Scholar Rock Reports Full Year 2021 Financial Results and Highlights Business Progress

March 7, 2022

- Progressing Phase 3 SAPPHIRE clinical trial evaluating apitegromab in patients with non-ambulatory Type 2 and 3 Spinal Muscular Atrophy (SMA)

- Two-year data from open label extension of TOPAZ Phase 2 trial in SMA expected by mid-2022

- Data from DRAGON Part B proof-of-concept trial evaluating SRK-181 to overcome checkpoint inhibitor resistance in cancer patients anticipated in 2022

- Ended 2021 with approximately $253 million in cash, cash equivalents, and marketable securities

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Mar. 7, 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 reported financial results for the full year ended December 31, 2021, and highlighted recent progress and upcoming milestones for its pipeline programs.

"We are excited by the momentum and engagement around our ongoing Phase 3 SAPPHIRE trial of apitegromab in spinal muscular atrophy, as well as our DRAGON Phase 1 trial of SRK-181 in solid tumors. These programs have significant potential to address unmet patient need in a differentiated way, as well as demonstrate the tremendous potential of Scholar Rock's platform, which focuses on inhibiting the activation of latent growth factors," said Nagesh Mahanthappa, Interim Chief Executive Officer of Scholar Rock. "While we progress these clinical programs, we also continue to build upon our world-leading expertise in selectively targeting the activation mechanisms of the TGFβ-superfamily growth factors and advance our preclinical pipeline focused on diseases such as fibrosis and regulation of iron metabolism. We look forward to providing more details about these programs at scientific meetings and through publications in the coming months."

Company Updates and Upcoming Milestones

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).*

- **Conducting SAPPHIRE Phase 3 Clinical Trial Evaluating Apitegromab in Patients with Non-Ambulatory Type 2 and 3 SMA.** The randomized, double-blind, placebo-controlled Phase 3 clinical trial is evaluating apitegromab as add-on therapy for patients on either nusinersen or risdiplam. The study design plans for approximately 156 patients aged 2-12 years old with non-ambulatory Type 2 and 3 SMA to be enrolled in the main efficacy population. Patients will be randomized 1:1:1 to receive for 12 months either apitegromab 20 mg/kg, apitegromab 10 mg/kg, or placebo by intravenous (IV) infusion every 4 weeks in addition to background SMN treatment.
- **Data from TOPAZ Phase 2 Open Label Extension Trial Expected to be Presented by mid-2022.** As of February 28, 2022, 55 of 57 patients remain enrolled in the long-term extension trial of apitegromab in Type 2 and Type 3 SMA.

SRK-181 *is a 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.*

- **Advancing Part B of the DRAGON Phase 1 POC Trial for SRK-181.** Based on the safety and pharmacokinetic data from Part A of the DRAGON Phase 1 trial, Scholar Rock is conducting the Part B dose expansion portion of the trial, in which SRK-181 is dosed at 1500 mg every three weeks (Q3W) in patients receiving an approved anti-PD-(L)1 therapy dosed Q3W, or 1000 mg every two weeks (Q2W) in patients receiving an approved anti-PD-(L)1 therapy dosed Q2W. Part B consists of multiple proof-of-concept cohorts focused upon evaluating the ability of SRK-181 to overcome primary resistance to anti-PD-(L)1 therapy. Each cohort will enroll up to 40 patients with various solid tumors, including urothelial carcinoma (UC), cutaneous melanoma (MEL), non-small cell lung cancer (NSCLC), clear cell renal cell carcinoma (ccRCC), and other solid tumors. The biomarker strategy in part B of DRAGON will explore early signs of SRK-181 activity, including target engagement and pathway modulation. This will include measuring effects on both circulating and tumor immune contexture, such as $CD8^+$ T cell infiltration and reductions in myeloid-derived suppressor cell (MDSC) populations as well as analysis of TGFβ-related pathway signaling. Early efficacy and safety data are anticipated in 2022.

Full Year 2021 Financial Results

For the full year ended December 31, 2021, net loss was $131.8 million or $3.59 per share compared to a net loss of $86.5.0 million or $2.81 per share for the year ended December 31, 2020.

- Revenue was $18.8 million for the year ended December 31, 2021 compared to $15.4 million for the year ended December 31, 2020. Revenue was related to the Gilead fibrosis-focused research collaboration, which concluded in December 2021.

- Research and development expense was $108.5 million for the year ended December 31, 2021 compared to $74.1 million for the year ended December 31, 2020. The increase year-over-year was primarily attributable to planned spend associated with apitegromab development, including costs associated with clinical drug supply manufacturing and costs associated with our SAPPHIRE trial, as well as higher personnel-related costs.
- General and administrative expense was $40.3 million for the year ended December 31, 2021 compared to $28.2 million for the year ended December 31, 2020. The increase year-over-year was primarily attributable to professional services and higher personnel-related costs, including filling key positions essential to progressing research, development and pre-commercial activities.

As of December 31, 2021, Scholar Rock had cash, cash equivalents, and marketable securities of approximately $253.0 million, which is expected to fund the Company's operations into mid-2023. "Continued operational excellence is an important priority for us in 2022 as we advance several programs across the continuum of drug development. As we continue to progress our SMA program with our Phase 3 SAPPHIRE trial, we are also encouraged that so many of the patients who participated in the TOPAZ study are still opting to receive apitegromab. We look forward to obtaining further insights into longer-term treatment outcomes and sharing that data in the coming months. In our immuno-oncology program, the DRAGON study has entered Part B and could produce meaningful data later this year and our research colleagues are pushing forward on several fronts as we continue to generate exciting programs from our scientific platform," said Ted Myles, COO and CFO of Scholar Rock.

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.

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," "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, or Part A of the Phase 1 clinical trial of 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 clinical trial of apitegromab in SMA or Part B of the Phase 1 clinical 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 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.

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

	Years Ended December 31

	2021	2020
Revenue	$ 18,816	$ 15,403
Operating expenses		
Research and development	108,468	74,062
General and administrative	40,269	28,219
Total operating expenses	148,737	102,281
Loss from operations	(129,921)	(86,878)
Other income (expense), net	(1,878)	395
Net loss	$ (131,799)	$ (86,483)
Net loss per share, basic and diluted	$ (3.59)	$ (2.81)
Weighted average common shares outstanding, basic and diluted	36,711,833	30,734,109

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

	December 31, 2021	December 31, 2020
Assets		
Cash, cash equivalents and marketable securities	$ 252,994	$ 341,031
Other current assets	12,325	3,373
Total current assets	265,319	344,404
Other assets	39,126	43,901
Total assets	$ 304,445	$ 388,305
Liabilities and Stockholders' Equity		
Current liabilities	$ 64,297	$ 42,564
Long-term liabilities	68,074	84,971
Total liabilities	132,371	127,535
Total stockholders' equity	172,074	260,770
Total liabilities and stockholders' equity	$ 304,445	$ 388,305

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