New Data Presented at Cure SMA Reveal Residual Unmet Needs in Young SMA Patients Treated With Gene Therapy and Suggest Further Potential of Using SPINRAZA® (nusinersen)

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- *RESPOND study is actively enrolling, with baseline characteristics reporting infants and toddlers have residual unmet medical needs in multiple areas after gene therapy; SPINRAZA treatment following gene therapy was well-tolerated*
- *Final safety data from DEVOTE Part A support the continued development of an investigational, higher dose of nusinersen; additional study phases are actively enrolling*
- *Biogen's continued R&D investments, including the ongoing DEVOTE, RESPOND and ASCEND studies, aim to assess options and inform therapy decisions for the SMA community*

CAMBRIDGE, Mass., June 15, 2022 (GLOBE NEWSWIRE) -- Biogen Inc. (Nasdaq: BIIB) will present new data from clinical studies aimed at assessing remaining unmet needs for people living with spinal muscular atrophy (SMA) and evaluating the potential impact of SPINRAZA® (nusinersen) in different patient populations at the SMA Research & Clinical Care Meeting hosted by Cure SMA this week in Anaheim, Calif. Biogen is a presenting sponsor of Cure SMA's 2022 Annual SMA Conference, the world's largest meeting dedicated to SMA research and care.

"The data we are presenting at this year's Cure SMA conference – including the latest updates from the RESPOND and DEVOTE studies – reinforce Biogen's commitment to evaluating the potential of SPINRAZA to further improve clinical outcomes for individuals with SMA," said Maha Radhakrishnan, M.D., Chief Medical Officer at Biogen. "There are key unmet needs within the SMA community and we are committed to addressing these through our ongoing research that includes active enrollment in three global clinical studies."

SMA Research Updates
Growing enrollment in the RESPOND study indicate there are residual unmet clinical needs in infants and toddlers with SMA following treatment with Zolgensma® (onasemnogene abeparvovec). The Phase 4 study is evaluating the clinical benefit and safety of SPINRAZA in infants and toddlers with SMA who have unmet needs following treatment with the gene therapy.

Since initial findings from nine patients were shared in March 2022, baseline and safety data from 16 patients enrolled in RESPOND (as of November 2021) are being presented. All enrolled study participants reported suboptimal clinical status across a variety of measures at baseline, with 13 of 16 showing this in multiple areas, including motor and respiratory functions and swallowing/feeding ability. After beginning SPINRAZA treatment, initial safety findings (median duration of 132.5 days) show three participants experienced a serious adverse event (AE) during the study period; none of these events were considered related to SPINRAZA treatment. The RESPOND study (NCT04488133) is currently enrolling participants at 20 sites worldwide; more information about the eligibility criteria is available at clinicaltrials.gov.

Biogen will also share final data from Part A of the ongoing, three-part DEVOTE study evaluating the safety and tolerability of investigational, higher doses of nusinersen.* Results from Part A, an open-label safety evaluation period in children and teens with later-onset SMA, suggest that a higher dosing regimen (28 mg) of nusinersen leads to higher levels of the drug in the cerebrospinal fluid and is generally well-tolerated, with most AEs reported considered to be mild in severity. The most common AEs reported were headache and procedural pain. Two serious AEs (fall, femur fracture) were reported in one participant during the study period. No AEs were considered related to nusinersen and some were related to treatment administration. The totality of Part A data supports further development of a higher dose of nusinersen.

Currently, Part B and Part C of DEVOTE evaluating an investigational, higher dose of nusinersen are enrolling at 52 sites worldwide. Information on the DEVOTE trial (NCT04089566) is available at clinicaltrials.gov.

Featured SPINRAZA Data Presentations Include:

- Results From the End of Part A of the Ongoing 3-Part DEVOTE Study to Explore Higher Doses of Nusinersen in SMA – *Friday, June 17, 2022 at 9:40 a.m. PT*
- Baseline Characteristics and Initial Safety Results in RESPOND: A Phase 4 Study of Nusinersen in Children With SMA Who Received Onasemnogene Abeparvovec – *Friday, June 17, 2022 at 10 a.m. PT*

**Nusinersen is currently commercialized under the brand name SPINRAZA® and the U.S. Food and Drug Administration-approved dose is 12 mg. As a foundation of care in SMA, more than 13,000 individuals have been treated with SPINRAZA worldwide.*[1]

About SPINRAZA® (nusinersen)
The SPINRAZA clinical development program encompasses 10 clinical studies, which have included more than 300 individuals across a broad spectrum of patient populations,[2] including two randomized controlled studies (ENDEAR and CHERISH). The ongoing SHINE and NURTURE open-label extension studies are evaluating the long-term impact of SPINRAZA. The most common adverse events observed in clinical studies were respiratory infection, fever, constipation, headache, vomiting and back pain. Laboratory tests can monitor for renal toxicity and coagulation abnormalities, including acute severe low platelet counts, which have been observed after administration of some ASOs.

Biogen licensed the global rights to develop, manufacture and commercialize SPINRAZA from Ionis Pharmaceuticals, Inc. (Nasdaq: IONS), the leader in antisense therapeutics. Please click here for Important Safety Information and full Prescribing Information for SPINRAZA in the U.S., or visit your respective country's product website.

About Spinal Muscular Atrophy (SMA)
SMA is a rare, genetic, neuromuscular disease that affects individuals of all ages. It is characterized by a loss of motor neurons in the spinal cord and

lower brain stem, resulting in progressive muscle atrophy and weakness.[3] SMA is caused by a deficiency in the production of survival motor neuron (SMN) protein due to a damaged or missing *SMN1* gene, with a spectrum of disease severity.[3] Some individuals with SMA may never sit; some sit but never walk; and some walk but may lose that ability over time.[4] In the absence of treatment, children with the most severe form of SMA would not be expected to reach their second birthday.[3]

SMA impacts approximately 1 in 10,000 live births,[5-8] is a leading cause of genetic death among infants[9] and causes a range of disability in teenagers and adults.[4]

About Biogen
As pioneers in neuroscience, Biogen discovers, develops, and delivers worldwide innovative therapies for people living with serious neurological 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, Sir Kenneth Murray, and Nobel Prize winners Walter Gilbert and Phillip Sharp. Today, Biogen has a leading portfolio of medicines to treat multiple sclerosis, has introduced the first approved treatment for spinal muscular atrophy, and developed the first and only approved treatment to address a defining pathology of Alzheimer's disease. Biogen is also commercializing biosimilars and focusing on advancing one of the industry's most diversified pipelines in neuroscience that will transform the standard of care for patients in several areas of high unmet need.

In 2020, Biogen launched a bold 20-year, $250 million initiative to address the deeply interrelated issues of climate, health, and equity. Healthy Climate, Healthy Lives™ aims to eliminate fossil fuels across the company's operations, build collaborations with renowned institutions to advance the science to improve human health outcomes, and support underserved communities.

We routinely post information that may be important to investors on our website at www.biogen.com. 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 nusinersen; the results of certain real-world data; our research and development program for the identification and treatment of SMA; clinical development programs, clinical trials and data readouts and presentations; the potential benefits and results from treatment of SMA; and risks and uncertainties associated with drug development and commercialization. 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 risks relating to the occurrence of adverse safety events and/or unexpected concerns that may arise from additional data or analysis, including from the DEVOTE, RESPOND and ASCEND studies; the risk that we may not fully enroll our clinical trials, or enrollment will take longer than expected; 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; regulatory authorities may require additional information or further studies; 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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