Reference number(s)
4563-A

SPECIALTY GUIDELINE MANAGEMENT

AMONDYS 45 (casimersen)

POLICY

I. INDICATIONS

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-Approved Indication
Amondys 45 is indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients who have a confirmed mutation of the DMD gene that is amenable to exon 45 skipping.

This indication is approved under accelerated approval based on an increase in dystrophin production in skeletal muscle observed in patients treated with Amondys 45. Continued approval for this indication may be contingent upon verification of a clinical benefit in confirmatory trials.

All other indications are considered experimental/investigational and not medically necessary.

II. DOCUMENTATION

Submission of the following information is necessary to initiate the prior authorization review:
A. Initial requests:
   1. Laboratory confirmation of Duchenne muscular dystrophy (DMD) diagnosis with a DMD gene mutation that is amenable to exon 45 skipping (refer to examples in Appendix).
   2. If applicable, medical records confirming a worsening in clinical status since receiving gene replacement therapy.
B. Continuation of therapy requests: documentation (e.g., chart notes) of response to therapy.

III. PRESCRIBER SPECIALTIES

This medication must be prescribed by or in consultation with a physician who specializes in the treatment of Duchenne muscular dystrophy (DMD).

IV. CRITERIA FOR INITIAL APPROVAL

Duchenne Muscular Dystrophy
Authorization of 6 months may be granted for treatment of DMD when all of the following criteria are met:
A. Genetic testing was conducted to confirm the diagnosis of DMD and to identify the specific type of DMD gene mutation.
B. The DMD gene mutation is amenable to exon 45 skipping (refer to examples in Appendix).
C. Treatment with Amondys 45 is initiated before the age of 14.

D. Member is able to achieve an average distance of at least 300 meters while walking independently over 6 minutes.
E. Member meets one of the following criteria:
   1. Member has not previously received gene replacement therapy for DMD (e.g., Elevidys).
   2. Member has previously received gene replacement therapy for DMD (e.g., Elevidys) and has experienced a worsening in clinical status since receiving gene replacement therapy (e.g., decline in ambulatory function).
F. Member will not exceed a dose of 30 mg/kg once weekly.

V. CONTINUATION OF THERAPY

Note: Members who were previously established on Amondys 45 and subsequently administered gene replacement therapy (e.g., Elevidys) must meet all initial criteria prior to re-starting Amondys 45.

Authorization of 12 months may be granted for members requesting continuation of therapy when all of the following criteria are met:
A. The member has demonstrated a response to therapy as evidenced by remaining ambulatory (e.g., able to walk with or without assistance, not wheelchair dependent).
B. The member will not exceed a dose of 30 mg/kg once weekly.

VI. APPENDIX

Examples of DMD gene mutations (exon deletions) amenable to exon 45 skipping (not an all-inclusive list):

1. Deletion of exon 44
2. Deletion of exon 46-47
3. Deletion of exon 46-48
4. Deletion of exon 46-49
5. Deletion of exon 46-51
6. Deletion of exon 46-53
7. Deletion of exon 46-55

VII. REFERENCES

1. Amondys 45 [package insert]. Cambridge, MA: Sarepta Therapeutics; February 2021.
2. ClinicalTrials.gov. Study of SRP-4045 and SRP-4053 in DMD patients (ESSENCE). Available at: https://clinicaltrials.gov/ct2/show/NCT02500381. Accessed March 1, 2021.
3. Fletcher, S., et. al. Dystrophin Isoform Induction In Vivo by Antisense-mediated Alternative Splicing. The American Society of Gene & Cell Therapy. 2010;18(6):1218-1223.
4. Polavarapu K, Preethish-Kumar V, Sekar D, et al. Mutation pattern in 606 Duchenne muscular dystrophy children with a comparison between familial and non-familial forms: a study in an Indian large single-center cohort. J Neurol. 2019;266(9):2177-2185.