



Biogen Provides Regulatory Update on High Dose Regimen of Nusinersen

September 23, 2025

CAMBRIDGE, Mass., Sept. 23, 2025 (GLOBE NEWSWIRE) -- [Biogen](#) Inc. (Nasdaq: BIIB) today announced that the U.S. Food and Drug Administration (FDA) issued a Complete Response Letter (CRL) for the Company's supplemental New Drug Application (sNDA) for the high dose regimen of nusinersen for the treatment of spinal muscular atrophy (SMA). The FDA letter requested an update to the technical information be included in the Chemistry Manufacturing and Controls (CMC) module of the sNDA.

The letter did not cite any deficiencies in the clinical data of the high dose regimen. The FDA provided options for resolution, and Biogen is planning to resubmit the application promptly based upon readily available information.

"While this outcome was unexpected, we remain committed to bringing the high dose regimen to people living with SMA," said Priya Singhal, M.D., M.P.H., Head of Development at Biogen. "We are working diligently to provide the necessary information to the FDA."

Biogen is working with regulatory authorities around the world to advance the high dose regimen as an additional dosing option for people living with SMA. The high dose regimen of SPINRAZA (nusinersen) was recently approved in Japan and is actively under review by the European Medicines Agency (EMA) and other global regulators.

About SPINRAZA

SPINRAZA (nusinersen) 12mg/5 mL injection is approved in more than 71 countries to treat infants, children and adults with spinal muscular atrophy (SMA). As a foundation of care in SMA, more than 14,000 individuals have been treated with SPINRAZA worldwide.¹

SPINRAZA has shown efficacy across ages and SMA types with a well-established safety profile based on data in patients treated up to 10 years,^{2,3} combined with unsurpassed real-world experience. The most common adverse events observed in clinical studies were respiratory infection, fever, constipation, headache, vomiting and back pain. Laboratory tests can monitor for renal toxicity and coagulation abnormalities, including acute severe low platelet counts, which have been observed after administration of some ASOs.

Biogen licensed the global rights to develop, manufacture and commercialize SPINRAZA from Ionis Pharmaceuticals, Inc. (Nasdaq: IONS). Please click here for [Important Safety Information](#) and [full Prescribing Information](#) for SPINRAZA in the U.S., or visit your respective country's product website.

About Biogen

Founded in 1978, Biogen is a leading biotechnology company that pioneers innovative science to deliver new medicines to transform patients' lives and to create value for shareholders and our communities. We apply deep understanding of human biology and leverage different modalities to advance first-in-class treatments or therapies that deliver superior outcomes. Our approach is to take bold risks, balanced with return on investment to deliver long-term growth.

We routinely post information that may be important to investors on our website at www.biogen.com. Follow us on social media - [Facebook](#), [LinkedIn](#), [X](#), [YouTube](#).

Biogen Safe Harbor

This news release contains forward-looking statements, including, among others, relating to: the potential benefits, efficacy and safety of higher doses of nusinersen (marketed as SPINRAZA); the potential to improve outcomes for, and address unmet needs of, patients with SMA; potential regulatory discussions, submissions and approvals and the timing thereof; the anticipated benefits, risks and potential of our collaboration arrangements; the potential of our commercial business and pipeline programs, including nusinersen; and risks and uncertainties associated with drug development and commercialization. These forward-looking statements may be accompanied by such words as "aim," "anticipate," "assume," "believe," "contemplate," "continue," "could," "estimate," "expect," "forecast," "goal," "guidance," "hope," "intend," "may," "objective," "outlook," "plan," "possible," "potential," "predict," "project," "prospect," "should," "target," "will," "would" or the negative of these words or other words and terms of similar meaning. Drug development and commercialization involve a high degree of risk, and only a small number of research and development programs result in commercialization of a product. Results in early-stage clinical trials may not be indicative of full results or results from later stage or larger scale clinical trials and do not ensure regulatory approval. You should not place undue reliance on these statements. Given their forward-looking nature, these statements involve substantial risks and uncertainties that may be based on inaccurate assumptions and could cause actual results to differ materially from those reflected in such statements.

These forward-looking statements are based on management's current beliefs and assumptions and on information currently available to management. Given their nature, we cannot assure that any outcome expressed in these forward-looking statements will be realized in whole or in part. We caution that these statements are subject to risks and uncertainties, many of which are outside of our control and could cause future events or results to differ materially from those stated or implied in this document, including, among others, uncertainty of our long-term success in developing, licensing, or acquiring other product candidates or additional indications for existing products; expectations, plans, prospects and timing of actions relating to product approvals, approvals of additional indications for our existing products, sales, pricing, growth, reimbursement and launch of our marketed and pipeline products; the potential impact of increased product competition in the biopharmaceutical and healthcare industry, as well as any other markets in which we compete, including increased competition from new originator therapies, generics, prodrugs and biosimilars of existing products and products approved under abbreviated regulatory pathways; our ability to effectively implement our corporate strategy; difficulties in obtaining and maintaining adequate coverage, pricing, and reimbursement for our products; the drivers for growing our business, including our dependence on collaborators and other third parties for the development, regulatory approval, and commercialization of products and other aspects of our business, which are outside of our full control; risks related to commercialization of biosimilars, which is subject to such risks related to our reliance on third-parties, intellectual property, competitive and market challenges and regulatory compliance; the risk that positive results in a clinical trial may not be replicated in subsequent or confirmatory trials or success in early stage clinical trials may not be predictive of results in later stage or large scale clinical trials or trials in other potential indications; risks associated with clinical trials, including our ability to adequately manage clinical activities, unexpected concerns that may arise from additional data or analysis obtained during clinical trials, regulatory authorities may require additional information or further studies, or may fail to approve or may delay approval of our drug candidates; and the occurrence of adverse safety events, restrictions on use with our products, or product liability claims; and any other risks and uncertainties that are described in reports we have filed with

the U.S. Securities and Exchange Commission, which are available on the SEC's website at www.sec.gov.

These statements speak only as of the date of this press release and are based on information and estimates available to us at this time. Should known or unknown risks or uncertainties materialize or should underlying assumptions prove inaccurate, actual results could vary materially from past results and those anticipated, estimated or projected. Investors are cautioned not to put undue reliance on forward-looking statements. A further list and description of risks, uncertainties and other matters can be found in our Annual Report on Form 10-K for the fiscal year ended December 31, 2024 and in our subsequent reports on Form 10-Q. Except as required by law, we do not undertake any obligation to publicly update any forward-looking statements whether as a result of any new information, future events, changed circumstances or otherwise.

References:

1. Based on commercial patients, early access patients, and clinical trial participants through December 31, 2022.
2. Core Data sheet, Version 13, October 2021. SPINRAZA. Biogen Inc, Cambridge, MA.
3. Finkle et al. Cure SMA 2024. "Final Safety and Efficacy Data From the SHINE Study in Participants With Infantile-Onset and Later-Onset SMA."

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