# Scholar Rock Reports Full Year 2020 Financial Results and Highlights Business Progress

March 9, 2021

*- TOPAZ Phase 2 interim data demonstrated proof-of-concept for apitegromab in patients with Type 2 and Type 3 spinal muscular atrophy (SMA); 12-month top-line results on-track for 2Q21*

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

*- Announced issuance of new U.S. patent providing protection for broad therapeutic use of apitegromab*

*- Ended 2020 with approximately $341 million in cash, cash equivalents, and marketable securities*

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Mar. 9, 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 reported financial results for the full year ended December 31, 2020 and highlighted recent progress and upcoming milestones for its pipeline programs.

“2021 is poised to further validate the therapeutic potential of our scientific platform, and we are starting the year with strong momentum as we continue to execute and advance our clinical trials in both SMA and immuno-oncology,” said Tony Kingsley, President and CEO of Scholar Rock. “All patients in the TOPAZ trial in SMA have now completed the 12-month treatment period and we are on-track to report top-line results next quarter. We also look forward to clinical read-outs from our SRK-181 DRAGON trial in cancer immunotherapy, along with continued progress with our earlier-stage programs.”

**Company Updates and Upcoming Milestones**

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

- **Top-Line Efficacy and Safety Data from the TOPAZ Phase 2 Trial Expected in 2Q21.** A total of 58 patients were enrolled across the three cohorts of the TOPAZ clinical trial (NCT03921528). With the exception of one patient who discontinued early from the trial for reasons unrelated to the study drug, all patients completed the 12-month treatment period and have elected to opt into the extension period. Top-line data from the 12-month treatment period will provide additional insights on apitegromab’s potential in improving motor function in patients with Type 2 and Type 3 SMA, including the potential for durability of effect and for further motor function increases observed from the 6-month interim analysis.
- **U.S. Patent Issued Providing Broad Therapeutic Use of Apitegromab.** The United States Patent Office (USPTO) has issued U.S. Patent 10,882,904 with an expiry of September 2036. The issued claims are broadly directed to the use of apitegromab to achieve two or more of the following therapeutic effects: increase muscle mass or function, decrease adipose-to-muscle ratios, decrease intramuscular fat infiltration, and prevent muscle loss or atrophy, without limiting to specific indications.
- **Identification of Second Indication for Apitegromab Planned for 2021.** Scholar Rock is evaluating multiple other diseases 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 TGFβ1 activation being developed with the aim of overcoming resistance to and increasing the number of patients who may benefit from checkpoint inhibitor therapy.*

- **Presented Preclinical Data at the TGFβ for Immuno-Oncology Drug Development Summit.** In January 2021, Scholar Rock participated in a panel discussion titled “Debating the Best Approach to Target TGF-β” and presented “Inhibition of TGFβ1 Activation with SRK-181 Overcomes Primary Resistance to Checkpoint Inhibition Therapy” at the TGFβ for Immuno-Oncology Drug Development Summit. The presentation provided an overview of the preclinical data demonstrating that combination treatment with a murine version of SRK-181 and an anti-PD-1 therapy led to tumor regression and an improved preclinical toxicity profile compared to less selective TGFβ inhibition.

- **DRAGON Phase 1 Trial Anticipated to Advance to Part B Dose Expansion in 2Q21.** SRK-181 is being evaluated in the two-part DRAGON trial (NCT04291079) in patients with locally advanced or metastatic solid tumors exhibiting primary resistance to anti-PD-(L)1 therapy. The main goals of Part A of the trial are to evaluate the safety and pharmacokinetics of SRK-181 and to determine the dosing regimen to be evaluated in Part B of the trial. As of March 8, 2021, dose escalation in Part A1 (SRK-181 as a single-agent) has progressed beyond the previously announced highest planned dose of 2400 mg every 3 weeks (Q3W) to 3000 mg Q3W to further characterize the upper bounds of the dose range, as permitted by

the protocol. Dose escalation in Part A2 of the trial (SRK-181 in combination with an approved anti-PD-(L)1 therapy) has progressed to 1600 mg Q3W.

The Part B dose expansion portion of the trial will consist of 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.

Initial clinical response and safety data from the DRAGON trial are expected in the second half of 2021. The ongoing COVID-19 pandemic may impact the enrollment and dosing of patients in the trial.

**Full Year 2020 Financial Results**

For the full year ended December 31, 2020, net loss was $86.5 million or $2.81 per share compared to a net loss of $51.0 million or $1.85 per share for the year ended December 31, 2019.

- Revenue was $15.4 million for the year ended December 31, 2020 compared to $20.5 million for the year ended December 31, 2019. Revenue was related to the Gilead fibrosis-focused collaboration that was executed in December 2018.
- Research and development expense was $74.1 million for the year ended December 31, 2020 compared to $54.2 million for the year ended December 31, 2019. The increase year-over-year was primarily attributable to costs associated with the apitegromab TOPAZ Phase 2 clinical trial, including clinical drug supply manufacturing, the SRK-181 DRAGON Phase 1 clinical trial, and higher personnel-related costs.
- General and administrative expense was $28.2 million for the year ended December 31, 2020 compared to $20.8 million for the year ended December 31, 2019. The increase year-over-year was primarily attributable to higher personnel-related costs and professional services.

As of December 31, 2020, Scholar Rock had cash, cash equivalents, and marketable securities of approximately $341.0 million.

**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, 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.

**Scholar Rock Holding Corporation**
**Condensed Consolidated Statements of Operations**
(unaudited)
(in thousands, except share and per share data)

| | Years Ended December 31 | |
|---|---|---|
| | **2020** | **2019** |
| Revenue | $ 15,403 | $ 20,492 |
| Operating expenses | | |
| Research and development | 74,062 | 54,217 |
| General and administrative | 28,219 | 20,817 |
| Total operating expenses | 102,281 | 75,034 |
| Loss from operations | (86,878) | (54,542) |
| Other income (expense), net | 395 | 3,542 |
| Net loss | $ (86,483) | $ (51,000) |
| Net loss per share, basic and diluted | $ (2.81) | $ (1.85) |
| Weighted average common shares outstanding, basic and diluted | 30,734,109 | 27,537,939 |

**Scholar Rock Holding Corporation**
**Condensed Consolidated Balance Sheets**
(unaudited)
(in thousands)

| | **December 31, 2020** | **December 31, 2019** |
|---|---|---|
| **Assets** | | |
| Cash, cash equivalents and marketable securities | $ 341,031 | $ 157,448 |
| Other current assets | 3,373 | 27,719 |
| Total current assets | 344,404 | 185,167 |
| Other assets | 43,901 | 11,214 |
| Total assets | $ 388,305 | $ 196,381 |
| **Liabilities and Stockholders' Equity** | | |
| Current liabilities | $ 42,564 | $ 32,814 |
| Long-term liabilities | 84,971 | 50,666 |
| Total liabilities | 127,535 | 83,480 |
| Total stockholders' equity | 260,770 | 112,901 |
| Total liabilities and stockholders' equity | $ 388,305 | $ 196,381 |

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