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New Data Show SPINRAZA™ (nusinersen) Significantly Reduces Risk of Death or Permanent Ventilation in Infantile-Onset Spinal Muscular Atrophy

Primary Endpoint from End of Study Analysis Presented at British Paediatric Neurology Association Annual Conference

Cambridge, Mass., January 13, 2017 – Biogen presented new data from the Phase 3 ENDEAR study of SPINRAZA™ (nusinersen), which demonstrated a statistically significant reduction in the risk of death or permanent ventilation in SPINRAZA-treated infants with spinal muscular atrophy (SMA) compared to untreated infants. The data were presented at the British Paediatric Neurology Association (BPNA) annual conference in Cambridge, UK, 11-13 January 2017.

In August 2016, Biogen reported that ENDEAR met its pre-specified primary endpoint at the interim analysis, the proportion of motor milestone responders as measured by the Hammersmith Infant Neurological Examination (HINE). Following the positive interim analysis, Biogen ended the study early so that all participants could have the option to receive SPINRAZA in an open-label extension study. Today, Biogen provided the first presentation of the pre-specified primary endpoint, time to death or permanent ventilation, from the end of study (EOS) analysis. The EOS results presented at BPNA include data from patients' final study visit, which occurred after the announcement that the study was being stopped and was not part of the interim analysis.

"Although ENDEAR was stopped early based on positive interim results, the study still demonstrated that a significantly greater number of infants treated with SPINRAZA survived and did not require permanent ventilation. These data further underscore the impact SPINRAZA may have on individuals living with this devastating disease," said Wildon Farwell, M.D., M.P.H., senior medical director, Clinical Development, Biogen. "We're very encouraged that individuals with SMA have already started treatment with SPINRAZA this week in the U.S., and we continue to work closely with regulatory agencies to bring this therapy to patients around the world as quickly as possible."

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SPINRAZA met the pre-specified primary endpoint at the ENDEAR EOS, demonstrating a statistically significant 47% reduction in the risk of death or permanent ventilation ($p<0.01$). In the EOS analysis, a greater percentage of untreated infants (68%) died or required permanent ventilation compared to infants treated with SPINRAZA (39%).

SPINRAZA demonstrated a favorable safety profile, with commonly reported adverse events including respiratory events and constipation, consistent with those expected in the general population of infants with SMA. Further EOS efficacy and safety results from ENDEAR will be presented at a future medical congress.

ENDEAR was a randomized, double-blind, sham-controlled study in patients with infantile-onset (most likely to develop Type 1) SMA. The EOS efficacy analysis included all patients (n=121) who had their final study visit after the interim analysis (n=78) and had the opportunity to attend the six-month study visit assessment.

SPINRAZA Program Status

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 2016.

Efficacy and safety data from the ENDEAR interim analysis, as well as open-label data in pre-symptomatic and symptomatic patients with, or likely to develop, Types 1, 2 and 3 SMA, supported the FDA approval of SPINRAZA in the U.S. for the treatment of SMA in pediatric and adult patients, representing the first approved treatment for individuals with SMA.[1]

In October 2016, the European Medicines Agency (EMA) validated Biogen's Marketing Authorization Application (MAA) for SPINRAZA, and the EMA's Committee for Medicinal Products for Human Use (CHMP) granted Accelerated Assessment status. In addition, Biogen has submitted regulatory filings in Japan, Canada and Australia and plans to initiate additional filings in other countries in 2017.

About SMA[2-6]

Spinal muscular atrophy (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 most severe life-threatening form, 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.

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To support awareness and education about SMA, Biogen has launched Together in SMA in the United States and Japan. Together in SMA is a program created to provide informational materials and resources to the SMA community. Learn more at www.TogetherinSMA.com (U.S.-only) and www.TogetherinSMA.jp/ (Japan-only).

About SPINRAZA™ (nusinersen)
SPINRAZA is being developed globally for the treatment of SMA.

SPINRAZA is an antisense oligonucleotide (ASO) that is designed to treat SMA caused by mutations in the 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.[7] 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 patients with SMA.

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

The most common adverse reactions reported for SPINRAZA were lower respiratory infection, upper 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 antisense oligonucleotides. Individuals may be at increased risk of bleeding complications. Renal toxicity, including potentially fatal glomerulonephritis, has been observed after administration of some antisense oligonucleotides. SPINRAZA is present in and excreted by the kidney.

For complete SPINRAZA U.S. prescribing information please visit www.SPINRAZA.com.

About Patient Support in the U.S.
As part of Biogen's commitment to patients and families living with SMA, the company has launched SMA360°™, which provides certain services that address nonmedical barriers to access in the U.S. These include logistical assistance, product education, insurance benefits investigations and financial assistance. A list of the SMA360° offerings is available at www.SPINRAZA.com.

SMA360° services from Biogen are available only to those eligible patients who have been prescribed SPINRAZA in the U.S. To learn more about the program and receive additional information about these services, please contact an SMA Support Coordinator at 1-844-4SPINRAZA (1-844-477-4672) Monday-Friday 8:30 a.m.-8:00 p.m. EST.

About Biogen
Through cutting-edge science and medicine, Biogen discovers, develops and delivers worldwide innovative therapies for people living with serious neurological, autoimmune and rare diseases. Founded in 1978, Biogen is one of the world's oldest independent biotechnology companies and patients worldwide benefit from its leading multiple sclerosis and innovative hemophilia therapies. For more information, please visit www.biogen.com. Follow us on Twitter.

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Biogen Safe Harbor

This press release contains forward-looking statements, including statements relating to the potential benefits, safety and efficacy of SPINRAZA, the status of current regulatory filings, and plans for additional regulatory filings in other jurisdictions. These statements may be identified by words such as “believe,” “except,” “may,” “plan,” “potential,” “will” and similar expressions, and are based on our current beliefs and expectations. You should not place undue reliance on these statements. These statements involve risks and uncertainties that could cause actual results to differ materially from those reflected in such statements, including 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, failure to obtain regulatory approvals in other jurisdictions, failure to protect intellectual property and other proprietary rights, product liability claims, third party collaboration risks, and the other risks and uncertainties that are described in the Risk Factors section of Biogen’s most recent annual or quarterly report and in other reports Biogen has filed with the U.S. Securities and Exchange Commission (SEC). Any forward-looking statements speak only as of the date of this press release and we assume no obligation to update any forward-looking statement.

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