Commercial PA Criteria

Effective: December 12, 2024

Prior Authorization: Miplyffa (arimoclomol)

Products Affected: Miplyffa (arimoclomol capsules)

Medication 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.

Covered Uses: Neurological manifestations of Niemann-Pick disease type C (NPC)

Exclusion Criteria: None

Required Medical Information:

1. Diagnosis
2. Medication History
3. Medical History

Prescriber Restriction: The medication is prescribed by, or in consultation with, a geneticist, endocrinologist, metabolic disorder subspecialist, neurologist, or a physician who specializes in the treatment of Niemann-Pick disease type C or related disorders

Age Restriction: Patient must be ≥ 2 years of age.

Coverage Duration: 12 months

Other Criteria:

1. Niemann-Pick disease type C. Approve if the patient meets ONE of the following (A **or** B):

A. Initial Therapy: Approve if the patient meets ALL of the following (i, ii, iii, iv, v, vi, **and** vii):

i. Patient is ≥ 2 years of age; **AND**

ii. Patient has one or more neurological symptom(s) of Niemann-Pick disease type C; **AND**
Note: Examples of neurologic symptoms of Niemann-Pick disease type C include loss of motor function, swallowing, and speech and cognitive impairment.

iii. Patient can walk independently or with assistance; **AND**

iv. The diagnosis is established by a genetic test showing biallelic pathogenic variants in either the NPC1 gene or NPC2 gene; **AND**

v. Patient does NOT have adult-onset Niemann-Pick disease type C; **AND**
Note: Adult-onset NPC is defined as the age of the first neurological symptom occurring > 15 years of age.

vi. The patient meets ONE of the following (a or b):

a. The medication will be taken in combination with miglustat; **OR**

b. According to the prescriber, patient is unable to take miglustat; **AND**

vii. The medication is prescribed by or in consultation with a geneticist, endocrinologist, metabolic disorder subspecialist, neurologist, or a physician who specializes in the treatment of Niemann-Pick disease type C or related disorders.

B. Patient is Currently Receiving Miplyffa. Approve if the patient meets ALL of the following (i, ii, iii, and iv):
   i. Patient does NOT have adult-onset Niemann-Pick disease type C; **AND**
   Note: Adult-onset Niemann-Pick disease type C is defined as the age of the first neurological symptom occurring > 15 years of age.
   ii. The patient meets ONE of the following (a or b):
      a. The medication will be taken in combination with miglustat; **OR**
      b. According to the prescriber, patient is unable to take miglustat; **AND**
   iii. According to the prescriber, patient has derived benefit from treatment defined as disease stabilization, slowed progression, or improvement; **AND**
   iv. The medication is prescribed by or in consultation with a geneticist, endocrinologist, metabolic disorder subspecialist, neurologist, or a physician who specializes in the treatment of Niemann-Pick disease type C or related disorders.

References:

1. Miplyffa™ capsules [prescribing information]. Frederiksberg, Denmark/Celebration, FL: Zevra Denmark A/S/Zevra Therapeutics; September 2024.

Policy Revision history

Rev #	Type of Change	Summary of Change	Sections Affected	Date
1	New Policy	New Policy	All	12/12/2024