



New Data at Cure SMA 2021 Highlight the Long-Term Efficacy of SPINRAZA® (nusinersen) and Biogen's Commitment to Innovation in SMA Therapy

June 10, 2021

- New data analysis suggests an investigational higher dose of SPINRAZA may lead to clinically meaningful improvements in motor function
- A NURTURE study analysis shows 92 percent of children who initiated SPINRAZA treatment as pre-symptomatic infants maintained the ability to swallow after approximately 4 years
- Among children and teens with later-onset SMA in the SHINE study, long-term treatment with SPINRAZA improved walking distance and reduced fatigue

CAMBRIDGE, Mass., June 10, 2021 (GLOBE NEWSWIRE) -- [Biogen](#) Inc. (Nasdaq: BIIB) today announced new research supporting the continued development of an investigational higher dose of SPINRAZA® (nusinersen) and additional data reinforcing the strength of SPINRAZA's clinical profile in improving the lives of individuals with spinal muscular atrophy (SMA) over the long term. These data are being presented at the virtual Cure SMA Research & Clinical Care Meeting taking place June 9-11, 2021.

"Intervention with SPINRAZA can meaningfully impact the trajectory of SMA, and we remain relentless in our aim of improving outcomes for people with SMA. We continue to better understand and explore SPINRAZA's potential with our new and ongoing global clinical studies," said Alfred Sandrock, Jr., M.D., Ph.D., Head of Research and Development at Biogen. "The data we are presenting at Cure SMA 2021 demonstrate the long-term benefits with SPINRAZA as individuals age. Additionally, a new analysis provides further support for the potential for a higher dose of SPINRAZA to offer even greater improvements in motor function for SMA patients."

New Findings Support Continued Development of an Investigational Higher Dose of SPINRAZA

An analysis of data from the Phase 2 CS3A and Phase 3 ENDEAR studies in children with infantile-onset SMA used pharmacokinetic (PK)/pharmacodynamic (PD) modelling to predict the potential efficacy of an investigational higher dose regimen of SPINRAZA. This analysis suggests that a higher dose of SPINRAZA may lead to a clinically meaningful increase in the CHOP INTEND (Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders) score beyond that already observed with the 12 mg approved dose. These findings further reinforce the scientific rationale for the evaluation of a higher dose of SPINRAZA in the ongoing DEVOTE study.

DEVOTE is a Phase 2/3 study evaluating the safety, tolerability and potential for even greater efficacy of SPINRAZA when administered at a higher dose than currently approved. It is a three-part study that includes an open-label safety evaluation cohort (Part A), a pivotal, double-blind, randomized, active-controlled treatment cohort (Part B) and an open-label cohort of patients transitioning from the approved 12 mg dosing regimen of SPINRAZA to the higher dose regimen (Part C).

As previously reported,¹ safety data from Part A (n=6; 28 mg) support continued development of a higher dose of SPINRAZA and an updated analysis presented at the meeting include data collected up to 10 months. Enrollment in the pivotal Part B of DEVOTE is ongoing and will evaluate a higher-dose regimen (two loading doses of 50 mg 14 days apart followed by 28 mg maintenance doses every four months) compared to the currently approved dosing regimen for SPINRAZA.² More information about DEVOTE is available at [ClinicalTrials.gov \(NCT04089566\)](https://clinicaltrials.gov/ct2/show/study/NCT04089566).

Data Reinforce Long-term Efficacy of SPINRAZA in a Broad Range of People with SMA

An analysis of data from the NURTURE study (n=25) shows 92 percent of patients who initiated SPINRAZA treatment as pre-symptomatic infants maintained the ability to swallow after a median of 3.8 years. This is in contrast with the natural history of SMA where impaired swallowing is expected for people with 2 or 3 *SMN2* copies³ and can lead to an increased risk of aspiration pneumonia, choking and failure to thrive. In this analysis, NURTURE study participants were consistently rated by their caregiver as, on average, never to rarely experiencing difficulty for the majority of measures related to general feeding, drinking liquids and eating solid foods. Additionally, all participants with 3 *SMN2* copies and 73 percent (11 of 15) of participants with 2 *SMN2* copies were reported by their caregiver as being fed exclusively by mouth.

In addition, post-hoc data from the open-label CS2-CS12 and SHINE extension studies indicate children and teens with later-onset SMA (n=14) showed improvement in walking distance over five years of SPINRAZA treatment and stabilization in fatigue.

In all analyses presented at the meeting, the safety profile of SPINRAZA was consistent with previously reported findings.

Featured SPINRAZA Data Presentations Include:

- Scientific Rationale for a Higher Dose of Nusinersen
- Part A Results From the Ongoing DEVOTE Study to Explore a Higher Dose of Nusinersen in SMA
- Preserved Swallowing Function in Infants Who Initiated Nusinersen Treatment in the Presymptomatic Stage of SMA: Results From the NURTURE Study
- Nusinersen in Later-onset Spinal Muscular Atrophy: Walking Distance and Fatigue in CS2/12 and SHINE Participants

About SPINRAZA® (nusinersen)

SPINRAZA is approved to treat infants, children and adults with spinal muscular atrophy (SMA) and is available in more than 50 countries. As a foundation of care in SMA, more than 11,000 individuals have been treated with SPINRAZA worldwide.⁴

SPINRAZA is an antisense oligonucleotide (ASO) that targets the root cause of SMA by continuously increasing the amount of full-length survival motor neuron (SMN) protein produced in the body.⁵ It is administered directly into the central nervous system, where motor neurons reside, to deliver treatment where the disease starts.⁵

SPINRAZA has demonstrated sustained efficacy across ages and SMA types with a well-established safety profile based on data in patients treated up to 7 years, combined with unsurpassed real-world experience.⁶ The SPINRAZA clinical development program encompasses 10 clinical studies, which have included more than 300 individuals across a broad spectrum of patient populations,⁶ including two randomized controlled studies (ENDEAR and CHERISH). The ongoing SHINE and NURTURE open-label extension studies are evaluating the long-term impact of SPINRAZA. 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), the leader in antisense therapeutics. 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 SMA

SMA is a rare, genetic, neuromuscular disease that affects individuals of all ages. It is characterized by a loss of motor neurons in the spinal cord and lower brain stem, resulting in progressive muscle atrophy and weakness.⁷ SMA is caused by a deficiency in the production of survival motor neuron (SMN) protein due to a damaged or missing SMN1 gene, with a spectrum of disease severity.⁷ Some individuals with SMA may never sit; some sit but never walk; and some walk but may lose that ability over time.⁸ In the absence of treatment, children with the most severe form of SMA would not be expected to reach their second birthday.⁷

SMA impacts approximately one in 11,000 live births in the U.S.,⁹ is a leading cause of genetic death among infants⁷ and causes a range of disability in teenagers and adults.⁸

About Biogen

At Biogen, our mission is clear: we are pioneers in neuroscience. Biogen discovers, develops and delivers worldwide innovative therapies for people living with serious neurological and neurodegenerative diseases as well as related therapeutic adjacencies. One of the world's first global biotechnology companies, Biogen was founded in 1978 by Charles Weissmann, Heinz Schaller, Kenneth Murray and Nobel Prize winners Walter Gilbert and Phillip Sharp. Today Biogen has the leading portfolio of medicines to treat multiple sclerosis, has introduced the first approved treatment for spinal muscular atrophy, commercializes biosimilars of advanced biologics and is focused on advancing research programs in multiple sclerosis and neuroimmunology, Alzheimer's disease and dementia, neuromuscular disorders, movement disorders, ophthalmology, neuropsychiatry, immunology, acute neurology and neuropathic pain.

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

Biogen Safe Harbor

This news release contains forward-looking statements, including statements made pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995, about the potential benefits, safety and efficacy of SPINRAZA; the results of certain real-world data and clinical studies of SPINRAZA; the identification and treatment of SMA; our research and development program for the treatment of SMA; the potential benefits and results from early treatment of SMA and/or higher dose SPINRAZA; the potential of our commercial business, including SPINRAZA; and risks and uncertainties associated with drug development and commercialization. These statements may be identified by words such as "aim," "anticipate," "believe," "could," "estimate," "expect," "forecast," "goal," "intend," "may," "plan," "possible," "potential," "will," "would" and other words and terms of similar meaning. You should not place undue reliance on these statements or the scientific data presented.

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 SPINRAZA; unexpected concerns that may arise from additional data, analysis or results obtained during clinical studies; regulatory authorities may require additional information or further studies, or may fail or refuse to approve or may delay approval of higher dose SPINRAZA; the occurrence of adverse safety events; risks of unexpected costs or delays; the risks of other unexpected hurdles; 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; and the direct and indirect impacts of the ongoing COVID-19 pandemic on our business, 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 are based on our current beliefs and expectations and speak only as of the date of this news release. We do not undertake any obligation to publicly update any forward-looking statements, whether as a result of new information, future developments or otherwise.

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