Scholar Rock Announces FDA Review of Apitegromab Biologics License Application (BLA) for Spinal Muscular Atrophy (SMA) to Progress with Second Fill-Finish Facility; Approval Decision Anticipated by September 30, 2026 Action Date

August 7, 2026

- *Today, August 7, 2026, Scholar Rock received customer notification from Catalent Indiana LLC (part of Novo Nordisk) that FDA classified the April 2026 site inspection of Catalent Indiana fill-finish facility as Official Action Indicated (OAI)*
- *Scholar Rock continuing to collaborate closely with FDA and under their guidance, will remove Catalent Indiana fill-finish facility from apitegromab BLA*
- *FDA review of second fill-finish facility progressing; Data package for FDA review of second fill-finish facility, as agreed to during March 2026 Type C meeting, has been submitted in advance of agreed upon timeline*
- *Robust supply of apitegromab commercial vials from second fill-finish facility available 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 Prescription Drug User Fee Act (PDUFA) date*
- *Company engaging with European Medicines Agency (EMA) on next steps to include Scholar Rock's second fill-finish facility in apitegromab Marketing Authorisation Application (MAA)*

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Aug. 7, 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 that the FDA review of the apitegromab Biologics License Application (BLA) for the treatment of children and adults with spinal muscular atrophy (SMA) is progressing with the Company's second fill-finish facility. The BLA remains on track for potential FDA approval by the September 30, 2026 Prescription Drug User Free Act (PDUFA) action date.

"We continue to be very pleased with our second fill-finish facility," said David L. Hallal, Board Chair and Chief Executive Officer of Scholar Rock. "The data package that we agreed upon with the FDA at our Type C meeting has been submitted, the FDA review of the facility is advancing, robust supply for commercialization is ready and awaiting packaging and labeling at a third-party site, and importantly, the facility has demonstrated a strong track record of compliance, including recent successful FDA and EMA inspections. As we work closely with the FDA on the final steps of the apitegromab BLA review process, we are ready to bring the world's first muscle-targeted treatment to children and adults with SMA."

Scholar Rock's apitegromab BLA submission in March 2026 included two fill-finish facilities, in alignment with FDA guidance. These facilities were Catalent Indiana LLC (part of Novo Nordisk) and a second facility, which provided two independent paths to a potential FDA approval. The Company received a customer notification from Catalent Indiana on August 7, 2026 that the FDA has classified the April 2026 site inspection of Catalent Indiana as Official Action Indicated (OAI). Scholar Rock will continue to collaborate closely with the FDA and under their guidance, will remove Catalent Indiana from the apitegromab BLA. FDA review of the apitegromab BLA will progress solely with the second fill-finish facility.

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 was submitted in advance of the agreed upon timeline, and Agency review of the data is progressing well. Robust supply from the second fill-finish facility is available, with vials from that facility on site at a third-party provider awaiting packaging and labeling for commercialization upon FDA approval.

The second fill-finish facility for apitegromab is a U.S.-based facility producing numerous commercial products and it is in good standing with FDA and EMA.

In Europe, Scholar Rock continues to engage with the European Medicines Agency (EMA) on next steps to include the Company's second fill-finish facility in the apitegromab Marketing Authorisation Application (MAA). The Company plans to provide updated timelines for a Committee for Medicinal Products for Human Use (CHMP) opinion upon alignment with the EMA.

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, 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 or EMA; 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 Catalent Indiana and a second U.S.-based fill-finish facility; expectations regarding commercial launch timing and readiness; 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; 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 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.

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