

DRUG POLICY

Miplyffa™(arimoclomol)

BENEFIT APPLICATION

Benefit determinations are based on the applicable contract language in effect at the time the services were rendered. Exclusions, limitations or exceptions may apply. Benefits may vary based on contract, and individual member benefits must be verified. Wellmark determines medical necessity only if the benefit exists and no contract exclusions are applicable. This medical policy may not apply to FEP. Benefits are determined by the Federal Employee Program.

DESCRIPTION

The intent of the policy is to provide coverage consistent with product labeling, FDA guidance, standards of medical practice, evidence-based drug information, and/or published guidelines. The indications below including FDA-approved indications and compendial uses are considered covered benefits provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-Approved Indications

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.

POLICY

Required Documentation

Niemann-Pick Disease Type C

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

1. Initial requests
 - A. Genetic or molecular test results confirming the diagnosis.
 - B. Medical records (e.g., chart notes) documenting neurological manifestations of disease and ambulation status.
 - C. Medical records (e.g., chart notes) of the baseline assessment for the 5-domain NPC clinical severity scale (NPCCSS) to establish baseline score.
2. 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

The requested 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.

Criteria for Initial Approval

Niemann-Pick Disease Type C

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

1. Member is 2 to 19 years of age.
2. Member has completed the NPC clinical severity scale (NPCCSS) assessment to establish baseline score.
3. Member is ambulatory (able to walk independently or with assistance).
4. The diagnosis is confirmed by either of the following:
 - A. Genetically confirmed variant in both alleles of NPC1 or NPC2.
 - B. 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).
5. Member has neurological manifestations of disease (e.g., loss of fine motor skills, swallowing, speech, ambulation).
6. The requested medication will be used in combination with miglustat.
7. 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

Niemann-Pick Disease Type C

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

1. Member meets the criteria for initial approval.
2. Member is experiencing benefit from therapy (e.g., stabilization or improvement in 5-domain NPCCSS score, fine motor skills, swallowing, speech, ambulation).

Other

Miplyffa (arimoclomol) is considered **not medically necessary** for members who do not meet the criteria set forth above.

Members currently receiving the requested medication as samples or via the manufacturer's patient assistance program will be required to meet the criteria for initial approval. This ensures that members are treated equally regardless of their provider's ability to access medication samples.

Dosing and Administration

Approvals may be subject to dosing limits in accordance with FDA-approved labeling, accepted compendia, and/or evidence-based practice guidelines.

Quantity Limits Apply

Medication	Standard Limit	FDA Recommended Dosing
Miplyffa (arimoclomol) 47 mg capsule	90 capsules per 30 days	The recommended oral dosage of Miplyffa, in combination with miglustat, for patients with an actual body weight of: <ul style="list-style-type: none">• 8 kg to 15 kg, is 47 mg three times a day
Miplyffa (arimoclomol) 62 mg capsule		
Miplyffa (arimoclomol) 93 mg capsule		

Miplyffa (arimoclomol) 124 mg capsule		<ul style="list-style-type: none"> • 15 kg to 30 kg, is 62 mg three times a day • 30 kg to 55 kg, is 93 mg three times a day • > 55 kg, is 124 mg three times a day
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PROCEDURES AND BILLING CODES

To report provider services, use appropriate CPT* codes, Alpha Numeric (HCPCS level 2) codes, Revenue codes, and/or ICD diagnostic codes.

REFERENCES

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

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

Geberhiwot T, Moro A, Dardis A, Ramaswami U, et al; International Niemann-Pick Disease Registry (INPDR). Consensus clinical management guidelines for Niemann-Pick disease type C. *Orphanet J Rare Dis*. 2018;13(1):50. doi: 10.1186/s13023-018-0785-7

POLICY HISTORY

Policy #: 05.05.69

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Revised:

Current Effective Date: March 7, 2025