Bristol Myers Squibb™

Submitted by:
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Dear NCCN Myelodysplastic Syndromes Guidelines Panel:

On behalf of Bristol Myers Squibb, we respectfully request the NCCN Guidelines Panel for Myelodysplastic Syndromes (MDS) review the enclosed REBLOZYL® (luspatercept-aamt) Prescribing Information, which includes a recently approved indication for patients with myelodysplastic syndromes with ring sideroblasts and myelodysplastic/myeloproliferative neoplasm with ring sideroblasts and thrombocytosis associated anemia[1]. This recently updated information is being sent for the panel's consideration as a follow-up to the REBLOZYL submission made by Bristol Myers Squibb on January 9, 2020 that included the Phase III clinical trial (MEDALIST) publication and the Prescription Drug User Fee Act (PDUFA) target, or action date for the use of REBLOZYL in MDS-associated anemia.

Specific Changes:
We respectfully request including REBLOZYL within the guidelines for the treatment of myelodysplastic/myeloproliferative neoplasm with ring sideroblasts and thrombocytosis-associated anemia with a Category 2A recommendation.

FDA Status:
On April 3, 2020, the U.S. Food and Drug Administration (FDA) granted approval for the use of REBLOZYL for the treatment of 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 RBC transfusions in patients who require immediate correction of anemia.

Rationale:
The updated Prescribing Information contains a post-hoc reclassification of the MEDALIST data using the World Health Organization (WHO) 2016 Diagnostic Criteria for MDS[2], which includes MDS/MPN-RS-T.

In the overall MEDALIST population, 37.9% (58/153) of REBLOZYL-treated patients and 13.2% (10/76) of placebo-treated patients achieved the primary endpoint of red blood cell transfusion independence ≥8 weeks (during Weeks 1-24), respectively (Common Risk Difference [95% CI]: 24.6 [14.5, 34.6]; $P<0.0001$). Among patients with a diagnosis of MDS/MPN-RS-T, 64.3% (9/14) and 22.2% (2/9) achieved the primary endpoint in the REBLOZYL and placebo arms, respectively.

Safety data has been updated to include results from patients treated on Phase II PACE study and Phase III MEDALIST study. Please see the enclosed full Prescribing Information.

Thank you for your consideration.

Sincerely,

Arpit Shah, PharmD
Associate Director, Global Medical Information

Chrystal U Louis, MD, MPH
Executive Director, US Medical Affairs, Myeloid

Reference List:

1. Celgene Corporation. Reblozyl (luspatercept-aamt) [Package Insert]. Summit, NJ: Celgene Corporation.
2. Arber DA, Orazi A, Hasserjian R, et al. The 2016 revision to the World Health Organization classification of myeloid neoplasms and acute leukemia. *Blood*. 2016;127(20):2391-2405.