Exondys 51 (eteplirsen)

Override(s)	Approval Duration
Prior Authorization	6 months

Medications	Dosing Limit
Exondys 51 (eteplirsen) 50mg/mL injection, solution, concentrate	30 mg/kg once weekly

APPROVAL CRITERIA

Initial requests for Exondys 51 (eteplirsen) 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 51 skipping; **AND**
III. Individual is age 7-13 years of age (NCT01396239 [Study 201] and NCT01540409 [Study 202]); **AND**
IV. Individual is using a corticosteroid; **AND**
V. Documentation is provided that shows individual must be able to walk an average distance between 200 and 400 meters (+/- 10%) while walking independently during 6MWT (NCT01396239 [Study 201] and NCT01540409 [Study 202]); **AND**
VI. Individual will not use with any other exon skipping agents for DMD (including but not limited to Vyondys 53).

Continuation of therapy with Exondys 51 (eteplirsen) 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 Exondys 51 (eteplirsen) 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.: 2020. 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: July 2, 2020.
3. DrugPoints® System [electronic version]. Truven Health Analytics, Greenwood Village, CO. Updated periodically.
4. Lexi-Comp ONLINE™ with AHFS™, Hudson, Ohio: Lexi-Comp, Inc.; 2020; Updated periodically.
5. Kole R, Krieg AM. Exon skipping therapy for Duchenne muscular dystrophy. *Ad Drug Del Rev*. 2015; 87:140-107.
6. Cirak S, Arechavala-Gomeza V, Guglieri M, et.al. Exon skipping and dystrophin restoration in patients with Duchenne muscular dystrophy after systemic phosphorodiamidate morpholino oligometer treatment: an open-label, phase 2, dose-escalation study. *Lancet*. 2011; 378:595-605.
7. Mendell JR, Rodino-Klapac LR, Sahenk Z, et.al. Eteplirsen for the treatment of Duchenne muscular dystrophy. *Ann Neurol.* 2013; 74:631-647.
8. Charleston JS, Schnell FJ, Dworzak J, et.al. Eteplirsen treatment for Duchenne muscular dystrophy: Exon skipping and dystrophin production. *Neurology*. 2018; 90:e2146-e2154.
9. Mendell JR, Goemans N, Lowes LP, et.al. Longitudinal effect of eteplirsen versus historical control on ambulation in Duchenne muscular dystrophy. *Ann Neurol.* 2016; 79:257-271.

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.