



Histology Independent Indications The HTA view

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15/12/17



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- To obtain a consolidated opinion from HTA's is pretty much close to impossible due to the large heterogeneity of methodologies in use
- Consequently new trial designs pose different problems for different HTA's
- With the help of Michelle Mujoomdar from EUnetHTA / ZIN we performed a 'quick and dirty' mini-survey among members of EUnetHTA and international HTA's.

Survey

- Questions were aimed at exploring the
 - Awareness around new trial designs
 - experience with them
 - acceptability in the health-economic context
 - specific problems arising from such trials
- 5 EU, Canada and Australia

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- Some agencies had experience assessing such trials, some mainly in early phase development, but not all
- No agency disapproved of such trials but similar methodological concerns were raised as discussed earlier
 - Statistical power
 - Uncontrolled aspects of some trial designs
 - Independence !

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- In some rare diseases the only feasible option (Pts/Reg/Ind/HTA?/Pay?)
- Allow answering multiple research questions in parallel (Pts/Reg/Ind/HTA/Pay?)
- While some advantages are identified for other stakeholders, the majority of HTA's couldn't see advantages for the health-economic assessment.

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- Lack of robust comparative data
- Translation of results into a relevant patient-level outcomes
- Lack of (statistical) methodology that ensures robust assessment of the (relative) effectiveness
- There is not enough experience at the moment to relieve the reluctance to endorse these approaches

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- Making reimbursement decisions based on a biomarker approach is not principally considered impossible by the HTA's included in the survey!
- The question is rather how the population is defined whether a standard of care can be established
- Baskets would by most HTA's be considered separately (see the earlier presentation)
- The acceptability of endpoints (hard versus surrogate, clinically relevant versus patient relevant) is not considered dependent on the 'type' of study.

Conclusion

- We need get our terminology aligned
- The elements that make up a study are the key, not the wrapper
- The design elements need to be agreed on at an early stage by all stakeholders
- Flexibility in designs is a pro, too much flexibility not
- Results need to be interpretable!
- Nothing is impossible, some things are just difficult

Thanks go to

- Michelle from ZIN for setting up the survey and tapping into her international network
- All HTA's that did try to answer our (not always clear) questions
- The organizers of the workshop for inviting also the HTA's to provide their opinion

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