



Imetelstat for Anemia in Myelodysplastic Syndrome

Questions for Deliberation and Voting: July 19th Public Meeting

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

Clinical Evidence

Patient Population: *Patients with lower risk myelodysplastic syndrome without the del(5q) mutation who are transfusion dependent* and ineligible for, or refractory to ESAs.*

1. Is the current evidence adequate to demonstrate that the net health benefit of imetelstat plus best supportive care is superior to that provided by best supportive care alone?

Yes No

Patient Population: *Patients with lower risk myelodysplastic syndrome without the del(5q) mutation who are transfusion dependent* and ineligible for, or refractory to ESAs, and are ring sideroblast positive.*

2. Is the current evidence adequate to demonstrate that the net health benefit of imetelstat plus best supportive care is superior to that provided by luspatercept plus best supportive care?

Yes No

Benefits Beyond Health and Special Ethical Priorities

Patient Population: *Patients with lower risk myelodysplastic syndrome without the del(5q) mutation who are transfusion dependent* and ineligible for, or refractory to ESAs.*

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

3. There is substantial unmet need despite currently available treatments.
4. 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 imetelstat plus best supportive care versus best supportive care alone:

Patient Population: *Patients with lower risk myelodysplastic syndrome without the del(5q) mutation who are transfusion dependent* and ineligible for, or refractory to ESAs.*

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

5. 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.
6. The treatment offers a substantial opportunity to improve access to effective treatment by means of its mechanism of action or method of delivery.

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 imetelstat plus best supportive care versus luspatercept plus best supportive care:

Patient Population: *Patients with lower risk myelodysplastic syndrome without the del(5q) mutation who are transfusion dependent* and ineligible for, or refractory to ESAs, and are ring sideroblast positive.*

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.

Long-Term Value for Money

Patient Population: *Patients with lower risk myelodysplastic syndrome without the del(5q) mutation who are transfusion dependent* and ineligible for, or refractory to ESAs.*

9. 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 imetelstat plus best supportive care compared to best supportive care alone at current pricing?
 - a. High long-term value for money at current pricing
 - b. Intermediate long-term value for money at current pricing
 - c. Low long-term value for money at current pricing
 - d. Evidence is insufficient to make a value determination

Patient Population: *Patients with lower risk myelodysplastic syndrome without the del(5q) mutation who are transfusion dependent* and ineligible for, or refractory to ESAs, and are ring sideroblast positive.*

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 imetelstat plus best supportive care compared to luspatercept plus best supportive care at current pricing?
 - a. High long-term value for money at current pricing
 - b. Intermediate long-term value for money at current pricing
 - c. Low long-term value for money at current pricing
 - d. Evidence is insufficient to make a value determination

** Transfusion-dependence defined as 2-4 red blood cell units transfused over 8 weeks*