



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

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?

a) Yes b) 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?

a) Yes b) No

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

a) Yes b) 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?+

 - 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** versus prophylaxis with **emicizumab**?+

 - 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.

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