CareSource Rxinnovations

PHARMACY POLICY STATEMENT
Georgia Medicaid

DRUG NAME	Miplyffa (arimoclomol)
BENEFIT TYPE	Pharmacy
STATUS	Prior Authorization Required

Miplyffa, approved by the FDA in 2024, 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. It is a small molecule that amplifies the heat shock response to target NPC protein misfolding and improve lysosomal function.
NPC is a rare neurovisceral lysosomal storage disease with manifestations that vary depending on the age of onset of neurological manifestations, from early infantile (visceral-neurodegenerative form) to adult (psychiatric-neurodegenerative form). It is caused by mutations in the *NPC1* or *NPC2* gene resulting in tissue accumulation of multiple lipids due to abnormal cellular trafficking by the involved proteins (NPC1 and 2).

Miplyffa (arimoclomol) will be considered for coverage when the following criteria are met:

Niemann-Pick disease type C (NPC)

For **initial** authorization:

1. Member is at least 2 years of age; AND
2. Medication must be prescribed by or in consultation with a neurologist, metabolic specialist, or geneticist; AND
3. Member has a diagnosis of NPC confirmed by one of the following:
   a) Genetic testing that shows mutations in both alleles of the *NPC1* or *NPC2* gene, or
   b) Mutation in one allele of *NPC1* or *NPC2*, AND either elevated plasma biomarkers (i.e., cholestane-triol or trihydroxy-cholanoyl-glycine and/or lysoSM-509 with normal or slightly elevated lysoSM) OR positive filipin testing; AND
4. Member has at least 1 neurological sign of disease; AND
5. Member does NOT have advanced neurological disease; AND
6. Miplyffa will be prescribed in combination with miglustat (PA required); AND
7. Miplyffa will NOT be prescribed in combination with Aqneursa.
8. **Dosage allowed/Quantity limit:** 90 capsules per 30 days.
   8 kg to 15 kg: 47 mg three times a day
   >15 kg to 30 kg: 62 mg three times a day
   >30 kg to 55 kg: 93 mg three times a day
   >55 kg: 124 mg three times a day

If all the above requirements are met, the medication will be approved for 6 months.

For **reauthorization**:
1. Chart notes must document improvement or stabilization of neurological signs and symptoms compared to baseline.

If all the above requirements are met, the medication will be approved for an additional 12 months.

CareSource considers Miplyffa (arimoclomol) not medically necessary for the treatment of conditions that are not listed in this document. For any other indication, please refer to the Off-Label policy.

DATE	ACTION/DESCRIPTION
10/22/2024	New policy for Miplyffa created.

References:

1. Miplyffa [prescribing information]. Zevra Therapeutics, Inc.; 2024.
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. Geberhiwot T, Moro A, Dardis A, et al. Consensus clinical management guidelines for Niemann-Pick disease type C. *Orphanet J Rare Dis*. 2018;13(1):50. Published 2018 Apr 6. doi:10.1186/s13023-018-0785-7
4. Patterson MC, Clayton P, Gissen P, et al. Recommendations for the detection and diagnosis of Niemann-Pick disease type C: An update. *Neurol Clin Pract*. 2017;7(6):499-511. doi:10.1212/CPJ.0000000000000399
5. Patterson MC, Vecchio D, Prady H, Abel L, Wraith JE. Miglustat for treatment of Niemann-Pick C disease: a randomised controlled study. *Lancet Neurol*. 2007;6(9):765-772. doi:10.1016/S1474-4422(07)70194-1

Effective date: 02/01/2026
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