Scholar Rock Reports First Quarter 2024 Financial Results and Highlights Business Progress

May 7, 2024

- On track to report topline data from pivotal Phase 3 SAPPHIRE trial in patients with Spinal Muscular Atrophy in 4Q 2024

- Initiating Phase 2 proof-of-concept trial evaluating apitegromab in obesity in June 2024

- Updated SRK-181 clinical data selected for oral presentation at American Society of Clinical Oncology (ASCO)

- New SRK-439 preclinical data selected for oral presentation at American Diabetes Association (ADA)

- Cash, cash equivalents and marketable securities of $238 million as of March 31, 2024; expected to support runway into 2H 2025

CAMBRIDGE, Mass.--(BUSINESS WIRE)--May 7, 2024-- Scholar Rock (NASDAQ: SRRK), a late-stage biopharmaceutical company focused on advancing innovative treatments for spinal muscular atrophy (SMA), cardiometabolic disorders, and other serious diseases where protein growth factors play a fundamental role, today reported financial results and corporate updates for the first quarter ended March 31, 2024.

“Scholar Rock is now only two quarters away from reporting topline data for our pivotal Phase 3 SAPPHIRE trial, which has the potential to build upon the promising data generated from our prior Phase 2 trial,” said Jay Backstrom, M.D., MPH, President & Chief Executive Officer of Scholar Rock. “As we look towards a potential commercial launch of apitegromab in SMA in 2025, we’re thrilled to be on the precipice of delivering a new class of treatment with potential to meaningfully impact people living with SMA.”

Dr. Backstrom continued, “In parallel, we continue to make important progress with our cardiometabolic program. As we advance SRK-439 towards IND, we plan to present new preclinical data at upcoming conferences that further support SRK-439’s differentiated product profile. Additionally, we remain on track to open enrollment in June for our Phase 2 proof-of-concept trial evaluating apitegromab in combination with GLP-1 agonist treatment in obesity, which is designed to assess the effect of our selective approach to blocking myostatin to preserve lean muscle mass as part of healthy weight management. As a leader in selective myostatin inhibition, it is a priority for us to apply our expertise in the cardiometabolic arena, where safe preservation of lean muscle mass is important.”

Company Highlights and Upcoming Milestones

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. Apitegromab is the only muscle-targeted therapy to show clinical proof-of-concept in SMA.

- **On track to report topline data from Phase 3 SAPPHIRE clinical trial in 4Q 2024**. If the trial is successful and apitegromab is approved, the Company expects to initiate a commercial product launch in 2025.
- **ONYX long-term extension study ongoing for patients from both the TOPAZ and SAPPHIRE studies.** More than 90% of patients on combination therapy have completed 4 years of apitegromab treatment and enrolled into ONYX.

Cardiometabolic Program

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

- **Initiating a Phase 2 proof-of-concept trial with apitegromab in combination with a GLP-1 receptor agonist (GLP-1 RA) in obesity in June.** The Phase 2 trial is a randomized, double-blind, placebo-controlled, multi-center study to evaluate the effect of apitegromab, a highly selective myostatin inhibitor, to preserve lean muscle mass as an adjunctive therapy in overweight and obese adults who are taking a GLP-1 RA. Data is expected in mid-2025 and will be used to guide clinical development of SRK-439. The Company plans to file an IND for SRK-439 for the treatment of obesity in 2025.
- **Preclinical data from the SRK-439 program selected for an oral presentation during the American Diabetes Association 84th Scientific Sessions being held June 21-24, 2024 in Orlando, Florida.** The presentation will include updates on SRK-439 and will be held on June 23 at 2:45 p.m. ET.

Immuno-Oncology Program

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

- **New SRK-181 data from the Phase 1 DRAGON proof-of-concept trial selected for an oral presentation at the ASCO Annual Meeting being held May 31- June 4, 2024 in Chicago.** Enrollment of the DRAGON trial was completed in December 2023, and patients who remain on the study continue to be treated. The presentation, "Phase 1 study (DRAGON) of SRK-181 (linavonkibart), a latent TGFβ1 inhibitor, combined with pembrolizumab in anti-PD1 resistant patients with advanced solid tumors: updated results of expansion part," will be presented on June 3 at 1:50 p.m. CT/2:50 p.m. ET. The Company will hold a webcast to discuss the data from the ASCO presentation on June 4 at 8:00 a.m. ET.

Corporate

- **Company hosting Investor & Analyst Day on May 22, 2024 in New York City.** Event to highlight the Company's myostatin inhibition programs in SMA and obesity.

First Quarter 2024 Financial Results

For the quarter ended March 31, 2024, net loss was $56.9 million or $0.59 per share compared to a net loss of $39.4 million or $0.49 per share for the quarter ended March 31, 2023.

- The Company did not record any revenue for the quarter ended March 31, 2024 or for the quarter ended March 31, 2023.
- Research and development expense was $43.1 million for the quarter ended March 31, 2024, compared to $29.7 million for the quarter ended March 31, 2023. The increase was primarily attributable to clinical trial and research study costs.
- General and administrative expense was $15.3 million for the quarter ended March 31, 2024, compared to $10.8 million for the quarter ended March 31, 2023. The increase was due to employee-related costs.
- As of March 31, 2024, Scholar Rock had cash, cash equivalents, and marketable securities of approximately $238 million, which is expected to fund the Company's anticipated operating and capital expenditure requirements into the second half of 2025.

"We are acutely focused on execution in 2024 as we prepare for the pivotal Phase 3 readout of apitegromab in SMA and continue to progress our cardiometabolic program through clinical development. Our cash position enables us to reach multiple upcoming key milestones as we prepare for our next phase of growth," said Ted Myles, Chief Operating Officer and Chief Financial Officer of Scholar Rock.

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-439

SRK-439 is a novel, preclinical, investigational myostatin inhibitor that has high in vitro affinity for pro- and latent myostatin and maintains myostatin specificity (i.e., no GDF11 or Activin-A binding), and is initially being developed for the treatment of obesity. Based on preclinical data, SRK-439 has the potential to support healthier weight management by preserving lean mass. The efficacy and safety of SRK-439 have not been established and SRK-439 has not been approved for any use by the FDA or any other regulatory agency.

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 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.

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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 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; 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 March 31, 2024, 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	
	2024	**2023**
Operating expenses		
Research and development	$ 43,094	$ 29,735
General and administrative	15,325	10,774
Total operating expenses	58,419	40,509
Loss from operations	(58,419)	(40,509)
Other income (expense), net	1,566	1,130
Net loss	$ (56,853)	$ (39,379)

Net loss per share, basic and diluted	$ (0.59)	$ (0.49)
Weighted average common shares outstanding, basic and diluted	95,892,601	79,610,059

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

	March 31, 2024	December 31, 2023
Assets		
Cash, cash equivalents and marketable securities	$ 238,432	$ 279,938
Other current assets	8,501	8,256
Total current assets	246,933	288,194
Other assets	20,352	22,841
Total assets	$ 267,285	$ 311,035
Liabilities and Stockholders' Equity		
Current liabilities	$ 37,506	$ 32,741
Long-term liabilities	47,006	53,076
Total liabilities	84,512	85,817
Total stockholders' equity	182,773	225,218
Total liabilities and stockholders' equity	$ 267,285	$ 311,035

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