Scholar Rock Provides Update on Global Apitegromab Regulatory Progress Across U.S., Europe, and Japan

August 21, 2026

- *Under FDA guidance, Scholar Rock has successfully removed Catalent Indiana LLC (part of Novo Nordisk) as a commercial fill-finish facility from apitegromab Biologics License Application (BLA) for spinal muscular atrophy (SMA); FDA review of BLA progressing well with Scholar Rock's alternate fill-finish facility*
- *Company is prepared for U.S. apitegromab launch immediately upon FDA approval, which may be granted at any time through September 30, 2026 Prescription Drug User Fee Act (PDUFA) date; robust supply of apitegromab commercial vials from alternate fill-finish facility available upon FDA approval*
- *Following Catalent Indiana Official Action Indicated (OAI) inspection classification by FDA, in alignment with European Medicines Agency (EMA), Scholar Rock has withdrawn apitegromab Marketing Authorisation Application (MAA) and will resubmit with alternate fill-finish facility to facilitate most expeditious path to Committee for Medicinal Products for Human Use (CHMP) opinion*
- *Japan Pharmaceuticals and Medical Devices Agency (PMDA) has agreed no additional clinical studies required for apitegromab regulatory submission; Scholar Rock now on track to submit apitegromab Japanese New Drug Application (JNDA) by YE 2026*

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Aug. 21, 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 world-leading platform in myostatin biology, today announced it has successfully removed Catalent Indiana LLC (part of Novo Nordisk) as a commercial fill-finish facility from its apitegromab Biologics License Application (BLA) for the treatment of spinal muscular atrophy (SMA) in the U.S., under guidance from FDA. FDA review of the BLA is progressing well with the Company's alternate fill-finish facility, and Scholar Rock continues to expect an FDA approval decision by the September 30, 2026 Prescription Drug User Fee Act (PDUFA) date.

Scholar Rock also announced that, following the August 7, 2026 customer notification that FDA classified the Catalent Indiana April 2026 general site inspection as Official Action Indicated (OAI), the Company has withdrawn its apitegromab Marketing Authorisation Application (MAA) via written procedure with the Committee for Medicinal Products for Human Use (CHMP), which concluded on August 20, 2026. Scholar Rock will remove Catalent Indiana as a commercial fill-finish facility from the apitegromab MAA and resubmit the application with the alternate fill-finish facility to facilitate the most expeditious path to a CHMP opinion.

In addition, Scholar Rock announced that it is now on track to submit a Japanese New Drug Application (JNDA) for apitegromab for the treatment of children and adults with SMA by year end 2026, following alignment with Japan's Pharmaceuticals and Medical Devices Agency (PMDA) that no additional clinical studies are required. This alignment is based on PMDA's assessment of the ability of apitegromab to meet the criteria as described in recent guidelines on core conditions for filing without Japanese clinical data.

"We are very pleased with the continued constructive and collaborative engagement we have with regulators both in the U.S. and Europe and look forward to commencing our US launch in Q3 pending FDA approval," said David L. Hallal, Board Chair and Chief Executive Officer of Scholar Rock. "In line with our ambition to serve patients with SMA in up to 50 countries around the world, we are also thrilled to announce that we now have alignment with PMDA in Japan to submit our apitegromab JNDA for the treatment of children and adults with SMA by year end. Together, our regulatory advancements reinforce our commitment to act with urgency on behalf of the SMA community globally."

In Japan, clinical studies in the Japanese population are typically required to support a JNDA filing. Scholar Rock engaged with PMDA regarding recent guidelines allowing JNDA submissions for specific orphan and rare disease drugs without domestic Japanese clinical trial data. The core conditions include a pivotal trial successfully completed outside of Japan, impractical patient recruitment due to small target patient populations, and available scientific and clinical evidence strongly indicating favorable benefit-risk profile for Japanese patients. Based on these criteria, the PMDA has agreed that an apitegromab JNDA may be filed without clinical data in Japanese patients.

Scholar Rock's alternate fill-finish facility for apitegromab is a U.S.-based facility producing numerous commercial products with a strong track record of regulatory compliance, including recent successful FDA and EMA inspections. Robust supply of apitegromab commercial vials from the alternate fill-finish facility is available to support the global launch of apitegromab following regulatory approvals.

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) 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.

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, 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 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 any regulatory authority, including the FDA, EMA or PMDA; the expected timing and outcome of FDA review of the accepted BLA for apitegromab by 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 the Company's fill-finish facility; expectations regarding commercial launch timing and readiness; expectations regarding the timing and submission of a JNDA; 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 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; whether the updated BLA, including an alternate 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, 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.

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