



**Inotersen and Patisiran for
Hereditary Transthyretin Amyloidosis: Effectiveness and Value
Draft Questions for Deliberation and Voting: September 13, 2018 Public Meeting**

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

Patient Population for all questions: Adults with hereditary transthyretin amyloidosis (hATTR)

Comparative Clinical Effectiveness

- 1) Is the evidence adequate to demonstrate that the net health benefit of inotersen plus best supportive care is superior to that provided by best supportive care alone?

Yes No

- 2) Is the evidence adequate to demonstrate that the net health benefit of patisiran plus best supportive care is superior to that provided by best supportive care alone?

Yes No

- 3) Is the evidence adequate to distinguish the net health benefit between inotersen and patisiran when added to best supportive care?

Yes No

Other Benefits – For Discussion during the Public Meeting

At the public meeting of Midwest CEPAC, panel members will be asked to comment on which of the following Other Benefits they find important to consider in thinking about the overall value of the two drugs under study.

When compared to **best supportive care alone**, does the addition of inotersen or patisiran offer one or more of the following “other benefits”? (yes, no, uncertain)

- a. This intervention offers reduced complexity that will significantly improve patient outcomes.
- b. This intervention will reduce important health disparities across racial, ethnic, gender, socioeconomic, or regional categories.
- c. This intervention will significantly reduce caregiver or broader family burden.
- d. This intervention offers a novel mechanism of action or approach that will allow successful treatment of many patients who have failed other available treatments.
- e. This intervention will have a significant impact on improving patients’ ability to return to work and/or their overall productivity.
- f. This intervention will have a significant positive impact outside the family, including on schools and/or communities.
- g. 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.
- h. There are other important benefits or disadvantages that should have an important role in judgments of the value of this intervention: _____

Contextual Considerations – For Discussion during the Public Meeting

At the public meeting of Midwest CEPAC, panel members will be asked to comment on which of the following Contextual Considerations they find important to consider in thinking about the overall value of the two drugs under study.

Are any of the following contextual considerations important in assessing inotersen’s or patisiran’s long-term value for money in patients? (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 best supportive care, there is significant uncertainty about the long-term risk of serious side effects of this intervention.
- e. Compared to best 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

1. For adults with hereditary transthyretin amyloidosis, given the available evidence on comparative clinical effectiveness and incremental cost effectiveness, and considering other benefits and contextual considerations, what is the long-term value for money of inotersen plus best supportive care compared with best supportive care alone?

High

Intermediate

Low

2. For adults with hereditary transthyretin amyloidosis, given the available evidence on comparative clinical effectiveness and incremental cost effectiveness, and considering other benefits and contextual considerations, what is the long-term value for money of patisiran plus best supportive care compared with best supportive care alone?

High

Intermediate

Low