Valoctocogene Roxaparvovec and Emicizumab for Hemophilia A without Inhibitors: Effectiveness and Value

Questions for Deliberation and Voting: October 30, 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 hemophilia A without inhibitors to factor VIII, is the evidence adequate to demonstrate that the net health benefit of **emicizumab** (Hemlibra, Genentech) is superior to that provided by prophylaxis with factor VIII?

   Yes No

Potential Other Benefits and Contextual Considerations

With ICER's 2020 value assessment framework update, ICER now uses a three-item Likert scale voting format.

2. Please vote 1, 2, or 3 on the following potential other benefits and contextual considerations as they relate to **emicizumab**. Refer to table below.

Likert Scale of Potential Other Benefits and Contextual Considerations		
1 (Suggests Lower Value)	**2 (Intermediate)**	**3 (Suggests Higher Value)**
Uncertainty or overly favorable model assumptions creates significant risk that base-case cost-effectiveness estimates are too optimistic.		Uncertainty or overly unfavorable model assumptions creates significant risk that base-case cost-effectiveness estimates are too pessimistic.
Very similar mechanism of action to that of other active treatments.		New mechanism of action compared to that of other active treatments.
Delivery mechanism or relative complexity of regimen likely to lead to much lower real-world adherence and worse outcomes relative to an active comparator than estimated from clinical trials.		Delivery mechanism or relative simplicity of regimen likely to result in much higher real-world adherence and better outcomes relative to an active comparator than estimated from clinical trials.
This intervention could reduce or preclude the potential effectiveness of future treatments.		This intervention offers the potential to increase access to future treatment that may be approved over the course of a patient's lifetime.
The intervention offers no special advantages to patients by virtue of presenting an option with a notably different balance or timing of risks and benefits.		The intervention offers special advantages to patients by virtue of presenting an option with a notably different balance or timing of risks and benefits.
This intervention will not differentially benefit a historically disadvantaged or underserved community.		This intervention will differentially benefit a historically disadvantaged or underserved community.
Small health loss without this treatment as measured by absolute QALY shortfall.		Substantial health loss without this treatment as measured by absolute QALY shortfall.
Small health loss without this treatment as measured by proportional QALY shortfall.		Substantial health loss without this treatment as measured by proportional QALY shortfall.
Will not significantly reduce the negative impact of the condition on family and caregivers vs. the comparator.		Will significantly reduce the negative impact of the condition on family and caregivers vs. the comparator.
Will not have a significant impact on improving return to work and/or overall productivity vs. the comparator.		Will have a significant impact on improving return to work and/or overall productivity vs. the comparator.
Other		Other

Long-Term Value for Money

3. 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 **emicizumab** 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