Cystic Fibrosis Transmembrance Conductance Regulator (CFTR) Potentiators:
Kalydeco (ivacaftor)
Orkambi (lumacaftor/ivacaftor)
Symdeko (tezacaftor/ivacaftor)
Trikafta (elexacaftor/tezacaftor/ivacaftor)
Effective 08/01/2021

Plan	☐ MassHealth ☒Commercial/Exchange	**Program Type**	☒ Prior Authorization ☒ Quantity Limit ☐ Step Therapy
Benefit	☒ Pharmacy Benefit ☐ Medical Benefit (NLX)		
Specialty Limitations	This medication has been designated specialty and must be filled at a contracted specialty pharmacy.		
Contact Information	**Specialty Medications**		
	All Plans	Phone: 866-814-5506	Fax: 866-249-6155
	Non-Specialty Medications		
	MassHealth	Phone: 877-433-7643	Fax: 866-255-7569
	Commercial	Phone: 800-294-5979	Fax: 888-836-0730
	Exchange	Phone: 855-582-2022	Fax: 855-245-2134
	Medical Specialty Medications (NLX)		
	All Plans	Phone: 844-345-2803	Fax: 844-851-0882
Exceptions			

Overview
CF is caused by genetic mutations in the CFTR protein. The CFTR protein is present in the respiratory epithelium and plays an important role in the regulation of airway surface liquid. Genetic mutations in the protein result in abnormal airway secretions, chronic endobronchial infection, and progressive airway obstruction. The CFTR potentiators treat the underlying cause of CF by targeting the defective CFTR protein to help facilitate increased chloride transport.

Coverage Guidelines:

Kalydeco (ivacaftor)

Authorization may be granted for members who are currently receiving treatment with Kalydeco for an FDA approved indication excluding when the product is obtained as samples or via manufacturer's patient assistance programs.

OR

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

1. Documentation of genetic testing to detect a mutation in the *CFTR* gene.
2. The member has one of the following mutations in the CFTR gene: A455E, A1067T, D110E, D110H, D579G, D1152H, D1270N, E56K, E193K, E831X, F1052V, F1074L, G178R, G551D, G551S, G1069R, G1244E, G1349D, K1060T, L206W, P67L, R74W, R117C, R117H, R347H, R352Q, R1070W, R1070W, S549N, S549R, S945L, S977F, S1251N, S1255P, 711+3A→G, 2789+5G→A, 3272-26A→G, 3849+10kbC→T.

3. The member is ≥ 6 months of age
4. Kalydeco will not be used in combination with Symdeko, Orkambi, or Trikafta

Orkambi (lumacaftor/ivacaftor)
Authorization may be granted for members who are currently receiving treatment with Orkambi for an FDA approved indication excluding when the product is obtained as samples or via manufacturer's patient assistance programs.
OR
Authorization may be granted for treatment of cystic fibrosis when all of the following criteria are met:
1. Documentation of genetic testing to detect a mutation in the *CFTR* gene.
2. The member is positive for the *F508del* mutation on both alleles of the *CFTR* gene.
3. The member is 2 years of age or older.
4. Orkambi will not be used in combination with Kalydeco, Symdeko, or Trikafta.

Symdeko (tezacaftor/ivacaftor)
Authorization may be granted for members who are currently receiving treatment with Symdeko for an FDA approved indication excluding when the product is obtained as samples or via manufacturer's patient assistance programs.
OR
Authorization may be granted for treatment of cystic fibrosis when all of the following criteria are met:
1. Documentation of genetic testing to detect a mutation in the *CFTR* gene.
2. The member has one of the following mutations in the CFTR gene: 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 the member is homozygous for the F508del mutation.
3. The member ≥ 6 years of age or older
4. Symdeko will not be used in combination with Kalydeco, Orkambi, or Trikafta

Trikafta (elaxacaftor/tezacaftor/ivacaftor)
Authorization may be granted for members who are currently receiving treatment with Trikafta for an FDA approved indication excluding when the product is obtained as samples or via manufacturer's patient assistance programs.
OR
Authorization may be granted for treatment of cystic fibrosis when all of the following criteria are met:
1. Documentation of genetic testing to detect a mutation in the *CFTR* gene.
2. The member is positive for the *F508del* mutation on one or more alleles of the *CFTR* gene.
3. The member is 12 years of age or older.
4. The member has previously trialed and experienced and inadequate response to Kalydeco, Symdeko, and Orkambi.
5. Trikafta will not be used in combination with Kalydeco, Symdeko, or Orkambi.

Continuation of Therapy
All members (including new members) requesting authorization for continuation of therapy must meet all initial authorization criteria.

Limitations
1. Initial approvals will be granted for 6 months
2. Reauthorizations will be granted for 12 months

3. The following quantity limits apply:

Kalydeco 150mg tablets	56 tablets per 28 days
Kalydeco 25mg, 50mg, or 75mg packets	56 packets per 28 days
Orkambi 200-125mg tablets	112 tablets per 28 days
Orkambi 150-188mg granules	56 packets per 28 days
Symdeko 50-75mg tablets	56 tablets per 28 days
Symdeko 100-150mg tablets	56 tablets per 28 days
Trikafta 100-50-75mg tablets	84 tablets per 28 days

References

1. Kalydeco [package insert]. Boston, MA: Vertex Pharmaceuticals Inc.; April 2019.
2. Mogayzel PJ, Naureckas ET, Robinson KA, et al. Cystic fibrosis pulmonary guidelines. Chronic medications for maintenance of lung health. *Am J Respir Crit Care Med*. 2013;187:680-689.
3. Orkambi [package insert]. Boston, MA: Vertex Pharmaceuticals Inc.; August 2018.
4. Symdeko [package insert]. Boston, MA: Vertex Pharmaceuticals Inc.; June 2019.
5. 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 Funtion Heterzygotes with Cystic Fibrosis. *N Engl J Med*. 2017; 377:2024-2035
6. 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
7. Trikafta (elexacaftor/tezacaftor/ivacaftor) [prescribing information]. Boston, MA: Vertex Pharmaceuticals Inc., January 2020.

Review History

05/20/2020 – Created and Reviewed P&T Mtg; Merged Orkambi, Symdeko, Trikafta and Kalydeco into one program. Effective 7/1/20.
05/19/2021 – Updated and Reviewed May P&T Mtg; Separated out Comm/Exch vs. MH UPPL; Added duration of approval to Limitations. Effective 08/01/2021.

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