Scholar Rock Reports Third Quarter 2023 Financial Results and Highlights Business Progress

November 7, 2023

- *Completed enrollment for pivotal Phase 3 SAPPHIRE trial evaluating apitegromab; topline data expected in 4Q 2024*
- *Expanding into cardiometabolic disorders with SRK-439, a novel investigational myostatin inhibitor*
- *Presented new data supporting proof of concept for SRK-181 in heavily pretreated patients with resistant metastatic ccRCC; completing enrollment of the Phase 1 DRAGON trial in December 2023*
- *Completed $98 million public offering, extending projected cash runway into second half of 2025*

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Nov. 7, 2023-- 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 reported financial results and corporate updates for the third quarter ended September 30, 2023.

“We have made significant progress across our pipeline over the last quarter, including completing enrollment of our Phase 3 SAPPHIRE trial of apitegromab, which was designed to build on the positive Phase 2 TOPAZ results. Based on the sustained improvement in motor function that was observed after 36 months of treatment in the TOPAZ trial, the favorable safety and tolerability profile, and the high continued participation rate in our long-term extension study, we believe apitegromab has the potential to be a transformative therapy for SMA patients,” said Jay Backstrom, M.D., MPH, President and Chief Executive Officer of Scholar Rock. “Further, we are excited to leverage our expertise in selective myostatin inhibition and expand into cardiometabolic disorders, including obesity. We believe our highly selective myostatin inhibitor SRK-439 has the potential to help patients retain lean muscle mass, which has been a key obstacle for many on GLP-1 receptor agonist therapy, and we plan to file an IND in 2025 to initiate clinical testing of SRK-439 in combination with GLP-1 receptor agonists.”

“Additionally, we recently presented new clinical and biomarker data from the Phase 1 DRAGON trial that demonstrated the therapeutic potential of SRK-181 in heavily pretreated patients with anti-PD-1 resistant clear cell renal cell carcinoma. We believe that the DRAGON trial has achieved the objective of establishing proof of concept that our highly selective approach to blocking latent TGFβ1 can restore sensitivity to a checkpoint inhibitor, most notably in those with anti-PD-1 resistant ccRCC and supports further development of SRK-181,” he added.

Recent Company Highlights and Upcoming Milestones

Spinal Muscular Atrophy (SMA) Program

Apitegromab is an investigational fully human monoclonal antibody that inhibits myostatin activation by selectively binding the pro- and latent forms of myostatin in skeletal muscle and is being developed as a potential first muscle-targeted therapy for the treatment of SMA.

- **Completed enrollment of its pivotal Phase 3 SAPPHIRE clinical trial.** The Company announced that it completed enrollment in September 2023, with topline data expected in the fourth quarter of 2024. If successful and if approved, the Company expects to initiate a commercial product launch in 2025.
- **Presented encore 36-month extension data from the Phase 2 TOPAZ trial at the 28th Annual Congress of the World Muscle Society in October.** The Company presented encore data from its Phase 2 TOPAZ trial extension period evaluating patient outcomes in SMA after 36 months of treatment of apitegromab, which showed long-term sustained improvements in motor function and in patient-reported outcome measures in patients with nonambulatory Types 2 and 3 SMA receiving survival motor neuron (SMN) therapy.
- **ONYX long-term extension study for patients from both the TOPAZ and SAPPHIRE studies remains ongoing.**

Immuno-Oncology Program

SRK-181 is an investigational selective inhibitor of latent TGFβ1 activation and is being developed with the aim of overcoming resistance to checkpoint therapy in patients with advanced cancer.

- **Presented clinical and biomarker data from Phase 1 DRAGON proof-of-concept trial at the SITC 38th Annual Meeting.** The Company presented new data from its Phase 1 DRAGON proof-of-concept trial. As of the August 29, 2023 data cutoff, SRK-181 data showed favorable tolerability and promising anti-tumor activity in heavily pretreated patients with clear cell renal cell carcinoma (ccRCC) resistant to anti-PD-1. Of 28 evaluable patients in the ccRCC cohort, six patients had confirmed partial responses and achieved a best tumor reduction of 33% to 93%. The objective response rate (ORR) was 21.4% and the disease control rate was 57%. Circulating granulocytic myeloid-derived suppressor cell (gMDSC) levels correlated with better clinical responsiveness in ccRCC patients treated with SRK-181 in combination with pembrolizumab. The Company believes these data from the Phase 1 DRAGON trial supports proof of concept and that the trial has met its primary objectives. The Company is completing enrollment of the trial in December 2023, while continuing to treat patients who remain on study.

Cardiometabolic Program

SRK-439 is a novel, preclinical investigational myostatin inhibitor that has high *in vitro* affinity for pro- and latent myostatin, maintenance of myostatin specificity (i.e., no GDF11 or Activin-A binding), and is initially being developed for the treatment of obesity.

- **Plans to initiate a Phase 2 proof-of-concept trial with apitegromab in combination with a GLP-1 receptor agonist (GLP-1 RA) in obesity in 2024.** As part of the Company's strategy to advance the development of SRK-439, it plans to initiate a Phase 2 proof-of-concept trial with apitegromab in combination with a GLP-1 RA in 2024, subject to IND clearance. Data from the clinical trial are expected in mid-2025 and will be used to inform further clinical development of SRK-439. The Company plans to file an IND for SRK-439 for the treatment of obesity in 2025.

Third Quarter 2023 Financial Results

For the quarter ended September 30, 2023, net loss was $42.4 million compared to a net loss of $43.3 million for the quarter ended September 30, 2022.

- The Company did not record any revenue for either the quarter ended September 30, 2023 or September 30, 2022.
- Research and development expense was $30.3 million for the quarter ended September 30, 2023, compared to $33.4 million for the quarter ended September 30, 2022.
- General and administrative expense was $13.3 million for the quarter ended September 30, 2023, compared to $10.5 million for the quarter ended September 30, 2022.
- As of September 30, 2023, Scholar Rock had cash, cash equivalents, and marketable securities of approximately $218.6 million, which in addition to approximately $92.5 million of net proceeds from the October 2023 equity offering, is projected to fund the Company's anticipated operating and capital expenditure requirements into the second half of 2025.

"Our upsized public offering achieved two key objectives: it enables us to bring our expertise to the cardiometabolic and obesity space with SRK-439, a highly selective myostatin inhibitor, and it extends our cash runway well past our upcoming SAPPHIRE Phase 3 data read out in Q4 2024," said Ted Myles, Chief Operating Officer and Chief Financial Officer of Scholar Rock. "We are executing against our plan and we are well positioned going into 2024."

Conference Call and Webcast
The Company's earnings conference call for the third quarter will be held at 8:00 a.m. ET on November 7, 2023. To access the conference call by phone, participants may register here to receive the dial-in number and unique PIN. A live webcast of the conference call will be available on the Investors & Media section of the Scholar Rock website at http://investors.scholarrock.com. An archived replay of the webcast will be available for approximately 90 days following the call.

About the Phase 3 SAPPHIRE Trial
SAPPHIRE is an ongoing randomized, double-blind, placebo-controlled, Phase 3 clinical trial evaluating the safety and efficacy of apitegromab in nonambulatory patients with Types 2 and 3 SMA who are receiving SMN-targeted therapy (either nusinersen or risdiplam). SAPPHIRE targeted enrolling approximately 156 patients aged 2-12 years old in the main efficacy population. These patients were randomized 1:1:1 to receive for 12 months either apitegromab 10 mg/kg, apitegromab 20 mg/kg, or placebo by intravenous (IV) infusion every 4 weeks. An exploratory population that targeted enrolling up to 48 patients aged 13-21 years old will also separately be evaluated. These patients were randomized 2:1 to receive either apitegromab 20 mg/kg or placebo. For more information about SAPPHIRE, visit www.clinicaltrials.gov. Apitegromab has not been approved for any use by the US FDA or any other health authority, and its safety and efficacy have not been established.

About SRK-181 & the Phase 1 DRAGON Proof-of-Concept Trial
SRK-181 is a selective inhibitor of TGFβ1 activation being developed to overcome primary resistance to checkpoint inhibitor therapy, such as anti-PD-(L)1 antibodies, in advanced cancer. TGFβ1 is the predominant TGFβ isoform expressed in many human tumor types. Based on analyses of various human tumors that are resistant to anti-PD-(L)1 therapy, data suggest that TGFβ1 is a key contributor to the immunosuppressive tumor microenvironment, excluding and preventing entry of cytotoxic T cells into the tumor, thereby inhibiting anti-tumor immunity. [1]

SRK-181 specifically targets the latent TGFβ1 isoform in a context-independent manner, designed to enable complete inhibition of TGFβ1 in all compartments within the tumor microenvironment. Scholar Rock believes that SRK-181 has the potential to overcome this immune cell exclusion and induce tumor regression when administered in combination with anti-PD-(L)1 therapy while potentially avoiding toxicities associated with non-selective TGFβ inhibition. The Phase 1 DRAGON proof-of-concept clinical trial (NCT04291079) in patients with locally advanced or metastatic solid tumors is ongoing. The trial is currently enrolling and dosing patients in multiple proof of concept cohorts conducted in parallel, including urothelial carcinoma (UC), cutaneous melanoma (MEL), non-small cell lung cancer (NSCLC), head and neck squamous cell carcinoma (HNSCC), and clear cell renal cell carcinoma (ccRCC). SRK-181 is an investigational product candidate, and its efficacy and safety have not been established. SRK-181 has not been approved for any use by the FDA or any other regulatory agency.

[1]Martin et al., Sci. Transl. Med. 12: 25 March 2020

About Scholar Rock
Scholar Rock is a biopharmaceutical company that discovers, develops, and delivers life-changing therapies for people with serious diseases that have high unmet need. As a global leader in the biology of the transforming growth factor beta (TGFβ) superfamily of cell proteins and named for the visual resemblance of a scholar rock to protein structures, the clinical-stage company is focused on advancing innovative treatments where protein growth factors are fundamental. Over the past decade, Scholar Rock has created a pipeline with the potential to advance the standard of care for neuromuscular disease, cardiometabolic disorders, cancer, and other conditions where growth factor-targeted drugs can play a transformational role.

Scholar Rock is the only company to show clinical proof of concept for a muscle-targeted treatment in spinal muscular atrophy (SMA). This commitment to unlocking fundamentally different therapeutic approaches is powered by broad application of a proprietary platform, which has

developed novel monoclonal antibodies to modulate protein growth factors with extraordinary selectivity. By harnessing cutting-edge science in disease spaces that are historically under-addressed through traditional therapies, Scholar Rock works every day to create new possibilities for patients. Learn more about the our approach at ScholarRock.com and follow @ScholarRock and on LinkedIn.

Availability of Other Information About 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.

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 growth, strategy, progress and timing of its clinical trials for apitegromab and SRK-181 and its preclinical programs, including SRK-439, and indication selection and development timing, including the therapeutic potential, clinical benefits and safety thereof, expectations regarding timing, success and data announcements of current ongoing preclinical and clinical trials, its cash runway, expectations regarding the achievement of important milestones, 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 or Part B of the Phase 1 clinical trial of SRK-181, are not predictive of, may be inconsistent with, or more favorable than, data generated from future or ongoing clinical trials of the same product candidates, including, without limitation, the Phase 3 clinical trial of apitegromab in SMA or Part B of the Phase 1 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 June 30, 2023, 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 September 30		Nine Months Ended September 30	
	2023	2022	2023	2022
Revenue	$ —	$ —	$ —	$ 33,193
Operating expenses				
Research and development	30,337	33,392	86,939	94,831
General and administrative	13,335	10,470	36,324	32,304
Total operating expenses	43,672	43,862	123,263	127,135
Loss from operations	(43,672)	(43,862)	(123,263)	(93,942)
Other income (expense), net	1,313	565	3,600	(1,305)
Net loss	$ (42,359)	$ (43,297)	$ (119,663)	$ (95,247)
Net loss per share, basic and diluted	$ (0.53)	$ (0.55)	$ (1.49)	$ (1.80)
Weighted average common shares outstanding, basic and diluted	80,606,438	79,336,161	80,115,143	52,958,447

Scholar Rock Holding Corporation
Condensed Consolidated Balance Sheets

(unaudited)
(in thousands)

	September 30, 2023	December 31, 2022
Assets		
Cash, cash equivalents and marketable securities	$ 218,635	$ 315,361
Other current assets	9,830	12,663
Total current assets	228,465	328,024
Other assets	22,224	30,144
Total assets	$ 250,689	$ 358,168
Liabilities and Stockholders' Equity		
Current liabilities	$ 26,730	$ 36,389
Long-term liabilities	56,294	61,544
Total liabilities	83,024	97,933
Total stockholders' equity	167,665	260,235
Total liabilities and stockholders' equity	$ 250,689	$ 358,168

View source version on businesswire.com: https://www.businesswire.com/news/home/20231107339655/en/

Investors & Media
Rushmie Nofsinger
Scholar Rock
rnofsinger@scholarrock.com
ir@scholarrock.com
857-259-5573

Source: Scholar Rock, Inc.