Biogen Exercises Option with Ionis to Develop and Commercialize Investigational ASO for SMA

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- BIIB115 is a preclinical investigational antisense oligonucleotide (ASO) that may have the potential for extended dosing intervals

- Biogen paid Ionis a $60 million one-time upfront payment

CAMBRIDGE, Mass. and CARLSBAD, Calif., Jan. 4, 2022 /PRNewswire/ -- Biogen Inc. (Nasdaq: BIIB) and Ionis Pharmaceuticals, Inc. (Nasdaq: IONS) today announced that Biogen exercised its option to obtain from Ionis a worldwide, exclusive, royalty-bearing license to develop and commercialize BIIB115/ION306. The companies have a broad strategic collaboration to develop novel therapies to treat neurological disorders. BIIB115 is an investigational antisense oligonucleotide (ASO) in development for spinal muscular atrophy (SMA) that may have the potential to help address additional unmet needs of patients as well as to be administered at extended dosing intervals. Biogen plans to advance BIIB115 to clinical trials to investigate safety, tolerability, pharmacokinetics, and efficacy.

"Combining Biogen's expertise in neurology with Ionis' leadership in antisense technology has led to SPINRAZA® (nusinersen) being a foundation of care in SMA," said Toby Ferguson, M.D., Ph.D., Vice President and Head of the Neuromuscular Development Unit at Biogen. "But unmet needs still remain for people impacted by SMA. We are excited to continue to pursue innovative treatments, such as BIIB115, that may have the potential to make a meaningful impact for patients in the SMA community."

SMA is characterized by loss of motor neurons in the spinal cord and lower brain stem, resulting in severe and progressive muscular atrophy. People with SMA do not produce enough survival motor neuron (SMN) protein, which is critical for the maintenance of motor neurons. BIIB115 is designed to target a root cause of SMA by increasing the production of functional SMN protein.

"SPINRAZA has transformed the standard of care for SMA, allowing patients to reach milestones that may have been previously unattainable and providing hope to families. BIIB115/ION306 represents another example of our productive collaboration with Biogen to discover and develop medicines that have potential to significantly benefit patients suffering from neurological diseases," said C. Frank Bennett, Ph.D., Executive Vice President, Chief Scientific Officer and Franchise Leader for Neurological Programs at Ionis.

As a part of the option exercise, Biogen made a one-time $60 million payment to Ionis in the fourth quarter of 2021. Future payments may include potential post-licensing development, regulatory and commercial milestone payments and royalties on annual worldwide net sales. Biogen will be solely responsible for the costs and expenses related to the development, manufacturing and potential future commercialization of BIIB115 following the option exercise.

About SPINRAZA® (nusinersen) injection, for intrathecal use 12 mg/5 mL
The SPINRAZA clinical development program encompasses 10 clinical studies, which have included more than 300 individuals across a broad spectrum of patient populations[1], 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.[2] 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.[2] Some individuals with SMA may never sit; some sit but never walk; and some walk but may lose that ability over time.[3] In the absence of treatment, children with the most severe form of SMA would usually not be expected to reach their second birthday.[2]

SMA impacts approximately 1 in 10,000 live births,[4-7] is a leading cause of genetic death among infants[8] and causes a range of disability in teenagers and adults.[3]

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 the leading portfolio of medicines to treat multiple sclerosis, has introduced the first approved treatment for spinal muscular atrophy, and is providing the first and only approved treatment to address a defining pathology of Alzheimer's disease. Biogen is also commercializing biosimilars and focusing on advancing the industry's most diversified pipeline 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.

About Ionis Pharmaceuticals, Inc.

For more than 30 years, Ionis has been the leader in RNA-targeted therapy, pioneering new markets and changing standards of care with its novel antisense technology. Ionis currently has three marketed medicines and a premier late-stage pipeline highlighted by industry-leading neurological and cardiometabolic franchises. Our scientific innovation began and continues with the knowledge that sick people depend on us, which fuels our vision of becoming one of the most successful biotechnology companies.

To learn more about Ionis visit www.ionispharma.com and follow us on Twitter @ionispharma.

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, including statements about potential clinical effects of BIIB115; the potential benefits, safety and efficacy of BIIB115; the clinical development program for BIIB115; the identification and treatment of SMA; our research and development program for the treatment of SMA; the potential of our commercial business and pipeline programs, including BIIB115; and risks and uncertainties associated with drug development and commercialization. These forward-looking statements may be accompanied by words such as "aim," "anticipate," "believe," "could," "estimate," "expect," "forecast," "intend," "may," "plan," "potential," "possible," "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 the development and potential commercialization of BIIB115; the risk that we may not fully enroll our clinical trials or enrollment will take longer than expected; unexpected concerns may arise from additional data, analysis or results obtained during our clinical trials; regulatory authorities may require additional information or further studies, or may fail or refuse to approve or may delay approval of our drug candidates, including BIIB115; the occurrence of adverse safety events; the risks of unexpected hurdles, 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; product liability claims; 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.

Ionis' Forward-looking Statements
This press release includes forward-looking statements regarding Ionis' business and the therapeutic and commercial potential of Ionis' technologies, BIIB115/ION306, SPINRAZA® (nusinersen) and other products in development. Any statement describing Ionis' goals, expectations, financial or other projections, intentions or beliefs is a forward-looking statement and should be considered an at-risk statement. Such statements are subject to certain risks and uncertainties, including those related to the impact COVID-19 could have on our business, and including but not limited to those related to our commercial products and the medicines in our pipeline, and particularly those inherent in the process of discovering, developing and commercializing medicines that are safe and effective for use as human therapeutics, and in the endeavor of building a business around such medicines. Ionis' forward-looking statements also involve assumptions that, if they never materialize or prove correct, could cause its results to differ materially from those expressed or implied by such forward-looking statements.

Although Ionis' forward-looking statements reflect the good faith judgment of its management, these statements are based only on facts and factors currently known by Ionis. As a result, you are cautioned not to rely on these forward-looking statements. These and other risks concerning Ionis' programs are described in additional detail in Ionis' annual report on Form 10-K for the year ended December 31, 2020, and the most recent Form 10-Q quarterly filing, which are on file with the SEC. Copies of these and other documents are available from the Company.

In this press release, unless the context requires otherwise, "Ionis," "Company," "we," "our," and "us" refers to Ionis Pharmaceuticals and its subsidiaries.

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