# Improving Evidence for Decision Making

## *IOM Workshop: Assessing and Improving Value in Cancer Care*

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# Value and Uncertainty

- Variety of definitions, all of which include some type of benefit
  - Improved health outcomes
  - Hope / Opportunity
  - Innovation
- Some definitions include cost
- All types of benefit have uncertainty
- In most (all?) definitions of value, reducing uncertainty helps in assessing value

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# The Problem

- The health care system fails to get best value from technology due in part to uncertainty
- Critical evidence gaps are common
  - 18,000 RCTs published each year, but….
  - Most reviews conclude: "…available evidence is limited or of poor quality"
- Decision makers must deal with large uncertainty when considering value

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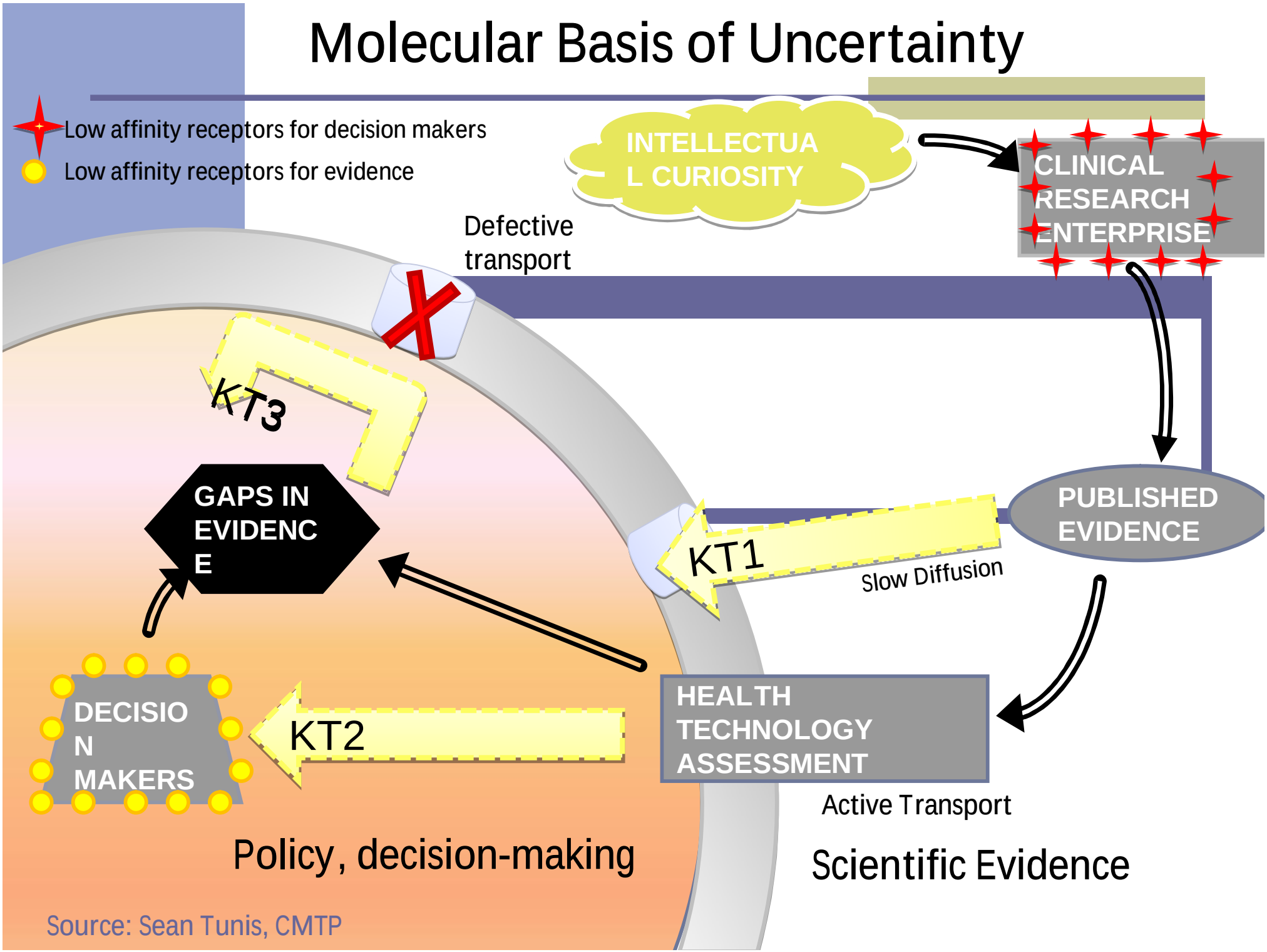
# Uncertainty and the Economy

- "better information about the costs and benefits of different treatment options, combined with new incentive structures reflecting the information….is essential to putting the country on a sounder long-term fiscal path." (Peter Orszag, June 2007)
- $1.1B in economic stimulus package for comparative effectiveness

# Molecular Basis of Uncertainty




Source: Sean Tunis, CMTP

# CMS Efforts to Improve Evidence

- Category  B IDE regulation (1996)
- Cover routine costs of clinical trials (2000)
- Working definition of “reasonable and necessary” (2000)
- Coverage with evidence development (2003)
- Promote pragmatic clinical trials (2003)
- Priorities for Sec 1013 of MMA (2004)
- MCAC becomes MedCAC (2005)
- Ad hoc efforts to work with NIH

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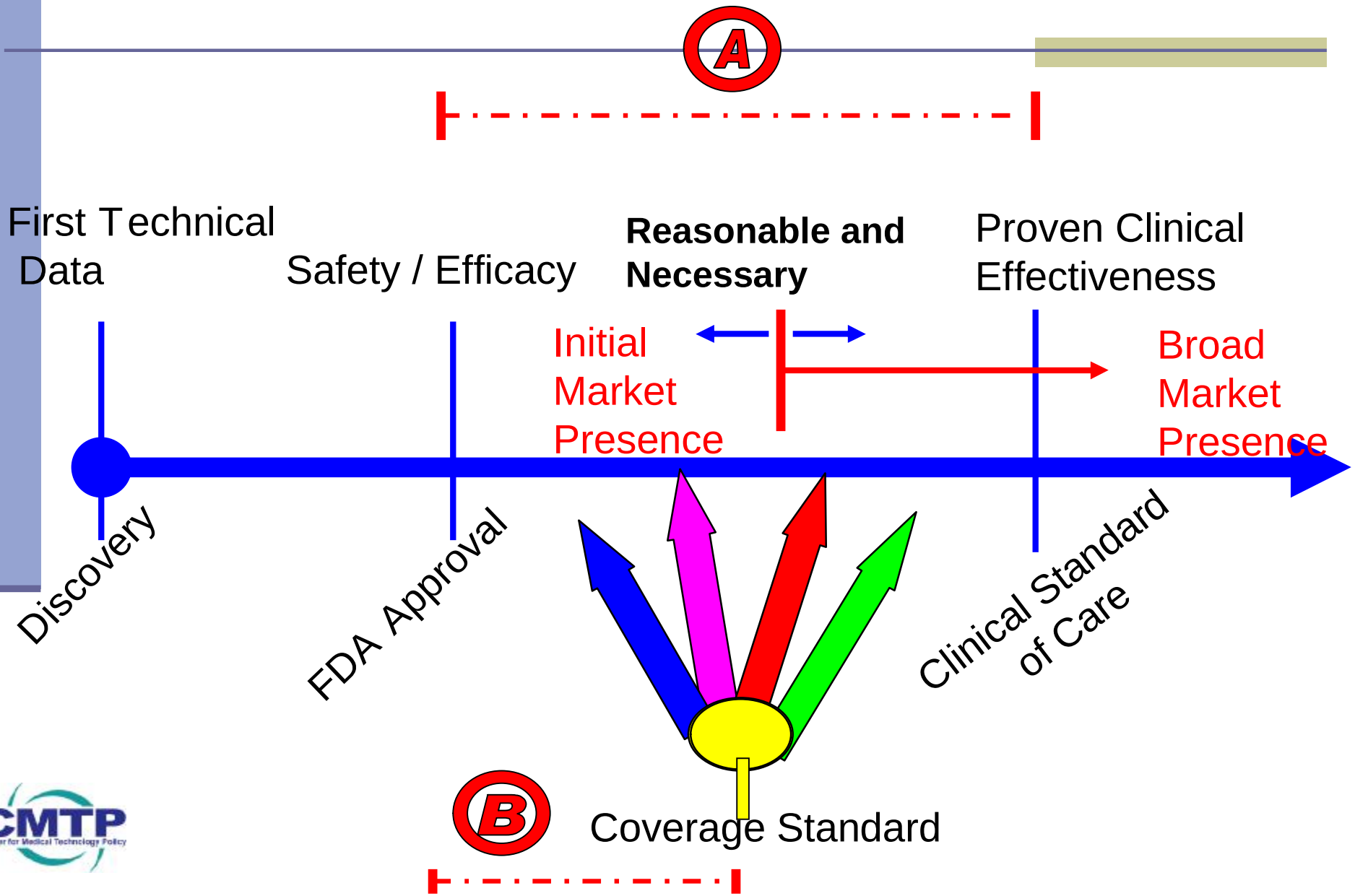
# Reasonable and Necessary

- Standard language adopted by CMS in 2000
  - "Adequate evidence to conclude that the item or service improves net health outcomes"
- Health outcomes are those that can be experienced by patients
- Major implications for influencing clinical research priorities and study design
  - Analogous to FDA "safety and efficacy"

# Natural History of Technology

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# Coverage with Evidence Development

- **Medicare's attempt to reduce uncertainty**
- **Links reimbursement to requirement for prospective data collection**
- **Medicare retains authority to approve study design**
- **Only (?) policy mechanism that aims to improve value by improving evidence**

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# Examples of Medicare CED

- Lung volume reduction surgery
- FDG-PET for suspected dementia
- Implantable defibrillators
- Off-label use of drugs for colorectal cancer
- FDG-PET for oncology
- Home testing for sleep apnea
- Artificial heart
- Coronary CT angiography (almost)

# Implantable Defibrillator Registry

- Medicare coverage expanded 01/05
- Registry intended for risk stratification
- 250k patients now in registry
- Baseline data interesting
  - Median age 74 (vs 60 in trials); LVEF higher
  - 3.6% complication rate
- No firing info or other outcomes data
  - Low priority for NHLBI, Industry, ACC/HRS
  - AHRQ has recently identified funds
  - Small fraction of $12.5B could have major ROI
  - Next time:  get industry/docs commitment first

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# CED Challenges

- Timing:  when coverage under review, may be too late for CED
- Methods
  - Difficult to design studies in coverage context
  - Registries provide broader access; ?? validity
  - RCTs viewed as equivalent of non-coverage
    - Large simple trials may help, but no examples
- Payers view as benefit expansion; Vendors opposite
- Unclear how best to fund clinical and research costs
- Private payers contract language
- May require a neutral coordinating entity

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# AHRQ Review: Tx of Clinically Localized Prostate Cancer

- Limited evidence on relative safety and effectiveness of major treatment options
  - prostatectomy, brachytherapy, radiation, active surveillance
- New technologies rapidly spreading without data
  - robotic surgery, proton beam
- Rigorous trials needed to compare treatment options, especially for side effects

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# Applying CED Lessons Learned

- Prostate Cancer: 186,000 new cases in 2008
- Proton Centers: “new nuclear arms race” (NYT)
  - More than twenty in development at $100m+ each
- Costs of Proton Beam Therapy about $85,000 per episode of treatment, more than 2X IMRT
- Proposed Study under ‘CED’: Proton Beam and IMRT in Treatment of Early Stage Prostate CA
- Multi-stakeholder collaboration including all major stakeholders: ASTRO, AHRQ, NCI, physicists, RTOG, vendors, plans, consumers, AUA
- Too late for true CED. Strategy to move forward despite fact that most plans already cover

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# Commercial Payer CED

- The CMTP multi-stakeholder group, with support from the California HealthCare foundation, has developed a Framework for implementing CED that includes:
  - A model of how different stakeholders would interact
  - Ways to incorporate CED into benefit language …or not
  - Criteria for selecting a research topic for a CED pilot project
  - Pragmatic research design criteria
  - Protections against anti-trust for plan sponsors
  - Transparent and accountable processes
- What's next?
  - Select high priority technology and implement

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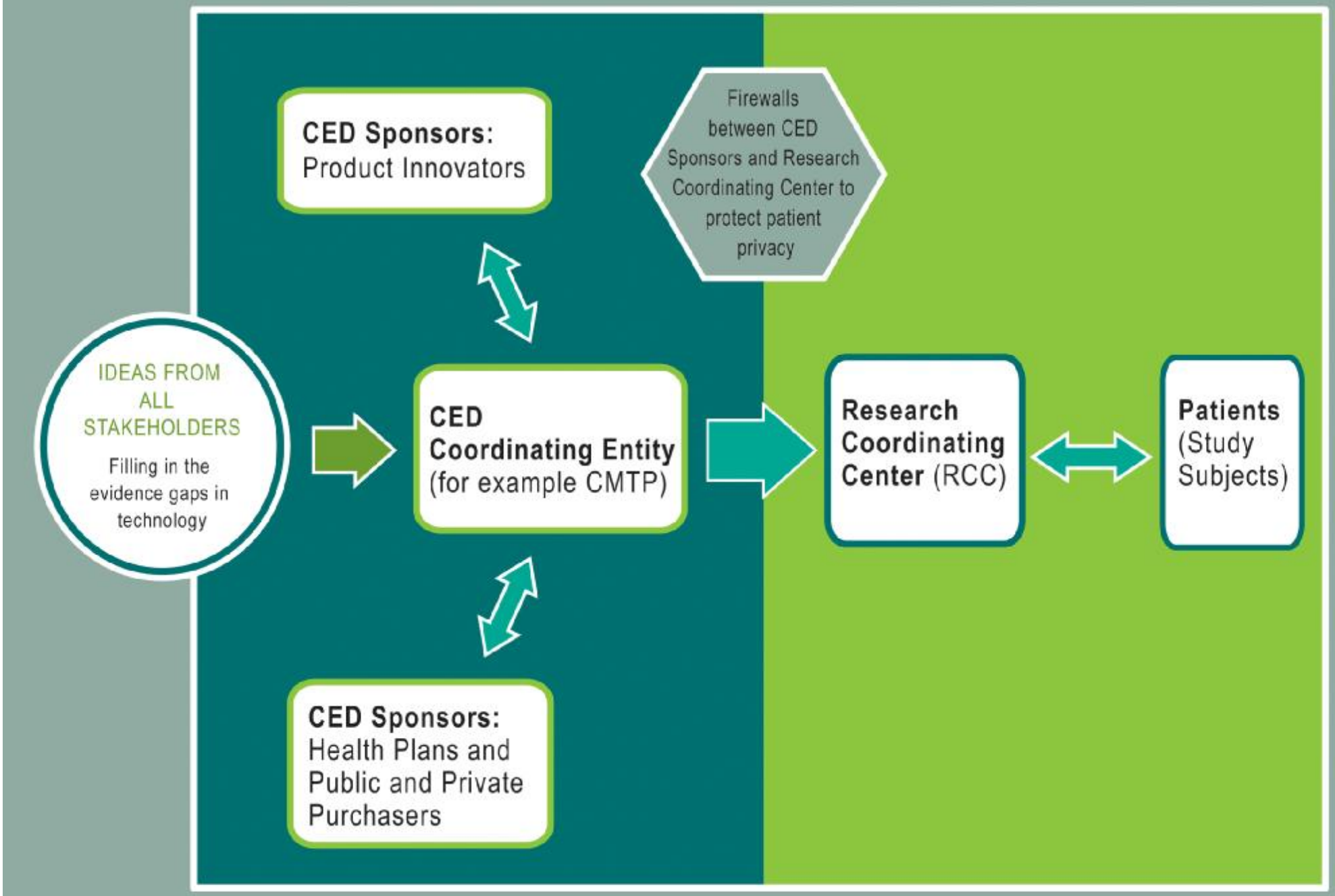
# The Coverage for Evidence Development Model (CED)



# Value and Uncertainty

- Variety of definitions, all of which include some type of benefit
- All types of benefit have uncertainty
- In most (all?) definitions of value, reducing uncertainty helps in assessing value
- Policy strategies to reduce uncertainty are still “experimental”

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# Brief Window for Evaluation

- "It is always too early to evaluate a new medical technology, until it is too late"
  - Doug Altman, quoting ??

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# Potential Topics for CED

- Devices/procedures for atrial fibrillation
- Biologics for treatment of osteoporosis, arthritis, cancer
- Molecular imaging
- Genetic tests and other molecular diagnostics
- Minimally invasive heart bypass surgery
- Merci Clot Retriever for Acute Ischemic Stroke

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# Manufacturers believe CED is going to happen

- How do product manufacturers view the impact of CED on their revenue stream?
  - The believe it will happen and are preparing for it
  - Some modeling predicts only 5% of expected revenue in the first three years of a CED study
  - Probability that product will meet or exceed evidence standards after study is completed is between 50% to 90%
  - Savings from inappropriate dissemination of new products will accrue to payers and be passed on to purchasers

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# Effectiveness Guidance Documents

- Analogous to FDA-guidance
- Targeted to product developers, clinical researchers
  - Recommendations for design of clinical studies to generate evidence that is adequate for decision making
  - “reasonable confidence” of improved health outcomes
- Started from insights from systematic reviews
- Multi-stakeholder advisory group, iterative draft and comment process
- Pilot projects
  - Gene expression profiling for breast cancer
  - Treatments for chronic wounds
  - Cardiac imaging

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# Pragmatic Clinical Trials Initiative

- Optimize design of phase III, IIIb trials to be maximally useful to post-FDA decision makers
- Clarify patient, clinician payer evidence needs
- Identify potential approach to more “pragmatic” designs
- Identify critical regulatory, methodological financial, operational barriers
- Develop PPCT guidance document
- FDA, CMS, CTTI, NICE others are confirmed

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