Landmark NURTURE Study of Infants with Spinal Muscular Atrophy (SMA) Treated Pre-Symptomatically with SPINRAZA® (nusinersen) Published in Neuromuscular Disorders

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- Treatment with SPINRAZA resulted in survival of 100 percent of infants, with none requiring permanent ventilation
- Motor milestones achieved included 100 percent of participants sitting without support and 88 percent walking independently; unprecedented outcomes in comparison to the natural history of the disease
- Study results underscore the established safety profile, proven efficacy and durability for up to nearly four years of SPINRAZA, the only SMA treatment approved for infants, children and adults

CAMBRIDGE, Mass., Oct. 02, 2019 (GLOBE NEWSWIRE) -- Biogen Inc. (Nasdaq: BIIB) today announced that the journal *Neuromuscular Disorders* has published data from NURTURE, the first study investigating a treatment targeting the underlying cause of spinal muscular atrophy (SMA) in infants treated pre-symptomatically. Data from the NURTURE study demonstrated that infants who initiated treatment with SPINRAZA prior to the onset of clinical symptoms attained unparalleled results compared to the natural history of the disease. As of March 2019 all participants were alive, without the need for permanent ventilation and experienced continuous improvements with the majority achieving motor milestones in timelines consistent with normal development. The results also demonstrated durability of effect with children making progress throughout the study.

“This pioneering study has far surpassed what we thought was possible and redefined our expectations of how early treatment helps individuals with SMA achieve optimal outcomes,” said Darryl De Vivo, M.D., Sidney Carter Professor of Neurology and Pediatrics, Columbia University Irving Medical Center in New York. “The extraordinary results achieved in this study underline the critical importance of newborn screening and early treatment of the clinically normal infants with SMA before the onset of any clinical symptoms.”

NURTURE is an ongoing, Phase 2, open-label study of 25 infants with the genetic diagnosis of SMA (most likely to develop SMA Type 1 or 2) who received their first dose of SPINRAZA in the pre-symptomatic stage and before six weeks old. The study, conducted at 15 sites in seven countries, has results up to 45.4 months. When compared with the natural history of the disease, the results are dramatic in their impact on changing the course of SMA.

As of March 2019 all infants in the study were 25 months or older, past the typical age of symptom onset for SMA Type 1 and Type 2, and were alive without the need for permanent ventilation. In comparison to the natural history of SMA, many of these infants would likely have passed away or require permanent ventilation on average by 13.5 months. In both the children with two and three copies of SMN2, treatment with SPINRAZA demonstrated rapid onset of improvement and durability of effect with their mean Children’s Hospital of Pennsylvania Infant Test of Neuromuscular Disorders (CHOP-INTEND) score of motor function reaching the maximum mean score of 64 for all participants with three copies of SMN2 (n=10) and a mean of 62.1 for those with two copies of SMN2 (N=15).

Additional highlights include:

- The majority of study participants achieved motor milestones in timeframes consistent with the World Health Organization (WHO) standards, with 100 percent sitting independently and 88 percent walking independently.
- Hammersmith Infant Neurologic Examination, Section 2 (HINE-2) development of motor function scores increased for all participants with the mean score for both those with two or three SMN2 copies approaching the maximum score of 26 points at the last assessment.
- SPINRAZA demonstrated longer term efficacy up to nearly four years, with participants continuing to make progress.
- SPINRAZA was well-tolerated with no new safety concerns identified after up to nearly four years of treatment.

These published results from the NURTURE study were previously presented at the 2019 Cure SMA Annual SMA Conference and the 5th Congress of the European Academy of Neurology.

About SPINRAZA® (nusinersen)[1-3]
SPINRAZA is the first therapy approved to treat infants, children and adults with spinal muscular atrophy (SMA) and is available in more than 40 countries. As of June 30, 2019, more than 8,400 individuals have been treated with SPINRAZA for up to nearly six years, based on patients across the post-marketing setting, Expanded Access Program (EAP) and clinical trial participants. SPINRAZA is the only SMA treatment to combine unsurpassed real-world experience and the highest level of clinical evidence across a broad spectrum of patient populations.

SMA is a rare, genetic, neuromuscular disease that is characterized by a loss of motor neurons in the spinal cord and lower brain stem, resulting in severe, progressive muscle atrophy and weakness. Approximately one in 10,000 live births have a diagnosis of SMA, and people of all ages are impacted by the disease. It is a leading genetic cause of infant mortality.

SPINRAZA, a foundation of care in SMA, is an antisense oligonucleotide (ASO), developed using Ionis’ proprietary technology that is designed to target a root cause of SMA by increasing the amount of full-length survival motor neuron (SMN) protein, which is critical to maintaining motor neurons. It is administered by intrathecal injection into the fluid surrounding the spinal cord where motor neurons reside to deliver the treatment where the disease starts.

SPINRAZA currently maintains the largest clinical data set in SMA based on data from over 300 patients across a broad range of SMA populations demonstrating a favorable benefit:risk profile. SPINRAZA was evaluated in two randomized, double-blind, sham-controlled studies (ENDEAR and CHERISH) in infantile and later-onset SMA patients and supported by open-label studies in pre-symptomatic infants (NURTURE) and individuals who were treated into adulthood with later-onset SMA (CS2/CS12). The most common adverse events observed were respiratory infection, fever, constipation, headache, vomiting and back pain. Meningitis and hydrocephalus have been observed in the post-marketing setting. Renal toxicity and coagulation abnormalities, including acute severe low platelet counts, have been observed after administration of some ASOs. Laboratory tests can monitor for these signs.

Biogen licensed the global rights to develop, manufacture and commercialize SPINRAZA from Ionis Pharmaceuticals, Inc. (Nasdaq: IONS), a leader in antisense therapeutics. Biogen and Ionis conducted an innovative clinical development program that moved SPINRAZA from its first dose in humans in 2011 to its first regulatory approval in five years.

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, and today 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, neuromuscular disorders, movement disorders, Alzheimer's disease and dementia, ophthalmology, immunology, neurocognitive disorders, acute neurology and pain.

We routinely post information that may be important to investors on our website at www.biogen.com. To learn more, please visit www.biogen.com and 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; the identification and treatment of SMA; our research and development program for the treatment of SMA; and the potential benefits and results from early treatment of SMA. 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 risks relating to the occurrence of adverse safety events and/or unexpected concerns that may arise from additional data or analysis; failure to obtain regulatory approvals in other jurisdictions; risks of unexpected 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; regulatory authorities may require additional information or further studies; product liability claims; and third party collaboration risks. 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.

References

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