PRESS RELEASE

Minoryx and Neuraxpharm complete enrolment in leriglitazone pivotal Phase 3 trial (CALYX) in adult patients with cerebral Adrenoleukodystrophy (cALD)

Top-line results expected in early 2028; trial to support future label expansion in the EU and to enable NDA filing in the US.

Mataró, Barcelona, Spain, and Düsseldorf, Germany, 9 September 2026 - Minoryx Therapeutics and Neuraxpharm Group (Neuraxpharm), announce that the last patient has been enrolled in the global Phase 3 clinical trial (CALYX) evaluating leriglitazone (NEZGLYAL®) in adult male patients with cerebral Adrenoleukodystrophy (cALD), an orphan neurodegenerative disease with no pharmacological therapeutic options.

In Europe, leriglitazone has received a positive opinion from the Committee for Medicinal Products for Human Use (CHMP) for the treatment of boys aged 2-12 with cALD and non-gadolinium enhancing lesions. Approval under exceptional circumstances from the European Commission (EC) is expected later this month and Neuraxpharm, a leading CNS specialist company and Minoryx's strategic partner in Europe, is preparing for the commercial launch.

"The full recruitment of CALYX is a major milestone towards confirming the disease-modifying potential of leriglitazone, expanding the label in the EU and obtaining approval for leriglitazone in the US," ***said Arun Mistry, Chief Medical Officer, Minoryx.*** *"With a read-out expected by early 2028, attaining this milestone enables the initiation of preliminary US regulatory and commercial infrastructure planning."*

"We are deeply grateful to the patients, families and global advocacy groups for supporting the trial and helping to advance a much-needed new therapeutic option for cALD," ***said Sílvia Pascual, VP Clinical Development, Minoryx.***

CALYX is a global, double-blind, randomized placebo-controlled Phase 3 clinical trial that has enrolled 41 adult male patients with cALD across selected centres of excellence in the US, Europe, South America and India. The primary endpoint evaluates survival and the secondary endpoints include the Loes score, major functional disabilities, activities of daily living and major neurocognitive impairment.

"Completion of enrolment in CALYX marks an important milestone in the development of leriglitazone and the generation of clinical evidence in adult patients with cALD," ***said Francisco Jurado, Chief Scientific Officer, Neuraxpharm****. "Together with the anticipated European Commission approval later this month, this achievement highlights the continued progress of the clinical development programme and its potential to support future regulatory applications across different cALD patient populations in Europe."*

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About CALYX

CALYX enrolled 41 adult, male cALD patients with gadolinium-enhancing brain lesions, and where HSCT (hematopoietic stem cell transplantation) is not recommended or refused by the patient. Patients were randomized either to leriglitazone or placebo with 1:1 randomization. The key exclusion criteria involved a Loes score greater than 12, and patients that previously received HSCT. The trial has an adaptive duration with an initial efficacy read-out at 18 months and if needed subsequent efficacy assessments at 27 and 36 months respectively with the option to complete the study at any of the three time points once statistical significance is reached. Statistical analysis will be carried out using 0.05 one-sided alpha at all time-points. The primary endpoint is 'time to death or bedridden with permanent ventilatory support'. A key secondary endpoint is radiological progression through the Loes score. Other secondary endpoints include major functional disabilities, activities of daily living and major neurocognitive impairment, as well as biomarkers in plasma such as neurofilament light chain. CALYX is intended to support label expansion in the EU and it is the NDA-enabling pivotal trial in the US as per agreement with the FDA.

About leriglitazone (NEZGLYAL®)

It is Minoryx Therapeutics' orally bioavailable, brain penetrating, selective PPAR gamma agonist. It has shown 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, it has shown clinical benefit in both paediatric cALD patients in the NEXUS[1] clinical trial and adult cALD patients in the ADVANCE[2] trial. 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 hematopoietic stem cell transplantation (HSCT). Data from ADVANCE showed that leriglitazone reduced cALD progression. More than 170 patients with cALD have received treatment to date. Leriglitazone has been granted orphan drug status for X-ALD by the FDA and the EMA, and Fast Track and Rare Paediatric Disease designation from the FDA for the treatment of X-ALD. Leriglitazone is also being investigated in a Phase 2a trial in pediatric patients with Rett syndrome with read-out by the end of 2026 (the TREE[3] trial). In Europe, it is exclusively licensed to Neuraxpharm.

Leriglitazone is pending European Commission approval, following a positive CHMP opinion issued in July 2026 for approval under exceptional circumstances in the European Union for the treatment of cerebral Adrenoleukodystrophy (cALD) in males with Adrenoleukodystrophy (ALD) aged 2 to

12 years with non-gadolinium (Gd) enhancing lesions (i.e. Gd negative) in brain Magnetic Resonance Imaging (MRI), with a Neurological Functional Score (NFS) of 0 or 1.

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 initially are non-gadolinium enhancing (i.e. Gd-negative) and they become gadolinium enhancing (ie. Gd-positive) as they progress, reflecting damage to the blood-brain barrier. Lesion progression causes severe symptoms such as loss of voluntary movement, inability to swallow, loss of communication, cortical blindness, 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 ages of 2-12, and up to 60% of adult patients with X-ALD will develop progressive cALD over time. There is currently no approved pharmacological treatment for cALD, while leriglitazone is awaiting a final decision by the European Commission following a positive CHMP opinion. In childhood, HSCT can arrest the disease. However, it is an invasive 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 for 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 programme, leriglitazone, 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, including 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 and Sambrinvest, and has support from a network of other organisations.

Minoryx was founded in 2011, is headquartered in Spain with Belgian facilities and has raised more than €150 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) disorders, including both psychiatric and neurological conditions, with a unique understanding of the CNS market.

Neuraxpharm is constantly innovating new products and solutions to address unmet patient needs and is expanding its portfolio through its pipeline, partnerships and acquisitions, including in the area of rare diseases.

The company has more than 1,000 employees and develops and commercialises CNS products through a direct presence in more than 20 countries in Europe, two in Latin America, one in the Middle East, 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 in Spain.

For more information, please visit https://www.neuraxpharm.com/

Forward-Looking Statements

This press release contains forward-looking statements that involve risks and uncertainties that could cause actual results to differ materially.

[1] NEXUS, a Phase 2/3, open-label clinical study designed to assess the efficacy and safety of leriglitazone in male paediatric patients with early stage cALD.
https://www.clinicaltrialsregister.eu/ctr-search/search?query=2019-000654-59

[2] 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 AMN with or without cALD.

[3] TREE, a Phase 2a, randomised (1:1), double-blind, placebo-controlled, clinical study, designed to evaluate the safety (and efficacy) of leriglitazone in paediatric female patients with Rett syndrome.
https://euclinicaltrials.eu/ctis-public/view/2024-514684-26-00?lang=en