

Vyondys 53[®] (golodirsen)

When requesting Vyondys 53[®] (golodirsen), 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

Vyondys 53 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 53 skipping. This indication is approved under the accelerated approval based on an increase in dystrophin in skeletal muscle observed in some patients treated with Vyondys 53. Continued approval for this indication may be contingent upon verification of a clinical benefit in a confirmatory trial.

Coverage Guidelines

The prescribing information for Vyondys 53 states that a clinical benefit has not been established. Due to lack of clinical efficacy data, approval is not recommended for Vyondys 53.

References

1. Vyondys 53 intravenous infusion [prescribing information]. Cambridge, MA: Sarepta Therapeutics, Inc.; June 2024.
2. Annestad EJ, Lund-Petersen I, Rasmussen M. Duchenne muscular dystrophy. *Tidsskr Nor Lægeforen*. 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. van Deutekom JC, Bremmer-Bout M, Janson AA, et al. Antisense-induced exon skipping restores dystrophin expression in DMD patient derived muscle cells. *Hum Mol Genet*. 2001;10(15):1547-1554.
6. Bladen CL, Salgado D, Monges S, et al. The TREAT-NMD DMD Global Database: analysis of more than 7,000 Duchenne muscular dystrophy mutations. *Hum Mutat*. 2015;36(4):395-402.
7. 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.
8. Frank DE, Schnell FJ, Akana C, et al. Increased dystrophin production with golodirsen in patients with Duchenne muscular dystrophy. *Neurology*. 2020;94(21):e2270-e2282.
9. Servais L, Mercuri E, Straub V, et al. Long-term safety and efficacy data of golodirsen in ambulatory patients with Duchenne muscular dystrophy amenable to exon 53 skipping: a first-in-human, multicenter, two-part, open-label, Phase 1/2 trial. *Nucleic Acid Ther*. 2022 Feb;32(1):29-39.
10. Sarepta Therapeutics. Study of SRP-4045 (Casimersen) and SRP-4053 (Golodirsen) in participants with Duchenne muscular dystrophy (DMD) (ESSENCE). In: ClinicalTrials.gov [Internet]. Bethesda (MD): National Library of Medicine (US). 2000- [cited 2023 December 10]. Available at: <https://clinicaltrials.gov/study/NCT02500381?intr=golodirsen&rank=3>. NLM Identifier: NCT02500381.