Geisinger

POLICIES AND PROCEDURE MANUAL

Policy: MBP 214.0

Section: Medical Benefit Pharmaceutical Policy

Subject: Vyondys 53 (golodirsen)

Applicable line of business:

Commercial	X	Medicaid	X
Medicare	X	ACA	X
CHIP	X		

I. Policy:
Vyondys 53 (golodirsen)

II. Purpose/Objective:
To provide a policy of coverage regarding Vyondys 53 (golodirsen)

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:
Vyondys 53 (golodirsen) is an antisense oligonucleotide which binds exon 53 of dystrophin pre-mRNA, which results in exclusion of exon 53 during mRNA processing in patients with genetic mutations that are amenable to exon 53 skipping. Exon 53 skipping causes production of a truncated dystrophin protein in these patients. Vyondys 53 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 53 skipping.

This indication is approved under accelerated approval based on an increase in dystrophin production in skeletal muscle observed in patients treated with Vyondys 53. 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

Vyondys 53 (golodirsen) will be considered medically necessary for all 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 to exon 53 skipping confirmed by a genetic counselor **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 Vyondys 53 is being given concurrently with oral corticosteroids unless intolerant or contraindicated **AND**
- Medical record documentation that patient will receive a dose consistent with the FDA approved labeling (maximum dose of 30mg/kg infused once weekly) **AND**
- Medical record documentation that the patient has not received prior treatment with gene therapy (e.g. Elevidys)*

Note: Exon Deletions* on the Duchenne Muscular Dystrophy Gene Theoretically Amenable to Exon 53 Skipping

3-52	4-52	5-52	6-52	9-52					
10-52	11-52	13-52	14-52	15-52	16-52	17-52	19-52		
21-52	23-52	24-52	25-52	26-52	27-52	28-52	29-52		
30-52	31-52	32-52	33-52	34-52	35-52	36-52	37-52	38-52	39-52
40-52	41-52	42-52	43-52	45-52	47-52	48-52	49-52		
50-52	52	54-58	54-61	54-63	54-64	54-66	54-76	54-77	

*The first number represents the first exon deleted. The last number is the last exon deleted. The dash (-) represents all exons in between the first and last exon deleted.

AUTHORIZATION DURATION: Initial approval will be for 6 months or less if the reviewing provider feels it is medically appropriate. Subsequent approvals will be for an additional 6 months or less if the reviewing provider feels it is medically appropriate and will require medical record documentation of the following:

- Medical record documentation that the member continues to benefit from treatment with golodirsen **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 Vyondys 53 continues to be given concurrently with oral corticosteroids unless intolerant or contraindicated **AND**

- Medical record documentation that the patient will continue to receive a dose consistent with the FDA approved labeling (maximum dose of 30mg/kg infused once weekly) **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.

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. Vyondys 53 [prescribing information]. Cambridge, MA: Sarepta Therapeutics Inc; February 2021.

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

Devised: 5/19/20

Revised: 7/9/20 (per DHS), 3/22/23 (LOB carve out, Medicaid PARP statement, Medicaid business segment), 12/28/23 (references added), 1/2/24 (gene therapy edit from 12/2023), 12/2/24 (LOB table, taglines)

Reviewed: 4/30/21, 3/23/22

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