pennsylvania
DEPARTMENT OF HUMAN SERVICES

MEDICAL ASSISTANCE BULLETIN

ISSUE DATE	EFFECTIVE DATE	NUMBER
December 14, 2020	January 5, 2021	*See below

SUBJECT

Prior Authorization of Duchenne Muscular Dystrophy (DMD) Antisense Oligonucleotides – Pharmacy Services

BY

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IMPORTANT REMINDER: All providers must revalidate the Medical Assistance (MA) enrollment of each service location every 5 years. Providers should log into PROMISe to check the revalidation dates of each service location and submit revalidation applications at least 60 days prior to the revalidation dates. Enrollment (revalidation) applications may be found at: https://www.dhs.pa.gov/providers/Providers/Pages/PROMISe-Enrollment.aspx.

PURPOSE:

The purpose of this bulletin is to:

1. Inform providers that the Department of Human Services (Department) will require prior authorization of prescriptions for Duchenne Muscular Dystrophy (DMD) Antisense Oligonucleotides.
2. Issue handbook pages that include the requirements for prior authorization and the type of information needed to evaluate the medical necessity of prescriptions for DMD Antisense Oligonucleotides submitted for prior authorization.

SCOPE:

This bulletin applies to all licensed pharmacies and prescribers enrolled in the Medical Assistance (MA) Program and providing services in the fee-for-service delivery system. Providers rendering services in the MA managed care delivery system should address any questions related to DMD Antisense Oligonucleotides to the appropriate managed care organization.

*01-20-55	09-20-54	27-20-50	33-20-51
02-20-48	11-20-48	30-20-47	
03-20-48	14-20-49	31-20-55	
08-20-58	24-20-49	32-20-47	

COMMENTS AND QUESTIONS REGARDING THIS BULLETIN SHOULD BE DIRECTED TO:

The appropriate toll-free number for your provider type.

Visit the Office of Medical Assistance Programs Web site at https://www.dhs.pa.gov/providers/Providers/Pages/Health%20Care%20for%20Providers/Contact-Information-for-Providers.aspx.

BACKGROUND:

The Department's Drug Utilization Review (DUR) Board meets semi-annually to review provider prescribing and dispensing practices for efficacy, safety, and quality and to recommend interventions for prescribers and pharmacists through the Department's Prospective Drug Use Review and Retrospective Drug Use Review programs.

DISCUSSION:

Exondys 51 (eteplirsen) is an antisense oligonucleotide that was approved by the U.S. Food and Drug Administration (FDA) in 2016 for the treatment of DMD. The FDA recently approved two additional antisense oligonucleotides, Vyondys 53 (golodirsen) and Viltepso (viltolarsen), for the treatment of DMD. The Department has required prior authorization for Exondys 51 (eteplirsen) since June 6, 2017.

During the October 21, 2020, meeting, the DUR Board recommended a requirement for prior authorization and corresponding guidelines to determine medical necessity of prescriptions for Vyondys 53 (golodirsen) and Viltepso (viltolarsen). The board also recommended combining the requirements for prior authorization for the three drugs in this class into one guideline titled DMD Antisense Oligonucleotides.

The guidelines to determine medical necessity of prescriptions for DMD Antisense Oligonucleotides, as recommended by the DUR Board, were subject to public review and comment and subsequently approved for implementation by the Department.

PROCEDURE:

The procedures for prescribers to request prior authorization of DMD Antisense Oligonucleotides are located in SECTION I of the Prior Authorization of Pharmaceutical Services Handbook. The Department will take into account the elements specified in the clinical review guidelines (which are included in the provider handbook pages in the SECTION II chapter related to DMD Antisense Oligonucleotides) when reviewing the prior authorization request to determine medical necessity.

As set forth in 55 Pa. Code § 1101.67(a), the procedures described in the handbook pages must be followed to ensure appropriate and timely processing of prior authorization requests for drugs that require prior authorization.

ATTACHMENTS:

Prior Authorization of Pharmaceutical Services Handbook - Updated pages

RESOURCES:

Prior Authorization of Pharmaceutical Services Handbook – SECTION I
Pharmacy Prior Authorization General Requirements

https://www.dhs.pa.gov/providers/Pharmacy-Services/Pages/Pharmacy-Prior-Authorization-General-Requirements.aspx

Prior Authorization of Pharmaceutical Services Handbook – SECTION II
Pharmacy Prior Authorization Guidelines
https://www.dhs.pa.gov/providers/Pharmacy-Services/Pages/Clinical-Guidelines.aspx

I. Requirements for Prior Authorization of Duchenne Muscular Dystrophy (DMD) Antisense Oligonucleotides

A. Prescriptions That Require Prior Authorization

All prescriptions for Duchenne Muscular Dystrophy (DMD) Antisense Oligonucleotides must be prior authorized.

B. Review of Documentation for Medical Necessity

In evaluating a request for prior authorization of a prescription for a DMD Antisense Oligonucleotide, the determination of whether the requested prescription is medically necessary will take into account whether the beneficiary:

1. Is prescribed the DMD Antisense Oligonucleotide for the treatment of a diagnosis that is indicated in the U.S. Food and Drug Administration (FDA)-approved package labeling OR a medically accepted indication; **AND**

2. Is prescribed the DMD Antisense Oligonucleotide by or in consultation with a neurologist with experience treating Duchenne muscular dystrophy; **AND**

3. Has documentation of a baseline evaluation, including a standardized assessment of motor function, by a neurologist with experience treating Duchenne muscular dystrophy; **AND**

4. Will receive concurrent corticosteroids unless contraindicated or intolerant**.**

   NOTE: If the beneficiary does not meet the clinical review guidelines listed above but, in the professional judgment of the physician reviewer, the services are medically necessary to meet the medical needs of the beneficiary, the request for prior authorization will be approved.

FOR RENEWALS OF PRIOR AUTHORIZATION FOR DMD ANTISENSE OLIGONUCLEOTIDES: The determination of medical necessity of a request for renewal of a prior authorization for a DMD Antisense Oligonucleotide that was previously approved will take into account whether the beneficiary:

1. Is prescribed the DMD Antisense Oligonucleotide by or in consultation with a neurologist with experience treating Duchenne muscular dystrophy; **AND**

2. Has documentation of an annual evaluation, including an assessment of motor function ability, by a neurologist with experience treating Duchenne muscular dystrophy; **AND**

3. Continues to benefit from the DMD Antisense Oligonucleotide based on the prescriber's assessment; **AND**

4. Will receive concurrent corticosteroids unless contraindicated or intolerant.

   NOTE: If the beneficiary does not meet the clinical review guidelines listed above but, in the professional judgment of the physician reviewer, the services are medically necessary to meet the medical needs of the beneficiary, the request for prior authorization will be approved.

C. Clinical Review Process

Prior authorization personnel will review the request for prior authorization and apply the clinical guidelines in Section B. above to assess the medical necessity of the request for a prescription for a DMD Antisense Oligonucleotide. If the guidelines in Section B. are met, the reviewer will prior authorize the prescription. If the guidelines are not met, the prior authorization request will be referred to a physician reviewer for a medical necessity determination. Such a request for prior authorization will be approved when, in the professional judgment of the physician reviewer, the services are medically necessary to meet the medical needs of the beneficiary.

D. References

1. Brushby, K. et.al Diagnosis and management of Duchenne muscular dystrophy, part 1: diagnosis, and pharmacological and psychosocial management. *The Lancet*; Published online November 30, 2009
2. Darras, B.T. Duchenne and Becker muscular dystrophy; Clinical features and diagnosis, Up To Date. Accessed October 1, 2020.
3. Gloss, D. et.al. Practice guideline update summary: Corticosteroid treatment of Duchenne muscular dystrophy Report of the Guideline Development Subcommittee of the American Academy of Neurology. Neurology 2016; 86: 465-472.
4. Darras, B.T. Duchenne and Becker muscular dystrophy: Glucocorticoid and disease-modifying treatment. Up To Date. Accessed October 1, 2020.
5. Exondys 51 (eteplirsen) prescribing information. Sarepta Therapeutics, ~~September 2016~~ July 2020.
6. Kinane, T.B, Mayer, O.H, Duda, P.W, et.al. Long-Term Pulmonary Function in Duchenne Muscular Dystrophy: Comparison of Eteplirsen-Treated Patients to Natural History. Journal of Neuromuscular Diseases 5 (2018) 47–58.
7. Mendell, J.R, Goemans, N, Lowes, L.P, et.al. Longitudinal Effect of Eteplirsen versus Historical Control on Ambulation in Duchenne Muscular Dystrophy. Annals of Neurology 2016;79:257–271.
8. Mendell J.R, Rodino-Klapac L.R, Sahenk Z, et.al. Eteplirsen Study G. Eteplirsen for the treatment of Duchenne muscular dystrophy. Annals of Neurology.2013;74(5):637-47.
9. Institute for Clinical and Economic Review, 2019. Final Evidence Report - Deflazacort, Eteplirsen, and Golodirsen for Duchenne Muscular Dystrophy.
10. Vyondys 53 (golodirsen) prescribing information. Sarepta Therapeutics, December 2019.
11. Viltepso (viltolarsen) prescribing information. Nippon Shinyaku Pharma, Inc. August 2020.