NC Medicaid
Outpatient Pharmacy Prior Approval Criteria
Casgevy

Effective Date: xx/xx/xxxx

Therapeutic Class Code: N1K
Therapeutic Class Description: Gene Therapy Agents- CD34+ Hematopoietic Stem Cells

Medication
Casgevy (exagamglogene autotemcel)

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.

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

1. 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.

2. **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

Clinical Coverage

- Beneficiary is ≥ 12 years of age; **AND**
- Provider has considered use of prophylaxis therapy for seizures prior to initiating myeloblative conditioning; **AND**
- Beneficiary has been screened and found negative for hepatitis B virus (HBV), hepatitis C virus (HCV), and human immunodeficiency virus 1&2 (HIV-1/HIV-2) in accordance with clinical guidelines prior to collection of cells (leukapheresis); **AND**
- Must not be administered concurrently with live vaccines while immunosuppressed; **AND**
- Beneficiary does not have history of hypersensitivity to dimethyl sulfoxide (DMSO) or dextran 40**; AND**
- Beneficiary has not received other gene therapies [e.g. Lyfgenia®(lovotibeglogene autotemcel), Zynteglo® (betibeglogene autotemcel), etc.]**; AND
- Beneficiary will not receive therapy concomitantly with any of the following:
  - Iron chelators for 7 days prior to mobilization and 6 months post-treatment (3 months post-treatment for non-myelosuppressive iron chelators); **AND**
  - Disease-modifying agents (e.g. hydroxyurea, or crizanlizumab) for at least 8 weeks prior to mobilization and conditioning; **AND**
- Beneficiary is a candidate for autologous hematopoietic stem cell transplant (HSCT) and has not had prior HSCT; **AND**
- For beneficiaries under 18 years of age, the beneficiary does not have a known and suitable 10/10 human leukocyte antigen matched related donor willing to participate in an allogenic HSCT; **AND**

Sickle Cell Disease

- Beneficiary has a confirmed diagnosis of sickle-cell disease with one of the following genotypes: βS/βS or βS/β0 or βS/β+ (Note: additional genotypes will be considered on a case-by-case basis based on disease severity) as determined by 1 of the following:
  - Identification of significant quantities of sickle cell hemoglobin (HbS) with or without an additional abnormal β-globin chain variant by hemoglobin assay; **OR**
  - Identification of biallelic HBB pathogenic variants where at least one allele is the p.Glu6Val pathogenic variant on molecular genetic testing; **AND**

- Beneficiary has symptomatic disease despite treatment with hydroxyurea at any point in the past OR add-on therapy (e.g. crizanlizumab etc.) OR has experienced intolerance; **AND**

- Beneficiary has experienced ≥ 2 serious vaso-occlusive events/crises requiring hospitalization (VOE/VOC)* in the previous year while adhering to the above therapy; **AND**

- Beneficiaryt will be transfused prior to apheresis to a total Hb < 11 g/dL and a HbS level <30% and patient will be transfused at least 8 weeks prior to initiation of myeloblative conditioning (with aforementioned Hb and HbS goals; **AND**

- Beneficiary will not receive granulocyte-colony stimulating factor (G-CSF) for the mobilization of hematopoietic stem cells (HSC) **AND**
- Must be prescribed in consultation with a board-certified hematologist with Sickle Cell Disease expertise.

Beta Thalassemia

- Beneficiary has a documented diagnosis of homozygous beta thalassemia or compound heterozygous beta thalassemia including β-thalassemia/hemoglobin E (HbE) as outlined by the following:
    - Beneficiary diagnosis is confirmed by *HBB* sequence gene analysis showing biallelic pathogenic variants; **OR**
    - Beneficiary has severe microcytic hypochromic anemia, absence of iron deficiency, anisopoikilocytosis with nucleated red blood cells on peripheral blood smear, and hemoglobin analysis that reveals decreased amounts or complete absence of hemoglobin A (HbA) and increased HbA_2 with or without increased amounts of hemoglobin F (HbF); **AND**
- Beneficiary has transfusion-dependent disease defined as a history of transfusions of at least 100 mL/kg/year or ≥10 units/year of packed red blood cells (pRBCs) in the 2 years preceding therapy; **AND**
- Beneficiary will be transfused prior to apheresis to a total Hb ≥ 11 g/dL for 60 days prior to myeloablative conditioning; AND
- Beneficiary does not have any of the following:
    - Severely elevated iron in the heart (i.e., patients with cardiac T2* less than 10 msec by magnetic resonance imaging [MRI] or left ventricular ejection fraction [LVEF] < 45% by echocardiogram); **OR**
    - Advanced liver disease [i.e., AST or ALT > 3 times the upper limit of normal (ULN), or direct bilirubin value > 2.5 times the ULN, or if a liver biopsy demonstrated bridging fibrosis or cirrhosis]

VOE/VOC is defined as an event requiring a visit to a medical facility for evaluation which results in a diagnosis of such being documented due to one (or more) of the following: acute pain, acute chest syndrome, acute splenic sequestration, acute hepatic sequestration, priapism lasting > 2 hours **AND necessitating subsequent interventions such as opioid pain management, non- steroidal anti-inflammatory drugs, RBC transfusion, etc.*

Renewal Criteria

- Coverage will not be renewed

Duration of Approval

- One treatment course

References

1 Casgevy [package insert]. Boston, MA; Vertex Inc.; January 2024

Criteria Change Log

xx/xx/xxxx	Criteria effective date