Effective date: 8/1/2019
Review date: 8/2019, 6/2020, 02/2021, 02/2022, 02/2023, 02/2024, 5/2025 Scope: Medicaid

SPECIALTY GUIDELINE MANAGEMENT

SYMDEKO (tezacaftor/ivacaftor)

POLICY

I. INDICATIONS

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-Approved Indication
Symdeko is indicated for the treatment of 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.

If the patient's genotype is unknown, an FDA-cleared CF mutation test should be used to detect the presence of *CFTR* mutation followed by verification with bi-directional sequencing when recommended by the mutation test instructions for use.

All other indications are considered experimental/investigational and are not medically necessary.

II. CRITERIA FOR INITIAL APPROVAL

Cystic Fibrosis
Authorization of 6 months may be granted for treatment of cystic fibrosis when all of the following criteria are met:

A. Documentation that genetic testing was conducted to detect a mutation in the *CFTR* gene.
B. The medication is prescribed by or in consultation with a pulmonologist.
C. The member is homozygous for the *F508del* mutation, or the member has one of the following mutations in the *CFTR* gene: A120T, A234D, A349V, A455E, A554E, A1006E, A1067T, D110E, D110H, D192G, D443Y, D443Y;G576A;R668C, D579G, D614G, D836Y, D924N, D979V, D1152H, D1270N, E56K, E60K, E92K, E116K, E193K, E403D, E588V, E822K, E831X, F191V, F311del, F311L, F508C, F508C;S1251N, F575Y, F1016S, F1052V, F1074L, F1099L, G126D, G178E, G178R, G194R, G194V, G314E, G551D, G551S, G576A, G576A;R668C, G622D, G970D, G1069R, G1244E, G1249R, G1349D, H939R, H1054D, H1375P, I148T, I175V, I336K, I601F, I618T, I807M, I980K, I1027T, I1139V, I1269N, I1366N, K1060T, L15P, L206W, L320V, L346P, L967S, L997F, L1324P, L1335P, L1480P, M152V, M265R, M952I, M952T, P5L, P67L, P205S, Q98R, Q237E, Q237H, Q359R, Q1291R, R31L, R74Q, R74W, R74W;D1270N, R74W;V201M, R74W;V201M;D1270N, R75Q, R117C, R117G, R117H, R117L, R117P, R170H, R258G, R334L, R334Q, R347H, R347L, R347P, R352Q, R352W, R553Q, R668C, R751L, R792G, R933G, R1066H, R1070Q, R1070W, R1162L, R1283M, R1283S, S549N, S549R, S589N, S737F, S912L, S945L, S977F, S1159F, S1159P, S1251N, S1255P, T338I, T1036N, T1053I, V201M, V232D, V562I, V754M,

V1153E, V1240G, V1293G, W1282R, Y109N, Y161S, Y1014C, Y1032C, 546insCTA, 711+3A→G, 2789+5G→A, 3272-26A→G, 3849+10kbC→T.

D. The member is at least 6 years of age.
E. Symdeko will not be used in combination with other ivacaftor containing medications.

III. CONTINUATION OF THERAPY

Authorization of 12 months may be granted for continued treatment in members requesting reauthorization for an indication listed in Section III who are experiencing benefit from therapy as evidenced by disease stability or disease improvement (e.g., improvement in FEV1 from baseline).

IV. QUANTITY LIMIT

Symdeko has a quantity limit of 2 tablets per day.

Age and Weight	FDA-Recommended Dosing
Adults, Pediatric patients aged 12 years and older, and Pediatric patients aged 6 to less than 12 years weighing 30kg or more	One tablet (containing tezacaftor 100 mg/ivacaftor 150 mg) in the morning and one tablet (containing ivacaftor 150 mg) in the evening
Pediatric patients aged 6 to less than 12 years weighing less than 30 kg	One tablet (containing tezacaftor 50 mg/ivacaftor 75 mg) in the morning and one tablet (containing ivacaftor 75 mg) in the evening

V. REFERENCES

1. Symdeko [package insert]. Boston, MA: Vertex Pharmaceuticals Inc.; December 2024
2. Rowe SM, Daines C, Ringshausen FC, Kerem E, Wilson J, Tullis E, Nair N, Simard C, Han L, Ingenito EP, McKee C, Lekstrom-Himes J, Davies JC. Tezacaftor-Ivacaftor in Residual Function Heterozygotes with Cystic Fibrosis. *N Engl J Med.* 2017; 377:2024-2035.
3. Taylor-Cousar JL, Munck A, McKone EF, et al. Tezacaftor–ivacaftor in patients with cystic fibrosis homozygous for Phe508del. *N Engl J Med* 2017; 377:2013-2023.