Policy and Procedure

DEPARTMENT: Pharmacy, Medical Directors	**DOCUMENT NAME:** Golodirsen (Vyondys 53)
PAGE: 1 of 5	**REPLACES DOCUMENT:**
APPROVED DATE: 8/1/2020	**RETIRED:**
EFFECTIVE DATE: 8/1/2020	**REVIEWED/REVISED:** 3/30/21, 11/22/21
PRODUCT TYPE: Star, Star Health, Star Kids, Star Plus, CHIP, CHIP Perinate	**REFERENCE NUMBER:** TX.PHAR.85

SCOPE:

Superior HealthPlan Pharmacy Department, Medical Directors

PURPOSE:

It is the policy of Superior HealthPlan (SHP) to follow state guidance for medical necessity review of golodirsen (Vyondys 53). 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.

BACKGROUND:

Description/Mechanism of Action:
Golodirsen (Vyondys 53) binds to exon 53 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:
Vyondys 53 injection is supplied in single-dose vials.
• Single-dose vials containing 100 mg/2 mL (50 mg/mL)

FDA Approved Indications:
Golodirsen (Vyondys 53) 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 53 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. . 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 golodirsen (Vyondys 53).
4. Documentation of genetic testing must confirm that the client's DMD gene is amenable to exon 53 skipping.
5. Documentation of baseline renal function test (i.e., Glomerulus Filtration Rate, GFR).
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. 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. Documentation that golodirsen (Vyondys 53) will not be used concomitantly with other exon skipping therapies for DMD.
9. 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.
10. The requested dosage is for no more than 30mg/kg once weekly
11. A maximum of 6 months may be approved by the Medical Director.

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. Requests for recertification/extension of prior authorization received after the current prior authorization expires will be denied for dates of service that occurred before the date the request is received.
3. All approvals or denials for continued therapy will be reviewed by a Superior Medical Director to continue coverage.
4. 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. Documentation of continual renal function testing while on golodirsen (Vyondys 53).
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. 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.
8. Documentation that golodirsen (Vyondys 53) will not be used concomitantly with other exon skipping therapies for DMD.
9. 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. Vyondys 53 should not be continued on clients who experience decreasing physical function while on the medication.
10. 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.
11. The requested dosage is for no more than 30mg/kg once weekly

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

Approval duration: 6 months

REFERENCES:

Vyondys 53 (golodirsen) [prescribing information]. Cambridge, MA: Sarepta Therapeutics Inc; February2021.
Texas Medicaid Provider Procedures Manual: Outpatient Drug Services Handbook

ATTACHMENTS:

DEFINITIONS/ABBREVIATIONS:

DMD = Duchenne Muscular Dystrophy

HCPCS Codes:

J1429-Injection, golodirsen 10mg

REVISION LOG

REVISION	DATE
Added OUTPATIENT DRUG SERVICES HANDBOOK MARCH 2021to references Added criteria #10 to continuation of therapy: Documentation includes a statement from prescribing clinician that the client has been compliant with the treatment.	3/30/2021

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Remove PDAC designation effective 12/1/21 Added max dosing to Initial Therapy and Continued Therapy Sections Reworded criteria #2 under Continued Therapy section to match TMPPM Manual Moved notes about dosage & administration schedule to a criteria point under the Initial and Continued Therapy sections Added DMD under Definitions/Abbreviations Updated References	11/22/21

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.