Betibeglogene Autotemcel for Beta Thalassemia

Draft Questions for Deliberation and Voting: June 17ᵗʰ Public Meeting

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

Patient population for all questions: *Patients living with transfusion-dependent thalassemia, typically defined as eight or more transfusions per year.*

Clinical Evidence

1. Given the currently available evidence, is the evidence adequate to demonstrate that the net health benefit of betibeglogene autotemcel is superior to that provided by standard clinical management (e.g., transfusion and chelation)?

<div align="center">

Yes   No

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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 beta thalassemia, 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

1. Acuity of need for treatment of individual patients based on short-term risk of death or progression to permanent disability
2. 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 betibeglogene autotemcel versus standard clinical management on the following outcomes that inform judgment of the overall long-term value for money of betibeglogene autotemcel?

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

1. Patients' ability to achieve major life goals related to education, work, or family life

2. Caregivers' quality of life and/or ability to achieve major life goals related to education, work, or family life
3. Patients' ability to manage and sustain treatment given the complexity of regimen
4. Society's goal of reducing health inequities
5. A potential advantage for therapies that offer a new treatment choice with a different balance or timing of risks and benefits that may be valued by patients with different risk preferences
6. A potential disadvantage for therapies that, if not successful, could reduce or even preclude the potential effectiveness of future treatments

Long-term Value for Money

1. 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 assumed pricing and payment plan with betibeglogene autotemcel versus standard clinical management?
    a. Low long-term value for money at assumed pricing and payment plan
    b. Intermediate long-term value for money at assumed pricing and payment plan
    c. High long-term value for money at assumed pricing and payment plan