Scholar Rock Provides Corporate Update and Highlights Priorities for 2022

January 10, 2022

- Initiated Phase 3 SAPPHIRE Clinical Trial Evaluating Apitegromab in Non-Ambulatory Patients with Type 2 and Type 3 Spinal Muscular Atrophy (SMA)

- Advanced DRAGON Phase 1 Study into Part B to Evaluate Potential for SRK-181 to Overcome Checkpoint Inhibitor Resistance in Cancer Patients

- Concluded Partnership with Gilead and Regained Rights to Advanced Preclinical Assets with Multiple Distinct Pharmacological Profiles

- Ended 2021 with Approximately $253 Million in Cash, Cash Equivalents, and Marketable Securities

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Jan. 10, 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 provided recent corporate updates and highlighted upcoming priorities for its pipeline programs in 2022.

“2021 was another transformative year for Scholar Rock, with positive data from both of our clinical programs, including from the TOPAZ Phase 2 trial for apitegromab, which is being developed for the improvement of motor function in patients with SMA; and Part A of the DRAGON Phase 1 proof-of-concept trial for SRK-181, being developed to overcome resistance to check point inhibitor therapy in cancer patients,” said Nagesh Mahanthappa, Ph.D., Interim CEO of Scholar Rock. “In 2022, we are thrilled to be advancing a pivotal Phase 3 trial of apitegromab and advancing our SRK-181 program to test our hypothesis that this highly selective and potent molecule can overcome resistance to checkpoint inhibitors thereby increasing the number of patients who may benefit from cancer immunotherapy. In addition, the preclinical pipeline has received a major boost as we have regained rights to assets discovered and developed during our research partnership with Gilead that have novel pharmacological profiles relevant to TGFβ-mediated diseases.”

2022 Priorities:

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

- **Robust Enrollment of the Phase 3 SAPPHIRE Trial Evaluating Apitegromab in Patients with Non-Ambulatory Type 2 and 3 Patients.** Scholar Rock has initiated the SAPPHIRE study. The study design plans for approximately 156 patients aged 2-12 years old with non-ambulatory Type 2/3 SMA to be enrolled in the main efficacy population. Patients will be randomized 1:1:1 to receive for 12 months either apitegromab 10 mg/kg, apitegromab 20 mg/kg, or placebo by intravenous (IV) infusion every 4 weeks added on top of background SMN treatment.
- **Progress TOPAZ Long-Term Extension to Two Year Readout.** As of January 6, 55 of 57 patients remain in the long-term extension trial of apitegromab in Type 2 and 3 SMA.
- **Advance Development Activities to Include Patients with Type 1 and Ambulatory SMA.**

SRK-181 is a potent and highly selective inhibitor of latent TGFβ1 activation being developed with the aim of overcoming primary resistance to and increasing the number of patients who may benefit from checkpoint inhibitor therapy.

- **Advance Progress in Part B of DRAGON Phase 1 Proof-of-Concept Trial.** Based on the safety and pharmacokinetic data from Part A of the DRAGON Phase 1 trial, Scholar Rock has initiated the Part B dose expansion portion of the trial, which is evaluating SRK-181 dosed 1500 mg every three weeks (Q3W) in patients receiving an approved anti-PD-(L)1 therapy dosed Q3W and 1000 mg every two weeks (Q2W) in patients receiving an approved anti-PD-(L)1 therapy dosed Q2W. Part B will enroll and dose patients in multiple proof of concept cohorts conducted in parallel, including;
    - Urothelial carcinoma (UC),
    - Cutaneous melanoma (MEL),
    - Non-small cell lung cancer (NSCLC),
    - Clear cell renal cell carcinoma (ccRCC),
    - Other solid tumors.

  Each cohort is expected to enroll up to 40 patients with various locally advanced or metastatic solid tumors who have demonstrated primary resistance to anti-PD-(L)1 therapy. Early efficacy and safety data are anticipated in 2022.

Advancing assets gained from the Gilead collaboration. In December 2018**,** Gilead Sciences and Scholar Rock entered into a three-year collaboration to discover and develop therapeutics that target TGFβ-driven signaling, a central regulator of fibrosis. Under the collaboration, Gilead had exclusive options to license worldwide rights to antibodies from certain TGFβ programs being developed by Scholar Rock. Scholar Rock received $80.0 million in proceeds upon signing the agreement and an additional $25.0 million preclinical milestone was achieved in December 2019 for the successful demonstration of efficacy in preclinical in vivo proof-of-concept studies. As of December 19, 2021 the collaboration period has concluded and on January 6, 2022, Gilead agreed that its option exercise period for all programs has been terminated.

- Scholar Rock regains rights to a suite of antibodies with novel pharmacological profiles that were discovered over the course of the collaboration.
- Of particular note, Scholar Rock has discovered antibodies that selectively inhibit the activation of latent TGFβ1 in the context of fibrotic extracellular matrix and that avoid perturbing TGFβ1 presented by cells of immune system. Such antibodies demonstrated significant antifibrotic activity in a variety of preclinical rodent models and safety at all doses tested in a non-GLP mouse safety study that we intend to publish in 2022.

“The novel anti-fibrotic antibodies discovered during this collaboration demonstrate the unique capabilities of the discovery platform we have built at Scholar Rock,” said Gregory Carven, CSO of Scholar Rock. “We are excited to continue the advancement of these assets as a part of the company’s growing preclinical pipeline.”

“We made great progress across our portfolio in 2021 and we’re carrying that momentum into 2022,” said Ted Myles, CFO and Head of Business Operations of Scholar Rock. “We recently strengthened our balance sheet through the use of our ATM and taking the $25 million second tranche of our debt facility with Silicon Valley Bank and Oxford Finance so that we have greater flexibility to continue to advance our clinical and pre-clinical programs. We have high conviction in our platform based on the exciting clinical data to date and we believe this puts us in a unique position as we advance our programs to serve patients’ needs.”

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/).

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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, 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, without limitation, that preclinical and clinical data, including the results from the Phase 2 trial of apitegromab, or Part A of the DRAGON clinical trial for SRK-181, 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 trial of apitegromab in SMA or the Phase 1 trial of SRK-181, 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 Quarterly Report on Form 10-Q for the quarter ended September 30, 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

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