Medical Policy

Utilization Management and Clinical Medical Policy

Policy Name:	Policy Number:	Scope:	Origination Date:	Effective Date:
Casimersen (Amondys 45®)	**MP-RX-FP-181-26**	☒ **MMM MA** ☒ **MMM MultiHealth**	**5/6/2026**	**5/6/2026**
			Last Review Date:	**Frequently Revision:**
			5/6/2026	**Annual**

Service Category:

☐ Anesthesia
☐ Surgery
☐ Radiology Procedures
☐ Pathology and Laboratory Procedures
☐ Medicine Services and Procedures
☐ Evaluation and Management Services
☐ DME/Prosthetics or Supplies
☒ Other: Part B Drugs

Service Description:

This document addresses the use of Amondys 45® (casimersen) in the treatment of Duchenne muscular dystrophy (DMD) in those with a mutation amenable to exon 45 skipping.

Background Information:

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. Estimated primary completion date for ESSENCE is October 2025. Inclusion criteria for the ESSENCE trial (NCT02500381) are found on clinicaltrials.gov and are listed as follows:

- 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

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.

Approved Indications

A. For the treatment of Duchenne muscular dystrophy (DMD) in patients who have a confirmed mutation of the DMD gene that is amenable to exon 51 skipping.

Other Uses

A. None.

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Medical Necessity Guidelines:

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)

A. Criteria For Initial Approval (*Provider must submit documentation [such as office chart notes, lab results, pathology reports, imaging studies, and any other pertinent clinical information] supporting the patient's diagnosis for the drug and confirming that the patient has met **all** approval criteria.)*

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.
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).

B. Criteria For Continuation of Therapy

i. MMM considers continuation of Amondys 45 therapy medically necessary in members requesting reauthorization for an indication listed in Section A Above (Criteria for Initial Approval) if the following criterion are met:
   A. Criteria above were met at initiation of therapy; **AND**
   B. Documentation is provided that individual remains ambulatory (with or without needing an assistive device, such as a cane or walker)

C. Authorization Duration

i. Initial Approval Duration: 6 months
ii. Reauthorization Approval Duration: 6 months

D. Conditions Not Covered

Any other use is considered experimental, investigational, or unproven, including the following (this list may not be all inclusive):

i. Requests for Exondys 51may not be approved when the above criteria (Section A: Criteria for Initial Approval) are not met and for all other indications.
   a. Concomitant use with another exon-skipping agent for DMD (including but not limited to Exondys 51, Vyondys 53).

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Limits or Restrictions:

A. Therapeutic Alternatives

The list below includes preferred alternative therapies recommended in the approval criteria and may be subject to prior authorization.

i. N/A

B. Quantity Limitations

Approvals may be subject to dosing limits in accordance with FDA-approved labeling, accepted compendia, and/or evidence-based practice guidelines. The chart below includes dosing recommendations as per the FDA-approved prescribing information.

Dosage Form & Strengths	Recommended Dosing/Limits
Amondys 45 injection 100 mg/2 mL (50 mg/mL) single-dose vial	• 30 mg/kg once weekly
Exceptions	
None	

Codes Information:

The following list(s) of procedure and/or diagnosis codes is provided for reference purposes only and may not be all inclusive. Inclusion or exclusion of a procedure, diagnosis or device code(s) does not constitute or imply member coverage or provider reimbursement policy. Benefit coverage for health services is determined by the member specific benefit plan document and applicable laws that may require coverage for a specific service. The inclusion of a code does not imply any right to reimbursement or guarantee claim payment. Other Policies and Guidelines may apply.

ICD-10 Diagnostic Codes:

Codes	Description
G71.01	Duchenne or Becker muscular dystrophy

HCPCS Codes:

Codes	Description
J1426	Injection, casimersen, 10 mg [Amondys 45]

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Reference Information:

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.
6. Wagner KR, Kuntz NL, Koenig E, et al. Safety, tolerability, and pharmacokinetics of casimersen in patients with Duchenne muscular dystrophy amenable to exon 45 skipping: A randomized, double-blind, placebo-controlled, dose-titration trial. Muscle Nerve. 2021;64(3):285-292. doi:10.1002/mus.27347

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.

Policy History:

Type of Review	Summary of Changes	P&T Approval Date	UM/CMPC Approval Date
Policy Inception	Elevance Health's Medical Policy adoption.	5/6/2026	5/6/2026