Generic Name: levacetylleucine

Therapeutic Class or Brand Name: Aqneursa

Applicable Drugs: N/A

Preferred: N/A

Non-preferred: N/A

Date of Origin: 2/24/2025

Date Last Reviewed / Revised: N/A

PRIOR AUTHORIZATION CRITERIA

(May be considered medically necessary when criteria I through IV are met)

I. Diagnosis of Niemann-Pick disease Type C (NPC) and following criteria A through D are met:
   A. At least ONE of the following
      1. Documentation of genetically confirmed mutations in both alleles of *NPC1* or *NPC2*
      2. Mutation in only one allele of *NPC1* or *NPC2 AND* either positive filipin staining or elevated cholestane-triol level (>2 X upper limit of normal)
   B. Has at least one neurological symptom (eg, hearing loss, vertical supranuclear gaze palsy, ataxia, dementia, dystonia, seizures, dysarthria, hypotonia, clumsiness, delayed developmental milestones, gelastic cataplexy or dysphagia).
   C. Patient weight is ≥ 15 kg
   D. Ambulates independently or with assistance

II. The medication is prescribed by or in consultation with geneticist, endocrinologist, metabolic disorder subspecialist, neurologist, neuromuscular specialist, or a physician who specializes in the treatment of Niemann-Pick disease type C

III. Request is for a medication with the appropriate FDA labeling, or its use is supported by current clinical practice guidelines.

IV. Refer to plan document for the list of preferred products. If requested agent is not listed as a preferred product, must have a documented failure, intolerance, or contraindication to a preferred product(s).

EXCLUSION CRITERIA

- Not to be given in combination with arimoclomol (Miplyffa)
- Avoid in those taking N-acetyl-DL-leucine and N-acetyl-D-leucine due to decreased efficacy

OTHER CRITERIA

- Dose may not exceed 4 grams per day
- Females of reproductive potential must verify negative pregnancy test prior to approval

Quantity / Days Supply Restrictions

- 15 kg to <25 kg: 1 gram twice a day orally
- 25 kg to <35 kg: 1 gram three times a day orally
- 35 kg or more: 2 g in morning, 1 gram in afternoon, and 1 gram in evening orally
- One packet contains equivalent of 1 gram of levacetylleucine
- Each carton contains 28 unit-dose packets
- Max supply: 112 packets (4 cartons) per 28 day supply

Approval Length

- **Authorization:** 6 months
- **Re-Authorization:** An updated letter of medical necessity or progress notes confirming the current medical necessity criteria are met and showing the medication is effective. Document tolerating therapy and response of disease stabilization, slowed progression (slow decline in ambulation, speech, swallow, stance), or improvement. Needs documentation patient is not pregnant if female is of reproductive potential.

Appendix

Click or tap here to enter text.

References

1. Aqneursa. Prescribing information. IntraBio Inc.; 2024. Accessed February 24, 2025. https://www.accessdata.fda.gov/drugsatfda_docs/label/2024/219132s000lbl.pdf.
2. Mengel E, Klünemann HH, Lourenço CM, Hendriksz CJ, Sedel F, Walterfang M, Kolb SA. Niemann-Pick disease type C symptomatology: an expert-based clinical description. Orphanet J Rare Dis. 2013;8:166. doi: 10.1186/1750-1172-8-166.
3. Bremova-Ertl T, Ramaswami U, Brands M, et al. Trial of *N*-Acetyl-l-Leucine in Niemann-Pick Disease Type C. N Engl J Med. 2024;390(5):421-431. doi: 10.1056/NEJMoa2310151.