ANALYSIS OF UTILITY VALUES USED IN NICE APPRAISALS OF HIGHLY SPECIALISED TECHNOLOGIES

PRM184

BACKGROUND

- The National Institute for Health and Care Excellence (NICE) develops technology appraisals (TA) to assess the clinical and cost-effectiveness of new and existing technologies within the NHS in England.
- Highly Specialised Technologies (HST) appraisals are recommendations on the use of highly specialised medicines and treatments for very rare conditions.
- NICE conducts TAs, including HST appraisals, using a ‘reference case’ to achieve a consistent approach to decision making across different technologies and disease areas. Although deviation from the reference case is permitted, such deviations must be clearly justified.
- The reference case stipulates that health effects should be expressed in terms of quality adjusted life years (QALYs). A QALY is calculated by weighting the time spent in a health state by a value (i.e. utility value) placed on the health-related quality of life (HRQoL) associated with the state.
- For the reference case, HRQoL or changes in HRQoL should be measured directly by patients and the utility of these changes should be based on public preferences using a choice-based method. Specifically, EuroQol EQ-5D is the preferred tool for the measurement of HRQoL, and thus calculation of utility values, in adults.
- The applicability of using the reference case in HST appraisals has been challenged, with stakeholders questioning the ability of the QAQL to accurately reflect health effects experienced by patients of very rare conditions.
- For example, there are concerns that EQ-5D does not fully capture all the benefits of highly specialised medicines, such as the impact on patients’ or their carers’ ability to go to school and/or work and the wider impact to patients’ families.
- Furthermore, very rare diseases often include genetically acquired disorders affecting children, which makes measuring utility values particularly challenging.
- Therefore, HST appraisals often include utility values from sources other than the EQ-5D.
- The incremental QALY gains associated with a new technology are critical to HST appraisal decision-making.
- From April 2017, the NICE HST interim process guide states that if there is compelling evidence that the treatment offers more than 10 incremental QALYs, an additional ‘QALY weighting’ can be applied. This results in increasing the incremental cost-effectiveness ratio (ICER) threshold used for decision making.

OBJECTIVES

NICE HST appraisals were evaluated to identify the included patient utility values and utility modifiers (e.g. carer disability, on-treatment utility increment and/or complications/disability) and:
- their type (e.g. EQ-5D)
- their source (e.g. clinical trial, patient/carer surveys, literature, expert opinion etc.)
- the associated commentary of the evidence review group (ERG) and appraisal committee
- their alignment to the NICE reference case
- the total incremental QALYs gained and their alignment to the ‘QALY weighting’ threshold

METHODS

Documents associated with NICE HST appraisals (Table 1) with published final guidance between January 2015 and May 2018 were reviewed.

Details of the technology, indication, source of utility values, comments relating to the utility values from the ERG and appraisal committee, incremental QALYs and final recommendation were systematically extracted and checked by a second author.

Utility value source publications were reviewed for clarification.

RESULTS

- In total, seven HST appraisals were reviewed (Table 2).

TABLE 2 – KEY UTILITY DATA FOR NICE HST APPRAISALS

<table>
<thead>
<tr>
<th>TECHNOLOGY</th>
<th>INDICATION</th>
<th>RECOMMENDATION</th>
<th>PUBLICATION DATE</th>
<th>PATIENT UTILITY VALUES</th>
<th>UTILITY MODIFIERS</th>
<th>COMPARISON</th>
<th>CONCLUSION</th>
</tr>
</thead>
<tbody>
<tr>
<td>HST1</td>
<td>Ocrelizumab (intraocular)</td>
<td>Recommended</td>
<td>28 Jun 15</td>
<td>Clinical trial and literature</td>
<td>On-treatment utility increment</td>
<td>Comparator (reduction)</td>
<td>Indefinite</td>
</tr>
<tr>
<td>HST2</td>
<td>Vennco (oral)</td>
<td>NPI-501</td>
<td>Optimised</td>
<td>16 Dec 15</td>
<td>HST interim process guide and literature</td>
<td>Comparator</td>
<td>Indefinite</td>
</tr>
<tr>
<td>HST3</td>
<td>Translumina (oral)</td>
<td>Oral</td>
<td>Optimised</td>
<td>30 Jul 16</td>
<td>HST interim process guide and literature</td>
<td>Comparator</td>
<td>Indefinite</td>
</tr>
<tr>
<td>HST4</td>
<td>Osmoticon (oral)</td>
<td>Oral</td>
<td>Optimised</td>
<td>16 Dec 15</td>
<td>HST interim process guide and literature</td>
<td>Comparator</td>
<td>Indefinite</td>
</tr>
<tr>
<td>HST5</td>
<td>Oculoglycan (oral)</td>
<td>Oral</td>
<td>Optimised</td>
<td>30 Jul 16</td>
<td>HST interim process guide and literature</td>
<td>Comparator</td>
<td>Indefinite</td>
</tr>
<tr>
<td>HST6</td>
<td>Orifcal (oral)</td>
<td>Oral</td>
<td>Optimised</td>
<td>16 Dec 15</td>
<td>HST interim process guide and literature</td>
<td>Comparator</td>
<td>Indefinite</td>
</tr>
<tr>
<td>HST7</td>
<td>Santen (oral)</td>
<td>NPI-501</td>
<td>Optimised</td>
<td>30 Jul 16</td>
<td>HST interim process guide and literature</td>
<td>Comparator</td>
<td>Indefinite</td>
</tr>
</tbody>
</table>

PATTER UTILITIES

Four HST appraisals collected patient utility values using EQ-5D, the preferred HST tool (Figure 1). Of these:
- One collected data directly from patients with the disease using clinical trial data, the preferred reference case approach
- Two collected data directly from patients with the disease using a patient survey
- One collected data used clinical experts’ ratings of patient health state vignettes

Three HST appraisals collected patient utility values from non-EQ-5D approaches (Figure 1). Of these:
- One used 36-item Short Form Survey (SF-36) data mapped to EQ-5D collected directly from patients with the disease using disease registry data
- One used health utilities index (HUI) data collected directly from patients with the disease using a patient survey
- One used multiple types and sources to derive utility values, including the use of utilities derived from the general population and a proxy disease

CONCLUSIONS AND IMPACT ON DECISION-MAKING

- On-treatment utility increments were included in three HST appraisals. Of these:
  - Two used EQ-5D either derived from clinical trial data (n=1) or clinical trial correlated with patient survey data (n=1)
  - One was a time trade-off in the UK general population (n=1)
- All seven HST appraisals also included various other utility modifiers: complication/disability (n=1), adverse event disability (n=1) and comparator disability (n=1) derived from various sources (e.g. published estimates from the literature, expert opinion and/or assumptions).

INTEGRAL QALYs

- Using the base case assumptions of the company submissions, when comparing the new technology with standard of care, the incremental QALYs ranged from 0.98 to 25.22.
- On the preferred assumptions of the ERG, when comparing the new technology with standard of care, the incremental QALYs’ ranged from 34 to 79.6.
- When based on the lower estimates of the ranges provided, only two HST appraisals were associated with >10 incremental QALYs to qualify for the additional ‘QALY weighting’

REFERENCES

4.www.decisionresourcesgroup.com/abacus

J. Macey,1 S. Knight,1 J. Tosh,1 J. Lee,2
1DRG Abacus, Bicester, UK
2DRG Abacus, Manchester, UK
International Society for Pharmacoeconomics and Outcomes Research (ISPOR) 10 – 14 November 2018, Barcelona, Spain