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Developing drugs for reimbursement - current and future challenges for statisticians
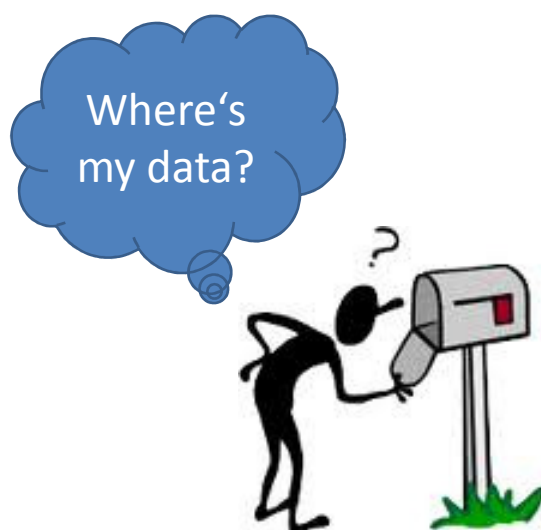
Martin Scott
Numerus

ROeS Dornbirn 2013

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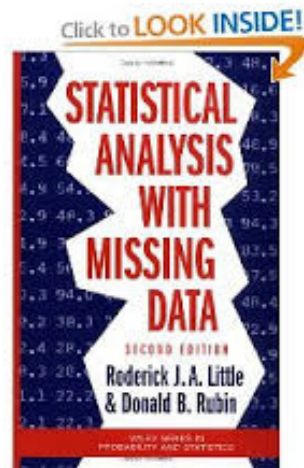
Challenges – past, present and future

Past challenges?

- Many

Missing data

Where's my data?

Missing data

Current challenges?

- Many

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Missing data and reimbusement

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The four hurdles

The ~~four~~ **only** hurdles

Generate data

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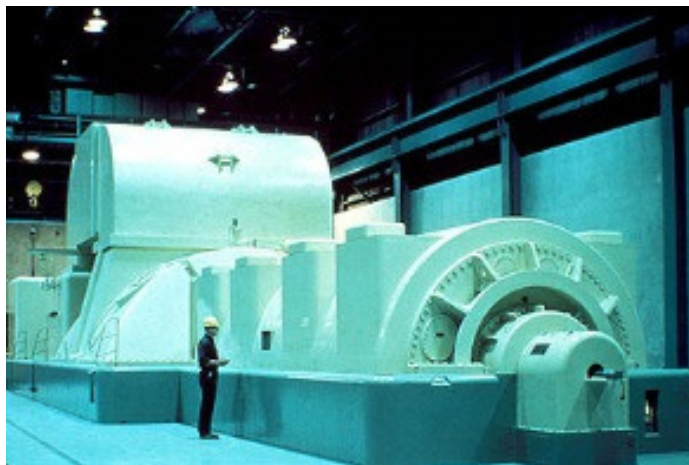
Our quest to generate data

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Generate data

What are the HTA
requirements?

Choose the right population

Choose the right endpoints
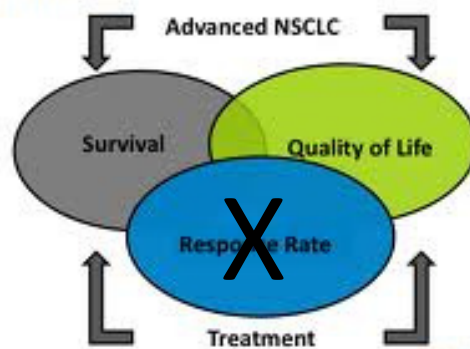
Plan (and power?) for subgroups

Our enemy

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Our enemy

Finance
Department

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Requires investment

What type of data for HTA?

Real world data

Real world data

- "…data collected under real life practice circumstances"

  (Lieven Annemans et al – ISPOR task force)

Real world data

- "…data collected under **real? life? practice?** circumstances"

  (Lieven Annemans et al – ISPOR task force)

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Do we already have a way forward?

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Which way?

TheITTWay

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ICH – E9

- **Intention-to-treat principle:** The principle that asserts that the effect of a treatment policy…

  …subjects should be followed up, assessed, and analyzed as members of that group irrespective of their compliance with the planned course of treatment

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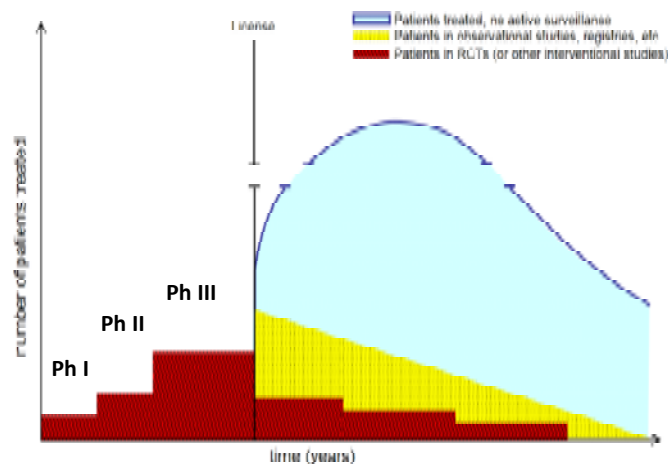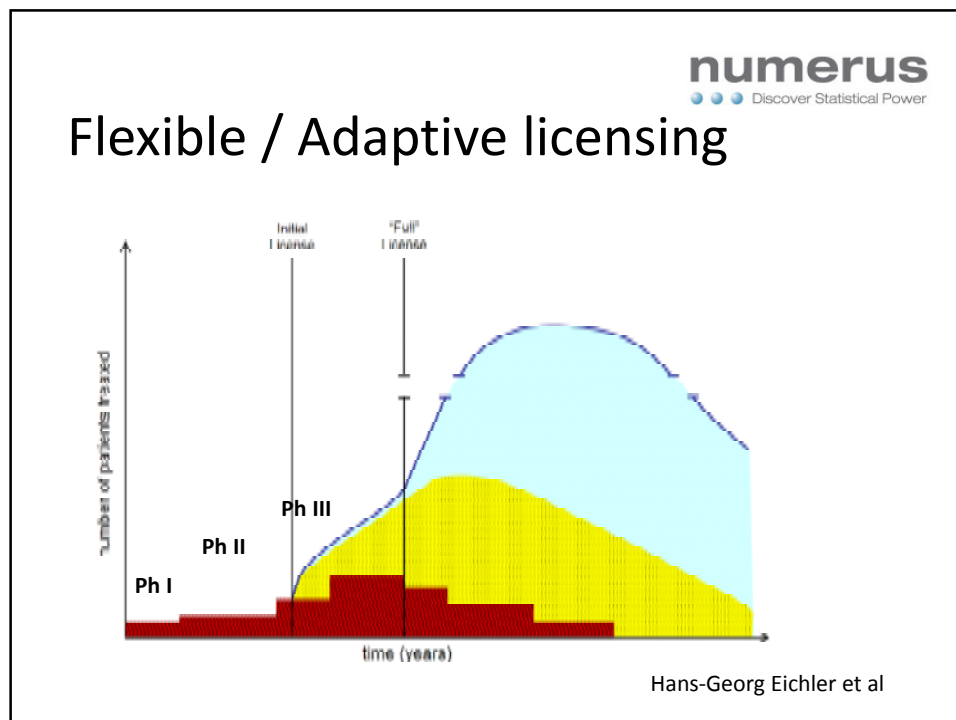
ITT – a full house

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Investment

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Generate data

- When is the data going to be generated?

- What's the cost? Who will pay?

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Current licensing

Hans-Georg Eichler et al

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Flexible / Adaptive licensing

Hans-Georg Eichler et al

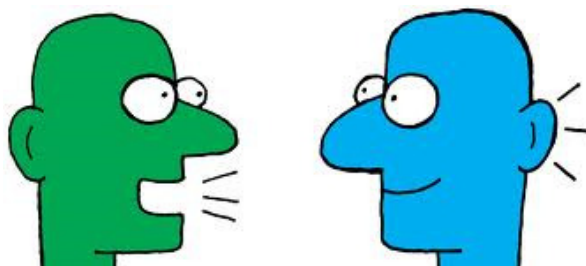
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Adaptive Licensing

- Licence at end of phase II (conditional on further trials)
- Conduct **BIG** phase III on "real world data", controlled or uncontrolled

Challenges for statisticians

- Which data do we collect for phase II? Which for phase III?
- What is convincing proof at phase II?
- How do we allocate type I / II error between phases II and III?
- If uncontrolled, how do we control? Which methods are available?

Biggest challenge for statisticians

Communicate