# Scholar Rock Reports First Quarter 2019 Financial Results and Highlights Business Progress

May 14, 2019

- *Commenced patient enrollment and dosing in TOPAZ, the Phase 2 clinical trial for SRK-015 in Type 2 and Type 3 Spinal Muscular Atrophy (SMA)*
- *Presented additional preclinical data from the SRK-181 cancer immunotherapy program at the American Association for Cancer Research (AACR) annual meeting demonstrating a highly specific inhibitor of TGFβ1 activation can overcome primary resistance to PD1 blockade across multiple tumor models*
- *Appointed Akshay Vaishnaw, M.D., Ph.D. to Scholar Rock's Board of Directors effective May 21, 2019*

CAMBRIDGE, Mass., May 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 March 31, 2019 and highlighted recent progress and upcoming milestones for its pipeline programs.

"2019 is off to a great start with the initiation of patient dosing in our Phase 2 TOPAZ trial of SRK-015 in SMA and the presentation of additional preclinical data outlining the potential of SRK-181 to expand the number of cancer patients who can benefit from checkpoint blockade therapies," said Nagesh Mahanthappa, Ph.D., President and CEO of Scholar Rock. "We remain focused on advancing these exciting programs while building an organization and team dedicated to the mission of developing novel therapies that can make a meaningful difference in the lives of patients."

**Company Highlights and Upcoming Milestones**

**SRK-015 Program:**

- **Commenced Patient Enrollment and Dosing in the TOPAZ Phase 2 Clinical Trial for SRK-015.** In May, Scholar Rock announced the initiation of patient enrollment and dosing in the 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. 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.

  Preliminary pharmacokinetics (PK) and pharmacodynamics (PD) data from a subset of patients are anticipated by the end of 2019. Interim safety and efficacy results for a subset of patients with six months of treatment exposure are expected in the first half of 2020 with top-line data for the 12-month treatment period expected beginning in the fourth quarter 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:**

- **Presented Additional Preclinical Data Further Supporting the Potential of SRK-181 to Overcome Primary Resistance to Checkpoint Blockade Therapy (CBT).** At the 2019 American Association for Cancer Research (AACR) annual meeting, Scholar Rock presented additional preclinical data showing that co-administration of SRK-181-mIgG1 (murine version of SRK-181) with an anti-PD1 antibody can drive tumor regression or control, resulting in a significant survival benefit compared to anti-PD1 monotherapy. This was demonstrated in the MBT-2 (bladder cancer) and Cloudman S91 (melanoma) mouse models that express TGFβ1, as well as in the EMT6 (breast cancer) mouse model, which expresses both TGFβ1 and TGFβ3 isoforms. Immune response data also revealed that combination treatment leads to infiltration and expansion of CD8+ T cells and a reduction of immunosuppressive myeloid cells, suggesting TGFβ1's multiple contributions to primary resistance to CBT.

- **Plan to Initiate Phase 1 Clinical Trial of SRK-181 in Patients with Solid Tumors 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 plans to develop SRK-181 for the treatment of tumors resistant to CBTs, such as anti-PD(L)1 antibodies, and to initiate a Phase 1

trial in patients with solid tumors in mid-2020.

**First Quarter 2019 Financial Results**

For the quarter ended March 31, 2019, net loss was $10.8 million or $0.42 per share compared to a net loss of $8.9 million or $3.18 per share for the quarter ended March 31, 2018.

- Research and development expense was $10.7 million for the quarter ended March 31, 2019 compared to $6.7 million for the quarter ended March 31, 2018. The increase year-over-year reflects preclinical and manufacturing costs for SRK-181, research costs associated with earlier pipeline programs, and increased personnel-related costs, slightly offset by a decrease year-over-year in manufacturing costs associated with SRK-015.

- General and administrative expense was $4.1 million for the quarter ended March 31, 2019 compared to $2.3 million for the quarter ended March 31, 2018. The increase year-over-year was primarily attributable to increased headcount, stock compensation, and higher operational fees associated with being a public company.

As of March 31, 2019, Scholar Rock had cash, cash equivalents, and marketable securities of $159.7 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.

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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 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 Annual Report on Form 10-K for the quarter ended December 31, 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 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 March 31, | |
|---|---|---|
| | **2019** | **2018** |
| Revenue | $ 3,106 | $ - |
| Operating expenses | | |
| Research and development | 10,739 | 6,701 |
| General and administrative | 4,070 | 2,315 |
| Total operating expenses | 14,809 | 9,016 |
| Loss from operations | (11,703) | (9,016) |
| Interest income, net | 948 | 144 |
| Other expense, net | — | (20) |
| Total other income (expense) | 948 | 124 |
| Net loss | $ (10,755) | $ (8,892) |
| Net loss per share, basic and diluted | $ (0.42) | $ (3.18) |
| Weighted-average common shares outstanding, basic and diluted | 25,592,659 | 2,795,497 |

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

| | **March 31, 2019** | **December 31, 2018** |
|---|---|---|
| **Assets** | | |
| Cash, cash equivalents and marketable securities | $ 159,666 | $ 175,645 |
| Other current assets | 2,362 | 2,296 |
| Total current assets | 162,028 | 177,941 |
| Other assets | 8,954 | 3,395 |
| Total assets | $ 170,982 | $ 181,336 |
| **Liabilities and Stockholders' Equity** | | |
| Current liabilities | $ 34,075 | $ 31,123 |
| Long-term liabilities | 39,384 | 43,590 |
| Total liabilities | 73,459 | 74,713 |
| Total stockholders' equity | 97,523 | 106,623 |
| Total liabilities and stockholders' equity | $ 170,982 | $ 181,336 |




Source: Scholar Rock