Viltepso® (viltolarsen)

When requesting Viltepso® (viltolarsen), 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

Viltepso 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 Viltepso. Continued approval for this indication may be contingent upon verification of a clinical benefit in a confirmatory trial.

Coverage Guidelines

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

References

1. Viltepso [prescribing information]. Paramus, NJ: Nippon Shinyaku; March 2021.
2. 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.
3. 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.
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. 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.
6. Clemens PR, Rao VK, Connolly AM, et al. Safety, tolerability, and efficacy of viltolarsen in boys with Duchenne muscular dystrophy amenable to exon 53 skipping. *JAMA Neurology*. 2020;77(8):982-991.
7. Clemens PR, Rao VK, Connolly AM, et al. Long-term functional efficacy and safety of vitolarsen in patients with Duchenne muscular dystrophy. J Neromusc Dis. 2022;9:490-501.
8. Clemens PR, Rao VK, Connolly AM, et al. Efficacy and safety of viltolarsen in boys with Duchenne muscular dystrophy: results from the phase 2, open-label, 4-year extension study. *J Neuromusc Dis*. 2023;439-447.

