Ionis announces license agreement with Recordati for zilganersen in Alexander disease (AxD) in all countries outside the U.S.

June 25, 2026

- Recordati brings deep rare disease expertise and proven capabilities in commercialization, access and country-specific regulatory environments -

- Ionis to independently commercialize zilganersen in the U.S. assuming FDA approval -

CARLSBAD, Calif.--(BUSINESS WIRE)--Jun. 25, 2026-- Ionis Pharmaceuticals, Inc. (Nasdaq: IONS) today announced that it has entered into a license agreement with Recordati ("Recordati"), under which Recordati obtained exclusive rights to develop and commercialize zilganersen, an investigational RNA-targeted medicine for Alexander disease (AxD), in all countries outside the U.S. Ionis will maintain sole commercial responsibility for zilganersen in the U.S. and will continue to lead development globally. Recordati will be responsible for regulatory filings and commercialization outside the U.S., including country-specific support for early access pathways based on local regulations and access dynamics.

"Recordati combines proven rare disease and global commercialization expertise with deep experience navigating regional and local regulatory environments and the infrastructure needed to reach patients outside the United States, making them the right partner to deliver zilganersen to patients with urgency. They recognize, as we do, the high unmet need for this rare, serious and often fatal neurodegenerative disease," said Brett P. Monia, Ph.D., chief executive officer, Ionis. "Together, we are committed to working closely to help bring zilganersen to people living with Alexander disease outside the United States and enable broader access to this important treatment."

Ionis plans to independently launch zilganersen in the U.S, which is currently under FDA review with a Prescription Drug User Fee Act (PDUFA) action date of September 22. As the potential first and only disease modifying treatment for AxD, zilganersen would also mark Ionis' first independent commercial launch in neurology, advancing the company's goal of bringing transformational medicines to people with serious neurological diseases.

Rob Koremans, Chief Executive Officer, Recordati, commented, "We are very pleased to partner with Ionis on the development and commercialization of zilganersen outside the United States. The addition of zilganersen further strengthens our Rare Diseases portfolio with a potentially transformative therapy for patients affected by Alexander disease, a devastating and progressive neurological disorder with significant unmet medical need. By combining Ionis' innovation and development expertise with Recordati's global rare disease capabilities, we believe we are well positioned to maximize the potential of zilganersen and bring a much-needed treatment option to patients with Alexander disease."

After disclosing positive top-line results, Ionis recently announced additional positive data from the pivotal study of zilganersen, highlighting potential treatment benefit across multiple AxD symptom domains and reinforcing zilganersen's positive impact on people living with AxD. The study met its primary endpoint in individuals ≥ 5 years of age, with zilganersen 50 mg demonstrating statistically significant and clinically meaningful stabilization of gait speed as assessed by the 10-Meter Walk Test (10MWT), a commonly used measure of gross motor function in neurologic disease, compared to control at Week 61. Secondary and exploratory endpoint results from patient/caregiver- and clinician-reported outcome assessments consistently favored zilganersen.

Zilganersen demonstrated a favorable safety and tolerability profile, with most adverse events (AE) mild or moderate in severity. Serious treatment-emergent adverse events (TEAEs) occurred less frequently in the zilganersen group compared to control (37.5% zilganersen 25 mg or 50 mg; 47.1% pooled control).

Under the terms of the agreement, Ionis receives an upfront payment of $30 million, and is eligible to receive additional payments based on achievement of milestones and a tiered royalty of up to the mid-20% range on annual net sales.

About Zilganersen

Zilganersen is an investigational antisense oligonucleotide medicine being evaluated as a treatment for people with Alexander disease (AxD). Zilganersen is designed to inhibit production of excess glial fibrillary acidic protein (GFAP) that accumulates because of disease-causing variants in the *GFAP* gene. The U.S. Food and Drug Administration (FDA) granted zilganersen Breakthrough Therapy, Orphan Drug and Rare Pediatric Disease designations for AxD. In addition, the European Medicines Agency (EMA) granted zilganersen Orphan Drug designation for AxD.

About Alexander Disease (AxD)

AxD is a rare, progressive and often fatal neurological disease that occurs in approximately 1 per 1 to 3 million people worldwide and affects a type of cell in the brain called astrocytes. Astrocytes have multiple roles in the brain to support neurons and oligodendrocytes, including maintenance of the myelin sheath that protects nerve fibers. AxD is caused by disease-causing variants in the glial fibrillary acidic protein (*GFAP*) gene and is generally characterized by progressive neurological deterioration resulting in loss of functional mobility, loss of independence and the inability to control muscles for large movements, swallowing and airway protection, though symptoms can vary depending on age of onset. AxD usually leads to death within 14-25 years after symptom onset. There are no approved disease-modifying medicines.

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 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 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 zilganersen, 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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