Zilganersen granted U.S. FDA Fast Track designation for people living with Alexander disease

October 1, 2024

- Zilganersen is the first investigational medicine in clinical development for adults and children living with Alexander disease, an ultra-rare neurological condition

CARLSBAD, Calif., Oct. 1, 2024 /PRNewswire/ -- Ionis Pharmaceuticals, Inc. (Nasdaq: IONS) announced today that the U.S. Food and Drug Administration (FDA) has granted zilganersen Fast Track designation for the treatment of children and adults with an ultra-rare, progressive and ultimately fatal neurological disorder known as Alexander disease (AxD). Topline data from the pivotal study of zilganersen is expected in the second half of 2025. The FDA grants investigational medicines Fast Track designation to facilitate the development and expedite the review of medicines that demonstrate the potential to treat serious conditions and fill an unmet medical need.

"With no approved treatments available for people living with AxD, receiving this Fast Track designation for zilganersen reflects the seriousness of this ultra-rare disease and the significant unmet need for treatment in this patient population," said Eugene Schneider, M.D., executive vice president and chief clinical development officer at Ionis. "Zilganersen was designed to address the underlying cause of disease and help improve the functioning of people living with AxD. We look forward to a data readout next year and working closely with the FDA to potentially bring forward the first approved AxD treatment."

The pivotal Phase 1-3 study of zilganersen in adults and children living with AxD completed enrollment earlier this year across 13 sites in eight countries. More information on the study (NCT04849741) is available at www.clinicaltrials.gov.

About Alexander Disease (AxD)
AxD is an ultra-rare, progressive and ultimately fatal type of leukodystrophy, which are a group of genetic disorders that affect the brain's white matter. AxD is estimated to occur in approximately one in one million to one in three million people worldwide and usually leads to death within 14 - 25 years after symptom onset. AxD can present throughout life as loss of independence and lack of ability to control muscles for swallowing, airway protection and purposeful movements, though the impact of AxD can vary depending on factors like age of onset. Diagnosing AxD is based on a combination of clinical presentation, brain magnetic resonance imaging (MRI) findings and genetic testing. AxD is caused by changes in the glial fibrillary acidic protein (GFAP) gene that lead to a build-up of abnormal proteins in cells, causing progressive damage to the nervous system. There are no medicines approved for people with AxD, and current treatments focus on managing their symptoms.

About Zilganersen (ION373)
Zilganersen is an investigational antisense oligonucleotide medicine being developed as a potential treatment for people with genetically confirmed Alexander disease (AxD). Zilganersen is designed to stop the excess glial fibrillary acidic protein (GFAP) production that accumulates because of disease-causing variants in the GFAP gene. In 2020, the U.S. Food and Drug Administration (FDA) granted zilganersen Orphan Drug designation and Rare Pediatric Disease designation. In addition, the European Medicines Agency (EMA) granted zilganersen Orphan Drug designation in 2019.

About Ionis Pharmaceuticals, Inc.
For three decades, Ionis has invented medicines that bring better futures to people with serious diseases. Ionis currently has five marketed medicines and a leading pipeline in neurology, cardiology, and other areas of high patient need. As the pioneer in RNA-targeted medicines, Ionis continues to drive innovation in RNA therapies in addition to advancing new approaches in gene editing. A deep understanding of disease biology and industry-leading technology propels our work, coupled with a passion and urgency to deliver life-changing advances for patients.

To learn more about Ionis, visit Ionis.com and follow us on X (Twitter) and LinkedIn.

Ionis Forward-looking Statements
This press release includes forward-looking statements regarding Ionis' business, and the therapeutic and commercial potential of Ionis' commercial medicines, zilganersen, additional medicines in development and technologies. 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 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. Except as required by law, we undertake no obligation to update any forward-looking statements for any reason. 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 Dec. 31, 2023, and most recent Form 10-Q, which are on file with the SEC. Copies of these and other documents are available at www.Ionis.com.

In this press release, unless the context requires otherwise, "Ionis," "Company," "we," "our" and "us" all refer to Ionis Pharmaceuticals and its subsidiaries.

Ionis Pharmaceuticals® is a registered trademark of Ionis Pharmaceuticals, Inc.

Zilganersen is an investigational medicine that has not been approved for the treatment of any disease by regulatory authorities.

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