# Scholar Rock Reports Second Quarter 2018 Financial Results and Updates on Business Progress

August 8, 2018

- *Completed Initial Public Offering Raising $86.3 Million in Gross Proceeds*
- *Initiated Enrollment in Multiple-Ascending Dose Portion of Phase 1 Clinical Trial for SRK-015*
- *On Track to Initiate Phase 2 Proof-of-Concept Study in Later-Onset SMA in 1Q19*
- *Cash and Cash Equivalents of $115.1 Million as of June 30, 2018*

CAMBRIDGE, Mass., Aug. 08, 2018 (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 second quarter ended June 30, 2018 and highlighted recent progress and upcoming milestones for its pipeline programs.

“Scholar Rock made transformative progress in the second quarter with the successful completion of our IPO and the initiation of the Company’s first clinical trial,” said Nagesh Mahanthappa, Ph.D, President and CEO of Scholar Rock. “We are well-positioned to continue to progress SRK-015 and build our pipeline of future product candidates focused on addressing neuromuscular disorders, cancer, fibrosis, and anemia. We plan to initiate a Phase 2 proof-of-concept study of SRK-015 to improve muscle function in patients with later-onset spinal muscular atrophy (SMA) in the first quarter of 2019.”

**Key Business and Clinical Highlights**

- **Successfully Completed IPO.** In May 2018, Scholar Rock successfully completed an initial public offering (IPO) of 6,164,000 shares of common stock, inclusive of the full exercise of the over-allotment option by the underwriters, raising gross proceeds of approximately $86.3 million.

- **Initiated Enrollment and Dosing in Multiple-Ascending Dose Portion of Phase 1 Clinical Trial for SRK-015.** SRK-015 is a selective inhibitor of the activation of myostatin and was granted Orphan Drug Designation (ODD) by the U.S. Food and Drug Administration (FDA) for the treatment of SMA in March 2018. 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 in healthy adult volunteers. Dosing has completed in the single-ascending dose portion of the study and has advanced to the multiple-ascending dose portion of the study.

**Upcoming Milestones**

- **On Track to Initiate Phase 2 Proof-of-Concept Study for SRK-015 in SMA in the First Quarter of 2019.** Pending supporting 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 later-onset SMA (Type 2 and Type 3) as a monotherapy or in conjunction with an approved survival motor neuron (SMN) upregulator therapy as background standard of care.

- **Identify Second Indication for SRK-015 in the First Half of 2019.** Scholar Rock is actively assessing numerous preclinical models in which the selective inhibition of the activation of myostatin may offer therapeutic benefit. Scholar Rock intends to identify a second indication for SRK-015 in the first half of 2019.

- **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 discovery and development of highly specific inhibitors of the activation of TGFβ1. Scholar Rock is progressing its evaluation of a number of selective inhibitors in multiple disease models and intends to nominate a product candidate and first indication in oncology, immuno-oncology or fibrosis by the end of the first half of 2019.

**Second Quarter 2018 Financial Results**

Net loss for the quarter ended June 30, 2018 was $14.7 million or $1.39 per share compared to a net loss of $5.9 million or $3.67 per unit for the same quarter last year.

- Research and development expense was $11.4 million for the quarter ended June 30, 2018, compared to $4.7 million in the same quarter in 2017. The $6.7 million increase year-over-year was primarily attributable to development and manufacturing costs associated with our lead product candidate, SRK-015, as well as employee costs related to increased headcount.

- General and administrative expense was $3.5 million for the quarter ended June 30, 2018, compared to $1.2 million in the same quarter in 2017. The $2.3 million increase year-over-year was mainly due to increased headcount and higher professional and consulting fees associated with ongoing business activities and operating as a public company.

As of June 30, 2018, Scholar Rock had cash, cash equivalents, and marketable securities of $115.1 million, compared to $58.0 million at the end of 2017. The cash balance is inclusive of the approximately $77.8 million in net proceeds from its IPO in May 2018. Scholar Rock believes the cash 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.

*Scholar Rock® is a registered trademark of Scholar Rock, Inc.*

**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 in SMA, the timing of its Phase 2 Proof-of-Concept study 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 and amount of time Scholar Rock's cash proceeds 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 and resources necessary to identify and develop product candidates on the expected timeline, the data generated from the Phase 1 clinical trial in SMA, competition from others 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 the final prospectus related to Scholar Rock's initial public offering filed with the Securities and Exchange Commission pursuant to Rule 424(b) of the Securities Act, 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**
**CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS**
**(Unaudited)**
**(In thousands, except unit, share, per unit and per share data)**

| | Three Months Ended June 30, | | Six Months Ended June 30, | |
|---|---|---|---|---|
| | 2018 | 2017 | 2018 | 2017 |
| Operating expenses: | | | | |
| Research and development | 11,424 | 4,711 | 18,125 | 8,279 |
| General and administrative | 3,460 | 1,177 | 5,775 | 2,350 |
| Total operating expenses | 14,884 | 5,888 | 23,900 | 10,629 |
| Loss from operations | (14,884) | (5,888) | (23,900) | (10,629) |
| Other income (expense): | | | | |
| Interest income, net | 213 | 11 | 357 | 23 |
| Other expense, net | (36) | (2) | (56) | (3) |
| Total other income | 177 | 9 | 301 | 20 |
| Net loss | $ (14,707) | $ (5,879) | $ (23,599) | $ (10,609) |
| Net loss per unit, basic and diluted | | $ (3.67) | | $ (6.62) |
| Net loss per share, basic and diluted | $ (1.39) | | $ (3.51) | |
| Weighted average common units outstanding, basic and diluted | | 1,603,088 | | 1,603,088 |
| Weighted average common shares outstanding, basic and diluted | 10,593,987 | | 6,716,283 | |
| | | | | |
| Comprehensive loss: | | | | |
| Net loss | $ (14,707) | $ (5,879) | $ (23,599) | $ (10,609) |
| Other comprehensive income: | | | | |
| Unrealized gain on marketable securities | 2 | 4 | 2 | 1 |
| Total other comprehensive income | 2 | 4 | 2 | 1 |
| Comprehensive loss | $ (14,705) | $ (5,875) | $ (23,597) | $ (10,608) |

**SCHOLAR ROCK HOLDING CORPORATION**
**CONSOLIDATED BALANCE SHEETS**
**(Unaudited)**
**(In thousands, except unit, share, and per share data)**

| | June 30, 2018 | December 31, 2017 |
|---|---|---|
| **Assets** | | |
| Current assets: | | |
| Cash and cash equivalents | $ 115,061 | $ 56,461 |
| Marketable securities | — | 1,498 |
| Prepaid expenses and other current assets | 1,856 | 1,242 |
| Total current assets | 116,917 | 59,201 |
| Property and equipment, net | 1,856 | 2,181 |
| Restricted cash | 205 | 205 |
| Other long-term assets | — | 50 |
| Total assets | $ 118,978 | $ 61,637 |
| **Liabilities, Convertible Preferred Stock and Stockholders' Equity (Deficit)** | | |
| Current liabilities: | | |
| Accounts payable | $ 2,598 | $ 1,359 |
| Accrued expenses | 3,356 | 2,796 |
| Deferred rent | — | 228 |
| Loan payable | 731 | 641 |
| Total current liabilities | 6,685 | 5,024 |
| Long-term portion of deferred rent | 681 | 468 |
| Long-term portion of loan payable | — | 398 |
| Warrant to purchase redeemable security | — | 37 |
| Total liabilities | 7,366 | 5,927 |

| | | |
|---|---|---|
| Commitments and contingencies (Note 9) | | |
| Convertible preferred stock (Series A-1, A-2, A-3, A-4, B and C), $0.001 par value; no shares authorized, issued or outstanding as of June 30, 2018; 43,157,651 shares authorized and 43,135,911 shares issued and outstanding as of December 31, 2017 (aggregate liquidation preference of $109,561 as of December 31, 2017) | — | 109,232 |
| Stockholders' equity (deficit): | | |
| Preferred stock, $0.001 par value; 10,000,000 and no shares authorized at June 30, 2018 and December 31, 2017, respectively; no shares issued and outstanding at June 30, 2018 and December 31, 2017, respectively | — | — |
| Common stock, $0.001 par value; 150,000,000 shares authorized and 25,243,684 shares issued and outstanding as of June 30, 2018, respectively; 60,000,000 shares authorized and 3,970,586 shares issued and outstanding as of December 31, 2017, respectively | 25 | 4 |
| Additional paid-in capital | 192,711 | 4,001 |
| Accumulated other comprehensive loss | — | (2) |
| Accumulated deficit | (81,124) | (57,525) |
| Total stockholders' equity (deficit) | 111,612 | (53,522) |
| Total liabilities, convertible preferred stock and stockholders' equity (deficit) | $ 118,978 | $ 61,637 |

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Source: Scholar Rock