Vyondys 53® (golodirsen) Injection

When requesting Vyondys 53® (golodirsen), the individual requiring treatment must be diagnosed with 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 Vyondya 53. Continued approval for this indication may be contingent upon verification of a clinical benefit in confirmatory trials.

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

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9. 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.

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