Assessment of Risk Benefit

Jürgen Kübler (presentor)
Thorkild Nielsen (facilitator)

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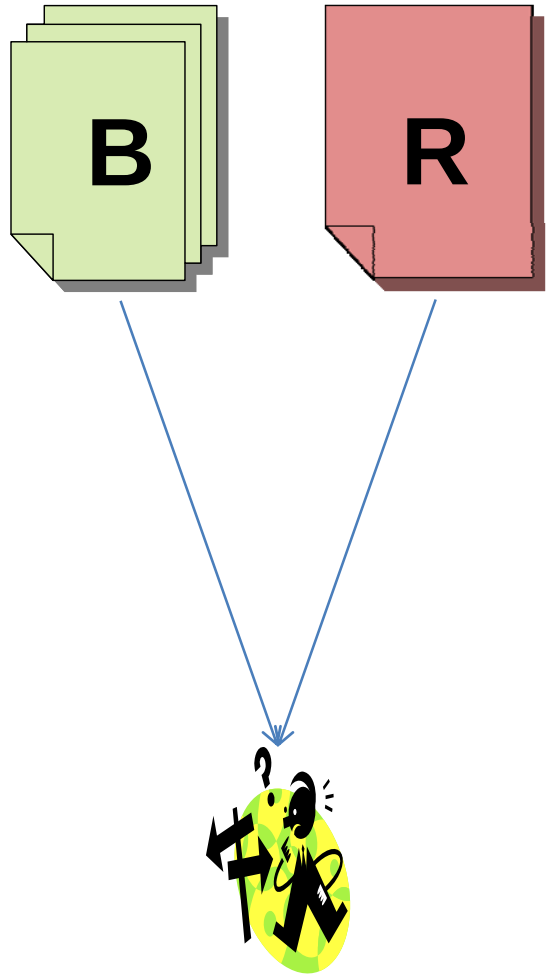
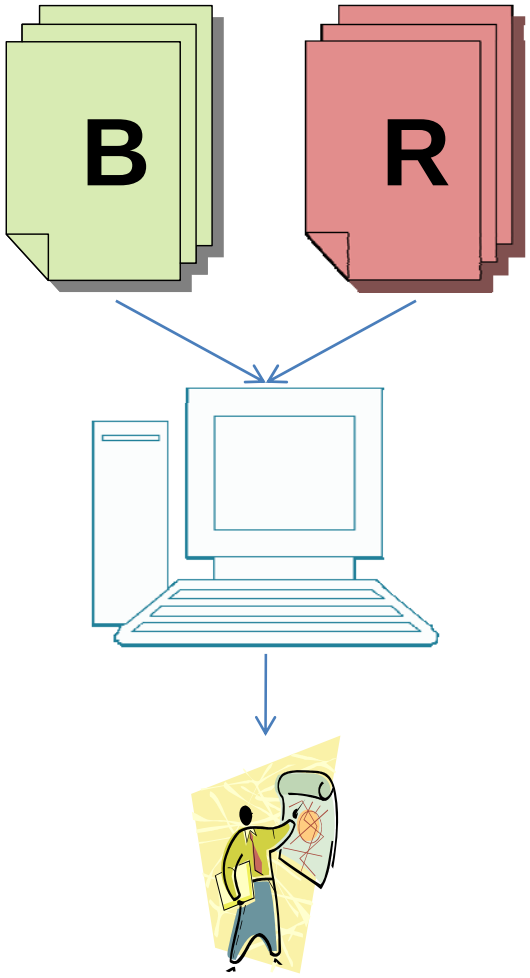
Cartoon deriding chronic disease epidemiology, for randomly generating fears by investigating seemingly unrelated risk factors and diseases

This cartoon contains a grain of truth: observational research is at its methodological best in discovering unexpected adverse effects.

Now and Tomorrow...





Current Positions & Perceptions
on Quantitative Benefit-Risk Assessments

- …it cannot replace the rigorous assessment of efficacy and safety individually

  Chuang-Stein et al, Drug Inf J 2008

- Quantitative benefit-risk assessment is not expected to replace qualitative evaluation

  EMEA CHMP, March 2008

- Progress in evaluating benefit:risk will be a function of how quickly the culture of quantitative safety assessment changes in the future

  O'Neill, Drug Inf J 2008

IMI Strategic Research Agenda

Over 350 senior representatives of patient organisations, universities, hospitals, regulatory authorities as well as small and large biopharmaceutical companies contributed to the elaboration of the IMI Strategic Research Agenda.

The IMI Strategic Research Agenda describes the bottlenecks in the biomedical research and development process as:

- predicting safety,
- predicting efficacy
- bridging gaps in knowledge management, and
- bridging gaps in education and training

Questions to be tackled

- Methodological development / consensus on approaches
  - Population based approaches vs patient preferences
  - Surrogates vs clinical endpoints
  - Can we learn from HTA?
- Preceived inbalance between efficacy and safety assessments
  - Can we learn form efficacy assessments
- Benefit-Risk management over time

Skills Sets in Benefit-Risk

- Prospective approaches in safety assessment
  - ‘Domain’ oriented summary of routine safety data vs. objective (medical question) driven approaches
- Observational research versus interventional research
  - External validity & benefit-risk in ‘real world’ setting
- Evidence synthesis
  - indirect/mixed treatment comparisons
  - Subgroups/subpopulations of interest
- Modelling life-time benefit and harm outcomes
  - use of surrogate endpoint

Suggested Points for Discussion

- As a statistical community, how should EFPSI or Professional statistical community engage with the IMI initiative?
- How should EFPSI or Professional statistical community link with Academia?
- How can EFPSI or Professional statistical community help to develop best practise?