Biogen Enhances Commitment to SMA Research with Data Presented at Annual SMA Conference

June 13, 2018

- Research includes clinical trial data across broad SMA patient populations, expanding scientific understanding of SMA and SPINRAZA® (nusinersen)
- Study presented examines neurofilament (pNF-H) as a potential biomarker for SMA, which could provide benefits for future research and deeper understanding of the disease

CAMBRIDGE, Mass., June 13, 2018 (GLOBE NEWSWIRE) -- Biogen (Nasdaq:BIIB) today announced it will present data from its SPINRAZA clinical development program – the largest of its kind, with more than six years of data – for spinal muscular atrophy (SMA) at the Cure SMA 2018 Annual SMA Conference in Dallas, TX (June 14-17, 2018).

"We are proud to support Cure SMA in advancing scientific understanding of the disease to improve the lives of individuals with SMA and their families. Data presented include analyses of disease burden, SPINRAZA treatment, and phosphorylated neurofilament heavy chain (pNF-H) in plasma and SMA clinical characteristics, which could help shape the future of SMA research and treatment," said Alfred Sandrock, M.D., Ph.D., executive vice president and chief medical officer at Biogen. "We remain dedicated to sharing our ongoing experience with SPINRAZA, the first and only approved treatment for SMA, among patients of all ages, while also building a portfolio of treatments for this devastating disease."

Presentations at the Annual SMA Conference – including data from Biogen's ongoing open-label trials, hospital care and potential biomarkers – reiterate SPINRAZA's effectiveness across broad SMA populations and expand scientific understanding of the disease and treatment options. One evaluation will feature pNF-H in plasma as a potential indicator of SMA disease activity that, through further research, could help provide a better understanding of the disease. Neurofilaments have been associated with disease activity across a range of neurological diseases.

Additional presentations include interim analyses from the SHINE and NURTURE studies, which assess SPINRAZA's safety and efficacy among those with infantile-onset SMA. A separate presentation will examine the experience and disease burden of untreated infants and children with SMA in hospitals, providing more context about the disease's natural history.

Biogen's SPINRAZA and SMA presentations include:

Platform presentations

- Phosphorylated Neurofilament Heavy Chain (pNF-H) As a Potential Biomarker of SMA Disease Activity: pNF-H Levels at Baseline and During Treatment in the Nusinersen Clinical Trial Program – *Friday, June 15, 11:40 a.m. CT*
- Longer-Term Assessment of the Safety and Efficacy of Nusinersen for the Treatment of Infantile-onset Spinal Muscular Atrophy (SMA): An Interim Analysis of the SHINE Study – *Saturday, June 16, 10:00 a.m. CT*
- Nusinersen in Infants Who Initiate Treatment in a Presymptomatic Stage of SMA: Interim Efficacy and Safety Results From the Phase 2 NURTURE Study – *Saturday, June 16, 10:20 a.m. CT*

Posters

- Survival and Ventilation Among Those with Type I SMA: Results From the 2017 Cure SMA Membership Survey*(joint presentation between Cure SMA and Biogen) – Poster 39 – Thursday, June 14, 4:30 p.m. CT*
- Characterization of Infant and Early Childhood Spinal Muscular Atrophy Patients and Their Transitions of Care Within U.S. Hospitals – *Poster 26 – Friday, June 15, 12:30 p.m. CT*

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 United States, 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 March 31, 2018, more than 4,100 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. To date, 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 we have not yet commercialized the medicine.

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.

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/.

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 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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