Scholar Rock Announces FDA Clearance of IND Application to Initiate Phase 2 Proof-of-Concept Trial with Apitegromab to Treat Obesity

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- *Randomized Phase 2 proof-of-concept trial with apitegromab in patients with obesity on GLP-1 therapies on track to initiate mid-2024*
- *Study aims to show the importance of selective myostatin inhibition in preservation of lean muscle mass as part of healthy, safe weight loss management*
- *Scholar Rock is also developing SRK-439, a novel investigational selective myostatin inhibitor, optimized for the treatment of obesity*

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Jan. 23, 2024-- Scholar Rock (NASDAQ: SRRK) (the Company), a late-stage biopharmaceutical company focused on advancing innovative treatments for spinal muscular atrophy (SMA), cardiometabolic disorders, and other serious diseases where protein growth factors play a fundamental role, today announced that the U.S. Food and Drug Administration (FDA) has cleared the company's Investigational New Drug (IND) application for its Phase 2 proof-of-concept trial of apitegromab to treat obesity in patients taking a GLP-1 receptor agonist (GLP-1 RA).

The Phase 2 trial is a randomized, double-blind, placebo-controlled, multi-center study to evaluate the effect of apitegromab, a highly selective myostatin inhibitor, to safely preserve lean muscle mass as an adjunctive therapy in overweight and obese adults who are taking a GLP-1 RA. Trial initiation is on track for mid-2024, and data from the apitegromab Phase 2 trial are expected in mid-2025. In parallel, Scholar Rock is developing SRK-439, a novel investigational selective myostatin inhibitor, optimized for the treatment of obesity. The Company plans to file an IND for SRK-439 in 2025.

"The FDA's acceptance of our IND application to study apitegromab in obesity allows us to assess the effect of our highly selective myostatin inhibitor on preserving lean muscle mass, and safety and tolerability of our approach when combined with a GLP-1 RA. The IND builds on our encouraging apitegromab clinical and safety data to date and allows us to inform the development of our cardiometabolic program with SRK-439, a novel preclinical selective myostatin inhibitor optimized for development in cardiometabolic disorders," said Jay Backstrom, M.D., MPH, President and Chief Executive Officer of Scholar Rock. "Maintaining lean mass during weight loss is important to overall metabolic health, and we look forward to initiating the Phase 2 proof-of-concept trial in apitegromab to validate our differentiated approach of selectively targeting only the pro- and latent forms of myostatin to retain muscle mass."

About SRK-439

SRK-439 is a novel, preclinical, investigational myostatin inhibitor that has high in vitro affinity for pro- and latent myostatin and maintains myostatin specificity (i.e., no GDF11 or Activin-A binding), and is initially being developed for the treatment of obesity. Based on preclinical data, SRK-439 has the potential to support healthier weight management by preserving lean mass and enhancing fat mass loss. The efficacy and safety of SRK-439 have not been established and SRK-439 has not been approved for any use by the FDA or any other regulatory agency.

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 to demonstrate clinical proof of concept in spinal muscular atrophy (SMA). 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. The efficacy and safety of apitegromab have not been established and apitegromab has not been approved for any use by the FDA or any other regulatory agency.

About Scholar Rock

Scholar Rock is a biopharmaceutical company that discovers, develops, and delivers life-changing therapies for people with serious diseases that have high unmet need. As a global leader in the biology of the transforming growth factor beta (TGFβ) superfamily of cell proteins and named for the visual resemblance of a scholar rock to protein structures, the clinical-stage company is focused on advancing innovative treatments where protein growth factors are fundamental. Over the past decade, Scholar Rock has created a pipeline with the potential to advance the standard of care for neuromuscular disease, cardiometabolic disorders, cancer, and other conditions where growth factor-targeted drugs can play a transformational role.

Scholar Rock is the only company to show clinical proof-of-concept for a muscle-targeted treatment in spinal muscular atrophy (SMA). This commitment to unlocking fundamentally different therapeutic approaches is powered by broad application of a proprietary platform, which has developed novel monoclonal antibodies to modulate protein growth factors with extraordinary selectivity. By harnessing cutting-edge science in disease spaces that are historically under-addressed through traditional therapies, Scholar Rock works every day to create new possibilities for patients. Learn more about our approach at ScholarRock.com and follow @ScholarRock and on LinkedIn.

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 Twitter and LinkedIn. The information that we post on our website or on 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.

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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, results and timing of its clinical trials for apitegromab and its preclinical programs, including SRK-439, regulatory feedback including with respect to the IND submitted in connection with the planned Phase 2 trial of apitegromab in combination with GLP-1 receptor agonists in obesity, and indication selection and development timing, including the therapeutic potential, clinical benefits and safety thereof, expectations regarding timing, success and data announcements of current ongoing preclinical and clinical trials, expectations regarding 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, that preclinical and clinical data, including the results from the Phase 2 clinical trial of apitegromab in SMA 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, including, without limitation, the Phase 3 clinical trial of apitegromab in SMA or Phase 2 clinical trial of apitegromab in obesity; Scholar Rock's ability to provide the financial support, resources and expertise necessary to identify and develop product candidates on their expected timelines; the data generated from Scholar Rock's nonclinical and preclinical studies and clinical trials; 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; Scholar Rock's ability to manage expenses and to obtain additional funding when needed to support its business activities and establish and maintain strategic business alliances and new business initiatives, and the impacts of public health pandemics such as COVID-19 on business operations and expectations, 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 September 30, 2023, 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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