



Eculizumab and Efgartigimod for the Treatment of Myasthenia Gravis

Draft Questions for Deliberation and Voting: September 24th Public Meeting

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

Clinical Evidence

Patient population for questions 1-5: Adults with generalized myasthenia gravis (gMG), defined by Myasthenia Gravis Foundation of America (MGFA) clinical classes of II to IV for whom conventional immunosuppressive therapies have not been effective or have not been tolerated, and who are anti-AChR antibody positive.

1. Given the currently available evidence, is the evidence adequate to demonstrate that the net health benefit of **eculizumab added to conventional therapy** is superior to that provided by **conventional therapy alone**?

Yes No

2. Given the currently available evidence, is the evidence adequate to demonstrate that the net health benefit of **efgartigimod added to conventional therapy** is superior to that provided by **conventional therapy alone**?

Yes No

3. Given the currently available evidence, is the evidence adequate to distinguish the net health benefit of **eculizumab** from that of **efgartigimod**?

Yes No

- a. If the answer to question 3 is yes, which therapy has the greater net health benefit?

a) eculizumab b) efgartigimod

If the answer to 3 is yes, we will plan to take separate votes on eculizumab and efgartigimod on their comparative clinical effectiveness versus intravenous immunoglobulin (IVIg) and rituximab.

4. Given the currently available evidence, is the evidence adequate to distinguish the net health benefit of **IVIg** from that of **eculizumab and efgartigimod**?

Yes No

a. If the answer to 4 is yes, which therapy has the greater net health benefit?

- a) IVIG b) eculizumab and efgartigimod

5. Given the currently available evidence, is the evidence adequate to distinguish the net health benefit of **rituximab** from that of **eculizumab and efgartigimod**?

Yes No

a. If the answer to 5 is yes, which therapy has the greater net health benefit?

- a) rituximab b) eculizumab and efgartigimod

Patient population for question 6: Adults with gMG, defined by MGFA clinical classes of II to IV for whom conventional immunosuppressive therapies have not been effective or have not been tolerated, and who are anti-AChR antibody negative.

6. Given the currently available evidence, is the evidence adequate to demonstrate that the net health benefit of **efgartigimod added to conventional therapy** is superior to that provided by **conventional therapy alone**?

Yes No

Contextual Considerations and Potential Other Benefits or Disadvantages

When making judgments of overall long-term value for money, what is the relative priority that should be given to any effective treatment for gMG, 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
3. Other (as relevant):

Please vote separately for each treatment under review on the following potential other benefits or disadvantages:

What are the relative effects of eculizumab versus conventional therapy on the following outcomes that inform judgment of the overall long-term value for money of eculizumab?

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

4. Patients' ability to achieve major life goals related to education, work, or family life
5. Caregivers' quality of life and/or ability to achieve major life goals related to education, work, or family life
6. Patients' ability to manage and sustain treatment given the complexity of regimen
7. Society's goal of reducing health inequities
8. Other (as relevant):

What are the relative effects of efgartigimod versus conventional therapy on the following outcomes that inform judgment of the overall long-term value for money of efgartigimod?

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

9. Patients' ability to achieve major life goals related to education, work, or family life
10. Caregivers' quality of life and/or ability to achieve major life goals related to education, work, or family life
11. Patients' ability to manage and sustain treatment given the complexity of regimen
12. Society's goal of reducing health inequities
13. Other (as relevant):

Long-term Value for Money

Patient population for question 1: Adults with gMG, defined by MGFA clinical classes of II to IV for whom conventional immunosuppressive therapies have not been effective or have not been tolerated, and who are anti-AChR antibody positive.

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 current pricing with **eculizumab added to conventional therapy** versus **conventional therapy 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

Patient population for question 2: Adults with gMG, defined by MGFA clinical classes of II to IV for whom conventional immunosuppressive therapies have not been effective or have not been tolerated.

2. 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 with **efgartigimod added to conventional therapy** versus **conventional therapy alone**?
 - a. Low long-term value for money at assumed pricing
 - b. Intermediate long-term value for money at assumed pricing
 - c. High long-term value for money at assumed pricing