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

November 12, 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*

- *Accelerated initiation of Phase 1 dose escalation and proof-of-concept clinical trial for SRK-181 in patients with solid tumors to the first quarter of 2020*

CAMBRIDGE, Mass., Nov. 12, 2019 (GLOBE NEWSWIRE) -- 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, 2019 and highlighted recent progress and upcoming milestones for its pipeline programs.

"The third quarter of 2019 has been a time of focused execution as we continue to advance our growing pipeline. Notably, we have been able to accelerate the development of SRK-181 and are now planning to initiate a Phase 1 dose escalation and proof-of-concept trial in patients with solid tumors in the first quarter of 2020, a testament to the strength of our team and scientific progress," said Nagesh Mahanthappa, Ph.D., President and CEO of Scholar Rock. "As we wrap up the year and look towards 2020 and beyond, we are well positioned to work towards a number of important milestones across our pipeline, including read-outs from the TOPAZ Phase 2 trial for SRK-015 and the initiation of our Phase 1 trial for SRK-181, that will further elucidate the potential of our product candidates in improving the lives of patients suffering from serious diseases."

Company Highlights and Upcoming Milestones

SRK-015 Program:

- **Preclinical and Phase 1 SRK-015 Healthy Volunteer Data Presented at World Muscle Society Congress.** In October 2019, Scholar Rock presented data for SRK-015, a highly selective inhibitor of myostatin activation, that further enforce the therapeutic rationale for the advancement of SRK-015 into the TOPAZ Phase 2 clinical trial. Preclinical studies demonstrated improved muscle strength following the administration of a mouse analog of SRK-015 (muSRK-015P) in mouse models of early and late stage restoration of SMN, the protein that is deficient in the genetic disease SMA. Multi-fold increases in serum latent myostatin levels following treatment with muSRK-015P were shown across animal species in mice, rats, and cynomolgus monkeys. In the Phase 1 clinical trial in adult healthy volunteers, SRK -015 was well-tolerated, had a well-behaved pharmacokinetic (PK) profile, and demonstrated robust and sustained target engagement.

  Scholar Rock is on track to report preliminary PK and pharmacodynamic (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.

- **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:

- **Accelerated Initiation of Phase 1 Dose Escalation and Proof-of-Concept Clinical Trial of SRK-181 in Patients with Solid Tumors to the First Quarter of 2020.** Scholar Rock intends to develop SRK-181, an isoform-selective inhibitor of TGFβ1 activation as a cancer immunotherapy in combination with anti-PD(L)1 antibodies. The company now plans to initiate a Phase 1 trial in the first quarter of 2020, in patients with locally advanced or metastatic solid tumors that exhibit primary resistance to anti-PD(L)1 antibodies. The two-part trial will consist of a dose escalation portion as both a single-agent and SRK-181 in combination with an approved anti-PD(L)1 antibody, as well as a dose expansion portion consisting of multiple tumor-specific cohorts evaluating SRK-181 in combination with an approved anti-PD(L)1 antibody.

RGMc Program:

- **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.

Third Quarter 2019 Financial Results

For the quarter ended September 30, 2019, net loss was $16.1 million or $0.55 per share compared to a net loss of $10.8 million or $0.44 per share for the quarter ended September 30, 2018.

- Revenue was $4.8 million for the quarter ended September 30, 2019 and was related to the Gilead Collaboration Agreement that was executed in December 2018.
- Research and development expense was $15.7 million for the quarter ended September 30, 2019 compared to $8.1 million for the quarter ended September 30, 2018. The increase year-over-year primarily reflects preclinical and manufacturing costs for SRK-181, clinical development costs associated with SRK-015, and higher personnel-related costs.
- General and administrative expense was $6.2 million for the quarter ended September 30, 2019 compared to $3.2 million for the quarter ended September 30, 2018. The increase year-over-year was primarily attributable to increased headcount, stock compensation, separation-related expense, and professional services.

As of September 30, 2019, Scholar Rock had cash, cash equivalents, and marketable securities of $176.1 million, compared to $175.6 million as of December 31, 2018.

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 June 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.

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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,	
	2019	**2018**	**2019**	**2018**
Revenue	$ 4,774	$ -	$ 12,919	$ -
Operating expenses				
Research and development	15,699	8,061	40,153	26,185
General and administrative	6,181	3,173	14,961	8,947
Total operating expenses	21,880	11,234	55,114	35,132
Loss from operations	(17,106)	(11,234)	(42,195)	(35,132)
Other income (expense), net	959	472	2,768	772
Net loss	$ (16,147)	$ (10,762)	$ (39,427)	$ (34,360)
Net loss per share, basic and diluted	$ (0.55)	$ (0.44)	$ (1.46)	$ (2.72)
Weighted average common shares outstanding, basic and diluted	29,232,158	24,310,681	26,929,215	12,647,032

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

	September 30, 2019	**December 31, 2018**
Assets		
Cash, cash equivalents and marketable securities	$ 176,082	$ 175,645
Other current assets	3,157	2,296
Total current assets	179,239	177,941
Other assets	9,303	3,395
Total assets	$ 188,542	$ 181,336
Liabilities and Stockholders' Equity		
Current liabilities	$ 32,522	$ 31,123
Long-term liabilities	34,230	43,590
Total liabilities	66,752	74,713
Total stockholders' equity	121,790	106,623
Total liabilities and stockholders' equity	$ 188,542	$ 181,336



Source: Scholar Rock