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 Cystic Fibrosis Transmembrane Regulator (CFTR) Modulators – 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:

1. Inform providers that the Department of Human Services (Department) will require prior authorization of prescriptions for Cystic Fibrosis Transmembrane Regulator (CFTR) Modulators.
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 CFTR Modulators 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 CFTR Modulators to the appropriate managed care organization.

BACKGROUND:

*01-20-49	09-20-48	27-20-44	33-20-45
02-20-42	11-20-42	30-20-41	
03-20-42	14-20-43	31-20-49	
08-20-52	24-20-43	32-20-41	

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.

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:

Trikafta (elexacaftor/tezacaftor/ivacaftor and ivacaftor) is the fourth CFTR Modulator approved by the U.S. Food and Drug Administration (FDA) for the treatment of cystic fibrosis. The Department has required prior authorization for the three CFTR Modulators previously approved by the FDA – Kalydeco (ivacaftor), Orkambi (lumacaftor/ivacaftor), and Symdeko (tezacaftor/ivacaftor) – since December 3, 2012, November 10, 2016, and May 1, 2017, respectively.

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 Trikafta (elexacaftor/tezacaftor/ivacaftor and ivacaftor). The board also recommended combining the guidelines for the four drugs in this therapeutic class into one handbook chapter titled CFTR Modulators.

The guidelines to determine medical necessity of prescriptions for CFTR Modulators, 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 CFTR Modulators 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 CFTR Modulators) 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 CFTR Modulators

A. Prescriptions That Require Prior Authorization

All prescriptions for Cystic Fibrosis Transmembrane Conductance Regulator (CFTR) Modulators must be prior authorized.

B. Review of Documentation for Medical Necessity

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

1. Is prescribed the CFTR Modulator for an indication that is included in the U.S. Food and Drug Administration (FDA)-approved package labeling OR a medically accepted indication; **AND**

2. Has a documented CFTR gene mutation consistent with FDA-approved package labeling; **AND**

3. Is age-appropriate according to FDA-approved package labeling, nationally recognized compendia, or peer-reviewed medical literature; **AND**

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

5. Is prescribed the CFTR Modulator by or in consultation with a pulmonologist or cystic fibrosis specialist; **AND**

6. Had all potential drug interactions addressed by the prescriber (such as discontinuation of the interacting drug, dose reduction of the interacting drug, or counseling of the beneficiary of the risks associated with the use of both medications when they interact); **AND**

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

8. Has documentation of baseline lab results as recommended in the FDA-approved package labeling; **AND**

9. If a prescription for a CFTR Modulator is for 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 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 CFTR MODULATORS: The determination of medical necessity of a request for renewal of a prior authorization for a CFTR Modulator that was previously approved will take into account whether the beneficiary:

1. Is prescribed the CFTR Modulator by or in consultation with a pulmonologist or cystic fibrosis specialist; **AND**

2. Had all potential drug interactions addressed by the prescriber (such as discontinuation of the interacting drug, dose reduction of the interacting drug, or counseling of the beneficiary of the risks associated with the use of both medications when they interact); **AND**

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

4. Based on the prescriber's assessment, continues to benefit from the CFTR Modulator; **AND**

5. Has documentation of results of recent lab monitoring as recommended in the FDA-approved package labeling; **AND**

6. Is continuing treatment with the prescribed CFTR Modulator based on recent lab results as recommended in the FDA-approved package labeling; **AND**

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

8. If a prescription for a CFTR Modulator is for 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 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 a prescription for a

CFTR Modulator. 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. Dose and Duration of Therapy

Requests for prior authorization of CFTR Modulators will be approved as follows:

1. Initial requests for prior authorization of CFTR Modulators and subsequent requests during the first year of therapy will be approved for up to 3 months of therapy.
2. Renewals of requests for prior authorization of CFTR Modulators after the first year of therapy that were previously approved will be approved for up to 12 months.

E. References

1. Institute for Clinical and Economic Review. Modulator Treatments for Cystic Fibrosis: Effectiveness and Value. Published June 7, 2018.
2. Katkin JP. Cystic fibrosis: genetics and pathogenesis. UpToDate. Accessed August 21, 2018.
3. Symdeko (package insert). Vertex Pharmaceuticals Incorporated, Boston, MA; December 2019.
4. Kalydeco (package insert). Vertex Pharmaceuticals Incorporated, Cambridge, MA; April 2019.
5. Katlin, HP. Cystic fibrosis: clinical manifestations and diagnosis. UpToDate. Accessed August 14, 2015.
6. Orkambi (package insert). Vertex Pharmaceuticals Incorporated, Cambridge, MA; July 2019.
7. Simon RH. Cystic fibrosis: overview of the treatment of lung disease. UpToDate. Accessed August 14, 2015.
8. TRIKAFTA (package insert). Vertex Pharmaceuticals Incorporated, Boston, MA: January 2020.