BlueCross BlueShield Federal Employee Program

MIPLYFFA
(arimoclomol)

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).

The mechanism by which Miplyffa exerts its clinical effects in patients with NPC is unknown. Miplyffa is an inhibitor of organic cationic transporter 2 (OCT2) (2).

Regulatory Status

FDA-approved indication: Miplyffa is indicated for use in combination with miglustat for the treatment of neurological manifestations of Niemann-Pick disease type C (NPC) in adult and pediatric patients 2 years of age and older (2).

Miplyffa contains warnings regarding hypersensitivity reactions and increased creatinine without affected glomerular function (2).

Miplyffa may cause embryofetal toxicity. Pregnant females should be advised of the potential risk to the fetus. Pregnancy planning and prevention should be considered for females of reproductive potential (2).

The safety and effectiveness of Miplyffa in pediatric patients less than 2 years of age have not been established (2).

Summary

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Miplyffa is indicated for use in combination with miglustat for the treatment of neurological manifestations of Niemann-Pick disease type C (NPC). Miplyffa contains warnings regarding hypersensitivity reactions, embryofetal toxicity, and increased creatinine. The safety and effectiveness of Miplyffa in pediatric patients less than 2 years of age have not been established (2).

Prior approval is required to ensure the safe, clinically appropriate, and cost-effective use of Miplyffa 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. Miplyffa [package insert]. Celebration, FL: Zevra Therapeutics, Inc.; September 2024.