Nature Medicine Publishes Results from the Pivotal DEVOTE Study of High-Dose Regimen of Nusinersen in Spinal Muscular Atrophy

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- Findings from DEVOTE support clinical benefits of the high-dose regimen of nusinersen (50 mg and 28 mg) in both treatment-naïve individuals and those previously treated with 12 mg nusinersen
- The high-dose regimen of nusinersen also slowed neurodegeneration more rapidly, as measured by neurofilament, than the 12 mg regimen

CAMBRIDGE, Mass., Feb. 04, 2026 (GLOBE NEWSWIRE) -- Biogen Inc. (Nasdaq: BIIB) today announced that *Nature Medicine* published results from the Phase 2/3 DEVOTE study evaluating the high-dose regimen of nusinersen, comprised of 50 mg/5 mL loading and 28 mg/5 mL maintenance doses, in spinal muscular atrophy (SMA). The high-dose regimen of nusinersen offers a more rapid loading regimen, two 50 mg doses 14 days apart, and a higher maintenance regimen, 28 mg, every 4 months, compared to the 12 mg nusinersen regimen (SPINRAZA®). The results showed the safety and effectiveness of the high-dose regimen of nusinersen across a broad range of people living with SMA, irrespective of age, prior treatment experience, and baseline functional status.

“One of the most notable changes seen with the higher dose regimen was a more rapid reduction in neurofilament, a marker of neurodegeneration. In DEVOTE, treatment with the high-dose regimen led to improvement across important domains like motor and bulbar function, respiratory health and hospitalizations, and survival,” said Richard Finkel, M.D., director, Center for Experimental Neurotherapeutics (CENT) at St. Jude Children’s Research Hospital. “Publication of the DEVOTE results in Nature Medicine further validate the importance of these data and the future role that I expect the high dose regimen will play as we look to continue to improve outcomes for people living with SMA.”

DEVOTE is a three-part study that enrolled 139 participants across ages and SMA types. Results from the pivotal cohort of the study (Part B) showed that treatment-naïve, symptomatic infants who received the high-dose regimen of nusinersen 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). Results favored the high-dose regimen relative to sham across secondary endpoints and trended in favor of the high-dose regimen over the currently approved 12mg regimen on key biomarker and efficacy measures.

“The publication of the DEVOTE data are an important step in our commitment to bring the high dose regimen of nusinersen to people living with SMA as quickly as possible,” said Stephanie Fradette, Pharm.D., Head of the Neuromuscular Development Unit at Biogen. “We are grateful to the participants, their families, study investigators and site staff, and our co-authors who have made the DEVOTE study and publication of these results a reality.”

In the open-label Part C (n=40) of DEVOTE, a diverse group of participants, age 4-65, transitioned to the high-dose regimen (one 50 mg dose four months after their last 12 mg dose, followed by the 28 mg maintenance regimen, every four months) after a median of 3.9 years on the 12 mg regimen. Participants experienced improvements in motor function after transitioning with mean increases of 1.8 points on the Hammersmith Functional Motor Scale – Expanded (HFMSE) and 1.2 points on the Revised Upper Limb Module (RULM) from baseline at Day 302.

The safety profile of the high-dose regimen of nusinersen was broadly consistent with the known safety profile of the 12 mg regimen. In the Part B infantile-onset cohort of 50 participants, the most common adverse events (AEs; ≥15% of participants) in the high-dose regimen group were pneumonia, respiratory failure, pyrexia, COVID-19, and upper respiratory tract infection. The most common serious AEs (occurring in at least 10% of participants in the high-dose regimen group) were: pneumonia, pneumonia aspiration, and respiratory failure.

The high-dose regimen of SPINRAZA (nusinersen) is approved in the European Union and Japan. The high-dose regimen is under review with the United States Food and Drug Administration (FDA) and has a Prescription Drug User Fee Act (PDUFA) action date of April 3.

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

About SPINRAZA
The high dose regimen of SPINRAZA (nusinersen) which is comprised of 50 mg/5 mL and 28 mg/5mL injections are approved in the European Union and Japan to treat infants, children and adults with spinal muscular atrophy (SMA). The high dose regimen of nusinersen is currently under review with the U.S. Food and Drug Administration (FDA) a Prescription Drug User Fee Act (PDUFA) action date of April 3, 2026. SPINRAZA 12 mg/5 mL injection is approved for SMA in more than 71 countries.[1]

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,[52,3] 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). For more information, visit your respective country's product website. For the U.S., please click here for 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 (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, 2022.
2. Core Data sheet, Version 13, October 2021. SPINRAZA. Biogen Inc, Cambridge, MA.
3. 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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