RXADVANCE
2 Park Central Drive
Southborough, MA 01772

Reblozyl (luspatercept-aamt) for Injection
Clinical Update

Clinical Update: FDA Approves Reblozyl (luspatercept-aamt) to Treat Anemia in Adults with Lower-Risk Myelodysplastic Syndromes (MDS).
FDA approval date: April 3, 2020

Reblozyl, the first and only erythroid maturation agent, promotes late-stage red blood cell maturation in animal models. Bristol Myers Squibb and Acceleron are jointly developing Reblozyl as part of a global collaboration. Reblozyl is currently approved in the U.S. for the treatment of:

- anemia in adult patients with beta thalassemia who require regular red blood cell transfusions, and
- anemia failing an erythropoiesis stimulating agent and requiring 2 or more red blood cell 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 red blood cell transfusions in patients who require immediate correction of anemia.

The approval of Reblozyl was based on the findings of MEDALIST, a Phase 3, randomized, double blind, placebo-controlled, multi-center study evaluating the efficacy and safety of Reblozyl in patients with IPSS-R-defined very low, low- and intermediate-risk non-del(5q) myelodysplastic syndromes (MDS) with ring sideroblasts. All patients were red blood cell (RBC) transfusion-dependent and were either refractory or intolerant to prior erythropoiesis-stimulating agent (ESA) therapy or were ESA naïve and unlikely to respond due to endogenous serum erythropoietin ≥200 U/L, and had no prior treatment with disease modifying agents.

In the trial, a significantly greater proportion of patients receiving Reblozyl achieved independence from RBC transfusions for at least eight weeks during the first 24 weeks of the trial compared with those receiving placebo, meeting the study's primary endpoint. Additionally, a significantly greater proportion of patients receiving Reblozyl vs. placebo achieved at least 12 weeks of independence from transfusions within the first 24 and 48 weeks of the study. The majority of treatment-emergent adverse events (TEAEs) in the trial were Grade 1-2. Grade 3 or 4 treatment-emergent adverse events were reported in 42.5% of patients who received Reblozyl and 44.7% of patients who received placebo. The most common (>10%) all-grade adverse reactions included fatigue, musculoskeletal pain, dizziness, diarrhea, dyspnea, nausea, hypersensitivity reactions, headache, and upper respiratory tract infection.