

## RECORDATI ANNOUNCES LICENSE AGREEMENT WITH IONIS PHARMACEUTICALS FOR ZILGANERSEN FOR THE TREATMENT OF ALEXANDER DISEASE IN ALL COUNTRIES OUTSIDE THE U.S.

*Agreement strengthens Recordati's Rare Diseases portfolio with a potential first-in-class treatment for Alexander disease*

*Zilganersen has demonstrated encouraging clinical data in pivotal study*

Milan, 24 June 2026 – Recordati today announced that it has entered into a license agreement with Ionis Pharmaceuticals, Inc. ("Ionis") for exclusive development and commercialization rights to zilganersen, an investigational RNA-targeted medicine for the treatment of Alexander disease (AxD), in all countries outside the U.S. 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. Ionis will maintain sole commercial responsibility for zilganersen in the U.S. and will continue to lead development globally.

**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."

**Brett P. Monia, Ph.D., Chief Executive Officer, Ionis**, added, "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. 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."

Alexander disease (AxD) is a rare, progressive and often fatal neurological disorder caused by mutations in the *GFAP* gene. The disease affects astrocytes, critical support cells in the brain, leading to progressive loss of neurological function, including mobility, independence, swallowing and breathing. Symptoms vary by age of onset, and patients often die within 14–25 years after symptoms begin. There are currently no approved disease-modifying therapies for AxD.

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  $\geq 5$  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

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(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, Recordati will pay Ionis an upfront payment of \$30 million, and Ionis 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 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 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.

*Recordati is an international pharmaceutical Group listed on the Italian Stock Exchange (XMI: REC), with roots dating back to a family-run pharmacy in Northern Italy in the 1920s. We are uniquely structured to provide treatments across specialty and primary care and rare diseases. Our fully integrated operations span clinical development, manufacturing of active ingredients and finished products, commercialization and licensing. We operate in approximately 150 countries across EMEA, the Americas and APAC, with around 4,700 employees. We believe that health is a fundamental right, not a privilege. Today, our purpose "Unlocking the full potential of life" aims to empower individuals to live life to the fullest, from common conditions to the rarest.*

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