



Phase 3 OASISplus data demonstrating benefit of donidalorsen for HAE patients who switched from other prophylactics published in JACI In Practice

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- Patients switched to donidalorsen without increased breakthrough attacks and experienced further reductions in mean attack rate from baseline –
- 84% of patients surveyed preferred donidalorsen over their prior treatment –
- Donidalorsen is currently under review with the U.S. FDA as a potential first-in-class prophylactic treatment for HAE; PDUFA date August 21 –

CARLSBAD, Calif.--(BUSINESS WIRE)--Jul. 21, 2025-- [Ionis Pharmaceuticals, Inc.](#) (Nasdaq: IONS) today announced that positive results from the Phase 3 OASISplus prospective switch study of donidalorsen in patients with hereditary angioedema (HAE) were published in [The Journal of Allergy and Clinical Immunology \(JACI\) In Practice](#). Results indicate that patients who switched to donidalorsen from prior prophylactic treatments showed a 62% further reduction in mean monthly HAE attack rate from baseline, and 84% of patients surveyed reported preference for donidalorsen.

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. Many patients continue to face unpredictable, painful breakthrough attacks, even while receiving prophylactic treatment. 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.

“In this study, we saw that patients were able to switch to donidalorsen from another prophylactic without an increase in attacks, and in fact, there was a reduction in mean attack rate. This translated to meaningful improvements in quality of life and disease control compared to baseline with their prior treatment,” said Kenneth Newman, M.D., senior vice president, clinical development, Ionis. “The study was designed to provide patients and physicians with data to inform switching to donidalorsen, and the results support our belief that donidalorsen has the potential to be the prophylactic treatment of choice for HAE, if approved.”

The Phase 3 OASISplus study included an open-label extension (OLE) cohort of patients continuing from the OASIS-HAE trial, as well as a prospective cohort to assess patients switching from both oral and injectable long-term prophylactic treatments to donidalorsen. The OASISplus switch cohort evaluated the safety and efficacy of donidalorsen administered every four weeks in patients who were previously treated with lanadelumab (n=32), C1-esterase inhibitor (n=22) or berotralstat (n=11) for at least 12 weeks prior to entering the study. Patients entered a 10-week baseline period during which they remained on their prior HAE prophylactic therapy before switching to donidalorsen.

Patients followed a pre-defined specific protocol to transition from their prior therapy to donidalorsen with no mean increase in breakthrough attacks observed during the switch. After 16 weeks of treatment with donidalorsen, patients experienced a 62% overall further reduction in mean HAE attack rate compared to baseline from their prior prophylactic treatment. HAE attack rate decreased by 65%, 41% and 73% for patients switching from lanadelumab, C1INH and berotralstat, respectively, after switching to donidalorsen.

The majority (84%) of patients reported a preference for donidalorsen over their prior therapy, citing better disease control, less time to administer, and less injection site pain or reactions. More patients reported well-controlled disease (93% versus 67% at baseline) after switching to donidalorsen, as measured by the Angioedema Control Test (AECT). The AECT is a standardized patient-reported questionnaire consisting of four questions related to the frequency, unpredictability and control of angioedema, designed to help identify patients with poor disease control. Quality-of-life measures also improved regardless of prior therapy.

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. One patient discontinued due to a TEAE not related to donidalorsen.

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 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 has 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](https://www.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 donidalorsen, our commercial medicines, 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.

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