



# 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.*

### Pediatric B-cell Acute Lymphoblastic Leukemia

*Patient Population for questions 1-4: Patients ages 0-25 years with B-cell acute lymphoblastic leukemia that is refractory or in second or greater relapse*

#### Clinical Evidence

1. Is the evidence adequate to demonstrate a net health benefit for treatment with tisagenlecleucel (Kymriah™, Novartis) versus treatment with clofarabine (Clolar®, Sanofi-Genzyme) or comparable chemotherapy (e.g., blinatumomab [Blincyto®, Amgen], 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?” (yes, no, uncertain)
  - a. This intervention provides significant direct patient health benefits that are not adequately captured by the QALY.
  - b. This intervention offers reduced complexity that will significantly improve patient outcomes.
  - c. This intervention will reduce important health disparities across racial, ethnic, gender, socioeconomic, or regional categories.
  - d. This intervention will significantly reduce caregiver or broader family burden.
  - e. This intervention offers a novel mechanism of action or approach that will allow successful treatment of many patients who have failed other available treatments.
  - f. This intervention will have a significant impact on improving patients’ ability to return to work and/or their overall productivity.
  - g. There are other important benefits or disadvantages that should have an important role in judgments of the value of this intervention: \_\_\_\_\_

3. Are any of the following contextual considerations important in assessing tisagenlecleucel’s long-term value for money? (yes, no, uncertain)
  - a. 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.
  - b. This intervention is intended for the care of individuals with a condition that represents a particularly high lifetime burden of illness.
  - c. This intervention is the first to offer any improvement for patients with this condition.
  - d. Compared to standard therapy, there is significant uncertainty about the long-term risk of serious side effects of this intervention.
  - e. Compared to standard therapy, there is significant uncertainty about the magnitude or durability of the long-term benefits of this intervention.
  - f. There are additional contextual considerations that should have an important role in judgments of the value 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

### Adult Aggressive B-cell Lymphoma

*Patient Population for questions 5-10: Patients ages 18 years and older aggressive B-cell lymphoma that is refractory or in second or greater relapse.*

### Clinical Evidence

5. Is the evidence adequate to demonstrate a net health benefit for treatment with axicabtagene ciloleucel (Yescarta™, Kite Pharma/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)
- a. This intervention provides significant direct patient health benefits that are not adequately captured by the QALY.
  - b. This intervention offers reduced complexity that will significantly improve patient outcomes.
  - c. This intervention will reduce important health disparities across racial, ethnic, gender, socioeconomic, or regional categories.
  - d. This intervention will significantly reduce caregiver or broader family burden.
  - e. This intervention offers a novel mechanism of action or approach that will allow successful treatment of many patients who have failed other available treatments.
  - f. This intervention will have a significant impact on improving patients’ ability to return to work and/or their overall productivity.
  - g. There are other important benefits or disadvantages that should have an important role in judgments of the value of this intervention: \_\_\_\_\_
9. Are any of the following contextual considerations important in assessing axicabtagene ciloleucel’s long-term value for money? (select all that apply)
- a. 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.
  - b. This intervention is intended for the care of individuals with a condition that represents a particularly high lifetime burden of illness.
  - c. This intervention is the first to offer any improvement for patients with this condition.
  - d. Compared to standard therapy, there is significant uncertainty about the long-term risk of serious side effects of this intervention.
  - e. Compared to standard therapy, there is significant uncertainty about the magnitude or durability of the long-term benefits of this intervention.
  - f. There are additional contextual considerations that should have an important role in judgments of the value 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