Geisinger

POLICIES AND PROCEDURE MANUAL

Policy: MBP 148.0

Section: Medical Benefit Pharmaceutical Policy

Subject: Exondys 51 (eteplirsen)

Applicable line of business:

Commercial	X	Medicaid	X
Medicare	X	ACA	X
CHIP	X		

I. Policy:
Exondys 51 (eteplirsen)

II. Purpose/Objective:
To provide a policy of coverage regarding Exondys 51 (eteplirsen)

III. Responsibility:
A. Medical Directors
B. Medical Management
C. Pharmacy Department

IV. Required Definitions
1. Attachment – a supporting document that is developed and maintained by the policy writer or department requiring/authoring the policy.
2. Exhibit – a supporting document developed and maintained in a department other than the department requiring/authoring the policy.
3. Devised – the date the policy was implemented.
4. Revised – the date of every revision to the policy, including typographical and grammatical changes.
5. Reviewed – the date documenting the annual review if the policy has no revisions necessary.

V. Additional Definitions
Medical Necessity or Medically Necessary means Covered Services rendered by a Health Care Provider that the Plan determines are:
a. appropriate for the symptoms and diagnosis or treatment of the Member's condition, illness, disease or injury;
b. provided for the diagnosis and the direct care and treatment of the Member's condition, illness disease or injury;
c. in accordance with current standards good medical treatment practiced by the general medical community;
d. not primarily for the convenience of the Member, or the Member's Health Care Provider; and
e. the most appropriate source or level of service that can safely be provided to the Member. When applied to hospitalization, this further means that the Member requires acute care as an inpatient due to the nature of the services rendered or the Member's condition, and the Member cannot receive safe or adequate care as an outpatient

Medicaid Business Segment
Medically Necessary — A service, item, procedure, or level of care compensable under the Medical Assistance program that is necessary for the proper treatment or management of an illness, injury, or disability is one that:

i. Will, or is reasonably expected to, prevent the onset of an illness, condition, injury or disability.
ii. Will, or is reasonably expected to, reduce or ameliorate the physical, mental or developmental effects of an illness, condition, injury or disability.
iii. Will assist the Member to achieve or maintain maximum functional capacity in performing daily activities, taking into account both the functional capacity of the Member and those functional capacities that are appropriate for Members of the same age.

DESCRIPTION:
Exondys 51 (eteplirsen) binds to exon 51 of dystrophin pre-messenger RNA (mRNA), resulting in exclusion of this exon during mRNA processing. Exon skipping allows for production of an internally truncated dystrophin protein. Eteplirsen is indicated in the treatment of Duchenne muscular dystrophy (DMD) in patients who have a confirmed mutation of the DMD gene that is amenable to exon 51 skipping.

This indication is approved under accelerated approval based on an increase in dystrophin in skeletal muscle observed in some patients treated with Exondys 51. A clinical benefit of Exondys 51 has not been established. Continued approval for this indication may be contingent upon verification of a clinical benefit in confirmatory trials.

CRITERIA FOR USE: Requires Prior Authorization by Medical Director or Designee

Exondys 51 (eteplirsen) will be considered medically necessary for the Medicare and Medicaid lines of business when ALL of the following criteria are met:

- Medical record documentation of interdisciplinary team involvement including, at a minimum, neurology, cardiology, pulmonology, and a genetic specialist (e.g. geneticist, genetic counselor, etc.) **AND**
- Medical record documentation of Duchenne's Muscular Dystrophy (DMD) confirmed by genetic testing **AND**
- Medical record documentation that the member has a confirmed mutation of the DMD gene that is amenable by exon 51 skipping confirmed by a genetic counselor (including, but not limited to mutation numbers: 45-50, 48-50, 49-50, 50, and 52) **AND**
- Medical record documentation of a baseline evaluation, including a standardized assessment of motor function by a neurologist with experience treating Duchenne muscular dystrophy **AND**
- Medical record documentation that Exondys 51 is being given concurrently with oral corticosteroids unless intolerant or contraindicated **AND**
- Medical record documentation that the patient has not received prior treatment with gene therapy (e.g. Elevidys)*

AUTHORIZATION DURATION: If approved, Exondys 51 should be approved for an authorization duration of 6 months. Subsequent authorizations will be for 6 months and require medical record documentation of the following:

- Medical record documentation that Exondys 51 is being given concurrently with oral corticosteroids unless intolerant or contraindicated **AND**
- Medical record documentation of an annual evaluation, including an assessment of motor function ability, by a neurologist with experience treating Duchenne muscular dystrophy **AND**
- Medical record documentation that the member continues to benefit from treatment of eteplirsen **AND**
- Medical record documentation that the patient has not received prior treatment with gene therapy (e.g. Elevidys)*

*Note: Requests for members that show decline in clinical status following treatment with Elevidys will be reviewed on a case-by-case basis.

Note: For Medicaid (GHP Family), any requests for services that do not meet criteria set in the PARP will be evaluated on a case-by-case basis.

Exondys 51 (eteplirsen) will be considered medically necessary for the commercial, exchange, and CHIP lines of business when ALL of the following criteria are met:

- Medical record documentation of interdisciplinary team involvement including, at a minimum, neurology, cardiology, pulmonology, and a genetic specialist (e.g. geneticist, genetic counselor, etc.) **AND**
- Medical record documentation of Duchenne's Muscular Dystrophy (DMD) confirmed by genetic testing **AND**
- Medical record documentation that the member has a confirmed mutation of the DMD gene that is amenable by exon 51 skipping confirmed by a genetic counselor (including, but not limited to mutation numbers: 45-50, 48-50, 49-50, 50, and 52) **AND**
- Medical record documentation of a baseline evaluation, including a standardized assessment of motor function by a neurologist with experience treating Duchenne muscular dystrophy **AND**
- Medical record documentation that Exondys 51 is being given concurrently with oral corticosteroids unless intolerant or contraindicated **AND**
- Medical record documentation that the patient is ambulatory (e.g. able to walk with assistance, not wheelchair bound, does not have full-time dependence on motorized wheelchairs or scooters for mobility) as proven by documentation of a 6-Minute Walk Test Distance (6MWT) within the past 3 months of initiation of Exondys 51 **AND**
- Medical record documentation that the patient has not received prior treatment with gene therapy (e.g. Elevidys)*

AUTHORIZATION DURATION: If approved, Exondys 51 should be approved for an authorization duration of 6 months. Subsequent authorizations will be for 6 months and require medical record documentation of the following:

- Medical record documentation that Exondys 51 is being given concurrently with oral corticosteroids unless intolerant or contraindicated **AND**
- Medical record documentation of an annual evaluation, including an assessment of motor function ability, by a neurologist with experience treating Duchenne muscular dystrophy **AND**
- Medical record documentation that the member continues to benefit from treatment of eteplirsen. **AND**
- Medical record documentation that the patient remains ambulatory (e.g. able to walk with assistance, not wheelchair bound, does not have full-time dependence on motorized wheelchairs or scooters for mobility) as proven by documentation of a follow-up 6-Minute Walk Test Distance (6MWT) within the past 6 months **AND**
- Medical record documentation that the patient has not received prior treatment with gene therapy (e.g. Elevidys)*

*Note: Requests for members that show decline in clinical status following treatment with Elevidys will be reviewed on a case-by-case basis.

LINE OF BUSINESS:
Eligibility and contract specific benefit limitations and/or exclusions will apply. Coverage statements found in the line of business specific benefit document will supersede this policy.

REFERENCES:

1. Exondys 51 [prescribing information]. Cambridge, MA: Sarepta Therapeutics Inc; January 2022.

This policy will be revised as necessary and reviewed no less than annually**.**

Devised: 1/17/17

Revised: 3/27/17 (per DHS), 5/8/17 (per DHS), 6/14/22 (Medicaid PARP statement), 6/2/23 (LOB carve out, Medicaid business segment), 12/30/23 (references added), 1/2/24 (gene therapy edit from 12/2023), 12/2/24 (LOB table, taglines)

Reviewed: 10/31/17, 9/28/18, 8/29/19, 8/26/20, 7/9/21

MA UM Committee approval: 12/31/23, 12/31/24