Generic Name: Arimoclomol

Therapeutic Class or Brand Name: Miplyffa

Applicable Drugs: N/A

Preferred: N/A

Non-preferred: N/A

Date of Origin: 2/24/2025

Date Last Reviewed / Revised: 3/24/2025

PRIOR AUTHORIZATION CRITERIA

(May be considered medically necessary when criteria I through VII are met)

I. Diagnosis of Niemann-Pick disease Type C (NPC) and the following criteria are met:
   A. At least ONE of the following
      1. Documentation of genetically confirmed mutations in both alleles of *NPC1* or *NPC2*
      2. Mutation in only one allele of *NPC1* or *NPC2 AND* either positive filipin staining or elevated cholestane-triol level (>2 X upper limit of normal)

II. Must be given in combination with miglustat

III. Has at least one neurological symptom (eg, hearing loss, vertical supranuclear gaze palsy, ataxia, dementia, dystonia, seizures, dysarthria, hypotonia, clumsiness, delayed developmental milestones, gelastic cataplexy or dysphagia).

IV. Minimum age requirement: 2 years of age

V. The medication is prescribed by or in consultation with geneticist, endocrinologist, metabolic disorder subspecialist, neurologist, neuromuscular specialist, or a physician who specializes in the treatment of Niemann-Pick disease type C

VI. Request is for a medication with the appropriate FDA labeling, or its use is supported by current clinical practice guidelines.

VII. Refer to plan document for the list of preferred products. If requested agent is not listed as a preferred product, must have a documented failure, intolerance, or contraindication to a preferred product(s).

EXCLUSION CRITERIA

- Severe epilepsy with seizures

OTHER CRITERIA

- Consider pregnancy planning and prevention for females of reproductive potential

QUANTITY / DAYS SUPPLY RESTRICTIONS

- Strengths available include 47 mg, 62 mg, 93 mg, 124 mg capsules

- Max 90 capsules per 30 day supply

Approval Length

- **Authorization:** 6 months
- **Re-Authorization:** An updated letter of medical necessity or progress notes confirming the current medical necessity criteria are met and showing the medication is effective. Document tolerating therapy and response of disease stabilization, slowed progression (slow decline in ambulation, speech, swallow, stance), or improvement.

Appendix

Click or tap here to enter text.

References

1. Miplyffa. Prescribing information. Zevra Therapeutics Inc. 2024. Accessed February 24, 2025. https://www.accessdata.fda.gov/drugsatfda_docs/label/2024/214927s000lbl.pdf
2. Mengel E, Patterson MC, Da Riol RM, et al. Efficacy and safety of arimoclomol in Niemann-Pick disease type C: Results from a double-blind, randomised, placebo-controlled, multinational phase 2/3 trial of a novel treatment. J Inherit Metab Dis. 2021;44(6): 1463-1480. doi:10.1002/jimd.12428
3. Mengel E, Klünemann HH, Lourenço CM, Hendriksz CJ, Sedel F, Walterfang M, Kolb SA. Niemann-Pick disease type C symptomatology: an expert-based clinical description. Orphanet J Rare Dis. 2013;8:166. doi: 10.1186/1750-1172-8-166.