Mass General Brigham
Health Plan

Medical Necessity Guidelines
Elevidys (delandistrogene moxeparvovec)

Policy Number: 072

Contents

Overview

Elevidys (delandistrogene moxeparvovec) is a gene therapy based on an adeno-associated virus that delivers a transgene encoding a micro-dystrophin protein designed to replace the function of the mutated *DMD* gene in patients with Duchenne muscular dystrophy (DMD).

FDA-approved indication

Elevidys is an adeno-associated virus vector-based gene therapy indicated in individuals at least 4 years of age:

- For the treatment of Duchenne muscular dystrophy (DMD) in patients who are ambulatory and have a confirmed mutation in the DMD gene.

Note: The FDA has removed the indication for use of Elevidys in non-ambulatory patients. They are investigating the risk of acute liver failure with serious outcomes as noted in the FDA Safety Communication dated July 18, 2025.

Medicare Advantage

Prior Authorization Required	Yes ☒ No ☐

Mass General Brigham Health Plan uses guidance from the Centers for Medicare and Medicaid Services (CMS) for medical necessity determinations for its Medicare Advantage plan members. National Coverage Determinations (NCDs), Local Coverage Determinations (LCDs), Local Coverage Articles (LCAs), and documentation included in the Medicare manuals are the basis for medical necessity determinations. When there is no guidance from CMS for the requested service, Mass General Brigham Health Plan's medical policies are used for medical necessity determinations. **At the time of Mass General Brigham Health Plan's most recent policy review, Medicare had:**

- Medicare Benefit Policy Manual Chapter 15: Covered Medical and Other Health Services

When CMS documentation references FDA labeling, Mass General Brigham Health Plan develops coverage criteria to clarify medical necessity of the requested services. Mass General Brigham Health Plan coverage

criteria align with FDA labeling without contradicting existing determinations and enhance the clarity of medical necessity requirements, documentation requirements, and clinical indications.

Coverage guidelines

Mass General Brigham Health Plan covers Elevidys when all of the following have been met:

1. Member is 4 or 5 years old
2. Diagnosis of DMD with a disease-causing mutation in the *DMD* gene
3. Anti-AAVrh74 total binding antibody titer <1:400
4. On a stable corticosteroid dose
5. Baseline measurements are recorded, within the past 3 months, for
    a. North Star Ambulatory Assessment, including scores and times on individual items
    b. Six-minute walk test (6MWT)
6. 6MWT ≥ 200 meters
7. Appropriate dosing
8. Prescriber is a specialist in neuromuscular disease

Exclusions

1. Deletion in exon 8 or exon 9 of the *DMD* gene
2. Current active infection
3. Prior treatment with delandistrogene moxeparvovec
4. Current treatment with antisense oligonucleotides

Mass General Brigham ACO

Prior Authorization Required	Yes ☒ No ☐

Prior authorization requests for Elevidys for Mass General Brigham ACO members should be submitted to the MassHealth Drug Utilization Review Program. Criteria for Elevidys are found in Table 76 - Neuromuscular Agents – Duchenne Muscular Dystrophy and Spinal Muscular Atrophy.

One Care and Senior Care Options (SCO)

Prior Authorization Required	Yes ☒ No ☐

Mass General Brigham Health Plan uses guidance from CMS for medical necessity determinations for its One Care and SCO plan members. NCDs, LCDs, LCAs, and documentation included in the Medicare manuals are the basis for medical necessity determinations. When there is no guidance from CMS for the requested service, or the member does not meet the medical necessity criteria for the requested service, Mass General Brigham Health Plan uses medical necessity guidelines from MassHealth. **See Medicare Advantage criteria and exclusions above. If Medicare Advantage criteria are not met, then MassHealth criteria are applied.**

Commercial and Qualified Health Plans

Prior Authorization Required	Yes ☒ No ☐

Prior authorization for Elevidys for Commercial and Qualified Health Plan members is managed by Prime Therapeutics. See the Prime Therapeutics policy for Elevidys for more information.

Codes

The following codes are included below for informational purposes only; inclusion of a code does not constitute or imply coverage or reimbursement.

Authorized Code	Code Description
J1413	Injection, delandistrogene moxeparvovec-rokl, per therapeutic dose

Summary of Evidence

Delandistrogene moxeparvovec (Elevidys), the first gene therapy for DMD, is a controversial therapy: it received traditional FDA approval for ambulatory patients 4 years and older, and accelerated approval for non-ambulatory patients in the same age group, despite a pivotal clinical trial that failed to meet its primary efficacy endpoint.

In a pioneering Phase 1/2a nonrandomized trial (SRP-9001-101) in 4 ambulatory patients with DMD aged 4-6 years, Mendell et al. (2020) demonstrated that delandistrogene had a good safety profile and produced a significant increase in micro-dystrophin expression within the gastrocnemius muscle. A trend toward improvement in North Star Ambulatory Assessment (NSAA) scores and reduction in CK levels was noted, though effects on other functional outcomes were variable. In long-term follow-up, improvements in functional outcomes appeared to be sustained at 4 years (Mendell et al. 2023).

Building on these findings, the first cohort of the nonrandomized phase 1b ENDEAVOR trial (SRP-9001-103) reported by Zaidman et al. (2023) focused on ambulatory patients aged 4 to less than 8 years. Again, the primary outcome of dystrophin expression was significantly increased. Exploratory functional endpoints, including NSAA score, showed a trend toward improvement. Outcomes have not yet been published for other cohorts in this trial, which include older ambulatory patients, younger ambulatory patients, and non-ambulatory patients.

The safety profile in the first cohort of ENDEAVOR was similar to the that observed previously; subsequent reports have highlighted the incidence of acute liver injury that was successfully managed with steroids and/or IVIG (Duvuru et al. 2025).

The pivotal trial for FDA approval was the phase 3, randomized, controlled, double-blinded EMBARK trial (SRP-9001-301). Although not yet published in a peer-reviewed journal, the data analysis submitted to the FDA is available (Zhou 2024). The primary efficacy endpoint, least-square mean change in NSAA score from baseline to 52 weeks, did not differ significantly between the group treated with delandistrogene and the placebo group (2.57 vs 1.92, p=0.244). Because the primary outcome did not show a significant difference, statistical inference was not performed on the key secondary outcomes of change from baseline to week 52 in time to rise from floor and in 10-minute walk/run test. A trend toward superiority was observed in the treatment group for both metrics, but the FDA analyst noted that analysis of these endpoints should be adjusted for multiple comparisons to reduce the risk of type I error.

The failure of the EMBARK trial to meet its primary efficacy endpoint raises doubt about the validity of dystrophin expression as a surrogate marker for clinically meaningful endpoints (Bhattacharyya et al. 2024). The decision to grant the product traditional approval on the basis of these data was made against the advice of FDA review teams and directors and has raised questions about the integrity of the FDA review process. The decision to grant accelerated approval for non-ambulatory patients, in whom clinical efficacy has not yet been demonstrated, was also controversial.

Although the balance of risks and benefits may favor treatment in an ambulatory population that otherwise lacks effective nonsteroidal disease-modifying therapies, further studies are needed to better assess long-term outcomes in different populations, and MGB Health Plan assesses that available evidence remains insufficient to determine efficacy in the non-ambulatory population and in children over the age of 5. MGB Health Plan does consider Elevidys to be medically necessary for ambulatory members aged 4-5 years with DMD, as described in the FDA's accelerated BLA approval of June 22, 2023, as this is the population with the best evidence of clinical benefit based on currently available data.

Effective Dates

April 2026: Ad hoc review. Reformatted policy. Clarified Medicare Advantage section and criteria hierarchy in the One Care and SCO section. Moved eligibility criteria to Medicare Advantage section. Added Prime Therapeutics information to Commercial and Qualified Health Plan section.
January 2026: Ad hoc review. Fixed formatting. Updated prior authorization table and added variation for One Care and SCO members.
October 2025: Annual review. Fixed code disclaimer. Updated FDA-approved indications.
April 2025: Ad hoc review. MassHealth variation updated to include new prior authorization process.
March 2025: Ad hoc review. Clarified FDA approved indication. Summary of evidence added. References updated.
April 2024: Effective date.

References

Bhattacharyya M, Miller LE, Miller AL, Bhattacharyya R. The FDA approval of delandistrogene moxeparvovec-rokl for Duchenne muscular dystrophy: a critical examination of the evidence and regulatory process. *Expert Opinion on Biological Therapy*. 2024;24(9):869-871.

Delandistrogene moxeparvovec [package insert]. Cambridge, MA: Sarepta Therapeutics, 2023.

Duvuru R, Neumann S, Beemarajan E, et al. Acute liver injury following delandistrogene moxeparvovec gene therapy requiring intravenous immunoglobulin. *Pediatric neurology 2025;*163:1-3.Klimchak AC, Sedita LE, Rodino-Klapac LR. Assessing the value of delandistrogene moxeparvovec (SRP-9001) gene therapy in patients with Duchenne muscular dystrophy in the United States. J Market Access & Health Policy 2023;11:2216518.

Mendell JR, Sahenk Z, Lehman K. Assessment of systemic delivery of rAAVrh74.MHCK7.micro-dystrophin in children with Duchenne muscular dystrophy: a nonrandomized controlled trial. JAMA Neurol. 2020;77(8):1122-1131.

Mendell JR, Sahenk Z, Lehman KJ, et al. Long-term safety and functional outcomes of delandistrogene moxeparvovec gene therapy in patients with Duchenne muscular dystrophy: A phase 1/2a nonrandomized trial. *Muscle & Nerve*. 2023;69:93-98.

Zaidman CM, Proud CM, McDonald CM, et al. Delandistrogene moxeparvovec gene therapy in ambulatory patients (aged ≥4 to <8 years) with Duchenne muscular dystrophy: 1-year interim results from Study SRP-9001-103 (ENDEAVOR). Ann Neurol. 2023 Nov; 94(5):955-968.

Zhou T. Statistical review – Elevidys. FDA website. 2024. Accessed 1/31/25 at https://www.fda.gov/media/179489/download?attachment.