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ASIA EDITION

European Commission grants marketing authorisation for NEZGLYAL, first pharmacological treatment for cALD

September 28, 2026 | News

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

Neuraxpharm Group and Minoryx Therapeutics have announced that the European Commission 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. The approval is based on results from the Phase 2/3 NEXUS 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 per 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 Member States, as well as Norway, Iceland and Liechtenstein.

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

The development programme continues, with enrolment now completed in the CALYX Phase 3 trial in adult male cALD patients with Gd-enhancing lesions, and the ongoing TREE Phase 2a trial in paediatric patients with Rett syndrome.

Read-outs are expected in early 2028 and by the end of 2026, respectively.