New SPINRAZA® (nusinersen) Data Reinforce Sustained Efficacy and Longer-Term Safety Across Broad Range of Spinal Muscular Atrophy (SMA) Patients

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- Treatment with SPINRAZA improved or stabilized motor function across patient populations, including young adults
- The longer-term safety profile of SPINRAZA was consistent among a broad spectrum of ages and SMA types
- New data add to the significant body of evidence underscoring the clinically meaningful and sustained benefit of SPINRAZA in toddlers, children and young adults treated for up to six and a half years

CAMBRIDGE, Mass., May 18, 2020 (GLOBE NEWSWIRE) -- Biogen Inc. (Nasdaq: BIIB) today announced additional data from the SPINRAZA (nusinersen) clinical development program that further demonstrate the sustained efficacy and longer-term safety of SPINRAZA in a broad range of patients with spinal muscular atrophy (SMA). These new data were selected for presentation at the 72nd American Academy of Neurology (AAN) annual meeting and will be available online via the 2020 AAN Science Highlights virtual platform.

“As the first approved treatment for SMA, SPINRAZA offers a significant data set that allows us to uniquely assess the safety and durability of repeated doses over time in individuals across age groups and varying disease severity,” said Alfred Sandrock, Jr., M.D., Ph.D., Executive Vice President, Research and Development at Biogen. “New data show that continuous treatment with SPINRAZA for up to six and a half years improved or stabilized motor function and disease activity in a broad spectrum of patients with SMA. These results are in stark contrast to the expected natural history of the disease. Further, in a progressive disease like SMA, stabilization is an important measure of treatment success, allowing patients to retain motor function that may otherwise be lost.”

New Data Reinforce Sustained Efficacy and Longer-Term Safety of SPINRAZA Across Age Groups and SMA Types
The SHINE open-label extension study (NCT02594124) has enrolled 292 patients (infants through teenagers) from five previous SPINRAZA clinical studies, including ENDEAR. New findings from the SHINE study show treatment with SPINRAZA resulted in motor function improvement or disease stabilization in toddlers, children and young adults who were treated continuously, some for up to six and a half years.

Key highlights include:

- Patients with infantile-onset SMA included in the ENDEAR-SHINE study (n=105) and who had earlier initiation of SPINRAZA treatment experienced the greatest benefit, and those with later initiation showed evidence of motor function stabilization or improvement.
- A separate analysis evaluated a cohort of seven young adults (Type 2 or 3) who began treatment with SPINRAZA as teenagers (aged 13 to nearly 16 years old) and have since been treated for up to six and a half years (range of 5.3 to 6.8 years). Most of these patients demonstrated generally stable or improved motor function throughout the follow-up period as assessed by the Hammersmith Functional Motor Scale Expanded (HFMSE), Revised Upper Limb Module and Upper Limb Module (RULM/ULM) and Six-Minute Walk Test (6MWT).
  - Results also measured the impact on participants’ caregivers via the Assessment of Caregiver Experience with Neuromuscular Disease (ACEND), with the majority reporting stable or decreased impact over the same period. ACEND is an outcomes instrument specifically designed to assess the caregiver impact experienced by caregivers raising children affected by neuromuscular disease, including physical, emotional and financial domains.[1]
- The durability of SPINRAZA was also demonstrated in individuals with later-onset SMA (n=126), as HFMSE and RULM scores were stable.
- In all SHINE presentations, the safety profile of SPINRAZA was consistent with previously reported findings.

AAN data presentations highlighted in this release include:

- Nusinersen in Infantile-onset Spinal Muscular Atrophy: Results From Longer-term Treatment From the Open-label SHINE Extension Study
- Longer-term Experience With Nusinersen in Young Adults With Spinal Muscular Atrophy: Results From the CS2/CS12 and SHINE Studies
- Longer-term Treatment With Nusinersen: Results in Later-onset Spinal Muscular Atrophy From the SHINE Study
- Safety Profile of Nusinersen in Presymptomatic and Infantile-Onset Spinal Muscular Atrophy (SMA): Interim Results From the NURTURE and ENDEAR-SHINE Studies

About SPINRAZA® (nusinersen)[2-4]
SPINRAZA is the first therapy approved to treat infants, children and adults with spinal muscular atrophy (SMA) and is approved in more than 50 countries. As of March 31, 2020, more than 10,000 individuals have been treated with SPINRAZA. It is the only SMA treatment to combine unsurpassed real-world experience with a robust level of clinical evidence across a broad spectrum of patient populations.

SMA is a rare, genetic, neuromuscular disease that is characterized by a loss of motor neurons in the spinal cord and lower brain stem that can result in severe, progressive muscle atrophy and weakness. Approximately one in 10,000 live births have a diagnosis of SMA, and people of all ages are impacted by the disease. It is a leading genetic cause of infant mortality.

SPINRAZA, a foundation of care in SMA, is an antisense oligonucleotide (ASO), developed using Ionis Pharmaceuticals’ proprietary technology that is designed to target a root cause of SMA by increasing the amount of full-length survival motor neuron (SMN) protein, which is critical to maintaining motor neurons. It is administered by intrathecal injection into the fluid surrounding the spinal cord where motor neurons reside to deliver the treatment where

the disease starts.

SPINRAZA currently maintains a robust clinical data set in SMA based on data from approximately 300 patients across a broad range of SMA populations demonstrating a favorable benefit:risk profile. SPINRAZA was evaluated in two randomized, double-blind, sham-controlled studies of infantile and later-onset SMA (ENDEAR and CHERISH, respectively) and supported by open-label studies that include pre-symptomatic infants (NURTURE), individuals with later-onset SMA (CS2/CS12) and an extension study of individuals who previously participated in the clinical development program (SHINE). The most common adverse events observed were respiratory infection, fever, constipation, headache, vomiting and back pain. Hypersensitivity, meningitis and hydrocephalus have been observed in the post-marketing setting. Renal toxicity and coagulation abnormalities, including acute severe low platelet counts, have been observed after administration of some ASOs. Laboratory tests can monitor for these signs.

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 that moved SPINRAZA from its first dose in humans in 2011 to its first regulatory approval in five years.

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. Today Biogen has the leading portfolio of medicines to treat multiple sclerosis, has introduced the first approved treatment for spinal muscular atrophy, commercializes biosimilars of advanced biologics and is focused on advancing research programs in multiple sclerosis and neuroimmunology, Alzheimer's disease and dementia, neuromuscular disorders, movement disorders, ophthalmology, immunology, neurocognitive disorders, acute neurology and pain.

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 identification and treatment of SMA; clinical development programs, clinical trials, and data readouts and presentations; and the potential benefits and results from early treatment of SMA. 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. 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 the occurrence of adverse safety events; risks of unexpected costs, delays or 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; regulatory authorities may require additional information or further studies; failure to obtain regulatory approvals in other jurisdictions; product liability claims; third party collaboration risks; and the direct and indirect impacts of the ongoing COVID-19 pandemic on our business, results of operations and financial condition. 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 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.

References:

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2. Based on Commercial Patients, Early Access Patients, and Clinical Trial Participants as of March 31, 2020.
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4. 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.

MEDIA CONTACT:
David Caouette
+ 1 617 679 4945
public.affairs@biogen.com

INVESTOR CONTACT:
Joe Mara
+1 781 464 2442
IR@biogen.com