BlueCross BlueShield
Federal Employee Program
Blue Cross Blue Shield Association
750 9th St NW, Suite 900
Washington, D.C. 20001
1-800-624-5060
Fax 1-877-378-4727

5.60.065

Section:	Prescription Drugs	**Effective Date:**	April 1, 2026
Subsection:	Central Nervous System Drugs	**Original Policy Date:**	October 11, 2024
Subject:	Aqneursa	**Page:**	1 of 4

Last Review Date: March 6, 2026

Aqneursa

Description

Aqneursa (levacetylleucine)

Background
Niemann-Pick disease Type C (NPC) is a rare progressive genetic disorder characterized by an inability of the body to transport cholesterol and other fatty substances inside of cells. This leads to the abnormal accumulation of these substances within various tissues of the body, including brain tissue, and can damage the affected area, which may lead to neurological manifestations. Neurological manifestations include seizures, dysphagia, cataplexy, dystonia, tremors, sleep disturbances, and psychiatric conditions (depression, obsessive compulsive disorder, bipolar disorder, hallucinations). Most cases of NPC are detected during childhood and progress to cause life-threatening complications by the second or third decade of life. NPC is caused by mutations in the NPC1 gene (NPC type 1C) or the NPC2 gene (NPC type 2C) and is inherited in the autosomal recessive manner (1).

Aqneursa inhibits P-glycoprotein however, the clinical significance of this finding has not been fully characterized. The distinct molecular target for Aqneursa in the treatment of NPC is unknown (2).

Regulatory Status
FDA-approved indication: Aqneursa is indicated for the treatment of neurological manifestations of Niemann-Pick disease type C (NPC) in adults and pediatric patients weighing ≥15 kg (2).

Aqneursa may cause embryo-fetal toxicity. For females of reproductive potential, verify that the patient is not pregnant prior to initiating treatment with Aqneursa. Females of reproductive potential should be advised to use effective contraception during treatment with Aqneursa and for 7 days after the last dose if Aqneursa is discontinued (2).

The safety and effectiveness of Aqneursa in pediatric patients weighing less than 15 kg have not been established (2).

Related policies

Miplyffa

Policy

This policy statement applies to clinical review performed for pre-service (Prior Approval, Precertification, Advanced Benefit Determination, etc.) and/or post-service claims.

Aqneursa may be considered **medically necessary** if the conditions indicated below are met.

Aqneursa may be considered **investigational** for all other indications.

Prior-Approval Requirements

Diagnosis

Patient must have the following:

Niemann-Pick disease type C (NPC)

AND ALL the following:

1. NPC diagnosis confirmed by genetic testing identifying disease-causing variants in the NPC1 or NPC2 genes
2. Aqneursa is being used for the neurological manifestations of NPC
3. Weight ≥ 15 kg
4. Females of reproductive potential **only**: pregnancy will be excluded before initiating treatment with Aqneursa, and patient will be advised to use effective contraception during treatment with Aqneursa and for 1 week after the last dose

Prior–Approval *Renewal* Requirements

Diagnosis

Patient must have the following:

Niemann-Pick disease type C (NPC)

AND ALL of the following:

1. Weight ≥ 15 kg
2. Neurological manifestations have improved or stabilized
3. Females of reproductive potential **only**: patient will be advised to use effective contraception during treatment with Aqneursa and for 1 week after the last dose

Policy Guidelines

Pre - PA Allowance

None

Prior - Approval Limits

Quantity 336 packets for oral suspension every 84 days

Duration 2 years

Prior–Approval *Renewal* Limits

Same as above

Rationale

Summary

Aqneursa is indicated for the treatment of neurological manifestations of Niemann-Pick disease type C (NPC). Aqneursa contains a warning regarding embryo-fetal toxicity. The safety and effectiveness of Aqneursa in pediatric patients weighing less than 15 kg have not been established (2).

Prior approval is required to ensure the safe, clinically appropriate, and cost-effective use of Aqneursa while maintaining optimal therapeutic outcomes.

References

1. Niemann Pick Disease Type C: National Organization for Rare Disorders. December 12, 2023. https://rarediseases.org/rare-diseases/niemann-pick-disease-type-c/.
2. Aqneursa [package insert]. Austin, TX: IntraBio Inc.; September 2024.

Policy History

Date	Action
October 2024	Addition to PA
March 2025	Annual review
March 2026	Annual review

Keywords

This policy was approved by the FEP® Pharmacy and Medical Policy Committee on March 6, 2026 and is effective on April 1, 2026.