

Comparative Effectiveness Research Looking Backward and Forward

Alliance for Health Policy Briefing



Sean Tunis, MD, MSc | 17 April 2019

CENTER FOR MEDICAL TECHNOLOGY POLICY

The Evidence Paradox

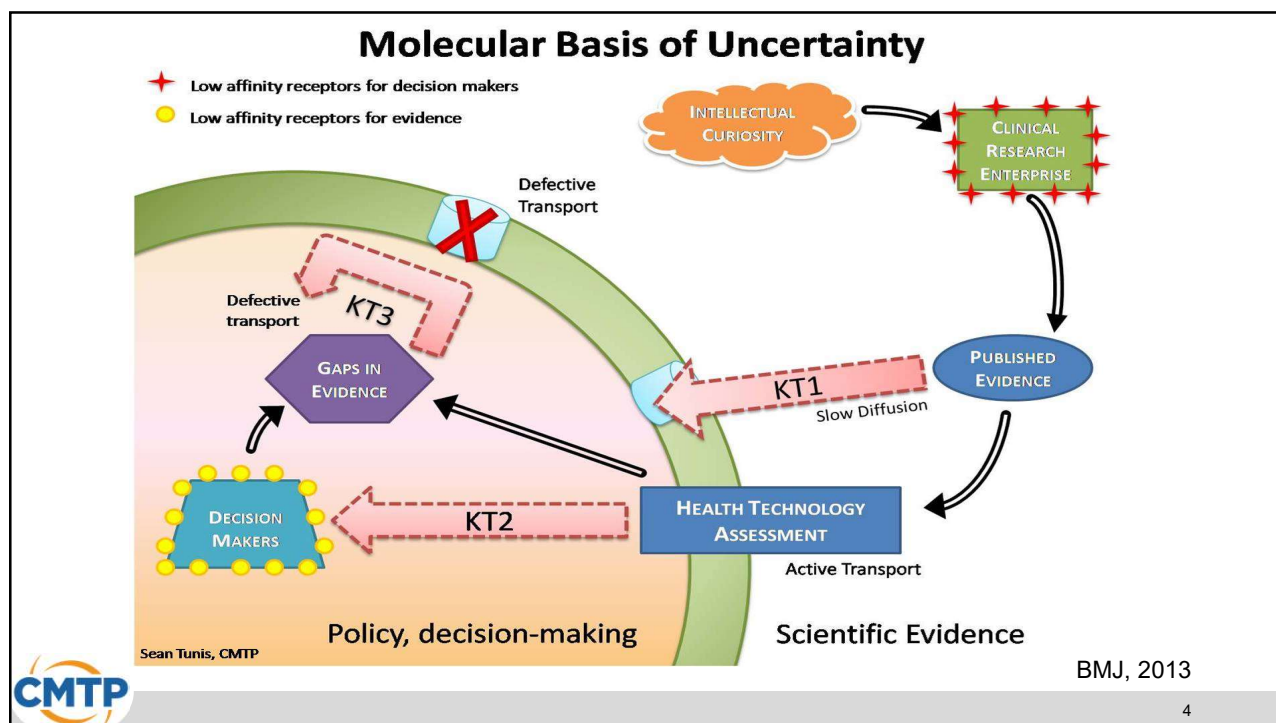
- 19,000+ RCTs published every year
- Tens of thousands of other clinical studies
- Systematic reviews intended to inform payers, guideline developers, patient education material regularly conclude that evidence is inadequate or poor quality



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What Was Missing?

- Research agenda targeted to decision makers
 - Patients, clinicians, payers, health systems
- Studies in broader patient populations
- Studies of care provide by “typical” clinicians
- Comparisons with current standard of care
- Outcomes that matter most to patients, clinicians



From CERI to PCORI

- Original draft legislation to create CER
- CERI became linked with “death panels” in ACA debate
- Renamed PCORI to emphasize patient focus
- PCORI leadership took this focus seriously
 - And avoided any hint of costs, cost-effectiveness, payers
- Patient-centered agenda led to key findings on nurse care coordinators, navigators, community-based care.
- Influenced NIH, FDA, and life sciences industry



Pragmatic Clinical Trials

Practical Clinical Trials

Increasing the Value of Clinical Research
for Decision Making in Clinical and Health Policy

JAMA[®]

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Decision makers in health care are increasingly interested in quality scientific evidence to support clinical and health policy. However, the quality of available scientific evidence is often

“Clinical trials for which the hypothesis and study design are developed specifically to answer the questions faced by decision makers”

PCORI and NIH Collaboratory have funded many of these

Best for longstanding questions: ASA dosing, duration of dialysis, when to take HTA drugs

TUNIS SR et al. *Jama*. 2003; 290: 1624-32



HTAi HEALTH TECHNOLOGY ASSESSMENT INTERNATIONAL™

RWE Emerging as Major Future CER Focus

Logos: abpi, CMT, CLINICAL TRIALS TRANSFORMATION INITIATIVE, Duke MARGOLIS CENTER for Health Policy, EUROPEAN MEDICINES AGENCY, eunetha, CADTH Evidence Driven., ICER INSTITUTE FOR CLINICAL AND ECONOMIC REVIEW, PCORnet The National Patient-Centered Clinical Research Network, INAHTA, The NEW ENGLAND JOURNAL of MEDICINE, FDA, imi innovative medicines initiative.

ISPOR/ISPE Summit on Real-World Evidence in Health Care Decision Making
October 20, 2017
Washington, DC, USA
http://www.ispe.org/RealWorldEvidence

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N Engl J Med 2016; 375:2293-2297 | December 8, 2016 | DOI: 10.1056/NEJMs1609216

New Technologies: Payment in Trials is Essential



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