What are pragmatic trials good for?

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Goals

• Explain distinction between pragmatic and explanatory trials
• Criticize standard view about pragmatic trials
  • Similarity thesis
  • Trade-off thesis
  • Straightforward extrapolation thesis
• How to improve problems with the standard view?
  • Framework
  • Additional causal evidence
• Conclusion
What are pragmatic trials?

• Opposite of explanatory trials
What are pragmatic trials?

- Opposite of explanatory trials
- Differ regarding:
  - eligibility criteria
What are pragmatic trials?

• Opposite of explanatory trials
• Differ regarding:
  • eligibility criteria
  • clinician expertise
What are pragmatic trials?

- Opposite of explanatory trials
- Differ regarding:
  - eligibility criteria
  - clinician expertise
  - compliance
The trade-off thesis

• There is a trade-off relationship between internal and external validity in medical trials.
• Pragmatic trials strike a more sensible balance between these two competing desiderata than explanatory trials do.
Against the trade-off thesis

- In some cases, internal validity can be increased with no costs to external validity
- In some cases, internal validity can be decreased with no gain (possibly a loss) to external validity
The Basic Model

\[ Y = \beta X + \gamma (X \ast W) + U \]

Y = outcome of interest
X = treatment variable
W = vector of interactive covariates
\( \beta, \gamma \) = the parameters for the marginal effect of an intervention on X
U = causes of Y which are independent of X and W
The Basic Model (example)

\[ Y = \beta X + \gamma (X \times W) + U \]

- \( Y \) = outcome of interest (headache intensity)
- \( X \) = treatment variable (aspirin intake)
- \( W \) = interactive covariates (interactive other medication)
- \( \beta, \gamma \) = the parameters for the marginal effect of an intervention on \( X \)
- \( U \) = causes of \( Y \) which are independent of \( X \) and \( W \) (head banging?)
Three kinds of idealization

• Homogenization with respect to U (other causes)
• Homogenization with respect to W (interactive other medication)
• Homogenization with respect to W (compliance)
The Basic Model (example)

\[ Y = \beta X + \gamma (X \ast W) + U \]

Y = outcome of interest (headache intensity)
X = treatment variable (aspirin intake)
W = interactive covariates (interactive other medication)
\( \beta, \gamma = \) the parameters for the marginal effect of an intervention on X
U = causes of Y which are independent of X and W (head banging?)
How can we improve?

- Framework (Mullers?)
- Additional evidence
  - Relevant covariates (and goals for extrapolating this)
  - Relation between distributions of covariates and effects
  - Distribution of covariates in target and experimental populations
How can we improve?

• Framework (Mullers?)
• Additional evidence
  • Relevant covariates and goals for extrapolation (Mechanistic Evidence)
  • Relation between distributions of covariates and effects (Mechanistic Evidence)
  • Distribution of covariates in target and experimental populations (Observational Evidence)
How can we get this additional evidence?

- Subgroup analysis
- Factorial experiment
- Collect more data on possible covariates during trials
Thank you

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