



Scholar Rock Presents TOPAZ Interim Analysis Data for Apitegromab in Spinal Muscular Atrophy at the 2021 Muscular Dystrophy Association (MDA) Virtual Clinical & Scientific Conference

March 15, 2021

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Mar. 15, 2021-- [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 its oral and poster presentations at the 2021 Muscular Dystrophy Association (MDA) Virtual Clinical & Scientific Conference, being held March 15-18.

Both presentations will include six-month interim analysis data from the TOPAZ Phase 2 clinical trial evaluating the potential of apitegromab, a highly selective inhibitor of latent myostatin, in improving motor function in patients with Type 2 and Type 3 Spinal Muscular Atrophy (SMA). Top-line clinical data from TOPAZ for the full 12-month treatment period are expected in the second quarter of 2021.

Oral presentation details:

- **Title:** Apitegromab, a Novel High-Affinity Anti-proMyostatin Monoclonal Antibody for Treating Spinal Muscular Atrophy: Results of a Phase 2 Interim Analysis
- **Date and Time:** Thursday, March 18, 2021, 2:00-2:15 pm EST
- **Presenter:** Amy Place, PhD, MBA, MS, RD, CLT on behalf of the apitegromab development team

Poster presentation details:

- **Title:** Apitegromab, a Novel High-Affinity Anti-Promyostatin Monoclonal Antibody for Treating Spinal Muscular Atrophy: Results of a Phase 2 Interim Analysis (Poster #51)
- **Dates:** Virtual poster presentations are available to view on the virtual conference platform from March 15-18, 2021

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 TGF β 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. Scholar Rock believes the inhibition of the activation of myostatin with apitegromab may promote a clinically meaningful improvement in motor function.

A Phase 2 clinical trial in patients with Type 2 and Type 3 SMA is ongoing (NCT03921528). The U.S. Food and Drug Administration (FDA) has granted Orphan Drug Designation (ODD) and Rare Pediatric Disease (RPD) designation, and the European Commission (EC) has granted Orphan Medicinal Product Designation, to apitegromab for the treatment of SMA. The effectiveness 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, fibrosis and anemia. 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 <https://scholarrock.com/> or follow Scholar Rock on Twitter ([@ScholarRock](#)) and LinkedIn (<https://www.linkedin.com/company/scholar-rock/>).

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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, SRK-181, and other product candidates and indication selection and development timing, its cash runway, 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," "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 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, competition from third parties that are developing products for similar uses, Scholar Rock's ability to obtain,

maintain and protect its intellectual property, the success of Scholar Rock's current and potential future collaborations, including its collaboration with Gilead, Scholar Rock's dependence on third parties for development and manufacture of product candidates including 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, 2020, 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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