BlueCross BlueShield
Federal Employee Program

AQNEURSA
(levacetylleucine)

RATIONALE FOR INCLUSION IN PA PROGRAM

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).

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.