Scholar Rock to Present Data from TOPAZ Ambulatory Cohort Analysis at the 2022 Muscular Dystrophy Association (MDA) Clinical & Scientific Conference

March 13, 2022

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Mar. 13, 2022-- Scholar Rock (NASDAQ: SRRK), a clinical-stage biopharmaceutical company focused on the treatment of serious diseases in which protein growth factors play a fundamental role, today announced that it will present a poster at the 2022 Muscular Dystrophy Association (MDA) Clinical & Scientific Conference (March 13 - 16, 2022).

The poster showcases data from an exploratory analysis of the ambulatory cohort in the Phase 2 TOPAZ trial evaluating apitegromab, a selective inhibitor of the activation of latent myostatin, in individuals with later-onset spinal muscular atrophy (SMA).

“We are enthusiastic about developing and investigating the therapeutic potential for apitegromab in both ambulatory as well as non-ambulatory forms of SMA,” said George Nomikos, M.D., Ph.D., Senior Vice President of Medical & Clinical Sciences and Head of the Muscle Therapeutic Area at Scholar Rock. “Hypothesis-generating insights from this analysis identify factors that may be more likely to be associated with motor function gains following apitegromab treatment in ambulatory patients with SMA.”

The TOPAZ study assessed the safety, tolerability, PK/PD and efficacy of apitegromab in Types 2 and 3 SMA. This post hoc analysis of the 12-month treatment data from the ambulatory type 3 SMA cohort explored the relationships between the efficacy of apitegromab (pooling data from both patients receiving it as monotherapy or in addition to background nusinersen) and factors such as pharmacokinetics and pharmacodynamics (PK/PD), age, body mass index, and the presence of scoliosis or joint contractures. The presence or absence of scoliosis and/or joint contractures at baseline were associated with differential responses in the Revised Hammersmith Scale (RHS) score after 12 months of treatment. Patients without scoliosis (n=12) had a mean change of +0.67 points from baseline in the RHS score, while patients with scoliosis (n=11) had a mean change of -1.45 from baseline in the RHS score. In addition, patients without joint contractures (n=10) had a mean change of +1.10 points from baseline in the RHS score, while patients with joint contractures (n=13) had a mean change of -1.46 from baseline in the RHS score.

The poster will be available in-person and virtually. Details are below:

Title: Apitegromab in SMA: An Analysis of PK/PD Relationships to Efficacy in Ambulatory Patients from the TOPAZ Trial (#72)
Presenter: Dr. Thomas Crawford, lead principal investigator of the TOPAZ trial and Co-Director, Muscular Dystrophy Association Clinic, Professor of Neurology at Johns Hopkins University
Poster Session Times:

- Sunday, March 13, 6:00 PM – 8:00 PM CST
- Monday, March 14, 10:00 AM – 8:00 PM CST
- Tuesday, March 15, 10:00 AM – 8:00 PM CST

Location: Gaylord Opryland Resort & Convention Center, Nashville, Tennessee, Ryman Hall B1-2

The Scholar Rock booth will be located in the exhibit hall (#206 and #208) and available virtually.

For conference information, visit www.mdaconference.org

About Apitegromab

Apitegromab is a selective inhibitor of the activation of myostatin and is an investigational product candidate for the treatment of patients with 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 inhibiting myostatin activation with apitegromab may promote a clinically meaningful improvement in motor function in patients with SMA. The U.S. Food and Drug Administration (FDA) has granted Fast Track (FTD), Orphan Drug (ODD) and Rare Pediatric Disease (RPD) 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 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, the Company 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. For more information, please visit www.ScholarRock.com or follow Scholar Rock on Twitter (@ScholarRock) and LinkedIn (https://www.linkedin.com/company/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 and timing of its clinical trials for apitegromab, 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 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 are not predictive of, may be inconsistent with, or more favorable than, data generated from future clinical trials of the same product candidate, including, without limitation, the Phase 3 clinical trial of apitegromab in SMA, 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 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 Annual Report on Form 10-K for the year ended December 31, 2021, 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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Source: Scholar Rock