What are pragmatic trials good for?

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"Inferring Policy from Evidence"

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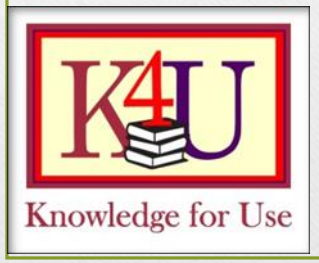

Goals

- 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

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  - eligibility criteria

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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

$$Y = \beta X + \gamma(X * W) + U$$

Y =outcome of interest

X = treatment variable

W = vector of interactive covariates

β, γ= 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)

$$Y = \beta X + \gamma(X * W) + U$$

Y =outcome of interest (headache intensity)

X = treatment variable (aspirin intake)

W = interactive covariates (interactive other medication)

β, γ= 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)

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$$Y = \beta X + \gamma(X * W) + U$$

Y =outcome of interest (headache intensity)

X = treatment variable (aspirin intake)

W = interactive covariates (interactive other medication)

β, γ= 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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