Scholar Rock Reports Full Year 2018 Financial Results and Highlights Business Progress

March 18, 2019

- *Initiated and Progressed Phase 1 Clinical Trial of SRK-015 in Healthy Volunteers, Transitioning to a Clinical-Stage Company*
- *Presented Compelling Preclinical Data from the SRK-181 Cancer Immunotherapy Program at the Society for Immunotherapy of Cancer's (SITC) 33rd Annual Meeting*
- *Announced Strategic Collaboration with Gilead Sciences to Discover and Develop Highly Specific Inhibitors of TGFβ activation for the Treatment of Fibrotic Diseases*
- *Completed Initial Public Offering Raising Approximately $86 million in Gross Proceeds*

CAMBRIDGE, Mass., March 18, 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 full year ended December 31, 2018 and highlighted progress in 2018 and upcoming milestones for its pipeline programs.

Key 2018 Accomplishments

- Completed initial public offering and raised approximately $86 million in gross proceeds.
- Entered into a strategic fibrosis-focused collaboration with Gilead Sciences, Inc. to discover and develop highly specific inhibitors of TGFβ activation ($80 million received upfront and eligible for up to an additional $1,450 million in potential milestone payments).
- Transitioned to a clinical-stage company with the initiation of a Phase 1 clinical trial of SRK-015 in healthy volunteers.
- Published preclinical data on the therapeutic benefit of inhibiting myostatin activation in mouse models of Spinal Muscular Atrophy (SMA) in peer-reviewed journal *Human Molecular Genetics.*
- Received Orphan Drug Designation from the U.S. Food and Drug Administration (FDA) and Orphan Medicinal Product Designation from the European Commission (EC) for SRK-015 for the treatment of SMA.
- Presented compelling preclinical data that demonstrate SRK-181-mIgG1 (murine version of SRK-181), a highly specific TGFβ1 inhibitor, can render resistant solid tumors vulnerable to PD1 blockade.
- Progressed antibody platform for neuromuscular disorders, cancer immunotherapy, fibrosis, and anemias.

"2018 was a year of remarkable progress with our platform and for establishing a strong foundation as we transitioned to a public company," said Nagesh Mahanthappa, Ph.D, President and CEO of Scholar Rock. "We are focused on advancing our growing portfolio of product candidates, including SRK-015 for the treatment of SMA and SRK-181 to overcome primary resistance to checkpoint blockade therapy, as we continue to pursue our mission of developing innovative therapies to address significant unmet medical needs of patients."

R&D Highlights and Upcoming Milestones

SRK-015 Program:

- **Presented Interim Results from Phase 1 Clinical Trial of SRK-015 in Healthy Volunteers.** In February 2019, Scholar Rock presented positive interim results from the Phase 1 clinical trial of SRK-015, a highly specific inhibitor of myostatin activation, which was observed to be well tolerated with no dose limiting toxicities identified up to the highest evaluated dose of 30 mg/kg. Pharmacodynamic data, measuring serum concentrations of latent myostatin, demonstrated robust and sustained target engagement, providing initial proof-of-mechanism for this unique therapeutic approach. Collectively, the favorable interim safety and tolerability, pharmacodynamic, and pharmacokinetic data supported the advancement of SRK-015 to a Phase 2 proof-of-concept clinical trial in patients with SMA.
- **Initiating Phase 2 Proof-of-Concept Trial for SRK-015 in SMA in First Quarter 2019.** Scholar Rock is initiating a Phase 2 proof-of-concept trial to assess the safety and efficacy of SRK-015. The trial will consist of three non-overlapping cohorts, each evaluating a distinct subpopulation of patients with Type 2 and Type 3 SMA and all patients will receive

SRK-015 dosed once every four weeks either as a monotherapy or in conjunction with an approved survival motor neuron (SMN) upregulator therapy. The primary efficacy endpoints will measure motor function through clinically meaningful outcome measures validated in SMA, such as the Hammersmith Functional Motor Scale Expanded (HFMSE) in non-ambulatory SMA and the Revised Hammersmith Scale (RHS) in ambulatory SMA, over a 12-month treatment period. Scholar Rock plans to provide additional details on the Phase 2 trial design at the time of initiating patient dosing in the second quarter of 2019. Interim safety and efficacy results for a subset of patients in each cohort with at least six months of treatment exposure are expected in the first half of 2020.

- **Plan to Identify Second Indication for SRK-015 in 2020.** Scholar Rock continues to see 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:

- **Nominated SRK-181 as Cancer Immunotherapy Product Candidate.** 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. SRK-181 is being developed for the treatment of tumors resistant to checkpoint blockade therapies (CBTs), such as anti-PD(L)1 antibodies. Scholar Rock plans to initiate a Phase 1 trial in patients with solid tumors in mid-2020.

- **Additional SRK-181-mIgG1 Preclinical Data to be Presented at Upcoming AACR Annual Meeting.** Scholar Rock will present additional preclinical data from multiple syngeneic mouse models of primary checkpoint resistance at the 2019 American Association for Cancer Research (AACR) Annual Meeting being held March 29-April 3, 2019. These studies demonstrate that co-administration of SRK-181-mIgG1 (murine version of SRK-181) with an anti-PD1 antibody permits effector T cell infiltration and expansion into the tumor microenvironment, resulting in tumor regression or control as well as significant survival benefit, while minimizing toxicities traditionally associated with pan-TGFβ inhibitors.

Full Year 2018 Financial Results

For the year ended December 31, 2018, net loss was $49.3 million or $3.15 per share compared to a net loss of $25.0 million or $15.30 per share for the year ended December 31, 2017.

- Research and development expense was $36.3 million for the year ended December 31, 2018 compared to $19.9 million for the year ended December 31, 2017. The increase year-over-year in both periods reflect 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 $14.4 million for the year ended December 31, 2018 compared to $5.1 million for the year ended December 31, 2017. The increase year-over-year was primarily attributable to increased headcount and higher professional and consulting fees associated with the IPO and collaboration with Gilead, as well as ongoing business activities and operations as a public company.

As of December 31, 2018, Scholar Rock had cash, cash equivalents, and marketable securities of $175.6 million, compared to $58.0 million as of December 31, 2017.

“We established a strong financial foundation in 2018 with contributions from our successful IPO and our strategic fibrosis collaboration with Gilead,” said Rhonda Chicko, Chief Financial Officer of Scholar Rock. “As illustrated by the recent announcement of positive interim Phase 1 results for SRK-015 and the nomination of SRK-181 as the first product candidate in our cancer immunotherapy program, we continue to focus on our robust pipeline of highly specific growth factor modulators across a diverse range of therapeutic areas.”

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 and Phase 2 clinical trials for SRK-015, its product candidate selection and development timing, including its plans and timing for developing SRK-181 and timing for identifying a second disease indication for SRK-015, 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, 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.

Scholar Rock Holding Corporation
Condensed Consolidated Statements of Operations
(unaudited)
(in thousands, except share and per share data)

	Years Ended December 31	
	2018	**2017**
Operating expenses		
Research and development	$ 36,310	$ 19,944
General and administrative	14,382	5,085
Total operating expenses	50,692	25,029
Loss from operations	(50,692)	(25,029)
Interest income, net	1,422	44
Other expense, net	(56)	(10)
Total other income (expense)	1,366	34
Net loss	$ (49,326)	$ (24,995)
Net loss per share, basic and diluted	$ (3.15)	$ (15.30)
Weighted-average common shares outstanding - basic and diluted	15,655,293	1,634,100

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

	December 31, 2018	**December 31, 2017**
Assets		
Cash, cash equivalents and marketable securities	$ 175,645	$ 57,959
Other current assets	2,296	$ 1,242
Total current assets	177,941	59,201

Other assets	3,395	2,436
Total assets	$ 181,336	$ 61,637
Liabilities and Stockholders' Equity		
Current liabilities	$ 31,123	$ 5,024
Long-term liabilities	43,590	903
Total liabilities	74,713	5,927
Convertible preferred stock	—	109,232
Total stockholders' equity (deficit)	106,623	(53,522)
Total liabilities, convertible preferred stock and stockholders' equity (deficit)	$ 181,336	$ 61,637

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