

## PHARMACY POLICY STATEMENT

### HAP CareSource™ Marketplace

<b>DRUG NAME</b>	<b>Aqneursa (levacetylleucine)</b>
<b>BENEFIT TYPE</b>	Pharmacy
<b>STATUS</b>	Prior Authorization Required

Aqneursa, approved by the FDA in 2024, is a modified amino acid indicated for the treatment of neurological manifestations of Niemann-Pick disease type C (NPC) in adults and pediatric patients weighing  $\geq 15$  kg. It enters pathways that correct metabolic dysfunction and improve energy production to ameliorate lysosomal dysfunction and reduce storage of unesterified cholesterol and sphingolipids.

NPC is a rare neurovisceral lysosomal storage disease with manifestations that vary depending on the age of onset of neurological manifestations, from early infantile (visceral-neurodegenerative form) to adult (psychiatric-neurodegenerative form). It is caused by mutations in the *NPC1* or *NPC2* gene resulting in tissue accumulation of multiple lipids due to abnormal cellular trafficking by the involved proteins (NPC1 and 2).

Aqneursa (levacetylleucine) will be considered for coverage when the following criteria are met:

#### Niemann-Pick disease type C (NPC)

For **initial** authorization:

- Member weighs at least 15 kg; AND
- Medication must be prescribed by or in consultation with a neurologist, metabolic specialist, or geneticist; AND
- Member has a diagnosis of NPC confirmed by one of the following:
  - Genetic testing that shows mutations in both alleles of the *NPC1* or *NPC2* gene, or
  - Mutation in one allele of *NPC1* or *NPC2*, AND either elevated plasma biomarkers (i.e., cholestane-triol or trihydroxy-cholanoyl-glycine and/or lysoSM-509 with normal or slightly elevated lysoSM) OR positive filipin testing; AND
- Member has at least mild disease-related neurological symptoms, but not severe; AND
- If of reproductive potential, prescriber attests the member is not pregnant; AND
- Aqneursa is not being prescribed in combination with Miplyffa.
- Dosage allowed/Quantity limit:** Granules for oral suspension. Dosage based on actual body weight, to be administered up to 3 times daily. 1 packet = 1 gram

Patient Body Weight	Morning Dose	Afternoon Dose	Evening Dose
15 to <25 kg	1 g	No Dose	1 g
25 to <35 kg	1 g	1 g	1 g
35 kg or more	2 g	1 g	1 g

QL: 112 packets per 28 days



***If all the above requirements are met, the medication will be approved for 6 months.***

For **reauthorization**:

1. Chart notes must document improvement or stabilization of neurological signs and symptoms compared to baseline.

***If all the above requirements are met, the medication will be approved for an additional 12 months.***

**HAP CareSource considers Aqneursa (levacetylleucine) not medically necessary for the treatment of conditions that are not listed in this document. For any other indication, please refer to the Off-Label policy.**

DATE	ACTION/DESCRIPTION
10/21/2024	New policy for Aqneursa created.

References:

1. Aqneursa [prescribing information]. IntraBio Inc.; 2024.
2. 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
3. Geberhiwot T, Moro A, Dardis A, et al. Consensus clinical management guidelines for Niemann-Pick disease type C. *Orphanet J Rare Dis*. 2018;13(1):50. Published 2018 Apr 6. doi:10.1186/s13023-018-0785-7
4. Patterson MC, Clayton P, Gissen P, et al. Recommendations for the detection and diagnosis of Niemann-Pick disease type C: An update. *Neurol Clin Pract*. 2017;7(6):499-511. doi:10.1212/CPJ.0000000000000399

Effective date: 04/01/2025

Revised date: 10/21/2024