Health Technology Assessment

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The Fourth Hurdle

There was general agreement that the fourth hurdle was the one to look out for.
What the Regulatory/HTA Interface might look like in the future – EMA Perspective*

Current paradigm

Assessors
- Regulators
- Payers

Assessment Focus
- Quality, Safety, Efficacy, (First 3 hurdles)
- Benefit-Risk Profile
- Relative Efficacy / - Effectiveness, Cost vs Health Benefit, Budget Impact (4th hurdle)

Studies / Data
- Emphasis on: RCT, most often placebo-controlled
- Active-controlled RCT; Observational studies, Cost-effectiveness/utility analyses, Budget impact analysis

Future paradigm?

Regulators
- Payers

Dedicated Relative Efficacy/- Effectiveness Assessment?

Quality, Safety, Efficacy, Benefit-Risk Profile

Cost vs Health Benefit, Budget Impact

Relative Efficacy / - Effectiveness

Emphasis on: RCT, more often active- and placebo-controlled

Cost-effectiveness/- utility analyses, Budget impact analysis

Active-controlled RCT, Adaptive phase III-IV trials
Observational studies, Meta-analysis

MA = Marketing Authorisation; RCT = randomised controlled trial

* How Regulatory Agencies could interact with Health Technology Bodies, Presentation at DIA, Berlin, March 2009, by Thomas Lonngren, EMA
Health Outcomes in Product Development

**Target/Candidate Selection**
- Reimbursement: desk research, external advice*
- Develop/test value hypotheses

**Phase I**
- Burden of Illness, unmet need
- Target endpoints and claims, PRO development
- Assess reimbursement and access needs

**Phase II/POC**
- Early economic modelling, pricing research
- PhIIb: Ensure clinical devt plans address payer needs
- Identify possible sub-populations

**Phase IIb**
- PRO validation (if required)
- PhII: Meaningful endpoints, comparators
- Burden of Illness Studies

**Phase III**
- ‘Value Dossier’ and local submissions
- Economic model, budget impact model(s)
- PRO analyses

**Phase IV/Post Marketing**
- ‘Real World ‘ effectiveness, cost-effectiveness
- ‘Managed Access’: further study commitments
- Adapt models, disease management tools

*External advisors/payer input obtained iteratively throughout product development

Based on: Sollano JA, Kirsch J, Bala MV, Chambers MG et. al. Clinical Pharmacology & Therapeutics (2008); 84, 2, 263–26
Relationship
clinical development – HEOR/HTA

• In the past: work in parallel w/o much interaction
  – HEOR/HTA „recycled“ the clinical study data

• Today & future:
  – Incorporation of HTA data needs into planning of clinical development programme
  – Joint PRO validation activities
  – Partnership between statisticians and 4th hurdle colleagues, e.g. Health Economics, HTA Policy/Corporate Affairs, Pricing
Skills sets in HTA

• Observational research versus interventional research
  – Linking “efficacy” with “effectiveness”

• Economic related outcomes
  – Health resource utilisation measures

• Evidence synthesis
  – indirect/mixed treatment comparisons
  – Subgroups/subpopulations of interest

• Modelling life-time clinical and cost outcomes
  – use of surrogate endpoints
Suggested points for discussion

• Is the HTA area seen as an area where statisticians should be involved?

• If yes,
  – Are statisticians in the industry sufficiently equipped to deal with the statistical challenges faced in HTA research?
  – How can EFPSI promote the statistical profession in the HTA area?