Medical Policy:

Amondys 45™ (casimersen) intravenous infusion

POLICY NUMBER	LAST REVIEW	ORIGIN DATE
MG.MM.PH.331	April 22, 2024	June 9, 2021

Definitions

Amondys 45 is an antisense oligonucleotide designed to bind to exon 45 of dystrophin pre-mRNA, resulting in exclusion of this exon during mRNA processing in patients with genetic mutations that are amenable to exon 45 skipping. These patients represent up to 8% of all patients with DMD. This genetic manipulation intends to restore the reading frame of the resulting mRNA. The result would be production of a shortened, but partially functional dystrophin protein as seen in less severe forms of muscular dystrophy (e.g., Becker muscular dystrophy).

Length of Authorization

Coverage will be provided for 6 months and may be renewed.

Dosing Limits [Medical Benefit]

The recommended dosage is 30mg/kg as an intravenous infusion over approximately 35 to 60 minutes once weekly.

Max Units (per dose and over time) [HCPCS Unit]:

• 350 billable units (3500 mg) every 7 days

Guideline

I. INITIAL APPROVAL CRITERIA

Coverage will be provided when the following criteria are met:

1. Duchenne muscular dystrophy (DMD)

A. Patient has a diagnosis of Duchenne Muscular Dystrophy (DMD) confirmed by genetic testing; **AND**
B. Specific type of DMD gene mutation which amenable to exon 45 skipping has been confirmed; **AND**
C. Patient retains meaningful voluntary motor function (e.g., patient is able to speak, manipulate objects using upper extremities, ambulate, etc.); **AND**
D. Patient is receiving physical and/or occupational therapy; **AND**
E. Baseline documentation of **ONE or more** of the following:
   i. Dystrophin level
   ii. Timed function tests (e.g., 6-minute walk test [6MWT], time to stand [TTSTAND], time to run/walk 10 meters [TTRW], time to climb 4 stairs [TTCLIMB], etc.)
   iii. Upper limb function (ULM) test
   iv. North Star Ambulatory Assessment (NSAA) score
   v. Forced Vital Capacity (FVC) percent predicted
F. Patient has been on a stable dose of corticosteroids for at least 6 months (unless contraindicated or intolerance); **AND**
G. Prescribing provider is a neurologist or DMD specialist

II. RENEWAL APPROVAL CRITERIA

Coverage can be renewed for six months based on the following conditions:

A. Stabilization of disease or absence of disease progression; **AND**
B. Absence of unacceptable toxicity from the drug.
C. Patient has responded to therapy compared to pretreatment baseline in **one or more** of the following (not all-inclusive):
   i. Increase in dystrophin level
   ii. Improvement in quality of life
   iii. Stability, improvement, or slowed rate of decline in one or more of the following:
      a. Timed function tests (e.g., 6-minute walk test [6MWT], time to stand [TTSTAND], time to run/walk 10 meters [TTRW], time to climb 4 stairs [TTCLIMB], etc.)
      b. Upper limb function (ULM) test
      c. North Star Ambulatory Assessment (NSAA) score
      d. Forced Vital Capacity FVC percent predicted

Applicable Procedure Codes

Code	Description
J1426	Injection, casimersen, 10 mg

Applicable NDCs

Code	Description
60923-0227-02	Injection, 100 mg/2 mL (50 mg/ mL) solution in a single-dose vial

ICD-10 Diagnoses

Code	Description
G71.01	Duchenne or Becker Muscular Dystrophy

Revision History

Company(ies)	DATE	REVISION
EmblemHealth & ConnectiCare	4/22/2024	Annual Review: Updated dosing limits, no criteria changes.
EmblemHealth & ConnectiCare	8/8/2023	Annual Review: Duchenne muscular dystrophy (DMD) Initial Criteria: Removed "Patient must be ambulatory (e.g. 6-minute walk test (6MWT) greater than or equal to 300 meters while walking independently, North star Ambulatory Assessment (NSAA) score of greater than 17, or achieved rise time (Gower's test) less than 7 seconds); **AND"** Added "Patient retains meaningful voluntary motor function (e.g., patient is able to speak, manipulate objects using upper extremities, ambulate, etc.); **AND** Patient is receiving physical and/or occupational therapy; **AND** Baseline documentation of **one or more** of the following: i. Dystrophin level ii. Timed function tests (e.g., 6-minute walk test [6MWT], time to stand [TTSTAND], time to run/walk 10 meters [TTRW], time to climb 4 stairs [TTCLIMB], etc.) iii. Upper limb function (ULM) test iv. North Star Ambulatory Assessment (NSAA) score v. Forced Vital Capacity (FVC) percent predicted" Renewal Criteria: Added "Patient has responded to therapy compared to pretreatment baseline in **one or more** of the following (not all-inclusive): i. Increase in dystrophin level ii. Improvement in quality of life iii. Stability, improvement, or slowed rate of decline in one or more of the following: a. Timed function tests (e.g., 6-minute walk test [6MWT], time to stand [TTSTAND], time to run/walk 10 meters [TTRW], time to climb 4 stairs [TTCLIMB], etc.) b. Upper limb function (ULM) test c. North Star Ambulatory Assessment (NSAA) score d. Forced Vital Capacity FVC percent predicted"
EmblemHealth & ConnectiCare	3/23/2022	Updated Procedure Code to J1426 and put on new template
EmblemHealth & ConnectiCare	6/9/2021	New Policy – went to 2Q2021 P&T, June 9, 2021

References

1. Amondys 45 intravenous Injection [package insert]. Cambridge, MA. Sarepta Therapeutics, Inc. Updated February 25, 2021. Accessed March 26, 2021. Available at: https://dailymed.nlm.nih.gov/dailymed/drugInfo.cfm?setid=e9e5fd44-eeda-4580-bba1-a734828bbcc3.

2. Amondys 45 intravenous Injection. IBM Micromedex® [database online]. Greenwood Village, CO. Truven Health Analytics. Available at: https://www.micromedexsolutions.com. Updated March 8, 2021. Accessed March 26, 2021.
3. CureDuchenne [Web site]. Available at: https://www.cureduchenne.org/.
4. Birnkrant DJ, Bushby K, Bann CM, et al. Diagnosis and management of Duchenne muscular dystrophy, part 1: diagnosis, and neuromuscular, rehabilitation, endocrine, and gastrointestinal and nutritional management. *Lancet Neurol*. 2018;17(3):251-267.
5. Shimizu-Motohashi Y, Murakami T, Kimura E, et al. Exon skipping for Duchenne muscular dystrophy: a systematic review and meta-analysis. *Orphanet J Rare Dis*. 2018;13(1):93.