Amondys 45™ (casimersen)
(Intravenous)

Effective Date: 05/01/2021
Review Date: 4/22/2021, 3/03/2022, 2/1/2023, 9/14/2023, 12/07/2023, 01/04/2024, 08/28/2024, 05/07/2025

Scope: Medicaid, Commercial, Medicare

I. Length of Authorization

Authorization is valid for 6 months and may be renewed.

II. Dosing Limits

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

- 1400 billable units every 28 days

III. Summary of Evidence

Amondys 45 (casimersen) is an antisense oligonucleotide for DMD that was approved via the accelerated approval pathway. The approval of Amondys 45 was based on the potential clinical benefit of increased levels of dystrophin protein, the surrogate endpoint, in patients with DMD to address hallmark symptoms of progressive muscle deterioration and weakness. The ESSENCE trial is a global, double blind, randomized, placebo-controlled trial in which 43 patients who had a muscle biopsy at baseline and Week 48 were evaluated for dystrophin level. Patients were males between the ages of 7 and 13 years of age. The patients who received Amondys 45 showed a statistically greater increase in dystrophin protein levels in skeletal muscle compared to patients on placebo (P = 0.004). The most common side effects observed in DMD patients treated with Amondys 45 were upper respiratory tract infections, cough, fever, headache, joint pain, and throat pain.

IV. Initial Approval Criteria [1-5]

Coverage is provided in the following conditions:

Medicare members who have previously received this medication within the past 365 days are not subject to Step Therapy Requirements.

Universal Criteria

- Patient is not on concomitant therapy with other DMD-directed antisense oligonucleotides (e.g., Exondys 51(eteplirsen), Vyondys 53 (golodirsen), Viltepso (viltolarsen), etc.); **AND**
- Patient has never received and will not receive therapy with Elevidys (delandistrogene moxeparvovec-rokl) within 6 months of this request; **AND**
- Patient serum cystatin C, urine dipstick, and urine protein-to-creatinine ratio (UPCR) are measured prior to starting therapy and periodically during treatment; **AND**

Duchenne muscular dystrophy (DMD) † Φ

- Patient must have a confirmed mutation of the *DMD* gene that is amenable to exon 45 skipping; **AND**
- Patient has been on a stable dose of corticosteroids, unless contraindicated or intolerant, for at least 6 months; **AND**
- Patient retains meaningful voluntary motor function (e.g., patient is able to speak, manipulate objects using upper extremities, ambulate, etc.); **AND**
- Patient should be receiving physical and/or occupational therapy; **AND**
- Baseline documentation of one or more of the following:
    - Dystrophin level
    - 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.) Upper limb function (ULM) test
    - North Star Ambulatory Assessment (NSAA)
    - Forced Vital Capacity (FVC) percent predicted

† FDA-labeled indication(s), **‡** Compendia recommended indication(s); **Φ** Orphan Drug

V. Renewal Criteria [1]

- Patient continues to meet universal and other indication-specific relevant criteria such as concomitant therapy requirements (not including prerequisite therapy), performance status, etc. identified in section IV; **AND**

- Absence of unacceptable toxicity from the drug. Examples of unacceptable toxicity include the following: severe hypersensitivity reactions, renal toxicity (e.g., glomerulonephritis, persistent increase in serum cystatin C, proteinuria), etc.; **AND**
- Patient has responded to therapy compared to pretreatment baseline in one or more of the following (not all-inclusive):
  - Increase in dystrophin level
  - Stability, improvement, or slowed rate of decline in timed function tests (e.g., time to stand [TTSTAND], 6-minute walk test [6MWT], time to run/walk 10 meters [TTRW], time to climb 4 stairs [TTCLIMB] or 4-stair climb [4SC])
  - Stability, improvement, or slowed rate of decline in upper limb function (ULM) test
  - Stability, improvement, or slowed rate of decline in North Star Ambulatory Assessment (NSAA) score
  - Stability, improvement, or slowed rate of decline in FVC% predicted
  - Improvement in quality of life

VI. Dosage/Administration

Indication	Dose
Duchenne muscular dystrophy	Administer 30 mg/kg via intravenous infusion once weekly. – Serum cystatin C, urine dipstick, and urine protein-to-creatinine ratio (UPCR) should be measured before starting therapy. Consider measurement of glomerular filtration rate prior to initiation of Amondys 45.

VII. Billing Code/Availability Information

HCPCS Code:

- J1426 – injection, casimersen, 10mg

NDC:

- Amondys 45 100 mg/2 mL single-dose vial: 60923-0227-xx

VIII. References

1. Amondys 45 [package insert]. Cambridge, MA; Sarepta Therapeutics, Inc.; March 2023. Accessed April 2025.

2. Topaloglu H, Gloss D, Moxley RT 3rd, et al. Practice guideline update summary: Corticosteroid treatment of Duchenne muscular dystrophy: Report of the Guideline Development Subcommittee of the American Academy of Neurology. Neurology. 2016 Jul 12;87(2):238.
3. Bushby K, Finkel R, Birnkrant DJ, et al. Diagnosis and management of Duchenne muscular dystrophy, part 1: diagnosis, and pharmacological and psychosocial management. Lancet Neurol; 2010 Jan; 9(1):77-93.
4. Bushby K, Finkel R, Birnkrant DJ, et al. Diagnosis and management of Duchenne muscular dystrophy, part 2: implementation of multidisciplinary care. Lancet Neurol; 2010 Jan; 9(2):177-189.
5. Sarepta Therapeutics. A Double-Blind, Placebo-Controlled, Multi-Center Study With an Open-Label Extension to Evaluate the Efficacy and Safety of SRP-4045 and SRP-4053 in Patients With Duchenne Muscular Dystrophy. Available from: h https://clinicaltrials.gov/ct2/show/NCT02500381?term=NCT02500381&draw=2&rank=1. NLM identifier: NCT02500381. Accessed July 1, 2024.
6. Darras BT, Urion DK, Ghosh PS. Dystrophinopathies. GeneReviews. www.ncbi.nlm.nih.gov/books/NBK1119/ (Accessed on July 1, 2024)
7. 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; 17:251.
8. Birnkrant DJ, Bushby K, Bann CM, et al. Diagnosis and management of Duchenne muscular dystrophy, part 2: respiratory, cardiac, bone health, and orthopaedic management. Lancet Neurol 2018; 17:347.
9. Moxley RT 3rd, Ashwal S, Pandya S, et al. Practice parameter: corticosteroid treatment of Duchenne dystrophy: report of the Quality Standards Subcommittee of the American Academy of Neurology and the Practice Committee of the Child Neurology Society. Neurology. 2005;64:13–20.
10. Gloss D, Moxley RT 3rd, Ashwal S, Oskoui M. Practice guideline update summary: Corticosteroid treatment of Duchenne muscular dystrophy: Report of the Guideline Development Subcommittee of the American Academy of Neurology. Neurology. 2016 Feb 2;86(5):465-72. Doi: 10.1212/WNL.0000000000002337. Reaffirmed on January 22, 2022.

Appendix 1 – Covered Diagnosis Codes

ICD-10	ICD-10 Description
G71.01	Duchenne or Becker muscular dystrophy

Appendix 2 – Centers for Medicare and Medicaid Services (CMS)

The preceding information is intended for non-Medicare coverage determinations. Medicare coverage for outpatient (Part B) drugs is outlined in the Medicare Benefit Policy Manual (Pub. 100-2), Chapter 15, §50 Drugs and Biologicals. In addition, National Coverage Determinations (NCDs) and/or Local Coverage Determinations (LCDs) may exist and compliance with these policies is required where applicable. Local Coverage Articles (LCAs) may also exist for claims payment purposes or to clarify benefit eligibility under Part B for drugs which may be self-administered. The following link may be used to search for NCD, LCD, or LCA documents: https://www.cms.gov/medicare-coverage-database/search.aspx. Additional indications, including any preceding information, may be applied at the discretion of the health plan.

Medicare Part B Covered Diagnosis Codes (applicable to existing NCD/LCA/LCD): N/A

Medicare Part B Administrative Contractor (MAC) Jurisdictions		
Jurisdiction	**Applicable State/US Territory**	**Contractor**
E (1)	CA, HI, NV, AS, GU, CNMI	Noridian Healthcare Solutions, LLC
F (2 & 3)	AK, WA, OR, ID, ND, SD, MT, WY, UT,	Noridian Healthcare Solutions, LLC
5	KS, NE, IA, MO	Wisconsin Physicians Service Insurance Corp (WPS)
6	MN, WI, IL	National Government Services, Inc. (NGS)
H (4 & 7)	LA, AR, MS, TX, OK, CO, NM	Novitas Solutions, Inc.
8	MI, IN	Wisconsin Physicians Service Insurance Corp (WPS)
N (9)	FL, PR, VI	First Coast Service Options, Inc.
J (10)	TN, GA, AL	Palmetto GBA
M (11)	NC, SC, WV, VA (excluding below)	Palmetto GBA
L (12)	DE, MD, PA, NJ, DC (includes Arlington & Fairfax counties and the city of	Novitas Solutions, Inc.
K (13 & 14)	NY, CT, MA, RI, VT, ME, NH	National Government Services, Inc. (NGS)
15	KY, OH	CGS Administrators, LLC

Policy Rationale: Amondys 45 was reviewed by the Neighborhood Health Plan of Rhode Island Pharmacy & Therapeutics (P&T) Committee. Neighborhood adopted the following clinical coverage criteria to ensure that its members use Amondys 45 according to Food and Drug Administration (FDA) approved labeling and/or relevant clinical literature. Neighborhood worked with network prescribers and pharmacists to draft these criteria. These criteria will help ensure its members are using this drug for a medically accepted indication, while minimizing the risk for adverse effects and ensuring more cost-effective options are used first, if applicable and appropriate. For Medicare members, these coverage criteria will only apply in the

absence of National Coverage Determination (NCD) or Local Coverage Determination (LCD) criteria. Neighborhood will give individual consideration to each request it reviews based on the information submitted by the prescriber and other information available to the plan.