Valuing a Cure: Are New Approaches Needed?

Goal: Sustainable Access to High-Value Care for All Patients

ICER value-based price benchmark

- Long-Term Value for Money
- Short-Term Affordability
- ICER Value-Based Price Benchmark
Challenges in Valuing Potential Cures

- Uncertainty regarding clinical effectiveness due to limitations in study design, outcome measures, and the size and duration of clinical trials
- Uncertainty regarding inclusion of additional elements of value that may be important for potential cures, but which are not part of standard cost-effectiveness methods

Potential Other Benefits particularly relevant for potential cures

- This intervention offers a novel mechanism of action that will allow successful treatment of patients for whom other treatments have failed.
- This intervention will have a significant positive impact outside the family, including on schools and/or communities.
- This intervention will have a significant impact on the entire “infrastructure” of care.
Challenges in Valuing Potential Cures

• Magnitudes of lifetime health gains and cost offsets that are far beyond those generated by nearly all traditional therapies
Options for value-based pricing of a cure

• New cure of a fatal disease of a 5 year-old child who would die in 10 years with standard Rx
• Assumed WTP threshold of $100,000/QALY

<table>
<thead>
<tr>
<th></th>
<th>Cost per year of current Rx</th>
<th>QALY gained</th>
<th>QALY gain price</th>
<th>Cost offset price component</th>
<th>“Value-based” price</th>
</tr>
</thead>
<tbody>
<tr>
<td>Standard CEA</td>
<td>$400,000</td>
<td>50</td>
<td>$5 million</td>
<td>$4 million</td>
<td>$9 million</td>
</tr>
<tr>
<td>QALY Price cap</td>
<td>$400,000</td>
<td>50</td>
<td>$5 million</td>
<td>$0</td>
<td>$5 million</td>
</tr>
<tr>
<td>Re-priced cost offset at $100,000 per QALY</td>
<td>$400,000</td>
<td>50</td>
<td>$5 million</td>
<td>$1 million</td>
<td>$6 million</td>
</tr>
<tr>
<td>Shared savings 50%</td>
<td>$400,000</td>
<td>50</td>
<td>$5 million</td>
<td>$2 million</td>
<td>$7 million</td>
</tr>
</tbody>
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Options for value-based pricing of a cure

• What are the criteria or principles by which we would choose to move to a modified approach?
  • Relative uncertainty in the durability of benefit
  • Magnitude of the health gain and/or cost offset
  • Federal investment in the basic science
  • Size of the intended population
  • Ability to pay through installments

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<th>QALY gain price</th>
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<th>Annual payment over 10 years</th>
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