Vyondys 53 (golodirsen)

Override(s)	Approval Duration
Prior Authorization	6 months

Medications	Dosing Limit
Vyondys 53 (golodirsen) 50 mg/mL intravenous solution	30 mg/kg once weekly

APPROVAL CRITERIA

Initial requests for Vyondys 53 (golodirsen) may be approved if the following criteria are met:

I. Individual has a confirmed diagnosis of Duchenne muscular dystrophy (DMD); **AND**
II. Documentation is provided that individual has a genetic mutation that is amenable to exon 53 skipping; **AND**
III. Individual is age 6-15 years (NCT02310906, Study 4053-101, Frank 2020); **AND**
IV. Individual is using a corticosteroid; **AND**
V. Documentation is provided that individual has a 6MWT (6 minute walk test) ≥ 250m (NCT02310906, Study 4053-101, Frank 2020); **AND**
VI. One of the following:
    A. NorthStar Ambulatory Assessment (NSAA) total > 17 (NCT02310906, Study 4053-101, Frank 2020), and documentation is provided; **OR**
    B. Rise (Gowers) time of < 7 seconds (NCT02310906, Study 4053-101, Frank 2020); **AND**
VII. Individual will not use with any other exon skipping agents for DMD (including but not limited to Exondys 51).

Continuation of therapy with Vyondys 53 (golodirsen) may be approved if the following criteria are met:

I. Criteria above were met at initiation of therapy; **AND**
II. Documentation is provided that individual remains ambulatory (with or without needing an assistive device, such as a cane or walker).

Requests for Vyondys 53 (golodirsen) may not be approved when the criteria above are not met and for all other indications.

Key References:

1. Clinical Pharmacology [database online]. Tampa, FL: Gold Standard, Inc.: 2019. URL: http://www.clinicalpharmacology.com. Updated periodically.
2. DailyMed. Package inserts. U.S. National Library of Medicine, National Institutes of Health website. http://dailymed.nlm.nih.gov/dailymed/about.cfm. Accessed: June 18, 2019.
3. DrugPoints® System [electronic version]. Truven Health Analytics, Greenwood Village, CO. Updated periodically.
4. Lexi-Comp ONLINE™ with AHFS™, Hudson, Ohio: Lexi-Comp, Inc.; 2019; Updated periodically.
5. Kole R, Krieg AM. Exon skipping therapy for Duchenne muscular dystrophy. *Ad Drug Del Rev*. 2015; 87:140-107.
6. Muntoni F, Frank D, Sarone V, et.al. Golodirsen Induces Exon Skipping Leading to Sarcolemmal Dystrophin Expression in Duchenne Muscular Dystrophy Patients With Mutations Amenable to Exon 53 Skipping (S22.001). Neurology Apr 2018, 90 (15 Supplement) S22.001.
7. Phase I/II Study of SRP-4053 in DMD Patients. NCT02310906. Available from: https://clinicaltrials.gov/ct2/show/NCT02310906?term=golodirsen&rank=2. Accessed July 25, 2019.
8. Shieh PB. Golodirsen Induces Exon Skipping Leading to Sarcolemmal Dystrophin Expression in Patients With Genetic Mutations Amenable to Exon 53 Skipping. Presented at: Carrell-Krusen Neuromuscular Symposium, February 22–23, 2018; Dallas, TX. Available at: https://cme.utsouthwestern.edu/sites/default/files/Session%207%20S-5%20Golodirsen%20Induces%20Dystrophin%20Expression-Shieh.pdf. Accessed on December 20, 2019.
9. FDA Clinical Review. Vyondys 53 (golodirsen). Available at: https://www.accessdata.fda.gov/drugsatfda_docs/nda/2019/211970Orig1s000MedR.pdf Accessed on January 22, 2020. and February 5, 2020.
10. FDA Other Reviews. Vyonds 53 (golodirsen). Appeal Granted. Available at: https://www.accessdata.fda.gov/drugsatfda_docs/nda/2019/211970Orig1s000AdminCorres.pdf Accessed on February 7, 2020.
11. Frank, DE, Schnell FJ, Akana C, et.al. Increased dystrophin production with golodirsen in patients with Duchenne muscular dystrophy. *Neurology*. 2020;94:e2270-e2282. Doi:10.1212/WNL.0000000000009233. Available from: https://n.neurology.org/content/neurology/94/21/e2270.full.pdf. Accessed July 23, 2020.

Federal and state laws or requirements, contract language, and Plan utilization management programs or polices may take precedence over the application of this clinical criteria.