NEW ENGLAND CEPAC
AN ICER PROGRAM
COMPARATIVE EFFECTIVENESS PUBLIC ADVISORY COUNCIL

Emicizumab for Hemophilia A: Effectiveness and Value

Draft Questions for Deliberation and Voting: March 29, 2018 Public Meeting

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

Patient population for all questions: *Patients with hemophilia A with inhibitors to factor VIII who will not be treated with immune tolerance induction (ITI) or for whom ITI has been unsuccessful. When necessary, age ranges are specified in voting questions.*

Comparative Clinical Evidence

1. Is the evidence adequate to demonstrate that prophylactic **emicizumab** provides a net health benefit compared with **no prophylactic therapy**?

   For patients < 12 years of age Yes No

   For patients ≥ 12 years of age Yes No

2. Is the evidence adequate to demonstrate that prophylactic **emicizumab** provides net health benefits compared with **prophylactic therapy with bypassing agents (BPAs)**?

   For patients < 12 years of age Yes No

   For patients ≥ 12 years of age Yes No

Other Benefits

3. When compared to prophylactic therapy with BPAs, does emicizumab offer one or more of the following "other benefits"? (yes, no, uncertain)

   a. This intervention provides significant direct patient health benefits that are not adequately captured by the QALY.
   b. This intervention offers reduced complexity that will significantly improve patient outcomes.

c. This intervention will reduce important health disparities across racial, ethnic, gender, socioeconomic, or regional categories.
d. This intervention will significantly reduce caregiver or broader family burden.
e. This intervention offers a novel mechanism of action or approach that will allow successful treatment of many patients who have failed other available treatments.
f. This intervention will have a significant impact on improving patients' ability to return to work and/or their overall productivity.
g. This intervention will have a significant positive impact outside the family, including on schools and/or communities.
h. This intervention will have a significant impact on the entire "infrastructure" of care, including effects on screening for affected patients, on the sensitization of clinicians, and on the dissemination of understanding about the condition, that may revolutionize how patients are cared for in many ways that extend beyond the treatment itself.
i. There are other important benefits or disadvantages that should have an important role in judgments of the value of this intervention: _____

Contextual Considerations

4. Are any of the following contextual consideration important in assessing emicizumab's long-term value for money? (yes, no, uncertain)

    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. This intervention is the first to offer any improvement for patients with this condition.
    d. Compared to supportive care, there is significant uncertainty about the long-term risk of serious side effects of this intervention.
    e. Compared to supportive care, there is significant uncertainty about the magnitude or durability of the long-term benefits of this intervention.
    f. There are additional contextual considerations that should have an important role in judgments of the value of this intervention: _____.

Long-term Value for Money

5. Given the available evidence on comparative clinical effectiveness and incremental cost effectiveness, and considering other benefits, disadvantages, and contextual considerations, what is the long-term value for money of **emicizumab** compared with **no prophylactic therapy**? *(The panel will only vote on this question if the incremental cost-effectiveness ratio for emicizumab compared with no prophylaxis is between $50,000 per quality-adjusted life year (QALY) and $175,000 per QALY.)*

6. Given the available evidence on comparative clinical effectiveness and incremental cost effectiveness, and considering other benefits, disadvantages, and contextual considerations, what is the long-term value for money of **emicizumab** compared with **prophylactic therapy with BPAs**? *(The panel will only vote on this question if the incremental cost-effectiveness ratio for emicizumab compared with BPAs is between $50,000 per QALY and $175,000 per QALY.)*

Low Intermediate High