

Ionis announces new donidalorsen data and presentations at the 2023 American Academy of Allergy, Asthma & Immunology Annual Meeting

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- Phase 2 open-label study data reinforce donidalorsen's potential to be a best-in-class prophylactic treatment for patients living with hereditary angioedema
- Ionis is preparing to launch donidalorsen, one of its three near-term commercial opportunities

CARLSBAD, Calif., Feb. 21, 2023 /PRNewswire/ -- [Ionis Pharmaceuticals, Inc.](#) (Nasdaq: IONS) today announced additional positive interim data from a Phase 2 open-label extension (OLE) study of donidalorsen, an investigational antisense medicine for the treatment of patients with hereditary angioedema (HAE). Positive interim data presented in November 2022 showed that treatment with donidalorsen resulted in an overall sustained mean reduction in HAE attack rates of 95% from baseline. In the latest update, patients treated for one year with donidalorsen showed a clinically meaningful 24-point mean improvement in their Angioedema Quality of Life (AE-QoL) total score relative to baseline with improvements observed in all domains. An improvement of 6 points or more is considered clinically meaningful¹.

These long-term, open-label data will be presented at the American Academy of Allergy Asthma & Immunology (AAAAI) Annual Meeting in San Antonio, Texas, February 24-27. Additional details can be found on the AAAAI [website](#).

"The improvement in quality of life demonstrated in patients treated with donidalorsen for one year were clinically meaningful and further support our belief in this medicine's potential to be a best-in-class prophylactic treatment for patients with HAE," said Richard S. Geary, Ph.D., executive vice president and chief development officer at Ionis. "We continue to be pleased with the progress of the Phase 3 OASIS study of donidalorsen, which remains on track to complete enrollment this year."

Poster titles:

- Poster #399: Phase 2 Open-Label Extension Study with Donidalorsen in Patients with Hereditary Angioedema: Updated Interim Analysis with Quality-of-Life Data
- Poster #400: Phase 2 Open-Label Extension Study with Donidalorsen Treatment in Patients with Hereditary Angioedema: Pharmacodynamic Data
- Poster #412: The Impact of Donidalorsen Taken Every 8 Weeks in Patients with Hereditary Angioedema

About the Phase 2 OLE Study

Patients who completed the blinded, placebo-controlled Phase 2 study were eligible for enrollment in the OLE study. There were 20 Type 1 or Type 2 HAE patients in the Phase 2 study, and 17 (85%) entered the OLE. Following a 13-week fixed-dose period where participants received subcutaneous donidalorsen 80 mg every four weeks, eight patients switched to subcutaneous donidalorsen 80 mg every eight weeks.

About Hereditary Angioedema (HAE)

HAE is a rare and potentially fatal genetic disease characterized by rapid and painful attacks of inflammation in the hands, feet, limbs, face, abdomen, larynx, and trachea. HAE is estimated to affect more than 20,000 patients in the U.S. and Europe and can be fatal if swelling occurs in the larynx. In patients with frequent or severe attacks, doctors frequently use prophylactic treatment approaches to prevent and reduce the severity of HAE attacks.

About Donidalorsen

Donidalorsen is an investigational antisense medicine that uses Ionis' advanced **L**igand-**C**onjugated **A**ntisense (LICA) technology and is designed to reduce the production of prekallikrein, or PKK, to treat patients with HAE. PKK plays an important role in the activation of inflammatory mediators associated with acute attacks of HAE.

About Ionis Pharmaceuticals, Inc.

For more than 30 years, Ionis has been the leader in RNA-targeted therapy, pioneering new markets and changing standards of care with its novel antisense technology. Ionis currently has three marketed medicines and a promising late-stage pipeline highlighted by industry-leading cardiovascular and neurological franchises. Our scientific innovation began and continues with the knowledge that sick people depend on us, which fuels our vision to become the leader in genetic medicine, utilizing a multi-platform approach to discover, develop and deliver life-transforming therapies.

To learn more about Ionis visit www.ionispharma.com and follow us on Twitter @ionispharma.

Ionis' Forward-looking Statements

This press release includes forward-looking statements regarding Ionis' business and the therapeutic and commercial potential of Ionis' technologies, donidalorsen and other products in development. 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 related to the impact COVID-19 could have on our business, and including but not limited to, those related to our commercial products and the medicines in our pipeline, and particularly 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. 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 Dec. 31, 2021, and the most recent Form 10-Q quarterly filing, 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" refers to Ionis Pharmaceuticals and its subsidiaries.

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¹ Weller K, et al. Allergy. 2016;71(8): 1203-1209.

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