DAWNZERA™ (donidalorsen) receives positive opinion from CHMP, recommended for approval in EU for hereditary angioedema (HAE)

November 14, 2025

- *Recommendation based on breadth of clinical evidence demonstrating significant HAE attack rate reduction with DAWNZERA*
- *European Commission decision expected in Q1 2026*

CARLSBAD, Calif.--(BUSINESS WIRE)--Nov. 14, 2025-- Ionis Pharmaceuticals, Inc. (Nasdaq: IONS) and Otsuka Pharmaceutical Co., Ltd. (Otsuka) today announced that the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency has adopted a positive opinion of DAWNZERA™ (donidalorsen) for the routine prevention of recurrent attacks of hereditary angioedema (HAE) in adults and adolescents aged 12 years and older. The positive opinion is now referred to the European Commission (EC) for an approval decision.

“We believe the positive opinion from the CHMP reflects the robust clinical evidence supporting DAWNZERA and its potential to deliver a meaningful benefit to people living with HAE in the EU,” said Brett P. Monia, Ph.D., chief executive officer, Ionis. “This advancement is made possible by the shared dedication of the teams at Ionis and Otsuka to bring DAWNZERA to as many people living with HAE as possible.”

The CHMP opinion was based on positive results from the Phase 3 OASIS-HAE and OASISplus studies, in which DAWNZERA demonstrated positive results across multiple measures of disease including significant and sustained reduction in mean monthly HAE attack rate, including when self-administered via autoinjector.

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 about 1 in 50,000 people worldwide.

DAWNZERA was approved by the U.S. Food and Drug Administration in August 2025 for prophylaxis to prevent attacks of HAE in adult and pediatric patients 12 years of age and older. Otsuka holds exclusive rights to bring donidalorsen to patients across Europe and Asia Pacific.

“We are encouraged by the CHMP’s positive opinion, a key milestone in advancing access to potentially life-changing treatments for the HAE community,” said Andy Hodge, president and CEO, Otsuka Pharmaceutical Europe Ltd. “We look forward to the forthcoming decision from the European Commission and remain committed to addressing the unmet needs of this patient population.”

IMPORTANT SAFETY INFORMATION

CONTRAINDICATIONS

DAWNZERA is contraindicated in patients with a history of serious hypersensitivity reactions, including anaphylaxis, to donidalorsen or any of the excipients in DAWNZERA.

WARNINGS AND PRECAUTIONS

Hypersensitivity Reactions

Hypersensitivity reactions, including anaphylaxis, have been reported in patients treated with DAWNZERA. If signs and symptoms of serious hypersensitivity reactions occur, discontinue DAWNZERA and institute appropriate therapy.

ADVERSE REACTIONS

Most common adverse reactions (incidence ≥ 5%) are injection site reactions, upper respiratory tract infection, urinary tract infection, and abdominal discomfort.

Please see full Prescribing Information for DAWNZERA.

About DAWNZERA™ (donidalorsen)

DAWNZERA™ (donidalorsen) is approved by the U.S. Food and Drug Administration for prophylaxis to prevent attacks of hereditary angioedema (HAE) in adult and pediatric patients 12 years of age and older. DAWNZERA is an RNA-targeted medicine designed to target plasma prekallikrein (PKK), which plays an important role in activating inflammatory mediators associated with acute attacks of HAE. For more information about DAWNZERA, visit DAWNZERA.com. DAWNZERA is not yet approved for any indication in Europe.

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, cardiometabolic 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), LinkedIn and Instagram.

Forward-looking Statements

This press release includes forward-looking statements regarding Ionis' business and the therapeutic and commercial potential of DAWNZERA, 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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