL study 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 progressing.** Scholar Rock has developed a high concentration subcutaneous formulation of apitegromab. The Company plans to engage with the FDA and EMA to align on the development path following the

regulatory approvals of apitegromab for SMA.

Facioscapulohumeral Muscular Dystrophy (FSHD) Program

- **Phase 2 FORGE trial initiated.** The Phase 2 randomized, double-blind, placebo-controlled trial, called FORGE, is expected to enroll approximately 60 patients with FSHD who will be randomized 1:1 to receive either apitegromab 10mg/kg IV or placebo every 4 weeks for 52 weeks. The primary endpoint is mean lean muscle volume (LMV) change from baseline at 12 months. Secondary endpoints include safety, pharmacokinetics/pharmacodynamics, and mean LMV change from baseline at 6 months. Several exploratory functional endpoints will also be assessed.

SRK-439

SRK-439 is a novel, investigational, subcutaneously administered myostatin inhibitor that binds to pro- and latent myostatin with high affinity and selectivity. Based on preclinical data, SRK-439 has the potential to potently inhibit myostatin and increase muscle mass.

- **Phase 1 study in healthy volunteers ongoing.** A Phase 1 study evaluating SRK-439 in healthy volunteers is progressing well with topline data anticipated in late 2026.

Second Quarter 2026 Financial Results

Scholar Rock reported a net loss of \$109.9 million, including stock-based compensation of \$19.7 million, for the quarter ended June 30, 2026, compared to a net loss of \$110.0 million, including stock-based compensation of \$24.4 million, for the quarter ended June 30, 2025. Net loss per common share was \$0.84 for the quarter ended June 30, 2026, compared to \$0.98 per common share for the quarter ended June 30, 2025.

- The Company did not record any revenue for the quarters ended June 30, 2026 and 2025.
- Research and development expense was \$58.2 million, including \$7.5 million in stock-based compensation, for the quarter ended June 30, 2026, compared to \$62.4 million, including \$5.8 million in stock-based compensation, for the quarter ended June 30, 2025.
- General and administrative expense was \$50.7 million, including \$12.2 million in stock-based compensation, for the quarter ended June 30, 2026, compared to \$49.7 million, including \$18.6 million in stock-based compensation, for the quarter ended June 30, 2025.
- As of June 30, 2026, Scholar Rock had cash, cash equivalents, and marketable securities of \$492.1 million. This reflects net cash proceeds of \$62.8 million from the Company's at-the-market (ATM) program during the quarter ended June 30, 2026.

Conference Call Information

Scholar Rock will host a conference call and webcast today, Thursday, August 6, at 8:00 a.m. ET to review its second 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 programs, 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, 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, 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 timing and outcome of EMA review and MAA approval; 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, including a second fill finish facility, 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 June 30, 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 June 30,		Six Months Ended June 30,	
	2026	2025	2026	2025
Operating expenses				
Research and development	\$ 58,234	\$ 62,401	\$ 110,048	\$ 111,079
General and administrative	50,667	49,708	100,869	78,120
Total operating expenses	108,901	112,109	210,917	189,199
Loss from operations	(108,901)	(112,109)	(210,917)	(189,199)
Other income (expense), net	(995)	2,078	(4,489)	4,445
Net loss	\$ (109,896)	\$ (110,031)	\$ (215,406)	\$ (184,754)
Net loss per share, basic and diluted	\$ (0.84)	\$ (0.98)	\$ (1.67)	\$ (1.65)
Weighted average common shares outstanding, basic and diluted	130,612,734	112,703,014	128,954,153	112,273,032

Scholar Rock Holding Corporation
Condensed Consolidated Balance Sheets
(unaudited)

(in thousands)

	June 30, 2026	December 31, 2025
Assets		
Cash, cash equivalents and marketable securities	\$ 492,088	\$ 367,563
Other current assets	15,095	17,584
Total current assets	507,183	385,147
Other assets	16,360	19,125
Total assets	<u>\$ 523,543</u>	<u>\$ 404,272</u>
Liabilities and Stockholders' Equity		
Current liabilities	\$ 72,242	\$ 55,419
Long-term liabilities	197,517	103,365
Total liabilities	269,759	158,784
Total stockholders' equity	253,784	245,488
Total liabilities and stockholders' equity	<u>\$ 523,543</u>	<u>\$ 404,272</u>

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Source: Scholar Rock