Source: *Neuraxpharm*

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NEZGLYAL® (leriglitazone) receives a positive CHMP opinion for the treatment of cerebral Adrenoleukodystrophy (cALD), a rare neurodegenerative disease

Recommendation based on NEXUS data; Minoryx and Neuraxpharm expect European Commission approval by the end of this quarter

BARCELONA, Spain and DÜSSELDORF, Germany, July 24, 2026 (GLOBE NEWSWIRE) -- Minoryx Therapeutics and Neuraxpharm Group, announce that the European Medicines Agency's (EMA) Committee for Medicinal Products for Human Use (CHMP) has recommended granting marketing authorisation approval under exceptional circumstances for NEZGLYAL® (leriglitazone) as a treatment for male cALD patients, aged 2-12 years with Gadolinium (Gd) negative brain lesions. European Commission approval is expected by the end of September 2026.

The opinion is based on results from the Phase 2/3 NEXUS[1] study and additional real-world evidence from compassionate use programmes. Cerebral adrenoleukodystrophy (cALD) is a debilitating disease characterised by demyelinating brain lesions that can progress rapidly, leading to acute neurological decline and death in three to four years. There are currently no approved pharmacological treatments for cALD in the EU.

"*The positive CHMP opinion is a regulatory validation, and we are very pleased that we will soon be able to provide a new therapeutic option to boys suffering from cALD. I would like to take the opportunity to thank physicians, patients, their families and the wider ALD community, we are*

enormously grateful for the support we received throughout these years," **said Marc Martinell, CEO, Minoryx.** *"The journey continues towards US approval and future European label-expansion, as we generate new data in adult male cALD patients with Gadolinium enhancing lesions from the ongoing CALYX* ^{2} *trial."*

"*cALD is a severe neurodegenerative disease and we look forward to bringing NEZGLYAL®, a long-awaited treatment, to European patients following EC approval later this year. This reflects Neuraxpharm's enduring commitment to delivering innovative solutions to CNS patients with significant unmet medical needs. Collaborations between Neuraxpharm and strategic partners like Minoryx throughout the clinical and regulatory process can deliver real-world impact for the CNS community. We remain committed to supporting Minoryx as we look ahead to obtaining European label-expansion in the future,*" **said Dr. Jörg Thomas Dierks, CEO, Neuraxpharm.**

Minoryx and Neuraxpharm entered into a license agreement under which Neuraxpharm will commercialise the product in Europe following marketing authorisation.

The development program continues, including the ongoing CALYX Phase 3 trial in adult male cALD patients with Gadolinium enhancing lesions and the TREE^{3} Phase 2a trial in pediatric patients with Rett syndrome, with read-outs expected by early 2028 and end of 2026 respectively.

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About the CHMP opinion
On 23 July 2026, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion recommending the granting of a marketing authorisation under exceptional circumstances for the medicinal product NEZGLYAL®, indicated 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.

It is expected that the European Commission will grant the Marketing Authorisation by the end of September 2026.

For more information, please visit Nezglyal | European Medicines Agency (EMA)

About leriglitazone (NEZGLYAL®)
It is Minoryx Therapeutics' orally bioavailable, brain penetrating, selective PPAR gamma agonist under development for CNS diseases. 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 clinical trial and adult cALD patients in the ADVANCE 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 Haematopoietic 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. In Europe, it is exclusively licensed to Neuraxpharm.

Leriglitazone has received a positive opinion under exceptional circumstances from the CHMP 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 pharmacological treatment available for cALD. 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), including both psychiatric and neurological disorders, 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] CALYX, a phase 3, multicentre, randomised (1:1), double-blind, placebo-controlled, clinical study, designed to compare the efficacy and safety of leriglitazone in male adult patients with progressive cALD. https://clinicaltrials.gov/study/NCT05819866

[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