THE USE OF EXTERNAL DATA FOR DECISION MAKING

BBS Spring Seminar

Panel discussion

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EXTRAPOLATION

RCT

Prediction

Routine data

Common disease

Rare disease

Small RCT

Borrowing

Registries
EVIDENCE SYNTHESIS

- **Pairwise meta-analysis**
  - comparing two treatments

- **Meta-regression**
  - including study-level covariates

- **Network meta-analysis**
  - comparing multiple treatments indirectly

- **RCT with historical controls**
  - integrating control group data from previous trials

- **Generalized (or cross design) synthesis**
  - combining data from different types of studies
HIERARCHICAL MODELS

Meta-analysis

Studies
(RCT, registry, …)

Patients

Example: Normal-normal hierarchical model (NNHM) for random-effects meta-analysis

\[ y_i | \theta_i \sim \text{Normal}(\theta_i, s_i^2) \quad \theta_i | \Theta, \tau \sim \text{Normal}(\Theta, \tau^2) \]
QUANTITIES OF INTEREST

Different quantities of interest in hierarchical models

- average effect ($\mu$) across studies
  - standard (pairwise) meta-analysis

- effect ($\theta_{k+1}$) of a future study
  - prediction / extrapolation: adult to children; bridging

- effect ($\theta_i$) of an individual study in the light of the other studies (shrinkage estimator)
  - small RCT with borrowing from registry

Inference of effects must account for between-study heterogeneity and potential biases.