

Leriglitazone has met the primary endpoint in NEXUS, the pivotal trial for pediatric patients with cerebral Adrenoleukodystrophy

Statistically significant difference in arrested disease in leriglitazone treated patients when compared to the natural history of the disease

Submission of European marketing authorization application (MAA) for leriglitazone expected mid-2025.

Barcelona, Spain and Düsseldorf, Germany – 11 December 2024 – Minoryx Therapeutics, a late-stage biotech company focused on the development of therapies for orphan central nervous system (CNS) disorders and Neuraxpharm Group (Neuraxpharm), a leading European specialty pharmaceutical company focused on the treatment of CNS disorders, today announced that the NEXUS trial has been completed and that the primary endpoint has been met. The parties intend to file for European Marketing Authorization of leriglitazone in pediatric and adult cerebral Adrenoleukodystrophy (cALD) patients by mid-2025.

The NEXUS trial was a 96-week, pivotal, open-label study designed to evaluate the efficacy and safety of once-daily oral dosing of leriglitazone in pediatric patients with cALD. The primary endpoint assessed the proportion of patients that had clinical and radiological arrested disease at week 96 or at a visit prior to hematopoietic stem cell transplant (HSCT). The evaluable population in the study consisted of 20 patients treated for a minimum of 24 weeks. All 20 patients remained clinically stable whilst on treatment and 7 out of 20 patients (35%) met the arrested disease criteria, which is significantly greater than the 10% self-arrested patients that would be expected from natural history ($p < 0.05$).

Leriglitazone was well tolerated in all children, there were no treatment-related serious adverse events nor discontinuations due to treatment related adverse events.

The full results of the NEXUS trial will be presented at upcoming neurology conferences.

“Cerebral ALD in boys is a devastating disease both for the patients and their families. Treatment options are limited, physicians and families of patients have constantly been searching for better tools and therapies to manage cALD in boys.” said **Patricia Musolino, Global Principal Investigator of Nexus**. *“The NEXUS results attest that leriglitazone addresses a critical unmet need for non-invasive treatments that can be administered at the moment brain lesions are identified to arrest or slow down lesion growth.”*

*“We are very pleased with the positive results from NEXUS demonstrating that leriglitzazone not only arrests the demyelinating brain lesions in children with cALD but also arrests clinical progression. These results are further supported by evidence from the cALD related endpoints of ADVANCE¹ in adult patients and the findings reported in cALD patients treated under compassionate use¹” **said Marc Martinell, CEO, Minoryx.** “Together with our partner, Neuraxpharm, we intend to submit the MAA to the EMA for this life-threatening condition as soon as possible.”*

Dr. Jörg-Thomas Dierks, CEO, Neuraxpharm *said*: *“cALD is a very serious neurological disorder with devastating outcomes for patients and their families. The results from this trial are extremely encouraging and we are fully committed to working with Minoryx to swiftly provide patients with an effective new treatment for this otherwise fatal and cruel disease.”*

Based on the successful results from NEXUS, Minoryx and Neuraxpharm have begun to compile the regulatory file for MAA submission in the European Union.

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About leriglitzazone

Leriglitzazone is Minoryx Therapeutics’ novel orally bioavailable, 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 neuroinflammation, demyelination, mitochondrial dysfunction, oxidative stress, and axonal degeneration. In clinical trials, leriglitzazone showed clinical benefit in both pediatric X-ALD patients in NEXUS and adult X-ALD patients in ADVANCE. Results from NEXUS demonstrate that pediatric cALD patients are clinically and radiologically stable after 96 weeks of treatment or at a visit prior to HSCT. Data from ADVANCE showed that leriglitzazone reduces the progression of lesions and the development of progressive cALD in adult patients. Another clinical trial in adult male patients with progressive cALD (CALYX²) is currently recruiting in the US, Argentina, Brazil and Europe. Leriglitzazone 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 NEXUS

NEXUS is a 96-week, pivotal, open-label, multicenter study (NEXUS; NCT04528706) of once-daily oral leriglitzazone in pediatric patients with cALD. NEXUS has enrolled 23 patients and the evaluable

population consisted of those patients treated for a minimum of 24 weeks (n=20). The primary endpoint assesses the proportion of evaluable patients that have clinically and radiologically arrested disease at week 96 or at a visit prior to HSCT (success criteria: one-sided 95% [CI] > 10%). Secondary endpoints include change from baseline in NFS and Loes score (LS). Change from baseline in lesion volume and plasma biomarker concentrations are exploratory endpoints.

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 is only available for a portion of patients. Gene therapy based HSCT is not globally available, and it requires myeloablative chemotherapy with associated comorbidities. In adults, experience in HSCT is very limited and this intervention is often not recommended.

In addition, all X-ALD patients reaching adulthood develop adrenomyeloneuropathy (AMN). This form progresses chronically and cALD patients with advanced AMN are largely ineligible to HSCT due to the poor prognosis of the treatment.

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, leriglitzone (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, Criteria BioVentures, 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/>

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 more than 1,000 employees and develops and commercializes CNS products through a direct presence in more than 20 countries in Europe, two in Latin America, one in the Middle East and globally via partners in more than 50 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

¹ ADVANCE, a pivotal phase 2/3 randomized, double-blind, placebo-controlled, clinical study with an open-label extension, was designed to assess the efficacy and safety of leriglitazone in male patients with X-ALD.

² CALYX, a phase 3, multicenter, randomized (1:1), double-blind, placebo-controlled, clinical study, has been designed to compare the efficacy and safety of leriglitazone in male adult patients with progressive cALD (<https://clinicaltrials.gov/study/NCT05819866>).