Clinical Criteria

Subject:	Nebraska Amondys 45 (casimersen)		
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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 age 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 May 2022 with estimated study completion in May 2023. 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. Currently, there are no published data or trial results available for casimersen.

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.

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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 confirmed 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 has been on a stable dose of oral corticosteroids (NCT02500381); **AND**
IV. Documentation is provided that individual has a 6MWT (6 minute walk test) ≥ 300 meters and less than 450 meters (NCT02500381); **AND**
V. Documentation is provided that individual has stable pulmonary function with forced vital capacity (FVC) equal to or greater than 50% predicted (NCT02500381); **AND**
VI. Individual will not use with any other exon skipping agents for DMD (including but not limited to Exondys 51, Vyondys 53).

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, such as a cane or walker).

Approval Duration: 6 months

Requests for Amondys 45 (casimersen) may not be approved when the criteria above are not met and for all other indications.

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

J3490	Unclassified drugs (when specified as (casimersen) Amondys 45)
J3590	Unclassified biologics (when specified as (casimersen) Amondys 45)
C9075	C9075-Injection, casimersen, 10 mg Amondys 45)

ICD-10 Diagnosis

	All diagnoses pend for NOC codes only
G71.00-G71.09	Muscular dystrophy

Document history

Reviewed: 08/20/2021
Document History:

- 08/20/2021 – Annual Review: No changes. Coding review: No changes.
- 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. Kole R, Krieg AM. Exon skipping therapy for Duchenne muscular dystrophy. *Ad Drug Del Rev*. 2015; 87:140-107.
2. ClinicalTrials.gov Internet. Bethesda (MD): National Library of Medicine (US). Available at: https://clinicaltrials.gov/ct2/home Accessed on January 28, 2021.
3. Amondys 45 package insert. Cambridge, MA: Sarepta Therapeutics, Inc.; 2021.

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.