Pharmacy Drug Policy Checklist

POLICY NAME	Casgevy (exagamglogene autotemcel)	POLICY #	3231P

Criteria

Coverage Criteria for Sickle Cell Disease

- ☐ **1.1** Diagnosis of sickle cell disease confirmed by genetic testing
  - Must have βS/βS, βS/β0 or βS/β+ genotype
- ☐ **1.2** 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
- ☐ **1.3** Prescribed by or in consultation with a hematologist (blood doctor) or other sickle cell specialist
- ☐ **1.4** Age 12 years or older
- ☐ **1.5** 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
- ☐ **1.6** 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

- ☐ **2.1** 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-β0/β0 genotype confirmed through genetic testing
- ☐ **2.2** Prescribed by or in consultation with a hematologist (blood doctor)
- ☐ **2.3** Age 12 years or older
- ☐ **2.4** Eligible for hematopoietic stem cell transplant but does not have a suitable HLA donor
- ☐ **2.5** 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

☐ **3.1** 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.

☐ **3.2** 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 βS/βS, βS/β0 or βS/β+ genotype or beta thalassemia with non-β0/β0 genotype. Use of Casgevy for the treatment of any other hematologic disorder is considered experimental and excluded from coverage.

☐ **3.3** 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).

☐ **3.4** 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.