



Inclisiran and Bempedoic Acid for Patients with Heterozygous Familial Hypercholesterolemia and for Secondary Prevention of ASCVD: Effectiveness and Value

Draft Questions for Deliberation and Voting at the February 5, 2021 Public Meeting

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

Clinical Evidence

Patient population for questions 1 and 2: All adult patients with established ASCVD and/or HeFH who have elevated LDL-C levels despite treatment with maximally tolerated oral lipid-lowering therapy.

1. Given today's evidence, is the evidence adequate to demonstrate that the net health benefit of adding **bempedoic acid alone** to usual care is superior to that provided by usual care alone?

Yes No

- a. If the answer to question 1 is no, is the evidence adequate to demonstrate the net health benefit of adding **bempedoic acid alone** to usual care is superior to that provide by usual care alone in patients who have statin-associated side effects ("statin intolerant")?

Yes No

- b. If the answer to question 1 is no, is the evidence adequate to demonstrate the the net health benefit of adding **bempedoic acid alone** to usual care is superior to that provide by usual care alone in patients with HeFH?

Yes No

2. Given today's evidence, is the evidence adequate to demonstrate that the net health benefit of adding **inclisiran** to usual care is superior to that provided by usual care alone?

Yes No

Contextual Considerations and Potential Other Benefits or Disadvantages

Please vote on the following contextual considerations relevant for both of the new treatments under review:

Question: When making judgments of overall long-term value for money, what is the relative priority that should be given to any effective treatment for the SECONDARY PREVENTION OF ASCVD, 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 the severity of the condition being treated
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:

Question: What are the relative effects of BEMPEDOIC ACID when added to maximally tolerated oral lipid-lowering therapy on the following outcomes that inform judgment of the overall long-term value for money of BEMPEDOIC ACID?

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. Health inequities
5. Other (as relevant): New treatment option for patients with statin intolerance

Question: What are the relative effects of INCLISIRAN versus PCSK9 INHIBITORS on the following outcomes that inform judgment of the overall long-term value for money of INCLISIRAN?

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

6. Patients' ability to achieve major life goals related to education, work, or family life
7. Caregivers' quality of life and/or ability to achieve major life goals related to education, work, or family life
8. Patients' ability to manage and sustain treatment given the complexity of regimen
9. Health inequities
10. Other (as relevant): New treatment option for patients with statin intolerance

Long-Term Value for Money

Patient population for question 5: All adult patients with established ASCVD and/or HeFH who have elevated LDL-C levels despite treatment with maximally tolerated statin therapy.

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 at current pricing of adding **bempedoic acid with ezetimibe** to usual care versus usual care with **ezetimibe**?
 - a. Low long-term value for money
 - b. Intermediate long-term value for money
 - c. High long-term value for money

Patient population for question 6: All adult patients with established ASCVD – with or without HeFH – who have elevated LDL-C levels and have statin-associated side effects (“statin intolerant”).

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 at current pricing of adding **bempedoic acid with ezetimibe** to usual care versus usual care with **ezetimibe**.
 - a. Low long-term value for money
 - b. Intermediate long-term value for money
 - c. High long-term value for money

Patient population for question 7: All adult patients with HeFH who have elevated LDL-C levels despite treatment with maximally tolerated statin therapy.

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 at current pricing of adding **bempedoic acid with ezetimibe** to usual care versus usual care with **ezetimibe**.
 - a. Low long-term value for money
 - b. Intermediate long-term value for money
 - c. High long-term value for money

Patient population for question 8: All adult patients with established ASCVD and/or HeFH who have elevated LDL-C levels despite treatment with maximally tolerated statin therapy.

4. 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 at current pricing of adding **inclisiran** to usual care versus **usual care alone**?
 - a. Low long-term value for money
 - b. Intermediate long-term value for money
 - c. High long-term value for money

Patient population for question 9: All adult patients with established ASCVD – with or without HeFH – who have elevated LDL-C levels and have statin-associated side effects (“statin intolerant”).

5. 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 at current pricing of adding **inclisiran** to usual care versus **usual care alone**?
 - a. Low long-term value for money
 - b. Intermediate long-term value for money
 - c. High long-term value for money

Patient population for question 10: All adult patients with HeFH who have elevated LDL-C levels despite treatment with maximally tolerated statin therapy.

6. 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 at current pricing of adding **inclisiran** to usual care versus **usual care alone**?
 - a. Low long-term value for money
 - b. Intermediate long-term value for money
 - c. High long-term value for money