Ionis treatment for Angelman syndrome receives orphan drug and rare pediatric disease designations from U.S. FDA

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CARLSBAD, Calif., June 13, 2022 /PRNewswire/ -- Ionis Pharmaceuticals, Inc. (Nasdaq: IONS), the leader in RNA-targeted therapeutics, announced today that the U.S. Food and Drug Administration (FDA) has granted orphan drug designation and rare pediatric disease designation to ION582, an investigational antisense medicine for the treatment of Angelman syndrome, a rare neurogenetic disorder caused by the loss of function of the maternally inherited Ubiquitin Protein Ligase E3A (UBE3A) gene. ION582 targets UBE3A.

Angelman syndrome, which affects an estimated one in 12,000 to 20,000 people globally,[1] presents early in life with profound and severe developmental delays in motor, language and cognitive functioning, seizures and ataxia. It is a neuro-developmental disorder that generally does not improve following symptom onset in early childhood, resulting in complete dependence on a caregiver. Currently, there are no disease-modifying treatments for Angelman syndrome.

"Receiving FDA orphan drug designation for ION582 reflects the important and urgent need for delivering an effective treatment to patients living with Angelman syndrome. Ionis is committed to working closely with regulators, clinical investigators, patients and caregivers to advance this novel treatment and make it available to those who need it," said C. Frank Bennett, Ph.D., executive vice president, chief scientific officer and franchise leader for neurological programs at Ionis.

Under the FDA's Orphan Drug Act, orphan drug status provides incentives, including tax credits, grants and waiver of certain administrative fees for clinical trials, and seven years of market exclusivity following drug approval. The FDA defines a rare pediatric disease as a serious or life-threatening disease that primarily affects individuals from birth to 18 years of age. Under the FDA's rare pediatric disease designation and voucher programs, if Ionis receives marketing approval for ION582 for Angelman syndrome, the company may qualify for a voucher that can be redeemed to receive a priority review of a subsequent marketing application for a different product.

ION582 is being evaluated in a Phase 1/2, open-label, dose-escalation clinical study in up to approximately 44 participants with Angelman syndrome. For more information on the HALOS Study (NCT05127226), visit clinicaltrials.gov.

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 cardiovascular and neurological franchises. Our scientific innovation began and continues with the knowledge that sick people depend on us, which fuels our vision of becoming a leading, fully integrated biotechnology company.

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 and the therapeutic and commercial potential of Ionis' technologies, ION582 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, 2021, 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.

[1] Mertz LG, Christensen R, Vogel I, Hertz JM, Nielsen KB, Gronskov K, Ostergaard JR. Angelman syndrome in Denmark. birth incidence, genetic findings, and age at diagnosis. Am J Med Genet A. 2013;161A:2197–203.

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