NC Medicaid Outpatient Pharmacy Prior Approval Criteria Cystic Fibrosis	Medicaid and Health Choice Effective Date: November 14, 2012 Amended Date:

Therapeutic Class Code: B0B, B0F

Therapeutic Class Description: CFTR (Cystic Fibrosis Transmembrane Conductance Regulator) Potentiator, and CFTR Potentiator and Corrector Combination

Medication
Kalydeco 150mg tablets Kalydeco 50mg granules Kalydeco 75mg granules Kalydeco 25mg granules
Orkambi 200mg/125mg tablets Orkambi 100mg/125mg tablets Orkambi 150/188mg granules Orkambi 100/125mg granules
Symdeko 50mg/75mg -75 mg tablets Symdeko 100/150 mg - 150 mg tablets
Trikafta

Eligible Beneficiaries
NC Medicaid (Medicaid) beneficiaries shall be enrolled on the date of service and may have service restrictions due to their eligibility category that would make them ineligible for this service.

NC Health Choice (NCHC) beneficiaries, ages 6 through 18 years of age, shall be enrolled on the date of service to be eligible, and must meet policy coverage criteria, unless otherwise specified. **EPSDT does not apply to NCHC beneficiaries**.

EPSDT Special Provision: Exception to Policy Limitations for Beneficiaries under 21 Years of Age

42 U.S.C. § 1396d(r) [1905(r) of the Social Security Act]

Early and Periodic Screening, Diagnostic, and Treatment (EPSDT) is a federal Medicaid requirement that requires the state Medicaid agency to cover services, products, or procedures for Medicaid beneficiaries under 21 years of age **if** the service is **medically necessary health care** to correct or ameliorate a defect, physical or mental illness, or a condition [health problem] identified through a screening examination (includes any evaluation by a physician or other licensed clinician). This means EPSDT covers most of the medical or remedial care a child needs to improve or maintain his/her health in the best condition possible, compensate for a health problem, prevent it from worsening, or prevent the development of additional health problems. Medically necessary services will be provided in the most economic mode, as long as the treatment made available is similarly efficacious to the service requested by the beneficiary's physician, therapist, or other licensed practitioner; the determination process does not delay the delivery of the needed service; and the determination does not limit the beneficiary's right to a free choice of providers.

EPSDT does not require the state Medicaid agency to provide any service, product, or procedure
a. that is unsafe, ineffective, or experimental/investigational.
b. that is not medical in nature or not generally recognized as an accepted method of medical practice or treatment.

Service limitations on scope, amount, duration, frequency, location of service, and/or other specific criteria described in clinical coverage policies may be exceeded or may not apply as long as the provider's documentation shows that the requested service is medically necessary "to correct or ameliorate a defect, physical or mental illness, or a condition" [health problem]; that is, provider documentation shows how the service, product, or procedure meets all EPSDT criteria, including to correct or improve or maintain the beneficiary's health in the best condition possible, compensate for a health problem, prevent it from worsening, or prevent the development of additional health problems.

EPSDT and Prior Approval Requirements

a. If the service, product, or procedure requires prior approval, the fact that the beneficiary is under 21 years of age does **NOT** eliminate the requirement for prior approval.
b. IMPORTANT ADDITIONAL INFORMATION about EPSDT and prior approval is found in the NCTracks Provider Claims and Billing Assistance Guide, and on the EPSDT provider page. The Web addresses are specified below.

NCTracks Provider Claims and Billing Assistance Guide:
https://www.nctracks.nc.gov/content/public/providers/provider-manuals.html

EPSDT provider page:
https://medicaid.ncdhhs.gov/medicaid/get-started/find-programs-and-services-right-you/medicaid-benefit-children-and-adolescents

Health Choice Special Provision: Exceptions to Policy Limitations for Health Choice Beneficiaries ages 6 through 18 years of age

EPSDT does not apply to NCHC beneficiaries. If a NCHC beneficiary does not meet the clinical coverage criteria within **the Outpatient Pharmacy prior approval** clinical coverage criteria, the NCHC beneficiary shall be denied services. Only services included under the Health Choice State Plan and the DMA clinical coverage policies, service definitions, or billing codes shall be covered for NCHC beneficiaries.

Criteria for Coverage- Kalydeco:

- Beneficiary has been diagnosed with Cystic Fibrosis
  and
- Beneficiary is age 4 months or greater
  and
- Beneficiary has a documented mutation in the CFTR gene that is responsive to ivacaftor. 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. (KALYDECO is not effective in patients with CF who are homozygous for the F508del mutation in the CFTR gene)
  and

- Dosing is 150mg taken every 12 hours (300mg/day total) or less
  and
- A baseline ALT and AST assessed prior to beginning therapy

Criteria for Coverage- Orkambi:

- Beneficiary has been diagnosed with Cystic Fibrosis
  and
- Beneficiary is age 2 or greater
  and
- Beneficiary is documented as homozygous for the *F508del* mutation in the *CFTR* gene. If the patient's genotype is unknown, an FDA-cleared CF mutation test should be used to detect the presence of the *F508del* mutation on both alleles of the *CFTR* gene.
  and
- Dosing is two tablets (each containing lumacaftor 200 mg/ivacaftor 125 mg) or less taken orally every 12 hours with fat-containing food
  and
- A baseline ALT and AST assessed prior to beginning therapy

Criteria for Coverage- Symdeko:

- Beneficiary has been diagnosed with Cystic Fibrosis
  and
- Beneficiary is 6 years of age or greater
  and
- Beneficiary is documented as homozygous for the F508del mutation in the CFTR gene or beneficiary has one mutation in the CFTR gene that is responsive to tezacaftor/ivacaftor. If the patient's genotype is unknown, an FDA-cleared CF mutation test should be used to detect the presence of the F508del mutation on both alleles of the CFTR gene.
  and
- Dosing is one tablet in the morning and one tablet in the evening
  and
- A baseline ALT and AST assessed prior to beginning therapy

Criteria for Coverage- Trikafta:

- Beneficiary has been diagnosed with Cystic Fibrosis
  and
- Beneficiary is ~~12~~ 6 year of age or greater
  and
- Beneficiary is documented to have at least one copy of the F508del mutation in the CFTR gene or beneficiary has one mutation in the CFTR gene that is responsive to tezacaftor/ivacaftor. If the beneficiary's genotype is unknown, an FDA-cleared CF mutation test should be used to confirm the presence of at least one F508del mutation.
  and
- Dosing does not exceed: two tablets (elexacaftor 100 mg, tezacaftor 50 mg and ivacaftor 75 mg) in the morning and one tablet (ivacaftor 150 mg) in the evening
  and
- Baseline ALT, AST, and bilirubin have been assessed prior to beginning therapy
  and

- If beneficiary less than 18 year of age: baseline ophthalmic examination has been performed

Procedures:

Length of therapy may be approved for up to 12 months.

References

1. Prescribing Information-Kalydeco® (ivacaftor) Vertex Pharmaceuticals, Inc., Cambridge, Massachusetts 02139. January 2012.
2. Prescribing Information Kalydeco®. Vertex Pharmaceuticals Incorporated Cambridge, MA; February 2014.
3. Prescribing Information Orkambi®. Vertex Pharmaceuticals Incorporated Boston, MA; April 2015.
4. Prescribing Information Orkambi®. Vertex Pharmaceuticals Incorporated Boston, MA; September 2016. Revised July 2019.
5. Prescribing Information Kalydeco®. Vertex Pharmaceuticals Incorporated Cambridge, MA; March 2015.
6. Prescribing Information Kalydeco®. Vertex Pharmaceuticals Incorporated Cambridge, MA; May 2017. Revised April 2019. Revised September 2020.
7. Prescribing Information Symdeko. Vertex Pharmaceuticals, Inc. Cambridge, MA; February 2018. Revised June 2019.
8. Prescribing Information Trikafta. Vertex Pharmaceuticals, Inc. Cambridge, MA; December 2020. Revised June 2021.

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Criteria Change Log

11/14/2012	Criteria effective date-Kalydeco only
08/01/2014	Added G1244E, G1349D, G178R, G551S, S1251N, S1255P, S549N, or S549R mutation in the CFTR gene. 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. (KALYDECO is not effective in patients with CF who are homozygous for the F508del mutation in the CFTR gene)
04/01/2015	Added R117H mutation
11/05/2015	Added Kalydeco gcn's 38138, 38139
03/09/2016	Added coverage for Orkambi
04/06/2017	Changed age for Kalydeco to 2 yrs and older and for Orkambi to 6 yrs and older
10/03/2017	Added genetic mutations E56K, K1060T, P67L, E193K, A1067T, R74W, L206W, G1069R, D110E, R347H, D579G, R1070Q, D1270N, D110H, R352Q, S945L, R1070W, R117C, A455E, S977F, F1074L, F1052V,or D1152H for Kalydeco
06/11/2018	Added information about Symdeko
02/26/2019	Age for Kalydeco changed from 2 or greater to 1 or greater
	Age for Symdeko changed from 12 or greater to 6 or greater. Updated GCN's with new products
11/03/2020	Removed GCN's Age for Kalydeco changed from 1 or greater to 6 months or greater. Age for Orkambi changed from 6 or greater to 2 or greater. Removed GCNs. Removed G551D, G1244E, G1349D, G178R, G551S, S1251N, S1255P, S549N, S549R, R117H, E56K, K1060T, P67L, E193K, A1067T, R74W, L206W, G1069R, D110E, R347H, D579G, R1070Q, D1270N, D110H, R352Q, S945L, R1070W, R117C, A455E, S977F, F1074L, F1052V, or D1152H and replaced with mutation in the CFTR gene that is responsive to ivacaftor. Updated EPSDT web addresses and info
11/03/2020	Added Trikafta
03/15/2021	Age for Kalydeco changed from 6 months or greater to 4 months or greater.
xx/xx/xxxx	add the wording "or mutation in the CFTR gene that is responsive" for Trikafta based on a change to the FDA labelling.
xx/xx/xxxx	Age for Trikafta changed from 12 years to 6 years or greater