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| Title: Duchenne Muscular Dystrophy Agents | Division: Medical Management Department: Utilization Management |
| Approval Date: 10/8/17 | LOB: Medicaid, HIV SNP, HARP, CHP, Medicare, UltraCare, MetroPlus Gold, Goldcare I&II, Essential Plan, QHP |
| Effective Date: 10/8/17 | Policy Number: UM-MP216 |
| Review Date: 2/28/2023 | Cross Reference Number: |
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1. POLICY DESCRIPTION:

Duchenne Muscular Dystrophy Agents

2. RESPONSIBLE PARTIES:

Medical Management Administration, Utilization Management, Integrated Care Management, Pharmacy, Claim Department, Providers Contracting.

3. DEFINITIONS:

Exondys 51 (eteplirsen) is indicated for Duchenne muscular dystrophy in patients who have a confirmed mutation of DMD gene that is amenable to exon 51 skipping. Eteplirsen binds to exon 51 of dystrophin pre-messenger RNA (mRNA) to exclude this exon during mRNA processing. Exon skipping allows an internally truncated dystrophin protein to be produced. Amondys 45 (casmieresen) is an antisense oligonucleotide indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients who have a confirmed mutation of the DMD gene that is amenable to exon 45 skipping. Vyondys 53 (golodirsen) is an antisense oligonucleotide indicated 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. Viltespo (viltolarsen) is an antisense oligonucleotide indicated 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.

4. POLICY:

Duchenne muscular dystrophy agents will be considered medically necessary when the following conditions of coverage have been met:

Initial Request:

- A. Patient must have a diagnosis of DMD **AND**
- B. Documentation of genetic testing must confirm the DMD gene mutation of the patient is amenable to exon 45, 51, or 53 skipping **AND**
- C. Documentation must confirm a stable dose of corticosteroids prior to starting therapy or a documented reason not to be on corticosteroids **AND**
- D. Documentation indicates kidney function testing prior to starting therapy (except for eteplirsen) **AND**
- E. Patient is not concurrently being treated with another exon skipping therapy for DMD
- F. Dosing must be in accordance with the FDA labeling:
 - i. Exondys 51, Amondys 45, Vyondys 53: 30 mg/kg weekly

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- ii. Viltepso: 80 mg/kg weekly

Approved for 24 weeks

Renewal Request

- A. The patient has a diagnosis of Duchenne muscular dystrophy (DMD) **AND**
 - i. All initial conditions of coverage have been met.* **AND**
 - ii. Dose is within FDA approved labeling

Approve for 24 weeks

5. LIMITATIONS/ EXCLUSIONS:

All other uses, including other forms of muscular dystrophy, are considered experimental/investigational and are not a covered benefit.

6. APPLICABLE PROCEDURE CODES:

| CPT | Description |
|-------|-------------------------------|
| J1428 | Injection, eteplirsen, 10 mg |
| J1426 | Injection, casmiersen, 10 mg |
| J1429 | Injection, golodirsen, 10 mg |
| J1427 | Injection, Viltolarsen, 10 mg |
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7. APPLICABLE DIAGNOSIS CODES:

| CODE | Description |
|--------|---------------------------------------|
| G71.01 | Duchenne or Becker muscular dystrophy |
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8. REFERENCES:

1. Exondys 51 [package insert]. Cambridge, MA: Sarepta Therapeutics, Inc, September 2016.
2. Anthony K, Feng L, Arechavala-Gomez V, et al. Exon skipping quantification by quantitative reverse transcription polymerase chain reaction in Duchenne muscular dystrophy patients treated with the antisense oligomer eteplirsen. Hum Gene Ther Methods. 2012 Oct;23(5):336-45.
3. 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.
4. 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.
5. Gold Standard, Inc. Exondys 51. Clinical Pharmacology [database online]. Available at: <http://www.clinicalpharmacology.com>.
6. Mendell JR, Rodino-Klapac LR, Sahenk Z, et al. Eteplirsen for the treatment of Duchenne muscular dystrophy. Ann Neurol. 2013 Nov;74(5):637-47.
7. Sarepta Therapeutics. Confirmatory Study of Eteplirsen in DMD Patients (PROMOVI). In: ClinicalTrials.gov [Internet]. Bethesda (MD): National Library of Medicine (US). 2000- [cited 2017 Jan 27]. Available from: https://clinicaltrials.gov/show/NCT_02255552 NLM Identifier: NCT 02255552.
8. Mendell JR, Goemans N, Lowes LP, et al. Longitudinal effect of eteplirsen versus historical control on ambulation in Duchenne muscular dystrophy. Ann Neurol. 2016 Feb;79(2):257-71.
9. American Society of Health System Pharmacists. AHFS Drug Information. Bethesda, MD. Electronic Version 2016. <http://www.online.lexi.com>. Accessed May 9, 2017.
10. Amondys 45 [package insert]. Cambridge, MA; Sarepta Therapeutics, Inc.: February 2021.
11. Vyondys 53 [package insert]. Cambridge, MA; Sarepta Therapeutics, Inc: February 2021.
12. Viltepso [package insert]. Paramus, NJ; NS Pharma, Inc, March 2021
13. New York State Department of Health Medicaid Update January 2022, Volume 38, Number 1. Available at: https://health.ny.gov/health_care/medicaid/program/update/2022/docs/mu_no1_jan22.pdf

REVISION LOG:

| REVISIONS | DATE |
|------------------|-------------|
| Creation date | |



Policy and Procedure

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| Annual Review | 10/25/2019 |
| Annual Review | 9/22/2021 |
| DOH Update Review | 2/17/2022 |
| Annual Review | 2/28/2023 |

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| Approved: | Date: | Approved: | Date: |
| Glendon Henry, MD Senior Medical Director | | Sanjiv Shah, MD Chief Medical Officer | |

Medical Guideline Disclaimer:

Property of Metro Plus Health Plan. All rights reserved. The treating physician or primary care provider must submit MetroPlus Health Plan clinical evidence that the patient meets the criteria for the treatment or surgical procedure. Without this documentation and information, Metroplus Health Plan will not be able to properly review the request for prior authorization. The clinical review criteria expressed in this policy reflects how MetroPlus Health Plan determines whether certain services or supplies are medically necessary. MetroPlus Health Plan 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). MetroPlus Health Plan 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 MetroPlus Health Plan, as some programs exclude coverage for services or supplies that MetroPlus Health Plan considers medically necessary. If there is a discrepancy between this guidelines and a member's benefits program, the benefits program will govern. In addition, coverage may be mandated by applicable legal requirements



Policy and Procedure

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of a state, the Federal Government or the Centers for Medicare & Medicaid Services (CMS) for Medicare and Medicaid members.

All coding and website links are accurate at time of publication.

MetroPlus Health Plan has adopted the herein policy in providing management, administrative and other services to our members, related to health benefit plans offered by our organization.