

Exondys 51™ (eteplirsen)



When requesting Exondys 51™ (eteplirsen), the individual requiring treatment must be diagnosed with the following FDA-approved indication or approved compendial uses 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 confirmatory trials.

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

- Exondys 51™ intravenous infusion [prescribing information]. Cambridge, MA: Sarepta Therapeutics, Inc.; October 2018.
- Annexstad EJ, Lund-Petersen I, Rasmussen M. Duchenne muscular dystrophy. *Tidsskr Nor Lægeforen*. 2014;134(14):1361-1364.
- Wood MJA. To skip or not to skip: that is the question for Duchenne muscular dystrophy. *Mol Ther*. 2013;21(12):2131-2132.
- 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.
- 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.
- Mendell JR, Rodino-Klapac LR, Sahenk Z, et al. Eteplirsen for the treatment of Duchenne muscular dystrophy. *Ann Neurol*. 2013;74(5):637-647.
- FDA briefing document for the Peripheral and Central Nervous System Drugs Advisory Committee Meeting. Eteplirsen (NDA 206488). April 25, 2016. Available at: <http://www.fda.gov/downloads/AdvisoryCommittees/CommitteesMeetingMaterials/Drugs/PeripheralandCentralNervousSystemDrugsAdvisoryCommittee/UCM497063.pdf>. Accessed on April 10, 2020.
- 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.
- Peripheral and Central Nervous System Drugs Advisory Committee. Eteplirsen. April 25, 2016. Available at: <http://www.fda.gov/downloads/AdvisoryCommittees/CommitteesMeetingMaterials/Drugs/PeripheralandCentralNervousSystemDrugsAdvisoryCommittee/UCM500822.pdf>. Accessed on April 10, 2020.
- 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.
- 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.
- 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(4):347-361.
- Birnkrant DJ, Bushby K, Bann CM, et al. Diagnosis and management of Duchenne muscular dystrophy, part 3: primary care, emergency management, psychosocial care, and transitions of care across the lifespan. *Lancet Neurol*. 2018;17(5):445-455.
- Khan N, Eliopoulos H, Han L, et al. Eteplirsen treatment attenuates respiratory decline in ambulatory and non ambulatory patients with Duchenne muscular dystrophy. *J Neuromuscul Dis*. 2019;6(2):213-225.

15. Bouchard M, Vogel LF, Apkon SD. Restoration of walking after surgical management of equinus in a non-ambulatory child with Duchenne muscular dystrophy: a case presentation. *PM R*. 2019;11(11):1240-1243.
16. Shimizu-Motohashi Y, Komaki H, Motohashi N, et al. Restoring dystrophin expression in Duchenne muscular dystrophy: current status of therapeutic approaches. *Pers Med*. 2019;7;9(1).
17. Charleston JS, Schnell FJ, Dworzak J, et al. Eteplirsen treatment for Duchenne muscular dystrophy: exon skipping and dystrophin production. *Neurology*. 2018;90(24):e2146-e2154.
18. Randeree L, Elick GD. Eteplirsen for paediatric patients with Duchenne muscular dystrophy: a pooled-analysis. *J Clin Neurosci*. 2018;49:1-6.
19. Alfano LN, Charleston JS, Connolly AM, et al. Long-term treatment with eteplirsen in nonambulatory patients with Duchenne muscular dystrophy. *Medicine (Baltimore)*. 2019;98(26):e15858.