curative

Cystic Fibrosis Transmembrane Conductance Regulator (CFTR) Prior Authorization

Drug(s) Applied:	Kalydeco, Trikafta, Symdeko

Criteria:
Drug(s) Applied will be approved when the requested medication is being used for an FDA approved indication and all of the following criteria are met:

I. Initial Therapy Criteria

A. Cystic Fibrosis, homozygous F508del mutation or mutation responsive (Kalydeco, Symdeko) as indicated by chart notes within past 6 months:

1. Diagnosis of Cystic Fibrosis as confirmed by genetic testing with ONE of the following:
    (1) Homozygous for F508del mutation in the CFTR gene (Symdeko only), **or**
    (2) At least one mutation responsive to the requested drug based on clinical and/or in vitro assay data (Symdeko or Kalydeco)
2. Patient's age is within FDA labeling for the requested indication for the requested agent, **and**
3. Patient will NOT be using the requested agent in combination with another CFTR modulator agent for the requested indication, **and**
4. Prescriber is a specialist, or has consulted with a specialist in the area of the patient's diagnosis (e.g. cystic fibrosis, pulmonologist)

Approval Duration: 6 months

B. Cystic Fibrosis, mutation responsive (Trikafta) as indicated by chart notes within past 6 months:

1. Diagnosis of Cystic Fibrosis as confirmed by genetic testing showing presence of at least one variant in the CFTR gene that is responsive based on clinical and/or in vitro data or results in production of CFTR protein **and**
2. Patient is at least 2 years of age, **and**
3. Patient will NOT be using the requested agent in combination with another CFTR modulator agent for the requested indication, **and**
4. Prescriber is a specialist, or has consulted with a specialist, in the area of the patient's diagnosis (e.g. cystic fibrosis, pulmonologist), **and**
5. Documentation of liver function test (LFT) results, including alanine

aminotransferase (ALT), aspartate aminotransferase (AST), alkaline phosphatase, and bilirubin within 1 month prior to starting the requested drug, **and**

6. Patient does not have severe hepatic impairment (Child-Pugh Class C)

Approval Duration: 6 months

II. Continued Therapy Criteria

A. Cystic Fibrosis, homozygous F508del mutation or mutation responsive (Kalydeco, Symdeko), as indicated by chart notes within past 6 months:

1. Patient has been previously approved for the requested agent through the plan's Prior Authorization process, or meets Initial Approval criteria **and**
2. Confirmed clinical improvement or stabilization with the requested agent from baseline (prior to treatment with the requested agent) as evidenced through at least one of the following:
    a) Improvement in FEV1or lung function tests, such as Cystic Fibrosis Questionnaire-Revised (CFQ-R) Respiratory Domain score, **or**
    b) Increase in weight/BMI, **or**
    c) Improvement in sweat chloride, **or**
    d) Reduced number of pulmonary exacerbations and/or pulmonary infections, **and**
3. The patient will NOT be using the requested agent in combination with another CFTR modulator agent for the requested indication, **and**
4. Prescriber is a specialist, or has consulted with a specialist, in the area of the patient's diagnosis (e.g. cystic fibrosis, pulmonologist)

Approval Duration: 12 months

B. Cystic Fibrosis, mutation responsive (Trikafta), as indicated by chart notes within past 6 months:

1. Patient has been previously approved for the requested agent through the plan's Prior Authorization process, or meets Initial Approval criteria **and**
2. Confirmed clinical improvement or stabilization with the requested agent from baseline (prior to treatment with the requested agent) as evidenced through at least one of the following:
    a) Improvement in FEV1or lung function tests, such as Cystic Fibrosis Questionnaire-Revised (CFQ-R) Respiratory Domain score, **or**
    b) Increase in weight/BMI, **or**
    c) Improvement in sweat chloride, **or**
    d) reduced number of pulmonary exacerbations and/or pulmonary infections, **and**

3. Patient will NOT be using the requested agent in combination with another CFTR modulator agent for the requested indication, **and**
4. Prescriber is a specialist, or has consulted with a specialist, in the area of the patient's diagnosis (e.g. cystic fibrosis, pulmonologist), **and**
5. Documentation within the last 365 days of liver function test (LFT) results, including alanine aminotransferase (ALT), aspartate aminotransferase (AST), alkaline phosphatase, and bilirubin, **and**
6. ONE of the following:
    a) Patient does not have evidence of elevated LFT results, **or**
    b) Patient is being followed by a hepatologist with notes indicating benefit outweighs risk, **and**
7. Patient does not have severe hepatic impairment (Child-Pugh Class C)

Approval Duration: 12 months

References:

1. Trikafta® [prescribing information]. Cambridge, MA: Vertex Pharmaceuticals Incorporated; December 2024.
2. Kalydeco® [prescribing information]. Cambridge, MA:Vertex Pharmaceuticals Incorporated. August 2023.
3. Symdeko® [prescribing information]. Cambridge, MA: Vertex Pharmaceuticals Incorporated. June 2022.
4. Southern KW, Addy C, Bell SC, et al. Standards for the care of people with cystic fibrosis; establishing and maintaining health. J Cyst Fibros. 2024;21-28.
5. Farrell PM, White TB, Ren CL, et al. Diagnosis of cystic fibrosis: consensus guidelines from the cystic fibrosis foundation. J Pediatr. 2017;181S:S4-S15.
6. Farrell PM, White TB, Howenstine MS, et al. Diagnosis of cystic fibrosis in screened populations. J Pediatr. 2017;181S:S33-S44.
7. Southern KW, Addy C, Bell SC, et al. Standards for the care of people with cystic fibrosis; establishing and maintaining health. J Cyst Fibros. 2024;21-28.
8. Ren CL, Morgan RL, Oermann C, et al. Cystic Fibrosis Pulmonary Guidelines: Use of CFTR Modulator Therapy in Patients with Cystic Fibrosis. Ann Am Thorac Soc. 2018 Mar;15(3):271-280.
9. Zemanick ET, Taylor-Cousar JL, Davies J, et al. A Phase 3 Open-Label Study of Elexacaftor/Tezacaftor/Ivacaftor in Children 6 through 11 Years of Age with Cystic Fibrosis and at Least One F508del Allele. Am J Respir Crit Care Med. 2021;203(12):1522.

Policy Owned by: Curative PBM team