Ionis' neurological franchise marks year of achievement

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In the news release, Ionis' neurological franchise marks year of achievement, issued 07-Jan-2020 by Ionis Pharmaceuticals, Inc. over PR Newswire, we are advised by the company that the last bullet in the bulleted list should read "administered to more than 9,300 patients in 40 countries" rather than "administered to more than 45,000 patients in 40 countries" as originally issued inadvertently. The complete, corrected release follows:

Ionis' neurological franchise marks year of achievement

- Potential breakthrough therapies for Huntington's, Alzheimer's, ALS and Parkinson's among numerous programs advancing in the clinic

- Neurological R&D team received several prestigious honors

- Advances in neurological research highlighted in leading peer-reviewed journals

CARLSBAD, Calif., Jan. 7, 2020 /PRNewswire/ -- The neurological franchise of Ionis Pharmaceuticals, Inc. (NASDAQ: IONS), the leader in RNA-targeted therapeutics, continued to shape the scientific and therapeutic landscape of the global biotechnology industry in 2019. By any metric, it was a remarkable year; from advancing potential breakthrough therapies in the clinic, to garnering prestigious awards and recognition in peer-reviewed journals, to generating more than $300 million in revenues. In all, Ionis' neurological franchise consists of two commercial medicines, eight drugs in late-stage development, six in early development and more than 30 in late discovery, targeting a broad range of conditions from rare diseases to those affecting millions of patients.

Through its partnerships, Ionis' neurological franchise achieved important milestones during the year, including:

- Enrolling the first patient in a pivotal study of IONIS-HTT_{Rx} (RG6042) for patients with Huntington's Disease (HD) by Roche
- Advancing tofersen, a drug designed to reduce production of superoxide dismutase 1 (SOD1) in patients with amyotrophic lateral sclerosis (ALS), to a phase 3 pivotal trial by Biogen
- Licensing of IONIS-$MAPT_{Rx}$ (BIIB080), a program currently in a Phase 1/2 clinical study in patients with mild Alzheimer's disease (AD) by Biogen
- Advancing an ongoing study by Biogen of IONIS-$C9_{Rx}$ (BIIB078) targeting the C9orf72 mutation in ALS
- Initiation of a clinical study of ION859 (BIIB094) as the first antisense drug to potentially treat people with Parkinson's disease
- Advancing six medicines that Ionis expects to enter the clinic in 2020, including a treatment for Angelman Syndrome.
- Advancement of 4 undisclosed neurological disease targets into drug discovery under Ionis' collaboration with Biogen
- Completion of a Phase 1 study of Ionis' TTR LICA drug and initiation of a Phase 3 study in patients with TTR amyloidosis with polyneuropathy
- Continued commercial success of SPINRAZA®. In first nine months of 2019, sales of the blockbuster increased nearly 25 percent from the same period in 2018. Marketed by Biogen, SPINRAZA is the first and only approved medicine for treatment of all forms of spinal muscular atrophy (SMA) and is a global foundation of care, administered to more than 9,300 patients in 40 countries

Promising new Ionis-owned therapies advancing into the clinic include ION283 for Lafora disease, ION373 for a form of leukodystrophy and ION716 for prion diseases.

"It's been a privilege to see Ionis' neurology program mature from its early days as a nascent project into the industry's leading neurological disease pipeline, one that is having a meaningful impact on patients," said C. Frank Bennett, Ph.D., Ionis' chief scientific officer and franchise leader for neurological programs. "What I am perhaps most proud of is that we're harnessing the power and efficiency of antisense technology to tackle some of the most severe diseases, bringing hope to patients who previously had none."

Dr. Bennett, a founding member of Ionis and a 2019 Breakthrough Prize laureate for his contributions to the discovery and development of SPINRAZA, was included on the 2019 "Highly Cited Researchers" list of the world's most influential researchers, for being among the top 1 percent of most-cited authors in their field.

Ionis' neurological franchise team also received the inaugural 2019 Sean M. Healey International Prize for Innovation in ALS. The global prize rewards excellence in research for exceptional discoveries leading to a transformative advance in therapy development in ALS. Other recipients of this year's prize included teams led by Timothy Miller, M.D., Ph.D., Don Cleveland, Ph.D., Richard C. Smith, M.D., Merit Cudkowicz, M.D., and Toby Ferguson, M.D.

Holly Kordasiewicz, Ph.D., vice president of Neurology at Ionis, was honored with the 2019 Oligonucleotide Therapeutics Society's Young Investigator Award. The award recognizes the outstanding achievements and contributions by a scientist in the field of oligonucleotide therapeutics who has recently received his or her doctoral degree. Dr. Kordasiewicz was acknowledged for her dedication to discovering transformative antisense medicines for devastating neurodegenerative diseases, including spinal muscular atrophy, Huntington's disease and amyotrophic lateral sclerosis.

Groundbreaking neurological research being done at Ionis was also highlighted in the scientific literature. In 2019, members of Ionis' neurological franchise team contributed 16 articles to leading peer-reviewed journals, including Nature Medicine, Nature Neuroscience, the Journal of Clinical Investigation and The New England Journal of Medicine (NEJM). "Targeting Huntingtin Expression in Patients with Huntington's Diseases" was among NEJM's Notable Articles of 2019, a collection of "practice-changing articles" selected by NEJM editors that are "improving patient care." Ionis authors of the paper were Drs. Bennett and Kordasiewicz, Eric E. Swayze, Ph.D., senior vice president, Research, Daniel A. Norris, Ph.D., director, Preclinical

Development, Tiffany Baumann, executive director, Regulatory Affairs, Anne V. Smith, Ph.D., executive director, Clinical Development and Roger M. Lane, M.D., vice president, Clinical Development.

About Ionis Pharmaceuticals, Inc.

As the leader in RNA-targeted drug discovery and development, Ionis has created an efficient, broadly applicable, drug discovery platform called antisense technology that can treat diseases where no other therapeutic approaches have proven effective. Our drug discovery platform has served as a springboard for actionable promise and realized hope for patients with unmet needs. We created the first and only approved treatment for both children and adults with spinal muscular atrophy as well as the world's first RNA-targeted therapeutic approved for the treatment of polyneuropathy in adults with hereditary transthyretin amyloidosis. Our sights are set on all the patients we have yet to reach with a pipeline of more than 40 novel medicines designed to treat a broad range of disease, including cardiovascular, neurological, infectious and pulmonary diseases and many more.

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

Ionis' Forward-looking Statement

This press release includes forward-looking statements regarding Ionis' business, financial guidance and the therapeutic and commercial potential of SPINRAZA (nusinersen) and Ionis' technologies and 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, particularly those inherent in the process of discovering, developing and commercializing drugs that are safe and effective for use as human therapeutics, and in the endeavor of building a business around such drugs. 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, 2018, 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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