

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

1. Exondys 51™ intravenous infusion [prescribing information]. Cambridge, MA: Sarepta Therapeutics; January 2022.
2. Annexstad EJ, Lund-Petersen I, Rasmussen M. Duchenne muscular dystrophy. *Tidsskr Nor Laegeforen*. 2014;134(14): 1361-1364.
3. Wood MJA. To skip or not to skip: that is the question for Duchenne muscular dystrophy. *Mol Ther*. 2013;21(12):2131-2132.
4. 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.
5. Flanigan KM, Voit T, Rosales XQ, et al. Pharmacokinetics and safety of single doses of drisapersen in non-ambulant subjects with Duchenne muscular dystrophy: results of a double-blind randomized clinical trial. *Neuromuscul Disord*. 2014;24(1):16-24.
6. Mendell JR, Rodino-Klapac LR, Sahenk Z, et al. Eteplirsen for the treatment of Duchenne muscular dystrophy. *Ann Neurol*. 2013;74(5):637-647.
7. FDA briefing document for the Peripheral and Central Nervous System Drugs Advisory Committee Meeting. Eteplirsen (NDA 206488). April 25, 2016.
8. Mendell JR, Goemans N, Lowes LP, et al. Longitudinal effect of eteplirsen versus historical control on ambulation in Duchenne muscular dystrophy. *Ann Neurol*. 2016;79(2):257-271.
9. Peripheral and Central Nervous System Drugs Advisory Committee. Eteplirsen. April 25, 2016 Accessed on April 21, 2021.
10. 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.
11. Kinane TB, Mayer OH, Duda PW, et al. Long-term pulmonary function in Duchenne muscular dystrophy: comparison of eteplirsen-treated patients to natural history. *J Neuromuscul Dis*. 2018;5(1):47-58.
12. McDonald CM, Sheih PB, Abel-Hamid HZ, et al; on behalf of the Italian DMD Telethon Registry Study Group, Leuven NMRC Registry Investigators, CINRG Duchenne Natural History Investigators, and PROMOTI Trial Clinical Investigators. Open-label evaluation of eteplirsen in patients with Duchenne muscular dystrophy amenable to exon skipping: PROMOTI trial. *J Neuromuscul Dis*. 2021;8:989-1001.
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): Nat Library of Medicine (US). 2000- [cited 2024 May 13]. Available <https://clinicaltrials.gov/ct2/show/NCT03992430?term=NCT03992430&draw=2&rank=1>. NLM Id: NCT03992430.