



# Gene Therapy for Hemophilia B and An Update on Gene Therapy for Hemophilia A: Effectiveness and Value

## Draft Questions for Deliberation and Voting: November 18, 2022 Public Meeting

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

*Patient Population for question 1: Adults  $\geq$  18 years of age with hemophilia B without inhibitors who would be appropriate for routine prophylaxis with factor replacement.*

### Clinical Evidence

1. Is the evidence adequate to demonstrate that the net health benefit of **etranacogene dezaparvovec** is superior to that provided by prophylaxis with Factor IX?

Yes                      No

*Patient Population for questions 2 – 3a: Adults  $\geq$  18 years of age with hemophilia A without inhibitors who would be appropriate for routine prophylaxis with factor replacement.*

2. Is the evidence adequate to demonstrate that the net health benefit of **valoctocogene roxaparvovec** is superior to that provided by prophylaxis with Factor VIII?

Yes                      No

3. Is the evidence adequate to distinguish the net health benefit between **valoctocogene roxaparvovec** and prophylaxis with emicizumab?

Yes                      No

- 3a. If the question to question 3 is yes, is the evidence adequate to demonstrate that the net health benefit of **valoctocogene roxaparvovec** is superior to that provided by **emicizumab**?

a} Yes                      b) No

3b. If the answer to 3a is no, is the evidence adequate to demonstrate that the net health benefit of **emicizumab** is superior to that provided by **valoctocogene roxaparvovec**?

a) Yes

b) 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 hemophilia A, 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

**When making judgments of overall long-term value for money, what is the relative priority that should be given to any effective treatment for hemophilia B, 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

6. Acuity of need for treatment of individual patients based on short-term risk of death or progression to permanent disability
7. Magnitude of the lifetime impact on individual patients of the condition being treated

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

**What are the relative effects of etranacogene dezaparvovec versus prophylaxis with Factor IX on the following outcomes that inform judgment of the overall long-term value for money of etranacogene dezaparvovec?**

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

8. Patients' ability to achieve major life goals related to education, work, or family life
9. Caregivers' quality of life and/or ability to achieve major life goals related to education, work, or family life
10. Patients' ability to manage and sustain treatment given the complexity of regimen

**What are the relative effects of valoctocogene roxaparvovec versus prophylaxis with emicizumab on the following outcomes that inform judgment of the overall long-term value for money of valoctocogene roxaparvovec?**

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

- 11. Patients' ability to achieve major life goals related to education, work, or family life
- 12. Caregivers' quality of life and/or ability to achieve major life goals related to education, work, or family life
- 13. Patients' ability to manage and sustain treatment given the complexity of regimen

**Long-Term Value for Money**

- 14. Given the available evidence on comparative effectiveness, incremental cost effectiveness, and potential other benefits or disadvantages, what is the long-term value for money of treatment at current pricing with **etranacogene dezaparvovec** versus prophylaxis with Factor IX?\*

  - a. Low long-term value for money at current pricing
  - b. Intermediate long-term value for money at current pricing
  - c. High long-term value for money at current pricing

- 15. Given the available evidence on comparative effectiveness, incremental cost effectiveness, and potential other benefits or disadvantages, what is the long-term value for money of treatment at current pricing with **valoctocogene roxaparvovec** versus prophylaxis with Factor VIII? <sup>+</sup>

  - a. Low long-term value for money at current pricing
  - b. Intermediate long-term value for money at current pricing
  - c. High long-term value for money at current pricing

- 16. Given the available evidence on comparative effectiveness, incremental cost effectiveness, and potential other benefits or disadvantages, what is the long-term value for money of treatment at current pricing with **valoctocogene roxaparvovec** and prophylaxis versus **emicizumab**? <sup>+</sup>

  - a. Low long-term value for money at current pricing
  - b. Intermediate long-term value for money at current pricing
  - c. High long-term value for money at current pricing

\*This vote will only be taken if a price becomes available for etranacogene dezaparvovec.

<sup>+</sup>This vote will only be taken if a price becomes available for valoctocogene roxaparvovec.