Scholar Rock Reports Full Year 2024 Financial Results and Highlights Business Progress

February 27, 2025

- Submitted apitegromab U.S. Biologics License Application (BLA) for patients with spinal muscular atrophy in January 2025; U.S. launch anticipated in 4Q 2025

- Remain on track to submit European Union marketing authorisation application (MAA) in 1Q 2025

- Topline data from Phase 2 EMBRAZE trial evaluating apitegromab vs placebo in adult patients with obesity receiving tirzepatide expected in 2Q 2025

- Filing of IND application for SRK-439, a highly selective investigational myostatin inhibitor designed for cardiometabolic indications, anticipated in 3Q 2025

- Ended 2024 with approximately $437 million in cash, cash equivalents, and marketable securities

- Management to host update call today at 8:15 a.m. ET

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Feb. 27, 2025-- 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, 2024.

“Following positive topline results from the SAPPHIRE trial demonstrating apitegromab’s potential to transform the lives of people living with spinal muscular atrophy while receiving SMN-targeted therapies, we have entered 2025 with tremendous momentum,” said Jay Backstrom, M.D., MPH, President & Chief Executive Officer of Scholar Rock. “We have submitted our BLA and remain on track to submit our MAA later this quarter and we are working with urgency to prepare for our anticipated commercial launch. In parallel, we plan to initiate our OPAL study to explore earlier treatment for SMA patients under two years of age and have two critical milestones ahead for our cardiometabolic program with the topline data readout of the EMBRAZE trial and IND application for SRK-439.”

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 patients with SMA. Apitegromab is the only muscle-targeted therapy candidate for patients with SMA to demonstrate statistically significant improvement and clinically meaningful benefit in a pivotal Phase 3 clinical trial as measured by the gold standard, SMA-specific Hammersmith Functional Motor Scale-Expanded (HFMSE) at week 52.

- **Submitted a biologics license application (BLA) to the U.S. Food and Drug Administration (FDA); on track to submit a marketing authorisation application (MAA) to the European Medicines Agency (EMA) in 1Q 2025.** BLA submission is based on the positive Phase 3 SAPPHIRE trial data that demonstrated a statistically significant and clinically meaningful improvement in motor function for patients receiving apitegromab compared to placebo, as measured by HFMSE. The FDA has granted Fast Track, Orphan Drug, and Rare Pediatric Disease designations, and the EMA has granted Priority Medicines and Orphan Medicinal Product designations to apitegromab in SMA. In anticipation of regulatory approvals, Scholar Rock is planning for a U.S. commercial launch in 4Q 2025, with European launch to follow.
- **Additional data from SAPPHIRE trial to be presented at the 2025 MDA Clinical & Scientific Conference in a poster presentation and an oral presentation on March 19 at 10:45 a.m. Central Time.**
- **Plans to initiate Phase 2 OPAL clinical trial in SMA patients under two years of age in 3Q 2025.** The trial will evaluate apitegromab in patients who have been or are continuing to be treated with any currently approved SMN therapy.

Apitegromab in other rare neuromuscular disorders

- **Expanding development of apitegromab in other rare neuromuscular disorders.** Building on the positive Phase 3 SAPPHIRE trial, the Company is exploring development of apitegromab in adjacent neuromuscular conditions where muscle weakness is a hallmark of the disease, such as Duchenne muscular dystrophy (DMD), Becker muscular dystrophy, Facioscapulohumeral muscular dystrophy, and amyotrophic lateral sclerosis, as part of our efforts to serve a growing number of patients suffering with devastating neuromuscular diseases.
- **Presenting preclinical data on a combination treatment approach with apitegromab in DMD at the MDA Clinical**

and Scientific Conference. The poster presentation includes initial preclinical data on an investigational approach combining the murine equivalent of apitegromab and a dystrophin corrector for addressing muscle defects in a DMD mouse model.

Cardiometabolic Program

SRK-439 is a novel, preclinical, investigational myostatin inhibitor that binds to pro- and latent myostatin with high affinity and is selective for myostatin (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 and sustainable weight management by preserving lean mass during weight loss.

- **Topline data expected in the second quarter of 2025 from the Phase 2 EMBRAZE proof-of-concept trial.** The trial is a randomized, double-blind, placebo-controlled, multi-center study evaluating the safety and efficacy of apitegromab, a highly selective investigational myostatin inhibitor, to preserve muscle mass in overweight and obese adults receiving tirzepatide. Trial outcomes will inform and guide our clinical development plans for SRK-439.
- **IND application for SRK-439 for the treatment of obesity in patients on standard of care GLP-1 RAs expected to be filed in 3Q 2025.** Scholar Rock disclosed preclinical data in January showing that SRK-439 protected against tirzepatide-induced muscle loss in diet induced obesity (DIO) mouse models. Lean mass preservation was seen with doses of SRK-439 as low as 0.3 mg/kg, and lean mass gain was observed at higher doses. The addition of SRK-439 with tirzepatide improved overall body composition with dose-dependent enhancement of fat mass loss.

Corporate

- **Entered into expanded credit facility with Oxford Finance.** The expanded facility replaces the prior facility that was in place with Oxford Finance and Silicon Valley Bank. The Company's outstanding debt balance is unchanged at $50 million. The new facility provides the Company with access to an additional $50 million at its discretion through December 31, 2025. An additional $50 million would be available to the Company upon FDA approval of apitegromab in SMA, and a final $50 million tranche would be available to the Company provided certain commercial milestones are met.
- **Successfully completed upsized $345 million public offering in October 2024.** The financing will fund the Company's planned commercial launch in SMA and continue advancement of priority programs.
- **Appointed Lisa Wyman as Chief Technical and Quality Officer (CTQO); Ted Myles, Chief Operating Officer (COO) & Chief Financial Officer (CFO) to support in transition to a new CFO.** Ms. Wyman brings more than 20 years of leadership and functional experience in technical and quality operations to Scholar Rock, where she will support the Company's ongoing growth and evolution to becoming a fully integrated biopharma company as it prepares for its first expected drug approval and commercial launch in 2025. As the Company transitions to a new organizational structure, the role of COO is being eliminated, and the Company has initiated a search for a new CFO. Mr. Myles will remain with the Company through mid-March and then will serve as a Senior Advisor for six months during the transition.

Full Year 2024 Financial Results

For the full year ended December 31, 2024, net loss was $246.3 million or $2.47 per share compared to a net loss of $165.8 million or $1.99 per share for the full year ended December 31, 2023.

- The Company did not record any revenue for the year ended December 31, 2024, or for the year ended December 31, 2023.
- Research and development expense was $184.6 million for the year ended December 31, 2024, compared to $121.9 million for the year ended December 31, 2023. The R&D spend was in line with company plans.
- General and administrative expense was $67.5 million for the year ended December 31, 2024, compared to $49.4 million for the year ended December 31, 2023. The increase was associated with employee-related costs, in addition to professional service fees.
- As of December 31, 2024, Scholar Rock had cash, cash equivalents, and marketable securities of approximately $437.3 million, which combined with cash available to the Company is expected to fund anticipated operating and capital expenditure requirements into the fourth quarter of 2026.

"Scholar Rock has entered 2025 from a position of financial strength, and the successful public offering completed in October 2024 will support both the planned commercial launch of apitegromab in SMA and the advancement our portfolio of highly selective myostatin inhibition programs," said Ted Myles, Chief Operating Officer and Chief Financial Officer of Scholar Rock. "The organization is well positioned to bring apitegromab to patients and to achieve key milestones in the year ahead."

Conference Call Information

Management will provide an update on the Company and discuss fourth quarter 2024 results via conference call on Thursday, February 27 at 8:15 am ET. To access the live conference call, participants may register here. The live audio webcast of the call will be available under "Events and Presentations" in the Investor Relations section of the Scholar Rock website at http://investors.scholarrock.com. To participate via telephone, please register in advance here. Upon registration, all telephone participants will receive a confirmation email detailing how to join the conference call, including the dial-in number along with a

unique passcode and registrant ID that can be used to access the call. An archived replay of the webcast will be available on the Company's website for approximately 90 days.

About Apitegromab

Apitegromab is an investigational fully human monoclonal antibody inhibiting myostatin activation by selectively binding the pro- and latent forms of myostatin in the skeletal muscle. It is the first muscle-targeted treatment candidate in spinal muscular atrophy (SMA) to demonstrate clinical success in a pivotal phase 3 clinical trial. Myostatin, a member of the TGFβ superfamily of growth factors, is expressed primarily by skeletal muscle cells, and the absence of its gene is associated with an increase in muscle mass and strength in multiple animal species, including humans. Scholar Rock believes that its highly selective targeting of pro- and latent forms of myostatin with apitegromab may lead to a clinically meaningful improvement in motor function in patients with SMA. The U.S. Food and Drug Administration (FDA) has granted Fast Track, Orphan Drug and Rare Pediatric Disease designations, and the European Medicines Agency (EMA) has granted Priority Medicines (PRIME) and Orphan Medicinal Product designations, to apitegromab for the treatment of SMA. Apitegromab has not been approved for any use by the FDA or any other regulatory agency.

About the Phase 3 SAPPHIRE Trial

SAPPHIRE was a randomized, double-blind, placebo-controlled Phase 3 clinical trial that evaluated the safety and efficacy of apitegromab in nonambulatory patients with Types 2 and 3 SMA who were receiving current standard of care (either nusinersen or risdiplam). SAPPHIRE enrolled 156 patients aged 2-12 years old in the main efficacy population. These patients were randomized 1:1:1 to receive either apitegromab 10 mg/kg, apitegromab 20 mg/kg, or placebo by intravenous (IV) infusion every 4 weeks for 12 months. An exploratory population including 32 patients aged 13-21 years old was also evaluated. These patients were randomized 2:1 to receive either apitegromab 20 mg/kg or placebo every 4 weeks for 12 months.

The SAPPHIRE trial met its primary endpoint for the main efficacy population with a statistically significant 1.8-point improvement (p=0.0192) based on apitegromab combined dose (10 mg/kg and 20 mg/kg) and standard of care (SOC) versus placebo and SOC (Hochberg multiplicity adjustment) as measured by the Hammersmith Functional Motor Scale-Expanded at week 52.

About the Phase 2 EMBRAZE Trial

EMBRAZE is a randomized, double-blind, placebo-controlled, Phase 2 proof-of-concept trial evaluating the efficacy, safety and pharmacokinetics of apitegromab in adults with a body mass index (BMI) of >27 (overweight) or a BMI of >30 (obese) and taking a GLP-1 RA (tirzepatide or semaglutide). The target enrollment of EMBRAZE is 100 subjects aged 18-65 who are overweight or obese without diabetes. As part of the study design, the treatment period is 24 weeks, and all subjects will receive a GLP-1 RA. In addition, all subjects will be randomized 1:1 to receive either apitegromab or placebo by intravenous (IV) infusion every four weeks during the 24-week treatment period. The primary endpoint is change from baseline at Week 24 in lean mass assessed by dual-energy X-ray absorptiometry. Secondary endpoints include additional weight loss measures, safety and tolerability, and pharmacokinetic outcomes. Exploratory endpoints at Weeks 24 and 32 include cardiometabolic parameters (e.g. HbA1c), body composition, and physical function.

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 cardiometabolic disorders, including obesity. Based on preclinical data, SRK-439 has the potential to support healthier weight management by preserving lean mass during weight loss. 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 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.

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.

The efficacy and safety of apitegromab, SRK-181, and SRK-439 have not been established and apitegromab, SRK-181, and SRK-439 have not been approved for any use by the FDA or any other regulatory agency.

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

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 X (formerly known as Twitter) and LinkedIn. The information that we post on our website or on X (formerly known as 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.

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 timing of any regulatory submissions, the therapeutic potential, clinical benefits and safety of any product candidates, expectations regarding timing, success and data announcements of current ongoing preclinical and clinical trials, its cash runway, its ability to use its expanded credit facility, expectations relating to commercial launch in the US in the fourth quarter of 2025, 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 3 SAPPHIRE trial will be sufficient to support regulatory approval, that the full results from the Phase 3 SAPPHIRE trial may differ from the topline data, that preclinical and clinical data, including the results from the Phase 2 or Phase 3 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; the data generated from Scholar Rock's nonclinical and preclinical studies and clinical trials; 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, and 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, 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)

	Years Ended December 31,	
	2024	**2023**
Operating expenses		
Research and development	$ 184,550	$ 121,900
General and administrative	67,504	49,395
Total operating expenses	252,054	171,295
Loss from operations	(252,054)	(171,295)
Other income (expense), net	5,760	5,506
Net loss	$ (246,294)	$ (165,789)
Net loss per share, basic and diluted	$ (2.47)	$ (1.99)

Weighted average common shares outstanding, basic and diluted	99,838,102	83,347,086

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

	December 31, 2024	December 31, 2023
Assets		
Cash, cash equivalents and marketable securities	$ 437,278	$ 279,938
Other current assets	13,887	8,256
Total current assets	451,165	288,194
Other assets	23,757	22,841
Total assets	$ 474,922	$ 311,035
Liabilities and Stockholders' Equity		
Current liabilities	$ 46,936	$ 32,741
Long-term liabilities	59,352	53,076
Total liabilities	106,288	85,817
Total stockholders' equity	368,634	225,218
Total liabilities and stockholders' equity	$ 474,922	$ 311,035

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