Scholar Rock Resubmits Biologics License Application (BLA) to FDA for Apitegromab for Treatment of Children and Adults with Spinal Muscular Atrophy (SMA)

March 31, 2026

- *Apitegromab BLA resubmission includes Catalent Indiana LLC (part of Novo Nordisk) and second U.S.-based fill-finish facility, aligned with FDA guidance from March 3, 2026 Type C meeting*
- *FDA and Novo Nordisk Q1 2026 interactions resulted in Scholar Rock's alignment with FDA to resubmit apitegromab BLA prior to FDA reinspection of Catalent Indiana facility*
- *FDA and Scholar Rock Q1 2026 interactions regarding accelerated progress in qualifying a second fill-finish facility resulted in alignment with FDA to include facility in BLA resubmission*
- *Company anticipates FDA acceptance of BLA within 30 days and a review period of up to 6 months from date of resubmission, with PDUFA action date expected in late September 2026*
- *Management to host a conference call today at 8:00am ET*

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Mar. 31, 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 announced it has resubmitted the Biologics License Application (BLA) for apitegromab for the treatment of children and adults with SMA to the U.S. Food and Drug Administration (FDA).

Apitegromab is the first and only muscle-targeted therapy to demonstrate statistically significant and clinically meaningful improvements in motor function in a pivotal Phase 3 clinical trial in patients with SMA receiving treatment with an SMN-targeted therapy.

"Our apitegromab BLA resubmission marks an important step forward in our mission to bring the world's first muscle-targeted therapy to children and adults living with SMA," said David L. Hallal, Chairman and Chief Executive Officer of Scholar Rock. "We continue to be encouraged by the FDA's engagement and shared sense of urgency as we work relentlessly for the SMA community. As we execute our plans for both Catalent Indiana and our second fill-finish facility, we look forward to anticipated apitegromab approvals and launches in both the U.S. and Europe this year."

In September 2025, Scholar Rock received a Complete Response Letter (CRL) from the FDA for apitegromab related to observations identified during a routine general site inspection of Catalent Indiana, LLC (part of Novo Nordisk). The observations were not specific to apitegromab and the CRL did not cite any other approvability concerns.

Since that time, Scholar Rock, Catalent Indiana, and the FDA have engaged in several constructive and collaborative interactions, including an in-person Type A meeting in November 2025 and a meeting between Catalent Indiana and the FDA early in the first quarter of 2026. During the meeting and an FDA site visit that followed, no additional corrective actions were requested by the FDA to Novo Nordisk's remediation plan. The decision to resubmit the apitegromab BLA prior to FDA reinspection of Catalent Indiana was made in alignment with the FDA.

The apitegromab BLA resubmission also includes a second U.S.-based fill-finish facility to strengthen the apitegromab supply chain and support future growing demand across the planned global commercial footprint. Following a positive Type C meeting on March 3, 2026, Scholar Rock aligned with the FDA to include the additional facility in the BLA resubmission based on the Company's accelerated commercial fill-finish timelines.

Scholar Rock anticipates FDA acceptance of the BLA within 30 days of resubmission and a review period of up to six months from the date of resubmission. A PDUFA action date is expected in late September 2026.

Updates to the apitegromab BLA for the resubmission were limited in scope and primarily composed of a standard safety update.

In Europe, the European Medicines Agency (EMA) review of the Marketing Authorisation Application (MAA) for apitegromab is progressing well, with a decision anticipated mid-2026.

The FDA has granted Fast Track, Orphan Drug, Priority Review, and Rare Pediatric Disease designations to apitegromab for the treatment of SMA. The European Medicines Agency (EMA) has granted Priority Medicines (PRIME) and Orphan Medicinal Product designations.

Conference Call Information

Scholar Rock will host a conference call and webcast today, Tuesday, March 31, at 8:00 a.m. ET. 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 Apitegromab

Apitegromab is an investigational fully human monoclonal antibody inhibiting myostatin activation by selectively binding the pro- and latent forms of myostatin in the skeletal muscle. It is the first muscle-targeted treatment candidate in spinal muscular atrophy (SMA) to demonstrate clinical success in a pivotal Phase 3 clinical trial. Myostatin, a member of the TGFβ superfamily of growth factors, is expressed primarily by skeletal muscle cells, and the absence of its gene is associated with an increase in muscle mass and strength in multiple animal species, including humans. Scholar Rock believes that its highly selective targeting of pro- and latent forms of myostatin with apitegromab may lead to a clinically meaningful improvement in motor function in patients with SMA. The U.S. Food and Drug Administration (FDA) has granted Fast Track, Orphan Drug and Rare Pediatric Disease designations, and the European Medicines Agency (EMA) has granted Priority Medicines (PRIME) and Orphan Medicinal Product designations, to apitegromab for the treatment of SMA. Apitegromab has not been approved for any use by the FDA or any other regulatory agency.

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 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 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 review of its BLA for apitegromab, expectations regarding approval and commercial launch timing in the U.S. and in Europe, expectations regarding a new fill-finish facility and the achievement of important milestones, 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 Catalent Indiana fill-finish facility in response to the FDA observations, and whether the updated BLA will be sufficient to support regulatory approval, risks related to delays in obtaining or failure to obtain FDA clearances or approvals and noncompliance with FDA regulations, information provided or decisions made by regulatory authorities; 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.

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