

Medications for the Treatment Niemann-Pick Disease Type C (NPC):
Aqneursa (levacetylleucine)
Miplyffa (arimoclomol)
Effective 06/01/2025

Plan	<input type="checkbox"/> MassHealth UPPL <input checked="" type="checkbox"/> Commercial/Exchange	Program Type	<input checked="" type="checkbox"/> Prior Authorization
Benefit	<input checked="" type="checkbox"/> Pharmacy Benefit <input type="checkbox"/> Medical Benefit		<input type="checkbox"/> Quantity Limit <input type="checkbox"/> Step Therapy
Specialty Limitations	This medication has been designated specialty and must be filled at a contracted specialty pharmacy.		
Contact Information	Medical and Specialty Medications		
	All Plans	Phone: 877-519-1908	Fax: 855-540-3693
Contact Information	Non-Specialty Medications		
	All Plans	Phone: 800-711-4555	Fax: 844-403-1029
Exceptions	N/A		

Overview

Aqneursa (levacetylleucine) is indicated for the treatment of neurological manifestations of Niemann-Pick disease type C (NPC) in adults and pediatric patients weighing at least 15 kilograms.

Miplyffa (arimoclomol) is indicated for use in combination with miglustat for the treatment of neurological manifestations of NPC in adults and pediatric patients that are 2 years of age or older.

Coverage Guidelines

Authorization may be granted for members new to the plan within the past 90 days who are currently receiving treatment with the requested medication, excluding when the product is obtained as samples or via manufacturer's patient assistance programs

OR

Authorization may be granted when the following criteria are met:

1. Member has a diagnosis of Niemann-Pick disease type C (NPC)
2. Diagnosis of NPC is confirmed by at least ONE of the following:
 - a. Genetically confirmed by mutations in both alleles of NPC1 or NPC2
 - b. Mutation in only one allele of NPC1 or NPC2 plus either positive or filipin staining or elevated cholestane triol/oxysterols (>2 times the upper limit of normal [ULN])
3. Member has at least one neurological symptom of the disease (e.g., hearing loss, vertical supranuclear gaze palsy, ataxia, dementia, dystonia, seizures, dysarthria, or dysphagia)
4. Requested medication is prescribed by or in consultation with a specialist knowledgeable in the treatment of NPC
5. **Requests for Aqneursa:**
 - a. Member meets ALL of the following:
 - i. Member weighs at least 15 kilograms
 - ii. Requested medication will not be used with Miplyffa (arimoclomol)

6. Requests for Miplyffa:

- a. Member meets ALL of the following:
 - i. Member is 2 years of age or older
 - ii. Member's treatment regimen will include miglustat
 - iii. Requested medication will not be used with Aqneursa (levacetylleucine)

Continuation of Therapy

Reauthorization requests will be approved when the following criteria are met:

1. Member demonstrates a positive clinical response to therapy (e.g., slowing of disease progression, improvement in neurological symptoms of the disease)
2. **Requests for Aqneursa:**
 - a. Requested medication will not be used in combination with Miplyffa (arimoclomol)
3. **Requests for Miplyffa:**
 - a. Member meets ALL of the following:
 - i. Member's treatment regimen includes miglustat
 - ii. Requested medication will not be used in combination with Aqneursa (levacetylleucine)

Limitations

1. Initial requests will be approved for 6 months
2. Reauthorization requests will be approved for 12 months
3. The following quantity limitations apply:

Drug Name	Quantity Limitations
Aqneursa (levacetylleucine) packets	4 packets per day
Miplyffa (arimoclomol) capsule	3 capsules per day

References

1. Aqneursa (levacetylleucine) [prescribing information]. Austin, TX: IntraBio Inc.; September 2024.
2. Bremova-Ertl T, Claassen J, Foltan T, et al. Efficacy and safety of N-acetyl-L-leucine in Niemann-Pick disease type C. *J Neurol*. 2022;269(3):1651-1662. doi:10.1007/s00415-021-10717-0
3. Bremova-Ertl T, Ramaswami U, Brands M, et al. Trial of N-acetyl-L-leucine in Niemann-Pick disease type C. *N Engl J Med*. 2024;390(5):421-431. doi:10.1056/NEJMoa2310151
4. Bremova-Ertl T, Schneider S. Current advancements in therapy for Niemann-Pick disease: Progress and pitfalls. *Expert Opin Pharmacother*. 2023;24(11):1229-1247. doi:10.1080/14656566.2023.2215386
5. 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. doi:10.1186/s13023-018-0785-7
6. 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
7. Miplyffa (arimoclomol) [prescribing information]. Celebration, FL: Zevra Therapeutics, Inc; September 2024.
8. Mullard A. FDA approves first two drugs for rare Niemann-Pick disease. *Nat Rev Drug Discov*. Published online September 27, 2024. doi:10.1038/d41573-024-00162-9



9. Patterson MC, Hendriksz CJ, Walterfang M, et al. Recommendations for the diagnosis and management of Niemann-Pick disease type C: An update. *Mol Genet Metab.* 2012;106(3):330-344. doi:10.1016/j.ymgme.2012.03.012
10. Patterson MC, Lloyd-Price L, Guldberg C, et al. Validation of the 5-domain Niemann-Pick type C Clinical Severity Scale. *Orphanet J Rare Dis.* 2021;16(1):79. doi:10.1186/s13023-021-01719-2
11. Patterson M, Mengel E, Guenther S, i Dali C. Long-term efficacy and safety evaluation of arimoclomol treatment in patients with Niemann Pick Type C – Data from 48 months open label trial. Poster presented at: Society for the Study of Inborn Errors of Metabolism Annual Symposium; September 3-6, 2024; Porto, Portugal.
12. 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

Review History

03/12/2025 – Created and reviewed at March P&T.

