

Reference number(s)
2516-A

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 cystic fibrosis (CF) in patients age 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. DOCUMENTATION

Submission of the following information is necessary to initiate the prior authorization review: For initial requests, genetic testing report confirming the presence of the appropriate *CFTR* gene mutation.

III. PRESCRIBER SPECIALTIES

This medication must be prescribed by or in consultation with a pulmonologist.

IV. CRITERIA FOR INITIAL APPROVAL

Cystic Fibrosis

Authorization of 12 months may be granted for treatment of cystic fibrosis when all of the following criteria are met:

- A. Genetic testing was conducted to detect a mutation in the *CFTR* gene.
- B. 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,

Reference number(s)
2516-A

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.

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

V. CONTINUATION OF THERAPY

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

VI. REFERENCES

1. Symdeko [package insert]. Boston, MA: Vertex Pharmaceuticals Inc.; June 2022.
2. Rowe SM, Daines C, Ringshausen FC, et al. 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.