New SPINRAZA® (nusinersen) Data Presented at Annual Congress of the World Muscle Society Demonstrate Benefits in Treating Presymptomatic Infants with Spinal Muscular Atrophy

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- NURTURE study participants were alive and did not require permanent ventilation, in contrast to natural history of spinal muscular atrophy (SMA)
- Study participants achieved motor milestones with 100 percent sitting independently and 88 percent able to walk
- Additional data feature biomarkers as an indicator for clinical development work in SMA

CAMBRIDGE, Mass., Oct. 06, 2018 (GLOBE NEWSWIRE) -- Biogen Inc. (Nasdaq: BIIB) today announced new interim results from NURTURE, an ongoing open-label, single-arm efficacy and safety study of SPINRAZA® (nusinersen) in 25 presymptomatic infants with SMA. The data are being presented today in a late-breaking session at the 23rd Annual Congress of the World Muscle Society (WMS) held in Mendoza, Argentina.

“The NURTURE study results demonstrate that early diagnosis and treatment with SPINRAZA has the potential to dramatically change the course of SMA,” said Wildon Farwell, M.D., senior medical director, clinical development at Biogen. “This is the longest available span of data on infants with SMA who began treatment in a presymptomatic period and indicates that children treated early with SPINRAZA can achieve motor milestones they would likely not attain without treatment.”

The interim analysis evaluated survival and respiratory intervention rates in infants (n=25) who were genetically diagnosed with SMA and began treatment in the presymptomatic stage of the disease. As of May 2018 all patients in the study were alive and none required tracheostomy or permanent ventilation. Additionally, 22 of the 25 participants were able to walk either with assistance or independently according to the motor milestone standard of the World Health Organization and all 25 were able to sit without support.

The motor skills of study participants were also evaluated using the Children’s Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP INTEND), an assessment which considers 16 different types of movement to create an overall score between zero and 64. The mean CHOP INTEND scores were 62.6 for study participants with three copies of the SMN2 gene and 61.0 for those with two copies of the gene.

All NURTURE study participants were 14 months or older at the time of the analysis. Participants included infants with two copies of the SMN2 gene (n=15) who are likely to develop a fatal, early-onset form of SMA known as Type 1, and infants with three copies of the SMN2 gene (n=10) who typically develop SMA Type 2 or 3. People living with SMA Types 2 and 3 may never be able to walk or will lose that ability over time. No new safety concerns were identified.

Additional research presented at WMS compared levels of phosphorylated neurofilament heavy chain (pNF-H) in plasma in more than 300 patients from SPINRAZA clinical trials, including those in the NURTURE study, and a control group of people without SMA. The data demonstrated that treatment with SPINRAZA is associated with a rapid decline followed by stabilization of pNF-H in plasma at levels close to those of healthy controls. The results are part of Biogen’s ongoing work to identify and validate biomarkers that could provide insight on the disease progression of SMA.

“We continue to develop tools to inform our clinical research and are encouraged by the potential of neurofilament as a biomarker for SMA, how it could further expand the scientific understanding of this rare disease and, more importantly, its potential impact on those living with SMA,” continued Farwell.

SPINRAZA Program Status

SPINRAZA is the first and only approved medicine for the treatment of spinal muscular atrophy (SMA) and is currently approved in the U.S., the European Union, Japan and Brazil, among other countries. Biogen has submitted regulatory filings in additional countries and plans to initiate additional filings in other countries. As of June 30, 2018, more than 5,000 individuals with SMA are being treated with SPINRAZA worldwide, based on patients across the post-marketing setting, Expanded Access Program (EAP) and clinical trial participants.

To support the urgent need for treatment for the most severely affected individuals living with SMA, Biogen initiated one of the largest, global, pre-approval EAPs in any rare disease, providing access to therapy free of charge. From its launch to June 30, 2018, the EAP has provided treatment access to more than 750 patients across 29 countries. Biogen also supports a Named Patient Sales Program (NPP), which allows access to SPINRAZA in countries where it is not commercially available.

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

About SMA[1-5]

SMA is a rare, genetic, neuromuscular disease that is characterized by loss of motor neurons in the spinal cord and lower brain stem, resulting in severe and progressive muscle atrophy and weakness. Ultimately, individuals with SMA may lose the ability to walk and can have difficulty performing the basic functions of life, such as breathing and swallowing, which results in significant healthcare intervention and caregiver assistance. Left untreated, the majority of infants with the most severe form of the disease do not live to see their second birthday without respiratory intervention.

Due to a deletion of, or mutation in, the SMN1 gene, people with SMA do not produce enough survival motor neuron (SMN) protein, which is critical for the maintenance of motor neurons. The severity of SMA correlates with the amount of SMN protein an individual has. People with Type 1 SMA, the form that requires the most intensive and supportive care, produce very little SMN protein and do not achieve the ability to sit without support or live beyond two years without respiratory support. People with Type 2 and Type 3 SMA produce greater amounts of SMN protein and have less severe, but still life-altering forms of SMA.

About SPINRAZA® (nusinersen)

SPINRAZA is an antisense oligonucleotide (ASO), developed using Ionis' proprietary antisense technology, that is designed to treat SMA caused by mutations or deletions in the SMN1 gene, located in chromosome 5q, that leads to a deficiency in SMN protein. SPINRAZA alters the splicing of SMN2 pre-mRNA in order to increase production of full-length SMN protein.[6] ASOs are short synthetic strings of nucleotides designed to selectively bind to target RNA and regulate gene expression. Through use of this technology, SPINRAZA has been shown to increase the amount of full-length SMN protein in individuals with SMA.[7]

SPINRAZA must be administered via intrathecal injection, which delivers therapies directly into the cerebrospinal fluid (CSF) around the spinal cord,[8] where motor neurons degenerate in individuals with SMA due to insufficient levels of SMN protein.[9]

In the clinical trial program, SPINRAZA demonstrated a favorable benefit-risk profile. The most common adverse reactions that occurred in the SPINRAZA group were respiratory infection and constipation. Serious adverse reactions of atelectasis were more frequent in SPINRAZA-treated patients. Coagulation abnormalities and thrombocytopenia, including acute severe thrombocytopenia, have been observed after administration of some ASOs. Individuals may be at increased risk of bleeding complications. Renal toxicity has been observed after administration of some ASOs. SPINRAZA is present in and excreted by the kidney.

Please click for Important Safety Information and full Prescribing Information in the U.S. and Europe, or visit your respective country's product website.

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. 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 and only approved treatment for spinal muscular atrophy and is focused on advancing neuroscience research programs in Alzheimer's disease and dementia, multiple sclerosis and neuroimmunology, movement disorders, neuromuscular disorders, pain, ophthalmology, neuropsychiatry and acute neurology. Biogen also manufactures and commercializes biosimilars of advanced biologics.

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 press 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 status of Biogen's current regulatory filings; Biogen's plans for additional regulatory filings in other jurisdictions; availability of patient access and reimbursement pathways, which may vary on a country-by-country basis; the identification and treatment of SMA; the potential of Biogen's 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" 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 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 commercialization of SPINRAZA, which may be impacted by, among other things, the level of preparedness of healthcare providers to treat patients, difficulties in obtaining or changes in the availability of reimbursement for SPINRAZA, the effectiveness of sales and marketing efforts, problems with the manufacturing process for SPINRAZA, the occurrence of adverse safety events and/or unexpected concerns that may arise from additional data or analysis; ; regulatory authorities may require additional information or further studies, or may fail or refuse to approve or may delay approval of SPINRAZA or expansion of product labeling; the occurrence of adverse safety events; risks of unexpected costs or delays; we may encounter 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 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 Securities and Exchange Commission. These statements are based on our current beliefs and expectations and speak only as of the date of this press 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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