Commercial/Healthcare Exchange PA Criteria

Effective: July 25th, 2018

Prior Authorization: Symdeko

Products Affected: Symdeko (tezacaftor/ivacaftor) oral tablet

Medication Description:

Symdeko is indicated for the treatment of patients ≥ 6 years of age with cystic fibrosis (CF) who are homozygous for the F508del mutation or who have at least one mutation in the cystic fibrosis transmembrane conductance regulator (CFTR) gene that is responsive to tezacaftor/ivacaftor based on *in vitro* data and/or clinical evidence.[1] If the patient's genotype is unknown, an FDA-cleared CF mutation test should be used to detect the presence of a CFTR mutation followed by verification with bi-directional sequencing when recommended by the mutation test instructions for use. Responsive CFTR mutations are based on: 1) a clinical forced expiratory volume in 1 second (FEV_1) response and/or 2) *in vitro* data in FRT cells, indicating that tezacaftor/ivacaftor increases chloride transport to ≥10% of untreated normal over baseline. CFTR gene mutations that are not responsive to ivacaftor alone are not expected to respond to Symdeko except for F508del homozygotes.

Covered Uses: patients with cystic fibrosis (CF) aged 6 years and older who are homozygous for the F508del mutation or who have at least one mutation in the cystic fibrosis transmembrane conductance regulator (CFTR) gene that is responsive to tezacaftor/ivacaftor based on in vitro data and/or clinical evidence.

Exclusion Criteria:

1. Cystic Fibrosis patients with unknown Cystic Fibrosis Transmembrane Regulator (CFTR) gene mutation.
2. Combination therapy with Orkambi or Kalydeco.

Required Medical Information:

1. Diagnosis
2. Cystic Fibrosis Transmembrane Regulator (CFTR) gene mutation (documentation required)
3. Current medical history
4. Current medication regimen

Age Restrictions: 6 years of age or older

Prescriber Restrictions: Prescribed by or in consultation with a pulmonologist or a physician who specializes in the treatment of cystic fibrosis (CF).

Coverage Duration:

Initial: 12 months

Continuation: 3 years

Other Criteria:

Approve Symdeko for 12 months in patients who meet the following criteria A AND B:

A. The patient meets ONE of the following conditions (i or ii):
   - **i.** The patient has at least one mutation in the cystic fibrosis transmembrane conductance regulator (CFTR) gene that is responsive to tezacaftor/ivacaftor: A455E, A1067T, D110E, D110H, D579G, D1152H, D1270N, E56K, E193K, E831X, F1052V, F1074L, K1060T, L206W, P67L, R74W, R117C, R347H, R352Q, R1070W, S945L, S977F, 711+3A→G, 2789+5G→A, 3272-26A→G, 3849+10kbC→T; **OR**
   - **ii.** The patient has two copies of the F508del mutation (homozygous); **AND**

B. The patient must be able to swallow tablets

References:

1. Symdeko™ tablets [prescribing information]. Cambridge, MA: Vertex Pharmaceuticals, Inc; February 2018

Policy Revision history

Rev #	Type of Change	Summary of Change	Sections Affected	Date
1	New Policy	New Policy	All	07/17/2018
2	Policy Update	Updated age to match FDA Label (changed from 12 to 6 years old)	Medication Description, Covered Uses, Age Restrictions	6/24/2019
3	Policy Update	Added continuation coverage duration of 3 years	Coverage Duration	7/1/2019