

# 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?

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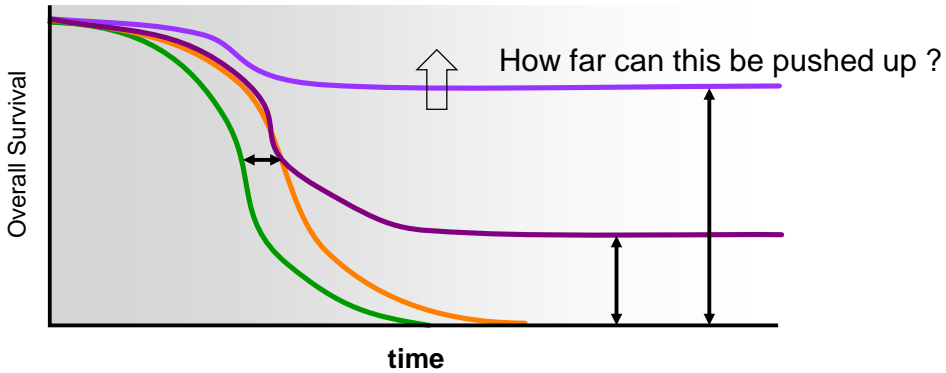


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**We want proof, not promise**

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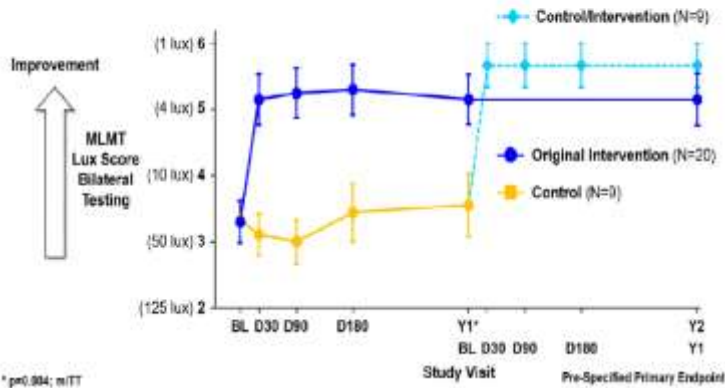
**We want proof, not promise**  
*But what about the value of hope?*



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

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

Figure 4.4. Observed Mean Bilateral MLMT Lux Score in Modified Intent-to-Treat Participants Out to 2-years in Phase III Study<sup>a</sup>



<sup>a</sup> p=0.004; mITT



**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**

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**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

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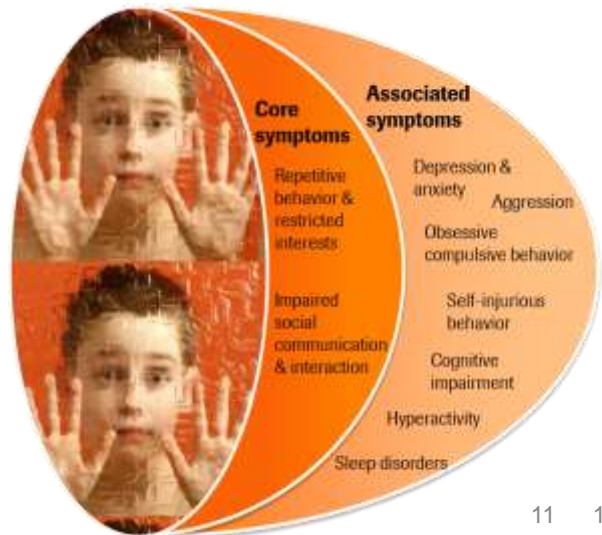
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**Our focus is on relevant patient benefits.**  
**These include mortality, morbidity and quality of life**

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**Our focus is 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?



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**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***