

New Hampshire Medicaid Fee-for-Service Program

Niemann-Pick Disease Type C Agents Criteria

Approval Date: June 5, 2025

Medications

Brand Names	Generic Names	Indication
Aqneursa™	levacetylleucine	• Treatment of neurological manifestations of Niemann-Pick disease type C (NPC) in adults and pediatric patients weighing greater than or equal to 15 kg
Miplyffa™	arimoclomol	• Treatment of neurological manifestations of NPC in combination with miglustat in adult and pediatric patients 2 years of age and older

Criteria for Approval

Aqneursa

1. Prescribed by or in consultation with a geneticist or a specialist in the area of the patient's diagnosis; **AND**
2. Patient has a diagnosis of Niemann-Pick disease Type C; **AND**
3. Genetic analysis confirms mutation in the NPC1 or NPC2 genes **AND**
4. The patient has disease-related neurological symptoms; **AND**
5. The patient weighs greater than or equal to 15 kg.

Initial approval period: 12 months

Criteria for Renewal

1. Patient has had clinical benefit with the requested drug; **AND**
2. Patient has not experienced any treatment-restricting adverse effects (e.g., abdominal pain, dysphagia, upper respiratory tract infections, and vomiting)

Renewal period: 12 months

Criteria for Denial

1. Failure to meet approval criteria.

Miplyffa

1. Prescribed by or in consultation with a geneticist or a specialist in the area of the patient's diagnosis; **AND**
2. Patient has a diagnosis of Niemann-Pick disease Type C; **AND**
3. Genetic analysis confirms mutation in the NPC1 or NPC2 genes **AND**
4. The patient has disease-related neurological symptoms; **AND**
5. Miplyffa will be used in combination with miglustat.

Initial approval period: 12 months

Criteria for Renewal

1. Patient has had clinical benefit with the requested drug; **AND**
2. Patient has not experienced any treatment-restricting adverse effects (e.g., abdominal pain, dysphagia, upper respiratory tract infections, and vomiting)

Renewal period: 12 months

Criteria for Denial

1. Failure to meet approval criteria.

References

Available upon request.

Revision History

Reviewed by	Reason for Review	Date Approved
DUR Board	New	04/08/2025
Commissioner designee	Approval	06/05/2025