DAWNZERA™ (donidalorsen) approved in the European Union for hereditary angioedema (HAE)

January 21, 2026

CARLSBAD, Calif.--(BUSINESS WIRE)--Jan. 21, 2026-- Ionis Pharmaceuticals, Inc. (Nasdaq: IONS) and Otsuka Pharmaceutical Co., Ltd. (Otsuka) today announced that the European Commission (EC) has approved DAWNZERA™ (donidalorsen) in the European Union (EU) for the routine prevention of recurrent attacks of hereditary angioedema (HAE) in adults and adolescents aged 12 years and older. The approval follows the positive opinion of the Committee for Medicinal Products for Human Use.

The approval is 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, with 94% overall mean monthly attack rate reduction at one year in the OASISplus open-label extension study. DAWNZERA is self-administered via subcutaneous autoinjector every four or eight weeks.

“The EU approval of DAWNZERA is an important milestone that reflects our ongoing commitment to bring this innovative medicine to people in need across the globe,” said Brett P. Monia, Ph.D., chief executive officer, Ionis. “As the first and only RNA-targeted therapy for HAE, we believe DAWNZERA has the potential to become the prophylactic therapy of choice for many patients across the EU. We extend our deepest gratitude to the patients, families and investigators who participated in our clinical trials and helped make this achievement possible.”

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.

“We are proud of the decision from the European Commission to authorize the use of DAWNZERA in HAE. This represents another key milestone in the collaboration between Otsuka and Ionis which aims to address unmet need in a challenging rare disease,” said Andy Hodge, President and CEO at Otsuka Pharmaceutical Europe Ltd. “We would like to thank all those at Otsuka and Ionis whose commitment has helped us introduce this medicine to patients.”

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 DAWNZERA to patients across Europe and Asia Pacific. With this approval, Ionis is eligible for a milestone payment of $15 million as well as tiered royalties of up to 30% on net product sales.

About DAWNZERA™ (donidalorsen)

DAWNZERA™ (donidalorsen) is approved in theU.S. and the EU 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.

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 Ionis Pharmaceuticals, Inc.

For three decades, Ionis has invented medicines that bring better futures to people with serious diseases. Ionis currently has marketed medicines and a leading pipeline in neurology, cardiometabolic disease 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.

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