Treatments for Anemia in Chronic Kidney Disease: Effectiveness and Value
Revised Questions for Deliberation and Voting
February 11, 2021 Virtual Public Meeting

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

Clinical Evidence

1. Given currently available evidence, in patients who have DI-CKD, is the evidence adequate to distinguish the net health benefit between roxadustat and ESAs?

   Yes  No

   1a. If the answer to Q1 is Yes: Based on the available evidence in patients who have DI-CKD, which therapy has a greater net health benefit: a) roxadustat, or b) ESAs?

      a) Roxadustat  b) ESAs

2. Given currently available evidence, in patients who have DI-CKD, is the evidence adequate to demonstrate that the net health benefit of roxadustat is superior to that provided by usual care (estimated by placebo arms)?

   Yes  No

3. Given currently available evidence, in patients who have DD-CKD, is the evidence adequate to distinguish the net health benefit between roxadustat and ESAs?

   Yes  No

   3a. If the answer to Q3 is Yes: Based on the available evidence in patients who have DD-CKD, which therapy has a greater net health benefit: a) roxadustat, or b) ESAs?

      a) Roxadustat  b) ESAs
Potential Other Benefits and Contextual Considerations

With ICER’s 2020 Value Assessment Framework update, we now use a three-item Likert scale voting format for potential other benefits and contextual considerations.

4. Please vote 1, 2, or 3 on the following potential other benefits and contextual considerations; for questions where a comparator or existing therapy is implied, please answer for roxadustat compared to ESAs.

<table>
<thead>
<tr>
<th>1 (Suggests Lower Value)</th>
<th>2 (Neutral)</th>
<th>3 (Suggests Higher Value)</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>DI-CKD:</strong> Uncertainty or overly favorable model assumptions creates significant risk that base-case cost-effectiveness estimates are too optimistic.</td>
<td></td>
<td><strong>DI-CKD:</strong> Uncertainty or overly unfavorable model assumptions creates significant risk that base-case cost-effectiveness estimates are too pessimistic.</td>
</tr>
<tr>
<td><strong>DD-CKD:</strong> Uncertainty or overly favorable model assumptions creates significant risk that base-case cost-effectiveness estimates are too optimistic.</td>
<td></td>
<td><strong>DD-CKD:</strong> Uncertainty or overly unfavorable model assumptions creates significant risk that base-case cost-effectiveness estimates are too pessimistic.</td>
</tr>
<tr>
<td>Very similar mechanism of action to that of other active treatments.</td>
<td></td>
<td>New mechanism of action compared to that of other active treatments.</td>
</tr>
<tr>
<td>Delivery mechanism or relative complexity of regimen likely to lead to much lower real-world adherence and worse outcomes relative to an active comparator than estimated from clinical trials.</td>
<td></td>
<td>Delivery mechanism or relative simplicity of regimen likely to result in much higher real-world adherence and better outcomes relative to an active comparator than estimated from clinical trials.</td>
</tr>
<tr>
<td>This intervention will not differentially benefit a historically disadvantaged or underserved community.</td>
<td></td>
<td>This intervention will differentially benefit a historically disadvantaged or underserved community.</td>
</tr>
<tr>
<td>Small health loss without this treatment as measured by absolute quality-adjusted life year (QALY) shortfall.</td>
<td></td>
<td>Substantial health loss without this treatment as measured by absolute QALY shortfall.</td>
</tr>
<tr>
<td>Small health loss without this treatment as measured by proportional QALY shortfall.</td>
<td></td>
<td>Substantial health loss without this treatment as measured by proportional QALY shortfall.</td>
</tr>
<tr>
<td>Will not significantly reduce the negative impact of the condition on family and caregivers vs. the comparator.</td>
<td></td>
<td>Will significantly reduce the negative impact of the condition on family and caregivers vs. the comparator.</td>
</tr>
<tr>
<td>Will not have a significant impact on improving return to work and/or overall productivity vs. the comparator.</td>
<td></td>
<td>Will have a significant impact on improving return to work and/or overall productivity vs. the comparator.</td>
</tr>
<tr>
<td>Other</td>
<td></td>
<td>Other</td>
</tr>
</tbody>
</table>
Long-Term Value for Money

5. 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 roxadustat versus ESAs in patients who have DI-CKD?

   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

6. 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 roxadustat versus ESAs in patients who have DD-CKD?

   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

---

1 As described in ICER’s Value Assessment Framework, questions on long-term value for money are subject to a value vote when an established net price is available or if net price estimates are felt to be extremely reliable.