Medical Drug Clinical Criteria

Subject:	Amondys 45 (casimersen)		
Document #:	CC-0189	**Publish Date**:	09/18/2023
Status:	Revised	**Last Review Date**:	08/18/2023

Table of contents

Overview

This document addresses the use of Amondys 45 (casimersen) in the treatment of Duchenne muscular dystrophy (DMD) with a mutation amenable to exon 45 skipping. DMD is a genetic disorder characterized by decrease in muscle mass over time, including progressive damage and weakness of facial, limb, respiratory and heart muscles. In DMD patients, dystrophin, a protein that is present in skeletal and heart muscles allowing the muscles to function properly, is either absent or found in very small amounts. In theory, exon 45 skipping allows for the creation of a shorter-than-normal, but partially functional, dystrophin protein in patients with a specific type of DMD mutation.

Per the Amondys 45 package insert, Amondys 45 was approved by the FDA under accelerated approval based on an increase in dystrophin production in skeletal muscle observed in patients treated with Amondys 45. Continued approval may be contingent upon verification of a clinical benefit in confirmatory trials. The label indicates there may be a risk of kidney toxicity with Amondys 45. Because of this, kidney function should be monitored. However, creatinine may not be a reliable indicator of renal function in DMD patients.

The ESSENCE trial is a phase 3 trial that includes both casimersen and golodirsen. Inclusion criteria for the ESSENCE trial (NCT02500381) are found on clinicaltrials.gov and are listed as follows:

- Males aged 7-13
- Genotypically confirmed DMD, with genetic deletion amenable to exon 45 or exon 53 skipping
- Stable dose of oral corticosteroids for at least 24 weeks
- Intact right and left biceps or 2 alternative upper muscle groups
- Mean 6MWT greater than or equal 300 meters and less than or equal to 450 meters
- Stable pulmonary function: forced vital capacity (FVC) equal to or greater than 50% predicted

Estimated primary completion date for ESSENCE is October 2025. An additional open label trial is being done (NCT03532542) that requires individuals have completed a clinical trial evaluating either casimersen or golodirsen, per protocol, with an estimated completion date of August 2026..

Prior to starting Amondys 45, serum cystatin C, urine dipstick, and urine protein-to-creatinine ratio should be measured. Amondys 45 is administered via a once weekly IV infusion at a dose of 30 mg/kg over 35-60 minutes.

Clinical criteria

When a drug is being reviewed for coverage under a member's medical benefit plan or is otherwise subject to clinical review (including prior authorization), the following criteria will be used to determine whether the drug meets any applicable medical necessity requirements for the intended/prescribed purpose.

Amondys 45 (casimersen)

Initial requests for Amondys 45 (casimersen) may be approved if the following criteria are met:

I. Individual has a diagnosis of Duchenne muscular dystrophy (DMD); **AND**
II. Documentation is provided that individual has a genetic mutation that is amenable to exon 45 skipping; **AND**
III. Individual is age 7-13 years (NCT02500381); **AND**
IV. Individual has been on a stable dose of oral corticosteroids (NCT02500381); **AND**
V. Documentation is provided that individual has a 6MWT (6 minute walk test) ≥ 300 meters and less than 450 meters (NCT02500381); **AND**

VI. Documentation is provided that individual has stable pulmonary function with forced vital capacity (FVC) equal to or greater than 50% predicted (NCT02500381).

Continuation of therapy with Amondys 45 (casimersen) may be approved if the following criterion are met:

I. Criteria above were met at initiation of therapy; **AND**
II. Documentation is provided that individual remains ambulatory (with or without needing an assistive device, including but not limited to a cane or walker).

Amondys 45 (casimersen) may not be approved for the following:

I. Individual is using another exon skipping agent for DMD (including but not limited to Exondys 51, Vyondys 53); **OR**,
II. When the above criteria are not met and for all other indications.

Approval Duration: 6 months

Quantity Limits

Amondys 45 (casimersen) Quantity Limits

Drug	Limit
Amondys 45 (casimersen)	30 mg/kg once weekly

Coding

The following codes for treatments and procedures applicable to this document are included below for informational purposes. Inclusion or exclusion of a procedure, diagnosis or device code(s) does not constitute or imply member coverage or provider reimbursement policy. Please refer to the member's contract benefits in effect at the time of service to determine coverage or non-coverage of these services as it applies to an individual member.

HCPCS

J1426	Injection, casimersen, 10 mg [Amondys 45]

ICD-10 Diagnosis

G71.00-G71.09	Muscular dystrophy

Document history

Revised: 08/18/2023
Document History:

- 08/18/2023 – Annual Review: Wording and formatting changes. Coding Reviewed: No changes.
- 08/19/2022 – Annual Review: No changes. Coding Reviewed: No changes.
- 08/20/2021 – Annual Review: No changes. Coding review: No changes. Effective 10/1/2021 Added J1426. Deleted 9/30/2021 - J3490, J3590, C9075. Delete all diagnoses pend.
- 03/01/2021 – Select review: Added new clinical criteria document with clinical criteria based on inclusion parameters of clinical trial NCT02500381; added quantity limit based on label. Coding Reviewed: Added HCPCS J3490, J3590, C9399. All diagnosis pend. Effective 7/1/21 Added C9075. Removed HCPCS C9399. Added ICD-10-CM G71.00-G71.09.

References

1. DailyMed. Package inserts. U.S. National Library of Medicine, National Institutes of Health website. http://dailymed.nlm.nih.gov/dailymed/about.cfm.
2. DrugPoints® System [electronic version]. Truven Health Analytics, Greenwood Village, CO. Updated periodically.
3. Lexi-Comp ONLINE™ with AHFS™, Hudson, Ohio: Lexi-Comp, Inc. Updated periodically.
4. Amondys 45 [package insert]. Cambridge, MA: Sarepta Therapeutics, Inc.; 2021.
5. Kole R, Krieg AM. Exon skipping therapy for Duchenne muscular dystrophy. *Ad Drug Del Rev*. 2015; 87:140-107.

Federal and state laws or requirements, contract language, and Plan utilization management programs or polices may take precedence over the application of this clinical criteria.