Submitted by:
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Date of Request: January 9, 2020

Dear NCCN Myelodysplastic Syndromes Guidelines Panel:

On behalf of Bristol-Myers Squibb Company, we respectfully request that the NCCN Guidelines Panel for Myelodysplastic Syndromes (MDS) review the enclosed data regarding the use of REBLOZYL® (luspatercept-aamt) in patients with very low-, low-, or intermediate-risk myelodysplastic syndromes (MDS)-associated anemia who require red blood cell transfusions. This data, recently published in the New England Journal of Medicine, is being sent for the panel's consideration as a follow-up to the REBLOZYL submission made by Celgene Corporation on November 20, 2019.[†]

Specific Changes:
As noted in the November 20, 2019 submission, we respectfully request including REBLOZYL within the algorithm for the treatment of symptomatic anemia in patients with MDS (with no deletion 5q +/- other cytogenetic abnormalities) with a Category 2A recommendation.

FDA Status:
REBLOZYL is indicated for the treatment of anemia in adult patients with beta thalassemia who require regular red blood cell (RBC) transfusions. REBLOZYL is not indicated for use as a substitute for RBC transfusions in patients who require immediate correction of anemia. Please see the enclosed full Prescribing Information.

The use of REBLOZYL in MDS is investigational. The US Food and Drug Administration (FDA) is evaluating REBLOZYL for the treatment of anemia in adults with very low- to intermediate-risk MDS who have ring sideroblasts and require RBC transfusions. The FDA has granted a Prescription Drug User Fee Act (PDUFA) target, or action date of April 4, 2020 for the use of REBLOZYL in MDS.

Rationale:
The November 20, 2019 submission was based on the results of the phase III clinical study evaluating the efficacy and safety of luspatercept-aamt compared to placebo in patients with MDS-associated anemia and included the oral presentation of data from that study. The attached full publication and supplementary appendix include additional efficacy and safety endpoints from the phase III study that were not included in the previously submitted data.

Your consideration of this submission is greatly appreciated.

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. Fenaux P, Platzbecker U, Mufti GJ, et al. Luspatercept in Patients with Lower-Risk Myelodysplastic Syndromes. *N Engl J Med*. 2020;382(2):140-151.

[†]Celgene has since been acquired by Bristol-Myers Squibb Company.