New Data for Nusinersen Underscore Biogen's Commitment to Advancing Clinical Research to Improve Outcomes in SMA

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- *New analyses from DEVOTE Part C further characterize the improvements in motor function in participants with SMA who transitioned to the investigational higher dose regimen of nusinersen from 12 mg SPINRAZA® (nusinersen)*
- *Final results from the landmark NURTURE study highlight the profound impact of early treatment with 12 mg SPINRAZA in clinically presymptomatic SMA with 92% of children achieving the ability to walk independently*

CAMBRIDGE, Mass., June 27, 2025 (GLOBE NEWSWIRE) -- Biogen Inc. (Nasdaq: BIIB) today announced new data that reinforce the clinical impact of nusinersen across a broad spectrum of individuals affected by spinal muscular atrophy (SMA). These latest findings from Part C of the DEVOTE trial evaluating a higher dose regimen of nusinersen and the NURTURE trial which evaluated the approved 12 mg regimen (SPINRAZA®) in clinically presymptomatic SMA were presented at the SMA Research & Clinical Care Meeting hosted by Cure SMA in Anaheim, Calif. Biogen's applications for the higher dose regimen of nusinersen are currently under review in the U.S., Europe, Japan and other global markets. The higher dose regimen of nusinersen comprises a more rapid loading regimen – two 50 mg doses 14 days apart – and a higher maintenance regimen – 28 mg every four months.

"As the SMA treatment landscape continues to evolve, we remain steadfast in our commitment to address the unmet needs of the community. The findings from Part C of the DEVOTE study further strengthen the growing body of evidence supporting the potential benefits of the higher dose regimen of nusinersen," said Stephanie Fradette, Pharm.D., Head of the Neuromuscular Development Unit at Biogen.

Improvements Observed With Higher Dose Regimen of Nusinersen in Previously Treated Patient Population
Detailed results from Part C of the DEVOTE study highlight the potential clinical benefits of an investigational higher dose of nusinersen in a broad range of individuals (n=38) who had been previously treated with nusinersen at the approved 12 mg dose for approximately four years (median: 3.9 years). Participants were 4 to 65 years of age and half (n=19) were ambulatory. Participants in Part C received one loading dose (50 mg) and two maintenance doses (28 mg each) of open-label higher dose nusinersen during the study period.

Most participants experienced improvements on the Hammersmith Functional Motor Scale – Expanded (HFMSE), Revised Upper Limb Module (RULM), and/or Clinical Global Impression of Change (CGI-C; assessed by investigator or caregiver) after transitioning to the higher dose regimen. These improvements were observed across phenotypes, functional status and age. For example, non-ambulatory participants improved by +2.5 (95% CI: 0.49, 4.56) on average on the HFMSE, and ambulatory participants improved by +1.1 (95% CI: -0.68, 2.89).

"These emerging data indicate that additional gains in function might be possible even in those with established disease who have been on therapy for years," said Richard Finkel, M.D., director, Center for Experimental Neurotherapeutics (CENT) at St. Jude Children's Research Hospital. "This effort to optimize the dosing of SPINRAZA is very exciting for the field and could fundamentally change how we treat our patients."

The safety profile of the higher dose regimen of nusinersen is broadly consistent with the known safety profile of 12 mg SPINRAZA. In the DEVOTE study overall, reported adverse events (AEs) included pneumonia, respiratory failure, pyrexia, COVID, upper respiratory tract infection, procedural pain and procedural headache. In Part C (n=40), AEs were reported in 37/40 participants, the majority of which were mild or moderate in severity. Serious AEs were reported by six participants (15%), none of which were considered by the investigator to be related to study treatment or administration.

Final NURTURE Data Redefine Expectations for Early Treatment
Final data from the eight-year, open-label NURTURE study highlight the impact of early intervention with 12 mg SPINRAZA in clinically presymptomatic infants with SMA.

At the study conclusion, all children who participated in NURTURE (n=25) were alive and experienced continued clinical benefits over the course of the study. No participants required permanent ventilation, and the majority (20 of 25 participants) went without any ventilatory support throughout the study. Ninety-two percent of participants achieved the ability to walk independently, many within normal developmental timeframes. Participants with elevated levels of neurofilament light chain (NfL) at baseline experienced rapid and sustained reductions in NfL after initiation of nusinersen, reinforcing the potential utility of NfL as an objective biomarker of disease activity and treatment response in SMA.

Nusinersen was generally well tolerated with no new safety concerns identified with eight years of follow-up. All participants had at least one AE, the majority of which were mild to moderate in severity; no AEs led to treatment discontinuation or study withdrawal.

About SPINRAZA
SPINRAZA (nusinersen) 12 mg/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] The currently approved 12 mg regimen for SPINRAZA is comprised of four loading doses administered over approximately 60 days, followed by maintenance dosing every four months thereafter.

SPINRAZA is an antisense oligonucleotide (ASO) that targets the underlying cause of motor neuron loss by continuously increasing the amount of full-length survival motor neuron (SMN) protein produced in the body.[2] It is administered directly into the central nervous system, where motor neurons reside, to deliver treatment where the disease starts.[2]

SPINRAZA has shown sustained efficacy across ages and SMA types with a well-established safety profile based on data in patients treated up to 10 years,[3,4] combined with unsurpassed real-world experience. The nusinersen clinical development program encompasses more than 10 clinical studies, which have included more than 460 individuals across a broad spectrum of patient populations, including two randomized controlled studies

(ENDEAR and CHERISH). 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 related to the potential clinical effects of a higher dose regimen of nusinersen; the potential benefits, safety and efficacy of higher dose regimen of nusinersen; the clinical development program for higher dose regimen of nusinersen; the identification and treatment of SMA; our research and development program for the treatment of SMA; the potential of our commercial business and pipeline programs, including SPINRAZA; and risks and uncertainties associated with drug development and commercialization. These forward-looking statements may be accompanied by words such as "aim," "anticipate," "believe," "could," "estimate," "expect," "forecast," "intend," "may," "plan," "potential," "possible," "will," "would" and 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 our forward-looking statements.

These statements involve risks and uncertainties that could cause actual results to differ materially from those reflected in such statements, including without limitation, uncertainty of success in the development and potential commercialization of higher dose regimen of nusinersen; the risk that we may not fully enroll our clinical trials or enrollment will take longer than expected; unexpected concerns may arise from additional data, analysis or results obtained during our clinical trials; regulatory authorities may require additional information or further studies, or may fail or refuse to approve or may delay approval of our drug candidates, including SPINRAZA; the occurrence of adverse safety events; the risks of unexpected hurdles, costs or delays; failure to protect and enforce our data, intellectual property and other proprietary rights and uncertainties relating to intellectual property claims and challenges; product liability claims; results of operations and financial condition. The foregoing sets forth many, but not all, of the factors that could cause actual results to differ from our expectations in any forward-looking statement. Investors should consider this cautionary statement, as well as the risk factors identified in our most recent annual or quarterly report and in other reports we have filed with the U.S. Securities and Exchange Commission. These statements speak only as of the date of this news release.

We do not undertake any obligation to publicly update any forward-looking statements.

References:

1. Based on commercial patients, early access patients, and clinical trial participants through December 31, 2022.
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3. Core Data sheet, Version 13, October 2021. SPINRAZA. Biogen Inc, Cambridge, MA.
4. 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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