Policy and Procedure

DEPARTMENT: Pharmacy, Medical Directors	**DOCUMENT NAME:** Eteplirsen (Exondys 51)
PAGE: 1 of 5	**REPLACES DOCUMENT:**
APPROVED DATE: 4/8/2019	**RETIRED:**
EFFECTIVE DATE: 4/8/2019	**REVIEWED/REVISED:** 4/17/2019, 10/11/19, 1/28/2020, 4/29/2020
PRODUCT TYPE: Star, Star Health, Star Kids, Star Plus, Chip, Chip Prenate	**REFERENCE NUMBER:** TX.PHAR.56

SCOPE:

Superior HealthPlan Pharmacy Department, Medical Directors

PURPOSE:

It is the policy of Superior HealthPlan to follow state guidance for medical necessity review of eteplirsen (Exondys 51). This medication is a pass through drug and should follow state guidance for medical necessity review for Medicaid/CHIP due to the manner in which it is reimbursed. All determinations will be performed by a Superior Medical Director. A SHP pharmacy clinician will review the prior authorization request and make a recommendation to the Medical Director but will not make the ultimate determination on any case.
Additionally, this medication is a Precision Drug. Centene's Precision Drug Action Committee (PDAC) creates a standardized approach for Centene to manage Precision Drugs and the associated costs for their administration, prior to members presenting with a request for one of these agents. All Precision Drug requests or potential requests must be reported to the PDAC for tracking, regardless of whether agents are carved out, passed through, etc. All Precision Drug medical necessity determinations will be supported by PDAC UM recommendation, utilizing specialist input as directed and allowed by turnaround times.

BACKGROUND:

Description/Mechanism of Action:
Eteplirsen (Exondys 51) 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.

Formulations:
Exondys 51 injection is supplied in single-dose vials.
• Single-dose vials containing 100 mg/2 mL (50 mg/mL)
• Single-dose vials containing 500 mg/10 mL (50 mg/mL)

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FDA Approved Indications:
Eteplirsen (Exondys 51) is approved for 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.

PROCEDURE:

I. Initial Approval Criteria

A. Duchenne muscular dystrophy (DMD)

1. All prior authorization approvals or denials will be determined by a Superior HealthPlan Medical Director.
2. Medical necessity determinations will be supported by PDAC UM recommendation. The SHP pharmacy clinician will review the UM recommendation with the prior authorization request for clinical appropriateness and make a recommendation to the SHP Medical Director, but will not make the ultimate determination on any case.
3. Documentation of a neurologist's consultation dated no more than six months prior to the initially requested authorization start date. The consultation must include the neurologist's name, credentials, contact information, and a recommendation for treatment with Exondys 51.
4. Documentation of genetic testing must confirm that the client's DMD gene is amenable to exon 51 skipping.
5. Member's age is birth through 19 years of age.
6. Current weight, including the date the weight was obtained; the weight must be dated no more than 30 days before the request date.
7. Documentation of baseline physical function. Testing tools used to measure the physical function must be age-appropriate for the child who is tested. The testing tool to be used includes but is not limited to: the Brooke Upper Extremity Scale, Baseline 6-minute walk test (6MWT), or the Pediatric Evaluation of Disability Inventory.
8. A maximum of 6 months may be approved by the Medical Director.

Note: Documentation of the member's dosage, administration schedule, number of injections to be administered during the prior authorization period, requested units per injection, and the dosage calculation must be submitted.

Approval duration: 6 months

II. Continued Therapy

A. Duchenne muscular dystrophy (DMD)

1. Currently receiving medication via the company benefit or member has previously met initial approval criteria or had received the drug from a previous Medicaid MCO (continuity of coverage).
2. Request for continuation must be received no earlier than 30 days before the current authorization period expires. The PA will be denied if received after the date of service requested.
3. All approvals or denials for continued therapy will be reviewed by a Superior Medical Director to continue coverage.
4. Medical necessity determinations will be supported by PDAC UM recommendation. The SHP pharmacy clinician will review the UM recommendation with the prior authorization request for clinical appropriateness and make a recommendation to the SHP Medical Director, but will not make the ultimate determination on any case.
5. Current weight, including the date the weight was obtained; the weight must be dated no more than 30 days before the request date.
6. Neurologist's consultation must be dated no more than one rolling year of the recertification/extension date that includes the name, credentials, and contact information for the consulting neurologist recommending ongoing treatment.
7. The Medical Director will check for improvement in or maintenance of baseline physical function. Providers must use the same testing instrument as used in the initial evaluation. If re-use of the initial testing instrument is not appropriate, for example, due to change in client status or restricted age range of the testing tool, the provider must explain the reason for the change. Exondys 51 should not be continued on clients who experience decreasing physical function while on the medication.

8. Documentation includes a statement from prescribing clinician that the client has been compliant with the treatment.

Note: Documentation of the member's dosage, administration schedule, number of injections to be administered during the prior authorization period, requested units per injection, and the dosage calculation must be submitted.

Approval duration: 6 months

REFERENCES:

Exondys 51 (eteplirsen) [prescribing information]. Cambridge, MA: Sarepta Therapeutics Inc; October 2018.

ATTACHMENTS:

DEFINITIONS/ABBREVIATIONS:

REVISION LOG

REVISION	DATE
Added information and criteria step regarding Centene's Precision Drug Action Committee (PDAC)	10/1/2019
Removed incorrect maximum dosing request from initial criteria. Removed pulmonary status criteria from continuation criteria.	10/11/2019

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Age expansion from 0-19 to 0-21 per VDP guidance	1/28/2020
Removed age restriction per VDP guidance	04/29/2020

POLICY AND PROCEDURE APPROVAL

Karen Tadlock, V.P., Pharmacy Operations — Approval on file

Dr. David Harmon, Sr. V.P., Chief Medical Officer — Approval on file

Pharmacy & Therapeutics Committee: — Approval on file

NOTE: The electronic approval retained in RSA Archer, Centene's P&P management software, is considered equivalent to a physical signature.