

**Louisiana Medicaid  
Eteplirsen (Exondys 51®)**

The *Louisiana Uniform Prescription Drug Prior Authorization Form* should be utilized to request clinical authorization for eteplirsen (Exondys 51®).

Additional Point-of-Sale edits may apply.

*Exondys 51® 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 51 skipping. This indication is approved under accelerated approval based on an increase in dystrophin in skeletal muscle observed in some patients treated with Exondys 51®. A clinical benefit of Exondys 51® has not been established. Continued approval for this indication may be contingent upon verification of a clinical benefit in confirmatory trials.*

**Approval Criteria**

- The following is true and is **stated on the request** – The recipient has a genetically confirmed diagnosis of Duchenne muscular dystrophy [ICD-10-CM code G71.0] and lab testing confirms that the mutation of the dystrophin (DMD) gene is amenable to exon 51 skipping; **AND**
- The recipient is at least 4 years of age, but not older than 19 years of age at initiation of therapy; **AND**
- The recipient has been on a stable dose of corticosteroids for at least 6 months prior to the date of the request. Each medication and date range of treatment must be **listed on the request**; **AND**
- Exondys 51® is prescribed by, or in consultation with, a neurologist experienced in the diagnosis and treatment of DMD; **AND**
- ~~The baseline dystrophin level has been determined and is stated on the request; AND~~
- The recipient has functional status that can be preserved as demonstrated by a valid measuring tool (name and result of the valid measuring tool is **stated on the request**); **AND**
- By submitting the authorization request, the prescriber attests to the following:
  - The prescribed dose does not exceed 30mg/kg once weekly; **AND**
  - The prescribing information for the requested medication has been thoroughly reviewed, including any Black Box Warning(s), Risk Evaluation and Mitigation Strategy (REMS), contraindications, minimum age requirements, recommended dosing, and prior treatment requirements; **AND**
  - All laboratory testing and clinical monitoring recommended in the prescribing information have been completed as of the date of the request and will be repeated as recommended; **AND**
  - The recipient has no concomitant drug therapies [including other RNA antisense agent(s) like golodirsen, or any other gene therapy] or disease states that limit the use of the requested medication and will not be receiving the requested medication in combination with any other medication that is contraindicated or not recommended per FDA labeling.

## Reauthorization Criteria

- The recipient started treatment before 20 years of age; **AND**
- Exondys 51<sup>®</sup> is prescribed by, or in consultation with, a neurologist experienced in the diagnosis and treatment of DMD; **AND**
- The following are true and the prescriber **states both on the request:**
  - **There has been a positive response to therapy; AND**
  - Functional status is reassessed with the same valid measuring tool, and the results show stable or improved functional status from baseline (**baseline and reassessment results** are stated on the request); **AND**
- By submitting the authorization request, the prescriber attests to the following:
  - The prescribed dose does not exceed 30mg/kg once weekly; **AND**
  - The prescribing information for the requested medication has been thoroughly reviewed, including any Black Box Warning(s), Risk Evaluation and Mitigation Strategy (REMS), contraindications, minimum age requirements, recommended dosing, and prior treatment requirements; **AND**
  - All laboratory testing and clinical monitoring recommended in the prescribing information have been completed as of the date of the request and will be repeated as recommended; **AND**
  - The recipient has no concomitant drug therapies [including other RNA antisense agent(s) like golodirsen, or any other gene therapy] or disease states that limit the use of the requested medication and will not be receiving the requested medication in combination with any other medication that is contraindicated or not recommended per FDA labeling.

**Duration of authorization approval, both initial and reauthorization: 6 months**

## References

Exondys 51 (eteplirsen) [package insert]. Cambridge, MA: Sarepta Therapeutics, Inc.; ~~October 2018~~ July 2020. <https://www.exondys51hcp.com/sites/default/files/2019-02/EXONDYS51PI.pdf>

National Institute of Health, U.S. Department of Health & Human Services. Duchenne muscular dystrophy. (2017, September 28). <https://rarediseases.info.nih.gov/diseases/6291/duchenne-muscular-dystrophy>

Mendell JR, Rodino-Klapac LR, Sahenk Z, et al; Eteplirsen Study Group. Eteplirsen for the treatment of Duchenne muscular dystrophy. *Ann Neurol*. 2013;74(5):637-647.

Revision	Date
Policy created.	December 2018
Added requirement for lab testing confirming DMD mutation, added age range, added DMD treatment experience to neurologist specialty, added specific concomitant use of other gene therapies, added functional status and ambulatory statements, added baseline and reauthorization dystrophin level and 6-MWT requirement, added reauthorization age restriction and positive response to therapy	January 2019
Removed FFS, added revision table, removed footer, modified ages of initiation, modified baseline and reauthorization ambulatory and 6-MWT requirement to use any valid measuring tool of functional status	January 2020
Removed dystrophin level requirement and updated package insert	September 2020