pennsylvania
DEPARTMENT OF HUMAN SERVICES

MEDICAL ASSISTANCE BULLETIN

ISSUE DATE	EFFECTIVE DATE	NUMBER
December 4, 2019	January 1, 2020	*See below

SUBJECT

Prior Authorization of Enzyme Replacements, Gaucher Disease – Pharmacy Services

BY

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Office of Medical Assistance Programs

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 issue updated handbook pages that include the requirements for prior authorization and the type of information needed to evaluate the medical necessity of prescriptions for Enzyme Replacements, Gaucher Disease 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 Enzyme Replacements, Gaucher Disease to the appropriate managed care organization.

BACKGROUND:

The Department of Human Services' (Department) Drug Utilization Review (DUR) Board meets semi-annually to review provider prescribing and dispensing practices for

*01-19-111	09-19-107	27-19-106	33-19-108
02-19-105	11-19-104	30-19-103	
03-19-104	14-19-103	31-19-111	
08-19-114	24-19-106	32-19-103	

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.

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:

During the September 13, 2019, meeting, the DUR Board recommended that all Enzyme Replacements, Gaucher Disease agents require prior authorization. The board also recommended revising the guideline for non-preferred Enzyme Replacements, Gaucher Disease agents to take into account the beneficiary's diagnosis and adding guidelines for requests of renewal of prior authorization for these agents.

The revisions to the guidelines to determine medical necessity of Enzyme Replacements, Gaucher Disease, 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 Enzyme Replacements, Gaucher Disease 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 Enzyme Replacements, Gaucher Disease) 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

I. Requirements for Prior Authorization of Enzyme Replacements, Gaucher Disease

A. Prescriptions That Require Prior Authorization

All prescriptions for Enzyme Replacements, Gaucher Disease agents must be prior authorized.

B. Review of Documentation for Medical Necessity

In evaluating a request for prior authorization of a prescription for an Enzyme Replacements, Gaucher Disease agent, the determination of whether the requested prescription is medically necessary will take into account whether the beneficiary:

1. Is prescribed the Enzyme Replacements, Gaucher Disease agent 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 age-appropriate according to FDA-approved package labeling, nationally recognized compendia, or peer-reviewed medical literature; **AND**

3. Is prescribed a dose that is consistent with FDA-approved package labeling, nationally recognized compendia, or peer-reviewed medical literature; **AND**

4. Does not have a history of a contraindication to the prescribed medication; **AND**

5. Is prescribed the Enzyme Replacements, Gaucher Disease agent by or in consultation with a specialist in the treatment of Gaucher disease; **AND**

6. For a non-preferred Enzyme Replacements, Gaucher Disease agent, has a history of therapeutic failure, contraindication, or intolerance of the preferred Enzyme Replacements, Gaucher Disease agents approved or medically accepted for the beneficiary's indication. See the Preferred Drug List (PDL) for the list of preferred Enzyme Replacements, Gaucher Disease agents at: https://papdl.com/preferred-drug-list; **AND**

7. For a diagnosis of Gaucher disease, has documentation of **both** of the following:

   a. **One** of the following:

      i. Enzyme assay demonstrating a deficiency of beta-glucocerebrosidase (glucosidase) activity
      ii. DNA testing confirming the diagnosis

   b. **One** of the following:

      i. Anemia,
      ii. Bone disease,

iii. Hepatomegaly,
iv. Interstitial lung disease,
v. Splenomegaly,
vi. Thrombocytopenia;

AND

8. If a prescription for an Enzyme Replacements, Gaucher Disease agent is in a quantity that exceeds the quantity limit, the determination of whether the prescription is medically necessary will also take into account the guidelines set forth in the Quantity Limits Chapter. The list of drugs that are subject to quantity limits, with accompanying quantity limits, is available at: https://www.dhs.pa.gov/providers/Pharmacy-Services/Pages/Quantity-Limits-and-Daily-Dose-Limits.aspx.

   NOTE: If the beneficiary does not meet the clinical review guidelines listed above but, in the professional judgement 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 ENZYME REPLACEMENTS, GAUCHER DISEASE AGENTS: The determination of medical necessity of a request for renewal of a prior authorization for an Enzyme Replacements, Gaucher Disease agent that was previously approved will take into account whether the beneficiary:

1. Is prescribed a dose that is consistent with FDA-approved package labeling, nationally recognized compendia, or peer-reviewed medical literature; **AND**

2. Is prescribed the Enzyme Replacements, Gaucher Disease agent by or in consultation with a specialist in the treatment of Gaucher disease; **AND**

3. Has documentation of improvement in disease severity since initiating treatment with the requested Enzyme Replacements, Gaucher Disease agent; **AND**

4. If a prescription for an Enzyme Replacements, Gaucher Disease agent is in a quantity that exceeds the quantity limit, the determination of whether the prescription is medically necessary will also take into account the guidelines set forth in the Quantity Limits Chapter. The list of drugs that are subject to quantity limits, with accompanying quantity limits, is available at: https://www.dhs.pa.gov/providers/Pharmacy-Services/Pages/Quantity-Limits-and-Daily-Dose-Limits.aspx.

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 a prescription for an Enzyme Replacements, Gaucher Disease agent. 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. Wang RY, Bodamer OA, et al. ACMG Standards and Guidelines. Lysosomal storage diseases: Diagnostic confirmation and management of presymptomatic individuals. *Genetics in Medicine*;13, (5), May 2011.
2. Hughes D, Sidransky E. Gaucher disease: Pathogenesis, clinical manifestations, and diagnosis. In: UpToDate [internet database]. Hahn SH, TePas E, eds. Waltham, MA: UpToDate. Updated May 21, 2019. Accessed July 29, 2019.
3. Hughes D, Sidransky E. Gaucher disease: Treatment. In: UpToDate [internet database]. Hahn SH, TePas E, eds. Waltham, MA: UpToDate. Updated April 10, 2018. Accessed July 29, 2019.