DO NOVEL VALUE MEASURES HAVE A PLACE IN EUROPEAN HTA?

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Why do we need to discuss novel value measures?

- Significant advances in understanding the science and biology of complex diseases with high unmet need, eg. cancer, neurodegenerative disorders, monogenic rare diseases
- Novel approaches incl cancer immuno-therapies, gene therapies and cell therapies
  - While response rates are high, treatment outcomes may vary, potential for durable benefit and even cures for an increasing percentage of patients
- Current HTA procedures were developed during a time when the focus was on blockbuster drugs in chronic diseases like cardiovascular, metabolism and respiratory.
  - While they are patient-centric, they do not provide a broad enough value perspective in areas of high unmet need and where benefits accrue beyond the patient
- How do we balance early access for treatments with high potential benefit with sufficient evidence?
Do novel value measures have a place in European HTA

- European HTA focused on patient relevant outcomes
  - Survival, morbidity, patient quality of life
- High bar for surrogate endpoints
  - No acceptance for PFS, DFS, ORR, pCR etc
- Need to demonstrate patient relevance for new endpoints
  - Can we link this to morbidity and patient quality of life?
  - Does it result in efficiency and process improvements in patient care?

We want proof, not promise
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But what about the value of hope?

Overall Survival vs. time

- Placebo control
- Cancer immuno-therapy
- Current standard of care including targeted therapies
- Combination: CIT+CIT, CIT+targeted therapy

How far can this be pushed up?

Institute for Clinical and Economic Review 2017
We want proof, not promise

*But what about the value of hope?*

- How do we achieve both static and dynamic efficiency?
- How do we value the potential for longterm durable benefit?
- How do we balance early access for high potential benefit with sufficient evidence?

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We pay for benefit, not for unmet need
We pay for benefit, not for unmet need

*Is it important to advance science and create real option value?*

- Alzheimer´s Disease is considered one of the biggest unmet needs and public health problem of the future.
- To date, over 100 molecules intended for modifying the course of Alzheimer´s Disease have failed in late stage clinical development
- We have reason to believe that the first medicines that slow the progression of AD will be approved in the next few years
- They will not be perfect, but they will increase our understanding of disease and pave the way for new more effective treatments
- They will preserve cognition and function so that patients may benefit from these more effective treatments

Our focus is on relevant patient benefits. These include mortality, morbidity and quality of life.
Our focus in on relevant patient benefits. *But isn’t there more than mortality, morbidity and quality of life?*

- Example: Autism spectrum disorders
- Only symptomatic therapies
- How do we value improved social communication & interaction?
- What about the impact on families?

Value measures still focused on traditional chronic diseases. *Need to include new components of value to reflect focus on unmet need and novel therapies*

- Need flexible approach to balance sufficient proof of longterm benefit with early access for patients in need
- As we approach previously untreatable diseases, we will increase our knowledge and tap into potential for further improvement of patient impact
- Because some of these diseases were not treated before, the direct costs have been low - need to provide more visibility into the full family and societal costs
- As we broaden the perspective on value, how will this affect our decision making?
Doing now what patients need next