



Atidarsagene Autotemcel for Metachromatic Leukodystrophy

Questions for Deliberation and Voting: September 29, 2023 Public Meeting

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

Clinical Evidence

Patient Population: *Children with presymptomatic late infantile MLD.*

1. Is the currently available evidence adequate to demonstrate that the net health benefit of atidarsagene autotemcel (arsa-cel) is superior to that provided by usual care?

Yes No

Patient Population: *Children with presymptomatic early juvenile MLD.*

2. Is the currently available evidence adequate to demonstrate that the net health benefit of arsa-cel is superior to that provided by usual care?

Yes No

Patient Population: *Children with early symptomatic early juvenile MLD.*

3. Is the currently available evidence adequate to demonstrate that the net health benefit of arsa-cel is superior to that provided by usual care?

Yes No

Contextual Considerations and Potential Other Benefits or Disadvantages

Please vote on the following contextual considerations:

Patient Population: *Children with presymptomatic late infantile MLD or presymptomatic early juvenile MLD*

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

4. Acuity of need for treatment of individual patients based on short-term risk of death or progression to permanent disability
5. Magnitude of the lifetime impact on individual patients of the condition being treated

Please vote on the following potential other benefits or disadvantages:

Patient Population: *Children with presymptomatic late infantile MLD or presymptomatic early juvenile MLD*

What are the relative effects of arsa-cel versus usual care on the following outcomes that inform judgment of the overall long-term value for money of arsa-cel?

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. Society's goal of reducing health inequities
9. Other: The entire "infrastructure" of care, including effects on screening for affected patients, on the awareness 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.

Long-Term Value for Money

10. 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 arsa-cel versus usual care?*
- 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

**This vote will only be taken if a price becomes available for arsa-cel.*