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

August 7, 2020

- On track to report interim efficacy and safety results from TOPAZ Phase 2 clinical trial of SRK-015 in patients with Type 2 and Type 3 Spinal Muscular Atrophy (SMA) in 4Q20

- Enrollment expected to advance to combination treatment of SRK-181 and an approved anti-PD-(L)1 therapy in Part A of DRAGON Phase 1 clinical trial in 3Q20; update on dose escalation expected in 4Q20

- Appointed Tony Kingsley as President and CEO and Ted Myles as CFO and Head of Business Operations; highly accomplished leaders with proven track records of growing and advancing biopharma companies

- Company hosting conference call at 8am ET to discuss financial results and introduce newest members of management

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Aug. 7, 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 second quarter ended June 30, 2020 and highlighted recent progress and upcoming milestones for its pipeline programs.

“I am impressed by the execution of the team, and momentum is rapidly building for our lead clinical programs, SRK-015 in SMA and SRK-181 in immuno-oncology, towards several potential value-creation opportunities in late 2020 and throughout 2021,” said Tony Kingsley, President and CEO of Scholar Rock. “Scholar Rock has an exceptional proprietary platform that has yielded multiple clinical candidates that have the potential to transform the lives of patients living with serious diseases, and I look forward to seeing the company continue to drive additional product candidates forward.”

Company Updates and Upcoming Milestones

SRK-015 Program for Spinal Muscular Atrophy:

SRK-015 is a highly selective inhibitor of latent myostatin being developed as the potential first muscle-directed therapy for the treatment of SMA.

- **On Track to Report Interim Efficacy and Safety Data from the TOPAZ Phase 2 Trial in the Fourth Quarter of 2020.** The interim efficacy, safety, pharmacokinetic (PK), and pharmacodynamic (PD) analysis will include data from 54 patients who have progressed through at least six months of treatment in the TOPAZ Phase 2 trial. The remaining three patients (one in Cohort 2 and two in Cohort 3) missed three doses of SRK-015 due to COVID-19-related restrictions at their trial site, and their 6-month assessments will be excluded from the interim analysis. These patients have resumed dosing and the Company is working closely with the trial site to schedule their next assessments.
    - To date, one patient (Cohort 1) has discontinued from the trial for reasons unrelated to the study drug and which occurred prior to the COVID-19 pandemic. All remaining 57 patients are continuing in the study.
    - As of August 1, 2020:
        - 56 of 57 patients have completed the 5-month visit.
        - 54 of 57 patients have completed the 6-month visit for the interim efficacy and safety analysis.
        - Eight of eight patients who have completed the 12-month treatment period have opted into the 12-month extension study.

With the progress to date for the completion of patient visits towards the interim analysis, Scholar Rock is working closely with the clinical trial sites through the ongoing COVID-19 pandemic to enable access to routine data monitoring activities to prepare for the interim analysis. The Company remains on track to report interim efficacy and safety data in the fourth quarter of 2020. Top-line data for the 12-month treatment period are expected in the first half of 2021. There may be further 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.

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 and meaningfully increasing the number of patients who may benefit from checkpoint inhibitor therapy.

- **Enrollment Advancing in DRAGON Phase 1 Proof-of-Concept Trial with Update on Dose Escalation Expected in the Fourth Quarter of 2020.** The DRAGON Phase 1 dose escalation and dose expansion trial is evaluating SRK-181 in patients with locally advanced or metastatic solid tumors. Dose escalation in Part A1 of the trial continues to progress, and the Company expects to advance to Part A2 of the trial to evaluate SRK-181 in combination with an approved anti-PD-(L)1

therapy in the third quarter of 2020 and to Part B of the trial in the first quarter of 2021. An update on dose escalation of SRK-181 as a single agent as well as in combination with anti-PD-(L)1 therapy is on track for the fourth quarter of 2020. Clinical response and safety data are anticipated in 2021.

The two-part DRAGON trial consists of a dose escalation portion (Part A) for SRK-181 as both a single-agent (Part A1) and in combination with an approved anti-PD-(L)1 therapy (Part A2), followed by a dose expansion portion (Part B) evaluating SRK-181 in combination with an approved anti-PD-(L)1 therapy in patients with 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. Patients will be administered SRK-181 IV every 3 weeks (Q3W), and additional dosing regimens may be explored in the future. Key objectives of the study include evaluating the efficacy, PK, and safety of SRK-181.

"We are encouraged by the high level of engagement of our trial investigators and patients. Patients in our SRK-015 TOPAZ trial are continuing their visits and thus far, we've seen a high rate of enrollment into the 12-month extension study," said Yung Chyung, M.D., Chief Medical Officer of Scholar Rock. "We are also pleased with the progress to date enrolling patients in our SRK-181 DRAGON trial, particularly given the backdrop of the ongoing COVID-19 pandemic. Important readouts from these trials will provide meaningful insights into the therapeutic potential of our product candidates as well as the power of our drug discovery platform."

Executive Leadership Updates:

- **Appointment of Tony Kingsley as President and Chief Executive Officer.** In July 2020, Scholar Rock announced that Tony Kingsley was being appointed President and Chief Executive Officer, effective August 1, 2020. Mr. Kingsley replaces Nagesh K. Mahanthappa, Ph.D., MBA, who chose to step down after serving in this role since 2012. Mr. Kingsley is a proven leader with a successful track record of driving growth, strategy and all facets of corporate operation. Dr. Mahanthappa continues to serve as a scientific advisor to the Company.
- **Appointment of Ted Myles as Chief Financial Officer and Head of Business Operations.** In July 2020, Scholar Rock announced the appointment of Ted Myles as Chief Financial Officer and Head of Business Operations, bringing more than 20 years of experience as a senior finance and operations executive with development and commercial stage biopharmaceutical companies. He had previously served on Scholar Rock's Board of Directors, including as chair of the audit committee and a member of the compensation committee.

Second Quarter 2020 Financial Results

For the quarter ended June 30, 2020, net loss was $19.3 million or $0.65 per share compared to a net loss of $12.5 million or $0.48 per share for the quarter ended June 30, 2019.

- Revenue was $3.9 million for the quarter ended June 30, 2020 compared to $5.0 million for the quarter ended June 30, 2019. Revenue was related to the Gilead fibrosis-focused collaboration that was executed in December 2018.
- Research and development expense was $17.0 million for the quarter ended June 30, 2020 compared to $13.7 million for the quarter ended June 30, 2019. The increase year-over-year is attributable to the acceptance of a customized antibody display library from Specifica, Inc., costs associated with the TOPAZ Phase 2 clinical trial for SRK-015, and higher personnel-related costs.
- General and administrative expense was $6.4 million for the quarter ended June 30, 2020 compared to $4.7 million for the quarter ended June 30, 2019. The increase year-over-year was primarily attributable to increased headcount and professional services.

As of June 30, 2020, Scholar Rock had cash, cash equivalents, and marketable securities of $141.2 million, which compares to cash, cash equivalents, and marketable securities of $157.4 million as of December 31, 2019.

Conference Call/Webcast:

Scholar Rock will host a conference call and audio webcast to discuss the second quarter 2020 financial results today at 8:00 a.m. Eastern Time. To participate in the call, please dial 833-519-1308 (domestic) or 914-800-3874 (international) and refer to conference ID: 5627485. A webcast of the call will also be available on the Investors & Media section of the Scholar Rock website at http://investors.scholarrock.com. An archived replay of the webcast will be available on Scholar Rock's website at: https://scholarrock.com/ for approximately 90 days following the presentation.

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 SRK-015, 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 nonclinical or preclinical study data, the potential of its proprietary platform, and the impact of COVID-19 on its clinical trials and its business and operations in general. 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 March 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)

	Three Months Ended June 30		Six Months Ended June 30	
	2020	2019	2020	2019
Revenue	$ 3,900	$ 5,039	$ 8,930	$ 8,145
Operating expenses				
Research and development	16,997	13,715	33,899	24,454
General and administrative	6,365	4,710	12,187	8,780
Total operating expenses	23,362	18,425	46,086	33,234
Loss from operations	(19,462)	(13,386)	(37,156)	(25,089)
Other income (expense), net	181	861	805	1,809
Net loss	$ (19,281)	$ (12,525)	$ (36,351)	$ (23,280)
Net loss per share, basic and diluted	$ (0.65)	$ (0.48)	$ (1.23)	$ (0.90)
Weighted average common shares outstanding, basic and diluted	29,690,280	25,922,833	29,608,814	25,758,658

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

	June 30, 2020	December 31, 2019
Assets		
Cash, cash equivalents and marketable securities	$ 141,176	$ 157,448

Other current assets	4,011	27,719
Total current assets	145,187	185,167
Other assets	11,173	11,214
Total assets	$ 156,360	$ 196,381
Liabilities and Stockholders' Equity		
Current liabilities	$ 32,567	$ 32,814
Long-term liabilities	41,619	50,666
Total liabilities	74,186	83,480
Total stockholders' equity	82,174	112,901
Total liabilities and stockholders' equity	$ 156,360	$ 196,381

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