New positive donidalorsen data to be presented at AAAAI/WAO Joint Congress highlight sustained HAE attack rate reduction and disease control

February 20, 2025

– Data also show significant and clinically meaningful improvements in quality-of-life measures in new analyses from Phase 3 and Phase 2 clinical program –

–With a PDUFA date of August 21, 2025, donidalorsen positioned to be Ionis' second independent commercial launch –

CARLSBAD, Calif.--(BUSINESS WIRE)--Feb. 20, 2025-- Ionis Pharmaceuticals, Inc. (Nasdaq: IONS) today announced that it will present additional data from the pivotal Phase 3 OASIS and OASISplus studies, as well as three year data from the Phase 2 open-label extension (OLE) study of donidalorsen, the company's investigational RNA-targeted prophylactic medicine for hereditary angioedema (HAE). Results will be presented at the 2025 American Academy of Allergy, Asthma & Immunology (AAAAI) / World Allergy Organization (WAO) Joint Congress in San Diego, California.

The New Drug Application (NDA) for donidalorsen to prevent attacks of HAE in adult and pediatric patients 12 years of age and older is currently under review with the U.S. Food and Drug Administration (FDA), with a target action date of August 21, 2025.

"While there's been notable advancement in the HAE treatment landscape, there is still an urgent need for a medicine that effectively reduces attacks, is well tolerated and simple to administer. Across the breadth of presentations at the congress, we believe the totality of the clinical evidence underscores the potential of donidalorsen to be the prophylactic treatment of choice for people living with HAE," said Kenneth Newman, M.D., senior vice president, head of clinical development, Ionis. "In new analyses from our OASISplus prospective switch cohort, donidalorsen continued to demonstrate the ability to reduce the HAE attack rate burden and improve quality of life in patients previously on other prophylactic treatments, with the simplicity of monthly or every two-month self-administration via an autoinjector. As a first-in-class RNA-targeted medicine, we believe donidalorsen has the potential to advance the prophylactic treatment paradigm for HAE."

Ionis will have 11 presentations, as follows. All final posters are available on Ionis' website .

- **Efficacy and Safety of Donidalorsen In Adolescent Patients with Hereditary Angioedema: A Subanalysis of the Phase 3 OASIS-HAE Study**
  - Featured Poster Presentation: March 2, 3:30-5:00pm PT (Poster 890)
  - Presenting Author: Joshua Jacobs
- **Hereditary Angioedema Disease Control after Switching to Donidalorsen from Prior Long-Term Prophylaxis: Results from the OASISplus Open-Label Extension Study**
  - Poster Presentation: March 2, 2025, 9:45-10:45am PT (Poster 601)
  - Presenting Author: Marc Riedl
- **Improvements in Quality-of-Life in Patients with HAE Receiving Donidalorsen: Post Hoc Analysis from the OASIS-HAE Study**
  - Poster Presentation: March 2, 2025, 9:45-10:45am PT (Poster 648)
  - Presenting Author: Danny Cohn
- **Patient-Reported Injection-Site Pain and Treatment Satisfaction after Switching from Long-Term Prophylaxis to Donidalorsen for the Treatment of Hereditary Angioedema: Results from the OASISplus Study**
  - Poster Presentation: March 2, 2025, 9:45-10:45am PT (Poster 599)
  - Presenting Author: Marc Riedl
- **Psychometric Validation of Angioedema Quality-of-Life Questionnaire in Hereditary Angioedema: Results from the OASIS-HAE Study**
  - Poster Presentation: February 28, 2025, 2:45-3:45pm PT (Poster 216)
  - Presenting Author: Aaron Yarlas
- **Long-Term Analysis of the Phase 2 Open-Label Extension of Donidalorsen in Patients With Hereditary Angioedema**
  - Poster Presentation: March 2, 2025, 9:45-10:45am PT (Poster 654)
  - Presenting Author: Michael Manning
- **Correlation between Subjective and Objective Disease Control in Hereditary Angioedema: Association between the Angioedema Control Test and Attack Rate**
  - Poster Presentation: March 2, 2025, 9:45-10:45am PT (Poster 647)
  - Presenting Author: Danny Cohn

- **Long-Term Prophylaxis for Hereditary Angioedema: Real-World Experience in Selected US Allergy Clinics**
  - Poster Presentation: March 2, 2025, 9:45-10:45am PT (Poster 739)
  - Presenting Author: Huamin Henry Li
- **The Patient Experience of Hereditary Angioedema: Findings from a Racially Diverse Sample of Adult Patients**
  - Poster Presentation: February 28, 2025, 2:45-3:45pm PT (Poster 215)
  - Presenting Author: Aaron Yarlas
- **Patient Preferences for Attributes of Prophylactic Treatment in Hereditary Angioedema: A Discrete-Choice Experiment**
  - Poster Presentation: March 2, 2025, 9:45-10:45am PT (Poster 696)
  - Presenting Author: Kathleen Villa
- **Participant Reported Ease-of-Use with a Prefilled, Single-dose, Disposable Autoinjector for the Treatment of Hereditary Angioedema**
  - Poster Presentation: March 2, 2025, 9:45-10:45am PT (Poster 699)
  - Presenting Author: Hetal Khatri

Ionis previously reported positive results from the Phase 3 OASIS-HAE and OASISplus studies. Data from both studies were first presented at the 2024 European Academy of Allergy and Clinical Immunology (EAACI) Annual Congress in Valencia, Spain, and results from OASIS-HAE were published in *The New England Journal of Medicine*.

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 people 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 six marketed medicines and a leading pipeline in neurology, cardiology and select 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), LinkedIn and Instagram.

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, 2024, 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.

View source version on businesswire.com: https://www.businesswire.com/news/home/20250219736352/en/

Ionis Investor Contact:

D. Wade Walke, Ph.D.
IR@ionis.com
760-603-2331

Ionis Media Contact:
Hayley Soffer
media@ionis.com
760-603-4679

Source: Ionis Pharmaceuticals, Inc.