Scholar Rock Reports Full Year 2023 Financial Results and Highlights Business Progress

March 19, 2024

- Pivotal Phase 3 SAPPHIRE trial in patients with Spinal Muscular Atrophy on track to report topline data in 4Q 2024

- Announced FDA clearance of IND application to initiate Phase 2 proof-of-concept trial with apitegromab to treat obesity; expected to commence in mid-2024

- Presented preclinical data supporting potential benefit of SRK-439 to preserve lean muscle mass as part of healthier weight management at Keystone Symposia

- Ended 2023 with approximately $280 million in cash, cash equivalents, and marketable securities, which is expected to fund operations into 2H 2025

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Mar. 19, 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 full year ended December 31, 2023.

“In 2023, Scholar Rock made significant progress across our industry-leading antimyostatin pipeline, and we are poised for a transformational year ahead. We remain on track to release topline data for our Phase 3 SAPPHIRE trial of apitegromab in spinal muscular atrophy in the fourth quarter of 2024, which if successful, will be the foundation of our neuromuscular franchise. Additionally, we remain on track to initiate a Phase 2 proof-of-concept trial with apitegromab in combination with a GLP-1 RA in mid-2024, as we continue to move SRK-439, our novel preclinical myostatin inhibitor for obesity, toward IND,” said Jay Backstrom, M.D., M.P.H., President & Chief Executive Officer of Scholar Rock. “As the only company to show clinical proof of concept in SMA with our selective antimyostatin approach, we are well positioned to further extend our leadership with a potential launch in SMA and clinical data in obesity anticipated in 2025."

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

- **On track to announce topline data from the Phase 3 SAPPHIRE clinical trial in 4Q 2024**. In September 2023, the Company announced that it completed enrollment of SAPPHIRE. Scholar Rock remains on track to report topline data from the Phase 3 trial in the fourth quarter of 2024. If the trial is successful and apitegromab is approved, the Company expects to initiate a commercial product launch in 2025.
- **Presented two posters at the Muscular Dystrophy Association Conference 2024 (MDA) in March 2024**. Earlier this month, Scholar Rock presented 36-month extension data from the Phase 2 TOPAZ trial at MDA showing long-term substantial and sustained improvements in motor function and improvements in patient- and caregiver-reported outcomes in patients with nonambulatory types 2 and 3 SMA receiving survival motor neuron (SMN) therapy. Scholar Rock also presented a literature review that describes the ongoing burden and significant unmet needs in SMA despite advancements in treatment. In addition, the Company hosted a “Muscle Matters in SMA” lunch symposium highlighting the experiences of a person living with SMA, a neurologist, and a physical therapist.
- **12-month data from Phase 2 TOPAZ trial published in *Neurology*.** These data demonstrated that apitegromab led to improved motor function in participants with types 2 and 3 SMA, supporting further study of apitegromab in the pivotal Phase 3 SAPPHIRE trial. The full manuscript titled, “Safety and Efficacy of Apitegromab in Patients With Spinal Muscular Atrophy Types 2 and 3” appeared in the March 12 issue of *Neurology*.
- **ONYX long-term extension study for patients from both the TOPAZ and SAPPHIRE studies is ongoing.**

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.

- **Announced FDA clearance of IND application to initiate a Phase 2 proof-of-concept trial of apitegromab to treat obesity in patients taking a GLP-1 receptor agonist (GLP-1 RA).** 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 safely preserve lean muscle mass as an adjunctive therapy in overweight and obese adults who are taking a GLP-1 RA. The clinical study is expected to commence in mid-2024 with data 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.
- **Presented preclinical SRK-439 data at the Keystone Symposia's *Obesity: Causes and Consequences* meeting in February 2024**. Preclinical data evaluating SRK-439 in combination with GLP-1 RAs supports the potential of SRK-439 to preserve lean muscle mass as part of healthy weight loss management. The Company intends to present additional preclinical SRK-439 data at upcoming conferences.

Corporate

- **Appointed Katie Peng to the Board of Directors.** In February 2024, Ms. Peng joined Scholar Rock's Board of Directors. As an accomplished biotechnology executive, she brings over three decades of deep commercial experience in neurology and rare diseases, including with Evrydsi® for SMA, as the Company prepares for the potential commercial launch of apitegromab. Ms. Peng is currently the Chief Commercial Officer at Denali Therapeutics.
- **Promoted Mo Qatanani, Ph.D. to Chief Scientific Officer.** In February 2024, Dr. Qatanani was promoted to Chief Scientific Officer of Scholar Rock. Dr. Qatanani joined Scholar Rock in 2021 and has been leading all research functions since 2022 and driving Scholar Rock's early-stage pipeline, including SRK-439. He brings over 15 years of discovery and translational research experience in developing and advancing multiple therapeutic modalities in the neuromuscular and cardiometabolic disease areas.
- **Scholar Rock to host an Investor & Analyst Day event on May 22, 2024 in New York City.** Event to highlight the Company's myostatin inhibition programs in SMA and obesity, including commercial readiness activities for apitegromab and the SRK-439 development plan.

Full Year 2023 Financial Results

For the full year ended December 31, 2023, net loss was $165.8 million or $1.99 per share compared to a net loss of $134.5 million or $2.26 per share for the full year ended December 31, 2022.

- The Company did not record any revenue for the year ended December 31, 2023. The Company recorded $33.2 million in revenue for the year ended December 31, 2022. The revenue in 2022 was attributable to recognition of the remaining revenue related to a collaboration agreement between the Company and Gilead Sciences.
- Research and development expense was $121.9 million for the year ended December 31, 2023, compared to $124.4 million for the year ended December 31, 2022. R&D spend was in line with company plans.
- General and administrative expense was $49.4 million for the year ended December 31, 2023, compared to $43.1 million for the year ended December 31, 2022. The increase was associated with planned increases in employee compensation and benefits expense as the Company increased total headcount during 2023.
- As of December 31, 2023, Scholar Rock had cash, cash equivalents, and marketable securities of approximately $280 million, which is expected to fund the Company's anticipated operating and capital expenditure requirements into the second half of 2025.

"We executed across our portfolio in 2023 and leveraged our world-leading expertise in myostatin inhibition with the formal launch of our cardiometabolic program. Additionally, we managed spend carefully throughout the year, and the capital raise in the fourth quarter extended our cash runway," said Ted Myles, Chief Operating Officer and Chief Financial Officer of Scholar Rock. "We are off to a strong start in 2024 and are well positioned for a pivotal year, looking to the SAPPHIRE data read-out as a critical inflection point for the company."

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 Annual Report on Form 10-K for the year ended December 31, 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)

	Years Ended December 31	
	2023	**2022**

Revenue	$ —	$ 33,193
Operating expenses		
Research and development	121,900	124,444
General and administrative	49,395	43,119
Total operating expenses	171,295	167,563
Loss from operations	(171,295)	(134,370)
Other income (expense), net	5,506	(132)
Net loss	$ (165,789)	$ (134,502)
Net loss per share, basic and diluted	$ (1.99)	$ (2.26)
Weighted average common shares outstanding, basic and diluted	83,347,086	59,611,656

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

	December 31, 2023	December 31, 2022
Assets		
Cash, cash equivalents and marketable securities	$ 279,938	$ 315,361
Other current assets	8,256	12,663
Total current assets	288,194	328,024
Other assets	22,841	30,144
Total assets	$ 311,035	$ 358,168
Liabilities and Stockholders' Equity		
Current liabilities	$ 32,741	$ 36,389
Long-term liabilities	53,076	61,544
Total liabilities	85,817	97,933
Total stockholders' equity	225,218	260,235
Total liabilities and stockholders' equity	$ 311,035	$ 358,168

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