



## Gene Therapies for Sickle Cell Disease

### Draft Questions for Deliberation and Voting: July 27<sup>th</sup>, 2023 Public Meeting

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

**Patient population for all questions:** Adolescents and adults with severe sickle cell disease (SCD) who do not have a matched sibling donor or haploidentical donor for hematopoietic stem cell transplantation (HSCT) or are too old for safe HSCT.

#### Clinical Evidence

1. Given the currently available evidence, is the evidence adequate to demonstrate that the net health benefit of lovotibeglogene autotemcel (lovo-cel) is superior to that provided by standard of care (i.e., hydroxyurea, chronic blood transfusions, pain medication, iron chelation)?

Yes                      No

2. Given the currently available evidence, is the evidence adequate to demonstrate that the net health benefit of exagamglogene autotemcel (exa-cel) is superior to that provided by standard of care (i.e., hydroxyurea, chronic blood transfusions, pain medication, iron chelation)?

Yes                      No

3. Given the currently available evidence, is the evidence adequate to distinguish the net health benefit between lovo-cel and exa-cel?

Yes                      No

***If yes, answer question 3a:***

- 3a. Given the currently available evidence, is the evidence adequate to demonstrate that the net health benefit of lovo-cel is superior to that provided by exa-cel?

Yes                      No

## Contextual Considerations and Potential Other Benefits or Disadvantages

*Please vote on the following contextual considerations:*

**When making judgments of overall long-term value for money, what is the relative priority that should be given to any effective treatment for SCD, on the basis of the following contextual considerations:**

1=Very low priority; 2=Low priority; 3=Average priority; 4=High priority; 5=Very high priority

4. Acuity of need for treatment of individual patients based on short-term risk of death or progression to permanent disability
5. Magnitude of the lifetime impact on individual patients of the condition being treated
6. Other (as relevant):

*Please vote on the following potential other benefits or disadvantages:*

**What are the relative effects of lovo-cel versus standard of care on the following outcomes that inform judgment of the overall long-term value for money of lovo-cel?**

1=Major negative effect; 2=Minor negative effect; 3=No difference; 4=Minor positive effect; 5=Major positive effect

7. Patients' ability to achieve major life goals related to education, work, or family life
8. Caregivers' quality of life and/or ability to achieve major life goals related to education, work, or family life
9. Patients' ability to manage and sustain treatment given the complexity of regimen
10. Society's goal of reducing health inequities
11. Other (as relevant)

**What are the relative effects of exa-cel versus standard of care on the following outcomes that inform judgment of the overall long-term value for money of exa-cel?**

1=Major negative effect; 2=Minor negative effect; 3=No difference; 4=Minor positive effect; 5=Major positive effect

12. Patients' ability to achieve major life goals related to education, work, or family life
13. Caregivers' quality of life and/or ability to achieve major life goals related to education, work, or family life
14. Patients' ability to manage and sustain treatment given the complexity of regimen
15. Society's goal of reducing health inequities
16. Other (as relevant)

## Long-Term Value for Money

17. 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 at current pricing with lovo-cel versus standard of care?
  - a. Low long-term value for money at assumed pricing
  - b. Intermediate long-term value for money at assumed pricing
  - c. High long-term value for money at assumed pricing
  
18. 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 at current pricing with exa-cel versus standard of care?
  - a. Low long-term value for money at assumed pricing
  - b. Intermediate long-term value for money at assumed pricing
  - c. High long-term value for money at assumed pricing