Generating Evidence for Decision Making

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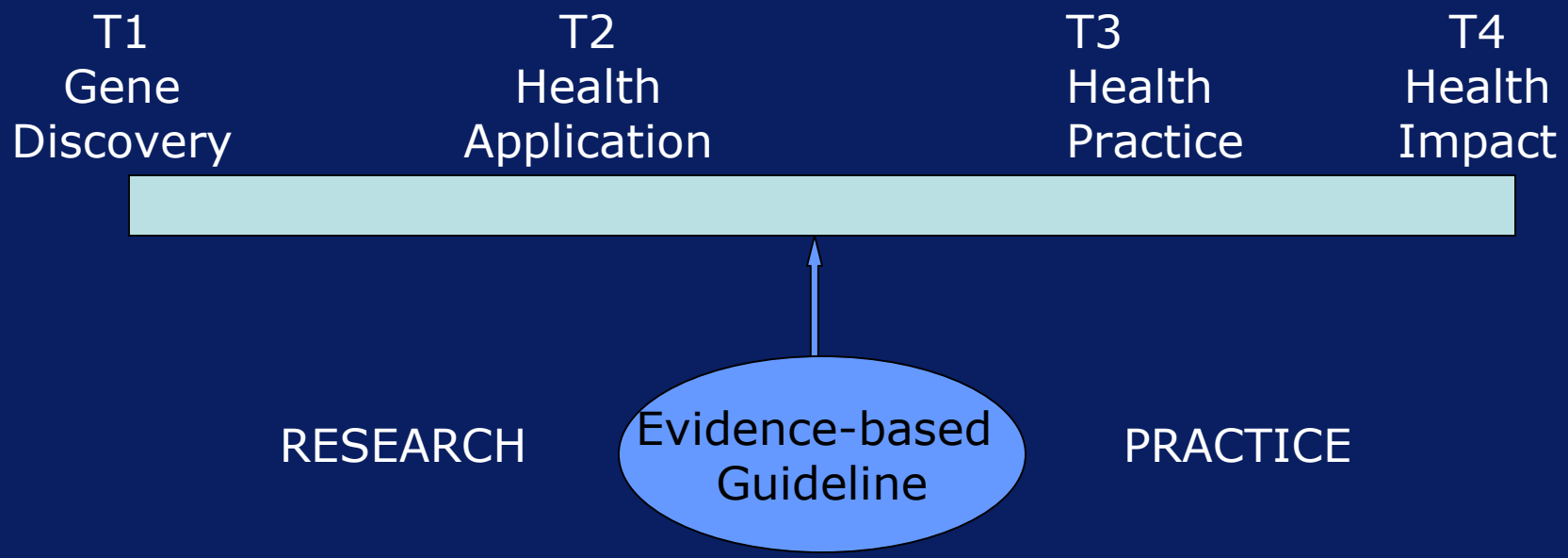
Issues

- Legitimization of health policy decisions will require:
  - Prospectively agreeing to evidentiary standards with a taxonomy of decisions consistent with the nature and importance of the decision to the population and/or individuals
  - A deliberative and inclusive process
- Taxonomy should not be formulaic or prescriptive but must allow consideration of:
  - Context: Need to determine a process for combining different kinds of information while making their relative contribution to decisions transparent

The Translational Process



How High Should the Evidence Bar Be?

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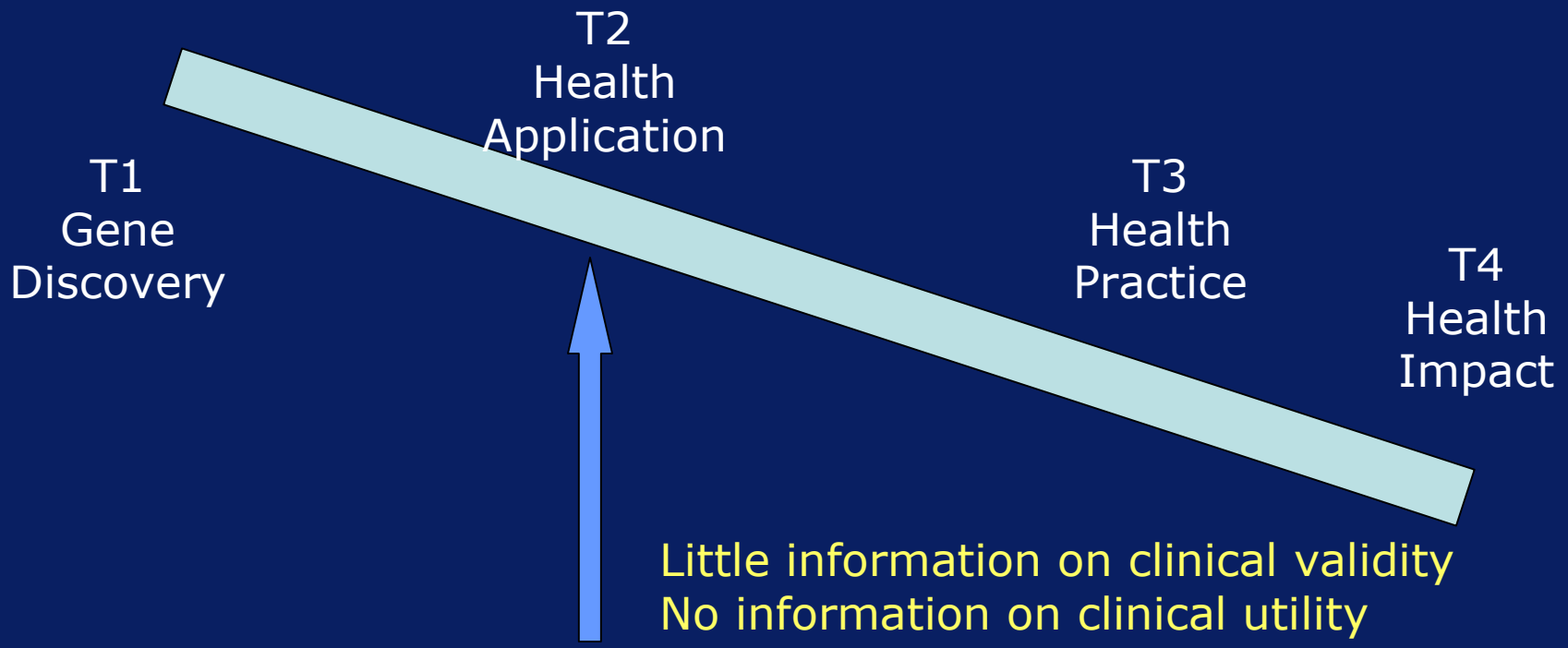
Lowering the Threshold for Translation into Practice



Little information on clinical validity
No information on clinical utility
Potentially no coverage
Potential for increased harms
Potential for increased benefits
Use based on expert opinion
Stimulate innovation

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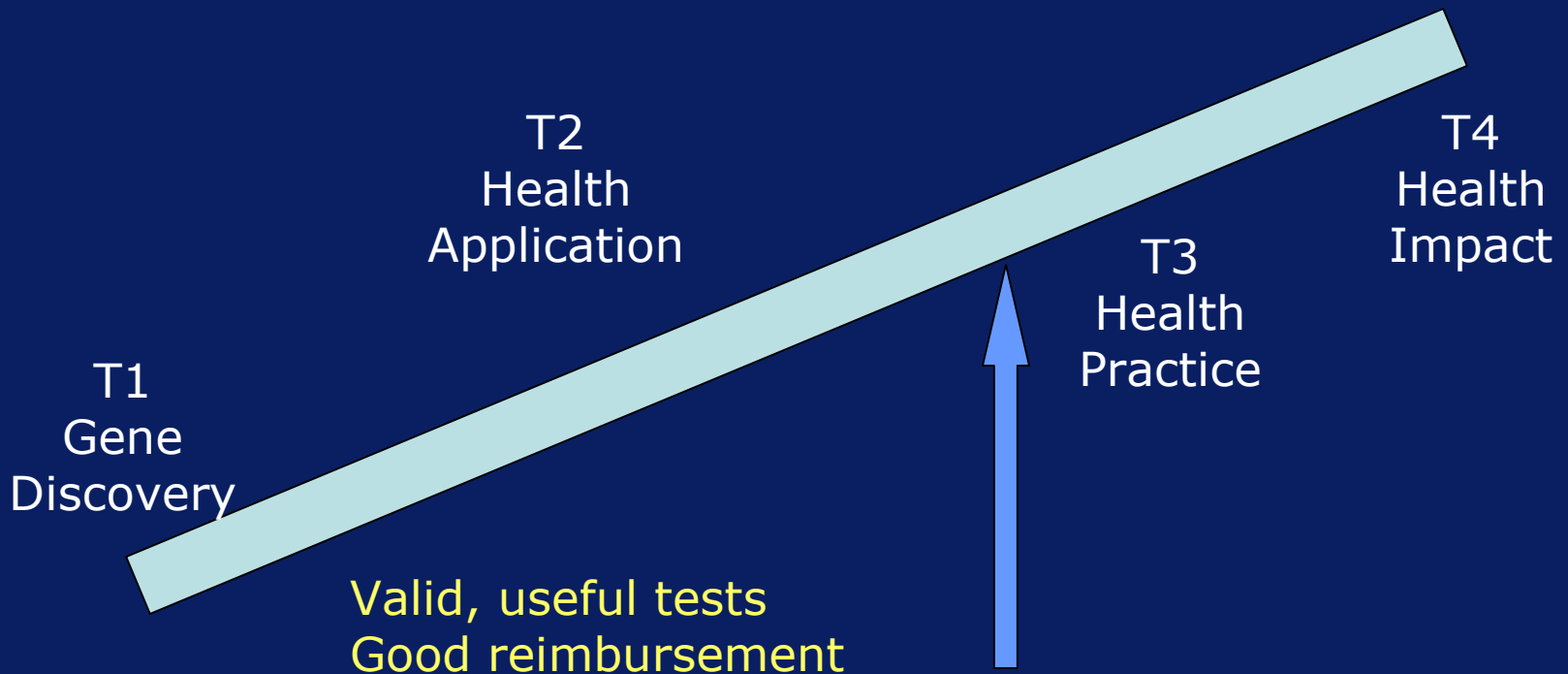
Raising the Evidentiary Threshold for Translation into Practice



Valid, useful tests
Good reimbursement
Lower incentive for innovation
Diminished potential for harms
Potential for diminished benefits
Strong evidence

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Evidence-based Decision Making

- What is the decision to be made?
- How does it affect the evidentiary standards?
- What are the relevant contextual factors?
- How does the information get integrated and applied?
- What processes are necessary to legitimize the decision?

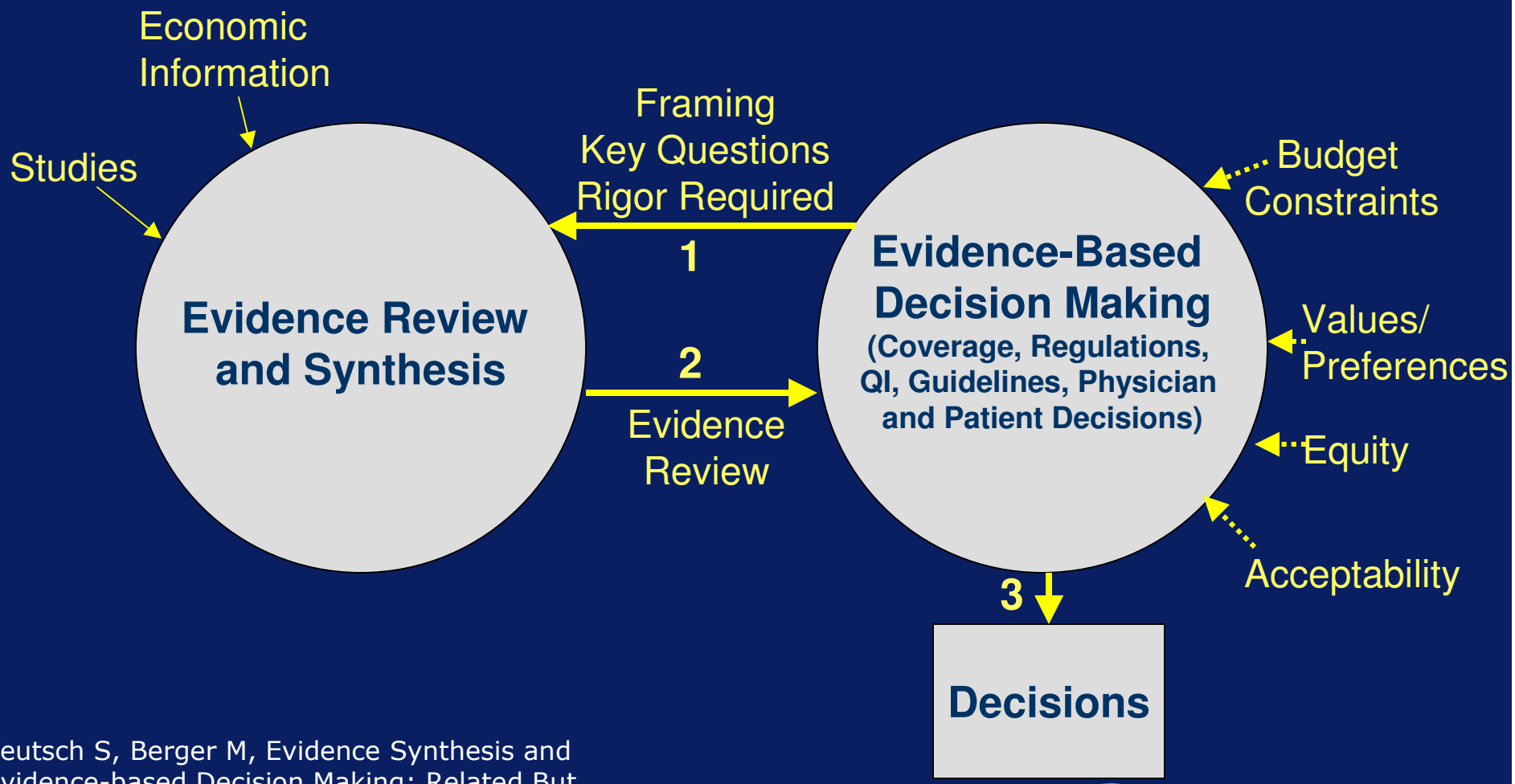
Uses

- Regulation
- Coverage
- Guidelines
- Quality Improvement Metrics
  - Pay-for-Performance
- Individual Decisions (Clinicians, Patients)

Information for Decision Making

- Quantitative
- Contextual

Dynamic Relationship Between Evidence Review & Synthesis and Evidence-based Decision Making



Teutsch S, Berger M, Evidence Synthesis and Evidence-based Decision Making: Related But Distinct Processes.

Medical Decision Making Sep-Oct 2005, pp. 487-489.

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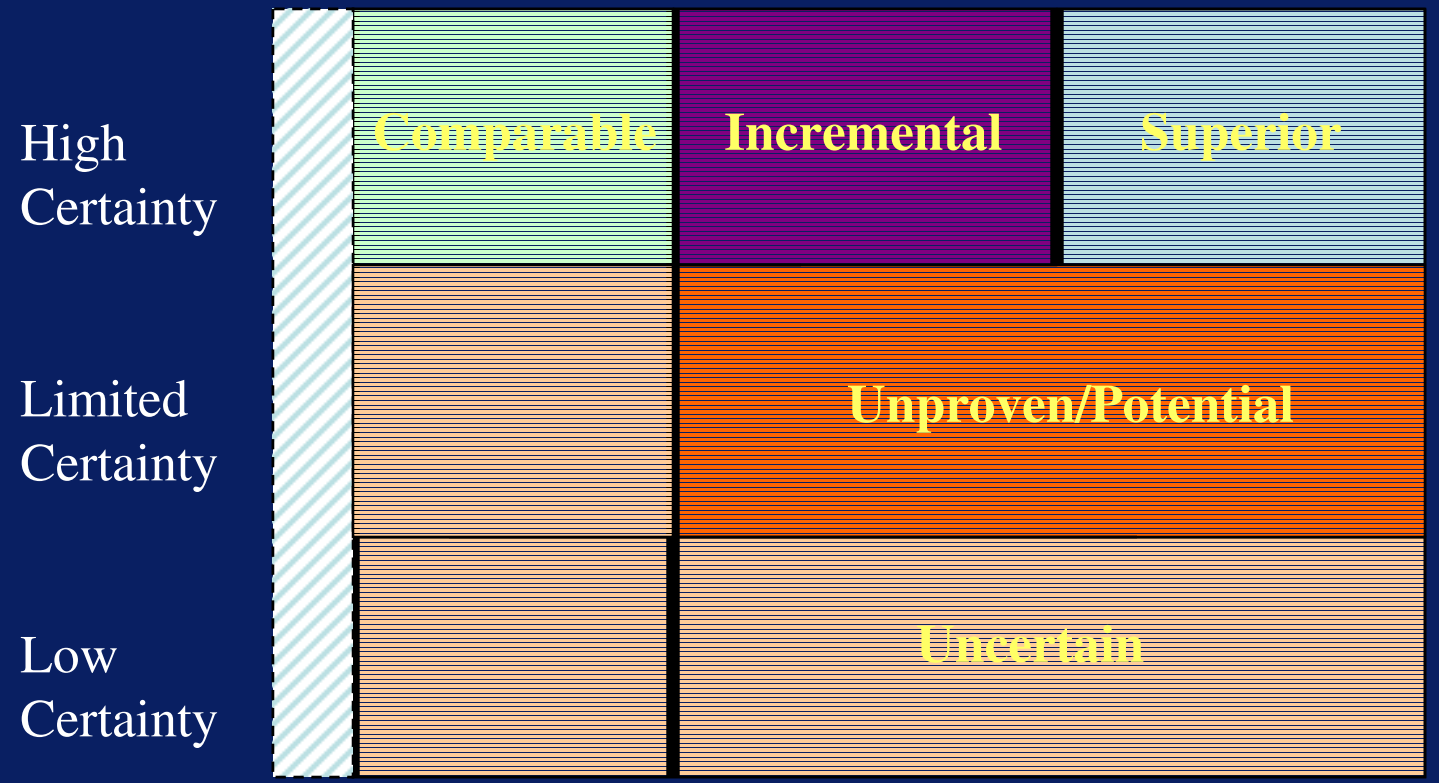
Quantitative Information for Decision Making

- Effectiveness
  - Certainty
  - Magnitude of effect (Net benefit)
- Cost / Cost Effectiveness
- Comparison to alternatives

EBM Roadmap Group: Comparative Clinical Effectiveness



Courtesy Steve Pearson and AHIP
Under development, draft Jan 2007

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Key Effectiveness Questions

- Efficacy: Can it work in controlled conditions?
- Safety: What are the possible harms?
- Effectiveness: Does it work in practice?
- Comparative effectiveness: Does it work better than alternatives?
- Clinical: Are there specific groups for whom it works better?
- Trade-offs: What is the balance of harms and benefits?

Proposed Evidentiary Standards for Clinical Recommendations as a Function of Treatment Goals and Acceptable Regret

(Adapted from Djulbegovic B, JCO, 2005)

Goals of Treatment	Certainty	Benefit-Harm Ratio	Regret	Evidentiary Standards	Examples
Prevention healthy individuals	High	Important trade-offs between the benefits and harms	High	Highest standard of experimental	Tamoxifen to prevent breast cancer
Cure	Low	Belief that intervention does more good than harm	Low	May accept lower level of evidence	Surgery of isolated liver metastasis of colorectal CA
Increased years of survival	Low	Belief that intervention does more good than harm	Low	May accept lower level of evidence	Imatinib in chronic myeloid leukemia;
Increased days to months of survival	High	Important trade-offs between the benefits and harms, or it is not clear	High	Highest standard of experimental evidence	Chemotherapy in metastatic lung cancer
Palliation (improvement of quality of life)	Low/ moderate	Belief that intervention does more good than harm	Low	May accept lower quality of evidence if costs are low	Morphine for pain control
Palliation (improvement of quality of life)	High	Uncertain whether the intervention does more good than harm	Moderate	High-quality particularly if costs are high	Bisphosphonates ; erythropoietin

Categories Of Genetic Test Applications And Some Characteristics Of How Clinical Validity And Utility Are Assessed

Application	Clinical Validity	Clinical Utility
Diagnosis	Association with disorder	Improved clinical outcomes Usefulness for decision-making End of diagnostic odyssey
Disease screening	Association with disorder	Improved health outcome Usefulness for decision making
Risk assessment/ Susceptibility	Association with future disorder	Improved health outcomes
Prognosis of diagnosed disease	Association with natural history	Improved health outcomes, or outcomes of value to patients, based on changes in patient management
Predicting treatment response	Association with a state that relates to drug efficacy or ADEs	Improved health outcomes or adherence based on drug selection or dosage

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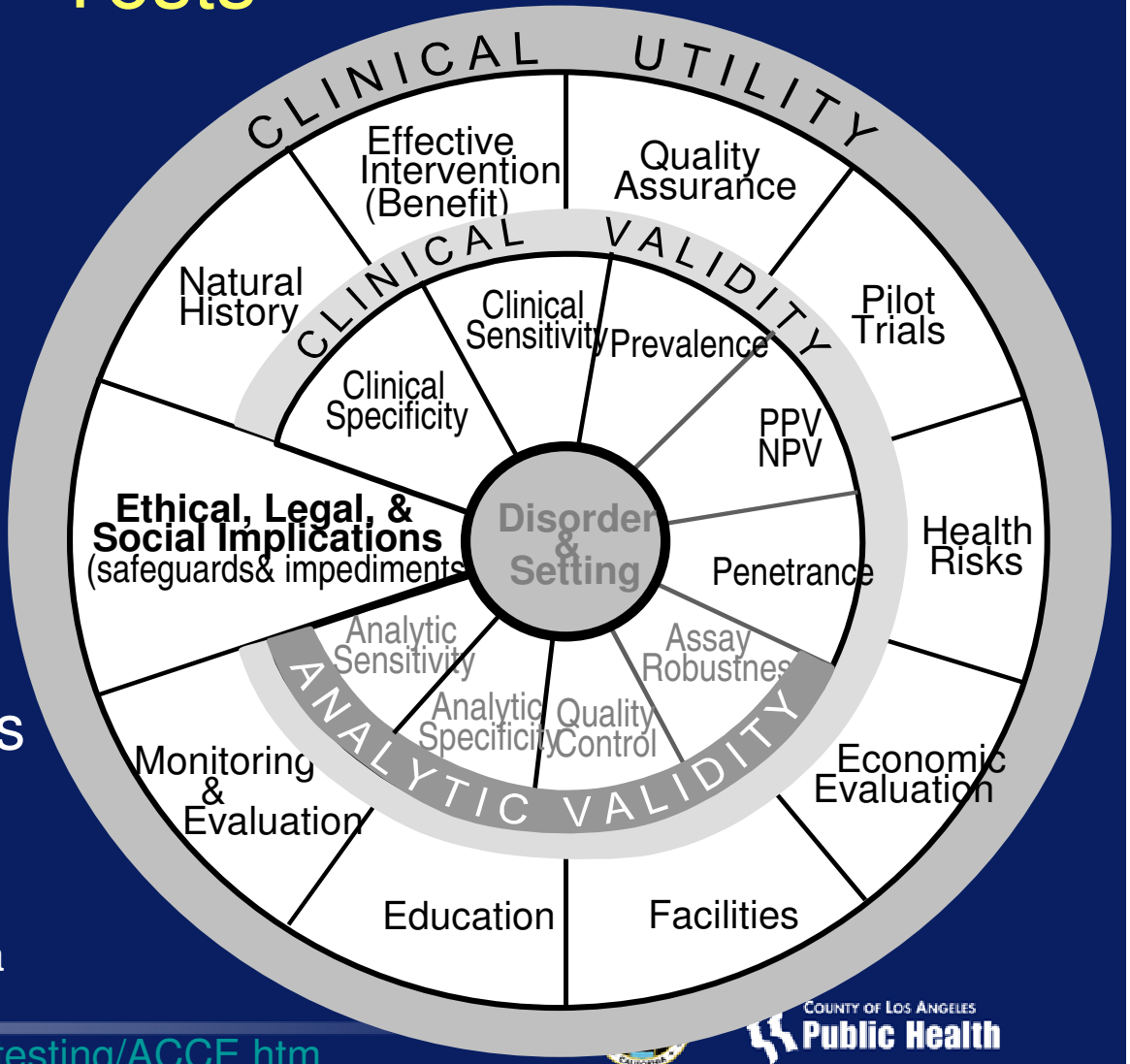
Source: EGAPP

A Multidisciplinary Evaluation of Genetic Tests

- ACCE
- Name reflects four components of evaluation
- Define test, disorder, and setting
- Analytic framework – 40+ targeted questions



Haddow JE, Palomaki GE: ACCE: A Model Process for Evaluating Data on Emerging Genetic Tests, 2003. http://www.cdc.gov/genomics/gtesting/ACCE.htm.

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Hierarchies of Data Sources and Study Designs for the Components of Evaluation3

Level	Analytic Validity	Clinical Validity	Clinical Utility
1	Collaborative study Summary data from well-designed external proficiency testing	Well designed longitudinal cohort studies Validated clinical decision rule	Meta-analysis of RCTs
2	Other proficiency testing Well designed peer-reviewed studies Expert panel reviewed FDA summaries	Well designed case-control studies	A single RCT
3	Less well designed peer-reviewed studies	Lower quality case-control and cross-sectional studies Unvalidated clinical decision rule	Controlled trial without randomization Cohort or case-control study
4	Other research, clinical laboratory or manufacturer data Studies on performance of the same basic methodology,	Case series Other research, clinical laboratory or manufacturer data Consensus guidelines Expert opinion	Case series Other studies, clinical laboratory or manufacturer data Consensus guidelines Expert opinion

Contextual Information for Decision Making (1)

- Clinical
  - Severity of the condition
  - Subgroup differences/generalizability
  - Availability of alternative treatments
  - Severity and frequency of harms
  - Risks of overuse or inappropriate use
- Economic
  - Budget impact
  - Budget constraints
  - Value

Contextual Information for Decision Making (2)

- Legal and Ethical considerations
  - Precedent
  - Federal and state regulatory constraints
  - Regret
- Feasibility
  - Current use
  - Infrastructure requirements
  - Acceptability: Partner and Stakeholder Interests
- Preferences / values
- Administrative
  - Options for targeting or limiting use to patients who would benefit most
  - Links to further evidence development

Process Considerations to Ensure Fairness & Reasonableness

- Clear “rules of the road”
- Deliberative process
- Transparency
- Appeals

Decision Factor Matrix

	Regulation	Coverage	Guidelines	QI	Individual Decisions
Efficacy					
Safety					
Effectiveness					
Comparative Effectiveness					
Cost/ CE					
Clinical Sit					
Legal/ Ethical					
Values/ Prefs					
Admin.					
Feasibility					
Stakeholders					

Decision Factor Matrix
(Straw Man for Discussion Only)

	Regulation	Coverage	Guidelines	QI	Individual Decisions
Efficacy					
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Values/ Prefs					
Admin.					
Feasibility					
Stakeholders					

Refining the Approach to Standards of Evidence

- Rethinking the hierarchy with new forms of evidence
- Aligning research efforts with application needs
- Accommodating the evolving role of observational data
- Decision-making when the evidence is insufficient