High Dose Regimen of Nusinersen Receives Positive CHMP Opinion for the Treatment of Spinal Muscular Atrophy

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- Positive CHMP opinion is based on data from the DEVOTE study which evaluated the high dose regimen of nusinersen in treatment-naive participants and those transitioning from the currently approved 12mg dose regimen[1]
- Final European Commission decision expected in January 2026
- SMA is a rare, genetic, neuromuscular disease that affects individuals of all ages[2]

CAMBRIDGE, Mass., Nov. 17, 2025 (GLOBE NEWSWIRE) -- Biogen Inc. (Nasdaq: BIIB) today announced the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) adopted a positive opinion recommending by consensus the approval of the high dose regimen of nusinersen for the treatment of 5q spinal muscular atrophy (SMA). 5q SMA is the most common form of the disease and represents approximately 95% of all SMA cases.[3] The CHMP's Positive Opinion will now be reviewed by the European Commission with a final decision expected in January 2026. If adopted by the European Commission, the high dose regimen will be an additional dosing option to the already approved 12 mg low dose regimen.

"While we've seen great progress over the past decade, there is urgency to do more to address the clear unmet needs of the SMA community," said Priya Singhal, M.D., M.P.H., Executive Vice President and Head of Development at Biogen. "The CHMP's positive opinion for the high dose regimen of nusinersen represents a promising advancement in our commitment to support the evolving needs of individuals living with SMA and deliver therapies that can enhance patient outcomes."

Nusinersen is currently commercialized under the brand name SPINRAZA® in over 71 countries at the label-approved dose regimen of 12 mg.

The CHMP's positive opinion is based on data from the three-part, Phase 2/3 DEVOTE study and its ongoing long-term extension which investigated the efficacy and safety of the high dose regimen of nusinersen in treatment-naïve and individuals previously-treated with the approved 12 mg dosing regimen. The high dose regimen comprises a loading regimen of two 50 mg doses 14 days apart and a maintenance regimen of 28 mg every four months for treatment-naïve patients. Those who transitioned to the high dose regimen from the 12 mg dose regimen received a single loading dose of 50 mg and then continued with the maintenance regimen of 28 mg every four months. During the study, the high dose regimen was given via an intrathecal injection by healthcare professionals experienced in doing lumbar punctures.

"The positive CHMP opinion for the high dose regimen of nusinersen is an important milestone for the SMA community," said Eugenio Mercuri, M.D., Ph.D., Professor of Pediatric Neurology at the Catholic University, Rome, Italy. "Based on the results from the DEVOTE study and my experience with patients receiving this novel regimen, I am confident that high dose nusinersen has the potential to bring meaningful benefits to people living with SMA."

The pivotal Part B cohort (n=75) met its primary endpoint where treatment-naïve, symptomatic infants who received the high dose regimen saw a statistically significant improvement compared to baseline in motor function as measured by the Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP-INTEND), when compared to a prespecified matched sham (untreated) group from the ENDEAR study* (mean difference: 26.19 points; +15.1 vs. -11.1, $p<0.0001$). The group treated with the high dose regimen of nusinersen experienced a reduction in the risk of death or permanent ventilation relative to the matched sham group (68% reduction, nominal $p=0.0006$).[1]

In the open-label Part C of DEVOTE, a broad range of individuals (n=38) transitioned to the high dose regimen after a median of 3.9 years on the approved 12 mg regimen. Participants experienced improvements in motor function with a mean increase of 1.8 points [SD=3.99] from baseline to Day 302 as measured by the Hammersmith Functional Motor Scale Expanded.[1]

The high dose regimen was generally well tolerated, with reported adverse events generally consistent with SMA and the known safety profile of nusinersen. No new safety concerns were observed with continued use of nusinersen in the long-term-extension study. In Part B of DEVOTE, the most common adverse events that occurred in at least 10% of participants treated with the high dose regimen of nusinersen and occurred at least 5% more frequently than the matched sham group were pneumonia, COVID-19, pneumonia aspiration, and malnutrition.[1]

Special warnings and precautions for use of nusinersen include adverse reactions as a part of the lumbar puncture procedure, low platelet counts and blood clotting abnormalities, renal toxicity and hydrocephalus (excessive buildup of cerebrospinal fluid in the brain).[4]

The new high dose regimen of SPINRAZA (nusinersen) was recently approved in Japan. The high dose regimen of nusinersen is currently under review with the U.S. Food and Drug Administration (FDA) with a decision expected by April 3, 2026. 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.

*ENDEAR is one of the two pivotal studies that formed the basis of regulatory approvals for SPINRAZA 12 mg.

About the DEVOTE Study[1]
DEVOTE was a Phase 2/3 randomized, controlled, dose-escalating study designed to evaluate the safety, tolerability, pharmacokinetics and efficacy of nusinersen when administered at a higher dose (50/28 mg). The study enrolled 145 participants across ages and SMA types at approximately 42 sites around the world. DEVOTE includes an open-label safety evaluation cohort (Part A), a double-blind, active control randomized treatment cohort (Part B), followed by an open-label treatment cohort (Part C) to assess the safety and tolerability of transitioning participants from the currently approved dose of SPINRAZA 12 mg to the higher dose regimen being tested in the study.

Part B was comprised of a pivotal cohort in treatment-naïve patients with infantile-onset SMA (n=75), and a supportive cohort in treatment-naïve patients with later-onset SMA (n=24). The primary endpoint of Part B measured the change from baseline on CHOP-INTEND at six months, comparing the higher dose regimen of nusinersen to a matched, untreated sham control group from the Phase 3 ENDEAR study. ENDEAR is one of

the two pivotal studies that formed the basis of regulatory approval for SPINRAZA 12 mg.

Part C was an open-label evaluation of the higher dose regimen in children and adults who transitioned from SPINRAZA 12 mg to the 50/28 mg regimen (n=40).

More information about the DEVOTE study (NCT04089566) is available at clinicaltrials.gov.

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.[5]

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,[6,7] 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). For more information, visit your respective country's product website. For the U.S., please click here Important Safety Information and full Prescribing Information.

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, decisions 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.

Digital Media Disclosure
From time to time we have used, or expect in the future to use, our investor relations website (investors.biogen.com), the Biogen LinkedIn account (linkedin.com/company/biogen) and the Biogen X account (x.com/biogen) as a means of disclosing information to the public in a broad, non-exclusionary manner, including for purposes of the SEC's Regulation Fair Disclosure (Reg FD). Accordingly, investors should monitor our investor relations website and this social media channel in addition to our press releases, SEC filings, public conference calls and webcasts, as the information posted on them could be material to investors.

References:

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3. Farrar MA, Kiernan MC. The Genetics of Spinal Muscular Atrophy: Progress and Challenges. Neurotherapeutics; 2015; 12:290–302.
4. European Medicines Agency. SPINRAZA Summary of Product Characteristics. Available at: https://www.ema.europa.eu/en/documents/product-information/spinraza-epar-product-information_en.pdf. Last accessed: November 2025.
5. Based on commercial patients, early access patients, and clinical trial participants through December 31, 2022.
6. Core Data sheet, Version 13, October 2021. SPINRAZA. Biogen Inc, Cambridge, MA.
7. Finkel RS, et al. Final Safety and Efficacy Data From the SHINE Study in Participants With Infantile-Onset and Later-Onset SMA. Presented at: Cure SMA Conference; 2024; Austin, Texas.

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