Scholar Rock Reports Third Quarter 2018 Financial Results and Highlights Business Progress

November 8, 2018

- *Completed Enrollment in Multiple Ascending Dose Portion of Phase 1 Clinical Trial for SRK-015; Interim Results Expected in 1Q19*
- *Plan to Initiate Phase 2 Proof-of-Concept Study with SRK-015 in Patients with Spinal Muscular Atrophy in 1Q19*
- *Presenting New Preclinical Data from TGFβ1 Cancer Immunotherapy Program at Upcoming Society for Immunotherapy of Cancer's (SITC) Annual Meeting*
- *On Track to Identify Product Candidate and First Indication in Oncology, Immuno-Oncology, or Fibrosis from TGFβ1 Program by the end of 1H19*

CAMBRIDGE, Mass., Nov. 08, 2018 (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 third quarter ended September 30, 2018 and highlighted recent progress and upcoming milestones for its pipeline programs.

"We continue to make important progress with our pipeline having now completed enrollment in the multiple ascending dose portion of the ongoing Phase 1 clinical trial for SRK-015 with interim results and the initiation of a Phase 2 proof-of-concept study in patients with Spinal Muscular Atrophy (SMA) expected in the first quarter of 2019," said Nagesh Mahanthappa, Ph.D, President and CEO of Scholar Rock. "We also look forward to our upcoming presentation of preclinical data at the SITC annual meeting that will demonstrate the ability of a highly specific inhibitor of TGFβ1 to render resistant solid tumors vulnerable to anti-PD1 while minimizing the toxicities traditionally associated with pan-TGFβ inhibitors."

Key R&D Highlights and Upcoming Milestones

SRK-015 Program:

- **Completed Enrollment in Multiple Ascending Dose Portion of Phase 1 Clinical Trial for SRK-015 with Interim Results Expected the First Quarter of 2019.** A placebo-controlled, double-blind Phase 1 clinical trial was initiated in May 2018 to evaluate the safety, tolerability, pharmacokinetics and pharmacodynamics of single and multiple ascending doses of intravenous SRK-015, a selective inhibitor of the activation of myostatin, in healthy adult volunteers. Enrollment in the single ascending dose and multiple ascending dose portions of the trial have been completed. Interim results from this ongoing Phase 1 trial are expected in the first quarter of 2019.
- **Plan to Initiate Phase 2 Proof-of-Concept Study for SRK-015 in SMA in the First Quarter of 2019.** Pending supportive safety data from the Phase 1 clinical trial, Scholar Rock plans to initiate a Phase 2 proof-of-concept study in the first quarter of 2019 to evaluate the safety and efficacy of SRK-015 in patients with SMA as a monotherapy or in conjunction with an approved survival motor neuron (SMN) upregulator therapy as background standard of care.
- **Plan to Identify Second Indication for SRK-015 in the First Half of 2019.** Scholar Rock is actively assessing numerous potential clinical settings in which the selective inhibition of the activation of myostatin may offer therapeutic benefit and intends to identify a second indication for SRK-015 in the first half of 2019.

TGFβ1 Program:

- **Presenting Preclinical Data at Upcoming SITC Annual Meeting Highlighting the Role of TGFβ1 Inhibition in Overcoming Primary Resistance to Anti-PD1.** At the Society for Immunotherapy of Cancer's (SITC) 33 rd Annual Meeting being held November 9-11, 2018, Scholar Rock will be presenting preclinical results demonstrating the ability of a highly specific inhibitor of TGFβ1, SRTβ1-Ab3, to render tumors vulnerable to checkpoint blockade therapy in syngeneic tumor models of primary resistance (Poster #550). Combination treatment with SRTβ1-Ab3 and anti-PD1 resulted in tumor regression or tumor control, and a significant survival benefit, while minimizing toxicities traditionally associated with pan-TGFβ inhibitors.
- **Plan to Nominate Product Candidate for TGFβ1 Program by the End of the First Half of 2019.** Scholar Rock's second antibody program is focused on the development of highly specific inhibitors of the activation of TGFβ1, and the company intends to nominate a product candidate and first indication in oncology, immuno-oncology or fibrosis by the end of the first half of 2019.

Third Quarter 2018 Financial Results

Net loss for the quarter ended September 30, 2018 was $10.8 million or $0.44 per share compared to a net loss of $5.9 million or $3.70 per unit for the same quarter last year.

- Research and development expense was $8.1 million for the quarter ended September 30, 2018, compared to $4.8 million in the same quarter in 2017. The $3.3 million increase year-over-year reflects development and manufacturing costs associated with lead product candidate, SRK-015, research costs associated with preclinical studies, as well as increased personnel-related costs to support continued progress with the pipeline.
- General and administrative expense was $3.2 million for the quarter ended September 30, 2018, compared to $1.2 million in the same quarter in 2017. The $2.0 million increase year-over-year was primarily attributable to increased headcount and higher professional and consulting fees associated with ongoing business activities and operating as a public company.

As of September 30, 2018, Scholar Rock had cash, cash equivalents, and marketable securities of $104.0 million, compared to $58.0 million at the end of 2017. Scholar Rock believes its cash and cash equivalents balance will be sufficient to fund operating expenses and capital expenditure requirements into the second half of 2020.

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 strategy, progress and timing of its Phase 1 clinical trial for SRK-015 and interim results from such clinical trial, the design and timing of its Phase 2 proof-of-concept study for SRK-015 in SMA, its product candidate selection and development timing, including timing for identifying a second disease indication for SRK-015 and the timing for identifying a product candidate and indication for its TGFβ1 program, the ability of its inhibitor of TGFβ1 to render tumors vulnerable to checkpoint blockade therapy, and the amount of time Scholar Rock's cash, cash equivalents, and marketable securities will fund the company's operations. 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 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, 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, 2018, 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 unit, share, per unit and per share data)

	Three Months Ended September 30		Nine Months Ended September 30	
	2018	2017	2018	2017
Operating expenses				
Research and development	$ 8,061	$ 4,778	$ 26,185	$ 13,057
General and administrative	3,173	1,164	8,947	3,514
Total operating expenses	11,234	5,942	35,132	16,571
Loss from operations	(11,234)	(5,942)	(35,132)	(16,571)
Interest income, net	472	9	828	32
Other expense, net	—	4	(56)	1
Total other income	472	13	772	33
Net loss	$ (10,762)	$ (5,929)	$ (34,360)	$ (16,538)
Net loss per share, basic and diluted	$ (0.44)		$ (2.72)	
Net loss per unit, basic and diluted		$ (3.70)		$ (10.32)
Weighted-average common shares outstanding - basic and diluted	24,310,681		12,647,032	
Weighted-average common units outstanding - basic and diluted		1,603,088		1,603,088

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

	September 30, 2018	December 31, 2017
Assets		
Cash, cash equivalents and marketable securities	$ 103,986	$ 57,959
Other current assets	2,201	$ 1,242
Total current assets	106,187	59,201
Other assets	2,831	2,436
Total assets	$ 109,018	$ 61,637
Liabilities and Stockholders' Equity		
Current liabilities	$ 5,761	$ 5,024
Long-term liabilities	914	903
Total liabilities	6,675	5,927
Convertible preferred stock	—	109,232
Total stockholders' equity (deficit)	102,343	(53,522)
Total liabilities, convertible preferred stock and stockholders' equity	$ 109,018	$ 61,637