UnitedHealthcare Pharmacy
Clinical Pharmacy Programs

Program Number	2025 P 2355-3
Program	Prior Authorization/Medical Necessity
Medication	Aqneursa™ (levacetylleucine)
P&T Approval Date	11/2024, 1/2025, 3/2025
Effective Date	6/1/2025

1. Background

Aqneursa (glycerol phenylbutyrate) is indicated for the treatment of neurological manifestations of Niemann-Pick disease type C (NPC) in adults and pediatric patients weighing ≥15 kg.

2. Coverage Criteria[a]:

A. Initial Authorization

1. **Aqneursa** will be approved based on **all** of the following criteria:

   a. **Both** of the following:

      (1) Diagnosis of Niemann-Pick disease type C (NPC)

      -AND-

      (2) Diagnosis has been genetically confirmed by mutation analysis of NPC1 and NPC2 genes

      -AND-

   b. Aqneursa is being used to treat neurological manifestations of NPC

      -AND-

   c. **One** of the following:

      (1) Aqneursa is prescribed in combination with miglustat

      -OR-

      (2) History of failure, contraindication, or intolerance to miglustat

      -AND-

   d. Patient is not receiving Aqneursa in combination with Miplyffa (arimoclomol)

      -AND-

   e. Aqneursa is prescribed by or in consultation with a provider with expertise in the

treatment of NPC

Authorization will be issued for 12 months.

B. <u>Reauthorization</u>

1. **Aqneursa** will be approved based on **<u>all</u>** of the following criteria:

   a. Documentation of positive clinical response to Aqneursa therapy (e.g., slowed disease progression from baseline based on assessment with NPC–specific scales)

   -AND-

   b. **<u>One</u>** of the following:

      (1) Aqneursa is prescribed in combination with miglustat

      -OR-

      (2) History of failure, contraindication, or intolerance to miglustat

   -AND-

   c. Patient is not receiving Aqneursa in combination with Miplyffa (arimoclomol)

   -AND-

   d. Aqneursa is prescribed by or in consultation with a provider with expertise in the treatment of NPC

   Authorization will be issued for 12 months.

[a] State mandates may apply. Any federal regulatory requirements and the member specific benefit plan coverage may also impact coverage criteria. Other policies and utilization management programs may apply.

3. Additional Clinical Rules:

- Notwithstanding Coverage Criteria, UnitedHealthcare may approve initial and re-authorization based solely on previous claim/medication history, diagnosis codes (ICD-10) and/or claim logic. Use of automated approval and re-approval processes varies by program and/or therapeutic class.
- Supply limits may be in place.

4. References:

1. Aqneursa [package insert]. Austin TX: IntraBio Inc.; September 2024.
2. 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

Program	Prior Authorization/Medical Necessity - Aqneursa (levacetylleucine)
Change Control	
11/2024	New program.
1/2025	Added criteria that Aqneursa not taken in combination with Miplyffa.
3/2025	Added criteria that Aqneursa taken in combination with miglustat or history of failure, contraindication, or intolerance to miglustat.