Spruce Biosciences Announces New Corporate Strategy and Acquisition of Tralesinidase Alfa for the Treatment of Sanfilippo Syndrome Type B (MPS IIIB)

April 15, 2025

Biologics License Application (BLA) Submission to U.S. FDA for Tralesinidase Alfa Enzyme Replacement Therapy (TA-ERT) Anticipated in 1H 2026

Spruce to Host Conference Call Today at 8:30 a.m. ET

SOUTH SAN FRANCISCO, Calif.--(BUSINESS WIRE)--Apr. 15, 2025-- Spruce Biosciences, Inc. (Nasdaq: SPRB), a late-stage biopharmaceutical company focused on developing and commercializing novel therapies for neurological disorders with significant unmet medical need, today announced the company's new corporate strategy and acquisition of tralesinidase alfa enzyme replacement therapy (TA-ERT) for the treatment of Sanfilippo Syndrome Type B (MPS IIIB).

"This is truly a transformative moment for Spruce as we focus our expertise in rare disease on a potential near-term commercial opportunity with TA-ERT in MPS IIIB," said Javier Szwarcberg, M.D., M.P.H., Chief Executive Officer of Spruce. "As a result of the strategic process initiated last year, Spruce acquired TA-ERT for the treatment of children with MPS IIIB, a neurodegenerative and ultimately fatal genetic disease. This new strategy opens a new chapter in our mission to provide transformative and life-changing therapies to patients affected by serious conditions with significant unmet medical need."

Dr. Szwarcberg continued, "In clinical studies, TA-ERT has been shown to significantly and durably normalize cerebral spinal fluid (CSF) heparan sulfate non-reducing end (HS-NRE) levels over a five-year period. Alignment has been achieved with the U.S. Food and Drug Administration (FDA) that HS-NRE could be used as a biomarker that may reasonably predict clinical benefit and serve as a basis for accelerated approval. Based on the existing clinical and non-clinical data, we anticipate submitting a BLA for TA-ERT to the FDA in the first half of 2026. We extend our gratitude to the patient and caregiver advocates, clinicians and industry leaders who have contributed to the TA-ERT program. With no FDA-approved treatments currently available, TA-ERT has the potential to be a groundbreaking advancement for patients and families impacted by MPS IIIB."

"The vision of the Spruce leadership team and their unrelenting commitment to serving patient communities with meaningful unmet need is on full display with the acquisition of TA-ERT," said Mike Grey, Executive Chairman of Spruce. "The confidence and support shown by our Board underscore the immense potential of the company's new portfolio and direction."

Corporate Strategy

- **Seek regulatory approval and maximize the U.S. commercial potential of TA-ERT for the treatment of MPS IIIB.** Spruce intends to seek U.S. accelerated approval of TA-ERT for MPS IIIB based on existing non-clinical and clinical data. As a condition of seeking such approval of a BLA from the FDA, Spruce will initiate a confirmatory trial. If the BLA is approved, Spruce intends to build a highly specialized commercial and medical affairs organization to support the commercialization of TA-ERT. Given that a relatively small number of clinicians and specialists treat most of the patients with MPS IIIB, the company believes this market can be effectively addressed with a modest-sized and targeted patient-centric field team, alongside various high-touch patient initiatives.
- **Commercialize globally through a patient-focused organization.** Spruce seeks to commercialize TA-ERT and its other investigational products throughout the developed world, including North America, the European Union (EU), the United Kingdom (U.K.), Latin America, Turkey, Asia, and other international markets. The company intends to establish its own commercial organization in the U.S., EU, and the U.K., and seek regional strategic collaborations and a network of third-party distributors in other international markets.
- **Focus on serious diseases with significant unmet medical need and clear biology.** Spruce focuses on diseases that have biology that is well understood. The company believes that developing drugs that directly impact known disease pathways will increase the probability of success of its development programs.

TA-ERT for the Treatment of MPS IIIB

Spruce entered into an Asset Purchase Agreement under which the company acquired an exclusive worldwide license agreement with BioMarin Pharmaceutical Inc. for TA-ERT and other enzyme replacement therapy products. TA-ERT is a fusion protein comprised of recombinant human alpha-N-acetylglucosaminidase (rhNAGLU) with modified human insulin-like growth factor 2 via an amino acid linker. TA-ERT is intended as an enzyme replacement therapy for the treatment of patients with MPS IIIB who lack rhNAGLU enzyme activity. In March 2024, in a Type C meeting with the FDA, the FDA confirmed that HS-NRE is deemed to be a surrogate biomarker reasonably likely to predict clinical benefit and could serve as a basis for accelerated approval. The FDA also confirmed that the completed clinical and non-clinical studies of TA-ERT were sufficient for a BLA submission and provided guidance around key design elements of a confirmatory trial, which must be initiated prior to potential accelerated approval of TA-ERT. TA-ERT has received fast-track designation, rare pediatric disease designation, and orphan drug designation in the U.S. and EU. Spruce intends to submit the BLA of TA-ERT for the treatment of MPS IIIB in the first half of 2026.

Financial Update

Spruce Biosciences also reported financial results for the year ended December 31, 2024.

As of December 31, 2024, Spruce had cash and cash equivalents of $38.8 million. The company expects its cash runway to fund its current operating plan through the end of 2025. 42.2 million shares of common stock are issued and outstanding and 21.4 million shares of common stock are reserved for issuance of warrants and equity securities as of December 31, 2024.

Please refer to Spruce's 2024 Annual Report on Form 10-K filed with the U.S. Securities and Exchange Commission today for the company's full annual 2024 financial results.

Conference Call Details

Spruce's management team will host a conference call today at 8:30 a.m. ET to discuss the business update. Analysts and investors can participate in the conference call by registering here.

An archived replay of the call will be available on the events section of the company's investor relations website for approximately 90 days.

About Spruce Biosciences

Spruce Biosciences is a late-stage biopharmaceutical company focused on developing and commercializing novel therapies for neurological disorders with significant unmet medical need. To learn more, visit www.sprucebio.com and follow us on X, LinkedIn, Facebook and YouTube.

Forward Looking Statements

Statements contained in this press release regarding matters that are not historical facts are "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995. Such forward-looking statements include statements regarding, among other things, the ability to seek accelerated approval of TA-ERT for MPS IIIB based on existing clinical data; the anticipated timing and conduct of our confirmatory trial for TA-ERT; the timing and likelihood of regulatory filings and approvals for TA-ERT, including our anticipated BLA Submission of TA-ERT for MPS IIIB in the first half of 2026; our ability to commercialize TA-ERT, if approved, in the United Sates and in international markets; the anticipated market opportunity and level of sales for TA-ERT for MPS IIIB, if approved; our ability to establish a commercial organization in the United States and leverage regional partnerships and a network of third-party distributors in international markets; our intended focus on serious diseases with significant unmet medical need and clear biology; and our expected future financing needs, are forward-looking statements. Because such statements are subject to risks and uncertainties, actual results may differ materially from those expressed or implied by such forward-looking statements. Words such as "anticipate", "continue", "will", "potential", "on track", "can", "intend", "expect" and similar expressions are intended to identify forward-looking statements. These forward-looking statements are based upon Spruce's current expectations and involve assumptions that may never materialize or may prove to be incorrect. Actual results could differ materially from those anticipated in such forward-looking statements as a result of various risks and uncertainties, which include, without limitation, risks and uncertainties associated with Spruce's business in general, the impact of geopolitical and macroeconomic events, and the other risks described in Spruce's filings with the U.S. Securities and Exchange Commission. All forward-looking statements contained in this press release speak only as of the date on which they were made and are based on management's assumptions and estimates as of such date. Spruce undertakes no obligation to update such statements to reflect events that occur or circumstances that exist after the date on which they were made, except as required by law.

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