



AMX0035 and Oral Edaravone for ALS

Draft Questions for Deliberation and Voting: August 19th Public Meeting

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

Patient population for all questions (unless otherwise specified): Adult persons with ALS.

Clinical Evidence

1. Given the currently available evidence, is the evidence adequate to demonstrate that the net health benefit of AMX0035 plus standard of care is superior to that provided by standard of care alone (i.e., multidisciplinary care that may involve treatment with riluzole and IV edaravone)?

Yes

No

Patient population for question 2: Adults with ALS who meet the narrow Study 19 criteria

2. Given the currently available evidence, is the evidence adequate to demonstrate that the net health benefit of oral edaravone plus standard of care is superior to that provided by standard of care alone (i.e., multidisciplinary care that may involve treatment with riluzole)?

Yes

No

Patient population for question 3: Adults with ALS who do not meet Study 19 criteria

3. Given the currently available evidence, is the evidence adequate to demonstrate that the net health benefit of oral edaravone plus standard of care is superior to that provided by standard of care alone (i.e., multidisciplinary care that may involve treatment with riluzole)?

Yes

No

Contextual Considerations and Potential Other Benefits or Disadvantages

Please vote on the following contextual considerations:

When making judgments of overall long-term value for money, what is the relative priority that should be given to any effective treatment for ALS, 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

Please vote on the following potential other benefits or disadvantages:

What are the relative effects of AMX0035 on the following outcomes that inform judgment of the overall long-term value for money of AMX0035?

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

3. Patients' ability to achieve major life goals related to education, work, or family life
4. Caregivers' quality of life and/or ability to achieve major life goals related to education, work, or family life

What are the relative effects of oral edaravone on the following outcomes that inform judgment of the overall long-term value for money of oral edaravone?

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

5. Patients' ability to achieve major life goals related to education, work, or family life
6. Caregivers' quality of life and/or ability to achieve major life goals related to education, work, or family life
7. Patients' ability to manage and sustain treatment given the complexity of regimen compared to IV edaravone

Long-term Value for Money

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 of AMX0035, at assumed pricing, compared to usual care?
 - 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

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 of oral edaravone, at assumed pricing, compared to usual care?
 - 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