U.S. Food and Drug Administration (FDA) Approves Reblozyl® (luspatercept-aamt), the First and Only Erythroid Maturation Agent, to Treat Anemia in Adults with Lower-Risk Myelodysplastic Syndromes (MDS)

Release Date:
Friday, April 3, 2020 5:08 pm EDT

Terms:
Corporate/Financial News  #anemia  #FDA  #MDS  #Reblozyl  $BMY  $XLRN  anemia  MDS  patients  Reblozyl

Dateline City:

The FDA approval marks the second indication for Reblozyl and the first new treatment option in over a decade for patients with MDS who require red blood cell (RBC) transfusions and have failed an erythropoiesis stimulating agent.

Reblozyl regulates late-stage RBC maturation to relieve patients from the burden of regular RBC transfusions.

In clinical trials, Reblozyl has shown to have significant benefit for the treatment of anemia in patients with myelodysplastic syndromes who have ring sideroblasts, said Guillermo Garcia-Manero, M.D., professor and chief of Section of Myelodysplastic Syndromes, Department of Leukemia, University of Texas MD Anderson Cancer Center. “Anemia is a serious consequence of MDS, requiring the majority of these patients to receive regular red blood cell transfusions, which can lead to additional complications, such as iron overload, transfusion site reactions and infections. In our current environment, we are reminded of the significant burden frequent blood transfusions can have on individuals and the healthcare system.”

“Today’s approval of Reblozyl is an important milestone for a majority of patients with myelodysplastic syndromes who have limited treatment options to address anemia associated with their disease. It also demonstrates our continued commitment to develop innovative products that improve the lives of patients living with serious diseases,” said Diane McDowell, M.D., vice president, Hematology Global Medical Affairs, Bristol Myers Squibb. “We are looking forward to making Reblozyl immediately available for this patient population.”

The FDA approval in MDS is based on results from the pivotal Phase 3 MEDALIST trial and marks the second indication for Reblozyl, which received its first approval in November 2019 for the treatment of anemia in adults with beta thalassemia who require regular RBC transfusions.

“We’re excited that Reblozyl has been approved to help even more patients in need of treatment options,” said Habib Dable, President and Chief Executive Officer, Acceleron. “We are enormously grateful to the patients, families and caregivers who participated in and supported the Reblozyl clinical trials, and to the researchers at Acceleron and beyond who, more than a decade ago, began this important quest to address patients’ chronic anemias.”

About MEDALIST
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.

Results from MEDALIST were published in the New England Journal of Medicine in January 2020.

About MDS
Myelodysplastic syndromes (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. Frequent transfusions are associated with an increased risk of iron overload, transfusion reactions and infections.

About Reblozyl®
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:
- 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.

Indication
REBLOZYL is indicated for the treatment of anemia in adult patients with beta thalassemia who require regular red blood cell (RBC) transfusions

REBLOZYL is indicated 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

Important Safety Information

WARNINGS AND PRECAUTIONS
Thrombosis/Thromboembolism
In adult patients with beta thalassemia, thromboembolic events (TEE) were reported in 8/223 (3.6%) REBLOZYL-treated patients. TEEs included deep vein thrombosis, pulmonary embolus, portal vein thrombosis, and ischemic stroke. Patients with known risk factors for thromboembolism (splenectomy or concomitant use of hormone replacement therapy) may be at further increased risk of TEE. Consider thromboprophylaxis in patients at increased risk of TEE. Monitor patients for signs and symptoms of thromboembolic events and institute treatment promptly.

Hypertension
Hypertension was reported in 10.7% (61/571) of REBLOZYL-treated patients. Across clinical studies, the incidence of Grade 3 to 4 hypertension ranged from 1.8% to 8.6%. In patients with beta thalassemia with normal baseline blood pressure, 13 (6.2%) patients developed systolic blood pressure (SBP) ≥130 mm Hg and 33 (16.6%) patients developed diastolic blood pressure (DBP) ≥80 mm Hg. In adult patients with MDS with normal baseline blood pressure, 26 (29.9%) patients developed SBP ≥130 mm Hg and 23 (16.4%) patients developed DBP ≥80 mm Hg. Monitor blood pressure prior to each administration. Manage new or exacerbations of preexisting hypertension using anti-hypertensive agents.

Embryo-Fetal Toxicity
REBLOZYL may cause fetal harm when administered to a pregnant woman. REBLOZYL caused increased post-implantation loss, decreased litter size, and an increased incidence of skeletal variations in pregnant rat and rabbit studies. Advise pregnant women of the potential risk to a fetus. Advise females of reproductive potential to use effective contraception during treatment and for at least 3 months after the final dose.

ADVERSE REACTIONS
Beta-Thalassemia

- Serious adverse reactions occurred in 3.6% of patients on REBLOZYL. Serious adverse reactions occurring in 1% of patients included cerebrovascular accident and deep vein thrombosis. A fatal adverse reaction occurred in 1 patient treated with REBLOZYL who died due to an unconfirmed case of acute myeloid leukemia (AML)

- Most common adverse reactions (at least 10% for REBLOZYL and 1% more than placebo) were headache (26% vs 24%), bone pain (20% vs 8%), arthralgia (19% vs 12%), fatigue (14% vs 13%), cough (14% vs 11%), abdominal pain (14% vs 12%), diarrhea (12% vs 10%) and dizziness (11% vs 5%)
Myelodysplastic Syndromes

- Grade ≥3 (>2%) adverse reactions included fatigue, hypertension, syncope and musculoskeletal pain. A fatal adverse reaction occurred in 5 (2.1%) patients
- The most common (>10%) adverse reactions included fatigue, musculoskeletal pain, dizziness, diarrhea, nausea, hypersensitivity reactions, hypertension, headache, upper respiratory tract infection, bronchitis, and urinary tract infection

LACTATION

It is not known whether REBLOZYL is excreted into human milk or absorbed systemically after ingestion by a nursing infant. REBLOZYL was detected in milk of lactating rats. When a drug is present in animal milk, it is likely that the drug will be present in human milk. Because many drugs are excreted in human milk, and because of the unknown effects of REBLOZYL in infants, a decision should be made whether to discontinue nursing or to discontinue treatment. Because of the potential for serious adverse reactions in the breastfed child, breastfeeding is not recommended during treatment and for 3 months after the last dose.

Please see full Prescribing Information for REBLOZYL.

Bristol Myers Squibb: Advancing Cancer Research

At Bristol Myers Squibb, patients are at the center of everything we do. The goal of our cancer research is to increase quality, long-term survival and make cure a possibility. We harness our deep scientific experience, cutting-edge technologies and discovery platforms to discover, develop and deliver novel treatments for patients.

Building upon our transformative work and legacy in hematology and Immuno-Oncology that has changed survival expectations for many cancers, our researchers are advancing a deep and diverse pipeline across multiple modalities. In the field of immune cell therapy, this includes registrational chimeric antigen receptor (CAR) T-cell agents for numerous diseases, and a growing early-stage pipeline that expands cell and gene therapy targets, and technologies. We are developing cancer treatments directed at key biological pathways using our protein homeostasis platform, a research capability that has been the basis of our approved therapies for multiple myeloma and several promising compounds in early to mid-stage development. Our scientists are targeting different immune system pathways to address interactions between tumors, the microenvironment and the immune system to further expand upon the progress we have made and help more patients respond to treatment. Combining these approaches is key to delivering new options for the treatment of cancer and addressing the growing issue of resistance to immunotherapy. We source innovation internally, and in collaboration with academia, government, advocacy groups and biotechnology companies, to help make the promise of transformational medicines a reality for patients.

About Bristol Myers Squibb

Bristol Myers Squibb is a global biopharmaceutical company whose mission is to discover, develop and deliver innovative medicines that help patients prevail over serious diseases. For more information about Bristol Myers Squibb, visit us at BMS.com or follow us on LinkedIn, Twitter, YouTube, Facebook and Instagram.

Celgene and Juno Therapeutics are wholly owned subsidiaries of Bristol-Myers Squibb Company. In certain countries outside the U.S., due to local laws, Celgene and Juno Therapeutics are referred to as, Celgene, a Bristol-Myers Squibb Company and Juno Therapeutics, a Bristol-Myers Squibb Company.

About Acceleron

Acceleron is a biopharmaceutical company dedicated to the discovery, development, and commercialization of therapeutics to treat serious and rare diseases. Acceleron’s leadership in the understanding of TGF-beta superfamily biology and protein engineering generates innovative compounds that engage the body’s ability to regulate cellular growth and repair.

Acceleron focuses its research and development efforts in hematologic and pulmonary diseases. In hematology, Acceleron and its global collaboration partner, Bristol-Myers Squibb, are co-promoting newly approved REBLOZYL® (luspatercept-aamt), the first and only approved erythroid maturation agent, in the United States and are developing luspatercept for the treatment of chronic anemia in myelofibrosis. Acceleron is developing sotatercept for the treatment of pulmonary arterial hypertension, having recently reported positive topline results of the Phase 2 PULSAR trial and actively enrolling patients in the Phase 2 SPECTRA trial.

For more information, please visit www.acceleronpharma.com. Follow Acceleron on social media: @AcceleronPharma and LinkedIn.

Bristol Myers Squibb Cautionary Statement Regarding Forward-Looking Statements

This press release contains “forward-looking statements” within the meaning of the Private Securities Litigation Reform Act of 1995 regarding, among other things, the research, development and commercialization of pharmaceutical products. All statements that are not statements of historical facts are, or may be deemed to be, forward-looking statements. Such forward-looking statements are based on historical performance and current expectations and projections about our future financial results, goals, plans and objectives and involve inherent risks, assumptions and uncertainties, including internal or external factors that could delay, divert or change any of them in the next several years, that are difficult to predict, may be beyond our control and could cause our future financial results, goals, plans and objectives to differ materially from those expressed in, or implied by, the statements. These risks, assumptions, uncertainties and other factors include, among others, whether Reblozyl for the additional indication described in this release will be commercially successful and that continued approval of Reblozyl for such additional indication described in this release may be contingent upon verification and description of clinical benefit in confirmatory trials. No forward-looking statement can be guaranteed. Forward-looking statements in this press release should be evaluated together with the many risks and uncertainties that affect Bristol Myers Squibb’s business and market, particularly those identified in the cautionary statement and risk factors discussion in Bristol Myers Squibb’s Annual Report on Form 10-K for the year ended December 31, 2019, as updated by our subsequent Quarterly Reports on Form 10-Q, Current Reports on Form 8-K and other filings with the Securities and Exchange Commission. The forward-looking statements included in this document are made only as of the date of this document and except as otherwise required by applicable law, Bristol Myers Squibb undertakes no obligation to publicly update or revise any forward-
looking statement, whether as a result of new information, future events, changed circumstances or otherwise.

**Acceleron Cautionary Statement Regarding Forward-Looking Statements**

This press release contains forward-looking statements about Acceleron's strategy, future plans and prospects, including statements regarding the development and commercialization of Acceleron's compounds, the timeline for clinical development and regulatory approval of Acceleron’s, the expected timing for reporting of data from ongoing clinical trials, and the potential of Reblozyl® (luspatercept-aamt) as a therapeutic drug. The words “anticipate,” “believe,” “could,” “estimate,” “expect,” “goal,” “intend,” “may,” “plan,” “potential,” “project,” “should,” “target,” “will,” “would,” and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words.

Actual results could differ materially from those included in the forward-looking statements due to various factors, risks and uncertainties, including, but not limited to, that the results of any clinical trials may not be predictive of the results or success of other clinical trials, that regulatory approval of Acceleron’s compounds in one indication or country may not be predictive of approval in another indication or country, that the development of Acceleron’s compounds will take longer and/or cost more than planned or accelerate faster than currently expected, that Acceleron or its collaboration partner, Bristol Myers Squibb Corporation (“BMS”), will be unable to successfully complete the clinical development of Acceleron’s compounds, that Acceleron or BMS may be delayed in initiating, enrolling or completing any clinical trials, and that Acceleron’s compounds will not receive regulatory approval or become commercially successful products. These and other risks and uncertainties are identified under the heading “Risk Factors” included in Acceleron’s most recent Annual Report on Form 10-K, and other filings that Acceleron has made and may make with the SEC in the future.

The forward-looking statements contained in this press release are based on management’s current views, plans, estimates, assumptions, and projections with respect to future events, and Acceleron does not undertake and specifically disclaims any obligation to update any forward-looking statements.

---


---

**Language:**

English

**Contact:**

Bristol Myers Squibb
Media Inquiries:
media@bms.com
609-252-3348

Rose Weldon
rose.weldon@bms.com

Investors:
Tim Power
609-252-7509
timothy.power@bms.com

Nina Goworek
908-673-9711
ngworek@celgene.com

Acceleron Pharma Inc.
Media:
Matt Fearer, (617) 301-9557
Director, Corporate Communication

Investors:
Todd James, IRC, (617) 649-9393
Senior Vice President, Corporate Affairs and Investor Relations

**Ticker Slug:**

Ticker: BMY
Exchange: NYSE
BMY announces #FDA approval of first new treatment in 15 years for patients with #MDS