Pharmacy Management Drug Policy

SUBJECT: Elevidys (delandistrogene moxeparvovec-rokl)
POLICY NUMBER: PHARMACY-117
EFFECTIVE DATE: 04/2024
LAST REVIEW DATE: 04/01/2024

If the member's subscriber contract excludes coverage for a specific service or prescription drug, it is not covered under that contract. In such cases, medical or drug policy criteria are not applied. This drug policy applies to the following line/s of business:

Policy Application

Category:	☒ Commercial Group (e.g., EPO, HMO, POS, PPO)	☒ Medicare Advantage
	☒ On Exchange Qualified Health Plans (QHP)	☐ Medicare Part D
	☒ Off Exchange Direct Pay	☒ Essential Plan (EP)
	☒ Medicaid & Health and Recovery Plans (MMC/HARP)	☒ Child Health Plus (CHP)
	☐ Federal Employee Program (FEP)	☐ Ancillary Services
	☒ Dual Eligible Special Needs Plan (D-SNP)	

DESCRIPTION:

Elevidys (delandistrogene moxeparvovec-rokl) is an adeno-associated virus vector-based gene therapy indicated for the treatment of ambulatory pediatric patients aged 4 through 5 years of age. It is designed to deliver the gene encoding the micro-dystrophin protein. The micro-dystrophin expressed by Elevidys is a shortened version that contains selected domains of dystrophin expressed in normal muscle cells. The drug is approved under accelerated approval which allows a surrogate endpoint (micro-dystrophin level) to be used for serious disease in which there is an unmet need for therapy.

The accelerated approval of Elevidys was based upon data from two ongoing clinical studies (Study 102 and Study 103) and safety data from three ongoing trials (Study 101, Study 102, and Study 103). Study 102 is a multicenter three-part Phase 2 study and Study 3 is a two-part open-label phase 1 study in five cohorts of boys with DMD defined by age and ambulatory status. For the subset of patients 4-5 years of age who received the FDA approved dosage of Elevidys, the mean change from baseline in Elevidys micro-dystrophin expression levels at Week 12 following Elevidys infusion was 95.7% (n=3; standard deviation [SD]: 17.9%) in Study 102 Parts 1 and 2, and 51.7% (n=11; SD: 41.0%) in Study 103 Cohort 1. Elevidys did not demonstrate a statistically significant treatment effect on functional outcomes; however, an exploratory subgroup analysis of the 16 participants (Elevidys: n=8; placebo: n=8) 4 through 5 years of age showed a numerical advantage for Elevidys compared to placebo in the change in North Star Ambulatory Assessment (NSAA) total score.

The Phase 3 EMBARK study is being conducted as the confirmatory trial for Elevidys to assess clinical benefit. On October 30, 2023, Sarepta announced topline results from EMBARK, which enrolled 125 patients with DMD between the ages of 4-7 years of age. The primary endpoint was not met as the change in NSAA total score from baseline at Week 52 (2.6 points in Elevidys-treated patients vs 1.9 points in placebo-treated) did not reach statistical significance (n=125, p=0.24). Key secondary endpoints, including Time to rise (TTR) and 10-meter walk test, showed statistically significant improvement (Change vs placebo LSM difference in seconds was -0.64 for TTR and -0.42 for 10-meter walk test). Full results from EMBARK will be shared at future medical meetings and publications will be pursued in a medical journal.

Elevidys is contraindicated in patients with any deletion in exon 8 and/or exon 9 in the DMD gene due to risk for immune-mediated myositis. Warnings/precautions involve acute serious liver injury, immune-mediated myositis, myocarditis, and pre-existing immunity against AAVrh74.

POLICY:

ELEVIDYS (DELANDISTROGENE MOXEPARVOVEC-ROKL)

Commercial/Essential/Medicaid Criteria:

Based upon our criteria and assessment of the peer-reviewed evidence, the use of Elevidys (delandistrogene moxeparvovec-rokl) has not been medically proven to be effective and, therefore, is considered investigational for the treatment of Duchenne muscular dystrophy (DMD). The justification for Elevidys (delandistrogene moxeparvovec-rokl) to be considered investigational is as follows:

1. Based upon our assessment of the peer-reviewed medical literature, there is inconclusive evidence that the drug has a definite positive effect on health outcomes.
2. Based upon our assessment of the peer-reviewed medical literature, there is inconclusive evidence that the drug, over time, leads to improvement in health outcomes (e.g., the beneficial effects of the service outweigh any harmful effects).
3. Based upon our assessment of peer-reviewed medical literature, there is inconclusive evidence that the drug provides improvement in health outcomes in standard conditions of medical practice, outside the clinical investigatory settings.

Refer to Corporate Medical Policy #11.01.03 Experimental or Investigational Services

Medicare criteria:

1. Medicare reviews are to follow the Local Coverage Determination (LCD) for Drugs and Biologicals, Coverage of, for Label and Off-Label Uses (L33394). The LCD can be found on the CMS website at: LCD - Drugs and Biologicals, Coverage of, for Label and Off-Label Uses (L33394) (cms.gov)

POLICY GUIDELINES:

1. Approval will be granted for 1 year if the above criteria are satisfied.
2. Prior authorization is contract dependent.
3. Not all contracts cover all Medical Infusible drugs. Refer to specific contract/benefit plan language for exclusions of Injectable Medications.
4. Elevidys is administered intravenously and will be considered for coverage under the medical benefit.

CODES:

Eligibility for reimbursement is based upon the benefits set forth in the member's subscriber contract. CODES MAY NOT BE COVERED UNDER ALL CIRCUMSTANCES. PLEASE READ THE POLICY AND GUIDELINES STATEMENTS CAREFULLY.
Codes may not be all inclusive as the AMA and CMS code updates may occur more frequently than policy updates.

HCPCS:

J1413 Elevidys (Effective 1/1/2024)

UPDATES:

Date	Revision
04/01/2024	Created & Implemented
11/23/2023	P&T Committee Review / Approval

REFERENCES:

1. Sarepta Therapeutics, Inc. Elevidys Package Insert; October 2023