



AETNA BETTER HEALTH®
Coverage Policy/Guideline

Name: Aqneursa

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Effective Date: 1/29/2025

Last Review Date: 12/6/2024

Applies to: Illinois
 Florida Kids

New Jersey
 Pennsylvania Kids

Maryland
 Virginia

Intent:

The intent of this policy/guideline is to provide information to the prescribing practitioner outlining the coverage criteria for Aqneursa under the patient's prescription drug benefit.

Description:

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

All other indications are considered experimental/investigational and not medically necessary.

Applicable Drug List:

Aqneursa

Policy/Guideline:

Documentation

Submission of the following information is necessary to initiate the prior authorization review:
Niemann-Pick Disease Type C¹

Initial requests:

- Genetic or molecular test results confirming the diagnosis.
- Medical records (e.g., chart notes) documenting neurological manifestations of disease.
- Medical records (e.g., chart notes) of the baseline assessment for the 5-domain NPC clinical severity scale (NPCCSS) to establish baseline score.

Continuation requests:

Chart notes or medical record documentation supporting positive clinical response (e.g., stabilization or improvement in 5-domain NPCCSS score, fine motor skills, swallowing, speech, ambulation).

Prescriber Specialties

This medication must be prescribed by or in consultation with an endocrinologist or physician who specializes in the treatment of metabolic disease and/or lysosomal storage disorders.

Initial Coverage Criteria

Niemann-Pick Disease Type C¹

Authorization of 12 months may be granted for treatment of Niemann-Pick disease, type C when ALL the following criteria are met:



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- Member weighs ≥ 15 kg.
- Member is 4 years of age to 64 years of age.
- Member has completed the NPC clinical severity scale (NPCCSS) assessment to establish baseline score.
- The diagnosis is confirmed by either of the following:
 - Genetically confirmed variant in both alleles of NPC1 or NPC2.
 - Mutation in only one allele of NPC1 or NPC2 plus either positive filipin staining or elevated cholestane-triol level (>2 times the upper limit of normal).
- Member has neurological manifestations of disease (e.g., loss of fine motor skills, swallowing, speech, ambulation).
- The requested medication will not be used in combination with Miplyffa (arimoclomol) for the treatment of neurological manifestations of Niemann-Pick disease type C.

Continuation of Therapy

Authorization of 12 months may be granted for continued treatment in members requesting reauthorization for an indication listed in the coverage criteria section when ALL the following criteria are met:

- Member meets the criteria for initial approval.
- Member is experiencing benefit from therapy (e.g., stabilization or improvement in 5-domain NPCCSS score, fine motor skills, swallowing, speech, ambulation).

Approval Duration and Quantity Restrictions:

Approval: 12 months

Quantity Level Limit: 112 packets per 28 days

References:

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