Reblozyl® (luspatercept-aamt) – New indication

- On April 3, 2020, Bristol Myers Squibb and Acceleron Pharma announced the FDA approval of Reblozyl (luspatercept-aamt), for the treatment of anemia failing an erythropoiesis stimulating agent and requiring 2 or more red blood cell (RBC) units over 8 weeks in adult patients with very low- to intermediate-risk myelodysplastic syndromes with ring sideroblasts (MDS-RS) or with myelodysplastic/myeloproliferative neoplasm with ring sideroblasts and thrombocytosis (MDS/MPN-RS-T).
    - Reblozyl is not indicated for use as a substitute for RBC transfusions in patients who require immediate correction of anemia.
- Reblozyl is also approved for the treatment of anemia in adult patients with beta thalassemia who require regular RBC transfusions.
- MDS are a group of closely related blood cancers characterized by ineffective production of healthy red blood cells, white blood cells and platelets, which can lead to anemia and frequent or severe infections. People with MDS who develop anemia often require regular blood transfusions to increase the number of healthy red blood cells in circulation.
- The approval of Reblozyl for the new indication was based on MEDALIST, a randomized, double-blind, placebo-controlled trial in 229 patients with MDS. Patients were randomized to Reblozyl or placebo and all patients received best supportive care, which included RBC transfusions as needed. The primary endpoint was the proportion of patients who were RBC transfusion independent, defined as the absence of any RBC transfusion during any consecutive 8-week period occurring entirely within weeks 1 through 24.
    - The primary endpoint was met in 37.9% and 13.2% of patients receiving Reblozyl and placebo, respectively (common risk difference: 24.6; 95% CI: 14.5, 34.6; $p < 0.0001$).
- The recommended starting dose of Reblozyl for the treatment of MDS is 1 mg/kg once every 3 weeks by subcutaneous injection for patients with anemia of MDS-RS or MDS/MPN-RS-T. Prior to each Reblozyl dose, the patient's hemoglobin and transfusion record should be reviewed. The dose should be titrated based on responses as outlined in the drug label.
    - Reblozyl should be reconstituted and administered by a healthcare professional.
    - Refer to the Reblozyl drug label for dosing in beta thalassemia.