Scholar Rock Announces FDA Approval of ISEMBYLD™ (apitegromab-mstn), the First and Only Muscle-Targeted Treatment for Children and Adults with Spinal Muscular Atrophy (SMA)

September 11, 2026

- *ISEMBYLD is approved for use in all adults and children ≥2 years of age with SMA who are currently receiving a survival motor neuron 2 (SMN2)-targeted treatment*
- *ISEMBYLD recommended dose of 10 mg/kg showed a robust, clinically meaningful 2.2-point improvement in motor function as measured by the gold-standard Hammersmith Functional Motor Scale-Expanded (HFMSE) compared to placebo, with all patients receiving SMN2-targeted background therapy (p = 0.0121*)*
- *ISEMBYLD showed a ≥ 3-point increase in HFMSE in 34.2% of patients compared to 13.5% of patients on placebo (odds ratio 3.8; p = 0.0125*)*
- *ISEMBYLD U.S. commercial launch underway with product available to ship in the coming days; Scholar Rock Supports™ dedicated support team now available to assist patients and caregivers*
- *Management to host investor call Monday, September 14, 2026, at 8:00 a.m. ET*

** nominal p-value*

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Sep. 11, 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 that the FDA has approved ISEMBYLD (apitegromab-mstn) for the treatment of spinal muscular atrophy (SMA) in adults and children two years of age and older who are currently receiving a survival motor neuron 2 (SMN2)-targeted treatment.

SMA is a rare, severe neuromuscular disease that results in irreversible loss of motor neurons and progressive muscle wasting, causing continuous motor function decline throughout life and diminishing the independence of both adults and children.

ISEMBYLD is the first and only muscle-targeted treatment to demonstrate motor function improvement in individuals with SMA currently receiving an SMN2-targeted treatment. In the Phase 3 randomized, placebo-controlled SAPPHIRE study, individuals receiving ISEMBYLD 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.

“Today’s FDA approval of ISEMBYLD marks a defining moment for the SMA community as we now launch the world’s first-ever muscle targeted treatment for children and adults living with SMA in the U.S.,” said David L. Hallal, Chairman and Chief Executive Officer of Scholar Rock. “After decades of failed industry-wide efforts to unlock the potential of myostatin inhibition, Scholar Rock has delivered a therapeutic breakthrough with ISEMBYLD. Our U.S. commercial team is now engaging physicians, SMA care teams, and payers on behalf of the SMA community and our Scholar Rock Supports™ team stands ready to provide dedicated, comprehensive assistance to patients and caregivers. I would like to extend my deepest thanks to our clinical study investigators, Cure SMA, and other patient advocacy groups for their dedication and support on this journey. Above all, I want to express my heartfelt gratitude to the patients and families affected by SMA who participated in our clinical trials for their trust in Scholar Rock and unwavering resilience every step of the way.”

“The approval of ISEMBYLD as the first-ever treatment to directly target the muscular component of SMA is a significant turning point for adults and children who have been waiting for innovative therapeutic options to improve motor function,” said Kenneth Hobby, President of Cure SMA. “We appreciate the FDA’s recognition, as reflected in this approval that supports access for a broad population within the SMA community, that improving motor function is a significant unmet need that must be addressed with urgency. Such improvements are fundamental to maintaining independence and to enabling participation in important activities of daily living from self-care to work and social interactions.”

With the approval of ISEMBYLD, Scholar Rock was awarded a Rare Pediatric Disease Priority Review Voucher, which may be used to obtain priority review for a future marketing application.

ISEMBYLD Robust Clinical Efficacy and Safety Profile from Phase 3 SAPPHIRE Study
The approval of ISEMBYLD was based on positive results from the Phase 3 pivotal, randomized, double-blind, placebo-controlled SAPPHIRE study. The SAPPHIRE study met its primary endpoint, and demonstrated a robust, clinically meaningful 2.2-point improvement in the gold-standard Hammersmith Functional Motor Scale-Expanded (HFMSE) in patients receiving ISEMBYLD 10 mg/kg and an SMN2-targeted treatment compared to patients receiving an SMN2-targeted treatment alone at one year (nominal p

= 0.0121; main efficacy population 2 - 12 years of age, n = 103). Additionally, 34.2% of ISEMBYLD-treated patients showed a ≥ 3-point increase in HFMSE compared to 13.5% of placebo-treated patients (odds ratio of 3.8; nominal p = 0.0125).

ISEMBYLD has a well-characterized safety profile. The safety database includes more than 500 individuals across all apitegromab clinical studies globally, some of whom have been on treatment for more than 7 years. Ninety-eight percent of participants treated in SAPPHIRE elected to continue in the ONYX long-term extension study. In the SAPPHIRE study, the most common adverse reactions were upper respiratory tract infections, vomiting, cough, other viral infections, headache, gastroenteritis, pharyngitis, and hypersensitivity. Fractures occurred in 9% of patients treated with ISEMBYLD 10 mg/kg vs. 2% in placebo.

For further information, see Important Safety Information below.

"Today's approval of ISEMBYLD marks a new era for the treatment of SMA," said Dr. Basil Darras, M.D., Associate Neurologist-in-Chief, Director of the Neuromuscular Center and Spinal Muscular Atrophy Program at Boston Children's Hospital, and a principal investigator in the SAPPHIRE study. "As neurologists, families consistently tell us that their top priority is gaining motor function, and we are now able to directly target the muscle, not just the motor neuron, for people living with SMA."

ISEMBYLD Commercial Availability and Patient Access
Scholar Rock Supports is now available to assist patients who have been prescribed ISEMBYLD. This program is designed to provide personalized support for patients and families, and includes help to understand insurance coverage, financial assistance programs for eligible patients, and disease and treatment education. Scholar Rock Supports will also help patients and families navigate their site of care options, as well as ongoing infusion logistics. Based on eligibility, infusions can be given at convenient locations including hospital, home, or infusion center. ISEMBYLD will be available to ship in the coming days.

Scholar Rock is working closely with top commercial and government payers to establish reliable, broad access to ISEMBYLD for appropriate patients.

Healthcare providers and patients can learn more about Scholar Rock's patient support services by visiting www.ScholarRockSupports.com or calling 833-777-5444 (833-SRRK-444). For more information about ISEMBYLD and U.S. Prescribing Information, visit www.ISEMBYLD.com.

Conference Call Information
Scholar Rock will host a conference call and webcast on Monday, September 14, 2026, 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 https://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 ISEMBYLD
ISEMBYLD is a fully human monoclonal IgG4 antibody that binds to promyostatin and latent myostatin and inhibits the activation of myostatin, blocking myostatin signaling. ISEMBYLD is approved in the United States for the treatment of spinal muscular atrophy (SMA) in adults and pediatric patients 2 years of age and older who are currently receiving a survival motor neuron 2 (SMN2)-targeted treatment.

For more information, visit www.ISEMBYLD.com.

Indication and Important Safety Information

What is ISEMBYLD (apitegromab-mstn)?
ISEMBYLD is a prescription medicine used to treat spinal muscular atrophy (SMA) in adults and children 2 years of age and older who are currently receiving a survival motor neuron 2 (SMN2)-targeted treatment.

IMPORTANT SAFETY INFORMATION
Before taking ISEMBYLD, tell your healthcare provider about all of your medical conditions, including if you:

- have a history of low bone density or bone fractures
- are pregnant or plan to become pregnant. If you are pregnant or are planning to become pregnant, ask your healthcare provider for advice before taking this medicine. It is not known if ISEMBYLD will harm your unborn baby. Tell your healthcare provider right away if you become pregnant during treatment with ISEMBYLD
- are breastfeeding or plan to breastfeed. It is not known if ISEMBYLD passes into breast milk. Talk to your healthcare provider about the best way to feed your baby while on treatment with ISEMBYLD

Tell your healthcare provider about all the medicines you take, including prescription and over-the-counter medicines, vitamins, and herbal supplements. Keep a list of them to show your healthcare provider, including your pharmacist, when you get a new medicine.

What are the possible side effects of ISEMBYLD?

- **Fractures.** Treatment with ISEMBYLD may increase the risk of bone fractures, including serious fractures. Bone fractures may happen with or without a fall or other injury. Your healthcare provider may consider stopping treatment with ISEMBYLD if you experience a bone fracture during treatment.

The most common side effects of ISEMBYLD include:

- upper respiratory tract infections
- vomiting
- cough
- viral infections
- headache
- stomach flu (gastroenteritis)
- sore throat (pharyngitis)
- hypersensitivity

These are not all of the possible side effects of ISEMBYLD. Call your healthcare provider for medical advice about side effects.

You are encouraged to report negative side effects of prescription drugs to the FDA. Visit http://www.fda.gov/medwatch or call 1-800-FDA-1088.

Please see full Prescribing Information and Patient Information.

About the SAPPHIRE Clinical Trial
SAPPHIRE (NCT05156320) was a global, multi-national, randomized, double-blind, placebo-controlled Phase 3 clinical trial that evaluated the safety and efficacy of ISEMBYLD (apitegromab-mstn) in a total of 188 patients in 9 countries with a diagnosis of 5q SMA who were 2 to 21 years of age. Patients were randomized in a 1:1:1 ratio to receive ISEMBYLD 20 mg/kg (2 times the recommended dosage), ISEMBYLD 10 mg/kg (the recommended dosage), or placebo, respectively, via intravenous infusion once every 4 weeks for approximately 1 year. All patients enrolled in this trial were receiving an approved SMN2-targeted treatment (either nusinersen or risdiplam).

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 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. Scholar Rock intends to commercialize ISEMBYLD (apitegromab-mstn) globally, beginning in the U.S. for individuals living with SMA who are 2 years of age and older and currently receiving a survival motor neuron 2 (SMN2)-targeted treatment.

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 and follow @ScholarRock on X and on LinkedIn.

Scholar Rock® is a registered trademark and ISEMBYLD ™is a 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: Scholar Rock's expectations regarding the U.S. commercial launch of ISEMBYLD, ISEMBYLD's ability to support a broad patient population, the potential approval and launch of apitegromab in other geographies, ISEMBYLD's ability to change patient lives, the anticipated benefits of ISEMBYLD for patients with SMA, and the Company's business strategy, plans and prospects. The words "may," "might," "will," "could," "would," "should," "expect," "anticipate," "plan," "believe," "intend,"

“estimate,” “potential,” “continue,” “target,” “goal,” and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Any forward-looking statements in this press release are based on the Company’s current expectations, intentions, and beliefs regarding future events, and are subject to risks and uncertainties that could cause actual results to differ materially from those expressed or implied by such statements. These risks and uncertainties include, without limitation: the Company’s ability to successfully commercialize ISEMBYLD in the U.S.; the Company’s ability to obtain and maintain regulatory approval of apitegromab in other jurisdictions; risks related to market acceptance, competition, pricing, reimbursement and access; manufacturing and supply chain risks; 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 ISEMBYLD, 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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