# FDA Approves Tisagenlecleucel for B-Cell ALL and Tocilizumab for Cytokine Release Syndrome

Kymriah is the first chimeric antigen receptor (CAR) T-cell immunotherapy approved by the FDA.

August 30, 2017 By Food and Drug Administration (FDA)

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On August 30, 2017, the U.S. Food and Drug Administration granted regular approval to tisagenlecleucel (KYMRIAH, Novartis Pharmaceuticals Corp.) for the treatment of patients up to age 25 years with B-cell precursor acute lymphoblastic leukemia (ALL) that is refractory or in second or later relapse.

Tisagenlecleucel is the first chimeric antigen receptor (CAR) T-cell immunotherapy approved by the FDA. Tisagenlecleucel consists of autologous T cells collected in a leukapheresis procedure that are genetically modified with a new gene containing a CAR protein allowing the T cells to identify and eliminate CD19-expressing normal and malignant cells.

Approval of tisagenlecleucel was based on a single-arm trial of 63 patients with relapsed or refractory pediatric precursor B-cell ALL, including 35 patients who had a prior hematopoietic stem-cell transplantation. Patients received a single dose of tisagenlecleucel intravenously within 2 to 14 days following the completion of lymphodepleting chemotherapy. Of the 63 patients who were evaluable for efficacy, the confirmed overall remission rate as assessed by independent central review was 82.5% (95% CI 70.9, 91.0), consisting of 63% of patients with complete remission (CR) and 19% with complete remission with incomplete hematological recovery (CRi). All patients with a confirmed CR or CRi were minimal residual disease negative by flow cytometry. Median remission duration was not reached (range: 1.2 to 14.1+ months).

The most common adverse reactions (incidence greater than 20%) are cytokine release syndrome, hypogammaglobulinemia, infections-pathogen unspecified, pyrexia, decreased appetite, headache, encephalopathy, hypotension, bleeding episodes, tachycardia, nausea, diarrhea, vomiting, viral infectious disorders, hypoxia, fatigue, acute kidney injury, and delirium. Grade 3 or 4 adverse events were noted in 84% of patients.

Serious adverse reactions such as CRS, including fatal CRS and CRS-associated disseminated intravascular coagulation with intracranial hemorrhage, prolonged cytopenia, infection, cardiac failure, and cardiac arrest occurred in patients receiving tisagenlecleucel. FDA approved

tisagenlecleucel with a Risk Evaluation and Mitigation Strategy.

The recommended tisagenlecleucel dose is one infusion of 0.2 to 5.0 x $10^6$ (CAR)-positive viable T cells per kg body weight intravenously for patients who are ≤ 50 kg, and 0.1 to 2.5 x $10^8$ total CAR-positive viable T cells intravenously for patients who are > 50 kg, administered 2 to 14 days after lymphodepleting chemotherapy.

Full prescribing information for tisagenlecleucel is available at: https://www.fda.gov/downloads/BiologicsBloodVaccines/CellularGeneTherapyProducts/ApprovedProducts/UCM573941.pdf

The FDA also approved today tocilizumab (ACTEMRA, Genentech Inc.) for the treatment of patients 2 years of age or older with cytokine release syndrome (CRS) that occurs with CAR T-cell therapy. In an analysis of data from clinical trials of CAR-T cells, 69% of patients with severe or life-threatening CRS had resolution of CRS within 2 weeks following one or two doses of tocilizumab.

Full prescribing information for tocilizumab is available at: https://www.accessdata.fda.gov/drugsatfda_docs/label/2017/125276s114lbl.pdf.

Healthcare professionals should report all serious adverse events suspected to be associated with the use of any medicine and device to FDA's MedWatch Reporting System by completing a form online at http://www.fda.gov/medwatch/report.htm, by faxing (1-800-FDA-0178) or mailing the postage-paid address form provided online, or by telephone (1-800-FDA-1088).

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