



Crizanlizumab, Voxelotor, and L-Glutamine for Sickle Cell Disease: Effectiveness and Value

Questions for Deliberation and Voting: March 26, 2020 Public Meeting

These questions are intended for the deliberation of the New England CEPAC voting body at the public meeting.

Clinical Evidence

1. For patients with SCD age 16 or older, is the evidence adequate to demonstrate that the net health benefit of **crizanlizumab** (Adakveo[®], Novartis AG) plus optimal usual care is superior to that provided by optimal usual care alone?
Yes No
2. For patients with SCD age 12 or older, is the evidence adequate to demonstrate that the net health benefit of **voxelotor** (Oxbryta[™], Global Blood Therapeutics, Inc.) plus optimal usual care is superior to that provided by optimal usual care alone?
Yes No
3. For patients with SCD age 5 or older, is the evidence adequate to demonstrate that the net health benefit of **L-glutamine** (Endari[™], Emmaus Medical, Inc.) plus optimal usual care is superior to that provided by optimal usual care alone?
Yes No

Potential Other Benefits and Contextual Considerations

Potential Other Benefits and Disadvantages

4. Does treating patients with **crizanlizumab** plus optimal usual care versus optimal usual care alone offer one or more of the following potential “other benefits?” (select all that apply)
 - a. This intervention will reduce important health disparities across racial, ethnic, gender, socioeconomic, or regional categories.
 - b. This intervention will significantly reduce caregiver or broader family burden.
 - c. This intervention offers a novel mechanism of action or approach that will allow successful treatment of many patients for whom other available treatments have failed.
 - d. This intervention will have a significant impact on improving patients’ ability to return to work and/or their overall productivity.
 - e. There are other important benefits or disadvantages that should have an important role in judgments of the value of this intervention: _____

5. Does treating patients with **voxelotor** plus optimal usual care versus optimal usual care alone offer one or more of the following potential “other benefits?” (select all that apply)
 - a. This intervention will reduce important health disparities across racial, ethnic, gender, socioeconomic, or regional categories.
 - b. This intervention will significantly reduce caregiver or broader family burden.
 - c. This intervention offers a novel mechanism of action or approach that will allow successful treatment of many patients for whom other available treatments have failed.
 - d. This intervention will have a significant impact on improving patients’ ability to return to work and/or their overall productivity.
 - e. There are other important benefits or disadvantages that should have an important role in judgments of the value of this intervention: _____

6. Does treating patients with **L-glutamine** plus optimal usual care versus optimal usual care alone offer one or more of the following potential “other benefits?” (select all that apply)
- 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 for whom other available treatments have failed.
 - This intervention will have a significant impact on improving patients’ ability to return to work and/or their overall productivity.
 - There are other important benefits or disadvantages that should have an important role in judgments of the value of this intervention: _____

Contextual Considerations

7. Are any of the following contextual considerations important in assessing **crizanlizumab**’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.
 - There is significant uncertainty about the long-term risk of serious side effects of this intervention.
 - There is significant uncertainty about the magnitude or durability of the long-term benefits of this intervention.
 - There are additional contextual considerations that should have an important role in judgments of the value of this intervention: _____.
8. Are any of the following contextual considerations important in assessing **voxelotor**’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.
 - There is significant uncertainty about the long-term risk of serious side effects of this intervention.
 - There is significant uncertainty about the magnitude or durability of the long-term benefits of this intervention.
 - There are additional contextual considerations 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 **L-glutamine'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. There is significant uncertainty about the long-term risk of serious side effects of this intervention.
 - d. There is significant uncertainty about the magnitude or durability of the long-term benefits of this intervention.
 - e. There are additional contextual considerations that should have an important role in judgments of the value of this intervention: _____.

Long-Term Value for Money

The New England CEPAC will not take value votes on any of the three treatments. Because we are conducting this meeting virtually, our ability to deliberate about fair value in public is impacted, notably because we will not be able to have as robust a conversation as we normally would about how to weigh potential other benefits, disadvantages, and contextual considerations in the overall assessment of value.