Golodirsen

NDC CODE(S) 60923-0465-XX VYONDYS 53 100MG/2ML Solution (SAREPTA THERAPEUTICS)

DESCRIPTION

Golodirsen is an antisense oligonucleotide of the phosphorodiamidate morpholino oligomer (PMO) subclass. PMOs are synthetic molecules in which the five-membered ribofuranosyl rings found in natural DNA and RNA are replaced by a six-membered morpholino ring. Each morpholino ring is linked through an uncharged phosphorodiamidate moiety rather than the negatively charged phosphate linkage that is present in natural DNA and RNA. Each morpholino subunit contains one of the heterocyclic bases found in DNA (adenine, cytosine, guanine, or thymine).

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.

POLICY

- Golodirsen for the treatment of Duchenne muscular dystrophy (DMD) is considered ***medically necessary*** if the medical appropriateness criteria are met. **(See Medical Appropriateness below.)**

- Golodirsen for the treatment of other conditions/diseases is considered ***investigational.***

MEDICAL APPROPRIATENESS

INITIAL APPROVAL

- Golodirsen is considered **medically appropriate** if **ALL** of the following:
  - Individual is not on concomitant therapy with other DMD-directed antisense oligonucleotides (i.e., eteplirsen, etc.)
  - **BCBST requirement**: Individual had an inadequate response, contraindication or intolerance to viltolarsen
  - Individual has a diagnosis of Duchenne muscular dystrophy (DMD) with **ALL** of the following:
    - Confirmed mutation of the DMD gene that is amenable to exon 53 skipping
    - On a stable dose of corticosteroids, unless contraindicated or intolerance, for at least 6 months
    - Retains meaningful voluntary motor function (e.g., patient is able to speak, manipulate objects using upper extremities, ambulate, etc.)
    - Receiving physical and/or occupational therapy
  - Baseline documentation of **ANY ONE** or more of the following:
    - Dystrophin level
    - 6-minute walk test (6MWT) or other timed function tests
    - Upper limb function (ULM) test
    - North Star Ambulatory Assessment (NSAA)
    - Forced Vital Capacity (FVC) percent predicted

RENEWAL CRITERIA

- Golodirsen is considered **medically appropriate** for renewal if **ALL** of the following:
  - Individual continues to meet initial approval criteria

- Absence of unacceptable toxicity from the drug, e.g., severe hypersensitivity reactions, renal toxicity/proteinuria, etc.
- Individual has responded to therapy compared to pretreatment baseline in one or more of the following (not all-inclusive):
  - Increase in dystrophin level
  - Stability, improvement, or slowed rate of decline in 6MWT or other timed function tests
  - Stability, improvement, or slowed rate of decline in ULM test
  - Stability, improvement, or slowed rate of decline in NSAA
  - Stability, improvement, or slowed rate of decline in FVC% predicted
  - Improvement in quality of life

INDICATION(S)	DOSAGE & ADMINISTRATION
Duchenne muscular dystrophy (DMD)	Administer 30 mg/kg via intravenous infusion once weekly - Measure glomerular filtration rate prior to initiation.
Store refrigerated at 2ºC to 8ºC. Do not freeze. Protect from light.	

LENGTH OF AUTHORIZATION

Coverage will be provided for 6 months and may be renewed

Refer to **DOSAGE LIMITS** below

APPLICABLE TENNESSEE STATE MANDATE REQUIREMENTS

BlueCross BlueShield of Tennessee's Medical Policy complies with Tennessee Code Annotated Section 56-7-2352 regarding coverage of off-label indications of Food and Drug Administration (FDA) approved drugs when the off-label use is recognized in one of the statutorily recognized standard reference compendia or in the published peer-reviewed medical literature.

IMPORTANT REMINDER

We develop Medical Policies to provide guidance to Members and Providers. This Medical Policy relates only to the services or supplies described in it. The existence of a Medical Policy is not an authorization, certification, explanation of benefits or a contract for the service (or supply) that is referenced in the Medical Policy. For a determination of the benefits that a Member is entitled to receive under his or her health plan, the Member's health plan must be reviewed. If there is a conflict between the Medical Policy and a health plan, the express terms of the health plan will govern.

ADDITIONAL INFORMATION

For appropriate chemotherapy regimens, dosage information, contraindications, precautions, warnings, and monitoring information, please refer to one of the standard reference compendia (e.g., the NCCN Clinical Practice Guidelines in Oncology (NCCN Guidelines®) published by the National Comprehensive Cancer Network®, Drugdex Evaluations of Micromedex Solutions at Truven Health, or The American Hospital Formulary Service Drug Information).

SOURCES

Lexicomp Online. (2020). AHFS DI. *Fluocinolone.* Retrieved September 14, 2020 from Lexicomp Online with AHFS.

Birnkrant, D. J., Bushby, K., Bann, C. M., Apkon, S. D., Blackwell, A., Colvin, M. K., et al. (2018). Diagnosis and management of Duchenne muscular dystrophy, part 3: primary care, emergency management, psychosocial care, and transitions of care across the lifespan. *Lancet Neurology,* 4422 (18), 30026-30027.

MICROMEDEX Healthcare Series. Drugdex Evaluations. (2020, August). *Golodirsen*. Retrieved September 14, 2020 from MICROMEDEX Healthcare Series.

U. S. Food and Drug Administration. (2019, December). Center for Drug Evaluation and Research. *VYONDYS 53 (golodirsen) injection, for intravenous use.* Retrieved September 14, 2020 from https://www.accessdata.fda.gov/drugsatfda_docs/label/2019/211970s000lbl.pdf.

EFFECTIVE DATE 4/2/21

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