Ionis doses first participant in Phase 1-2 ASCEND study of ION337 in Dravet syndrome

July 13, 2026

CARLSBAD, Calif.--(BUSINESS WIRE)--Jul. 13, 2026-- Ionis Pharmaceuticals, Inc. (Nasdaq: IONS) today announced that the first participant has been dosed in the Phase 1-2 ASCEND study of ION337, an investigational RNA-targeted medicine for people living with Dravet syndrome, a rare, severe and lifelong neurological disorder. Dravet syndrome typically begins in infancy and is associated with prolonged seizures, developmental delays, cognitive impairments and an increased risk of sudden death.

“The first participant to receive ION337 in the ASCEND study marks an important step toward advancing a potential disease modifying therapy for people living with Dravet syndrome,” said Holly Kordasiewicz, Ph.D., executive vice president, chief development officer, Ionis. “ION337 is our first wholly owned medicine developed using Ionis’ advanced NMA technology, which is designed to enhance the potency of our medicines and potentially support infrequent intrathecal dosing of every six months or less. This milestone reflects a new wave of scientific innovation across Ionis’ advancing neurology pipeline for people living with serious neurological conditions. We look forward to advancing the development of ION337 alongside the Dravet syndrome community.”

ASCEND (NCT07531745) is an open-label, Phase 1-2 study evaluating the safety and tolerability of ION337 in children aged 2 to 12 years with a clinical diagnosis of Dravet syndrome. The study consists of two parts: an initial 6-month single ascending dose (SAD) component, followed by a 24-month multiple ascending dose (MAD) component with dosing of ION337 every 6 months, and an additional 7-month safety follow-up period.

ION337 uses next-generation N-Methylacetamide modifications (NMA technology) designed to enhance the potency of splice modulating antisense oligonucleotides (ASOs). This advanced molecular design is intended to provide sustained activity, supporting infrequent dosing.

About ION337

ION337 is an investigational RNA-targeted medicine designed to increase production of the $Na_V1.1$ protein, which is reduced in people with Dravet syndrome (DS) caused by certain *SCN1A* gene variants. The U.S. Food and Drug Administration (FDA) has granted ION337 Fast Track designation for the treatment of Dravet syndrome.

About Dravet Syndrome (DS)

Dravet syndrome is a rare, severe and lifelong developmental epileptic encephalopathy that impacts 1 in 30,000 individuals globally. Dravet syndrome typically presents in infancy and is commonly associated with prolonged, treatment-resistant seizures. People living with this condition may also experience developmental delays, speech and language difficulties, movement or balance problems and behavioral or attention challenges. Dravet syndrome is typically caused by a change in one copy of the *SCN1A* gene that leaves the brain with insufficient quantities of a protein called $Na_V1.1$, disrupting signaling and leading to excessive brain activity. Current therapies are primarily focused on symptomatic management of seizures, with generally poor long-term prognosis. There are currently no approved disease modifying therapies.

About Ionis Neurology

Ionis has been at the forefront of discovering and developing leading neurological disease medicines, including SPINRAZA® (nusinersen), the first approved treatment for spinal muscular atrophy, WAINUA® (eplontersen), a medicine to treat hereditary transthyretin-mediated amyloid polyneuropathy (ATTRv-PN), and QALSODY® (tofersen) for SOD1-ALS. The clinical-stage neurology portfolio includes 13 investigational medicines, of which six are wholly owned by Ionis. Ionis' investigational portfolio includes medicines for which there are few or no disease modifying treatments, such as rare diseases including Alexander disease, Angelman syndrome, prion disease, multiple system atrophy and Huntington’s disease, as well as more common conditions such as Alzheimer's disease.

About Ionis Pharmaceuticals, Inc.

For more than 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 additional 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 ION337, our commercial medicines, additional medicines in development and technologies and our expectations regarding development and regulatory milestones. 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, 2025, 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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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

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