13 November 2025 Rev. 2[1]
EMA/114031/2025
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[2] (initial authorisation)

Aqneursa

levacetylleucine

On 24 July 2025, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation for the medicinal product Aqneursa[3], intended for the treatment of neurological manifestations of Niemann-Pick Type C (NPC) disease in adults and children from 6 years of age weighing at least 20 kg. The CHMP also concluded that the active substance, levacetylleucine, could not be considered a new active substance; this conclusion was confirmed on 13 November 2025 after re-examination of the initial opinion.

The applicant for this medicinal product is IntraBio Ireland Ltd.

Aqneursa will be available as 1 g granules for oral suspension. The active substance of Aqneursa is levacetylleucine, a modified form of the amino acid leucine (ATC code: 'not yet assigned') that targets underlying processes of neurological dysfunction. While the mechanism of action of levacetylleucine is not yet fully understood, non-clinical studies have demonstrated that it corrects energy metabolism, including improved production of adenosine triphosphate, the main source of energy for cerebellar tissues and cells.

The benefits of Aqneursa are an improvement in neurological signs, symptoms and functioning in patients with NPC disease after 12 weeks of treatment, as measured using the scale for the assessment and rating of ataxia (SARA), compared with placebo, as observed in a randomised, double-blind, placebo-controlled, 2-period crossover phase 3 study.

The most common side effect with Aqneursa is flatulence.

The full indication is:

> Aqneursa is indicated for the treatment of neurological manifestations of Niemann-Pick type C (NPC) disease, in combination with miglustat, or as a monotherapy in patients where miglustat

[1] This document was updated on 13 November 2025 to include the outcome of the re-examination and to remove the previous text announcing the request for re-examination
[2] Summaries of positive opinion are published without prejudice to the Commission decision, which will normally be issued 67 days from adoption of the opinion
[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

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is not tolerated, in adults and children aged 6 years and older and weighing at least 20 kg.

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.