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European Commission grants marketing authorisation for NEZGLYAL® (leriglitazone), the first pharmacological treatment approved for cerebral Adrenoleukodystrophy (cALD), a rare neurodegenerative disease

Approval is based on NEXUS data and follows positive CHMP opinion in July; first European launch is expected by end of 2026

BARCELONA, Spain and DÜSSELDORF, Germany, Sept. 25, 2026 (GLOBE NEWSWIRE) -- Neuraxpharm Group and Minoryx Therapeutics today announce that the European Commission (EC) has granted marketing authorisation under exceptional circumstances for NEZGLYAL® (leriglitazone), an orally bioavailable, brain penetrating, selective PPAR gamma agonist, as a treatment for male cALD patients, aged 2-12 years with Gadolinium (Gd)-negative brain lesions.

The therapy is the first approved pharmacological treatment for cALD in the European Union (EU). The approval is based on results from the Phase 2/3 NEXUS[1] study and additional real-world evidence from compassionate use programmes.

The first European launch is expected in Germany by the end of the year, with additional launches in Europe anticipated pending completion of national reimbursement negotiations. Neuraxpharm

is also evaluating appropriate access pathways for eligible patients.

"*With childhood cALD, neurodegeneration is irreversible, so it is critical to halt disease progression early, ideally, before symptoms surface and signs of neuroinflammation appear. Until now, there were no pharmacological treatment options for early intervention. Invasive procedures, such as hematopoietic stem cell transplantation, are available for more progressed patients. However, they are donor-dependent and can only be applied within a very narrow time window*," **said Dr. Caroline Sevin, MD, PhD, of CRMR LeukoFrance, Hôpital du Kremlin Bicêtre, France:** "*That we now have a pharmacological treatment for early intervention is a major advance in our treatment of cALD.*"

"*cALD is a rapidly progressing neurodegenerative disease which severely impacts the lives of patients and their families, underlining the critical need for treatments which can halt or slow disease progression and improve quality of life,*" **said Dr. Jörg Thomas Dierks, CEO of Neuraxpharm**. *"Today's announcement reinforces our commitment at Neuraxpharm to advancing innovative medicines that target CNS diseases with significant unmet clinical need. We're looking forward to working with the local authorities, and the medical and patient communities to bring NEZGLYAL® to eligible individuals across the region as quickly as possible."*

"*This approval represents a significant milestone for the cALD community and recognises years of breakthrough research and collaboration between clinicians and patient organisations. We are very grateful for their continued support,*" **said Marc Martinell, CEO of Minoryx.** "*Our development efforts continue as we generate more data towards expanding the label within X-ALD and other orphan indications.*"

Cerebral adrenoleukodystrophy (cALD) is a debilitating neurodegenerative disease characterised by demyelinating brain lesions that can progress rapidly, leading to acute neurological decline and death in three to four years. It predominantly affects the brain and is an aggressive form of X-linked adrenoleukodystrophy (X-ALD), which has an incidence of approximately 6-8/100,000 live births.

NEZGLYAL® (leriglitazone) is an oral medicine taken daily and offers a non-invasive yet disease-modifying cALD treatment option for Gd-negative children.

The EC approval is valid across all 27 European Union (EU) Member States, as well as Norway, Iceland and Liechtenstein.

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

The development programme continues, with enrolment now completed in the CALYX[2] Phase 3 trial in adult male cALD patients with Gd-enhancing lesions, and the ongoing TREE[3] Phase 2a trial in paediatric patients with Rett syndrome. Read-outs are expected in early 2028 and by the end of 2026, respectively.

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About the European Commission (EC) approval
On 21 September 2026, the European Commission (EC) granted marketing authorisation for NEZGLYAL® (leriglitazone). 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.

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

About NEZGLYAL® (leriglitazone)
Leriglitazone is an orally bioavailable, brain penetrating, selective PPAR gamma agonist developed by Minoryx and approved as the first pharmacological treatment for cALD in the European Union for male cALD patients, aged 2-12 years with Gadolinium (Gd)-negative brain lesions in brain Magnetic Resonance Imaging (MRI), with a Neurological Functional Score (NFS) of 0 or 1. 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[4] 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. Detailed recommendations for the use of NEZGLYAL® are described in the Summary of Product Characteristics (SmPC), which is available on the EMA website in all official European Union languages.

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.

cALD with Gd-positive lesions 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 cALD with Gd-positive lesions over time. Leriglitazone is the first EU approved pharmacological treatment 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, has been approved in the European Union to treat cerebral Adrenoleukodystrophy (cALD) in male patients, aged 2-12 years with Gadolinium (Gd)-negative brain lesions. It is also being developed to treat other orphan CNS diseases like Rett Syndrome. 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 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.

References
[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 assess the efficacy and safety of leriglitazone in male adult cALD patients with Gd-positive lesions.
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

[4]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.
https://www.thelancet.com/journals/laneur/article/PIIS1474-4422(22)00495-1/abstract