Chimeric Antigen Receptor T-Cell Therapies for B-Cell Cancers: Effectiveness and Value

Draft Questions for Deliberation and Voting
March 2, 2018 Public Meeting

These questions are intended for the deliberation of the CTAF voting body at the public meeting.

Population 1: Pediatric B-cell Acute Lymphoblastic Leukemia

Clinical Evidence

Patient Population for questions 1-6: Patients ages 0-25 years with B-cell acute lymphoblastic leukemia that is refractory or in second or greater relapse.

1. Is the evidence adequate to demonstrate a net health benefit for treatment with tisagenlecleucel (Kymriah™, Novartis) versus treatment with clofarabine or comparable chemotherapy (e.g., blinatumomab, multi-agent chemotherapy including clofarabine)?

   Yes No

Contextual Considerations/Other Benefits

2. Does treating patients with tisagenlecleucel offer one or more of the following "other benefits?" (select all that apply)

☐ This intervention provides significant direct patient health benefits that are not adequately captured by the QALY.
☐ This intervention offers reduced complexity that will significantly improve patient outcomes.
☐This intervention will reduce important health disparities across racial, ethnic, gender, socioeconomic, or regional categories.
☐This intervention will significantly reduce caregiver or broader family burden.
☐This intervention offers a novel mechanism of action or approach that will allow successful treatment of many patients who have failed other available treatments.
☐ This intervention will have a significant impact on improving return to work and/or overall productivity.

3. Are any of the following contextual considerations important in assessing tisagenlecleucel's long-term value for money? (select all that apply)

☐ This intervention is intended for the care of individuals with a condition of particularly high severity in terms of impact on length of life and/or quality of life.
☐ This intervention is intended for the care of individuals with a condition that represents a particularly high lifetime burden of illness.
☐ This intervention is the first to offer any improvement for patients with this condition.
☐ Compared to standard therapy there is significant uncertainty about the longterm risk of serious side effects of this intervention.
☐ Compared to standard therapy, there is significant uncertainty about the magnitude or durability of the long-term benefits of this intervention.

Long-term Value for Money

4. Given the available evidence on comparative effectiveness and incremental cost-effectiveness, and considering other benefits, disadvantages, and contextual considerations, what is the long-term value for money of treatment with tisagenlecleucel versus treatment with clofarabine?

a. Low b. Intermediate c. High

Population 2: Adult Aggressive B-cell Lymphoma

Clinical Evidence

Patient Population for questions 7-12: Patients ages 18 years and older aggressive B-cell lymphoma that is refractory or in second or greater relapse.

5. Is the evidence adequate to demonstrate a net health benefit for treatment with axicabtagene ciloleucel (Yescarta™, Kite/Gilead) versus treatment with the regimens assessed in the SCHOLAR-1 trial?

   Yes No

6. Is the evidence adequate to demonstrate a net health benefit for treatment with tisagenlecleucel versus treatment with the regimens assessed in the SCHOLAR-1 trial?

   Yes No

7. Is the evidence adequate to distinguish the net health benefit between axicabtagene ciloleucel and tisagenlecleucel?

   Yes No

Contextual Considerations/Other Benefits

8. Does treating patients with axicabtagene ciloleucel offer one or more of the following "other benefits?" (select all that apply)

☐ This intervention provides significant direct patient health benefits that are not adequately captured by the QALY.
☐ This intervention offers reduced complexity that will significantly improve patient outcomes.
☐This intervention will reduce important health disparities across racial, ethnic, gender, socioeconomic, or regional categories.
☐This intervention will significantly reduce caregiver or broader family burden.
☐This intervention offers a novel mechanism of action or approach that will allow successful treatment of many patients who have failed other available treatments.
☐ This intervention will have a significant impact on improving return to work and/or overall productivity.

9. Are any of the following contextual considerations important in assessing axicabtagene ciloleucel's long-term value for money? (select all that apply)

☐ This intervention is intended for the care of individuals with a condition of particularly high severity in terms of impact on length of life and/or quality of life.
☐ This intervention is intended for the care of individuals with a condition that represents a particularly high lifetime burden of illness.
☐ This intervention is the first to offer any improvement for patients with this condition.
☐ Compared to standard therapy there is significant uncertainty about the longterm risk of serious side effects of this intervention.
☐ Compared to standard therapy, there is significant uncertainty about the magnitude or durability of the long-term benefits of this intervention.

Long-term Value for Money

10. Given the available evidence on comparative effectiveness and incremental cost-effectiveness, and considering other benefits, disadvantages, and contextual considerations, what is the long-term value for money of treatment with axicabtagene ciloleucel versus treatment with the regimens assessed in the SCHOLAR-1 trial?

a. Low b. Intermediate c. High