



Scholar Rock to Advance Antimyostatin Program to Develop Novel Therapies for Cardiometabolic Disorders

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- Company plans to expand into cardiometabolic disorders, leveraging its experience in myostatin inhibition
- Preclinical data support advancing SRK-439, a novel investigational myostatin inhibitor for the treatment of cardiometabolic disorders, with an initial focus on obesity, towards an IND submission in 2025
- To inform the development of SRK-439, Company plans to initiate a Phase 2 proof-of-concept trial of apitegromab in combination with a GLP-1 receptor agonist in obesity in 2024 with data readout expected in mid-2025
- Data from pivotal Phase 3 SAPPHIRE trial in SMA expected in Q4 2024

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Oct. 11, 2023-- Scholar Rock (NASDAQ: SRRK), a Phase 3, clinical-stage biopharmaceutical company focused on the treatment of serious diseases in which protein growth factors play a fundamental role, today announced it plans to advance SRK-439, a novel investigational myostatin inhibitor for the treatment of obesity towards an investigational new drug application (IND) submission in 2025. As part of the Company's strategy to advance the development of SRK-439, it plans to initiate a Phase 2 proof-of-concept trial with apitegromab in combination with a GLP-1 receptor agonist (GLP-1 RA) in 2024, subject to IND acceptance. Data from the clinical trial are expected in mid-2025 and will be used to inform further clinical development of SRK-439.

"As a long-standing leader in targeting myostatin with a highly selective antibody platform, we are excited to advance our cardiometabolic program. We have discovered multiple, highly selective myostatin inhibitors and see potential with SRK-439 to retain lean muscle mass, a concern that is commonly associated with weight loss strategies, including treatments currently on the market for obesity, a common, serious and costly public health issue affecting adults and children globally. As apitegromab has shown to date in spinal muscular atrophy (SMA), muscle-targeted therapies that are well tolerated have the potential to represent a new class of treatments across a wide range of diseases," said Jay Backstrom, M.D., M.P.H., President and Chief Executive Officer of Scholar Rock. "While we continue to expand the development of our pipeline, our top priority remains advancing the apitegromab program in SMA. We recently completed full enrollment of the pivotal Phase 3 SAPPHIRE trial of apitegromab in SMA, for which we expect to read out topline data in Q4 2024."

Cardiometabolic program

Muscle plays a key role in metabolic functions and energy homeostasis. Leveraging proven expertise in antimyostatin and its effect on increasing muscle mass, the Company has been developing myostatin-selective inhibitors to address cardiometabolic disorders, including obesity. Scholar Rock's platform has generated multiple antibody candidates, including apitegromab, that selectively target pro- and latent forms of myostatin. The Company believes the selectivity of these antibody candidates enables a favorable risk-benefit profile for patients with cardiometabolic disorders.

SRK-439, a novel antimyostatin antibody candidate developed by Scholar Rock, has attractive properties, including high *in vitro* affinity for pro- and latent myostatin, maintenance of myostatin specificity (i.e., no GDF11 or Activin-A binding), and robust *in vivo* efficacy in preclinical models. Studies in diet-induced obese mice showed that SRK-439 in combination with GLP-1 receptor agonist (GLP-1 RA) achieved:

- Dose-dependent reversal of lean mass loss during GLP-1 RA-mediated weight loss; and
- Enhancement of fat mass loss mediated by GLP-1 RA treatment.

"Cardiometabolic disorders are creating growing global medical challenges and contribute to the development of serious health conditions, such as cardiovascular disease, Type 2 diabetes, and certain cancers," said Mo Qatanani, Ph.D., Senior Vice President and Head of Research. "Leveraging our unique scientific platform, Scholar Rock has developed SRK-439 with a proposed mechanism of action that aims to safely maintain muscle mass, while enhancing body fat loss, thereby potentially enhancing metabolic health and potentially offering a unique opportunity to address a high unmet need in treating metabolic conditions, including obesity. We look forward to presenting preclinical data on this new asset from our pipeline at upcoming scientific conferences."

About Scholar Rock

Scholar Rock is a clinical-stage biopharmaceutical company focused on the discovery and development of innovative medicines for the treatment of serious diseases in which signaling by protein growth factors plays a fundamental role. Scholar Rock is creating a pipeline of novel product candidates with the potential to transform the lives of patients suffering from a wide range of serious diseases, including neuromuscular disorders, cancer, and fibrosis. Scholar Rock's approach to targeting the molecular mechanisms of growth factor activation enabled it to develop a proprietary platform for the discovery and development of monoclonal antibodies that locally and selectively target these signaling proteins at the cellular level. By developing product candidates that act in the disease microenvironment, Scholar Rock intends to avoid the historical challenges associated with inhibiting growth factors for therapeutic effect. Scholar Rock believes its focus on biologically validated growth factors may facilitate a more efficient development path.

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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, any development plans, strategy and progress for apitegromab and SRK-439, the timing of its clinical trials, anticipated clinical data, and therapeutic benefits for apitegromab and SRK-439, and other product candidates and indication selection and development timing, 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 apitegromab, SRK-439 and its other product candidates and its 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, expectations regarding the therapeutic potential of SRK-439 for the treatment of obesity, planned timing for an IND submission for SRK-439, that preclinical and clinical data, including the results from the Phase 2 clinical trial of apitegromab and preclinical studies of SRK-439, are not predictive of, may be inconsistent with, or more favorable than, data generated from future clinical trials of the same product candidates, including, without limitation, the Phase 3 clinical trial of apitegromab in SMA and preclinical studies of SRK-439, Scholar Rock's ability to provide the financial support, resources and expertise necessary to identify and develop product candidates on the expected timeline, the data generated from Scholar Rock's SAPHIRE Phase 3 clinical trial, information provided or decisions made by regulatory authorities, 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, 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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