

# Payer Perspectives on Large Simple Trials

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Workshop: LSTs & Knowledge Generation in A Learning Health System

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# How evidence is used to inform medical policy

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- Definition of a “Covered Service” as a contract term
- Use of a defined, structured review process, with an explicit hierarchy of evidence
- Separate consideration of **clinical efficacy and effectiveness** (to determine whether the service is eligible for coverage) from **costs and cost-effectiveness** (to guide approaches to promoting optimal quality and affordability)
- Policy Context: Medical Technology innovation valuable, but also can contribute to high and rising health spending without commensurate clinical benefits (too much “flat of the curve” health care)
- “New” Medical Technologies:
  - May not be all that “new”
  - May be “new” but with unclear clinical utility
  - May be new, with known clinical utility, but overused or with costs significantly greater than benefits
  - Need to promote “high value” innovation:
  - Often “disruptive” to existing care
  - Strong competitor to existing approaches (“faster, better, cheaper”, lower cost setting)
  - Significant clinical value or significant impact on costs (ideally, both)

# Common, Typical Questions:

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- Does it work? How strong is the evidence?
- Is a proposed new treatment safe (relative to other available treatments and the natural history of the disease)?
- What specific populations would benefit ? What specific populations would not?
- How does the procedure, service, drug or device improve health outcomes?
- What are the advantages, harms and alternatives to the proposed treatment?
- What is the clinical evidence of effectiveness and safety of the proposed treatment?
- How does it work in the “real world”?
- Which study design will answer safety and effectiveness questions specific to the treatment under review?
  - What questions can be addressed through retrospective observational series?
  - What questions will prospective multi-site observational series answer?
- How do we consider the strength of the evidence (e.g. GRADE) vs. the strength of the recommendations?

# Some Perspectives on Large, Simple Trials (LSTs) and Learning Health Systems

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- Potential to accelerate “speed to answer” vs. other study designs
- LSTs may offer more generalizable answers to relevant questions
- Simplicity can cut both ways, however:
  - Need reliable randomization and truly comparable cohorts
  - Need a 360 view of the what happens (side effects, system level effects)
  - Delivery context itself as an variable
- Some questions, while “answerable” via a LST, really need a traditional RCT
- “Knowledge” generated by a Learning Health System vs. “research”
  - Human subjects protection, consent
  - Research vs. operations/quality improvement
- Changing Policy Context: PPACA Provisions Expanding Coverage for Clinical Trials
  - Cancer and life threatening conditions
  - Phase I-IV

# Creating the Future by Inventing It:

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Need greater “speed to answer” and “speed to use”

Speed to answer:

- Understand the right questions earlier

- Create a “learning health system” where information is gathered, analyzed, disseminated as care is being delivered

- Capitalize on the opportunities created by “Big Data”—sophisticated analysis of observational data

- Increase use of modeling/simulation approaches

Speed to use:

- Better dissemination; clinical decision support

- Specialty society guidance, performance assessment and feedback

- Benefit design and incentives e.g. Value-Based Benefits

Need for new (and large) data sets: phenotypes, functional status, patient-reported outcomes

Also continue to need prospective trials: LSTs can play an important role here

**We need to work together to promote high-value innovation!**