Elevidys® (delandistrogene moxeparvovec-rokl)
(Intravenous)

Effective Date: 10/1/2023
Review Date: 9/14/2023, 12/7/2023, 01/04/2024, 8/28/2024
Scope: Medicaid, Commercial, Medicare-Medicaid Plan (MMP)

I. Length of Authorization

Coverage will be provided for one dose per lifetime and may not be renewed.

II. Dosing Limits

A. Quantity Limit (max daily dose) [NDC Unit]:

- 1 kit (based on weight chart below)

B. Max Units (per dose and over time) [HCPCS Unit]:

- 1 therapeutic dose (up to 70 vials [700 mL] based on weight chart below)

III. Summary of Evidence

Elevidys (delandistrogene moxaparvvovec-rokl) is a gene therapy indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients at least 4 years of age with a confirmed mutation in the DMD gene. The DMD indication in non-ambulatory patients is approved under accelerated approval based on expression of Elevidys micro-dystrophin, with continued approval contingent upon verification and description of clinical benefit in a confirmatory trial. Elevidys is contraindicated in DMD patients that carry any deletion in exon 8 and/or exon 9 due to serious adverse events of serious liver injury, immune-mediated myositis, and myocarditis. Elevidys' FDA approval was based on a statistically significant increase in quantity of micro-dystrophin expression at 12 weeks, with a mean change of 40.7-54.2 in Study 102 Part 1, Study 102 Part 2, and Study 103 (combined n=61). The most common adverse reactions across studies (incidence ≥5%) were vomiting and nausea, liver injury, pyrexia, and thrombocytopenia.

IV. Initial Approval Criteria [1-11]

Submission of medical records (chart notes) related to the medical necessity criteria is REQUIRED on all requests for authorizations. Records will be reviewed at the time of submission. Please

provide documentation related to diagnosis, step therapy, and clinical markers (i.e. genetic and mutational testing) supporting initiation when applicable.

Coverage is provided in the following conditions:

Duchenne Muscular Dystrophy (DMD) † Φ [1-11]

- Patient is at least 4 years of age; **AND**
- Patient is not on concomitant therapy with DMD-directed antisense oligonucleotides (e.g., Vyondys 53 (golodirsen), Amondys 45 (casimersen), Viltepso (viltolarsen), Exondys 51 (eteplirsen), etc.); **AND**
- Patient has a not received a DMD-directed antisense oligonucleotide within the past 30 days; **AND**
- Patient does not have an active infection, including clinically important localized infections; **AND**
- Patient has been on a stable dose of a corticosteroid, unless contraindicated or intolerant, prior to start of therapy and will be used concomitantly with a corticosteroid regimen pre- and post- infusion *(refer to the package insert for recommended corticosteroid dosing during therapy)*; **AND**
- Patient troponin-I levels will be monitored at baseline and subsequently as clinically indicated; **AND**
- Patient will have baseline liver function assessed prior to and following therapy for at least 3 months and as indicated; **AND**
- Patient has a confirmed documented mutation of the *DMD* gene between exons 18-58; **AND**
- Diagnosis of DMD along with prescribing Elevidys is made by or in consultation with, a pediatric neuromuscular specialist with expertise in the diagnosis of DMD
- Patient is receiving physical and/or occupational therapy; **AND**
- Patient must have a baseline anti-AAVrh74 total binding antibody titer of < 1:400 as measured by ELISA *(Note: An FDA authorized test for the detection of AAVrh74 total binding antibodies is not currently available. Currently available tests may vary in accuracy and design.)*; **AND**
- Patient does NOT have any deletion in exon 8 and/or exon 9 in the DMD gene; **AND**
- Patient has not been treated with Elevidys (delandistrogene moxeparvovec-rokl) in the past; **AND**
- Patient will undergo treatment at a manufacturer approved Qualified Treatment Center (QTC); **AND**
- MMP members who have previously received this medication within the past 365 days are not subject to Step Therapy Requirements.

† FDA Approved Indication(s); ‡ Compendium Recommended Indication(s); Φ Orphan Drug

V. Renewal Criteria

Coverage cannot be renewed.

VI. Dosage/Administration

Indication	Dose
Duchenne Muscular Dystrophy	• For patients weighing less than 70 kg, the recommended dose is 1.33×10^{14} vector genomes per kilogram (vg/kg) of body weight (or 10 mL/kg body weight) • For patients weighing 70 kg or greater, the recommended dose is 9.31×10^{15} vector genomes (equal to 700mL/70 vials) as a total fixed dose***** <u>Calculate the dose for patients weighing less than 70 kg as follows</u>: Elevidys dose (in mL) = patient body weight (in kilogram) x 10 *The multiplication factor 10 represents the per kilogram dose (1.33×10^{14} vg/kg) divided by the amount of vector genome copies per mL of the Elevidys suspension (1.33×10^{13} vg/mL).* Number of vials needed = Elevidys dose (in mL) divided by 10 (round to the nearest number of vials). ***Note:** There is limited safety data available in non-ambulatory patients weighing 70 kg or greater, who received the maximum dose of ELEVIDYS, 9.31×10^{15} vg, in clinical trials
– *Immune responses to the AAVrh74 vector can occur after administration of Elevidys. To reduce the risk associated with an immune response, corticosteroids should be administered starting 1 day prior to Elevidys infusion. Initiate a corticosteroid regimen following the appropriate schedule. This regimen is recommended for a minimum of 60 days after the infusion unless earlier tapering is clinically indicated. See the PI for the recommended corticosteroid regimen dose modification for patients with liver function abnormalities following Elevidys infusion. If acute serious liver injury is suspected, a consultation with a specialist is recommended.* – *For patients previously taking corticosteroids at baseline, taper off the additional peri-Elevidys corticosteroids (back to baseline corticosteroid dose) over 2 weeks, or longer as needed. For patients not previously taking corticosteroids at baseline, taper the added peri-Elevidys corticosteroids off (back to no corticosteroids) over 4 weeks, or longer, as needed, and the corticosteroids should not be stopped abruptly.*	
• Elevidys is shipped frozen at ≤ -60 °C. Elevidys can be refrigerated but must be used within 14 days of receipt. DO NOT RE-FREEZE.	

- Elevidys is an adeno-associated virus vector-based gene therapy. Follow precautions for viral vector shedding for one month after the infusion.
- For single-dose intravenous infusion only.

VII. Billing Code/Availability Information

HCPCS code:

- J1413 - Injection, delandistrogene moxeparvovec-rokl, per therapeutic dose; 1 billable unit = 1 therapeutic dose

NDC:

Elevidys kit sizes:

Patient Weight (kg)	Total Vials (and mL) per Kit	NDC	Patient Weight (kg)	Total Vials (and mL) per Kit	NDC
10.0 – 10.4	10 (100)	60923-0501-10	40.5 – 41.4	41 (410)	60923-0532-41
10.5 – 11.4	11 (110)	60923-0502-11	41.5 – 42.4	42 (420)	60923-0533-42
11.5 – 12.4	12 (120)	60923-0503-12	42.5 – 43.4	43 (430)	60923-0534-43
12.5 – 13.4	13 (130)	60923-0504-13	43.5 – 44.4	44 (440)	60923-0535-44
13.5 – 14.4	14 (140)	60923-0505-14	44.5 – 45.4	45 (450)	60923-0536-45
14.5 – 15.4	15 (150)	60923-0506-15	45.5 – 46.4	46 (460)	60923-0537-46
15.5 – 16.4	16 (160)	60923-0507-16	46.5 – 47.4	47 (470)	60923-0538-47
16.5 – 17.4	17 (170)	60923-0508-17	47.5 – 48.4	48 (480)	60923-0539-48
17.5 – 18.4	18 (180)	60923-0509-18	48.5 – 49.4	49 (490)	60923-0540-49
18.5 – 19.4	19 (190)	60923-0510-19	49.5 – 50.4	50 (500)	60923-0541-50
19.5 – 20.4	20 (200)	60923-0511-20	50.5 – 51.4	51 (510)	60923-0542-51
20.5 – 21.4	21 (210)	60923-0512-21	51.5 – 52.4	52 (520)	60923-0543-52
21.5 – 22.4	22 (220)	60923-0513-22	52.5 – 53.4	53 (530)	60923-0544-53
22.5 – 23.4	23 (230)	60923-0514-23	53.5 – 54.4	54 (540)	60923-0545-54
23.5 – 24.4	24 (240)	60923-0515-24	54.5 – 55.4	55 (550)	60923-0546-55
24.5 – 25.4	25 (250)	60923-0516-25	55.5 – 56.4	56 (560)	60923-0547-56
25.5 – 26.4	26 (260)	60923-0517-26	56.5 – 57.4	57 (570)	60923-0548-57
26.5 – 27.4	27 (270)	60923-0518-27	57.5 – 58.4	58 (580)	60923-0549-58
27.5 – 28.4	28 (280)	60923-0519-28	58.5 – 59.4	59 (590)	60923-0550-59
28.5 – 29.4	29 (290)	60923-0520-29	59.5 – 60.4	60 (600)	60923-0551-60
29.5 – 30.4	30 (300)	60923-0521-30	60.5 – 61.4	61 (610)	60923-0552-61
30.5 – 31.4	31 (310)	60923-0522-31	61.5 – 62.4	62 (620)	60923-0553-62
31.5 – 32.4	32 (320)	60923-0523-32	62.5 – 63.4	63 (630)	60923-0554-63

32.5 – 33.4	33 (330)	60923-0524-33	63.5 – 64.4	64 (640)	60923-0555-64
33.5 – 34.4	34 (340)	60923-0525-34	64.5 – 65.4	65 (650)	60923-0556-65
34.5 – 35.4	35 (350)	60923-0526-35	65.5 – 66.4	66 (660)	60923-0557-66
35.5 – 36.4	36 (360)	60923-0527-36	66.5 – 67.4	67 (670)	60923-0558-67
36.5 – 37.4	37 (370)	60923-0528-37	67.5 – 68.4	68 (680)	60923-0559-68
37.5 – 38.4	38 (380)	60923-0529-38	68.5 – 69.4	69 (690)	60923-0560-69
38.5 – 39.4	39 (390)	60923-0530-39	≥ 69.5	70 (700)	60923-0561-70
39.5 – 40.4	40 (400)	60923-0531-40			
The total number of vials in each kit corresponds to the dosing requirement for the individual patient, based on the patient's body weight. Each kit includes a specified number of Elevidys vials (with a minimum					

VIII. References

1. Elevidys [package insert]. Cambridge, MA; Sarepta Therapeutics, Inc.; October 2023. Accessed July 2024.
2. Topaloglu H, 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 Jul 12;87(2):238.
3. Bushby K, Finkel R, Birnkrant DJ, et al. Diagnosis and management of Duchenne muscular dystrophy, part 1: diagnosis, and pharmacological and psychosocial management. Lancet Neurol; 2010 Jan; 9(1):77-93.
4. Bushby K, Finkel R, Birnkrant DJ, et al. Diagnosis and management of Duchenne muscular dystrophy, part 2: implementation of multidisciplinary care. Lancet Neurol; 2010 Jan; 9(2):177-189.
5. Birnkrant DJ, Bushby K, Bann CM, et al. Diagnosis and management of Duchenne muscular dystrophy, part 1: diagnosis, and neuromuscular, rehabilitation, endocrine, and gastrointestinal and nutritional management. Lancet Neurol 2018; 17:251.
6. Birnkrant DJ, Bushby K, Bann CM, et al. Diagnosis and management of Duchenne muscular dystrophy, part 2: respiratory, cardiac, bone health, and orthopaedic management. Lancet Neurol 2018; 17:347.
7. Moxley RT 3rd, Ashwal S, Pandya S, et al. Practice parameter: corticosteroid treatment of Duchenne dystrophy: report of the Quality Standards Subcommittee of the American Academy of Neurology and the Practice Committee of the Child Neurology Society. Neurology. 2005;64:13–20.

8. Gordon LB, Brown WT, Collins FS. Hutchinson-Gilford Progeria Syndrome. GeneReviews. https://www.ncbi.nlm.nih.gov/books/NBK1121/ (Accessed on November 25, 2020).
9. Scott E, Eagle M, Mayhew A, et al. Development of a Functional Assessment Scale for Ambulatory Boys with Duchenne Muscular Dystrophy. Physiother. Res. Int. 17 (2012) 101–109.
10. Mercuri E, Coratti G, Messina S. et al. Revised North Star Ambulatory Assessment for Young Boys with Duchenne Muscular Dystrophy. PLoS ONE, 11(8), e0160195.

Appendix 1 – Covered Diagnosis Codes

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

Appendix 2 – Centers for Medicare and Medicaid Services (CMS)

The preceding information is intended for non-Medicare coverage determinations. Medicare coverage for outpatient (Part B) drugs is outlined in the Medicare Benefit Policy Manual (Pub. 100-2), Chapter 15, §50 Drugs and Biologicals. In addition, National Coverage Determinations (NCDs) and/or Local Coverage Determinations (LCDs) may exist and compliance with these policies is required where applicable. Local Coverage Articles (LCAs) may also exist for claims payment purposes or to clarify benefit eligibility under Part B for drugs which may be self- administered. The following link may be used to search for NCD, LCD, or LCA documents: https://www.cms.gov/medicare-coverage-database/search.aspx. Additional indications, including any preceding information, may be applied at the discretion of the health plan.

Medicare Part B Covered Diagnosis Codes (applicable to existing NCD/LCA/LCD): N/A

Medicare Part B Administrative Contractor (MAC) Jurisdictions		
Jurisdiction	**Applicable State/US Territory**	**Contractor**
E (1)	CA, HI, NV, AS, GU, CNMI	Noridian Healthcare Solutions, LLC
F (2 & 3)	AK, WA, OR, ID, ND, SD, MT, WY, UT, AZ	Noridian Healthcare Solutions, LLC
5	KS, NE, IA, MO	Wisconsin Physicians Service Insurance Corp (WPS)
6	MN, WI, IL	National Government Services, Inc. (NGS)
H (4 & 7)	LA, AR, MS, TX, OK, CO, NM	Novitas Solutions, Inc.
8	MI, IN	Wisconsin Physicians Service Insurance Corp (WPS)
N (9)	FL, PR, VI	First Coast Service Options, Inc.
J (10)	TN, GA, AL	Palmetto GBA

M (11)	NC, SC, WV, VA (excluding below)	Palmetto GBA
L (12)	DE, MD, PA, NJ, DC (includes Arlington & Fairfax counties and the city of Alexandria in VA)	Novitas Solutions, Inc.
K (13 & 14)	NY, CT, MA, RI, VT, ME, NH	National Government Services, Inc. (NGS)
15	KY, OH	CGS Administrators, LLC

Policy Rationale:

Elevidys was reviewed by the Neighborhood Health Plan of Rhode Island Pharmacy & Therapeutics (P&T) Committee. Neighborhood adopted the following clinical coverage criteria to ensure that its members use Elevidys according to Food and Drug Administration (FDA) approved labeling and/or relevant clinical literature. Neighborhood worked with network prescribers and pharmacists to draft these criteria. These criteria will help ensure its members are using this drug for a medically accepted indication, while minimizing the risk for adverse effects and ensuring more cost-effective options are used first, if applicable and appropriate. For INTEGRITY (Medicare-Medicaid Plan) members, these coverage criteria will only apply in the absence of National Coverage Determination (NCD) or Local Coverage Determination (LCD) criteria. Neighborhood will give individual consideration to each request it reviews based on the information submitted by the prescriber and other information available to the plan.