Ionis Pharmaceuticals Receives Orphan Drug Designation from the US FDA for IONIS-HTT Rx for the Treatment of Patients with Huntington's Disease

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First therapy designed to directly target the cause of disease

CARLSBAD, Calif., Jan. 5, 2016 /PRNewswire/ -- Ionis Pharmaceuticals, Inc. (NASDAQ: IONS) today announced that the U.S. Food and Drug Administration has granted Orphan Drug Designation to IONIS-HTT_{Rx} for the treatment of patients with Huntington's disease (HD). IONIS-HTT_{Rx} is the first therapy to enter clinical development that is designed to directly target the cause of the disease by reducing the production of the protein responsible for HD. IONIS-HTT_{Rx} has also been granted orphan drug designation by the European Medicines Agency for the treatment of patients with HD.

"HD is a rare genetic neurological disease in which patients experience deterioration of both mental abilities and physical control. Although the toxic protein produced from the huntingtin (HTT) gene in HD patients has been a target of interest for many years, IONIS-HTT_{Rx} is the first therapy to enter clinical development that is designed to treat the underlying cause of this fatal disease. The granting of Orphan Drug Designation in both the US and Europe highlights the significant need for a drug that could transform the treatment of HD," said C. Frank Bennett, Ph.D., senior vice president of research at Ionis Pharmaceuticals.

The Orphan Drug Act provides for economic incentives to encourage the development of drugs for diseases affecting fewer than 200,000 people in the United States. Orphan drug designation entitles seven years of market exclusivity in the United States if market approval is granted for IONIS-HTT_{Rx} for the treatment of patients with HD. Additional incentives include tax credits related to clinical trial expenses, an exemption from the FDA-user fee, and FDA assistance in clinical trial design.

ABOUT IONIS and ROCHE

Roche and Ionis are collaborating to develop antisense drugs to treat HD. The alliance combines Ionis' antisense expertise with Roche's scientific knowledge in developing neurodegenerative therapeutics. To date, Ionis has earned $52 million in upfront and milestone payments from its relationship with Roche and is eligible to earn additional milestone payments as the drug progresses in development, as well as royalties on sales of IONIS-HTT_{Rx} if it is commercialized. Roche has the option to license IONIS-HTT_{Rx} from Ionis through the completion of the Phase 1/2a study. Prior to option exercise, Ionis is responsible for the discovery and development of IONIS-HTT_{Rx}. If Roche exercises its option, it will assume responsibility for global development, regulatory and commercialization activities for the drug.

CHDI Foundation, Inc. provided financial and scientific support to Ionis' HD drug discovery program through a development collaboration with Ionis. Over time, CHDI will be reimbursed for its support of Ionis' program out of milestone payments received by Ionis.

ABOUT IONIS-HTT_{Rx} and Huntington's Disease

IONIS-HTT_{Rx} is a Gen. 2.0+ antisense drug in development for the treatment of Huntington's disease. IONIS-HTT_{Rx} is designed to reduce the production of all forms of the huntingtin (HTT) protein, which is the protein responsible for HD. As such, IONIS-HTT_{Rx} offers a unique approach to treat all patients with HD.

HD is a rare genetic, progressive neurological disease resulting in deterioration in mental abilities and physical control. HD is referred to as a triplet repeat disorder, and is one of a large family of genetic diseases in which certain gene sequences are mistakenly repeated. In HD, the gene that encodes for the HTT protein contains a trinucleotide sequence that is repeated in the gene more than 36 times. The resulting HTT protein is toxic and gradually damages neurons in the brain. Symptoms of HD usually appear between the ages of 30 to 50 years, and continually worsen over a 10 to 25 year period. Ultimately, the weakened individual succumbs to pneumonia, heart failure or other complications. Presently, there is no effective disease modifying treatment, and current approaches only focus on managing the severity of some disease symptoms.

ABOUT IONIS PHARMACEUTICALS, INC.

Ionis Pharmaceuticals is the leading company in RNA-targeted drug discovery and development focused on developing drugs for patients who have the highest unmet medical needs, such as those patients with severe and rare diseases. Using its proprietary antisense technology, Ionis has created a large pipeline of first-in-class or best-in-class drugs, with over a dozen drugs in mid- to late-stage development. Drugs currently in Phase 3 development include volanesorsen, a drug Ionis is developing and plans to commercialize through its wholly owned subsidiary, Akcea Therapeutics, to treat patients with familial chylomicronemia syndrome and familial partial lipodystrophy; IONIS-TTR_{Rx}, a drug Ionis is developing with GSK to treat patients with all forms of TTR amyloidosis; and nusinersen, a drug Ionis is developing with Biogen to treat infants and children with spinal muscular atrophy. Ionis' patents provide strong and extensive protection for its drugs and technology. Additional information about Ionis is available at www.ionispharma.com.

IONIS PHARMACEUTICALS' FORWARD-LOOKING STATEMENT

This press release includes forward-looking statements regarding Ionis' alliance with Roche, the development, activity, therapeutic potential, commercial potential and safety of IONIS-HTT_{Rx}. 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, 2014, and its most recent quarterly report on Form 10-Q, which are on file with the SEC. Copies of these and other documents are available from the Company.

In December, 2015, the Company changed its name from Isis Pharmaceuticals, Inc. to Ionis Pharmaceuticals, Inc.

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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