Kymriah™ (tisagenlecleucel) – New indication

- On May 1, 2018, Novartis announced the FDA approval of Kymriah (tisagenlecleucel) for the treatment of adult patients with relapsed or refractory large B-cell lymphoma after 2 or more lines of systemic therapy including diffuse large B-cell lymphoma (DLBCL) not otherwise specified, high grade B-cell lymphoma and DLBCL arising from follicular lymphoma.
    - Kymriah is not indicated for the treatment of patients with primary central nervous system lymphoma.
- Kymriah is also indicated for the treatment of patients up to 25 years of age with B-cell precursor acute lymphoblastic leukemia (ALL) that is refractory or in second or later relapse.
- DLBCL is the most common form of non-Hodgkin lymphoma. For patients who relapse or don't respond to initial therapy, there are limited treatment options that provide durable responses, and the median life expectancy is approximately 6 months.
- The safety and efficacy of Kymriah in refractory DLBCL was evaluated in an open-label, single-arm trial in adults who had received ≥ 2 lines of chemotherapy or relapsed from hematopoietic stem cell transplantation. Following 2 to 11 days after completing lymphodepleting chemotherapy, Kymriah was administered as a single intravenous (IV) infusion. Bridging chemotherapy was permitted to control disease burden.
    - Complete response was achieved in 32% of patients (95% CI: 21.5%, 44.8%).
    - The median duration of response had not been reached at the time of analysis.
    - The overall response rate was 50% (95% CI: 37.6%, 62.4%).
- Kymriah carries a boxed warning for cytokine release syndrome and neurological toxicities.
    - Kymriah is available through a Risk Evaluation and Mitigation Strategy (REMS) program to inform and educate healthcare professionals about Kymriah's risks.
- The most common adverse reactions (> 20%) with Kymriah use in DLBCL patients were cytokine release syndrome, infections (pathogens unspecified), pyrexia, diarrhea, nausea, fatigue, hypotension, edema, and headache.
- In adult patients with DLBCL, Kymriah is provided as a single-dose of 0.6 to 6.0 x 10^8 chimeric antigen receptor-positive viable T-cells for IV infusion.
    - Refer to the Kymriah drug label for dosing information in ALL.
- To ensure that eligible patients have access to Kymriah, Novartis continues to collaborate with the Centers for Medicare and Medicaid Services (CMS) on the creation of an appropriate value-based pricing approach.