

POLICY NAME	Casgevy (exagamglogene autotemcel)	POLICY #	3231P
--------------------	------------------------------------	-----------------	-------

Criteria

Coverage Criteria for Sickle Cell Disease

- ☐ Diagnosis of sickle cell disease confirmed by genetic testing
 - Must have $\beta S/\beta S$, $\beta S/\beta 0$ or $\beta S/\beta +$ genotype
- ☐ Documented severe disease as evident by history of recurrent vaso-occlusive crises defined as at least 2 events per year in the last 2 years prior to therapy
- ☐ Prescribed by or in consultation with a hematologist (blood doctor) or other sickle cell specialist
- ☐ Age 12 years or older
- ☐ Documented trial and failure of standard of care including hydroxyurea, Endari, Oxbryta, or Adakveo
 - Standard of care treatments must be discontinued for 2 months prior to Casgevy infusion
- ☐ Review of clinical information confirming that patient has met all of the above requirements for treatment completed by both a pharmacist and medical director

Coverage Criteria for Transfusion Dependent Beta-Thalassemia

- ☐ Diagnosis of transfusion dependent beta thalassemia with a history of requiring at least 100 mL/kg/year or 10 units/year of RBC transfusions in the past 2 years
 - Must be non- $\beta 0/\beta 0$ genotype confirmed through genetic testing
- ☐ Prescribed by or in consultation with a hematologist (blood doctor)
- ☐ Age 12 years or older
- ☐ Eligible for hematopoietic stem cell transplant but does not have a suitable HLA donor
- ☐ Review of clinical information confirming that patient has met all of the above requirements for treatment completed by both a pharmacist and medical director

Exclusion Criteria – Any of the following prevents coverage

- ☐ Significant liver dysfunction
 - Patients with advanced liver disease were excluded from clinical trials (NCT03745287). Safety and efficacy has not been established in this patient population.

- ☐ **Diagnosis of any hematologic disorder other than sickle cell disease or transfusion dependent beta thalassemia**
 - Casgevy has only been studied in patients with confirmed diagnosis of sickle cell disease with the $\beta S/\beta S$, $\beta S/\beta 0$ or $\beta S/\beta +$ genotype or beta thalassemia with non- $\beta 0/\beta 0$ genotype. Use of Casgevy for the treatment of any other hematologic disorder is considered experimental and excluded from coverage.

- ☐ **Prior treatment with an allogenic or autologous stem cell transplant**
 - Patients with prior stem cell transplant or those with eligible matched donors were excluded from clinical trials. Safety and efficacy of Casgevy in patients with a history of stem cell transplant has not been established and treatment is not recommended in these patients (package insert).

- ☐ **Casgevy will not be covered in patients who have previously received Lyfgenia or any other gene therapy**
 - Safety and efficacy has not been established in patients who were previously treated any gene therapy.