Ionis announces FDA acceptance of New Drug Application for donidalorsen for prophylactic treatment of HAE

November 4, 2024

- *Donidalorsen will be a first-in-class RNA-targeted medicine for hereditary angioedema, assuming approval*
- *Donidalorsen PDUFA date set for August 21, 2025*
- *Donidalorsen has the potential to be Ionis' second independent commercial launch*

CARLSBAD, Calif., Nov. 4, 2024 /PRNewswire/ -- Ionis Pharmaceuticals, Inc. (Nasdaq: IONS) announced today that the U.S. Food and Drug Administration (FDA) has accepted for review the New Drug Application (NDA) for donidalorsen, an investigational RNA-targeted medicine for prophylaxis to prevent attacks of hereditary angioedema (HAE) in adult and pediatric patients 12 years of age and older. The FDA has set an action date of August 21, 2025 under the Prescription Drug User Fee Act (PDUFA). The FDA application was based on positive results with monthly and bi-monthly dosing in the pivotal Phase 3 OASIS-HAE and OASISplus (open label extension (OLE) and switch) studies, as well as the ongoing Phase 2 OLE study.

HAE is a rare and potentially life-threatening genetic condition that involves recurrent attacks of severe swelling (angioedema) in various parts of the body, including the hands, feet, genitals, stomach, face and/or throat. Donidalorsen is designed to reduce the production of prekallikrein (PKK), interrupting the pathway that leads to HAE attacks.

"Despite currently available treatments, many people living with HAE continue to experience painful and potentially life-threatening breakthrough attacks. Based on the totality of clinical evidence from the Phase 3 OASIS-HAE and OASISplus studies, as well as new three-year results from our Phase 2 OLE study, we believe that donidalorsen has the potential to advance the prophylactic treatment paradigm for people living with HAE," said Brett Monia, Ph.D., chief executive officer of Ionis. "With the FDA acceptance of our donidalorsen NDA, we are poised for our second independent launch next year, assuming approval, which will allow us to continue to deliver on our goal to bring a steady cadence of medicines to patients with serious diseases."

The FDA previously granted donidalorsen Orphan Drug Designation in 2023. Otsuka, which has exclusive rights to commercialize donidalorsen in Europe and Asia Pacific, is preparing to submit a Marketing Authorization Application (MAA) to the European Medicines Agency (EMA) this year.

Ionis recently presented new results from the Phase 3 and Phase 2 OLE studies at the 2024 American College of Allergy, Asthma & Immunology (ACAAI) Annual Scientific Meeting, which demonstrate that donidalorsen delivered significant and sustained reductions in HAE attacks, with overall sustained mean reduction in HAE attack rates of 96% from baseline maintained up to three years in the ongoing Phase 2 OLE study.

Across all three studies, donidalorsen was well-tolerated, with no serious treatment-emergent adverse events (TEAEs) related to donidalorsen. Most adverse events (AEs) were mild or moderate in severity, and injection site reactions were the most common AE.

The donidalorsen ACAAI e-poster presentations can be found on Ionis' website.

About Hereditary Angioedema (HAE)
HAE is a rare and potentially life-threatening genetic condition that involves recurrent attacks of severe swelling (angioedema) in various parts of the body, including the hands, feet, genitals, stomach, face and/or throat. HAE is estimated to affect more than 20,000 patients in the U.S. and Europe. In the U.S., doctors frequently use prophylactic treatment approaches to prevent and reduce the severity of HAE attacks in patients.

About Donidalorsen
Donidalorsen is an investigational RNA-targeted medicine designed to target prekallikrein (PKK), which plays an important role in activating inflammatory mediators associated with acute attacks of hereditary angioedema (HAE). By reducing the production of PKK, donidalorsen could be an effective prophylactic approach to preventing HAE attacks, if approved.

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

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 our commercial medicines, donidalorsen, 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 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 December 31, 2023, and most recent Form 10-Q, which are on file with the Securities and Exchange Commission. 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" all refer to Ionis Pharmaceuticals and its subsidiaries.

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