



Scholar Rock Resubmits Apitegromab Marketing Authorisation Application (MAA) to European Medicines Agency (EMA) for Treatment of Children and Adults with Spinal Muscular Atrophy (SMA)

October 8, 2026

- *Apitegromab MAA resubmission includes alternate fill-finish facility in place of Catalent Indiana LLC (part of Novo Nordisk), in alignment with guidance from EMA*
- *Alternate fill-finish facility in MAA is the same site that supported September 11, 2026 U.S. FDA approval of ISEMBYLD™ (apitegromab-mstn) for the treatment of children and adults with SMA*
- *Company anticipates a Committee for Medicinal Products for Human Use (CHMP) opinion in H1 2027*

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Oct. 8, 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 leading platform in myostatin biology, today announced it has resubmitted the Marketing Authorisation Application (MAA) for apitegromab for the treatment of children and adults with spinal muscular atrophy (SMA) to the European Medicines Agency (EMA).

Informed by ongoing dialogue with the EMA, the resubmitted apitegromab MAA includes Scholar Rock's alternate fill-finish facility in place of the Catalent Indiana fill-finish facility. The alternate fill-finish facility is the same site that supported the U.S. FDA approval of ISEMBYLD (apitegromab-mstn) in September 2026.

"With the launch of ISEMBYLD now underway in the U.S., we are working with urgency to bring this first-ever muscle-targeted therapy to children and adults living with SMA in Europe," said David L. Hallal, Chairman and Chief Executive Officer of Scholar Rock. "Consistent with our ongoing dialogue with EMA, we are pleased with the expeditious resubmission of our apitegromab MAA. This marks a decisive step forward in advancing our mission to bring apitegromab to the more than 35,000 SMA patients globally who are receiving an SMN2-targeted therapy."

Apitegromab, marketed as ISEMBYLD™ in the United States, is the first and only muscle-targeted treatment to demonstrate motor function improvement in individuals with SMA currently receiving a survival motor neuron 2 (SMN2)-targeted treatment. In the Phase 3 randomized, placebo-controlled SAPPPIRE study, individuals receiving apitegromab demonstrated a robust, clinically meaningful improvement in motor function after one year of treatment while those on an SMN2-targeted treatment alone experienced a loss of motor function.

In August 2026, Scholar Rock informed the Committee for Medicinal Products for Human Use (CHMP) of the withdrawal of its previous MAA due to a lack of GMP compliance at the Catalent Indiana fill-finish facility. With the inclusion of the alternate facility in the MAA resubmission, the Company anticipates a CHMP opinion in the first half of 2027.

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 from this facility is available, with vials on site at a third-party provider awaiting packaging and labeling for commercialization upon approval from the European Commission.

About Apitegromab

Apitegromab is a 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 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. In the Phase 3 SHAPPPIRE study, apitegromab treatment resulted in clinically meaningful improvements in motor function in patients with SMA on background survival motor neuron 2 (SMN2)-targeted therapy as compared to patients receiving an SMN2-targeted therapy alone. In September 2026, the U.S. Food and Drug Administration (FDA) approved apitegromab, under the brand name ISEMBYLD™ (apitegromab-mstn) in the United States, for the treatment of SMA in adults and pediatric patients 2 years of age and older who are currently receiving a SMN2-targeted treatment. Prior to its U.S. approval, apitegromab was granted Fast Track, Orphan Drug and Rare Pediatric Disease designations from the FDA for the treatment of SMA. The European Medicines Agency (EMA) has granted Priority Medicines (PRIME) and Orphan Medicinal Product designations to apitegromab for the treatment of SMA.

About Scholar Rock

Scholar Rock is delivering muscle-targeted breakthroughs to transform the treatment of spinal muscular atrophy (SMA) and other rare neuromuscular diseases where muscle atrophy remains a critical unmet need. Following FDA approval in September 2026, Scholar Rock is commercializing ISEMBYLD (apitegromab-mstn) in the U.S. for people living with SMA who are 2 years of age and older and currently receiving a survival motor neuron 2 (SMN2)-targeted treatment. The company plans to expand access to

ISEMBYLD globally, in up to 50 countries worldwide.

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 about our neuromuscular franchise at [ScholarRock.com](https://www.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 expectations concerning the regulatory review of the resubmitted apitegromab Marketing Authorisation Application, including the anticipated timing of a CHMP opinion; the potential approval and commercialization of apitegromab in Europe and other jurisdictions; the anticipated benefits of apitegromab for patients with SMA; the ability of regulatory approval in Europe to constitute a decisive step forward in Scholar Rock’s mission to bring apitegromab to SMA patients globally; Scholar Rock’s European and global commercial strategy; and the Company’s business strategy, plans and prospects. The use of words such as “may,” “might,” “will,” “could,” “would,” “should,” “expect,” “anticipate,” “plan,” “believe,” “intend,” “estimate,” “potential,” “continue,” “target,” “goal,” and similar expressions are intended to identify such forward-looking statements, although not all forward-looking statements contain these identifying words. 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: the timing and outcome of regulatory review of the resubmitted apitegromab MAA; whether the resubmitted MAA, including the alternate fill finish facility, will be sufficient to support regulatory approval in Europe; the possibility that EMA or other regulatory authorities may require additional information, data, inspections or manufacturing changes; the Company’s ability to obtain and maintain regulatory approval of apitegromab in Europe and other jurisdictions; manufacturing and supply-chain risks, including risks related to third-party manufacturers and regulatory inspections; the Company’s ability to successfully commercialize ISEMBYLD in the United States and, if approved, apitegromab in other jurisdictions; risks related to market acceptance, competition, pricing, reimbursement and access; Scholar Rock’s ability to obtain, maintain and protect its intellectual property; and Scholar Rock’s dependence on third parties for the development and manufacture of ISEMBYLD and apitegromab, 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.

View source version on businesswire.com: <https://www.businesswire.com/news/home/20261008025279/en/>

Scholar Rock:

Investors

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

Media

Jeff Smith
682-401-8428
Molly MacLeod, Ph.D.
802-579-5995
media@scholarrock.com

Source: Scholar Rock