



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

Draft 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 best usual care is superior to that provided by best 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 best usual care is superior to that provided by 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 best usual care is superior to that provided by usual care alone?
Yes No

Potential Other Benefits and Contextual Considerations

Potential Other Benefits and Disadvantages

4. Does treating patients with **crizanlizumab** plus best usual care versus best 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 best usual care versus best 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 best usual care versus best 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: _____

Contextual Considerations

7. Are any of the following contextual considerations important in assessing **crizanlizumab**’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: _____.
8. Are any of the following contextual considerations important in assessing **voxelotor**’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: _____.

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

As described in ICER's value assessment framework, questions on long-term value for money are subject to a value vote when incremental cost-effectiveness ratios for the interventions of interest are between \$50,000 and \$175,000 per QALY in the primary "base case" analysis.

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 at current pricing with **crizanlizumab** plus best usual care versus best usual care alone?
- 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
11. 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 **voxelotor** plus best usual care versus best usual care alone?
- 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
12. 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 **L-glutamine** plus best usual care versus best usual care alone?
- 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