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

May 7, 2020

- Progressing TOPAZ Phase 2 clinical trial of SRK-015 in patients with Spinal Muscular Atrophy; interim efficacy and safety results delayed by approximately one quarter to 4Q20 due to impact of COVID-19 pandemic on clinical trial sites

- Commenced dosing of patients with solid tumors that exhibit primary resistance to anti-PD(L)1 therapy in the DRAGON Phase 1 clinical trial of SRK-181; update on dose escalation expected in 4Q20

CAMBRIDGE, Mass.--(BUSINESS WIRE)--May 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 first quarter ended March 31, 2020 and highlighted recent progress and upcoming milestones for its pipeline programs.

"We are humbled by the tenacity and commitment of the patients and clinicians with whom we have the privilege to work as we continue to adjust to the challenges of the COVID-19 pandemic," said Nagesh Mahanthappa, Ph.D., President and CEO of Scholar Rock. "It is with a primary focus on the safety and well-being of our patients, clinical investigators and employees, that we are committed to continuing the execution of the SRK-015 TOPAZ Phase 2 and the SRK-181 DRAGON Phase 1 trials. Though we may continue to experience uncertainty during these unprecedented times, Scholar Rock is planning for additional updates on both programs later this year."

Company Updates and Upcoming Milestones

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

- **Anticipate Interim Efficacy and Safety Data from the TOPAZ Phase 2 Trial in the Fourth Quarter of 2020.** COVID-19-related restrictions have impacted patient access to clinical trial sites, which have led to missed or delayed doses or assessments (including efficacy) in some patients and has affected Scholar Rock's ability to monitor trial data collected by sites. As a result, Scholar Rock now plans to report interim efficacy, safety, pharmacokinetic (PK), and pharmacodynamic (PD) results in the fourth quarter of 2020 for patients across the three cohorts who have progressed through at least six months of the treatment period. This is an approximately one quarter delay compared to the previous guidance of interim 6-month results in mid-2020.

  As of May 1, 2020, the majority of patients have either completed or are on track to complete their 6-month visit for the interim efficacy and safety analysis. For patients who have had their 6-month visit impacted, Scholar Rock is working closely with clinical trial sites on scheduling to minimize any additional delays in dosing and assessments.

- Enrollment of the TOPAZ proof-of-concept trial was completed in January 2020. 58 patients with Type 2 or Type 3 SMA were enrolled across the three cohorts (23 patients in Cohort 1, 15 patients in Cohort 2, and 20 patients in Cohort 3).
- 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.
- As of May 1, 2020, 41 of 57 patients have completed the 6-month visit for the interim efficacy and safety analysis.
- As of May 1, 2020, 51 of 57 patients have completed the 5-month visit.
- Approximately 70% of patients are enrolled in the U.S. and the remaining 30% of patients are enrolled in Europe, including Italy, Spain, and Netherlands.

  Top-line data for the 12-month treatment period are now expected in the first half of 2021 as compared to the previous guidance of the fourth quarter of 2020 and first quarter 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.

- **Identification of Second Indication for SRK-015 Planned for 2020.** Scholar Rock continues to evaluate multiple potential opportunities beyond SMA, for which the selective inhibition of the activation of myostatin with SRK-015 may offer therapeutic benefit.

SRK-181 Program for Immuno-Oncology:

- **Update on Dose Escalation from the DRAGON Phase 1 Proof-of-Concept Trial in Solid Tumors Expected in the Fourth Quarter of 2020.** SRK-181 is a potent and highly selective inhibitor of latent transforming growth factor beta 1 (TGFβ1) activation and is being developed with an aim of expanding anti-tumor responses from immunotherapy by overcoming primary resistance to anti-PD-1 or anti-PD-L1 therapy. The DRAGON Phase 1 dose escalation and dose expansion trial evaluating SRK-181 in patients with locally advanced or metastatic solid tumors was initiated in the first quarter of 2020. Scholar Rock continues to activate clinical trial sites and recently commenced patient enrollment and dosing. Update on dose escalation of SRK-181 as a single agent as well as in combination with anti-PD-(L)1 therapy is expected in the fourth quarter of 2020, subject to the pace of enrollment as impacted by the COVID-19 pandemic. Clinical response and safety data are anticipated in 2021. Timing of data read-outs may be further impacted by the COVID-19 pandemic.

  The two-part DRAGON trial consists of a dose escalation portion (Part A) for SRK-181 as both a single-agent and in combination with an approved anti-PD-(L)1 therapy, 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.

- **Published Preclinical Data Detailing a Potent and Selective Inhibitor of TGFβ1 Overcoming Primary Resistance to Checkpoint Inhibition.** In March 2020, Scholar Rock's preclinical data establishing the therapeutic rationale for evaluating a potent and highly selective inhibitor of TGFβ1 activation to overcome primary resistance to checkpoint inhibitor therapy were published in the peer-reviewed journal *Science Translational Medicine*[(1)]. Selective inhibition of latent TGFβ1 activation with SRK-181 has demonstrated in preclinical studies the potential to overcome primary resistance, which, if replicated in clinical studies, could meaningfully expand the number of patients who could benefit from checkpoint inhibitor therapy. In preclinical studies, an improved safety profile was observed for SRK-181 as compared to conventional inhibitors of TGFβ signaling.

RGMc Program for Iron-Restricted Anemias:

- **Nomination of a Product Candidate from the RGMc Program Planned in 2020.** Scholar Rock is evaluating a number of highly specific inhibitors of repulsive guidance molecule C (RGMc) and plans to nominate an antibody as its third product candidate in 2020. RGMc's known function is localized to hepatocytes and the identification of RGMc selective-antibodies may offer the potential for liver-specific modulation of BMP6 signaling to address iron-restricted anemias.

Additional Corporate Updates:

- **Chief Scientific Officer, Alan Buckler, Ph.D., to Step Down Effective June 1, 2020.**
  Alan Buckler, Ph.D., has decided to leave Scholar Rock to pursue other opportunities and will remain in his position through June 1 to ensure a smooth transition. Alan has been instrumental in helping to build a highly talented research team that has established Scholar Rock's proprietary discovery platform as well as advanced two product candidates through research, discovery and preclinical development into the clinic.

  On June 1, 2020, Gregory Carven, Ph.D., current Senior Vice President of Biologics, will be named to the new position of Head of Research to continue delivering on the Company's commitment to drug discovery. Gregory is a gifted scientist with more than 15 years of experience in the discovery and development of antibody therapeutics and is a co-inventor of pembrolizumab. Since joining Scholar Rock in 2014, Gregory has led the Company's biologics discovery, design, and manufacturing initiatives and played a central role in the discovery and preclinical development of both the SRK-015 and SRK-181 product candidates.

- **Announced Issuance of U.S. Patent Broadly Relevant to Antibodies that Modulate TGFβ Activation.** The United States Patent Office (USPTO) issued U.S. Patent No. 10,597,443 with an expiry of May 2034 broadly covering methods for making activation modulators of TGFβ that utilize Scholar Rock's proprietary platform approach of targeting the precursor form of growth factors. This broadly relates to TGFβ activation inhibitors that are specific to the context of presenting molecules, such as GARP and LTBP, as well as activation inhibitors that are not dependent on a specific presenting molecule, such as SRK-181.

First Quarter 2020 Financial Results

For the quarter ended March 31, 2020, net loss was $17.1 million or $0.58 per share compared to a net loss of $10.8 million or $0.42 per share for the quarter ended March 31, 2019.

- Revenue was $5.0 million for the quarter ended March 31, 2020 and was related to the Gilead fibrosis-focused collaboration (the “Gilead Collaboration Agreement”) that was executed in December 2018.
- Research and development expense was $16.9 million for the quarter ended March 31, 2020 compared to $10.7 million for the quarter ended March 31, 2019. The increase year-over-year primarily reflects manufacturing costs and costs associated with the initiation of the DRAGON Phase 1 clinical trial for SRK-181, costs associated with the TOPAZ Phase 2 clinical trial for SRK-015, and higher personnel-related costs, slightly offset by lower early development costs.
- General and administrative expense was $5.8 million for the quarter ended March 31, 2020 compared to $4.1 million for the quarter ended March 31, 2019. The increase year-over-year was primarily attributable to increased headcount and professional services.

As of March 31, 2020, Scholar Rock had cash, cash equivalents, and marketable securities of $160.6 million, which is inclusive of a $25 million payment from Gilead for the achievement of the preclinical milestone under the Gilead Collaboration Agreement. This compares to cash, cash equivalents, and marketable securities of $157.4 million as of December 31, 2019.

(1) Martin, C., Datta, A., Schürpf, T., et al. Selective inhibition of TGFβ1 activation overcomes primary resistance to checkpoint blockade therapy by altering tumor immune landscape, *Science Translational Medicine,* 2020 Mar 25; 12(536), eaay8456.

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 newly elucidated understanding of 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, 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 Annual Report on Form 10-K for the year ended December 31, 2019, 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 March 31	
	2020	**2019**
Revenue	$ 5,030	$ 3,106

Operating expenses		
Research and development	16,902	10,739
General and administrative	5,822	4,070
Total operating expenses	22,724	14,809
Loss from operations	(17,694)	(11,703)
Other income (expense), net	624	948
Net loss	$ (17,070)	$ (10,755)
Net loss per share, basic and diluted	$ (0.58)	$ (0.42)
Weighted average common shares outstanding, basic and diluted	29,527,349	25,592,659

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

	March 31, 2020	December 31, 2019
Assets		
Cash, cash equivalents and marketable securities	$ 160,648	$ 157,448
Other current assets	3,525	27,719
Total current assets	164,173	185,167
Other assets	11,529	11,214
Total assets	$ 175,702	$ 196,381
Liabilities and Stockholders' Equity		
Current liabilities	$ 27,100	$ 32,814
Long-term liabilities	49,955	50,666
Total liabilities	77,055	83,480
Total stockholders' equity	98,647	112,901
Total liabilities and stockholders' equity	$ 175,702	$ 196,381

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