Mass General Brigham
Health Plan

Neuromuscular Agents
Exondys 51 (eteplirsen)
Effective 06/01/2025

Plan	☒ MassHealth UPPL ☐Commercial/Exchange	**Program Type**	☒ Prior Authorization ☐ Quantity Limit ☐ Step Therapy
Benefit	☐ Pharmacy Benefit ☒ Medical Benefit		
Specialty Limitations	N/A		
Contact Information	**Medical and Specialty Medications** All Plans | Phone: 877-519-1908 | Fax: 855-540-3693		
	Non-Specialty Medications All Plans | Phone: 800-711-4555 | Fax: 844-403-1029		
Notes	Exondys 51 is also available on the pharmacy benefit. Please see the MassHealth Drug List for coverage and criteria.		

Overview
Exondys 51 (eteplirsen) is an antisense oligonucleotide 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.

Coverage Guidelines
Authorization may be reviewed on a case by case basis for members who are new to the plan currently receiving treatment with requested medication excluding when the product is obtained as samples or via manufacturer's patient assistance programs.
OR
Authorization may be granted for members when all the following criteria are met, and documentation is provided:

1. The member has a diagnosis of Duchenne Muscular Dystrophy
2. Documentation of a confirmed out of frame deletion in the DMD that is amenable to exon 51 skipping
3. The prescribing physician is a neuromuscular neurologist or consult notes from a neuromuscular neurology office are provided
4. The member is ambulatory as defined by a current six-minute walk test (6MWT - distance walked in six minutes in meters) of ≥ 200 meters (test must have been observed or completed by the treating provider, or ordered by the treating provider and completed by a qualified medical practitioner)
5. Dosing is appropriate (30 mg/kg intravenously every week)
6. Member has received a corticosteroid for at least 6 months prior and member will continue to use a corticosteroid in combination with the requested agent **OR** a demonstrated contraindication to corticosteroids
7. Member has at least a baseline measurement for ALL of the following timed function tests as shown in medical records (tests must have been observed or completed by the treating provider, or ordered by the treating provider and completed by a qualified medical practitioner):
    a. Timed ten-meter walk/run (time in seconds)

b. Timed floor (supine) to stand (time in seconds)
c. Timed four-step descend (time in seconds)
d. Timed four-step climb (time in seconds)
e. Timed sit to stand (time in seconds)

8. Member has not previously received treatment with a gene therapy for DMD

Continuation of Therapy

Reauthorizations may be approved when **ALL** the following is met:

1. The member remains ambulatory as defined by a current 6MWT of ≥ 200 meters (test must have been observed or completed by the treating provider, or ordered by the treating provider and completed by a qualified medical practitioner)
2. The member has a stable or improving pattern of 6MWTs as shown in medical records with results of a pretreatment baseline and all interim results (all previous 6MWTs results must be included)
3. Dosing remains appropriate
4. The member continues to utilize corticosteroid in combination with the requested agent **OR** demonstrated contraindication to corticosteroids
5. Member has a stable or improving pattern of observed performance on at least TWO of the following five timed function tests as shown in medical records (all results for all tests must be included with the date of performance; tests must have been observed or completed by the treating provider, or ordered by the treating provider and completed by a qualified medical practitioner):
    a. Timed ten-meter walk/run (time in seconds)
    b. Timed floor (supine) to stand (time in seconds)
    c. Timed four-step descend (time in seconds)
    d. Timed four-step climb (time in seconds)
    e. Timed sit to stand (time in seconds)
6. Prior to therapy with Exondys 51, member has not previously received treatment with a gene therapy for DMD

Limitations

1. Initial and reauthorization approvals may be granted for 6 months.

References

1. Exondys 51 [package insert]. Cambridge (MA): Sarepta Therapeutics, Inc.; 2025 January.
2. Frank DE, Schnell FJ, Akana C, et al. Increased dystrophin production with golodirsen in patients with Duchenne muscular dystrophy. Neurology 2020; 94:e2270
3. FDA grants accelerated approval to first targeted treatment for rare Duchenne muscular dystrophy mutation. https://www.fda.gov/news-events/press-announcements/fda-grants-accelerated-approval-first-targeted-treatment-rare-duchenne-muscular-dystrophy-mutation (Accessed on December 17, 2019).

Review History

02/08/2023 - Reviewed and created for Feb P&T; from SGM to Custom. Matched MH UPPL criteria. Effective 4/1/23.
07/12/2023 – Reviewed and updated for P&T. Minor language update. No clinical changes. Effective 7/31/23.
05/15/25 – Reviewed and updated for P&T. Performed annual medical criteria review. Policy has been updated to better reflect agents with prior authorization on medical benefit. Updated formatting and references. Extended approval duration to 6 months. Added criterion that member must not have used any gene therapy prior. Effective 6/1/25.