New Data Presented at MDA Clinical Conference Show Benefit in Motor Function for Infants, Teens and Young Adults Treated With SPINRAZA® (nusinersen)

March 12, 2018

- NURTURE study results reported that all study participants (25 infants) were alive and experienced improved motor function at the time of reporting, compared to a decline seen in SMA natural history
- One-hundred percent of NURTURE participants achieved the age-expected World Health Organization motor milestone of sitting without support – a development never seen with SMA Type 1
- Case series conducted on SPINRAZA-treated teens and young adults with SMA Type 2 and 3 (17 to 19 years old upon their last visit) demonstrated stable or improved motor function and improved quality of life

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Biogen (Nasdaq: BIIB) today announced new interim Phase 2 results from NURTURE, the ongoing open-label, single-arm study evaluating the efficacy and safety of SPINRAZA® (nusinersen) among pre-symptomatic infants with spinal muscular atrophy (SMA). In NURTURE, all infants treated with SPINRAZA were alive, did not require permanent ventilation and showed improvement in motor function and motor milestone achievements as of July 5, 2017, compared to the disease's natural history. This study, along with a case series demonstrating SPINRAZA's effectiveness among teens and young adults, will be presented at the Muscular Dystrophy Association (MDA) Clinical Conference on March 11-14, 2018, in Arlington, Virginia.

"These results reinforce SPINRAZA's effectiveness as the first and only approved treatment for SMA and demonstrate once again the benefit it can bring to individuals with SMA, including infants, teens and young adults," said Alfred Sandrock, M.D., Ph.D., executive vice president and chief medical officer at Biogen. "SPINRAZA is supported by the largest well-controlled SMA clinical development program conducted to date and we are committed to ongoing research in SMA. Biogen looks forward to working with healthcare providers and institutions in order to provide teens and young adults with SMA access to SPINRAZA, a treatment that has demonstrated significant benefit in the maintenance of motor function for these patients."

In the NURTURE study, SPINRAZA was administered to infants six weeks old or younger (n=25), who were in the pre-symptomatic stage, genetically-diagnosed with SMA and had two or three copies of the SMN2 gene (n=15 for two copies (most likely to develop Type 1 SMA); n=10 for three copies (most likely to develop Type 2 SMA)). At the time of this interim analysis, infants had been followed for up to 25.6 months – well beyond the typical timeframe when most infants with Type 1 SMA would have required permanent ventilation or died. The interim analysis, titled, "Nusinersen in Infants Who Initiate Treatment in a Pre-Symptomatic State of SMA: Interim Efficacy and Safety Results from the Phase 2 NURTURE Study," showed that all infants were alive and none required tracheostomy or permanent ventilation.

"The NURTURE findings document the continuing benefits that SPINRAZA provides for patients with SMA who initiated treatment in early infancy while clinically pre-symptomatic, including age-appropriate developmental gains in motor function and motor milestone achievements," said Dr. Darryl C. De Vivo, M.D., lead study author, Columbia University Medical Center, New York. "The treated infants in the NURTURE study had genetic SMA and were likely to clinically develop Type 1 or 2, yet with enough observation time they have all achieved independent sitting and the majority have developed the ability to walk."

NURTURE participants also achieved a mean CHOP INTEND score, which measures general motor function among infants with SMA, of 58.4 at last visit (out of a maximum score of 64). Many continue to improve and maintain these scores beyond a point in time at which untreated individuals with Type 1 SMA would experience a significant decline. Overall, the study showed that SPINRAZA was well-tolerated and no new safety concerns were identified.

Also presented at the MDA Clinical Conference was a case series, "Nusinersen Experience in Teenagers and Young Adults With Spinal Muscular Atrophy," which showcased SPINRAZA's stabilizing or improving effect on teens and young adults with Type 2 or 3 SMA.

In the case series, participants (n=5) were 14 to 15 years old at the start of Study CS2, and 17 to 19 years old at the time of their last visit in the extension Study CS12. One participant was Type 2 and four were Type 3, and all received multiple doses of SPINRAZA over 2.5 years of observation. The results included improvement on the Hammersmith Functional Motor Scale–Expanded (HFMSE); stabilization on the Upper Limb Module (ULM); improvement in the Six-Minute Walk Test (6MWT); and stable or improved scores on the Assessment of Caregiver Experience with Neuromuscular Disease (ACEND).

"The case series demonstrated SPINRAZA's effectiveness in teens and young adults with SMA Type 2 or 3," said Dr. John Day, M.D., Ph.D., lead study author, Stanford University Medical Center, California. "The study participants demonstrated stable or slightly improved motor function and quality of life during two years of treatment – and even afterward, we have continued to see improved and maintained stability and motor benefits with SPINRAZA. This differs importantly from untreated teens and young adults with SMA, who experience a decline in motor function, specifically in reduced walking distance and upper limb activities, as well as in health-related quality of life."

For more information about SPINRAZA and prescribing information in the United States, please visit www.SPINRAZA.com. Prescribing information in the European Union is available at http://www.ema.europa.eu/ema/.

SPINRAZA Program Status
SPINRAZA is the first and only approved medicine for the treatment of SMA and is currently approved in the United States, the European Union, Brazil, Japan, Switzerland, Australia, South Korea, and Canada. Biogen has submitted regulatory filings in additional countries and plans to initiate additional filings in other countries. According to commercial, Early Access Program and clinical trial participant patient data as of December 31, 2017, more than 3,200 individuals with SMA are being treated with SPINRAZA worldwide.

Globally, starting in 2016, in response to the urgent need for treatment for the most severely affected individuals living with SMA, Biogen sponsored one of the largest, pre-approval Expanded Access Programs (EAP) in rare disease, free of charge.

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 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 characterized by loss of motor neurons in the spinal cord and lower brain stem, resulting in severe and progressive muscular atrophy and weakness. Ultimately, individuals with the most severe type of SMA can become paralyzed and have difficulty performing the basic functions of life, like breathing and swallowing.

Due to a loss of, or defect 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. 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 being developed globally for the treatment of SMA.

SPINRAZA is an antisense oligonucleotide (ASO), 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 SMN protein deficiency. 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 the potential to increase the amount of full-length SMN protein in individuals with SMA.

SPINRAZA must be administered via intrathecal injection, which delivers therapies directly to the cerebrospinal fluid (CSF) around the spinal cord,[7] where motor neurons degenerate in individuals with SMA due to insufficient levels of SMN protein.[8]

SPINRAZA demonstrated a favorable benefit-risk profile. The most common adverse reactions reported for SPINRAZA were upper respiratory infection, lower 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.

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. Founded in 1978 as one of the world's first global biotechnology companies by Charles Weissman, 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 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 relating to 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, and availability of patient access and reimbursement pathways, which may vary on a country-by-country basis. These forward-looking statements may be accompanied by words such as "aim," "anticipate," "believe," "could," "estimate," "except," "forecast," "intend," "may," "plan," "potential," "possible," "will," and other words and terms of similar meaning. Drug development and commercialization involve a high degree of risk. 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 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 to approve or may delay approval of Biogen's drug candidates or expansion of product labeling; Biogen may encounter other unexpected hurdles which may be impacted by, among other things, the occurrence of adverse safety events, failure to obtain regulatory approvals in certain jurisdictions, or failure to protect intellectual property and other proprietary rights; product liability claims; or third party collaboration risks. The foregoing sets forth many, but not all, of the factors that could cause actual results to differ from Biogen's expectations in any forward-looking statement. Investors should consider this cautionary statement, as well as the risk factors identified in Biogen's most recent annual or quarterly report and in other reports Biogen has filed with the U.S. Securities and Exchange Commission. These statements are based on Biogen's current beliefs and expectations and speak only as of the date of this press release. Biogen does 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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