Biogen's SPINRAZA® (nusinersen) Receives Positive Recommendation from NICE for Funding in the United Kingdom for the Treatment of Infants, Children and Adults with Spinal Muscular Atrophy

May 14, 2019

- SPINRAZA becomes the first therapy recommended in the United Kingdom for 5q spinal muscular atrophy to treat all age groups, including patients who are pre-symptomatic
- More than 7,500 individuals have been treated with SPINRAZA worldwide in over 40 countries across the Expanded Access Program, clinical trials and post-marketing setting*
- Therapy is supported by a comprehensive clinical data set including new data on the longer-term durability and safety profile presented at the American Academy of Neurology Annual meeting

CAMBRIDGE, Mass., May 14, 2019 (GLOBE NEWSWIRE) -- Biogen Inc. (Nasdaq: BIIB) today announced that The National Institute for Health and Care Excellence (NICE) in the United Kingdom has recommended funding for SPINRAZA (nusinersen) on the National Health Service (NHS). The positive recommendation is for the treatment of infants, children and adults with 5q spinal muscular atrophy (SMA), including pre-symptomatic and symptomatic SMA Types 1, 2 and 3. SMA is a rare, debilitating and life-threatening disease that results in severe, progressive muscular atrophy and weakness.

"We applaud the decision by NICE to recommend funding for SPINRAZA in the United Kingdom. This is a momentous occasion for patients and their families and the result of a strong collaboration between Biogen, NICE, NHS and the SMA community," said Chirfi Guindo, Executive Vice President, Global Product Strategy and Commercialization at Biogen. "We are committed to working with authorities to find solutions to fund innovation and provide broad patient access through value-based contracting programs and by enabling governments to leverage savings created by our biosimilars portfolio."

Established in 1999, NICE provides advice and standards on value in healthcare to the NHS. The NICE recommendation was based on the comprehensive set of data for nusinersen highlighting its clinically meaningful benefits for individuals in all age groups with SMA. New data on the efficacy and safety of nusinersen was presented at the 71st Annual Meeting of the American Academy of Neurology in Philadelphia (May 4-10). SPINRAZA is an antisense oligonucleotide (ASO) that targets the underlying cause of the disease in order to increase production of full-length survival motor neuron protein.

The decision builds on Biogen's commitment to find solutions to provide broad access to innovative therapies by collaborating closely with governments and communities around the world on new business models. In Europe, a key component of that work is Biogen's portfolio of biosimilars — biologic medicines that are similar to currently available biologic therapies known as originators. Biosimilar products benefit patients and are strategically important as Biogen works with payers and health systems globally with the goal of creating room in healthcare budgets to provide access for patients to innovative therapies. In Europe, approximately 145,000 patients have been treated with a Biogen biosimilar and, based on internal estimates, Biogen expects the uptake to contribute an estimated healthcare savings of up to 1.8 billion euros in 2019.

About SPINRAZA® (nusinersen)[1-4]
SPINRAZA is the first approved medicine for the treatment of spinal muscular atrophy (SMA) and is currently available in more than 40 countries. As of March 31, 2019, more than 7,500 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.

SPINRAZA is an antisense oligonucleotide (ASO) developed using Ionis' proprietary antisense technology that is designed to treat the root cause of SMA. SPINRAZA alters the splicing of SMN2 pre-mRNA in order to increase production of full-length spinal motor neuron (SMN) protein. 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. SPINRAZA is administered via intrathecal injection, which delivers therapies directly into the cerebrospinal fluid (CSF) around the spinal cord, where motor neurons degenerate in individuals with SMA due to insufficient levels of SMN protein.

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.

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, 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[2,5]
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. About 1 in 10,000 live births have a diagnosis of SMA. Ultimately, individuals with SMA can lose the ability to walk and 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 (SMA Type 1) do not live beyond their second birthday without respiratory intervention. People with childhood or adult onset SMA (Type 2 or 3) produce greater amounts of SMN protein resulting in less severe, but still life-altering forms of the disease.

Due to a deletion of, or mutation in, the SMN1 gene, people with SMA do not produce enough 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 SMA Type 1, 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 typically live beyond two years without respiratory support. People with SMA Type 2 and Type 3 produce greater amounts of SMN protein and have less severe, but still life-altering forms of SMA.

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 and only approved treatment for spinal muscular atrophy and is focused on advancing neuroscience research programs in multiple sclerosis and neuroimmunology, Alzheimer's disease and dementia, movement disorders, neuromuscular disorders, acute neurology, neurocognitive disorders, pain and ophthalmology. Biogen also 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 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 status of current regulatory filings; and the potential of our commercial business, including SPINRAZA and our biosimilars portfolio. 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. 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 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; 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; risks related to our dependence on third parties for the development and commercialization of biosimilars; risks of legal actions, regulatory scrutiny or other challenges to biosimilars; 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 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.

Reference:
* As of March 31, 2019, more than 7,500 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.

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