Pharmacy Medical Necessity Guidelines:
Cystic Fibrosis Medications

Effective: August 1, 2023

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Applies to:

Commercial Products
☒ Harvard Pilgrim Health Care Commercial products; Fax: 617-673-0988
☒ Tufts Health Plan Commercial products; Fax: 617-673-0988
CareLinkSM – Refer to CareLink Procedures, Services and Items Requiring Prior Authorization

Public Plans Products
☒ Tufts Health Direct – A Massachusetts Qualified Health Plan (QHP) (a commercial product); Fax: 617-673-0988

Note: While you may not be the provider responsible for obtaining prior authorization, as a condition of payment you will need to ensure that prior authorization has been obtained.

Overview

Food and Drug Administration – Approved Indications
The cystic fibrosis transmembrane conductance regulator (CFTR) potentiators are indicated for:
• Kalydeco (ivacaftor)
  Treatment of cystic fibrosis (CF) in patients age 1 month and older who have one mutation in the CFTR gene that is responsive to ivacaftor based on clinical and/or in vitro assay data
• Orkambi (lumacaftor/ivacaftor)
  Treatment of CF in patients aged 1 year and older who are homozygous for the F508del mutation in the CFTR gene. The efficacy and safety of Orkambi (lumacaftor/ivacaftor) have not been established in patients with CF other than those homozygous for the F80del mutation.
• Symdeko (tezacaftor/ivacaftor)
  Treatment of patients with CF aged 6 years and older who are homozygous for the F508del mutation or who have at least one mutation in the CFTR gene that is responsive to tezacaftor/ivacaftor based on in vitro data and/or clinical evidence.
• Trikafta (elexacaftor/tezacaftor/ivacaftor and ivacaftor)
  Treatment of CF in patients aged 2 years and older who have at least one F508del mutation in the CFTR gene or a mutation in the CFTR gene that is responsive based on in vitro data.

Clinical Guideline Coverage Criteria
The plan may authorization coverage of a Cystic Fibrosis Medication when all of the following criteria are met:

Kalydeco
1. Documented diagnosis of cystic fibrosis
   AND
2. Prescribed by or in consultation with a pulmonologist or physician specializing in the treatment of cystic fibrosis
   AND
3. The patient is at least 1 month of age
   AND
4. Documentation of a cystic fibrosis mutation test confirming one of the CFTR gene mutations responsive to Kalydeco
Orkambi
1. Documented diagnosis of cystic fibrosis
2. Prescribed by or in consultation with a pulmonologist or physician specializing in the treatment of cystic fibrosis
3. The patient is at least 1 year of age
4. Documentation confirming the patient is homozygous for the Phe508del (F508del) mutation in the CFTR gene

Symdeko
1. Documented diagnosis of cystic fibrosis
2. Prescribed by or in consultation with a pulmonologist or physician specializing in the treatment of cystic fibrosis
3. The patient is at least 6 years of age
4. Documentation of one (1) of the following:
   a. Patient is homozygous for the Phe508del (F508del) mutation in the CFTR gene
   b. Patient has at least one mutation in the CFTR gene that is responsive to Symdeko

Trikafta
1. Documented diagnosis of cystic fibrosis
2. Prescribed by or in consultation with a pulmonologist or physician specializing in the treatment of cystic fibrosis
3. The patient is at least 2 years of age
4. Documentation of one (1) of the following:
   a. Patient has at least one Phe508del (F508del) mutation in the CFTR gene
   b. Patient has at least one mutation in the CFTR gene that is responsive to Trikafta

Limitations
1. For a non-formulary medication request, please refer to the Pharmacy Medical Necessity Guidelines for Formulary Exceptions and submit a formulary exception request to the plan as indicated.

Codes
None

References

Approval And Revision History
September 13, 2022: Reviewed by the Pharmacy & Therapeutics Committee.

- July 11, 2023: Expanded age requirements of Kalydeco and Trikafta based on updated package labeling (effective August 1, 2023).

Background, Product and Disclaimer Information
Pharmacy Medical Necessity Guidelines have been developed for determining coverage for plan benefits and are published to provide a better understanding of the basis upon which coverage decisions are made. The plan makes coverage decisions on a case-by-case basis considering the individual member's health care needs. Pharmacy Medical Necessity Guidelines are developed for selected therapeutic classes or drugs found to be safe, but proven to be effective in a limited, defined population of patients or clinical circumstances. They include concise clinical coverage criteria based on current literature review, consultation with practicing physicians in the service area who are medical experts in the particular field, FDA and other government agency policies, and standards adopted by national accreditation organizations. The plan revises and updates Pharmacy Medical Necessity Guidelines annually, or more frequently if new evidence becomes available that suggests needed revisions.

For self-insured plans, coverage may vary depending on the terms of the benefit document. If a discrepancy exists between a Pharmacy Medical Necessity Guideline and a self-insured Member's benefit document, the provisions of the benefit document will govern.

Treating providers are solely responsible for the medical advice and treatment of members. The use of this policy is not a guarantee of payment or a final prediction of how specific claim(s) will be adjudicated. Claims payment is subject to member eligibility and benefits on the date of service, coordination of benefits, referral/authorization and utilization management guidelines when applicable, and adherence to plan policies and procedures and claims editing logic.