DRAWING INFERENCES ABOUT EFFECTS OF MEDICAL TREATMENT

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The debate about whether we can derive useful information about treatment effects from observational data has been going on for a long time.

EVIDENCE PYRAMID (ONE VIEW)

- Systematic Reviews and Meta-analyses
- Randomized Controlled Double Blind Studies
- Cohort Studies
- Case Control Studies
- Case Series
- Case Reports
- Ideas, Editorials, Opinions
- Animal research
- In vitro ('test tube') research
BUT—THINGS ARE NOT ALWAYS SO CRISP AND CLEAR

- Sometimes randomized trials yield inconsistent results
- Sometimes a meta-analysis shows results inconsistent with a large follow-up RCT
- Sometimes an observational result is so compelling that everyone believes it even without randomized trials
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- Sometimes randomized trials yield inconsistent results
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- Sometimes an observational result is so compelling that everyone believes it even without randomized trials
- Lots of questions are difficult or impossible with randomized trials
RCTs ARE PROBLEMATIC WHEN

- We are concerned about whether a product that is no more effective than competing products is adequately safe
- We are concerned about the possibility of rare but serious adverse events (even when the product is more effective than competing products)

Improved approaches to studying these questions in observational databases are needed
WE NEED TO WORRY ABOUT A GROWING PERSPECTIVE THAT IF YOU HAVE LOTS OF DATA YOU AUTOMATICALLY HAVE THE ANSWERS
I visited 23andMe in the summer of 2013.
They were very excited about the possibility of collecting genetic data on millions of people, and then getting data on the medications they took and their health outcomes.
They were sure that with all those data they’d be able to quickly see what treatments were best for certain genetic profiles.
They had no clue about any of the issues discussed here over the past 2 days.
MY TAKE

- We need to make the process of doing randomized trials more efficient
- The move toward building randomized clinical trials into health systems, so that research data are derived from health system data, is a very positive development
- We need to come to grips with the unnecessary burdens we have built into our systems of research oversight
- Observational studies are needed to study safety issues, but will not be able to detect small effects reliably
- There is insufficient understanding of what we give up when we move away from randomized trials
A PLUG

- Penn’s 2015 Conference on Statistical Issues in Clinical Trials will focus on issues in pragmatic trials
- April 15, 2015 in Philadelphia
- Great group of speakers and faculty
- Look for announcements in December