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| AETNA BETTER HEALTH®<br>Coverage Policy/Guideline  |                          |
| Name: Miplyffa   | Page: 1 of 3             |
| Effective Date: 6/9/2025   | Last Review Date: 5/2025 |
| Applies to: <input checked="" type="checkbox"/> Illinois <input type="checkbox"/> Florida <input checked="" type="checkbox"/> Florida Kids <input checked="" type="checkbox"/> New Jersey <input checked="" type="checkbox"/> Maryland <input type="checkbox"/> Michigan <input checked="" type="checkbox"/> Pennsylvania Kids <input checked="" type="checkbox"/> Virginia <input type="checkbox"/> Kentucky PRMD |                          |

### Intent:

The intent of this policy/guideline is to provide information to the prescribing practitioner outlining the coverage criteria for Miplyffa under the patient's prescription drug benefit.

### Description:

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.

All other indications are considered experimental/investigational and not medically necessary.

### Applicable Drug List:

Miplyffa

### Policy/Guideline:

#### **Documentation**

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

Niemann-Pick Disease Type C<sup>1</sup>

#### Initial requests:

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

#### 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**

This 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.



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### **Initial Coverage Criteria**

#### **Niemann-Pick Disease Type C<sup>1</sup>**

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

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

**Authorization of 12 months may be granted for continued treatment in members requesting reauthorization for an indication listed in the coverage criteria section when ALL the following criteria are met:**

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

### **Approval Duration and Quantity Restrictions:**

**Approval:** 12 months

#### **Quantity Level Limit:**

- Miplyffa 47 mg capsules: 90 per 30 days
- Miplyffa 62 mg capsules: 90 per 30 days
- Miplyffa 93 mg capsules: 90 per 30 days
- Miplyffa 124 mg capsules: 90 per 30 days

### **References:**



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