Scholar Rock Reports First Quarter 2022 Financial Results and Updates Strategic Priorities

May 16, 2022

- On track for Phase 2 apitegromab TOPAZ two-year data by mid-year

- Progressed enrollment for both Phase 3 apitegromab SAPPHIRE clinical trial & Phase 1 SRK-181 DRAGON clinical trial

- Restructuring reduces operating expenses, prioritizes R&D activity on completing SAPPHIRE trial and extends cash runway into the fourth quarter of 2023

- Yung Chyung, MD, has decided to step down as Chief Medical Officer effective June 30, 2022

CAMBRIDGE, Mass.--(BUSINESS WIRE)--May 16, 2022-- Scholar Rock (NASDAQ: SRRK), a Phase 3 clinical-stage biopharmaceutical company focused on the treatment of serious diseases in which protein growth factors play a fundamental role, today provided a business update and reported financial results for the first quarter ended March 31, 2022.

Business Strategy & Update

- **Prioritize R&D Activities to Support Progression and Completion of the Apitegromab Phase 3 SAPPHIRE Trial in Spinal Muscular Atrophy.** Scholar Rock is focusing its expertise in inhibiting the activation of latent growth factors on completing the Phase 2 TOPAZ extension clinical trial and the Phase 3 SAPPHIRE clinical trial, both of which are evaluating the potential of apitegromab to address motor function impairments in patients with spinal muscular atrophy (SMA). The SRK-181 program for the treatment of patients with solid tumors showing primary resistance to anti-PD-(L)1 therapy remains a priority with strategic value. While investment in the existing scientific platform continues, the Company is narrowing the scope of discovery programs it expects to pursue.
- **Focused Strategy to Increase Operational Efficiency, Reduce Cash Burn and Extend Runway.** The Company today announced a restructuring of its business to prioritize and focus on its clinical stage assets resulting in a reduction to its workforce by approximately 25%, in addition to the curtailment of previously planned hiring. The reduction in operating expenses relates to general and administrative spend and research activities related to earlier stage programs. In conjunction with the pipeline prioritization, these changes are expected to extend the cash runway into the fourth quarter of 2023.

“We are highly confident in the transformative potential of apitegromab based upon the Phase 2 TOPAZ data we have released to date. We look forward to presenting two-year extension trial data in the coming weeks,” said Nagesh Mahanthappa, Ph.D., Founding Chief Executive Officer of Scholar Rock. “Based upon the strength of the data released to date and our commitment to the SMA community, this narrower focus and associated headcount reduction is necessary to execute our core mission -- completing the Phase 3 SAPPHIRE trial to bring apitegromab, our highly innovative therapeutic candidate, to patients suffering with this devastating disease.”

Dr. Mahanthappa continued, “Parting with employees who have dedicated their talent and expertise to evolving a scientific platform into a Phase 3-stage company is very difficult, and we are extremely grateful for their contributions to our mission to support patients and help position us to potentially deliver the next big innovation to patients with SMA.”

Changes to Development Leadership

The Company also announced today that Yung Chyung, M.D. has decided to step down as Chief Medical Officer (CMO) to explore new career opportunities, including his interest in global health. Dr. Chyung will remain with Scholar Rock through June 30 to work with the executive team and the development organization on a transition plan, and a retained search for a new CMO is ongoing.

“I am incredibly proud of the progress we’ve achieved to date at Scholar Rock and am humbled to have served alongside the amazing and talented employees across the company,” said Dr. Chyung. “I am enthusiastic about the potential of apitegromab and SRK-181 to transform the lives of individuals impacted by serious diseases. With operational momentum continuing to build and the outstanding, high performing teams in place, I feel this is an appropriate time for me to explore my other long-term career interests, such as global health.”

“The Board and I sincerely thank Yung for his ingenuity and leadership over the years, which have brought the apitegromab program forward through a successful Phase 2 proof-of-concept study and now into Phase 3, creating hope for patients and families suffering from SMA that muscle-directed therapy could have the potential to make a meaningful impact on their lives,” said Dr. Mahanthappa. “While the search for a new CMO is a top priority and we are committed to identifying a medical leader with a strong track record in late-stage drug development and product launch, the significant depth of talent in our development team allows us to continue to build momentum in the SAPPHIRE pivotal and Phase 1 DRAGON proof-of-concept clinical trials without disruption.”

Pipeline Updates

Apitegromab is a selective inhibitor of myostatin activation being developed as the potential first muscle-directed therapy for the treatment of spinal muscular atrophy (SMA).

- **Enrollment Ongoing for Phase 3 SAPPHIRE Clinical Trial Evaluating Apitegromab in Patients with Non-Ambulatory Type 2 and 3 SMA.** The randomized, double-blind, placebo-controlled Phase 3 SAPPHIRE clinical trial is evaluating apitegromab for patients on either nusinersen or risdiplam. The clinical trial design plans for approximately 156 patients aged 2-12 years old with non-ambulatory Type 2 and 3 SMA to be enrolled in the main efficacy population.
- **Clinical Data from the Phase 2 TOPAZ 24-Month Extension Trial to be Presented at Cure SMA Annual Meeting in June**.
- **Data from the Phase 2 TOPAZ Trial Presented at the Muscular Dystrophy Association (MDA) Clinical & Scientific Conference and the American Academy of Neurology Annual Meeting in March, as well as the European Paediatric Neurology Society Congress in April.**

SRK-181 is a selective inhibitor of latent TGFβ1 activation being developed with the aim of overcoming primary resistance to and increasing the number of patients who may benefit from checkpoint inhibitor therapy.

- **Enrollment Ongoing for Part B of the Phase 1 DRAGON POC Clinical Trial for SRK-181.** Part B consists of multiple proof-of-concept cohorts focused upon evaluating the ability of SRK-181 to overcome primary resistance to anti-PD-(L)1 therapy. Each cohort will enroll up to 40 patients with various solid tumors, including urothelial carcinoma (UC), cutaneous melanoma (MEL), non-small cell lung cancer (NSCLC), clear cell renal cell carcinoma (ccRCC), and other solid tumors. The biomarker strategy in part B of DRAGON will explore early signs of SRK-181 activity, including target engagement and pathway modulation. This will include measuring effects on both circulating and tumor immune contexture, such as CD8+ T cell infiltration and reductions in myeloid-derived suppressor cell (MDSC) populations as well as analysis of TGFβ-related pathway signaling. Initial evidence of drug activity and safety data are anticipated in 2022.

First Quarter 2022 Financial Results

For the quarter ended March 31, 2022, net loss was $8.0 million or $0.21 per share compared to a net loss of $27.7 million or $0.76 per share for the quarter ended March 31, 2021.

- Revenue was $33.2 million for the quarter ended March 31, 2022 compared to $4.7 million for the quarter ended March 31, 2021 and was related to the Gilead fibrosis-focused research collaboration, which was executed in December 2018 and concluded in December 2021.
- Research and development expense was $29.4 million for the quarter ended March 31, 2022 compared to $22.5 million for the quarter ended March 31, 2021. The increase year-over-year primarily reflects increased clinical costs associated with apitegromab and higher personnel costs. The Company expects research expenses to decline beginning in the third quarter of 2022 due to the portfolio updates and workforce reduction announced today.
- General and administrative expense was $10.8 million for the quarter ended March 31, 2022 compared to $9.4 million for the quarter ended March 31, 2021. The increase year-over-year was primarily attributed to higher personnel costs. As a result of our reduction in force, we expect our employee-related expenses to decline in the second half of the year as compared to the first half of 2022.
- As of March 31, 2022, Scholar Rock had cash, cash equivalents, and marketable securities of approximately $210 million. The Company expects that the updates and changes announced today will fund its anticipated operating and capital expenditure requirements into the fourth quarter of 2023.

“While we have paused many of our discovery programs, we are continuing to progress selected pre-clinical programs which best exemplify the value of our platform. As a platform company, business development remains an important part of our strategy and we continue to explore partnerships for these programs which could be a source of non-dilutive capital in the future,” said Ted Myles, Chief Operating Officer and Chief Financial Officer at Scholar Rock. “Our streamlined focus and structure allow us to channel the majority of our resources to supporting the SAPPHIRE trial and SRK-181, which we believe are key near- and long-term value drivers.”

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, and fibrosis. Scholar Rock’s approach to targeting 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/). Investors and others should note that we communicate with our investors and the public using our company website www.scholarrock.com, including, but not limited to, company disclosures, investor presentations and FAQs, Securities and Exchange Commission filings, press releases, public conference call transcripts and webcast transcripts, as well as on Twitter and LinkedIn. The information that we post on our website or on Twitter or LinkedIn could be deemed to be material information. As a result, we encourage investors, the media and others interested to review the information that we post there on a regular basis. The contents of our website or social media shall not be deemed incorporated by reference in any filing under the Securities Act of 1933, as amended.

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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 apitegromab, SRK-181, and other product candidates and indication selection and development timing, its cash runway, the ability of any product candidate to perform in humans in a manner consistent with earlier nonclinical, preclinical or clinical trial data, and the potential of its product candidates and proprietary platform. The use of words such as "may," "might," "could," "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, without limitation, that preclinical and clinical data, including the results from the Phase 2 clinical trial of apitegromab, or Part A of the Phase 1 clinical trial of SRK-181, are not predictive of, may be inconsistent with, or more favorable than, additional data generated from clinical trials of the same product candidate, including, without limitation, the TOPAZ extension clinical trial and the Phase 3 SAPPHIRE clinical trial of apitegromab in SMA or Part B of the Phase 1 DRAGON clinical trial of SRK-181, 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, information provided or decisions made by regulatory authorities, 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, without limitation, to supply any clinical trials, 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, and the impacts of public health pandemics such as COVID-19 on business operations and expectations, 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 March 31, 2022, 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)

	Three Months Ended March 31	
	2022	**2021**
Revenue	$ 33,193	$ 4,708
Operating expenses		
Research and development	29,366	22,549
General and administrative	10,760	9,366
Total operating expenses	40,126	31,915
Loss from operations	(6,933)	(27,207)
Other income (expense), net	(1,017)	(464)
Net loss	$ (7,950)	$ (27,671)
Net loss per share, basic and diluted	$ (0.21)	$ (0.76)
Weighted average common shares outstanding, basic and diluted	37,456,574	36,380,438

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

	March 31, 2022	**December 31, 2021**
Assets		
Cash, cash equivalents and marketable securities	$ 210,211	$ 252,994
Other current assets	18,375	12,325
Total current assets	228,586	265,319
Other assets	37,391	39,126
Total assets	$ 265,977	$ 304,445

Liabilities and Stockholders' Equity		
Current liabilities	$ 33,373	$ 64,297
Long-term liabilities	61,288	68,074
Total liabilities	94,661	132,371
Total stockholders' equity	171,316	172,074
Total liabilities and stockholders' equity	$ 265,977	$ 304,445

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