Elevidys (delandistrogene moxeparvovec-rokl)
PAM – 082

Iowa Medicaid Program	Prior Authorization	**Effective Date***	06/20/2024
Revision Number	1	**Last Reviewed**	04/18/2025
Reviewed By	Medicaid Medical Director	**Next Review**	04/17/2026
Approved By	Medicaid Clinical Advisory Committee	**Approved Date**	04/18/2025

Overview

Medication: [1]	delandistrogene moxeparvovec-rokl
Brand Name:	Elevidys®
Pharmacologic Category:	Adeno-Associated Virus (AAV) vector-based gene therapy; recombinant, non-replicating
FDA-Approved Indications:	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; • For the treatment of DMD in patients who are non-ambulatory and have a confirmed mutation in the *DMD* gene. ▶ Accelerated Approval: The DMD indication in non-ambulatory patients is approved under accelerated approval based on expression of ELEVIDYS micro-dystrophin (noted hereafter as "micro-dystrophin") in skeletal muscle. Continued approval for this indication may be contingent upon verification and description of clinical benefit in a confirmatory trial(s). Contraindication: Elevidys is contraindicated in patients with any deletion in exon 8 and/or exon 9 in the *DMD* gene.
How Supplied:	• Elevidys® is shipped frozen in 10 mL vials. • Supplied as a customized kit to meet dosing requirements for each patient. Each kit contains ten (10) to seventy (70) single-dose vials.
Dosage and Administration:	• For single-dose intravenous infusion only • Select patients for treatment with anti-AAVrh74 total binding antibody titers < 1:400 • Dose is measured in vector genomes (vg) and is based on body weight of the patient: ○ Weight less than 70 kg: dose is 1.33×10^{14} vg/kg (10 mL/kg) ○ Weight 70 kg or greater: dose is 9.31×10^{15} vg (700 mL)
Benefit Category:	Medical

* HCPCS code effective 1/1/2024. Criteria included in this policy corresponds to the expanded indication approved by the FDA on 6/20/2024, thus 6/20/2024 is the effective date for this criteria document.

Descriptive Narrative

Duchenne muscular dystrophy (DMD) is a type of dystrophinopathy which occurs as a result of mutations (primarily deletions) in the dystrophin gene. Dystrophin is a protein that is present in skeletal and heart muscles allowing the muscles to function properly. The principal symptom of DMD is weakness, as muscle fiber degeneration is the primary pathologic process.

The dystrophinopathies are inherited as X-linked recessive traits and have varying clinical characteristics, with DMD being associated with the most severe clinical symptoms. In DMD, dystrophin is either absent or found in very small amounts. The majority of mutations of the dystrophin gene are deletions of one or more exons, which are found in approximately 60 to 65 percent of patients with DMD.[2]

Delandistrogene moxeparvovec-rokl (Elevidys®)

Elevidys® is a recombinant gene therapy designed to deliver the gene encoding the ELEVIDYS micro-dystrophin protein. It is administered as a one-time gene transfer infusion using the adeno-associated virus serotype rh74 (rAAVrh74) vector to deliver the micro-dystrophin-encoding gene to skeletal and cardiac muscle tissue. Cells that receive the modified gene produce a micro-dystrophin (a shortened form of the naturally occurring dystrophin protein). Researchers believe that recipients of the modified dystrophin gene will have a milder, Becker-type muscular dystrophy phenotype.

Elevidys® is the first approved gene therapy for Duchenne muscular dystrophy (DMD). It was initially approved by the FDA under an accelerated approval program in June of 2023 for the treatment of ambulatory pediatric patients aged 4 through 5 years with DMD with a confirmed mutation in the *DMD* gene. In June of 2024 the FDA granted traditional approval to Elevidys® for the treatment of ambulatory patients older than 4 years of age and accelerated approval for non-ambulatory patients aged 4 years and older.

Definitions

- **Ambulatory:** Able to walk, with or without an assistive device, such as a cane or walker (in contrast to “non-ambulatory”: unable to walk and requiring use of a wheelchair on a regular basis).
- **Adeno-associated virus (AAV):** A small virus that infects humans and is not known to cause disease. Modified (non-replicating) AAVs are frequently used as viral vectors for gene therapy.

- **Becker muscular dystrophy (BMD):** A type of muscular dystrophy that is similar to but not as severe as Duchenne muscular dystrophy (DMD). BMD has a later onset and milder symptoms than DMD but can affect the heart in a manner similar to DMD.
- **Dystrophin:** A protein that is required for muscles to function properly. This protein is missing or found in inadequate amounts in individuals with DMD.
- **Gene replacement therapy:** A medical treatment that introduces or alters genetic material to replace the function of a missing or dysfunctional gene with the goal of lessening or eliminating a disease process that results from genetic dysfunction; also known as gene therapy.
- **Surrogate endpoint:** A marker, such as a physical sign, laboratory measurement, or radiographic image or biomarker that is "reasonably likely" to predict clinical benefit, but in and of itself does not measure clinical benefit (such as changes in survival or symptoms).
- **X-linked recessive trait:** A mutation in the gene on the X-chromosome. The phenotype is always expressed in males (who have only one X chromosome) and in females who have mutations in both of their X chromosomes.

Guidelines

In 2005, the American Academy of Neurology issued guidelines on cortico-steroid treatment of Duchenne muscular dystrophy (DMD).[3] The guidelines (updated in 2016 and reaffirmed in 2022) include these recommendations:

Prednisone, offered as an intervention for patients with DMD:
- Should be used to improve strength (Level B) and may be used to improve times motor function (Level C);
- Should be used to improve pulmonary function (Level B);
- May be used to reduce the need for scoliosis surgery (Level C);
- May be used to delay the onset of cardiomyopathy by 18 years of age (Level C).

Deflazacort, offered as an intervention for patients with DMD, may be used to:
- Improve strength and timed motor function and delay age at loss of ambulation by 1.4–2.5 years (Level C);
- Improve pulmonary function (Level C);
- Reduce the need for scoliosis surgery (Level C);
- Delay the onset of cardiomyopathy by 18 years of age (Level C);
- Increase survival at 5 and 15 years of follow-up (Level C).

Care considerations for DMD were last published in April 2018, and while they do mention the implications of emerging genetic and molecular therapies for DMD, gene therapy such as Elevidys® had not yet been FDA-approved and so is not a part of the official guidance.[4,5,6]

Criteria

Prior authorization is required.

Elevidys® is considered medically necessary when **ALL** of the following are met:

1. Diagnosis of Duchenne muscular dystrophy (DMD) and **BOTH** of the following apply:
   a. Genetic testing confirms a mutation in the *DMD* gene; **AND**
   b. The mutation is not a deletion in exon 8 and/or exon 9; **AND**
2. Member meets **ALL** of the following
   a. Four (4) years of age or older; **AND**
   b. Baseline anti-AAVrh74 antibody titers have been measured and are less than 1:400; **AND**
   c. No previous treatment with Elevidys®; **AND**
   d. No clinical signs or symptoms of infection evident at the time of Elevidys® administration; **AND**
3. Member will receive a corticosteroid regimen prior to and for a minimum of 60 days following receipt of Elevidys®, in accordance with FDA-approved labeling; **AND**
4. Elevidys® is not prescribed concurrently with exon-skipping therapies (e.g., Amondys 45™, Exondys 51®, Viltepso™, Vyondys 53™);
   a. If member is currently on exon-skipping therapy, member must discontinue prior to treatment with Elevidys® (and not restart after treatment with Elevidys®); **AND**
5. Prescribed by, or in consultation with, a neurologist with expertise in the management of DMD; **AND**
6. Elevidys® dose is based on member body weight and does not exceed:
   a. 1.33×10^{14} vector genomes per kilogram (vg/kg) of body weight for a member weighing less than 70 kg; **OR**
   b. 9.31×10^{15} vector genomes (vg) for a member weighing 70 kg or greater.

Elevidys® is **NOT** considered medically necessary for continuation of therapy (indicated for one-time treatment only). Requests for repeat treatment will not be authorized.

Approval Duration and Quantity Limits

	Initial Authorization	Subsequent Authorization(s)
Approval Duration	One course of treatment per lifetime, to be administered only for members 4 years of age or older who meet criteria above.	Not applicable.
Quantity Limits	One-time dose based on member weight, not to exceed: • 1.33×10^{14} vector genomes (vg) per kilogram body weight (10 mL/kg) for member < 70 kg; • 9.31×10^{15} vg (700 mL) for member ≥ 70 kg.	

Coding and Product Information

The following list(s) of codes and product information are provided for reference purposes only and may not be all inclusive. The inclusion of a code does not imply any right to reimbursement or guarantee claim payment, nor does the exclusion of a code imply that its association to the HCPCS code is inappropriate.

HCPCS	Description
J1413	Injection, delandistrogene moxeparvovec-rokl, per therapeutic dose

ICD-10	Description
G71.01	Duchenne or Becker muscular dystrophy

NDC *	Dosage	Indicated for Patient Weight	Vials per Kit	Volume per Kit	Units per Kit
60923-0501-10	per treatment dose	10.0 – 10.4 kg	10	100 mL	1
60923-0502-11	per treatment dose	10.5 – 11.4 kg	11	110 mL	1
60923-0503-12	per treatment dose	11.5 – 12.4 kg	12	120 mL	1
60923-0504-13	per treatment dose	12.5 – 13.4 kg	13	130 mL	1
60923-0505-14	per treatment dose	13.5 – 14.4 kg	14	140 mL	1
60923-0506-15	per treatment dose	14.5 – 15.4 kg	15	150 mL	1
60923-0507-16	per treatment dose	15.5 – 16.4 kg	16	160 mL	1
60923-0508-17	per treatment dose	16.5 – 17.4 kg	17	170 mL	1
60923-0509-18	per treatment dose	17.5 – 18.4 kg	18	180 mL	1
60923-0510-19	per treatment dose	18.5 – 19.4 kg	19	190 mL	1
60923-0511-20	per treatment dose	19.5 – 20.4 kg	20	200 mL	1
60923-0512-21	per treatment dose	20.5 – 21.4 kg	21	210 mL	1
60923-0513-22	per treatment dose	21.5 – 22.4 kg	22	220 mL	1
60923-0514-23	per treatment dose	22.5 – 23.4 kg	23	230 mL	1
60923-0515-24	per treatment dose	23.5 – 24.4 kg	24	240 mL	1
60923-0516-25	per treatment dose	24.5 – 25.4 kg	25	250 mL	1
60923-0517-26	per treatment dose	25.5 – 26.4 kg	26	260 mL	1
60923-0518-27	per treatment dose	26.5 – 27.4 kg	27	270 mL	1
60923-0519-28	per treatment dose	27.5 – 28.4 kg	28	280 mL	1
60923-0520-29	per treatment dose	28.5 – 29.4 kg	29	290 mL	1
60923-0521-30	per treatment dose	29.5 – 30.4 kg	30	300 mL	1
60923-0522-31	per treatment dose	30.5 – 31.4 kg	31	310 mL	1
60923-0523-32	per treatment dose	31.5 – 32.4 kg	32	320 mL	1
60923-0524-33	per treatment dose	32.5 – 33.4 kg	33	330 mL	1
60923-0525-34	per treatment dose	33.5 – 34.4 kg	34	340 mL	1
60923-0526-35	per treatment dose	34.5 – 35.4 kg	35	350 mL	1
60923-0527-36	per treatment dose	35.5 – 36.4 kg	36	360 mL	1
60923-0528-37	per treatment dose	36.5 – 37.4 kg	37	370 mL	1
60923-0529-38	per treatment dose	37.5 – 38.4 kg	38	380 mL	1
60923-0530-39	per treatment dose	38.5 – 39.4 kg	39	390 mL	1
60923-0531-40	per treatment dose	39.5 – 40.4 kg	40	400 mL	1
60923-0532-41	per treatment dose	40.5 – 41.4 kg	41	410 mL	1
60923-0533-42	per treatment dose	41.5 – 42.4 kg	42	420 mL	1
60923-0534-43	per treatment dose	42.5 – 43.4 kg	43	430 mL	1
60923-0535-44	per treatment dose	43.5 – 44.4 kg	44	440 mL	1

NDC *	Dosage	Indicated for Patient Weight	Vials per Kit	Volume per Kit	Units per Kit
60923-0536-45	per treatment dose	44.5 – 45.4 kg	45	450 mL	1
60923-0537-46	per treatment dose	45.5 – 46.4 kg	46	460 mL	1
60923-0538-47	per treatment dose	46.5 – 47.4 kg	47	470 mL	1
60923-0539-48	per treatment dose	47.5 – 48.4 kg	48	480 mL	1
60923-0540-49	per treatment dose	48.5 – 49.4 kg	49	490 mL	1
60923-0541-50	per treatment dose	49.5 – 50.4 kg	50	500 mL	1
60923-0542-51	per treatment dose	50.5 – 51.4 kg	51	510 mL	1
60923-0543-52	per treatment dose	51.5 – 52.4 kg	52	520 mL	1
60923-0544-53	per treatment dose	52.5 – 53.4 kg	53	530 mL	1
60923-0545-54	per treatment dose	53.5 – 54.4 kg	54	540 mL	1
60923-0546-55	per treatment dose	54.5 – 55.4 kg	55	550 mL	1
60923-0547-56	per treatment dose	55.5 – 56.4 kg	56	560 mL	1
60923-0548-57	per treatment dose	56.5 – 57.4 kg	57	570 mL	1
60923-0549-58	per treatment dose	57.5 – 58.4 kg	58	580 mL	1
60923-0550-59	per treatment dose	58.5 – 59.4 kg	59	590 mL	1
60923-0551-60	per treatment dose	59.5 – 60.4 kg	60	600 mL	1
60923-0552-61	per treatment dose	60.5 – 61.4 kg	61	610 mL	1
60923-0553-62	per treatment dose	61.5 – 62.4 kg	62	620 mL	1
60923-0554-63	per treatment dose	62.5 – 63.4 kg	63	630 mL	1
60923-0555-64	per treatment dose	63.5 – 64.4 kg	64	640 mL	1
60923-0556-65	per treatment dose	64.5 – 65.4 kg	65	650 mL	1
60923-0557-66	per treatment dose	65.5 – 66.4 kg	66	660 mL	1
60923-0558-67	per treatment dose	66.5 – 67.4 kg	67	670 mL	1
60923-0559-68	per treatment dose	67.5 – 68.4 kg	68	680 mL	1
60923-0560-69	per treatment dose	68.5 – 69.4 kg	69	690 mL	1
60923-0561-70	per treatment dose	69.5 kg and above	70	700 mL	1

* Labeler: Sarepta Therapeutics, Inc. (60923)

Compliance

1. Should conflict exist between the policy and applicable statute, the applicable statute shall supersede.
2. Federal and State law, as well as contract language, including definitions and specific contract provisions or exclusions, take precedence over medical policy and must be considered first in determining eligibility for coverage.
3. Medical technology is constantly evolving, and Iowa Medicaid reserves the right to review and update medical policy on an annual or as-needed basis.

Medical necessity guidelines have been developed for determining coverage for member benefits and are published to provide a better understanding of the basis upon which coverage decisions are made. Medical necessity guidelines are developed for selected physician-administered medications found to be safe and proven to be effective in a limited, defined population or clinical circumstances. They include concise clinical coverage criteria based on current literature review, consultation with practicing physicians in the service area who are medical experts in the particular field, FDA and other government agency policies, and standards adopted by national accreditation organizations.

Criteria are revised and updated annually, or more frequently if new evidence becomes available that suggests needed revisions.

References

[1] Elevidys® prescribing information (08/2024). Sarepta Therapeutics, Inc.: Cambridge, MA. Available online: www.elevidyshcp.com. Accessed November 1, 2024.

[2] Darras BT. Duchenne and Becker muscular dystrophy: Genetics and pathogenesis. Dashe JF, ed. UpToDate. Waltham, MA: UpToDate Inc. www.uptodate.com. Accessed February 28, 2025.

[3] Gloss D, Moxley RT 3rd, et al. Practice guideline update summary: Corticosteroid treatment of Duchenne muscular dystrophy: Report of the Guideline Development Subcommittee of the American Academy of Neurology. Neurology. 2016 Feb 2;86(5):465-72. PMID: 26833937. Reaffirmed in 2022.

[4] Birnkrant, David J et al. "Diagnosis and management of Duchenne muscular dystrophy, part 1: Diagnosis, and neuromuscular, rehabilitation, endocrine, and gastrointestinal and nutritional management." The Lancet. Neurology vol. 17,3 (2018): 251-267.

[5] Birnkrant, David J et al. "Diagnosis and management of Duchenne muscular dystrophy, part 2: Respiratory, cardiac, bone health, and orthopaedic management." The Lancet. Neurology vol. 17,4 (2018): 347-361.

[6] Birnkrant, David J et al. "Diagnosis and management of Duchenne muscular dystrophy, part 3: Primary care, emergency management, psychosocial care, and transitions of care across the lifespan." The Lancet. Neurology vol. 17,5 (2018): 445-455.

Development of utilization management criteria may also involve research into other state Medicaid programs, other payer policies, consultation with experts and review by the Medicaid Clinical Advisory Committee (CAC). These sources may not be referenced individually unless they are specifically published and are otherwise applicable to the criteria at issue.

Criteria Change History

Change Date	Changed By	Description of Change	Version
[mm/dd/yyyy]	CAC		
Signature			

Change Date	Changed By	Description of Change	Version
[mm/dd/yyyy]	CAC		
Signature			

Change Date	Changed By	Description of Change	Version
04/18/2025	CAC	Criteria implementation.	1
Signature William (Bill) Jagiello, DO			

CAC = Medicaid Clinical Advisory Committee