



# Sonpiretigene Isteparvovec for Advanced Retinitis Pigmentosa

## Draft Questions for Deliberation and Voting: April 11<sup>th</sup>, 2025 Public Meeting

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

**Patient Population for all questions:** *People with advanced retinitis pigmentosa (RP) with severe vision loss.*

**Note for all questions:** *Usual care may include low vision aids, mobility training and support, and vision related rehabilitation.*

### Clinical Evidence

1. For patients with advanced RP, is the current evidence adequate to demonstrate that the net health benefit of sonpiretigene is greater than that of usual care?

Yes                  No

### 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 about advanced RP:**

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

2. There is substantial unmet need despite currently available treatments.
3. This condition is of substantial relevance for people from a racial/ethnic group that have 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 sonporetigene versus usual care:**

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

4. 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.
5. The treatment offers a substantial opportunity to improve access to effective treatment by means of its mechanism of action or method of delivery.
6. Other: as determined pre-meeting by ICER team based on input from patients, clinical experts, and appraisal committee members

### **Long-Term Value for Money**

7. 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 sonporetigene compared to usual care at assumed pricing?\*

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

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