NC Division of Medical Assistance **Medicaid and Health Choice**
Outpatient Pharmacy **Amended Date:**
Prior Approval Criteria
~~Kalydeco (ivacaftor)~~ Cystic Fibrosis

DRAFT

Therapeutic Class Code: B0B, B0F

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

Medication	Generic Code Number(s)
Kalydeco 150mg tablet	31312
Kalydeco 50mg granules	38138
Kalydeco 75mg granules	38139
Orkambi 200mg/125mg tablet	39008

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
EPSDT DOES NOT ELIMINATE THE REQUIREMENT FOR PRIOR APPROVAL IF PRIOR APPROVAL IS REQUIRED. Additional information on EPSDT guidelines may be accessed at http://www.ncdhhs.gov/dma/epsdt/.

Criteria for Coverage- Kalydeco:

- Beneficiary has been diagnosed with Cystic Fibrosis

and

- Beneficiary is age 6 or greater

and

- Beneficiary has a documented G551D, 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)

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 12 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

Procedures:

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

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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.