Ionis treatment for Alexander disease granted orphan drug status from EMA

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CARLSBAD, Calif., Feb. 11, 2020 /PRNewswire/ -- Ionis Pharmaceuticals, Inc. (NASDAQ: IONS), the leader in RNA-targeted therapeutics, announced today that the European Medicines Agency (EMA) has granted orphan drug designation to ION373 for the treatment of people with Alexander disease, a severe, progressive and debilitating condition that can eventually result in death due to loss of control over autonomic functions like breathing. Infants and young children who develop the condition rarely survive beyond their teenage years or young adulthood.

Orphan drug designation by the EMA provides regulatory and financial 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. Upon approval, drugs that have been granted orphan designation by the EMA receive market exclusivity for 10 years in the EU.

Alexander disease affects approximately 500 people in the EU. It is caused by a mutation in a protein that creates overproduction of glial fibrillary acidic protein (GFAP) in the brain. ION373 is designed to stop the mutated gene from producing excess GFAP.

"We look forward to working closely with European regulators, clinical investigators, Alexander disease patients and their families to advance this important medicine and make it available to those who need it," said Brett P. Monia, Ph.D., Ionis' chief executive officer. "Receiving orphan drug status reflects the urgent need for ION373, which is among a number of wholly-owned, novel drugs in the Ionis pipeline that we are prioritizing."

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 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, cardiovascular, 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, 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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