# Ionis treatment for Alexander disease receives orphan drug designation from U.S. FDA

September 30, 2020

**- U.S. orphan drug status follows similar designation by the European Medicines Agency**

CARLSBAD, Calif., Sept. 30, 2020 /PRNewswire/ -- Ionis Pharmaceuticals, Inc. (NASDAQ: IONS), the leader in antisense therapeutics, announced today that the U.S. Food and Drug Administration (FDA) has granted orphan drug designation to ION373 for the treatment of people with Alexander disease, a severe, progressive and debilitating rare neurodegenerative disease that can result in death. Infants and young children who develop the condition rarely survive beyond their teenage years or young adulthood. Alexander disease has been estimated to occur in about one in one million births. Orphan drug designation is granted by the FDA to drugs and biologics intended for treatment, prevention or diagnosis of a rare disease or condition that affects fewer than 200,000 people in the U.S. at the time of designation.

Alexander disease is caused by a mutation in a protein resulting in overproduction of glial fibrillary acidic protein (GFAP) in the brain. ION373 is an Ionis-owned investigational antisense medicine designed to stop the mutated gene from producing excess GFAP.

Under the FDA's Orphan Drug Act, orphan drug status provides incentives, including waiver of certain administrative fees, grants and tax credits for clinical trials, and seven years of market exclusivity following drug approval. Earlier this year, the European Medicines Agency (EMA) granted orphan drug designation to ION373. EMA provides regulatory, financial and market incentives to develop therapies for life threatening or chronically debilitating conditions affecting not more than five in 10,000 people in the European Union (EU) and for which there is no satisfactory method of diagnosis, prevention or treatment.

"Receiving FDA orphan drug status for ION373 reflects the urgent need for a novel medicine to treat Alexander disease. We look forward to working closely with regulators, clinical investigators, Alexander disease patients and their families to advance this important medicine and make it available to those who need it," said Frank Bennett, Ph.D., Ionis' chief scientific officer and franchise leader for neurological programs.

ION373 is one of several Ionis-owned investigational medicines designed to treat neurological diseases. Others include ION716 (Prion disease), ION283 (Lafora disease) and ION363 (amyotrophic lateral sclerosis or ALS).

**About Alexander disease**

Alexander disease (AxD) is a rare neurological condition characterized as a leukodystrophy, or a disease affecting the myelin sheath (the fatty insulation that protects a nerve fiber and supports signal conduction). Two major types of AxD have been defined. Type I onset typically occurs before 4 years of age and patients can experience head enlargement, seizures, limb stiffness, delayed or declining cognition, and lack of growth. Type II onset typically occurs after the age of 4 and symptoms can include difficulty speaking, swallowing, and making coordinated movements. AxD is most often fatal. There are treatments that can relieve symptoms, but there is no disease modifying therapy yet available to patients.

**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 all patients, 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 potentially treat a broad range of disease, including neurological, cardio-renal, metabolic, infectious, and pulmonary diseases.

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 ION373 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, 2019, 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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Ionis Pharmaceuticals Investor Contact: D. Wade Walke, Ph.D., Vice President, Investor Relations, 760-603-2741; Ionis Pharmaceuticals Media Contact: Roslyn Patterson, Vice President, Marketing and Communications, 760-603-4679