Scholar Rock Provides Corporate Update and Highlights Priorities for 2021

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- TOPAZ Phase 2 trial evaluating apitegromab in spinal muscular atrophy on-track to report top-line results from the 12-month treatment period in 2Q21

- SRK-181 DRAGON Phase 1 trial enrolling patients with solid tumors in Part A2; initial clinical response and safety data anticipated 2H21

- Ended 2020 with approximately $340 million in cash, cash equivalents, and marketable securities

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Jan. 11, 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 provided a corporate update and highlighted priorities for 2021.

“2020 was a transformative year for Scholar Rock with significant progress made both clinically and operationally with positive interim data from the TOPAZ trial providing initial proof-of-concept of apitegromab’s potential in SMA and showing the potential benefit of inhibiting the latent forms of growth factors,” said Tony Kingsley, President and CEO of Scholar Rock. “In 2021, we will further elucidate the potential of our product candidates through data from our TOPAZ Phase 2 trial in SMA and our DRAGON Phase 1 trial in cancer immunotherapy as well as build upon our scientific platform towards additional indications.”

2021 Expected Milestones:

Apitegromab is a highly selective inhibitor of latent myostatin activation being developed as the potential first muscle-directed therapy for the treatment of spinal muscular atrophy (SMA).

- **Top-line Efficacy and Safety Data from TOPAZ Phase 2 Trial in SMA Anticipated in 2Q21.** A total of 58 patients were enrolled across the three cohorts of the TOPAZ clinical trial with one discontinuation to date. Positive six-month interim analysis data were announced in October 2020, demonstrating apitegromab’s potential to improve motor function in patients with Type 2 and Type 3 SMA. The top-line 12-month efficacy and safety data are expected in the second quarter of 2021.

  As of January 8, 2021, 56 patients have completed the 12-month study and all 56 have opted into the extension period.

- **Identification of Second Indication for Apitegromab Planned for 2021.** With the demonstration of initial proof-of-concept from the TOPAZ interim analysis, Scholar Rock is actively evaluating multiple other disorders for which the selective inhibition of the activation of myostatin may offer therapeutic benefit.

SRK-181 is a potent and highly selective inhibitor of latent transforming growth factor beta 1 (TGFβ1) activation being developed with an aim of overcoming resistance to and increasing the number of patients who may benefit from, checkpoint inhibitor therapy.

- **Initial Clinical Response and Safety Data from the DRAGON Trial are Anticipated in the Second Half of 2021.** SRK-181 is being evaluated in the two-part DRAGON Phase 1 trial in patients with locally advanced or metastatic solid tumors exhibiting primary resistance to anti-PD-(L)1 therapy. Dose escalation in Part A of the DRAGON trial is progressing with the highest planned dose of 2400 mg now being evaluated in Part A1 (SRK-181 as a single-agent) and the 800 mg dose being currently evaluated in Part A2 (SRK-181 in combination with an approved anti-PD-(L)1 therapy).

  The DRAGON trial is anticipated to advance to Part B in the second quarter of 2021, which is expected to encompass multiple cohorts, including urothelial carcinoma, cutaneous melanoma, non-small cell lung cancer, and other solid tumors. Each cohort will enroll up to 40 patients with locally advanced or metastatic solid tumors for which they have been treated with an approved anti-PD-(L)1 therapy and have demonstrated primary resistance. Patients in Part B will be treated with SRK-181 in combination with an approved anti-PD-(L)1 therapy.

“We enter 2021 with positive momentum across our programs. We believe we’ve demonstrated the potential of our novel scientific approach to treating a wide array of disease areas for which growth factors play a critical role in disease progression,” said Ted Myles, CFO and Head of Business Operations of Scholar Rock. “Our strong balance sheet, which can fund operations into 2023, provides flexibility as we continue to invest in developing important therapies for patients in need.”

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 www.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 Quarterly Report on Form 10-Q for the quarter ended September 30, 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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