FDA Issues Complete Response Letter (CRL) for Apitegromab as a Treatment for Patients with Spinal Muscular Atrophy (SMA) Solely Related to Observations Identified at Catalent Indiana LLC Fill-Finish Facility

September 23, 2025

- *No other approvability issues cited in CRL*
- *Scholar Rock intends to resubmit the apitegromab Biologics License Application (BLA) upon resolution of Catalent Indiana LLC-related observations*

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Sep. 23, 2025-- 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 announced that the U.S. Food and Drug Administration (FDA) has issued a Complete Response Letter (CRL) for the apitegromab Biologics License Application (BLA) for the treatment of patients with spinal muscular atrophy (SMA). The CRL is related to observations identified during a routine general site inspection of Catalent Indiana LLC, a third-party fill-finish facility which was acquired by Novo Nordisk A/S in December 2024. The observations are not specific to apitegromab. The CRL did not cite any other approvability concerns, including apitegromab's efficacy and safety data or the third-party drug substance manufacturer.

The Catalent Indiana observations were discussed during Scholar Rock's second quarter 2025 earnings announcement. Catalent Indiana submitted a comprehensive response in early August 2025 to address the observations noted by the FDA. Following that submission, Catalent Indiana has continued to work rapidly to take corrective action and has kept the FDA apprised of that progress.

Upon Catalent Indiana's successful remediation of the FDA observations, Scholar Rock will resubmit the apitegromab BLA. Scholar Rock believes that the FDA will be able to act expeditiously on the application once the manufacturing issues have been resolved.

"We are continuing to work closely with Catalent Indiana on the FDA's manufacturing observations so that we can resubmit the apitegromab BLA as soon as possible," said David L. Hallal, Chairman and Chief Executive Officer of Scholar Rock. "We remain focused on working hand-in-hand with the FDA to pursue approval of the first and only muscle-targeted treatment for people living with SMA."

"While we are disappointed that the availability of a muscle-targeted treatment approach for patients with SMA has been delayed, we remain enthusiastic about the transformative potential of apitegromab," said Kenneth Hobby, President of Cure SMA. "Muscle strength and motor function are significant unmet needs for many in the SMA community and are fundamental to independence. A gain in motor function can allow someone to participate in important activities of daily living from self-care to work and social interactions, and as such, we urgently await the availability of the first-ever treatment with the potential to address the muscular component of SMA."

Apitegromab was granted Orphan Drug Designation, Rare Pediatric Disease Designation, Priority Review, and Fast Track designation, which recognizes the potential to treat a serious condition and fill an unmet medical need.

Outside of the U.S., the apitegromab marketing authorisation application (MAA) is under review by the European Medicines Agency and a decision is anticipated near mid-2026. European launch is anticipated in the second half of 2026, with Germany expected to be the first European market with patient access.

The referenced Catalent Indiana fill-finish facility located in Bloomington, Indiana is currently owned and operated by Novo Nordisk. This facility is no longer part of Catalent, Inc., and Catalent, Inc. does not operate the facility.

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 SMA
Spinal muscular atrophy (SMA) is a rare, severe, genetic neuromuscular disease. The disease is characterized by the irreversible loss of motor neurons, atrophy of the voluntary muscles of the limbs and trunk, and progressive muscle wasting that causes continuous motor function decline throughout life and can diminish the independence of both children and adults. Motor function decline in SMA patients is affected by motor neuron health and muscle responsiveness. SMN-targeted treatments are designed to prevent motor neuron loss but do not directly address muscle. It is estimated that approximately 35,000 SMA patients globally have been treated with an SMN-targeted treatment.

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, the timing of any regulatory responses and anticipated approvals, the therapeutic potential, clinical benefits and safety of any product candidates, 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, 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, whether the FDA observations related to Catalent Indiana are resolvable in a timely manner or at all, 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; 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, 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.

View source version on businesswire.com: https://www.businesswire.com/news/home/20250923756342/en/

Scholar Rock:

Investors
Laura Ekas, Ph.D.
ir@scholarrock.com
917-439-0374

Media
Molly MacLeod
media@scholarrock.com
802-579-5995

Source: Scholar Rock