Scholar Rock Reports Second Quarter 2019 Financial Results and Highlights Business Progress

August 14, 2019

- *Enrollment on track in the TOPAZ Phase 2 clinical trial of SRK-015 in patients with Type 2 and Type 3 Spinal Muscular Atrophy for preliminary PK/PD results by year-end*
- *Presented positive final results from the Phase 1 clinical trial of SRK-015 in healthy volunteers at the Cure SMA Annual Conference*
- *Completed public offering of common stock raising approximately $51.8 million in gross proceeds to advance portfolio of product candidates for the treatment of neuromuscular disorders, cancer, and anemia*

CAMBRIDGE, Mass., Aug. 14, 2019 (GLOBE NEWSWIRE) -- Scholar Rock Holding Corporation (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 quarter ended June 30, 2019 and highlighted recent progress and upcoming milestones for its pipeline programs.

"Throughout the first half of 2019, we made significant progress both clinically and financially as we advanced SRK-015 into a Phase 2 trial in SMA, SRK-181 towards the initiation of a Phase 1 trial in cancer immunotherapy in mid-2020, as well as successfully completed a follow-on offering to ensure that we are well-capitalized to support our portfolio of product candidates and overall company growth," said Nagesh Mahanthappa, Ph.D., President and CEO of Scholar Rock. "We look forward to building upon this momentum as we continue to progress our broad pipeline, with a number of important milestones at the end of this year and into 2020."

Company Highlights and Upcoming Milestones

SRK-015 Program:

- **Positive Final Results from the Phase 1 Clinical Trial of SRK-015 in Healthy Volunteers Presented at the Cure SMA Annual Conference.** In June 2019, Scholar Rock presented positive detailed results from the Phase 1 trial of SRK-015, which consisted of data from both the single-ascending and multiple-ascending dose portions of the trial. SRK-015 was observed to be well-tolerated with no dose-limiting toxicities identified in up to the highest evaluated dose of 30 mg/kg. The pharmacologic profile showed robust engagement of latent myostatin, which was saturated and sustained up to at least Day 140 after three doses of SRK-015 given once every two weeks at 20 mg/kg or 30 mg/kg. In contrast, no meaningful change was observed in the latent myostatin biomarker concentrations in subjects who received placebo.

- **Preliminary Pharmacokinetic (PK) and Pharmacodynamic (PD) Data from TOPAZ Phase 2 TrialExpected by End of 2019.** Patient enrollment is underway in the TOPAZ Phase 2 proof-of-concept trial to assess the safety and efficacy of SRK-015 in patients with Type 2 and Type 3 Spinal Muscular Atrophy (SMA). Approximately 55 patients are anticipated to be enrolled in the U.S., Canada, and Europe across three distinct and parallel cohorts and treated with SRK-015, either as a monotherapy or in conjunction with an approved survival motor neuron (SMN) upregulator therapy.

  Scholar Rock is on track to report preliminary PK/PD data for a subset of patients in the Phase 2 TOPAZ trial by the end of 2019, which will provide initial observations on target engagement of latent myostatin in patients with Type 2 and Type 3 SMA. These preliminary PK/PD results will be followed by interim safety and efficacy results for a subset of patients with six months of treatment exposure expected in the first half of 2020 and top-line data for the full 12-month treatment period expected beginning in the fourth quarter of 2020.

- **Details of Scholar Rock-Developed Biomarker Assay that Quantifies Serum Latent Myostatin Published in Peer-Reviewed Journal.** In July 2019, Scholar Rock published details of its immunoassay used to measure serum latent myostatin in peer-reviewed journal *Society of Laboratory Automation and Screening Discovery.* The publication, "A Sensitive and Selective Immunoassay for the Quantitation of Serum Latent Myostatin After In-Vivo Administration of SRK-015, a Selective Inhibitor of Myostatin Activation,"[(1)] highlights the ability of the immunoassay to quantify serum latent myostatin without cross-reactivity to promyostatin, mature myostatin, or closely related members of the TGFβ superfamily and the establishment of the robustness, reproducibility, and stability of the immunoassay.

  Through use of this biomarker assay, robust and sustained target engagement of latent myostatin has been observed in both preclinical studies and the Phase 1 clinical trial in healthy volunteers, establishing proof-of-mechanism of Scholar Rock's therapeutic approach of targeting the latent form of growth factors.

- **Identification of Second Indication for SRK-015 Planned for 2020.** Scholar Rock continues to evaluate multiple potential opportunities for which SRK-015 could offer clinical benefit and is assessing additional potential clinical settings in which the selective inhibition of the activation of myostatin may offer therapeutic benefit.

SRK-181 Program:

- **Initiation of the Phase 1 Clinical Trial of SRK-181 in Patients with Solid Tumors Anticipated in Mid-2020.** In March 2019, Scholar Rock selected SRK-181, a highly specific inhibitor of TGFβ1 activation, as the first product candidate in its TGFβ1 cancer immunotherapy program based on the strength of preclinical data and human translational insights. Scholar Rock intends to develop SRK-181 for the treatment of tumors resistant to checkpoint blockade therapies (CBTs), such as anti-PD(L)1 antibodies, and believes there is significant opportunity to expand the number of patients who can benefit from CBTs.

  Scholar Rock plans to initiate a Phase 1 trial in patients with primary resistance to approved checkpoint blockade therapies in mid-2020 with initial clinical data anticipated by the end of 2021.

RGMc Program:

- **Nomination of a Product Candidate from the RGMc Program Planned in First Half of 2020.** For its third product candidate, Scholar Rock is evaluating a number of highly specific inhibitors of repulsive guidance molecule C (RGMc) and plans to nominate an antibody in the first half of 2020. RGMc is a co-receptor of bone morphogenetic protein 6 (BMP6), a member of the TGFβ superfamily that plays an important role in iron metabolism. 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 Update:

- **Public Offering of Common Stock Completed inJune/July 2019.** Scholar Rock successfully completed a public offering of 3,450,000 shares of common stock, inclusive of the full exercise of the over-allotment option by the underwriters, raising gross proceeds of approximately $51.8 million.

Second Quarter 2019 Financial Results

For the quarter ended June 30, 2019, net loss was $12.5 million or $0.48 per share compared to a net loss of $14.7 million or $1.39 per share for the quarter ended June 30, 2018.

- Research and development expense was $13.7 million for the quarter ended June 30, 2019 compared to $11.4 million for the quarter ended June 30, 2018. The increase year-over-year reflects preclinical and manufacturing costs for SRK-181 and higher personnel-related costs, partially offset by a decrease year-over-year in manufacturing costs associated with SRK-015.

- General and administrative expense was $4.7 million for the quarter ended June 30, 2019 compared to $3.5 million for the quarter ended June 30, 2018. The increase year-over-year was primarily attributable to increased headcount, stock compensation, and higher operational fees associated with operating as a public company.

As of June 30, 2019, Scholar Rock had cash, cash equivalents, and marketable securities of $185.1 million, compared to $175.6 million as of December 31, 2018. The cash balance as of June 30, 2019 is inclusive of the approximately $42.2 million in net proceeds from the public offering of 3,000,000 shares of common stock and exclusive of $6.3 million in net proceeds from the underwriter's purchase of an additional 450,000 shares of common stock that was settled in July 2019.

1. Cote, S. M., Jackson, J., Pirruccello-Straub, M., Carven, G. J., & Wawersik, S. (2019). A Sensitive and Selective Immunoassay for the Quantitation of Serum Latent Myostatin after In Vivo Administration of SRK-015, a Selective Inhibitor of Myostatin Activation. *SLAS DISCOVERY: Advancing Life Sciences R&D*.

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.

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

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

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,	
	2019	2018	2019	2018
Revenue	$ 5,039	$ -	$ 8,145	$ -
Operating expenses				
Research and development	13,715	11,424	24,454	18,125
General and administrative	4,710	3,460	8,780	5,775
Total operating expenses	18,425	14,884	33,234	23,900
Loss from operations	(13,386)	(14,884)	(25,089)	(23,900)
Other income (expense), net	861	177	1,809	301
Net loss	$ (12,525)	$ (14,707)	$ (23,280)	$ (23,599)
Net loss per share, basic and diluted	$ (0.48)	$ (1.39)	$ (0.90)	$ (3.51)
Weighted-average common shares outstanding, basic and diluted	25,922,833	10,593,987	25,758,658	6,716,283

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

	June 30, 2019	December 31, 2018
Assets		
Cash, cash equivalents and marketable securities	$ 185,131	$ 175,645
Other current assets	3,247	2,296
Total current assets	188,378	177,941
Other assets	8,533	3,395
Total assets	$ 196,911	$ 181,336
Liabilities and Stockholders' Equity		
Current liabilities	$ 32,049	$ 31,123
Long-term liabilities	35,907	43,590
Total liabilities	67,956	74,713
Total stockholders' equity	128,955	106,623
Total liabilities and stockholders' equity	$ 196,911	$ 181,336

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Source: Scholar Rock