23 July 2026
EMADOC-1829012207-57936
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (initial authorisation)

Nezglyal
lericlitazone

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[2] for the medicinal product Nezglyal[3], intended for the treatment of cerebral adrenoleukodystrophy (cALD).

The applicant for this medicinal product is Minoryx Therapeutics S.L.

Nezglyal will be available as oral suspension (13.66 mg/mL). The active substance of Nezglyal is leriglitazone (ATC code: A16AX23). Leriglitazone is an active metabolite of pioglitazone, and a selective peroxisome proliferator-activated receptor gamma (PPARγ) agonist that crosses the blood-brain barrier (BBB). Lerigltazone has been reported to have an antioxidant and neuroprotective activity in the central nervous system by modulating critical pathways involved in cALD progression and consequently decreasing neuroinflammation, enhancing BBB integrity, promoting myelination and improving mitochondrial function.

The benefits of Nezglyal were shown in an open-label study which measured the effects of leriglitazone on disease progression prior to haematopoietic stem cell transplantation (HSCT), in boys aged 2 to 12 years old with cALD.

Of the 20 boys who could be evaluated, seven (35%) showed no meaningful worsening of their condition (clinically and radiologically arrested disease) after receiving treatment for up to 96 weeks (or until they underwent transplantation). Of the nine boys who presented with gadolinium-negative cerebral lesions at baseline, six showed stabilisation (6/9 [66.7%]), while of the 11 boys who presented with gadolinium-positive cerebral lesions, one showed stabilisation (1/11 [9.1%.]).

This proportion was higher than what would normally be expected if the disease was left to progress naturally.

[1] Summaries of positive opinion are published without prejudice to the Commission decision, which will normally be issued 67 days from adoption of the opinion
[2] In exceptional circumstances, an authorisation may be granted subject to certain specific obligations, to be reviewed annually. This happens when the applicant can show that they are unable to provide comprehensive data on the efficacy and safety of the medicinal product, due to the rarity of the condition it is intended for, limited scientific knowledge in the area concerned, or ethical considerations involved in the collection of such data.
[3] This product was designated as an orphan medicine during its development. EMA will now review the information available to date to determine if the orphan designation can be maintained

Official address Domenico Scarlattilaan 6 ● 1083 HS Amsterdam ● The Netherlands
Address for visits and deliveries Refer to www.ema.europa.eu/how-to-find-us
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The most common side effects in children (2 to 12 years of age) were weight increase, eyelid oedema, leukopenia and neutropenia.

The full indication is:

Nezglyal is 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.

Nezglyal should be initiated and monitored by a physician with experience in the management of neurodegenerative diseases.

Detailed recommendations for the use of this product will be described in the summary of product characteristics (SmPC), which will be published on the EMA website in all official European Union languages after the marketing authorisation has been granted by the European Commission.