CENTER FOR DRUG EVALUATION AND RESEARCH

APPLICATION NUMBER:

761136Orig2s000

NON-CLINICAL REVIEW(S)

MEMORANDUM

Date: December 31, 2019
To: File for BLA 761136
From: Michael L Manning, PhD
Pharmacology-Toxicology Reviewer
Division of Hematology Oncology Toxicology (DHOT)
Office of Oncologic Diseases (OOD)
Through: Haleh Saber, PhD
Deputy Director
Division of Hematology Oncology Toxicology (DHOT)
Office of Oncologic Diseases (OOD)
Subject: Nonclinical review for BLA 761136-Orig 2
BLA: 761136
Applicant: Celgene Corporation
Drug: REBLOZYL® (luspatercept-aamt)

REBLOZYL® (luspatercept-aamt) is a recombinant fusion protein comprised of a modified form of the extracellular domain of human activin receptor type IIB and a human IgG1 Fc domain. Luspatercept-aamt was approved in November 2019 for the treatment of anemia in adult patients with beta thalassemia who require regular red blood cell (RBC) transfusions. The current application seeks to add a new indication: for the treatment of adult patients with very low- to intermediate-risk myelodysplastic syndromes (MDS)-associated anemia who have ring sideroblasts and require RBC transfusions.

The nonclinical pharmacology and toxicology information necessary to support the approval of the current application was reviewed to support the approval of the previous application and is applicable to the current indication of MDS-associated anemia. The product label will be revised to 1) describe the activity of luspatercept in MDS-associated anemia based on nonclinical studies, and 2) update the animal-to-human exposure ratios based on the recommended dose(s) of luspatercept-aamt in patients with MDS. Additional revisions may be made to the product label, as deemed necessary.

There are no nonclinical issues that would prevent the approval of this application.

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/s/

MICHAEL L MANNING
12/31/2019 08:18:46 AM

HALEH SABER
12/31/2019 08:20:34 AM