

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 skipping

Coverage Guidelines

The individual must meet **all** of the following criteria for initial approval:

- Diagnosis of Duchenne muscular dystrophy
- Confirmed mutation of the Duchenne muscular dystrophy (DMD) gene that is amenable to exon 51 skipping

The individual must meet the following criteria for reauthorization:

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

Approval duration (initial and reauthorization): 12 months

Dosing Recommendation

- 30 milligrams per kilogram of body weight once weekly

References

1. Exondys 51™ prescribing information. Cambridge, MA: Sarepta Therapeutics; Revised September 2016. Accessed September 19, 2019.
2. Klasco RK (Ed): DRUGDEX® system. Thomson Micromedex, Greenwood Village, CO. Accessed September 19, 2019.
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. Accessed September 19, 2019.