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Scholar Rock Receives FDA Fast Track Designation for Apitegromab Facioscapulohumeral Muscular Dystrophy (FSHD) Program as Participant Dosing Commences in Phase 2 FORGE Trial

- *Apitegromab for FSHD granted Fast Track and Orphan Drug designations by FDA*
- *Dosing underway in Phase 2 FORGE trial; FSHD program expands Scholar Rock's global leadership in myostatin biology across rare neuromuscular diseases*
- *FORGE builds on clinically validated mechanism of apitegromab, the first and only muscle-targeted therapy to show statistically significant and clinically meaningful improvement in motor function in a Phase 3 clinical study of patients with spinal muscular atrophy (SMA) receiving an SMN-targeted therapy*
- *FSHD trial supported by favorable translational preclinical data in FSHD mouse model demonstrating increases in muscle mass, strength, and endurance*

CAMBRIDGE, Mass., September 2, 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 has granted Fast Track and Orphan Drug designations to apitegromab for the treatment of people living with FSHD. In addition, the Company announced that participant dosing is underway in the Phase 2 FORGE clinical trial evaluating apitegromab, an investigational fully human monoclonal antibody designed to inhibit myostatin activation, in people living with FSHD. FSHD is a progressive, hereditary neuromuscular disease characterized by muscle atrophy, weakness, and functional decline.

"We are very pleased to receive both Fast Track and Orphan Drug designations for our apitegromab FSHD program, which underscores the urgency of advancing new treatment options for the FSHD community," said David L. Hallal, Chairman and Chief Executive Officer of Scholar Rock. "With participant dosing now underway in our Phase 2 FORGE study, we are one step closer to realizing the broader potential of our world-leading myostatin platform and bringing a potentially transformative muscle-targeted therapy to people living with FSHD worldwide."

FORGE is a Phase 2 randomized, double-blind, placebo-controlled, multi-center clinical trial designed to evaluate the efficacy, safety, pharmacokinetics, and pharmacodynamics of apitegromab as a monotherapy in adults with genetically confirmed FSHD (**NCT07435129**).

The study will enroll approximately 60 participants randomized one-to-one to receive apitegromab 10 mg/kg or placebo intravenously every four weeks for 52 weeks. The primary endpoint is percent change from baseline in total lean muscle volume (LMV) as measured by MRI at Week 52. Secondary endpoints include percent change from baseline in total LMV at Week 24, change from baseline in additional muscle parameters such as muscle fat fraction at Weeks 24 and 52, and safety and tolerability. Additional exploratory endpoints will also be evaluated.

“Our preclinical data from the FlexDux4 model and prior literature suggest that, in people living with FSHD, anabolic stimuli can result in muscle hypertrophy and improved motor function. Apitegromab therefore holds important potential to impact this disease,” said Akshay Vaishnaw, M.D., Ph.D., President of Research and Development at Scholar Rock. “We believe FORGE, which assesses changes in lean muscle volume as well as a range of exploratory functional endpoints, will enable robust evaluation of apitegromab and inform on the drug’s potential to drive meaningful functional outcomes.”

Scholar Rock’s FSHD program is supported by translational preclinical **data**, which demonstrated that a murine form of apitegromab increased muscle mass, strength, and endurance in the gold-standard FLExDUX4 FSHD mouse model. Additional background on the Phase 2 FORGE trial design is available **here**.

Fast Track designation is granted to investigational therapies that are intended to treat serious or life-threatening conditions and have the potential to address significant unmet medical needs. The designation is intended to expedite the development and review of eligible therapies.

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 Facioscapulohumeral Muscular Dystrophy (FSHD)
Facioscapulohumeral muscular dystrophy (FSHD) is a rare, progressive, and debilitating hereditary neuromuscular disease characterized by muscle atrophy, weakness, and functional decline. The disease typically affects muscles of the face, shoulders, upper arms, trunk, and lower extremities, often resulting in impaired mobility, reduced independence, chronic pain, fatigue, and diminished quality of life. The symptoms of FSHD can vary in both presentation and severity. The diagnosed prevalence of FSHD is estimated to be 1 in 20,000 individuals, suggesting there are approximately 40,000 people living with FSHD in the United States and European Union. However, the disease is underdiagnosed. There are currently no approved therapies for the treatment of FSHD.

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.

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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 progress, timing, conduct, enrollment, design and the results of the Phase 2 FORGE clinical trial evaluating apitegromab in people living with FSHD; the therapeutic potential, clinical benefits and safety of apitegromab in FSHD, including its potential to increase muscle mass, strength and endurance; the potential of apitegromab to address a significant unmet need in FSHD; the ability of the FORGE trial and its endpoints to evaluate the biological and functional effects of myostatin inhibition and inform the potential of apitegromab in FSHD; the ability of preclinical data and clinical data with apitegromab to predict results in FSHD; estimates regarding the prevalence of FSHD, including the number of people living with diagnosed or undiagnosed FSHD in the United States, the European Union and other jurisdictions; the extent to which Scholar Rock's expansion into FSHD may expand or reinforce its leadership in myostatin biology across rare neuromuscular diseases; the potential and expected benefits associated with Fast Track and Orphan Drug designations; the ability of any product candidate to perform in humans in a manner consistent with earlier nonclinical, preclinical or clinical trial data; and the broader potential of Scholar Rock's anti-myostatin platform and product candidates across rare neuromuscular diseases. 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 the FORGE trial will enroll or progress as anticipated; whether the design and endpoints of the FORGE trial will be sufficient to evaluate the efficacy, safety, pharmacokinetics, pharmacodynamics, biological activity or functional effects of apitegromab in FSHD; whether preclinical and clinical data, including the results from the FORGE trial, will support further development or regulatory approval of apitegromab for FSHD; that nonclinical and preclinical data,

including data generated in a murine form of apitegromab in an FSHD mouse model, and clinical data of patients with SMA may not be predictive of, or may be inconsistent with, or may be more favorable than data generated in the FORGE trial or other future clinical trials; that estimates of the prevalence of FSHD and the number of people living with diagnosed or undiagnosed FSHD may prove to be inaccurate; that Fast Track and Orphan Drug designations may not result in a faster development or regulatory review process or increase the likelihood of regulatory approval; Scholar Rock's ability to provide the financial support, resources and expertise necessary to conduct the FORGE trial and develop apitegromab 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; 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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