



# Therapies for Spinal Muscular Atrophy: Effectiveness and Value

## Draft Questions for Deliberation and Voting: August 1, 2025 Public Meeting

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

### Clinical Evidence

1. For children aged 2-12 years with SMA Type 2 or 3, is the currently available evidence adequate to demonstrate that the net health benefit of apitegromab in addition to standard of care (risdiplam or nusinersen) is greater than that of standard of care alone?

Yes                      No

2. For people with SMA previously treated with onasemnogene abeparvovec, is the currently available evidence adequate to demonstrate that the net health benefit of risdiplam is greater than that of no additional treatment?

Yes                      No

3. For people with SMA previously treated with onasemnogene abeparvovec, is the currently available evidence adequate to demonstrate that the net health benefit of nusinersen is greater than that of no additional treatment?

Yes                      No

4. For clinically presymptomatic infants with SMA, is the currently available evidence adequate to distinguish the net health benefit among nusinersen, onasemnogene abeparvovec and risdiplam as first line therapy?

Yes                      No

a) If yes, which is best?

A. Nusinersen              B. Onasemnogene abeparvovec              C. Risdiplam

## Benefits Beyond Health and Special Ethical Priorities

**To help inform judgments of overall long-term value for money, please indicate your level of agreement with the following statements:**

1=Strongly Disagree; 2=Disagree; 3=Neutral; 4=Agree; 5=Strongly Agree

5. There is substantial unmet need despite currently available treatments.

6. This condition is of substantial relevance for people from a racial/ethnic group that has not been equitably served by the healthcare system.

**To help inform judgments of overall long-term value for money, please indicate your level of agreement with the following statements based on the relative effects of apitegromab in addition to standard of care (risdiplam or nusinersen) versus standard of care alone:**

1=Strongly Disagree; 2=Disagree; 3=Neutral; 4=Agree; 5=Strongly Agree

7. The treatment is likely to produce substantial improvement in caregivers' quality of life and/or ability to pursue their own education, work, and family life.

8. The treatment offers a substantial opportunity to improve access to effective treatment by means of its mechanism of action or method of delivery.

9. Other: as determined pre-meeting by ICER team based on input from patients, clinical experts, and appraisal committee members.

## Long-Term Value for Money

10. Given the available evidence on comparative clinical effectiveness and incremental cost effectiveness, and considering benefits beyond health and special ethical priorities, what is the long-term value for money of apitegromab in addition to standard of care (risdiplam or nusinersen) compared to standard of care alone at [current/assumed] pricing?\*

- a. High long-term value for money at [current/assumed] pricing
- b. Intermediate long-term value for money at [current/assumed] pricing
- c. Low long-term value for money at [current/assumed] pricing

*\*This vote will only be taken if a price becomes available for apitegromab.*