Medical Policy:

Vyondys 53 (golodirsen) injection

POLICY NUMBER	LAST REVIEW	ORIGIN DATE
MG.MM.PH.201	January 2, 2024	January 1, 2020

The treating physician or primary care provider must submit to EmblemHealth, or ConnectiCare, as applicable (hereinafter jointly referred to as "EmblemHealth"), the clinical evidence that the member meets the criteria for the treatment or surgical procedure. Without this documentation and information, EmblemHealth will not be able to properly review the request preauthorization or post-payment review. The clinical review criteria expressed below reflects how EmblemHealth determines whether certain services or supplies are medically necessary. This clinical policy is not intended to pre-empt the judgment of the reviewing medical director or dictate to health care providers how to practice medicine. Health care providers are expected to exercise their medical judgment in rendering appropriate care. Health care providers are expected to exercise their medical judgment in rendering appropriate care.

EmblemHealth established the clinical review criteria based upon a review of currently available clinical information (including clinical outcome studies in the peer reviewed published medical literature, regulatory status of the technology, evidence-based guidelines of public health and health research agencies, evidence-based guidelines and positions of leading national health professional organizations, views of physicians practicing in relevant clinical areas, and other relevant factors). EmblemHealth expressly reserves the right to revise these conclusions as clinical information changes and welcomes further relevant information. Each benefit program defines which services are covered. The conclusion that a particular service or supply is medically necessary does not constitute a representation or warranty that this service or supply is covered and/or paid for by EmblemHealth, as some programs exclude coverage for services or supplies that EmblemHealth considers medically necessary.

If there is a discrepancy between this guideline and a member's benefits program, the benefits program will govern. Identification of selected brand names of devices, tests and procedures in a medical coverage policy is for reference only and is not an endorsement of any one device, test or procedure over another. In addition, coverage may be mandated by applicable legal requirements of a state, the Federal Government or the Centers for Medicare & Medicaid Services (CMS) for Medicare and Medicaid members. All coding and web site links are accurate at time of publication.

EmblemHealth may also use tools developed by third parties, such as the MCG™ Care Guidelines, to assist us in administering health benefits. The MCG™ Care Guidelines are intended to be used in connection with the independent professional medical judgment of a qualified health care provider and do not constitute the practice of medicine or medical advice. EmblemHealth Services Company, LLC, has adopted this policy in providing management, administrative and other services to EmblemHealth Plan, Inc., EmblemHealth Insurance Company, EmblemHealth Services Company, LLC, and Health Insurance Plan of Greater New York (HIP) related to health benefit plans offered by these entities. ConnectiCare, an EmblemHealth company, has also adopted this policy. All of the aforementioned entities are affiliated companies under common control of EmblemHealth Inc.

Definitions

Vyondys (golodirsen) is designed to bind to exon 53 of dystrophin pre-mRNA resulting in exclusion of this exon during mRNA processing in patients with genetic mutations that are amenable to exon 53 skipping. Exon 53 skipping is intended to allow for production of an internally truncated dystrophin protein in patients with genetic mutations that are amenable to exon 53 skipping.

Length of Authorization

Coverage will be provided for 6 months and may be renewed.

Dosing Limits [Medical Benefit]

Max Units (per dose and over time):

30 milligrams per kilogram once weekly; 350 billable units (3500 mg) every 7 days

Guideline

I. Initial Approval Criteria

Vyondys *may be considered medically necessary if one of the below conditions are met* ***AND*** *use is consistent with the medical necessity criteria that follows:*

1. **Duchenne muscular dystrophy (DMD)**
   A. In patients who have a confirmed mutation of the DMD gene that is amenable to exon 53 skipping; **AND**
   B. Patient has been on a stable dose of corticosteroids, unless contraindicated or intolerance, for at least 6 months; **AND**
   C. Patient retains meaningful voluntary motor function (e.g., patient is able to speak, manipulate objects using upper extremities, ambulate, etc.); **AND**
   D. Patient is receiving physical and/or occupational therapy; **AND**
   E. Baseline documentation of **ONE** or more of the following:
      i. Dystrophin level
      ii. Timed function tests (e.g., 6-minute walk test [6MWT], time to stand [TTSTAND], time to run/walk 10 meters [TTRW], time to climb 4 stairs [TTCLIMB], etc.)
      iii. Upper limb function (ULM) test
      iv. North Star Ambulatory Assessment (NSAA) score
      v. Forced Vital Capacity (FVC) percent predicted

Limitations/Exclusions

Vyondys is not considered medically necessary for when any of the following selection criteria is met:

1. Disease progression while on DRUG.
2. Indications not supported by CMS recognized compendia or acceptable peer reviewed literature may be deemed as not approvable and therefore not reimbursable.
3. Renal function should be monitored; creatinine may not be a reliable measure of renal function in DMD patients.
4. There are no clinical studies demonstrating use in the geriatric population (≥ 65 years of age) as DMD is largely a disease of children and young adults
5. Patient is not on concomitant therapy with other DMD-directed antisense oligonucleotides (e.g., eteplirsen, casimersen, viltolarsen, etc.)

II. Renewal Criteria

1. Patient continues to meet INITIAL APPROVAL CRITERIA; **AND**
2. Absence of unacceptable toxicity from the drug. Examples of unacceptable toxicity include reactions (including anaphylaxis), thrombosis, coagulopathy, severe hepatotoxicity, pancreatitis, etc.; **AND**
3. Patient has responded to therapy compared to pretreatment baseline in one or more of the following (not all-inclusive):
   i. Increase in dystrophin level
   ii. Improvement in quality of life
   iii. Stability, improvement, or slowed rate of decline in one or more of the following:

a. Timed function tests (e.g., 6-minute walk test [6MWT], time to stand [TTSTAND], time to run/walk 10 meters [TTRW], time to climb 4 stairs [TTCLIMB], etc.)
b. Upper limb function (ULM) test
c. North Star Ambulatory Assessment (NSAA) score
d. Forced Vital Capacity (FVC) percent predicted

Dosage/Administration

Indication	Dose
Duchenne muscular dystrophy (DMD)	• 30 milligrams per kilogram once weekly • Administer as an intravenous infusion over 35 to 60 minutes • Dilution required prior to administration

Applicable Procedure Codes

Code	Description
J1429	Injection, golodirsen, 10 mg (VYONDYS 53®). J-Code effective date: 07/01/2020

Applicable NDCs

Code	Description
60923-0465-02	Vyondys, single use vial; 50 mg/mL powder for injection

ICD-10 Diagnoses

Code	Description
G71.01	Duchenne or Becker muscular dystrophy

Revision History

Company(ies)	DATE	REVISION
EmblemHealth & ConnectiCare	1/2/2024	Annual Review: Updated length of authorization from 12 months to 6 months Initial Criteria: DMD: Added: "Patient has been on a stable dose of corticosteroids, unless contraindicated or intolerance, for at least 6 months; **AND** Patient retains meaningful voluntary motor function (e.g., patient is able to speak, manipulate objects using upper extremities, ambulate, etc.); **AND** Patient is receiving physical and/or occupational therapy; **AND** Baseline documentation of **ONE** or more of the following: Dystrophin level, Timed function tests (e.g., 6-minute walk test [6MWT], time to stand [TTSTAND], time to run/walk 10 meters [TTRW], time to climb 4 stairs [TTCLIMB], etc.) , Upper limb function (ULM) test, North Star Ambulatory Assessment (NSAA) score, Forced Vital Capacity (FVC) percent predicted" Limitations and Exclusions: Added: "Patient is not on concomitant therapy with other DMD-directed antisense oligonucleotides (e.g., eteplirsen, casimersen, viltolarsen, etc.)" Renewal Criteria: Removed "Disease stabilization or improvement as evidenced by a complete response [CR]"

		Added: “Patient has responded to therapy compared to pretreatment baseline in one or more of the following (not all-inclusive): Increase in dystrophin level, Improvement in quality of life, Stability, improvement, or slowed rate of decline in one or more of the following: Timed function tests (e.g., 6-minute walk test [6MWT], time to stand [TTSTAND], time to run/walk 10 meters [TTRW], time to climb 4 stairs [TTCLIMB], etc.), Upper limb function (ULM) test, North Star Ambulatory Assessment (NSAA) score, Forced Vital Capacity (FVC) percent predicted”
EmblemHealth & ConnectiCare	3/21/2023	Annual Review: no revisions
EmblemHealth & ConnectiCare	1/18/2023	Transfer to New Template
EmblemHealth & ConnectiCare	06/10/2020	Added J-Code(J1429): Injection, golodirsen, 10 mg (VYONDYS 53®). J-Code effective date: 07/01/2020.
EmblemHealth & ConnectiCare	04/20/2020	In Limitations/Exclusions: the geriatric population is defined as (≥ 65 years of age). There are no clinical studies demonstrating use in the geriatric population (≥ 65 years of age) as DMD is largely a disease of children and young adults.
EmblemHealth & ConnectiCare	01/01/2020	New medical policy (approved in Medical Policy Subcommittee on 02/06/2020)

References

1. Product Information: VYONDYS 53™ intravenous injection, golodirsen intravenous injection. Sarepta Therapeutics Inc (per FDA), Cambridge, MA, 2019.