Scholar Rock Reports Full Year 2019 Financial Results and Highlights Business Progress

March 12, 2020

- Initiated the Phase 1 proof-of-concept clinical trial of SRK-181, a potent and highly selective inhibitor of TGFβ1 activation, in patients with locally advanced or metastatic solid tumors

- On track to report interim safety and efficacy data from the TOPAZ Phase 2 clinical trial of SRK-015 in patients with Type 2 and Type 3 Spinal Muscular Atrophy in mid-2020

CAMBRIDGE, Mass.--(BUSINESS WIRE)-- 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, 2019 and highlighted recent progress and upcoming milestones for its pipeline programs.

“The year is off to a strong start with the initiation of the Phase 1 trial of SRK-181 in immuno-oncology and the completion of enrollment in the SRK-015 TOPAZ Phase 2 trial in SMA,” said Nagesh Mahanthappa, Ph.D., President and CEO of Scholar Rock. “We now have two product candidates in the clinic with data expected from both programs later this year that will offer initial insights on their potential to treat patients with SMA and solid tumors, respectively. Scholar Rock is well-positioned to continue to execute and build momentum across its portfolio of product candidates.”

Company Highlights and Upcoming Milestones

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

- **Published Structural Insights into the Mechanism by which SRK-015 Targets the Precursor or Latent Form of the Myostatin Growth Factor.** In February 2020, the Journal of Biological Chemistry[(1)] published *“Structural basis for specific inhibition of extracellular activation of pro- or latent myostatin by the monoclonal antibody SRK-015”* detailing the integrated structural and biochemical approaches used to elucidate the unique molecular mechanism of SRK-015, a highly specific inhibitor of the activation of latent myostatin. The publication offers the first structural insights into the approach of targeting precursor forms to modulate growth factor activation. This approach could be applied to the discovery of specific modulators across members of the TGFβ superfamily or, even more broadly, to other signaling proteins that require precursor processing for activation.

- **On Track to Report Interim Safety and Efficacy Data from TOPAZ Phase 2 Trial in Mid-2020.** In January 2020, Scholar Rock announced the completion of enrollment in the TOPAZ proof-of-concept trial for SRK-015 with a total of 58 patients with Type 2 or Type 3 SMA enrolled across 19 sites in the U.S. and Europe. Scholar Rock plans to report interim safety and efficacy results for all patients enrolled across the three cohorts of the trial following six months of treatment exposure in mid-2020. Top-line data for the full 12-month treatment period are expected beginning in the fourth quarter of 2020 and into the first quarter of 2021.

- **Presented Preliminary Data Highlighting Patient Demographics and Baseline Characteristics from the TOPAZ Trial at SMA Europe 2nd International Scientific Congress.** In February 2020, Scholar Rock presented two posters at the SMA Europe 2nd International Scientific Congress highlighting key preclinical and Phase 1 data, in addition to preliminary pharmacokinetic (PK)/pharmacodynamic (PD) and baseline characteristics from the TOPAZ Phase 2 trial of SRK-015 in patients with Type 2 and Type 3 SMA. Preliminary demographic and baseline characteristic data for patients in the TOPAZ trial are in-line with previously published studies in SMA, including the evaluation of patients treated with nusinersen.

- **Announced Preliminary Pharmacokinetic and Pharmacodynamic Results from TOPAZ Trial.** In November 2019, Scholar Rock announced results from a planned preliminary PK/PD analysis of 29 patients across all three cohorts of the TOPAZ trial. The analysis showed dose-proportional drug exposure and dose-dependent increases of up to 100-fold in serum latent myostatin levels following treatment with SRK-015. These biomarker results confirmed the presence of latent myostatin in patients with SMA and provides the first evidence of successful pharmacologic engagement of a latent growth factor in a human disease setting.

- **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:

- **Initial Clinical Data from SRK-181 Phase 1 Dose Escalation and Dose Expansion Trial in Solid Tumors Expected in the Second Half of 2020.** In the first quarter of 2020, Scholar Rock initiated a Phase 1 proof-of-concept trial for SRK-181 in patients with locally advanced or metastatic solid tumors. The two-part trial will consist 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 antibody, followed by a dose expansion portion (Part B) evaluating SRK-181 in combination with an approved anti-PD-(L)1 antibody in multiple tumor-specific cohorts, including urothelial carcinoma, cutaneous melanoma, non-small cell lung cancer, and other solid tumors. Key objectives of the study include evaluating the safety and pharmacokinetics of SRK-181 and the efficacy of SRK-181 in combination with anti-PD-(L)1 therapy in the treatment of solid tumors exhibiting primary resistance to anti-PD-(L)1 therapy. Initial clinical results, including biomarker data, from Part A of the Phase 1 trial are expected in the second half of 2020 with clinical response and safety data from Part B anticipated throughout 2021.

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.

Corporate Highlight:

- **Achieved First Milestone of $25 Million in Strategic Fibrosis Collaboration with Gilead Sciences.** In December 2019, Scholar Rock achieved a $25 million preclinical milestone under the strategic fibrosis-focused collaboration with Gilead Sciences, Inc. for the successful demonstration of efficacy in preclinical *in vivo* proof-of-concept studies. This initial milestone advances the collaboration to develop potent and selective inhibitors of latent TGFβ activation for the potential treatment of patients suffering from fibrotic diseases.

Full Year 2019 Financial Results

For the year ended December 31, 2019, net loss was $51.0 million or $1.85 per share compared to a net loss of $49.3 million or $3.15 per share for the year ended December 31, 2018.

- Revenue was $20.5 million for the year ended December 31, 2019 and was related to the Gilead Collaboration Agreement that was executed in December 2018.

- Research and development expense was $54.2 million for the year ended December 31, 2019 compared to $36.3 million for the year ended December 31, 2018. The increase year-over-year primarily reflects preclinical and manufacturing costs for SRK-181 and higher personnel-related costs, slightly offset by lower manufacturing costs for SRK-015 and early development costs.

- General and administrative expense was $20.8 million for the year ended December 31, 2019 compared to $14.4 million for the year ended December 31, 2018. The increase year-over-year was primarily attributable to increased headcount and professional services.

As of December 31, 2019, Scholar Rock had cash, cash equivalents, and marketable securities of $157.4 million, compared to $175.6 million as of December 31, 2018. A $25 million payment was received in January 2020 from Gilead for the achievement of the preclinical milestone under the strategic fibrosis-focused collaboration.

(1) Dagbay, K., Treece, E., Streich Jr, F., Carven, G., et al. Structural basis for specific inhibition of extracellular activation of pro- or latent myostatin by the monoclonal antibody SRK-015, *Journal of Biological Chemistry*, https://www.jbc.org/cgi/doi/10.1074/jbc.RA119.012293

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, and the ability of any product candidate to perform in humans in a manner consistent with nonclinical or preclinical study data. 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, and 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 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 September 30, 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)

	Years Ended December 31	
	2019	**2018**
Revenue	$ 20,492	$ —
Operating expenses		
Research and development	54,217	36,310
General and administrative	20,817	14,382
Total operating expenses	75,034	50,692
Loss from operations	(54,542)	(50,692)
Other income (expense), net	3,542	1,366
Net loss	$ (51,000)	$ (49,326)
Net loss per share, basic and diluted	$ (1.85)	$ (3.15)
Weighted average common shares outstanding, basic and diluted	27,537,939	15,655,293

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

	December 31, 2019	**December 31, 2018**
Assets		
Cash, cash equivalents and marketable securities	$ 157,448	$ 175,645
Other current assets	27,719	2,296
Total current assets	185,167	177,941

Other assets	11,214	3,395
Total assets	$ 196,381	$ 181,336
Liabilities and Stockholders' Equity		
Current liabilities	$ 32,814	$ 31,123
Long-term liabilities	50,666	43,590
Total liabilities	83,480	74,713
Total stockholders' equity	112,901	106,623
Total liabilities and stockholders' equity	$ 196,381	$ 181,336

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Scholar Rock Contact:
Investors/Media
Catherine Hu
chu@scholarrock.com
917-601-1649

Media Contact:
The Yates Network
Kathryn Morris
kathryn@theyatesnetwork.com
914-204-6412

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