This English version is intended to be a reference material for the convenience of users. In the event of inconsistency between the Japanese original and this English translation, the former shall prevail.

Summary of Investigation Results

Tisagenlecleucel

February 25, 2021

Non-proprietary name

Tisagenlecleucel (Novartis Pharma K.K.)

Branded name (Marketing authorization holder)

Kymriah suspension for intravenous infusion

Indications

1. Relapsed or refractory CD19 positive B-cell acute lymphoblastic leukaemia only when:
   - First-episode patients have failed to achieve remission by 2 or more lines of standard chemotherapy, or
   - Relapsed patients have failed to achieve remission by 1 or more lines of standard therapy, or
   - Patients are ineligible for allogeneic haematopoietic stem cell transplant or have had a relapse after allogeneic haematopoietic stem cell transplant.
2. Relapsed or refractory CD19 positive diffuse large B-cell lymphoma, limited to patients who are not eligible for autologous haematopoietic stem cell transplant or who have had a relapse after autologous haematopoietic stem cell transplant and only when:
   - First-episode patients have failed to achieve complete remission by 2 or more lines of chemotherapy, or relapsed patients have failed to achieve complete response to 1 or more lines of chemotherapy or have achieved complete response followed by a relapse, or
   - Patients with transformed follicular lymphoma have received 2 or more lines of chemotherapy in total with 1 or more lines performed after transformation and have failed to achieve complete response to the line(s) of post-transformation chemotherapy or have achieved complete response followed by a relapse.

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Summary of revisions

"Infusion reaction, anaphylaxis" should be added to the Clinically Significant Adverse Reactions section.

Investigation results and background of the revision

Cases of infusion reaction or anaphylaxis have been reported in patients treated with tisagenlecleucel overseas. MHLW/PMDA in consultation with expert advisors concluded that revision of the package insert was necessary.

Number of cases and patient mortalities reported in Japan during the previous 3 fiscal years

No cases of infusion reaction or anaphylaxis have been reported to date.
(Japanese market launch: May 2019)

The expert advisors present at the Expert Discussion regarding the current investigation were nominated based on their conflict of interest declarations concerning the relevant products, pursuant to the "Rules for Convening Expert Discussions, etc., by the Pharmaceuticals and Medical Devices Agency" (PMDA Administrative Rule No. 20-8, dated December 25, 2008).

Pharmaceuticals and Medical Devices Agency

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