

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

- For the treatment of Duchenne muscular dystrophy in an individual who has a confirmed mutation of the Duchenne muscular dystrophy gene that is amenable to exon 51 skipping¹

Approved Off-label Compendial uses²

- No off-label uses meet evidence standards

Coverage Guidelines

Duchenne muscular dystrophy¹

- Diagnosis of Duchenne muscular dystrophy¹
- Confirmed mutation of the Duchenne muscular dystrophy (DMD) gene that is amenable to exon 51 skipping^{1,3}

For reauthorization:

- Individual has shown benefit from the drug (eg, improvement in dystrophin levels or in 6-minute walk distance [6MWD])¹

Approval duration: 12 months

Additional Information for Prescribers

- None

References

1. Exondys 51™ prescribing information. Cambridge, MA: Sarepta Therapeutics; Revised September 2016. <http://exondys51hcp.com/sites/default/files/EXONDYS%2051%E2%84%A2%20Prescribing%20Information.pdf>. Accessed January 31, 2017.
2. Klasco RK (Ed): DRUGDEX® system. Thomson Micromedex, Greenwood Village, CO. Accessed January 31, 2017. <https://www.micromedexsolutions.com>.
3. Bushby K, Finkel R, Birnkrant DJ, et al. Diagnosis and management of Duchenne muscular dystrophy, part 1: diagnosis, and pharmacological and psychosocial management. *Lancet Neurol*. 2010;9:77-93. [http://www.thelancet.com/pdfs/journals/laneur/PIIS1474-4422\(09\)70271-6.pdf](http://www.thelancet.com/pdfs/journals/laneur/PIIS1474-4422(09)70271-6.pdf). Accessed January 31, 2017.