Scholar Rock Reports Third Quarter 2020 Financial Results and Highlights Business Progress

November 9, 2020

- Positive interim analysis data from the TOPAZ Phase 2 trial demonstrated proof-of-concept for apitegromab (SRK-015) in patients with Type 2 and Type 3 Spinal Muscular Atrophy

- Progress update on dose escalation in Part A of the SRK-181 DRAGON Phase 1 trial to be presented at the Society for Immunotherapy of Cancer (SITC) Congress; no dose-limiting toxicities observed as of the data cutoff

- Raised $230 million in gross proceeds through a public offering to advance apitegromab, SRK-181 and preclinical programs

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Nov. 9, 2020-- 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 third quarter ended September 30, 2020 and highlighted recent progress and upcoming milestones for its pipeline programs.

“We achieved a significant milestone with the TOPAZ interim analysis proof-of-concept results. The data support apitegromab’s potential to improve motor function for patients with Type 2 and Type 3 SMA and importantly, highlight the therapeutic potential of our platform targeting the latent forms of growth factors, which includes SRK-181 in immuno-oncology,” said Tony Kingsley, President and CEO of Scholar Rock. “As we look to the remainder of 2020 and into 2021, I am very excited for the potential of the company as we continue to advance and expand our pipeline across a wide range of serious diseases where growth factors may play a role, including other neuromuscular disorders, cancer, and fibrosis.”

Company Updates and Upcoming Milestones

Apitegromab (SRK-015) Program for Spinal Muscular Atrophy (SMA):

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

- **Six-Month Interim Data from TOPAZ Demonstrate Meaningful Potential for Apitegromab to Improve Motor Function for Patients with SMA.** In October 2020, Scholar Rock announced positive proof-of-concept data from a pre-planned six-month interim analysis of the TOPAZ Phase 2 trial, which enrolled 58 patients with Type 2 and Type 3 SMA across three parallel cohorts. Key findings included:
    - Treatment with apitegromab led to improvements in the Hammersmith scale scores (primary efficacy endpoint that measures motor function) in all three cohorts of patients with Type 2 and Type 3 SMA.
    - Substantial proportion of patients in each cohort attained ≥3-point improvement in Hammersmith scores.
    - Dose response in the primary efficacy endpoint was observed in the randomized, double-blind Cohort 3, with apitegromab high dose (20 mg/kg) attaining a 5.6 point mean improvement at six-months over baseline compared to low dose (2 mg/kg) attaining a 2.4 point mean improvement over baseline.
    - Pharmacokinetic and pharmacodynamic data supported the clinically observed dose response; apitegromab high dose (20 mg/kg) yielded higher levels of drug exposure and target engagement than low dose (2 mg/kg).
    - No safety signals were identified from the interim analysis. The five most frequently reported treatment-emergent adverse events (TEAEs) were headache, upper respiratory tract infection, pyrexia, nasopharyngitis, and cough.
    - Detailed interim analysis results from the TOPAZ trial were issued on October 27, 2020: *Scholar Rock Announces Positive Proof-of-Concept Data from TOPAZ Phase 2 Trial Interim Analysis of SRK-015 in Patients with Type 2 and Type 3 Spinal Muscular Atrophy*.

  Top-line data for the 12-month treatment period are expected in the second quarter of 2021. Twelve-month results could provide additional insights on the potential durability of effect and the potential for further improvements in motor function, as well as additional safety data. There may be impacts on the timing of future doses and assessments for patients in the trial as the effects of the COVID-19 pandemic continue to evolve.

  As of November 5, 2020, 45 of 45 patients who have completed the 12-month study have opted into the extension period.

- **Product candidate, SRK-015, now referred to as apitegromab.** The United States Adopted Names (USAN) Council has approved the use of the non-proprietary name apitegromab for SRK-015, the Company’s product candidate for the treatment of patients with SMA. The USAN Council is responsible for selecting simple, informative and unique non-proprietary (generic) drug names and establishes logical nomenclature classifications based on pharmacological and/or chemical relationships. The council consists of a Food and Drug Administration (FDA) liaison, one member-at-large,

and one representative from each of the following: The American Medical Association (AMA), United States Pharmacopeia (USP) and the American Pharmacists Association (APhA).

SRK-181 Program for Immuno-Oncology:

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

- **Progress Update on Dose Escalation in Part A of the DRAGON Phase 1 Trial to be Presented at the Society for Immunotherapy of Cancer (SITC) Annual Meeting.** A poster presentation titled: "*DRAGON: Phase 1 trial of SRK-181, a latent TGFβ1 inhibitor in combination with anti-PD-(L)1 inhibitors for patients with solid tumors unresponsive to anti-PD-(L)1 therapy alone,*" is being presented at the SITC congress being held November 9-14, 2020. The poster outlines the dose escalation scheme and provides an update on progress of patient enrollment and advancement in dose levels. As of October 1, 2020, eight patients have been dosed in Part A1 of the trial, which evaluates SRK-181 as a monotherapy. No dose-limiting toxicities had been observed up to the 800 mg dose and the 1600 mg dose is currently under evaluation. Part A2 of the trial, which evaluates SRK-181 in combination with an approved anti-PD-(L)1 therapy, has enrolled two patients at the 240 mg dose as of October 1, 2020.

  The two-part DRAGON Phase 1 clinical trial consists of a dose escalation portion (Part A) and a dose expansion portion (Part B) to evaluate SRK-181 in combination with an approved anti-PD-(L)1 therapy in patients with locally advanced or metastatic solid tumors exhibiting primary resistance to that anti-PD-(L)1 therapy. Part B will encompass multiple cohorts that are expected to include urothelial carcinoma, cutaneous melanoma, non-small cell lung cancer, and other solid tumors. Scholar Rock anticipates several updates from the DRAGON trial over the next year, including updates on dose escalation, an advancement to Part B that is expected in the first quarter of 2021, and clinical response and safety data in the second half of 2021. There may be impacts on the enrollment rate and dosing of patients in the trial as the effects of the COVID-19 pandemic continue to evolve.

- **Presentation of Preclinical TGFβ1 Data at the Festival of Biologics, World Immunotherapy Congress 2020.** On November 5, 2020, Scholar Rock presented previously announced preclinical data at the virtual Festival of Biologics. The presentation titled: "Selective inhibition of TGFβ1 activation overcomes primary resistance to checkpoint inhibition therapy" detailed the potential of a potent and select inhibitor of latent TGFβ1 activation, such as SRK-181, in overcoming the dose-limiting toxicities associated with non-selective approaches to targeting TGFβ. In mouse tumor models that recapitulate key features of primary resistance to checkpoint inhibitor therapy, combination treatment with SRK-181-mIgG1 (murine version of SRK-181) and an anti-PD-1 therapy resulted in tumor regression or control and survival benefit.

Third Quarter 2020 Financial Results

For the quarter ended September 30, 2020, net loss was $23.6 million or $0.79 per share compared to a net loss of $16.1 million or $0.55 per share for the quarter ended September 30, 2019.

- Revenue was $3.0 million for the quarter ended September 30, 2020 compared to $4.8 million for the quarter ended September 30, 2019. Revenue was related to the Gilead fibrosis-focused collaboration that was executed in December 2018.
- Research and development expense was $18.4 million for the quarter ended September 30, 2020 compared to $15.7 million for the quarter ended September 30, 2019. The increase year-over-year is primarily attributable to costs associated with the apitegromab TOPAZ Phase 2 clinical trial, including clinical drug supply manufacturing, as well as higher personnel-related costs.
- General and administrative expense was $8.3 million for the quarter ended September 30, 2020 compared to $6.2 million for the quarter ended September 30, 2019. The increase year-over-year was primarily attributable to higher personnel-related costs.

As of September 30, 2020, Scholar Rock had cash, cash equivalents, and marketable securities of $116.3 million. On October 19, 2020, Scholar Rock announced the closing of a $50 million debt facility, of which $25 million was funded at closing. On November 2, 2020, Scholar Rock announced the closing of a public offering of common stock and prefunded warrants and full exercise of the underwriters' option to purchase additional shares of common stock. The aggregate gross proceeds to Scholar Rock from this offering were $230 million, before deducting underwriting discounts and commissions and other offering expenses.

"With the recent public offering and the addition of the debt facility, we have significantly strengthened our balance sheet and extended our cash runway into 2023," said Ted Myles, CFO and Head of Business Operations of Scholar Rock. "We are fully investing in our clinical product candidates, apitegromab and SRK-181, as well as our preclinical programs and the underlying scientific platform to develop 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 June 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.

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

	Three Months Ended September 30		Nine Months Ended September 30	
	2020	2019	2020	2019
Revenue	$ 3,037	$ 4,774	$ 11,967	$ 12,919
Operating expenses				
Research and development	18,383	15,699	52,282	40,153
General and administrative	8,272	6,181	20,459	14,961
Total operating expenses	26,655	21,880	72,741	55,114
Loss from operations	(23,618)	(17,106)	(60,774)	(42,195)
Other income (expense), net	57	959	862	2,768
Net loss	$ (23,561)	$ (16,147)	$ (59,912)	$ (39,427)
Net loss per share, basic and diluted	$ (0.79)	$ (0.55)	$ (2.02)	$ (1.46)
Weighted average common shares outstanding, basic and diluted	29,779,114	29,232,158	29,665,995	26,929,215

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

	September 30, 2020	December 31, 2019
Assets		
Cash, cash equivalents and marketable securities	$ 116,264	$ 157,448
Other current assets	3,117	27,719
Total current assets	119,381	185,167
Other assets	44,040	11,214
Total assets	$ 163,421	$ 196,381
Liabilities and Stockholders' Equity		
Current liabilities	$ 32,869	$ 32,814
Long-term liabilities	67,575	50,666
Total liabilities	100,444	83,480
Total stockholders' equity	62,977	112,901
Total liabilities and stockholders' equity	$ 163,421	$ 196,381

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