# MEDICAL POLICY STATEMENT

| Original Effective Date | Next Annual Review Date | Last Review / Revision Date |
|---|---|---|
| 10/06/2015 | 10/06/2016 | 10/06/2015 |
| **Policy Name** | | **Policy Number** |
| **Cystic Fibrosis** | | **SRx-0025** |

Medical Policy Statements prepared by CSMG Co. and its affiliates (including CareSource) are derived from literature based on and supported by clinical guidelines, nationally recognized utilization and technology assessment guidelines, other medical management industry standards, and published MCO clinical policy guidelines. Medically necessary services include, but are not limited to, those health care services or supplies that are proper and necessary for the diagnosis or treatment of disease, illness, or injury and without which the patient can be expected to suffer prolonged, increased or new morbidity, impairment of function, dysfunction of a body organ or part, or significant pain and discomfort. These services meet the standards of good medical practice in the local area, are the lowest cost alternative, and are not provided mainly for the convenience of the member or provider. Medically necessary services also include those services defined in any Evidence of Coverage documents, Medical Policy Statements, Provider Manuals, Member Handbooks, and/or other policies and procedures.

Medical Policy Statements prepared by CSMG Co. and its affiliates (including CareSource) do not ensure an authorization or payment of services. Please refer to the plan contract (often referred to as the Evidence of Coverage) for the service(s) referenced in the Medical Policy Statement. If there is a conflict between the Medical Policy Statement and the plan contract (i.e., Evidence of Coverage), then the plan contract (i.e., Evidence of Coverage) will be the controlling document used to make the determination.

For Medicare plans please reference the below link to search for Applicable National Coverage Descriptions (NCD) and Local Coverage Descriptions (LCD):

## A. SUBJECT

**Cystic Fibrosis**

- Aminoglycoside Antibiotic
  - **Bethkis** (tobramycin Inhalation solution)
  - **Tobi** (tobramycin Inhalation solution & podhaler)
  - **Kitabis** (tobramycin solution)
- Monobactam Antibiotic
  - **Cayston** (aztreonam inhalation solution)
- Transmembrane conductance regulator (CFTR)
  - **Kalydeco** (ivacaftor)
  - **Orkambi** (lumacaftor/ivacaftor**)**
- DNase enzyme
  - **Pulmozyme** (dornase alfa inhalation solution)

## B. BACKGROUND

The CareSource Medication Policies are therapy class policies that are used as a guide when determining health care coverage for our members with benefit plans covering prescription drugs. Medication Policies are written on selected prescription drugs requiring prior authorization or Step-Therapy. The Medication Policy is used as a tool to be interpreted in conjunction with the member's specific benefit plan.

The intent of the Cystic Fibrosis Program is to encourage appropriate selection of patients for therapy according to product labeling and/or clinical guidelines and/or clinical studies, and also to encourage use of preferred agents.

## C. DEFINITIONS

N/A

**D. POLICY**

I. CareSource will approve the use of **Bethkis** (tobramycin Inhalation solution), **Tobi** (tobramycin Inhalation solution & podhaler), **Cayston** (aztreonam inhalation solution), **Kitabis** (tobramycin solution), **Kalydeco** (ivacaftor), **Orkambi** (lumacaftor/ivacaftor), and **Pulmozyme** (dornase alfa inhalation solution) and consider their use as medically necessary when the following criteria have been met for Cystic Fibrosis:

A. **Bethkis** (tobramycin Inhalation solution) is an aminoglycoside antibiotic used for the treatment of lung infections in patients with cystic fibrosis. **Bethkis** (tobramycin Inhalation solution) will be considered medically necessary when the following criteria have been met:
   1. Patient has a diagnosis of Cystic Fibrosis
   2. Patient has a positive culture for Pseudomonas aeruginosa
   3. Prescribed by a pulmonologist or an infectious disease specialist
   4. Patient is 6 years of age and older

B. **Tobi** (tobramycin Inhalation solution & podhaler) and generic tobramycin is an aminoglycoside antibiotic used for the treatment of lung infections in patients with cystic fibrosis. **Tobi** (tobramycin Inhalation solution & podhaler) will be considered medically necessary when the following criteria have been met:
   1. Patient has a diagnosis of Cystic Fibrosis
   2. Patient has a positive culture for Pseudomonas aeruginosa
   3. Prescribed by a pulmonologist or an infectious disease specialist
   4. Patient is 6 years of age and older

C. **Kitabis** (tobramycin solution) is an aminoglycoside antibiotic used for the treatment of lung infections in patients with cystic fibrosis. **Kitabis** (tobramycin solution) will be considered medically necessary when the following criteria have been met:
   1. Patient has a diagnosis of Cystic Fibrosis
   2. Patient has a positive culture for Pseudomonas aeruginosa
   3. Prescribed by a pulmonologist or an infectious disease specialist
   4. Patient is 6 years of age and older
   5. Patient has had a trial of tobramycin solution and is documented in clinical notes to be ineffective

D. **Cayston** (aztreonam inhalation solution) is an antibiotic used for the treatment of lung infections in patients with cystic fibrosis. **Cayston** (aztreonam inhalation solution) will be considered medically necessary when the following criteria have been met:
   1. Patient has a diagnosis of Cystic Fibrosis
   2. Patient has a positive culture for Pseudomonas aeruginosa
   3. Prescribed by a pulmonologist or an infectious disease specialist
   4. Patient is 7 years of age and older
   5. Patient with forced expiratory volume in 1 second ($FEV_1$) > 25% or ≤ 75% predicted

E. **Kalydeco** (ivacaftor) is a cystic fibrosis transmembrane conductance regulator (CFTR) potentiator indicated for the treatment of cystic fibrosis. Kalydeco (ivacaftor) will be considered medically necessary when the following criteria have been met:
   1. Patient has a diagnosis of Cystic Fibrosis
   2. Prescribed by a pulmonologist
   3. Patient is 2 years of age and older
   4. Patient has had genetic testing and has one of the following mutations in the CFTR gene: G551D, G1244E, G1349D, G178R, G551S, S1251N, S1255P, S549N, S549R or R117H

F. **Orkambi** (lumacaftor/ivacaftor is a cystic fibrosis transmembrane conductance regulator (CFTR) potentiator and CFTR corrector combination indicated for the treatment of cystic fibrosis. Orkambi (lumacaftor/ivacaftor) will be considered medically necessary when the following criteria have been met:

1. Patient has a diagnosis of Cystic Fibrosis
2. Prescribed by a pulmonologist
3. Patient is 12 years of age and older
4. Patient has had genetic testing and has two copies (homozygous) of the F508del mutation (F508del/F508del) in their CFTR gene.

G. **Pulmozyme** (dornase alfa inhalation solution) is a recombinant DNase enzyme indicated in conjunction with standard therapies for the management of cystic fibrosis (CF). **Pulmozyme** (dornase alfa inhalation solution) will be considered medically necessary when the following criteria have been met:
1. Patient has a diagnosis of Cystic Fibrosis
2. Prescribed by a pulmonologist
3. Patient is 5 years of age and older
4. Patient with > 40% forced vital capacity (FVC) predicted

**Note:** Documented diagnosis must be confirmed by contemporaneous portions of the individual's medical record which will confirm the presence of disease and will need to be supplied with prior authorization request. These medical records may include, but not limited to test reports, chart notes from provider's office or hospital admission notes.

ALL other uses of Bethkis (tobramycin Inhalation solution), Tobi (tobramycin Inhalation solution & podhaler), Cayston (aztreonam inhalation solution), Kitabis (tobramycin solution), Kalydeco (ivacaftor), Orkambi (lumacaftor/ivacaftor), and Pulmozyme (dornase alfa inhalation solution) are considered experimental/investigational and therefore, will follow CareSource's Off-Label policy.

**Note**: Patient is required to have completed the trial listed in the above criteria unless the patient is unable to tolerate, has a contraindication, or a loss of response. Documentation such as chart notes or pharmacy claims may be requested.

**Refer to the product package insert for dosing, administration and safety guidelines.**

**For Medicare Plan members, reference the Applicable National Coverage Determinations (NCD) and Local Coverage Determinations (LCD). Compliance with NCDs and LCDs is required where applicable.**

## E. CONDITIONS OF COVERAGE

For Medicare Plan members, reference the below link to search for Applicable National Coverage Descriptions (NCD) and Local Coverage Descriptions (LCD): If there is no NCD or LCD present, reference the CareSource Policy for coverage.

**HCPCS**

J7682 Bethkis (tobramycin Inhalation solution)
J7682 Tobi (tobramycin Inhalation solution & podhaler)
J3490 Cayston (aztreonam inhalation solution)
J7682 Kitabis (tobramycin solution)
J3490 Kalydeco (ivacaftor)
J3490 Orkambi (lumacaftor/ivacaftor)
J7639 Pulmozyme (dornase alfa inhalation solution)

**CPT**

81220 CFTR (cystic fibrosis transmembrane conductance regulator) (eg, cystic fibrosis) gene analysis; common variants (eg, ACMG/ACOG guidelines)

81221 CFTR (cystic fibrosis transmembrane conductance regulator) (eg, cystic fibrosis) gene analysis; known familial variants
81222 CFTR (cystic fibrosis transmembrane conductance regulator) (eg, cystic fibrosis) gene analysis; duplication/deletion variants
81223 CFTR (cystic fibrosis transmembrane conductance regulator) (eg, cystic fibrosis) gene analysis; full gene sequence

**Step Therapy**
Under some plans, including plans that use an open or closed formulary, some of the medications in this policy may be subject to step-therapy. Refer to the CareSource formulary tool or PDL for further guidance.

**Place of Service**
Office, Outpatient, Home
**Preferred place of service is in the home.*
**Note:** CareSource supports administering medications in various settings, as long as those services are furnished in the most appropriate and cost effective setting that are supportive of the patient's medical condition and unique needs and condition. The decision on the most appropriate setting for administration is based on the member's current medical condition and any required monitoring or additional services that may coincide with the delivery of the specific medication.

**AUTHORIZATION PERIOD**
Approved initial authorizations are valid for 12 months. Continued treatment may be considered when the member has shown biological response to treatment. **ALL** authorizations are subject to continued eligibility.

## F. RELATED POLICIES/RULES

## G. REVIEW/REVISION HISTORY

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|---|---|
| Date Issued: | 10/06/2015 |
| Date Reviewed: | 10/06/2015 |
| Date Revised: | |

## H. REFERENCES


1. Pulmozyme [Package Insert]. South San Francisco, CA; Genentech, Inc.: December 2014.
2. Orkambi [Package Insert]. Boston, MA; Vertex Pharmaceuticals, Inc.: July 2015.
3. Kalydeco. [Package Insert]. Boston, MA; Vertex Pharmaceuticals, Inc.: March 2015.
4. Cayston. [Package Insert]. Foster City, CA; Gilead Sciences, Inc.: May 2014.
5. Kitabis [Package Insert]. Midlothian, VA; Catalent Pharma Solutions, LLC: November 2014.
6. Tobi [Package Insert]. East Hanover, NJ; Novartis Pharmaceuticals, Corp: October 2015.
7. Bethkis [Package Insert]. Woodstock, IL; Catalent Pharma Solutions: October 2013.
8. Peter J. Mogayzel, Jr., Edward T. Naureckas, Karen A. Robinson, Gary Mueller, Denis Hadjiliadis, Jeffrey B. Hoag, Lisa Lubsch, Leslie Hazle, Kathy Sabadosa, Bruce Marshall, and the Pulmonary Clinical Practice Guidelines Committee "Cystic Fibrosis Pulmonary Guidelines", American Journal of Respiratory and Critical Care Medicine, Vol. 187, No. 7 (2013), pp. 680-689. doi: 10.1164/rccm.201207-1160OE
9. Peter J. Mogayzel, Jr., Edward T. Naureckas, Karen A. Robinson, Cynthia Brady, Margaret Guill, Thomas Lahiri, Lisa Lubsch, Jane Matsui, Christopher M. Oermann, Felix Ratjen, Margaret Rosenfeld, Richard H. Simon, Leslie Hazle, Kathy Sabadosa, and Bruce C. Marshall "Cystic Fibrosis Foundation Pulmonary Guideline. Pharmacologic Approaches to Prevention and Eradication of Initial Pseudomonas aeruginosa Infection", Annals of the

American Thoracic Society, Vol. 11, No. 10 (2014), pp. 1640-1650. doi: 10.1513/AnnalsATS.201404-166OC
10. Cystic Fibrosis - CFTR Gene and Mutation Panel. (2015). MCG. ACG: A-0597 (AC). Retrieved July 29, 2015.
11. Antibiotics, inhaled. (2015). MCG. ACG: A-0313 (AC). Retrieved July 29, 2015.

This guideline contains custom content that has been modified from the standard care guidelines and has not been reviewed or approved by MCG Health, LLC.

**The medical Policy Statement detailed above has received due consideration as defined in the Medical Policy Statement Policy and is approved.**