Policy Title:	Vyondys 53 (golodirsen) (Intravenous)		
		Department:	PHA
Effective Date:	03/06/2020		
Review Date:	3/6/2020		
Revision Date:			

Purpose: To support safe, effective and appropriate use of Vyondys 53 (golodirsen).
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Scope: Medicaid, Exchange, Medicare-Medicaid Plan (MMP)

Policy Statement:

Vyondys 53 (golodirsen) is covered under the Medical Benefit when used within the following guidelines. Use outside of these guidelines may result in non-payment unless approved under an exception process.

Procedure:

Coverage of Vyondys 53 (golodirsen) will be reviewed prospectively via the prior authorization process based on criteria below.

Initial Criteria:

- Patient has a diagnosis of Duchenne muscular dystrophy (DMD) by, or in consultation with, a neurologist with expertise in the diagnosis of DMD; AND
- Vyondys 53 is prescribed by, or in consultation with, a neurologist with expertise in the treatment of DMD; AND
- Provider submits medical records (e.g., chart notes, laboratory values) confirming the mutation of the DMD gene is amenable to exon 53 skipping; AND
- Vyondys 53 will only be covered for patients with the mutation of DMD gene that is amenable to the exon 53 skipping and Vyondys 53 will NOT be covered for other forms of muscular dystrophy; AND
- Patient has been on a stable dose of corticosteroids for at least 6 months; AND
- Provider submits the following assessments which were completed within the last 30 days:
    - Stable cardiac function with left ventricular ejection fraction (LVEF) > 50%;AND
    - Stable pulmonary function with predicted forced vital capacity (FVC) ≥ 50%;AND
- One of the following documentations are submitted with the request:
    - Submission of medical records (e.g., chart notes, laboratory values) confirming that the patient has a 6- Minute Walk Time (6MWT) ≥ 300 meters while walking independently (e.g., without side-by-side assist, cane, walker, wheelchair, etc.) prior to beginning Vyondys 53 therapy; OR

- Both of the following:
  - Submission of medical records (e.g., chart notes) confirming that the patient is ambulatory without needing an assistive device (e.g., without side-by-side assist, cane, walker, wheelchair, etc.); AND
  - One of the following:
    - Patient has achieved a score of greater than 17 on the North Star Ambulatory Assessment (NSAA); OR
    - Patient has achieved a time to rise from the floor (Gower's test) of less than 7 seconds; AND
- Vyondys 53 dosing for DMD is in accordance with the United States Food and Drug Administration approved labeling: maximum dosing of 30 mg/kg infused once weekly; AND
- Vyondys 53 is not used concomitantly with other exon skipping therapies for DMD;
- MMP members who have previously received this medication within the past 365 days are not subject to Step Therapy Requirements

Continuation of Therapy Criteria:

- Vyondys 53 is prescribed by, or in consultation with, a neurologist with expertise in the treatment of DMD; AND
- Patient is responding positively to therapy as indicted by all of the following assessed within the last 30 days:
  - Submission of medical records (e.g., chart notes) confirming that the patient is ambulatory without needing an assistive device (e.g., without side-by-side assist, cane, walker, wheelchair, etc.); AND
  - Stable cardiac function with LVEF > 50%; AND
  - Stable pulmonary function with predicted FVC ≥ 50%; AND
- Vyondys 53 dosing for DMD is in accordance with the United States Food and Drug Administration approved labeling: maximum dosing of 30 mg/kg infused once weekly; AND
- Vyondys 53 is not used concomitantly with other exon skipping therapies for DMD

Coverage durations:

- Initial coverage: 3 months
- Continuation of therapy coverage: 6 months

*** Requests will also be reviewed to National Coverage Determination (NCD) and Local Coverage Determinations (LCDs) if applicable.***

Dosage/Administration:

Indication	Dose
Duchenne muscular dystrophy	30 mg/kg via intravenous infusion once weekly

Investigational use: All therapies are considered investigational when used at a dose or for a condition other than those that are recognized as medically accepted indications as defined in any one of the following standard reference compendia: American Hospital Formulary Service Drug information (AHFS-DI), Thomson Micromedex DrugDex, Clinical Pharmacology, Wolters Kluwer Lexi-Drugs, or Peer-reviewed published medical literature indicating that sufficient evidence exists to support use. Neighborhood does not provide coverage for drugs when used for investigational purposes.

Applicable Codes:
Below is a list of billing codes applicable for covered treatment options. The below tables are provided for reference purposes and may not be all inclusive. Requests received with codes from tables below do not guarantee coverage. Requests must meet all criteria provided in the procedure section.

The following HCPCS/CPT codes are:

HCPCS/CPT Code	Description
C9399	Unclassified drugs or biologicals

References:

1. Vyondy 53 [package insert]. Cambridge, MA: Sarepta Therapeutics, Inc, December 2019.
2. Bushby K, Finkel R, Birnkrant DJ, Case LE, Clemens PR, Cripe L, et al. Diagnosis and management of Duchenne muscular dystrophy, part 1: diagnosis, and pharmacological and psychosocial management. Lancet Neurol; 2010 Jan; 9(1):77 93.

3. Bushby K, Finkel R, Birnkrant DJ, et al. (2010) Diagnosis and management of Duchenne muscular dystrophy, part 2: implementation of multidisciplinary care. Lancet Neurol; 2010 Jan; 9(2):177-189.
4. Birnkrant DJ, Bushby K, Bann CM, et al. Diagnosis and management of Duchenne muscular dystrophy, part 1: diagnosis, and neuromuscular, rehabilitation, endocrine, and gastrointestinal and nutritional management. Lancet Neurol. 2018;17(3):251-267. doi: 10.1016/S1474-4422(18)30024.
5. Exondys 51 [package insert]. Cambridge, MA: Sarepta Therapeutics, Inc, October 2018.
6. Frank DE, Mercuri E, Servais, L, et al. Golodirsen induces exon skipping leading to sarcolemmal dystrophin expression in patients with genetic mutations amenable to exon 53 skipping. Poster presented at: Annual Clinical Genetics Meeting of the American College of Medical Genetics and Genomics; April 2-6, 2019; Seattle, WA
7. Muntoni F, Frank DE, Morgan J, et al. Golodirsen induces exon skipping leading to sarcolemmal dystrophin expression in patients with genetic mutations amenable to exon 53 skipping [abstract]. Neuromuscul Disord. 2018;28:S5. Abstract D01.
8. Muntoni F, Frank DE, Sardone V, et al. SRP-4053 induces exon skipping leading to sarcolemmal dystrophin expression in Duchenne muscular dystrophy patients amenable to exon 53 skipping. Poster presented at: 22nd International Annual Congress of the World Muscle Society; October 3-7 2017; Saint Malo, France.
9. Study of SRP-4045 and SRP-4053 in DMD Patients (ESSENCE) https://clinicaltrials.gov/ct2/show/NCT02500381?term=golodirsen&cond=Duchenne+Muscular+Dystrophy&rank= 3. Accessed July 22, 2019.
10. Institute for Clinical and Economic Review (ICER). Deflazacort, eeplirsen, and golodirsen for Duchenne muscular dystrophy: Effectiveness and value: Evidence Report. https://icer-review.org/wpcontent/uploads/2018/12/ICER_DMD_Evidence Report_071119.pdf. July 11, 2019,Accessed July 22, 2019