



CORRECTING and REPLACING Scholar Rock Reports Fourth Quarter and Full Year 2025 Financial Results and Recent Business Highlights

March 3, 2026

- *Apitegromab Biologics License Application (BLA) resubmission and U.S. launch, following FDA approval, are anticipated in 2026 for the treatment of children and adults with spinal muscular atrophy (SMA)*
- *FDA completed constructive meeting with Catalent Indiana, LLC (part of Novo Nordisk), with discussion of remediation progress and no additional corrective actions requested by FDA*
- *Scholar Rock plans to resubmit BLA upon successful FDA reinspection of Catalent Indiana*
- *Apitegromab Marketing Authorisation Application (MAA) review ongoing, with EMA decision anticipated in mid-2026; European launch expected in H2 2026, starting with Germany*
- *Secured new debt facility, providing up to \$550 million in non-dilutive capital to support commercialization of apitegromab and strategic advancement of key pipeline programs*
- *Cash, cash equivalents, and marketable securities of \$367.6 million as of December 31, 2025*
- *Management to host conference call today at 8:00 a.m. ET*

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Mar. 3, 2026-- In the release, the first paragraph of the Fourth Quarter and Full Year 2025 Financial Results section, second sentence should read: Net loss per common share was \$0.76 for the quarter ended December 31, 2025, compared to \$0.61 per common share for the quarter ended December 31, 2024. (instead of Net loss per common share was \$0.88 for the quarter ended December 31, 2025, compared to \$0.61 per common share for the quarter ended December 31, 2024.) In the Condensed Consolidated Statements of Operations table, Net loss per share, basic and diluted for the Three Months Ended December 31, 2025 should read: \$(0.76) (instead of \$(0.88)) and the Weighted average common shares outstanding, basic and diluted for the Three Months Ended December 31, 2025 should read: 120,449,524 (instead of 103,087,377).

The updated release reads:

SCHOLAR ROCK REPORTS FOURTH QUARTER AND FULL YEAR 2025 FINANCIAL RESULTS AND RECENT BUSINESS HIGHLIGHTS

- *Apitegromab Biologics License Application (BLA) resubmission and U.S. launch, following FDA approval, are anticipated in 2026 for the treatment of children and adults with spinal muscular atrophy (SMA)*
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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 fourth quarter and full year ended December 31, 2025, and provided an update on recent company developments.

"Our highest priority is to serve children and adults living with SMA by bringing apitegromab through the regulatory review process as quickly as possible," said David L. Hallal, Chairman and Chief Executive Officer of Scholar Rock. "To that end, we are encouraged by the FDA's continued engagement and shared sense of urgency as Novo Nordisk works expeditiously to remediate its Catalent Indiana facility. We are ready to resubmit our apitegromab BLA following successful reinspection of the site by the FDA."

Mr. Hallal continued, "As we prepare to usher in the next phase of innovation for patients with SMA, we continue to strengthen our financial position while aggressively advancing our pipeline and expect 2026 to be a transformative year for Scholar Rock."

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 spinal muscular atrophy (SMA) to demonstrate a statistically significant and clinically meaningful benefit in a pivotal Phase 3 clinical trial (SAPPHIRE).

SMA Program

- **BLA resubmission and U.S. launch, following approval, expected in 2026.** A meeting between FDA and Catalent Indiana occurred early in the first quarter of 2026. The meeting was constructive and included a discussion of Novo Nordisk's progress remediating the Catalent Indiana facility. No additional corrective actions were requested by FDA. Scholar Rock plans to resubmit the apitegromab BLA following a successful reinspection of the site.
- **U.S. commercial team preparing for launch.** The commercial team is expanding its reach and deepening relationships with key stakeholders, including SMA treatment centers and payers. The team's focus includes educating on the importance of addressing the full motor unit, which consists of the motor neuron and the muscle.
- **European Medicines Agency (EMA) regulatory review ongoing.** A decision by EMA on the apitegromab Marketing Authorisation Application (MAA) is expected in mid-2026. The European team continues to engage with key stakeholders on SMA disease awareness and education initiatives. The Company is planning for an apitegromab launch in Europe in the second half of 2026, beginning with Germany.
- **Advancing key activities at second fill-finish facility.** Technology transfer continues at a second U.S.-based fill-finish facility to strengthen supply continuity and support future commercial demand. Engineering runs are underway with additional manufacturing runs planned through the second quarter of 2026. Scholar Rock expects to submit a supplemental BLA (sBLA) for this fill-finish facility later in 2026.
- **Phase 2 OPAL clinical trial ongoing.** Enrollment and patient dosing continue in the Phase 2 OPAL study (NCT07047144). The trial is designed to evaluate 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.
- **Development activities for subcutaneous apitegromab progressing.** 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, and further development activities are ongoing, including planned FDA and EMA regulatory engagements.

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. The IND application is cleared, and the Company continues to anticipate the initiation of a Phase 2 randomized, double-blind, placebo-controlled trial, called FORGE, 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 potentially inhibit myostatin and increase muscle mass.

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

Corporate Update

- **Secured new debt facility for up to \$550 million in non-dilutive capital from funds managed by Blue Owl Capital (NYSE: OWL).** This debt facility is expected to support commercialization of apitegromab and strategic advancement of key pipeline programs. The debt facility matures in February 2032, and consists of the following:
 - \$100 million, which became available at closing and was used to retire Scholar Rock's prior debt facility with Oxford Finance;
 - An additional \$100 million to be drawn down in the first quarter of 2026;
 - Up to \$150 million available upon FDA approval of apitegromab; and
 - An option for additional incremental facilities of up to \$200 million at the mutual consent of Scholar Rock and Blue Owl Capital.

Fourth Quarter and Full Year 2025 Financial Results

Scholar Rock reported a net loss of \$91.0 million, including stock-based compensation of \$19.4 million, for the quarter ended December 31, 2025, compared to a net loss of \$66.5 million, including stock-based compensation of \$9.5 million, for the quarter ended December 31, 2024. Net loss per common share was \$0.76 for the quarter ended December 31, 2025, compared to \$0.61 per common share for the quarter ended December 31, 2024. For the full year ended December 31, 2025, Scholar Rock reported a net loss of \$377.9 million, including stock-based compensation of \$75.6 million, compared to a net loss of \$246.3 million for the year ended December 31, 2024, including stock-based compensation of \$36.6 million. Net loss per common share was \$3.29 for

the full year ended December 31, 2025, compared to \$2.47 per common share for the full year ended December 31, 2024.

- The Company did not record any revenue for the quarters ended December 31, 2025 and 2024, or for the full years ended December 31, 2025 and 2024.
- Research and development expense was \$46.9 million, including \$5.3 million in stock-based compensation, for the quarter ended December 31, 2025, compared to \$50.4 million, including \$4.0 million in stock-based compensation, for the quarter ended December 31, 2024. For the full year ended December 31, 2025, research and development expense was \$208.4 million, including \$20.7 million in stock-based compensation, compared to \$184.5 million, including \$16.0 million in stock-based compensation, for the full year ended December 31, 2024.
- General and administrative expense was \$45.0 million, including \$14.1 million in stock-based compensation, for the quarter ended December 31, 2025, compared to \$19.0 million, including \$5.5 million in stock-based compensation, for the quarter ended December 31, 2024. For the full year ended December 31, 2025, general and administrative expense was \$176.2 million, including \$54.9 million in stock-based compensation, compared to \$67.5 million, including \$20.6 million in stock-based compensation, for the full year ended December 31, 2024.
- As of December 31, 2025, Scholar Rock had cash, cash equivalents, and marketable securities of \$367.6 million. This reflects \$60.4 million from the exercise of outstanding warrants for the quarter ended December 31, 2025.

Conference Call Information

Scholar Rock will host a conference call and webcast today, Tuesday, March 3, at 8:00 a.m. ET to review its fourth quarter and full year 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, expectations regarding resubmission and timing of its BLA for apitegromab upon the successful FDA reinspection of Catalent Indiana, expectations regarding commercial launch timing in the U.S. 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, 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 Annual Report on Form 10-K for the year ended December 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.

Scholar Rock Holding Corporation
Condensed Consolidated Statements of Operations

(unaudited)

(in thousands, except share and per share data)

	Three Months Ended December 31,		Years Ended December 31,	
	2025	2024	2025	2024
Operating expenses				
Research and development	\$ 46,875	\$ 50,365	\$ 208,440	\$ 184,550
General and administrative	45,021	18,992	176,205	67,504
Total operating expenses	91,896	69,357	384,645	252,054
Loss from operations	(91,896)	(69,357)	(384,645)	(252,054)
Other income (expense), net	931	2,903	6,706	5,760
Net loss	<u>\$ (90,965)</u>	<u>\$ (66,454)</u>	<u>\$ (377,939)</u>	<u>\$ (246,294)</u>
Net loss per share, basic and diluted	<u>\$ (0.76)</u>	<u>\$ (0.61)</u>	<u>\$ (3.29)</u>	<u>\$ (2.47)</u>
Weighted average common shares outstanding, basic and diluted	120,449,524	109,520,287	114,701,154	99,838,102

Scholar Rock Holding Corporation
Condensed Consolidated Balance Sheets

(unaudited)

(in thousands)

	December 31, 2025	December 31, 2024
Assets		
Cash, cash equivalents and marketable securities	\$ 367,563	\$ 437,278
Other current assets	17,584	13,887
Total current assets	385,147	451,165
Other assets	19,125	23,757
Total assets	<u>\$ 404,272</u>	<u>\$ 474,922</u>
Liabilities and Stockholders' Equity		
Current liabilities	\$ 55,419	\$ 46,936
Long-term liabilities	103,365	59,352
Total liabilities	158,784	106,288
Total stockholders' equity	245,488	368,634
Total liabilities and stockholders' equity	<u>\$ 404,272</u>	<u>\$ 474,922</u>

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