International Prix Galien Recognizes SPINRAZA® as Best Biotechnology Product

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Rare disease therapy honored with seven Prix Galien awards, including six independent country recognitions, for innovation in treating spinal muscular atrophy

CAMBRIDGE, Mass., Nov. 29, 2018 (GLOBE NEWSWIRE) -- Biogen Inc. (Nasdaq: BIIB) has been awarded the 2018 International Prix Galien as Best Biotechnology Product for SPINRAZA (nusinersen), the first and only treatment for spinal muscular atrophy (SMA). The prestigious honor marks the seventh Prix Galien for SPINRAZA, following country recognitions in the U.S., Germany, Italy, Belgium-Luxembourg, the Netherlands and the U.K. The international award was presented at a ceremony in Dakar, Senegal on November 28, 2018.

"We are honored that the Galien Foundation continues to recognize the groundbreaking science and profound clinical impact of SPINRAZA with this international award," said Michel Vounatsos, chief executive officer of Biogen. "The ability to bring the first and only therapy to people living with SMA is the result of a close collaboration with the SMA community, academia, researchers and our colleagues at Ionis, which has made a meaningful difference in this area of high unmet medical need."

The International Prix Galien is given every two years by Prix Galien International Committee members in recognition of excellence in scientific innovation to improve human health. To qualify for International Prix Galien consideration, nominees must have won a Prix Galien in an individual member country.

SMA is a progressive, debilitating neuromuscular disease that impacts infants, children and adults. It affects approximately one in every 10,000 live births and results in muscle weakness which can take away a person's ability to walk, eat and ultimately breathe. [1] The U.S. Food and Drug Administration (FDA) approved SPINRAZA on December 23, 2016, under priority review for the treatment of SMA in pediatric and adult patients. In June 2017 SPINRAZA was granted marketing authorization in the European Union to treat individuals with 5q SMA, the most common form of the disease representing approximately 95 percent of all SMA cases.[2]

About The Galien Foundation
The Galien Foundation fosters, recognizes and rewards excellence in scientific innovation to improve the state of human health. The Foundation oversees and directs activities in the USA for the Prix Galien, an international award that recognizes outstanding achievements in improving the human condition through the development of innovative therapies. The Prix Galien was created in France in 1970 in honor of Galen, the father of medical science and modern pharmacology.

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, and Japan, among other countries. Biogen has submitted regulatory filings in additional countries and plans to initiate additional filings in other countries. As of September 30, 2018 nearly 6,000 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[1,3-6]
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)[7-10]
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. 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 must be 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.

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, relating to the potential benefits, safety and efficacy of SPINRAZA; the results of certain real-world data; the status of our current regulatory filings; our plans for additional regulatory filings in other jurisdictions; planning and timing for commercial launch; 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," "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 U.S. 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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