PRESS RELEASE

Leriglitazone Marketing Authorization Application submitted for treatment of cerebral adrenoleukodystrophy has been validated by European Medicines Agency

Barcelona, Spain and Düsseldorf, Germany – 23 July 2025 – 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 announce that the Marketing Authorization Application (MAA) for Minoryx's lead candidate leriglitazone (NEZGLYAL®) has been submitted to the European Medicines Agency (EMA) for the treatment of paediatric and adult male patients with cerebral adrenoleukodystrophy (cALD). EMA has now validated the MAA file and it is under review by the Committee for Medicinal Products for Human Use (CHMP).

This new MAA is based on recent data from the successfully concluded NEXUS study, a 96-week, pivotal, open-label study designed to evaluate the efficacy and safety of once-daily oral dosing of leriglitazone in paediatric patients with cALD. The study met the primary endpoint and all 20 evaluable patients remained clinically stable whilst on treatment and 7 out of 20 evaluable patients (35%) met the arrested disease criteria, which is significantly greater than the 10% self-arrest rate that would be expected from natural history ($p<0.05$). Leriglitazone was well tolerated with no discontinuations due to adverse events.

The submission is further supported by data from the previously concluded ADVANCE study, a 116 patient, double-blind, placebo-controlled study conducted in Europe and the United States in which leriglitazone reduced the incidence and progression of cerebral lesions in adults. Furthermore, data from the ongoing extensive compassionate use program in adult and paediatric patients with cALD have also been submitted. Leriglitazone was generally well tolerated in the ADVANCE study and compassionate use programme.

"We believe the new and positive data from Nexus provide compelling evidence for the efficacy of leriglitazone giving hope to patients and their families that a new treatment will be available in the near future," **said Marc Martinell, CEO, Minoryx**. *"Leriglitazone, if approved, would be the only pharmacological treatment for patients suffering from this devastating orphan disease with a major unmet medical need."*

"We are very pleased that the clinical development programme and compassionate use data have shown the efficacy of leriglitazone across all age ranges," **said Arun Mistry, Chief Medical Officer, Minoryx.** *"We will continue to work closely with the EMA through the evaluation process to maximize the chance for this therapy to reach all patients in desperate need."*

Dr. Jörg-Thomas Dierks, CEO, Neuraxpharm said; *"The EMA's validation of the leriglitazone MAA submission is a key step forward as we look to bring this much needed treatment to patients. Given the terrible impact of this devastating disease and the lack of effective options, we remain hopeful of a positive outcome from the CHMP."*

The development of leriglitazone continues with the ongoing CALYX trial in adult patients with cALD and the TREE study in paediatric Rett patients. Both studies are open to recruitment.

About leriglitazone (NEZGLYAL®)

Leriglitazone 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 favourable 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, leriglitazone showed clinical benefit in both paediatric X-ALD patients in NEXUS and adult X-ALD patients in ADVANCE. Results from NEXUS demonstrate that paediatric cALD patients are clinically

and radiologically stable after over 96 weeks of treatment or at a visit prior to haematopoietic stem cell transplantation (HSCT). Data from ADVANCE showed that leriglitazone stabilises lesion growth and reduces the incidence of progressive cALD therefore delaying or preventing life threatening progression of the disease in adult patients. Another clinical trial in adult male patients with progressive cALD (CALYX2) is currently recruiting in the US, Argentina, Brazil, Europe and India. 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 NEXUS

NEXUS is a 96-week, pivotal, open-label, multicentre study (NEXUS; NCT04528706) of once-daily oral leriglitazone in paediatric patients with cALD. NEXUS enrolled 23 patients and the evaluable population consisted of those patients treated for a minimum of 24 weeks (n=20). The primary endpoint assessed the proportion of evaluable patients that had clinically and radiologically arrested disease at week 96 or at a visit prior to HSCT (success criteria: one-sided 95% [CI] > 10%). Secondary endpoints included 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 characterised 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 three to four 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, 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 with 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 TREE

TREE2 is a 36-week, phase 2a, randomised 1:1, placebo-controlled study of once-daily oral leriglitazone in female paediatric patients aged 5-12 with Rett syndrome conducted at Hospital Sant Joan de Déu, Barcelona, Spain. The study will enroll 24 patients that will undergo 36 weeks of treatment and 4 weeks of follow-up. The primary endpoint is safety. In addition, the study assesses a number of secondary and exploratory endpoints including the Rett Syndrome Behaviour Questionnaire (RSBQ), the Vineland Adaptive Behaviour Scale (VABS), and the Rett Syndrome Motor Evaluation Scale (RESMES) including various biomarkers.

About Rett Syndrome

Rett syndrome is a rare genetic neurological and developmental disorder that primarily affects females. It is almost exclusively caused by a de-novo genetic mutation in the MECP2 gene on the X-chromosome. The disorder causes a progressive loss of motor skills and language. Babies born with Rett syndrome initially seem to develop as

expected, however they subsequently lose the skills they previously had such as the ability to crawl, walk, communicate and use of their hands. Over time, these children experience increasing problems with the use of muscles that control movement, coordination and communication. Most patients also experience seizures and have intellectual disabilities. Rett syndrome affects about 1 in 10-20.000 females and natural history suggest that many patients live into middle age.

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 programme, 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, 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 organisations.

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 40 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 commercialices CNS products through a direct presence in more than 20 countries in Europe, two in Latin America, one in the Middle East, one in Australia 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

Neuraxpharm, has licensed the rights to leriglitazone in Europe and Sperogenix has licensed the rights to China.

1 ADVANCE, a pivotal phase 2/3 randomised, 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, multicentre, randomised (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).