Exondys 51™ (eteplirsen)

When requesting Exondys 51™ (eteplirsen), 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

Exondys 51 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 51 skipping. This indication is approved under the accelerated approval based on an increase in dystrophin in skeletal muscle observed in some patients treated with Exondys 51. Continued approval for this indication may be contingent upon verification of a clinical benefit in a confirmatory trial.

Coverage Guidelines

The prescribing information for Exondys 51 states that a clinical benefit has not been established. Due to the lack of clinical efficacy data, approval is not recommended for Exondys 51.

References

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7. FDA briefing document for the Peripheral and Central Nervous System Drugs Advisory Committee Meeting. Eteplirsen (NDA 206488). April 25, 2016.
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13. Sarepta Therapeutics. A study to compare safety and efficacy of a high dose of eteplirsen in participants with Duchenne muscular dystrophy (DMD) (MIS51ON). In: ClinicalTrials.gov [Internet]. Bethesda (MD): National Library of Medicine (US). 2000- [cited 2024 May 13]. Available at: https://clinicaltrials.gov/ct2/show/NCT03992430?term=NCT03992430&draw=2&rank=1. NLM Identifier: NCT03992430.