FDA Approves New High Dose Regimen of SPINRAZA® (nusinersen) for Spinal Muscular Atrophy

March 30, 2026

- The approval was anchored on data from the pivotal DEVOTE study that investigated the efficacy and safety of the High Dose Regimen of SPINRAZA in treatment-naïve and previously treated SPINRAZA patients
- High Dose SPINRAZA will be available in the United States in the coming weeks and is also approved in the European Union, Switzerland and Japan

CAMBRIDGE, Mass., March 30, 2026 (GLOBE NEWSWIRE) -- Biogen Inc. (Nasdaq: BIIB) today announced that the High Dose Regimen of SPINRAZA® (nusinersen), which is comprised of 50 mg/5 mL and 28 mg/5 mL doses, was approved by the U.S. Food and Drug Administration (FDA) for the treatment of spinal muscular atrophy (SMA). Backed by more than 10 years of clinical data supporting the Low Dose Regimen of SPINRAZA (12 mg), High Dose SPINRAZA was designed to deliver a higher concentration of drug through both the loading and maintenance dosing phases, to provide a new option in response to the ongoing needs of the community.

The High Dose Regimen of SPINRAZA, which will be available in the coming weeks, enables an accelerated loading phase for those new to SPINRAZA treatment – with two 50 mg doses administered 14 days apart – followed by 28 mg maintenance dose injections every four months thereafter. Patients transitioning from the Low Dose Regimen would follow their existing dosing schedule at four-month intervals after a single High Dose loading phase.

“Optimizing the dose of nusinersen builds on a therapy that we already know can change lives. The high dose regimen demonstrated meaningful clinical benefit while maintaining a well characterized safety profile,” said Richard Finkel, M.D., director, Center for Experimental Neurotherapeutics (CENT) at St. Jude Children’s Research Hospital. “I believe High Dose Spinraza will play an important role in the future of SMA care.”

The FDA approval is based on data from the three-part, Phase 2/3 DEVOTE study. Results from the pivotal cohort of the study showed treatment-naïve, symptomatic infants who received High Dose SPINRAZA experienced statistically significant improvements 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, $p<0.0001$).

“Over the past decade, Biogen has continued to listen, learn, and innovate to help advance care for people living with SMA,” said Priya Singhal, M.D., M.P.H., Executive Vice President and Head of Development at Biogen. “With more than 10 years of clinical data on SPINRAZA, the development of the High Dose Regimen reflects both the strength of that foundation and our unwavering commitment to the SMA community to optimize treatment options. We are grateful to the community for their support and contributions toward this milestone.”

In the DEVOTE study, the safety profile of the High Dose Regimen of SPINRAZA was generally consistent with the known safety of the Low Dose Regimen. The most common adverse reactions occurring in at least 10% of SPINRAZA-treated patients who received the High Dose Regimen and occurred at least 5% more frequently than in historic matched sham-control were: pneumonia, COVID-19, pneumonia aspiration, and malnutrition in patients with infantile-onset SMA. COVID-19 was not discovered at the time of ENDEAR, the study from which the matched sham-control was taken.

“Nearly ten years ago, the approval of SPINRAZA marked a turning point in SMA care and changed what the community believed was possible, with Biogen becoming a trusted partner for thousands of people living with SMA. Today’s approval of High Dose SPINRAZA makes progress in addressing unmet needs of the SMA community,” said Kenneth Hobby, President of Cure SMA. “Biogen understands the needs of the SMA community and has remained a committed and engaged partner to advance research that can improve the daily lives of people living with SMA.”

High Dose SPINRAZA is also approved in the European Union, Switzerland, and Japan, and Biogen is working with regulatory authorities and national health authorities around the world to progress this 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.

WHAT IS SPINRAZA?

SPINRAZA® (nusinersen) is a prescription medicine used to treat spinal muscular atrophy (SMA) in pediatric and adult patients.

IMPORTANT SAFETY INFORMATION

Increased risk of bleeding complications has been observed after administration of similar medicines. Your healthcare provider should perform blood tests before you start treatment with SPINRAZA and before each dose to monitor for signs of these risks. Seek medical attention if unexpected bleeding occurs.

Increased risk of kidney damage, including potentially fatal acute inflammation of the kidney, has been observed after administration of similar medicines. Your healthcare provider should perform urine testing before you start treatment with SPINRAZA and before each dose to monitor for signs of this risk.

The most common side effects of SPINRAZA in the Low Dose Regimen in infantile- and later- onset SMA include lower respiratory infection, constipation, fever, headache, vomiting, back pain, and post-lumbar puncture syndrome.

The most common side effects of SPINRAZA in the High Dose Regimen in infantile-onset SMA include pneumonia, COVID-19, pneumonia aspiration, and malnutrition. COVID-19 was not discovered at the time of the studies for the Low Dose Regimen.

These are not all of the possible side effects of SPINRAZA. Call your healthcare provider for medical advice about side effects. You may report side effects to FDA at 1-800-FDA-1088.

Before taking SPINRAZA, tell your healthcare provider if you are pregnant or plan to become pregnant.

Please see full Prescribing Information.
This information is not intended to replace discussions with your healthcare provider.

About the DEVOTE Study
DEVOTE was a Phase 2/3 randomized, controlled, dose-escalating study designed to evaluate the safety, tolerability, pharmacokinetics, and efficacy of SPINRAZA 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 included 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 high 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 mg/5 mL and 28 mg/5 mL regimen (n=40).

About SPINRAZA
The High Dose Regimen of SPINRAZA® (nusinersen) comprising a 50 mg/5 mL loading dose and 28 mg/5 mL maintenance dose injections is currently approved in the U.S., European Union, Switzerland, and Japan to treat infants, children and adults with spinal muscular atrophy (SMA). SPINRAZA 12 mg/5 mL injection is approved for SMA in more than 71 countries.[1] SPINRAZA is administered intrathecally by, or under the direction of, healthcare professionals experienced in performing lumbar punctures.

The Low Dose Regimen of 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).

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, Instagram, 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 a High Dose Regimen of SPINRAZA® (nusinersen); the potential to advance care and improve outcomes for, and address unmet needs of, patients with SMA; potential regulatory discussions, submissions, decisions and approvals and the timing thereof, including the approval of a High Dose Regimen of SPINRAZA; 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, 2025 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 (https://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:

1. Based on commercial patients, early access patients, and clinical trial participants through December 31, 2024.
2. Core Data sheet, Version 13, October 2021. SPINRAZA. Biogen Inc, Cambridge, MA.
3. Finkel 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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