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**PRESS RELEASE****Rare Disease Day 2024 - Call for greater awareness of X-linked adrenoleukodystrophy and cerebral adrenoleukodystrophy – special film launched**

**Barcelona, Spain and Düsseldorf, Germany – 29 February, 2024** – Neuraxpharm Group (Neuraxpharm), a leading European specialty pharmaceutical company focused on the treatment of central nervous system (CNS) disorders, and Minoryx Therapeutics, a late stage biotech company focused on the development of therapies for orphan central nervous system disorders, reaffirm their commitment on Rare Disease Day 2024 to finding better treatments for patients suffering from a range of rare diseases, such as leukodystrophy, including X-linked adrenoleukodystrophy (X-ALD) and more specifically cerebral adrenoleukodystrophy (cALD).

This Rare Disease Day, to raise awareness of this disease, Neuraxpharm and Minoryx announce the publication of a special information film with the participation of world renowned expert Professor Fanny Mochel, the Director of the Reference Center for Leukodystrophies - La Pitié-Salpêtrière Hospital, Paris, and Karen Harrison, Support Services Manager of Alex, The Leukodystrophy Charity (Alex-TLC). In the film, Professor Mochel describes the disease and the need for regular screening to identify patients as early as possible, while Karen Harrison speaks powerfully about the patient experience and need for better treatments.

The film can be found on Neuraxpharm's and Minoryx's websites, YouTube and LinkedIn channels.

**Dr. Jörg-Thomas Dierks, Chief Executive Officer of Neuraxpharm, said:** *“The impact this disease has on patients and their families cannot be overstated. At Neuraxpharm, we are committed to bringing better treatment to patients so that we can improve the lives of those affected by this devastating disease. We hope that this film will raise awareness and understanding of the condition.”*

**Marc Martinell, Chief Executive Officer of Minoryx said:** *“The X-ALD community deserve all our support to help them face the extremely challenging consequences of this disease, which in the case of cALD are fatal. We want to raise awareness of the diseases and the importance of regular monitoring and early detection as these are critical factors for timely management of these patients.”*

On 26 January 2024, Neuraxpharm and its partner Minoryx Therapeutics stated that they are seeking a re-examination of leriglitzzone, Minoryx's novel brain-penetrant and selective PPAR gamma agonist, by the European Medicines Agency's Committee for Medicinal Products for Human Use (CHMP) for conditional approval for patients with cALD. Minoryx and Neuraxpharm strongly believe that leriglitzzone has a positive benefit / risk balance in patients with cALD.

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## **About X-ALD and cALD**

X-linked adrenoleukodystrophy (X-ALD) is an orphan neurodegenerative disease. The global incidence of X-ALD is approximately 6-8/100,000 live births. Boys and adult men with X-ALD can, at any point in their lifetime, develop cALD, which is characterized by demyelinating brain lesions that may become rapidly progressive, leading to acute neurological decline and death. These lesions can produce severe symptoms such as loss of voluntary movements, inability to swallow, loss of communication, cortical blindness and total incontinence and death with a mean survival of 3 to 4 years.

Progressive cALD occurs in 31-35% of ALD patients in childhood with typical onset between the age of 2-12 and up to 60% of adult patients, with X-ALD will develop progressive cALD over time. There is currently no pharmacological treatment available for cALD. In childhood, Hematopoietic Stem Cell Transplantation (HSCT) can arrest the disease. However, it is an aggressive procedure and only available for a portion of patients; autologous HSCT is not globally available; this still requires hemo-ablation therapy with associated comorbidities and long-term safety data is being gathered. In adults, experience in HSCT is very limited and the intervention is often not recommended.

In addition, all X-ALD patients reaching adulthood develop adrenomyeloneuropathy (AMN), characterized by progressive spastic paraparesis, as well as progressive deterioration of balance and sensory function, and development of incontinence. This form progresses chronically with onset of symptoms typically in adulthood, affecting both men and women, and has poor prognosis.

## **About leriglitazone**

Leriglitazone is Minoryx Therapeutics's novel orally bioavailable and selective PPAR gamma agonist with a potential first-in-class and best-in-class profile for CNS diseases. It has demonstrated brain penetration and a favorable safety profile. It showed robust preclinical proof-of-concept in animal models of multiple diseases by modulating pathways leading to mitochondrial dysfunction, oxidative stress, neuroinflammation, demyelination and axonal degeneration. In clinical trials, leriglitazone showed clinical benefit in both adult X-ALD patients in ADVANCE and pediatric X-ALD patients in NEXUS. Data from ADVANCE showed that leriglitazone reduces the progression of lesions and the development of progressive cALD. Results on radiological stabilization seen in NEXUS after 24 weeks of treatment were similar to those attained with Hematopoietic Stem Cell Transplant (HSCT) or ex-vivo gene therapy, hence it is expected that leriglitazone could provide a comparable clinical benefit to cALD patients. Leriglitazone has been granted orphan drug status for X-ALD from the FDA and the EMA and Fast Track and Rare Pediatric Disease designation from the FDA for the treatment of X-ALD.

## **About the Neuraxpharm Group**

Neuraxpharm is a leading European specialty pharmaceutical company focused on the treatment of the central nervous system (CNS), including both psychiatric and neurological disorders. It has a unique understanding of the CNS market built over 35 years.

Neuraxpharm is constantly innovating, with new products and solutions to address unmet patient needs and is expanding its portfolio through its pipeline, partnerships and acquisitions.

The company has c.1,000 employees and develops and commercializes CNS products through a direct presence in more than 20 countries in Europe, two in Latin America, and globally via partners in more than 40 countries. Neuraxpharm is backed by funds advised by Permira.

Neuraxpharm manufactures many of its pharmaceutical products at Neuraxpharm Pharmaceuticals (formerly Laboratorios Lesvi) in Spain.

For more information, please visit [www.neuraxpharm.com](http://www.neuraxpharm.com)

## About Minoryx

Minoryx Therapeutics is a registration stage biotech company focusing on the development of novel therapies for orphan central nervous system (CNS) diseases with high unmet medical needs. The company's lead program, leriglitazone (MIN-102), a novel, brain penetrant and selective PPAR gamma agonist, is being developed to treat X-linked adrenoleukodystrophy (X-ALD) and other orphan CNS diseases. The company is backed by a syndicate of experienced investors, which includes Columbus Venture Partners, CDTI Innvierte, Caixa Capital Risc, Fund+, Ysios Capital, Roche Venture Fund, Kurma Partners, Chiesi Ventures, S.R.I.W, Idinvest Partners / Eurazeo, SFPI-FPIM, HealthEquity, Sambrinvest and Herrecha, and has support from a network of other organizations.

Minoryx was founded in 2011, is headquartered in Spain with Belgian facilities and has so far raised more than €120 million.

For more information, please visit <https://www.minoryx.com/>