BlueCross BlueShield
Federal Employee Program

REBLOZYL
(luspatercept-aamt)

RATIONALE FOR INCLUSION IN PA PROGRAM

Background

Reblozyl (luspatercept-aamt) is a recombinant fusion protein that binds several endogenous TGF-β superfamily ligands, thereby diminishing Smad2/3 signaling. Reblozyl promotes erythroid maturation through differentiation of late-stage erythroid precursors (normoblasts). In a model of β-thalassemia, Reblozyl decreased abnormally elevated Smad2/3 signaling and improved hematology parameters associated with ineffective erythropoiesis (1).

Regulatory Status

FDA-approved indications: Reblozyl is an erythroid maturation agent indicated for the treatment of: (1)

- Anemia in adult patients with beta thalassemia who require regular red blood cell (RBC) transfusions.
- Anemia without previous erythropoiesis stimulating agent use (ESA-naïve) in adult patients with very low- to intermediate-risk myelodysplastic syndromes (MDS) who may require regular red blood cell (RBC) transfusions.
- Anemia failing an erythropoiesis stimulating agent and requiring 2 or more RBC units over 8 weeks in adult patients with very low- to intermediate-risk myelodysplastic/ myeloproliferative neoplasm with ring sideroblasts and thrombocytosis (MDS/MPN-RS-T).

Limitations of Use: Reblozyl is not indicated for use as a substitute for RBC transfusions in patients who require immediate correction of anemia (1).

Thromboembolic events have been reported in patients taking Reblozyl. These events may include deep vein thromboses, pulmonary embolus, portal vein thrombosis, and ischemic strokes. Thromboprophylaxis may be considered in patients with beta thalassemia at increased risk for thromboembolic events (1).

Hypertension has also been reported in patients treated with Reblozyl. Blood pressure should be monitored prior to each administration. Extramedullary hematopoietic masses (EHM) have also been observed. EHM are clumps of blood cell precursors that form when tissues other than bone

marrow produce blood cells. In cases of patients with EHM and serious complications, Reblozyl should be discontinued (1).

Reblozyl may cause fetal harm when administered to a pregnant woman. Pregnant women should be advised of the potential risk to a fetus. Females of reproductive potential should use an effective method of contraception during treatment with Reblozyl and for at least 3 months after the final dose (1).

Hemoglobin (Hgb) should be assessed and reviewed prior to each administration. If a RBC transfusion occurred prior to dosing, the pretransfusion Hgb must be considered for dosing purposes (1).

The safety and effectiveness of Reblozyl in pediatric patients less than 18 years of age have not been established (1).

Summary

Reblozyl (luspatercept-aamt) is a recombinant fusion protein that binds several endogenous TGF-β superfamily ligands, thereby diminishing Smad2/3 signaling. Reblozyl promotes erythroid maturation through differentiation of late-stage erythroid precursors (normoblasts). In a model of β-thalassemia, Reblozyl decreased abnormally elevated Smad2/3 signaling and improved hematology parameters associated with ineffective erythropoiesis. The safety and effectiveness of Reblozyl in pediatric patients less than 18 years of age have not been established (1).

Prior approval is required to ensure the safe, clinically appropriate, and cost-effective use of Reblozyl while maintaining optimal therapeutic outcomes.

References

1. Reblozyl [package insert]. Summit, NJ: Celgene Corporation; August 2023.