Medical Benefit Drug Policy

Elevidys (delandistrogene moxeparvovec-rokl) suspension, for intravenous infusion

Policy Number: MC/PC 010
Effective Date: September 1, 2025

Instructions for Use

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Coverage Rationale

Duchenne Muscular Dystrophy (DMD)

For initial coverage of Elevidys for Duchenne Muscular Dystrophy (DMD), the following will be required:

- Submission of medical records (e.g., chart notes) confirming all of the following:
  - Genetic testing to confirm the diagnosis of DMD and mutation in the DMD gene **and**
  - No deletion in exon 8 or exon 9 in the DMD gene is present **and**
  - Patient is at least 4 years of age **and**
  - Patient is ambulatory without needing an assistive device (e.g., without side-by-side assist, cane, walker, wheelchair, etc.) **and**
  - Anti-AAVrh74 total binding antibody titers are less than 1:400 **and**
  - Patient will receive a corticosteroid regimen prior to and following the administration of Elevidys in line with the FDA-approved recommendations in the labeling **and**
  - Patient does not have preexisting liver impairment, chronic hepatic condition or acute liver disease **and**
- Submission of medical records (e.g., chart notes) documenting results of all of the following baseline laboratory values and provider attests that these laboratory values will be monitored after administration according to the FDA-approved recommendations in the labeling:
  - Liver function [i.e., clinical exam, GGT (gamma-glutamyl transferase), total bilirubin]
  - Platelet counts
  - Troponin-I **and**
- Provider attests that liver function (clinical exam, GGT, and total bilirubin) will be monitored weekly for the first 3 months following Elevidys infusion and monitoring will be continued thereafter if clinically indicated as per FDA-approved recommendations in the labeling **and**
- Submission of medical records (e.g., chart notes) confirming patient has a left ventricular ejection fraction of greater than or equal to 40 percent (%) **and**
- Provider attests that patient does not have clinical signs or symptoms of infection **and**

- Provider attests that patient will not receive exon-skipping therapies fo Exondys 51 (eteplirsen), Viltepso (viltolarsen), Vyondys 53 (golodirsen) treatment **and**
- Prescribed by a neurologist with expertise in the treatment of DMD at an authorized treatment center with expertise in gene therapy **and**
- Submission of medical records (e.g., chart notes) confirming that prescriber completed a shared decision-making conversation has occurred, regarding the potential risks of treatment with Elevidys, including but not limited to acute serious liver injury **and**
- Provider attests that patient has never received Elevidys treatment in their lifetime

Applicable Codes

The following list(s) of procedure and/or diagnosis codes is provided for reference purposes only and may not be all inclusive. Listing of a code in this policy does not imply that the service described by the code is a covered or non-covered health service. Benefit coverage for health services is determined by the member specific benefit plan document and applicable laws that may require coverage for a specific service. The inclusion of a code does not imply any right to reimbursement or guarantee claim payment. Other Policies and Guidelines may apply.

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

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

Background

Duchenne muscular dystrophy (DMD) is an X-linked neuromuscular disease caused by mutations in the DMD gene that result in the absence or near-absence of functional dystrophin protein in muscle cells. It is characterized by progressive loss of skeletal and cardiac muscle function, resulting in loss of ambulation and respiratory or cardiac failure (Institute for Clinical and Economic Review [ICER] 2019). DMD is the most common pediatric muscular dystrophy, with an incidence of around 400 to 600 cases per year and a prevalence of approximately 9000 to 12,000 males in the United States (U.S.) (ICER 2019, Klimchak 2023). The estimated incidence worldwide is 1 in 3500 to 5000 live male births (Mendell et al 2013).

Elevidys is a recombinant adeno-associated viral serotype rh74 (AAVrh74)-based gene therapy designed to deliver a copy of the gene encoding a micro-dystrophin protein expressed in normal muscle cells.

Clinical Evidence

The initial approval of Elevidys was based on an ongoing, 2-part, Phase 2, double-blind (DB), multi-center (MC), placebo-controlled (PC), crossover (XO), randomized controlled trial (RCT) in 41 ambulatory DMD patients 4 through 7 years of age for up to 96 weeks. Part 1 was a 48-week DB, PC, RCT in which patients received Elevidys (n = 20) or placebo (n = 21). Part 2 was a 48-week DB, PC, RCT in which patients who received placebo during Part 1 were treated with Elevidys, and patients who were treated with Elevidys during Part 1 received placebo. Part 3 is an ongoing, open-label (OL), follow-up period.

The first co-primary endpoint was expression of Elevidys micro-dystrophin in skeletal muscle at Week 12. The absolute quantity of Elevidys micro-dystrophin was measured by western blot assay, adjusted by muscle content, and expressed

as a percent of control (i.e., as percent levels of normal, wild-type dystrophin i
muscular dystrophy (BMD)) in muscle biopsy samples. The quantification of mi
change in Elevidys-treated patients vs placebo patients by western blot analysis of muscle biopsies.

- At Week 12 of Part 1, the mean change from baseline of Elevidys micro-dystrophin compared to control was 23.82% (range, -0.64 to 131.67).
- At Week 12 of Part 2, the mean change from baseline of Elevidys micro-dystrophin compared to control was 39.64% (range, -1.13 to -90.43).

The second co-primary endpoint was change in North Star Ambulatory Assessment (NSAA) total score, a functional scale designed for ambulatory DMD patients, at Week 48. In Part 1, Elevidys did not demonstrate a statistically significant effect of treatment vs placebo in the change in NSAA from baseline. Analysis of the 4- to 5-year-old subgroup demonstrated a statistically significant change in NSAA at Week 48. In Part 2, the least square mean (LSM) change of NSAA score from baseline was 1.3 points in the Elevidys group, and the between-group difference was 2.0 ($p = 0.0009$). There was a subgroup analysis of the 4- to 5-year-old subgroup and the 6- to 7-year-old subgroup. The 4- to 5-year-old subgroup demonstrated a statically significant change in NSAA scores of 2.5, but the analysis of 6- to 7-year-old demonstrated a -0.7 difference between treatment groups that was not statistically significant.

Study 103, the ENDEAVOR study, is an ongoing Phase 1b, MC, OL, SA, single-dose trial in ambulatory and non-ambulatory patients. It is a 48-patient multi-cohort, and the primary data from cohort 1 of 20 four- to 7-year-old ambulatory boys supported the initial approval of Elevidys. Additional data from cohort 2 through 5 was later provided to support the expanded indication in non-ambulatory patients and in expanded age groups beyond 4 and 5 years of age. The primary endpoint was change in micro-dystrophin expression as measured by western blot at Week 12.

- At Week 12, ambulatory patients in cohort 1, 2, 4, and 5a showed a numerical increase in micro-dystrophin by western blot of 51.0% (standard deviation [SD], 47).
- At Week 12, non-ambulatory patients in cohort 3 and cohort 5b showed a numerical increase in micro-dystrophin by western blot of 40.1% (SD, 35.9).

An exploratory endpoint was change in NSAA score at Week 52.

- The mean NSAA score in patients treated with Elevidys was 22.1 points at baseline and 26.1 points at Week 52, exhibiting a 4-point increase.

Study 301, the EMBARK study, is an ongoing 2-part, Phase 3, DB, MC, PC, RCT including 125 ambulatory patients 4 to 7 years of age. This confirmatory trial led to the expanded age indication for use of Elevidys in patients beyond 4 or 5 years of age. Cohort 1 patients received a single IV infusion of Elevidys on Day 1 of Year 1 followed by a subsequent placebo IV infusion 1 year later. Cohort 2 patients received a single placebo IV infusion during Day 1 of Year 1 followed by a single Elevidys IV infusion 1 year later.

- The primary endpoint was change in NSAA score at Week 52, and Elevidys did not demonstrate a statistically significant effect vs placebo at Week 52.
- Secondary endpoints of time to rise (TTR), 10-meter walk/run test (10MWR), and micro-dystrophin expression showed a statistically significant improvement.

Place in therapy

Clinical guidelines published by the American Academy of Neurology (AAN) (Gloss et al 2016 [reaffirmed 2022]) and the DMD Care Considerations Working Group (funded by the Centers for Disease Control and Prevention [CDC]) (Birnkrant et al 2018) recognize corticosteroids as the mainstay of DMD treatment. The well-established benefits of long-term glucocorticoid therapy include loss of ambulation at a later age, preserved upper limb and respiratory function, and avoidance of scoliosis surgery.

Although the DMD Care Considerations Working Group acknowledged the app ations were provided to inform its place in therapy (Birnkrant et al 2018). Clinical guic ed since the FDA approvals of Vyondys 53, Viltepso, Amondys 45, Agamree, Duvyzat, or Elevidys.

U.S. Food and Drug Administration (FDA)

This section is to be used for informational purposes only. FDA approval alone is not a basis for coverage.

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.
- For the treatment of DMD in patients who are non-ambulatory and have 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. Continued approval for this indication may be contingent upon verification and description of clinical benefit in a confirmatory trial(s).

References

1. 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(3):251-267.
2. Elevidys [package insert]. Cambridge, MA: Sarepta Therapeutics, Inc.; September 2024.
3. Institute for Clinical and Economic Review (ICER). Deflazacort, eteplirsen, and golodirsen for Duchenne muscular dystrophy: Effectiveness and value: Final evidence report. https://icer-review.org/material/dmd-final-evidence-report/. August 15, 2019. Accessed August 6, 2025.
4. Gloss D, Moxley RT, Ashwal S, Oskoui M. Practice guideline update summary: Corticosteroid treatment of Duchenne muscular dystrophy. Report of the guideline development subcommittee of the American academy of neurology. Neurology. 2016;86(5):465-472.
5. Klimchak AC, Sedita LE, Rodino-Klapac LR, et al. Assessing the value of delandistrogene moxeparvovec (SRP-9001) gene therapy in patients with Duchenne muscular dystrophy in the United States. J Mark Access Health Policy. 2023 May 26;11(1):2216518.
6. Mendell JR, Lloyd-Puryear M. Report of MDA muscle disease symposium on newborn screening for Duchenne muscular dystrophy. Muscle Nerve. 2013 Jul;48(1):21-6.

Policy History/Revision Information

Date	Summary of Changes
5/15/2025	Approved by OptumRx P&T Committee
07/16/2025	Update to coverage rationale section. Added criteria "Patient is ambulatory".
8/21/2025	Update to coverage rationale section to require submission of medical records and requirements of baseline labs and testing and additional requirements for post-treatment monitoring. Provider attestations were included to highlight a shared decision-making process for treatment.

Instructions for Use

This Medical Benefit Drug Policy provides assistance in interpreting standard benefit plans. When deciding coverage, the member specific benefit plan document must be referenced as the terms of the member specific benefit plan may differ from the standard plan. In the event of a conflict, the member specific benefit plan document governs. Before using this policy, please check the member specific benefit plan document and any applicable federal or state mandates. The insurance reserves the right to modify its Policies and Guidelines as necessary. This Medical Benefit Drug Policy is provided for informational purposes. It does not constitute medical advice.

OptumRx may also use tools developed by third parties to assist us in administering health benefits. OptumRx Medical Benefit Drug Policies are intended to be used in connection with the independent professional medical judgment of a qualified health care provider and do not constitute the practice of medicine or medical advice.

Archived Policy Versions (Internal Only)

Effective Date	Policy Number	Policy Title
mm/dd/yyyy – mm/dd/yyyy	######	Title of Policy Hyperlinked to KL or Other Internal Location

Nondiscrimination & Language Access Policy

Discrimination is Against the Law. Aspirus Health Plan, Inc. complies with applicable Federal civil rights laws and does not discriminate on the basis of race, color, national origin, age, disability, or sex, (including sex characteristics, including intersex traits; pregnancy or related conditions; sexual orientation, gender identity and sex stereotypes), consistent with the scope of sex discrimination described at 45 CFR § 92.101(a)(2). Aspirus Health Plan, Inc. does not exclude people or treat them less favorably because of race, color, national origin, age, disability, or sex.

Aspirus Health Plan, Inc.:
Provides people with disabilities reasonable modifications and free appropriate auxiliary aids and services to communicate effectively with us, such as:

- Qualified sign language interpreters.
- Written information in other formats (large print, audio, accessible electronic formats, other formats).

Provides free language assistance services to people whose primary language is not English, which may include:

- Qualified interpreters.
- Information written in other languages.

If *you* need reasonable modifications, appropriate auxiliary aids and services, or language assistance services, contact the Nondiscrimination Grievance Coordinator at the address, phone number, fax number, or email address below.

If *you* believe that Aspirus Health Plan, Inc. has failed to provide these services or discriminated in another way on the basis of race, color, national origin, age, disability, or sex, *you* can file a *grievance* with:

Nondiscrimination Grievance Coordinator
Aspirus Health Plan, Inc.
PO Box 1890
Southampton, PA 18966-9998
Phone: 1-866-631-5404 (TTY: 711)
Fax: 763-847-4010
Email: customerservice@aspirushealthplan.com

You can file a *grievance* in person or by mail, fax, or email. If *you* need help filing a *grievance*, the Nondiscrimination Grievance Coordinator is available to help *you*.

You can also file a civil rights complaint with the U.S. Department of Health and Human Services, Office for Civil Rights, electronically through the Office for Civil Rights Complaint Portal, available at https://ocrportal.hhs.gov/ocr/portal/lobby.jsf, or by mail or phone at:

U.S. Department of Health and Human Services
200 Independence Avenue, SW
Room 509F, HHH Building
Washington, D.C. 20201
1.800.368.1019, 800.537.7697 (TDD)

Complaint forms are available at http://www.hhs.gov/ocr/office/file/index.html. This notice is available at Aspirus Health Plan, Inc.'s website: https://aspirushealthplan.com/webdocs/70021-AHP-NonDiscrim_Lang-Assist-Notice.pdf.

Language Assistance Services

Albanian: KUJDES: Nëse flitni shqip, për ju ka në dispozicion shërbime të asistencës gjuhësore, pa pagesë.Telefononi në 1-800-332-6501 (TTY: 711).

Arabic تنبيه: إذا كنت تتحدث اللغة العربية، فإن خدمات المساعدة اللغوية متاحة لك مجاناً. اتصل بن اعلى رقم الهاتف1-800-332-6501(رقم هاتف الصم والبك : 711)

French: ATTENTION: Si vous parlez français, des services d'aide linguistique vous sont proposés gratuitement. Appelez le 1-800-332-6501 (ATS: 711).

German: ACHTUNG: Wenn Sie Deutsch sprechen, stehen Ihnen kostenlos sprachliche Hilfsdienstleistungen zurVerfügung. Rufnummer: 1-800-332-6501 (TTY: 711).

Hindi: _यान द_: य_द आप ि हंदी बोलते ह_ तो आपके ि लए मु_त म_ भाषा सहायता सेवाएं उपल_ध ह_।1-800-332-6501 (TTY: 711) पर कॉल कर_।

Hmong: LUS CEEV: Yog tias koj hais lus Hmoob, cov kev pab txog lus, muaj kev pab dawb rau koj. Hu rau 1-800-332-6501 (TTY: 711).

Korean: 주의: 한국어를 사용하시는 경우, 언어 지원 서비스를 무료로 이용하실 수 있습니다.1-800-332-6501 (TTY: 711)번으로 전화해 주십시오.

Polish: UWAGA: Jeżeli mówisz po polsku, możesz skorzystać z bezpłatnej pomocy językowej. Zadzwoń pod numer1-800-332-6501 (TTY: 711).

Russian: ВНИМАНИЕ: Если вы говорите на русском языке, то вам доступны бесплатные услуги перевода.Звоните 1-800-332-6501 (телетайп: 711).

Spanish: ATENCIÓN: si habla español, tiene a su disposición servicios gratuitos de asistencia lingüística. Llame al1-800-332-6501 (TTY: 711).

Tagalog: PAUNAWA: Kung nagsasalita ka ng Tagalog, maaari kang gumamit ng mga serbisyo ng tulong sa wika nangwalang bayad. Tumawag sa 1-800-332-6501 (TTY: 711).

Traditional Chinese: 注意：如果您使用繁體中文， 您可以免費獲得語言援助服務。請 致電 1-800-332-6501 (TTY: 711)

Vietnamese: CHÚ Ý: Nếu bạn nói Tiếng Việt, có các dịch vụ hỗ trợ ngôn ngữ miễn phí dành cho bạn. Gọi số 1-800-332-6501 (TTY: 711).

Pennsylvania Dutch: Wann du Deitsch (Pennsylvania German / Dutch) schwetzscht, kannscht du mitaus Koschte ebbergricke, ass dihr helft mit die englisch Schprooch. Ruf selli Nummer uff: Call 1-800-332-6501 (TTY: 711).

Lao: ໂປດຊາບ: ຖ້າວ່າ ທ່ານເວົ້າພາສາ ລາວ, ການບໍລິການຊ່ວຍເຫຼືອດ້ານພາສາ,ໂດຍບໍ່ເສັງຄ່າ, ແມ່ນມີພ້ອມໃຫ້ທ່ານ. ໂທຣ 1-800-332-6501 (TTY: 711).