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# Press Release

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## U.S. Food and Drug Administration (FDA) Accepts for Priority Review Bristol-Myers Squibb's Biologics License Application (BLA) for Lisocabtagene Maraleucel (liso-cel) for Adult Patients with Relapsed or Refractory Large B-Cell Lymphoma

U.S. FDA set a target action date of August 17, 2020

Application based on results from the TRANSCEND NHL 001 trial, the largest study of CD19-directed CAR T cells to support a BLA to date

Liso-cel is a CD19-directed CAR T-cell product with a defined composition of CAR+ viable T cells consisting of purified CD8+ and CD4+ T cells

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PRINCETON, N.J.--(BUSINESS WIRE)--[Bristol-Myers Squibb Company](#) (NYSE: BMY) today announced that the U.S. Food and Drug Administration (FDA) has accepted for Priority Review its Biologics License Application (BLA) for lisocabtagene maraleucel (liso-cel), the company's autologous anti-CD19 chimeric antigen receptor (CAR) T-cell immunotherapy with a defined composition of purified CD8+ and CD4+ CAR T cells for the treatment of adult patients with relapsed or refractory (R/R) large B-cell lymphoma after at least two prior therapies. The FDA has set a Prescription Drug User Fee Act (PDUFA) goal date of August 17, 2020.

“There remains a critical need for additional therapies in large B-cell lymphoma, particularly for relapsed or refractory patients,” said Stanley Frankel, M.D., senior vice president, Cellular Therapy Development, Bristol-Myers Squibb. “Based on the TRANSCEND NHL 001 data, liso-cel has the potential to expand treatment options for those affected by this aggressive blood cancer who did not respond to initial therapies or whose disease has relapsed. This BLA acceptance and Priority Review designation is an important step as we work to improve treatment for these patients in need.”

The BLA, submitted by Juno Therapeutics, a wholly owned subsidiary of Bristol-Myers Squibb Company, is based on the safety and efficacy results from the TRANSCEND NHL 001 trial, evaluating liso-cel in 268 patients with R/R large B-cell lymphoma, including diffuse large B-cell lymphoma (DLBCL), high-grade lymphoma, primary mediastinal B-cell lymphoma and Grade 3B follicular lymphoma. TRANSCEND NHL 001 is the largest study of CD19-directed CAR T cells to support a BLA to date and was recently the subject of an oral presentation at the 61st American Society of Hematology Annual Meeting and Exposition.

According to the FDA, a Priority Review designation will direct overall attention and resources to the evaluation of applications for drugs that, if approved, would be significant improvements in the safety or effectiveness of the treatment, diagnosis, or prevention of serious conditions when compared to standard applications.

Liso-cel was previously granted Breakthrough Therapy and Regenerative Medicine Advanced Therapy designations by the FDA for R/R aggressive large B-cell non-Hodgkin lymphoma, including DLBCL, not otherwise specified (de novo or transformed from indolent lymphoma), PMBCL or Grade 3B FL, and Priority Medicines (PRIME) scheme by the European Medicines Agency for R/R DLBCL.

Liso-cel is an investigational compound that is not approved for use in any country.

### **About Large B-cell Lymphoma**

Diffuse large B-cell lymphoma (DLBCL) is the most common of large B-cell lymphomas. It is an aggressive form of non-Hodgkin lymphoma (NHL), accounting for three out of every

five cases. Approximately one-third of patients with DLBCL relapse after receiving first-line treatment, and about 10% have refractory disease. Historically, median life expectancy for patients who relapse or are refractory to current standard of care treatments is approximately six months.

### **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 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 Lisocabtagene Maraleucel (liso-cel)**

Liso-cel is an investigational chimeric antigen receptor (CAR) T-cell therapy designed to target CD19, which is a surface glycoprotein expressed during normal B-cell development

and maintained following malignant transformation of B cells. Liso-cel aims to target CD19-expressing cells through a CAR construct that includes an anti-CD19 single-chain variable fragment (scFv) targeting domain for antigen specificity, a transmembrane domain, a 4-1BB costimulatory domain hypothesized to increase T-cell proliferation and persistence, and a CD3-zeta T-cell activation domain. The defined composition of CAR-positive viable T-cells (consisting of CD8 and CD4 components) in liso-cel may reduce product variability; however, the clinical significance of defined composition is unknown.

### **About TRANSCEND NHL 001**

TRANSCEND NHL 001 is an open-label, multicenter, pivotal phase 1 study to determine the safety, antitumor activity, and pharmacokinetics of liso-cel in patients with R/R B-cell NHL, including DLBCL, HGL, PMBCL, Grade 3B FL. Mantle cell lymphoma is investigated in a separate cohort. The primary outcome measures included treatment-related adverse events, dose-limiting toxicities and objective response rate. Key secondary outcome measures included complete response rate, duration of response, and progression-free survival. The TRANSCEND program is a broad clinical program evaluating liso-cel in multiple disease states and treatment stages.

### **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](https://www.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.

### **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, the possibility of unfavorable results from additional studies involving liso-cel, that such product candidate may not receive regulatory approval for the indications described in this release in the currently anticipated timeline or at all and, if approved, whether such product candidate for such additional indications described in this release will be commercially successful. No forward-looking statement can be guaranteed. It should be noted that a priority review designation does not change the standards for FDA approval. 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, 2018, 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.*

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