DO NOVEL VALUE MEASURES HAVE A PLACE IN EUROPEAN HTA?

ISPOR-EU panel, Nov 12, 2018, 3.45 - 4.45pm [Breakout Session #2 (IP6)]

Participants

Moderator
Ross Maclean, MD, SVP, Head of Medical Affairs, Precision Value & Health, USA

Panelists
Louis Garrison, PhD, Professor Emeritus, Dept. of Pharmacy, University of Washington, USA
Mark Sculpher, PhD, Professor, Centre for Health Economics, University of York, UK
Jens Grueger, PhD, SVP and Head, Global Access, Hoffman La Roche, Switzerland
The Panel: Diverse perspectives

- Recap on the role of QALY in HTA and place the QALY in the wider context of other “Elements of Value”;
- Comment on how novel value measures address static vs. dynamic opportunity costs; and
- Introduce issue of future generation willingness-to-pay for today’s innovation.

Lou Garrison

The Panel: Diverse perspectives

- Explore if/how the benefits from innovation equate to the value delivered and that “value” requires one to identify and compare benefits that are socially and fiscally meaningful to the opportunity costs;
- Affirm that healthcare systems can only afford to pay for specific, measurable benefits; and
- Challenge whether the US perspective adequately addresses opportunity costs?

Mark Sculpher
The Panel: Diverse perspectives

- Describe how novel treatments may confer benefits not captured in existing approaches and that an HTA “average” benefit does not capture the unique, patient-centric benefits of some therapies;
- Highlight that healthcare systems not equipped to consider some benefits e.g., improved QoL; and
- Challenge different market perspectives on value.

Jens Grueger

Poll: Q1. In HTA, to what extent should the QALY be supplemented by other measures of value?
Poll: Q2. Should patient's perspectives include only those patients who stand to benefit from a new product (0) or also include those who will forgo benefit as a result of its funding (1)?
Poll: Q3. Do you think health status utility measures (and thus QALY) adequately include the patient's perspective?
Scientific innovation is forcing a broader view on value
The public’s view on valuing life

40 million people from 233 countries/territories completed an online survey.

A variation of the “trolley problem” in philosophy, applied to self-driving cars … think of the center lane vs. the bicycle lane.

Strong preferences for saving:
- Humans > animals
- Many people > Few people
- Children > Elderly

Geo-cultural differences


The impact of innovation

MELANOMA - Initial increase in lung nodules (at 6 weeks and 3 months) followed by complete regression (at 10 months).

NSCLC – Partial response (at 15 months) in liver and lungs.

The impact of innovation

The value of a violin virtuoso?

Link here.
A few provocative thoughts to get us started …
Elements of value

The value of simply knowing …

The medical diagnostic process informs patient and provider knowledge. Novel technologies such as Next Generation Sequencing (NGS) have the potential to transform the depth, breadth and consequences of such knowledge (aka “personal utility”), in turn delivering value. For example:

- 45% of adult respondents were willing to pay for information on a variant for which there was no effective treatment available\(^1\).
- 27% of the general population would want “secondary findings” information for disorders with severe quality-of-life consequences, irrespective of whether effective medical treatment was available\(^2\).
- Parents were interested in the return of highly penetrant non-medically actionable conditions in children, particularly if manifestations were more severe (e.g., earlier age of onset and greater level of disability)\(^3\).

References:
Is society’s view of “value” every changing?

Do you view the 12 elements of value as static or dynamic, and if dynamic, how do innovators and payers keep up?

Accounting for the patient perspective

- **Payers**: How does the patient perspective translate into economic value to justify treatment costs?
- **Patients**: How will this treatment impact the patient and their day-to-day life?
- **Physicians**: How can patient quality of care, satisfaction and outcomes be improved in a meaningful, measurable way?
- **Policymakers**: How can value and quality assessments better account for and incorporate the patient perspective?
Complementary methods to understanding the patient perspective

### Revealed preference studies
- Understand how patients make decisions based upon observed behavior
- Can use existing retrospective data

### Discrete choice experiments
- Quantify value of treatment attributes
- Better understand patient decision-making regarding treatment choice

### Heterogeneity assessments
- Quantify variation in patient behavior and outcomes to support personalized treatment
- Can use causal inference methods

Obvious yet not easily measurable

Regarding “specific, measurable benefits”, how should aspects of life that are perhaps more “intangible” be accommodated?
How high is the “Fourth Hurdle”?

- What matters most to the patient about a particular treatment?
  - Survival?
  - Treatment toxicity or side effects?
  - Insurance coverage?
- What prevents a patient from adhering to the medication?
  - What factors facilitate adherence?
- What elements of symptom improvement are most valuable to patients?
  - Functional ability?
  - Quality of life?
- What are the impacts of treatment-related costs on the patient?
  - Out-of-pocket costs
  - Ancillary costs

Innovation driving Rx options, better outcomes and need for data

Example: Drugs approved to treat NSCLC

1998 (n=6)

- Cisplatin
- Docetaxel
- Gemcitabine Hydrochloride
- Irinotecan
- Paclitaxel
- Vinorelbine Tartrate

2018 (n=26)

- Afatinib Dimaleate
- Alectinib
- Atezolizumab
- Bevacizumab
- Brigatinib
- Carboplatin
- Ceritinib
- Crizotinib
- Dabrafenib
- Docetaxel
- Erlotinib Hydrochloride
- Everolimus
- Gefitinib

- Gemcitabine Hydrochloride
- Mechloretamine Hydrochloride
- Methotrexate
- Nectitumumab
- Nivolumab
- Osimertinib
- Paclitaxel
- Paclitaxel Albumin-stabilized Nanoparticle Formulation
- Pembrolizumab
- Pemetrexed Disodium
- Ramucirumab
- Trametinib
- Vinorelbine Tartrate

"Uneasy lies the head that wears a crown."

- Convenience
- Mode of administration
- Uncertainty
- Fear
- Hope
- Insurance value
- Option value
- Financial burden
- Side effect tolerance
- Family / caregiver considerations
- Treatment goals

Does the burden-of-proof for quantifying all these elements of value rest with the innovator, or it is shared more broadly?

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Thank you for your attention!

Questions?
Patient perspective is increasingly considered in value assessment

- Frameworks for evaluating cost-effectiveness are increasingly considering a broader perspective and more patient-centered impacts.
- Advocacy organizations appear keen to defend patient-centered endpoints.

References: