Reference number(s)
6673-A

Specialty Guideline Management
Miplyffa (arimoclomol)

Products Referenced by this Document

Drugs that are listed in the following table include both brand and generic and all dosage forms and strengths unless otherwise stated. Over the counter (OTC) products are not included unless otherwise stated.

Brand Name	Generic Name
Miplyffa	arimoclomol

Indications

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-approved Indications[1]

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.

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

Documentation

Submission of the following information is necessary to initiate the prior authorization review:

Niemann-Pick Disease Type C[1]

Initial requests:

- Genetic or molecular test results confirming the diagnosis.

- Medical records (e.g., chart notes) documenting neurological manifestations of disease and ambulation status.
- 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.

Coverage Criteria

Niemann-Pick Disease Type C[1]

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

- Member is 2 to 19 years of age.
- Member has completed the NPC clinical severity scale (NPCCSS) assessment to establish baseline score.
- Member is ambulatory (able to walk independently or with assistance).
- 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 be used in combination with miglustat.
- The requested medication will not be used in combination with Aqneursa (levacetylleucine) 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 of 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).

References

1. Miplyffa [package insert]. Celebration, FL: Zevra Therapeutics, Inc.; September 2024.