Biogen and Ionis Win Prestigious Prix Galien Award for SPINRAZA as Best Biotechnology Product

October 27, 2017

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Biogen (NASDAQ: BIIB) and Ionis have been awarded the prestigious 2017 Prix Galien USA Award for Best Biotechnology Product for SPINRAZA® (nusinersen). The Prix Galien USA Award recognizes extraordinary achievement in scientific innovation that improves the state of human health. The award was presented at a ceremony in New York City on October 26, 2017.

"We are humbled to be recognized by the Galien Foundation with Ionis for SPINRAZA, the first and only therapy to treat the devastating condition of spinal muscular atrophy," said Michel Vounatsos, chief executive officer at Biogen. "SPINRAZA's profound clinical impact is certainly the most rewarding outcome for all of us involved, and I want to thank our Biogen colleagues around the world for their passion and commitment to making a difference in this area of medical need."

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. SMA is a rare disease and leading genetic cause of death in infants marked by progressive, debilitating muscle weakness taking away a person's ability to walk, eat and ultimately breathe. SPINRAZA is an approved therapy for the treatment of SMA.

For more information about SPINRAZA and prescribing information in the United States, please visit www.SPINRAZA.com.

SPINRAZA Program Status
SPINRAZA is the first approved medicine for the treatment of SMA and is currently approved in the United States, the European Union, Brazil, Japan, Switzerland and Canada. Biogen has submitted regulatory filings in additional countries and plans to initiate additional filings in other countries.

Biogen licensed the global rights to develop, manufacture and commercialize SPINRAZA from Ionis Pharmaceuticals, 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 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.

About SMA [1-5]
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 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 Pharmaceuticals' 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 survival motor neuron (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 antisense oligonucleotides. Individuals may be at increased risk of bleeding complications. Renal toxicity has been observed after administration of some antisense oligonucleotides. 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 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, 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 current regulatory filings, 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,” “except,” “forecast,” “intend,” “may,” “plan,” “potential,” “possible,” “will” and other words and terms of similar meaning. You should not place undue reliance on these statements or the scientific data presented. Drug development and commercialization involve a high degree of risk. 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, 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; or 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, failure to obtain regulatory approvals in other jurisdictions, 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 our 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 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.

1. Darras B, Markowitz J, Monani U, De Vivo D. Chapter 8 - Spinal Muscular Atrophies. In: Vivo BTD, ed. Neuromuscular Disorders of Infancy, Childhood, and Adolescence (Second Edition). San Diego: Academic Press; 2015:117-145.

2. Lefebvre S, Burglen L, Reboullet S, et al. Identification and characterization of a spinal muscular atrophy-determining gene. Cell.1995;80(1):155-165.

3. Mailman MD, Heinz JW, Papp AC, et al. Molecular analysis of spinal muscular atrophy and modification of the phenotype by SMN2. Genet Med. 2002;4(1):20-26.

4. Monani UR, Lorson CL, Parsons DW, et al. A single nucleotide difference that alters splicing patterns distinguishes the SMA gene SMN1 from the copy gene SMN2. Hum Mol Genet. 1999;8(7):1177-1183.

5. Peeters K, Chamova T, Jordanova A. Clinical and genetic diversity of SMN1-negative proximal spinal muscular atrophies. Brain.2014;137(Pt 11):2879-2896.

6. Hua Y, Sahashi K, Hung G, Rigo F, Passini MA, Bennett CF, Krainer AR. Antisense correction of SMN2 splicing in the CNS rescues necrosis in a type III SMA mouse model. Genes Dev. 2010 Aug 1; 24(15):16344-44.

7. Evers MM, Toonen LJ, van Roon-Mom WM. Antisense oligonucleotides in therapy for neurodegenerative disorders. Adv Drug Deliv Rev. 2015;87:90-103.

8. Lunn MR, Wang CH. Spinal muscular atrophy. Lancet. 2008;371(9630):2120-2133.

Contact:

Biogen
MEDIA CONTACT:
Ligia Del Bianco, +1 617-914-6778
public.affairs@biogen.com
or
INVESTOR CONTACT:
Ben Strain, +1 781-464-2442
IR@biogen.com