



Prophylaxis for Hereditary Angioedema with Lanadelumab and C1 Inhibitors: Effectiveness and Value

Draft Questions for Deliberation and Voting: October 25, 2018 Public Meeting

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

Patient Population for all questions: Patients with Type 1 and Type 2 Hereditary Angioedema (HAE 1/2) who are eligible for long-term prophylactic therapy.

Clinical Evidence

1. Is the evidence adequate to demonstrate that the net health benefits of long-term prophylaxis with C1 inhibitors for HAE 1/2 are superior to on-demand therapy only?
Yes No
2. Is the evidence adequate to distinguish the net health benefits among the C1 inhibitors (Cinryze, Ruconest, Haegarda) for long-term prophylactic therapy for HAE 1/2?
Yes No
3. Is the evidence adequate to demonstrate that the net health benefits of long-term prophylaxis with lanadelumab for HAE 1/2 are superior to on-demand therapy only?
Yes No

Potential Other Benefits and Contextual Considerations

4. Does treating HAE 1/2 patients with long-term prophylactic therapy offer one or more of the following potential “other benefits?” (select all that apply)
 - a. **Haegarda** offers reduced complexity that will significantly improve patient outcomes.
 - b. **Lanadelumab** offers reduced complexity that will significantly improve patient outcomes.
 - c. This intervention will reduce important health disparities across racial, ethnic, gender, socioeconomic, or regional categories.
 - d. This intervention will significantly reduce caregiver or broader family burden.
 - e. **Lanadelumab** offers a novel mechanism of action or approach that will allow successful treatment of many patients for whom other available treatments have failed.
 - f. This intervention will have a significant impact on improving patients’/caregivers’ ability to return to work or school and/or their overall productivity.
 - g. This intervention will have a significant positive impact outside the family, including on schools and/or communities.
 - h. 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.
 - i. There are other important benefits or disadvantages that should have an important role in judgments of the value of this intervention: _____

5. Are any of the following contextual considerations important in assessing the long-term value for money of long-term prophylactic therapy for HAE 1/2? (select all that apply)
 - 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 on-demand treatment only, there is significant uncertainty about the long-term risk of serious side effects of using **C1 inhibitors**.
 - e. Compared to on-demand treatment only, there is significant uncertainty about the long-term risk of serious side effects of using **lanadelumab**.
 - f. Compared to on-demand treatment only, there is significant uncertainty about the magnitude or durability of the long-term benefits of using **C1 inhibitors**.
 - g. Compared to on-demand treatment only, there is significant uncertainty about the magnitude or durability of the long-term benefits of using **lanadelumab**.
 - h. There are additional contextual considerations that should have an important role in judgments of the value of this intervention: _____.

Long-term Value for Money

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 of long-term prophylaxis of HAE with **Cinryze** versus on-demand therapy?

Low

Intermediate

High

7. 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 long-term prophylaxis of HAE with **Ruconest** versus on-demand therapy?

Low

Intermediate

High

8. 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 long-term prophylaxis of HAE with **Haegarda** versus on-demand therapy?

Low

Intermediate

High

9. 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 long-term prophylaxis of HAE with **lanadelumab**¹ versus on-demand therapy?

Low

Intermediate

High

¹ Note: This vote will be taken only if lanadelumab has a publicly-announced price at the time of the October 25 public meeting.