

Clinical Policy Title:	arimoclomol
Policy Number:	RxA.880
Drug(s) Applied:	Miplyffa
Original Policy Date:	4/10/2025
Last Review Date:	12/11/2025
Line of Business Policy Applies to:	All lines of business (except Medicare)

Criteria

I. Initial Approval Criteria

A. Niemann-Pick disease type C (must meet all):

1. Diagnosis of Niemann-Pick disease type C (NPC);
2. Member has one of the following mutations, confirmed by genetic testing (a or b):
 - a. Mutations in both alleles of NPC1 or NPC2;
 - b. Mutation in one allele AND either a positive filipin-staining or elevated cholestane triol/oxysterols (>2× ULN);
3. Documentation of neurological manifestation of the disease;
4. Member is not using Miplyffa in combination with Aqneursa;
5. Member is currently receiving miglustat and has been on a stable dose for 6 consecutive months;
6. Member can walk independently or with assistance.

Approval duration

All Lines of Business (except Medicare): 12 months

II. Continued Therapy Approval

1. Auto-approval based on lookback functionality within the past 120 days as a proxy for member responding positively to therapy with RxAdvance initial approval

Approval duration

All Lines of Business (except Medicare): 12 months

References

1. Mengel E, Patterson MC, Da Rioli 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 Inher Metab Dis.* 2021;44(6):1463-1480. doi:10.1002/jimd.12428

Review/Revision History	Review/Revised Date	P&T Approval Date
Policy established.	04/10/2025	04/10/2025
Policy reviewed	12/11/2025	12/11/2025

This clinical policy has been developed to authorize, modify, or determine coverage for individuals with similar conditions. Specific care and treatment may vary depending on individual need and benefits covered by the plan. This policy is not intended to dictate to providers how to practice medicine, nor does it constitute a contract or guarantee regarding payment or results. This document may contain prescription brand name drugs that are trademarks of pharmaceutical manufacturers that are not affiliated with RxAdvance.