Amondys 45® (casimersen)

When requesting Amondys 45® (casimersen IV infusion), the individual requiring treatment must be diagnosed with the following FDA-approved indication and meet the specific coverage guidelines and applicable safety criteria for the covered indications.

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 the accelerated approval based on an increase in dystrophin in skeletal muscle observed in some patients treated with Amondys 45. Continued approval for this indication may be contingent upon verification of a clinical benefit in a confirmatory trial.

Coverage Guidelines

The prescribing information for Amondys 45 states that a clinical benefit has not been established. Due to the lack of clinical efficacy data, approval is not recommended for Amondys 45.

References

1. Amondys 45 intravenous infusion [prescribing information]. Cambridge, MA: Sarepta; March 2023.
2. 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(3):251-267.
3. Shimizu-Motohashi Y, Murakami T, Kimura E, et al. Exon skipping for Duchenne muscular dystrophy: a systematic review and meta-analysis. *Orphanet J Rare Dis*. 2018;13(1):93.
4. US National Institutes of Health. In: ClinicalTrials.gov [Internet]. Bethesda (MD): National Library of Medicine (US). 2000- [cited 2024 Feb 13]. Available from: https://clinicaltrials.gov/ct2/show/NCT02500381. Search term: NCT02500381.