Clinical Utility and Comparative Effectiveness Research

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SACGHS Clinical Utility and Comparative Effectiveness Research Task Force

SACGHS Members

- **Marc Williams (Chair)**
- Sylvia Au
- Paul Billings
- Gwen Darien
- Andrea Ferreira-Gonzalez
- Sheila Walcoff
- Paul Wise

Agency Experts

- Scott Bowen, CDC
- Denise Geolot, HRSA
- Elizabeth Mansfield, FDA
- Michael Lauer, NHLBI
- Gurvaneet Randhawa, AHRQ

Task Force Charge

- To determine which issues, if any, SACGHS should explore in the areas of clinical utility (CU) and comparative effectiveness research (CER)

- Immediate focus of the task force was assessing federal funding of CER projects that concern genetics and genomics

Federal Funding for CER

- American Recovery and Reinvestment Act of 2009 (ARRA) appropriated $400 million to the NIH, $300 million to AHRQ, and $400 million to the Office of the Secretary, HHS, for CER

- $400 million for Secretary must be used either to "conduct, support, or synthesize" CER or to "encourage the development and use of clinical registries, clinical data networks, and other forms of electronic health data that can be used to generate or obtain outcomes data."

Funding for CER (continued)

- ARRA also required Secretary to task the Institute of Medicine (IOM) with report recommending national priorities for CER funds appropriated to Secretary

- ARRA requires Secretary to consider IOM recommendations as well as the recommendations of the Federal Coordinating Council for Comparative Effectiveness Research (FCCCER) in spending $400 million appropriated to Office of the Secretary

Task Force Strategy

- Review recommendations from IOM and FCCCER and identify those relating to genetics and genomics,
- assess the degree to which projects NIH and ARHQ support with their CER funds satisfy any IOM and FCCCER recommendations relating to genetics and genomics,
- and identify those recommended studies or projects relating to genetics and genomics that are not yet funded.
- SACGHS could then recommend that the Office of the Secretary fund those genetic-or genomics-related projects that were recommended by either IOM or FCCCER but not funded by NIH or AHRQ

Federal Coordinating Council for CER

- FCCCER is composed of senior Federal officials, most of whom are physicians, with responsibility for health-related programs

- FCCCER issued a report on June 30, 2009 that recognizes that CER can promote personalized medicine by examining the effectiveness of interventions by patient subgroup
  - Lewin report is specifically focused on how CER and personalized medicine complement one another

FCCCER Report

- Recommends that the primary investment of the Secretary's funds be in creating data infrastructure for CER (e.g., patient registries)

- Recommends secondary, but significant investments for dissemination and translation of CER, CER studies focused on priority populations, and CER studies focused on priority types of interventions
  - Priority populations identified include racial and ethnic minorities, persons with disabilities, persons with multiple chronic conditions, the elderly, and children
  - CER studies on priority types of interventions could involve comparing different medical home models or comparing surgery versus medical management

FCCCER Report (continued)

- Report notes, “As the Secretary develops HHS’s full portfolio of ARRA investments, it will be critical to consider both CER and health IT holistically . . . .”
  - As such, SACGHS may want to continue to encourage health IT policy that supports collection of genetic information useful for CER
  - In addition, barriers to genomic data sharing are also barriers to CER

IOM Report

- Report released on June 30, 2009
- Generated 100 prioritized research topics and 10 recommendations
- Prioritized research topics that explicitly mention genetics or genomics:
  - First quartile priority
    - to compare the effectiveness of genetic and biomarker testing with usual care in preventing and treating breast, colorectal, prostate, lung, and ovarian cancer
  - Third quartile priority
    - to compare the effectiveness of biomarker information (including genetic information) with standard care in motivating behavior change and improving clinical outcomes
- 8 other prioritized research topics could conceivably include genetics/genomics within scope, but not explicitly mentioned

NIH Analysis of IOM Report

- NIH reviewed all 100 of the recommended study topics
  - most of the 100 IOM study topics are already being studied through ongoing NIH research projects
  - Review by task force identified numerous funded projects in genetics/personalized medicine space

IOM Report

- Recommendations of particular relevance to SACGHS:
  - #7: HHS should devote sufficient resources to research and innovation in the methods of CER
    - Innovation in methods for CU studies is also needed
  - #8: HHS should help develop large-scale, clinical and administrative data networks for use in CER
    - Goal raises privacy and informed consent issues that likely overlap with issues raised by genomic data sharing
    - Reflects ongoing efforts to create such data networks (CA-BIG, NCBI efforts)
    - Recommendation implies need to collect clinic level data. How does meaningful use relate to this issue?

Analysis of NIH ARRA-funded CER Grants

- Several funded projects will fulfill particular CER studies relating to genetics that IOM recommended:
  - 24 specifically funded under CER
  - See details Tab #4
- Many other (50-100s) address genomic/personalized medicine issues not directly related to IOM top 100
  - Good coverage across a range of conditions
  - Some use CER methods even if not funded by CER funds
- Many funded projects also would serve as investments in data infrastructure and in dissemination and translation of CER findings, consistent with FCCCER's recommendations

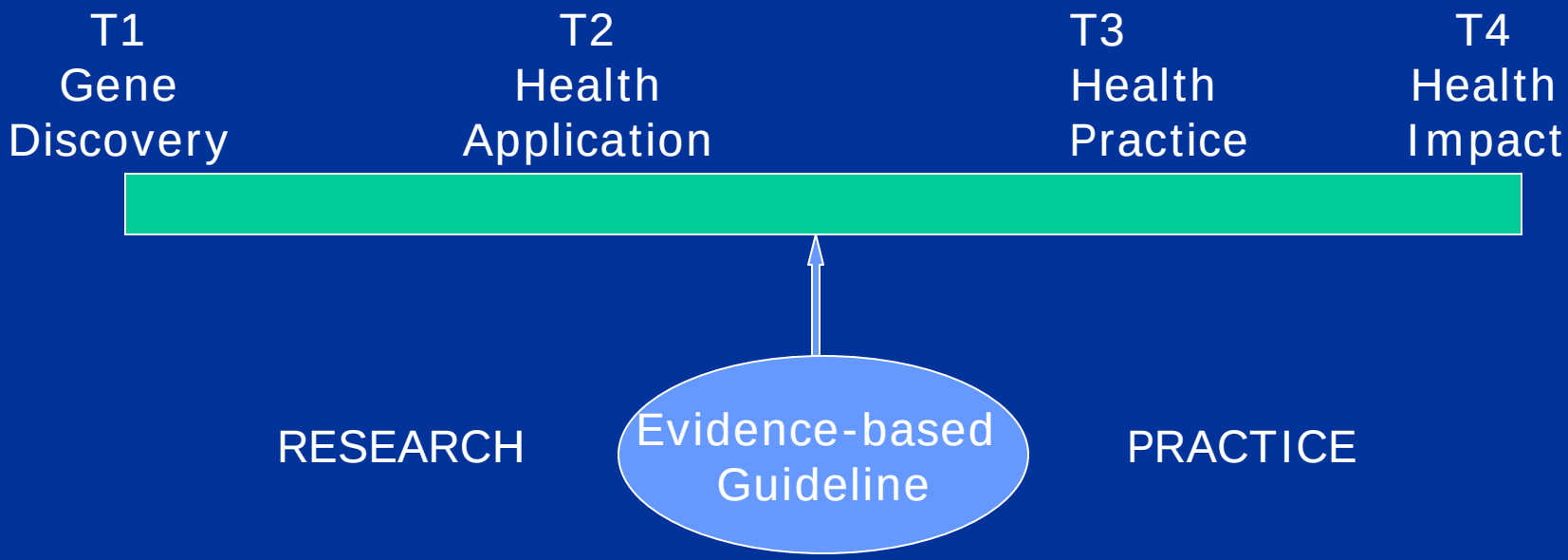
Analysis of AHRQ ARRA-funded CER Grants

- Grants:
  - a) CHOICE and iADAPT announcements are closed. Rough estimate of applicants – 118 and 91, respectively (final # subject to change). Titles indicate only a small proportion will have a focus on genomics but detailed reading of the applications may reveal otherwise.
  - b) PROSPECT and EDM announcements still open.
- Based on titles ~10% may have something to do with genomics
- For all of these grants, reviews, funding decisions, and awards will be done before close of fiscal year 2010 (i.e., end of September)

Gaps

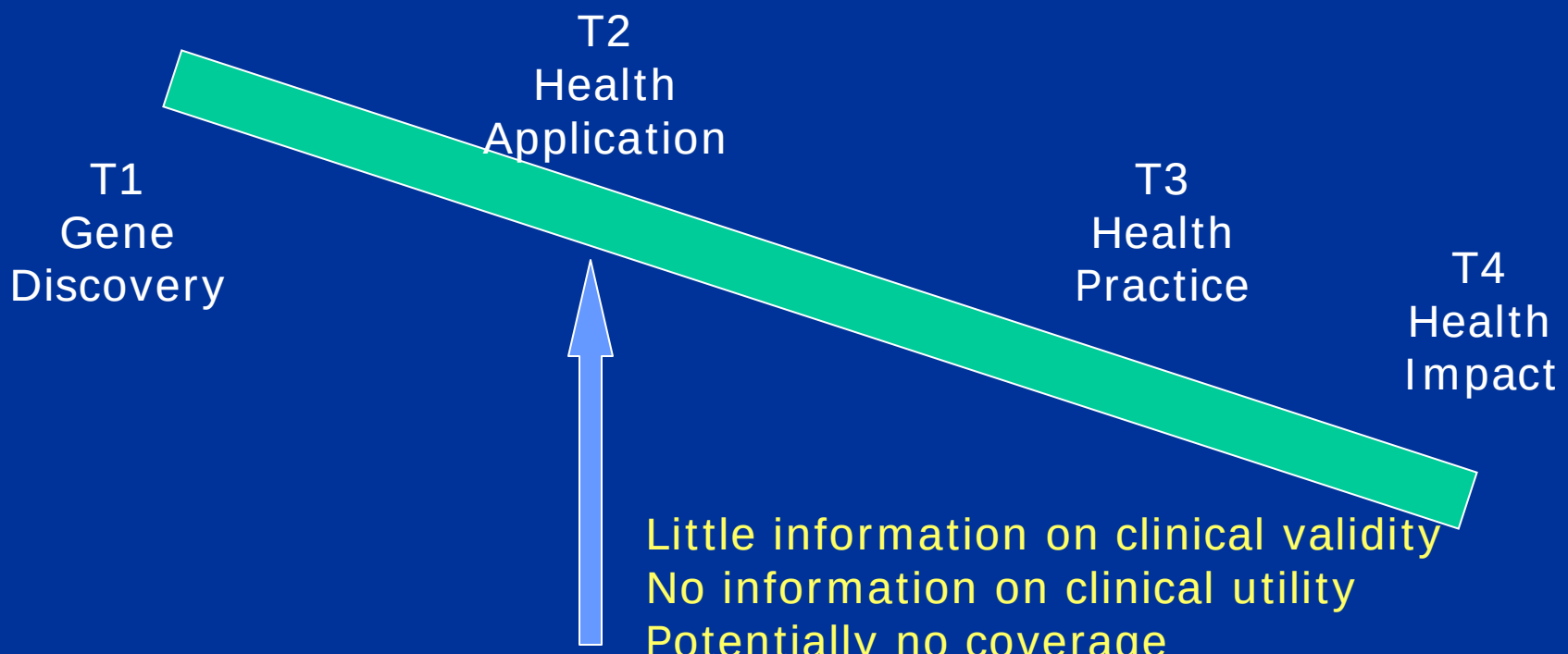
- Definition of adequate evidentiary standards for different applications
- Third quartile IOM priority Healthcare Delivery System-I
- Coordination of efforts

The Translational Process



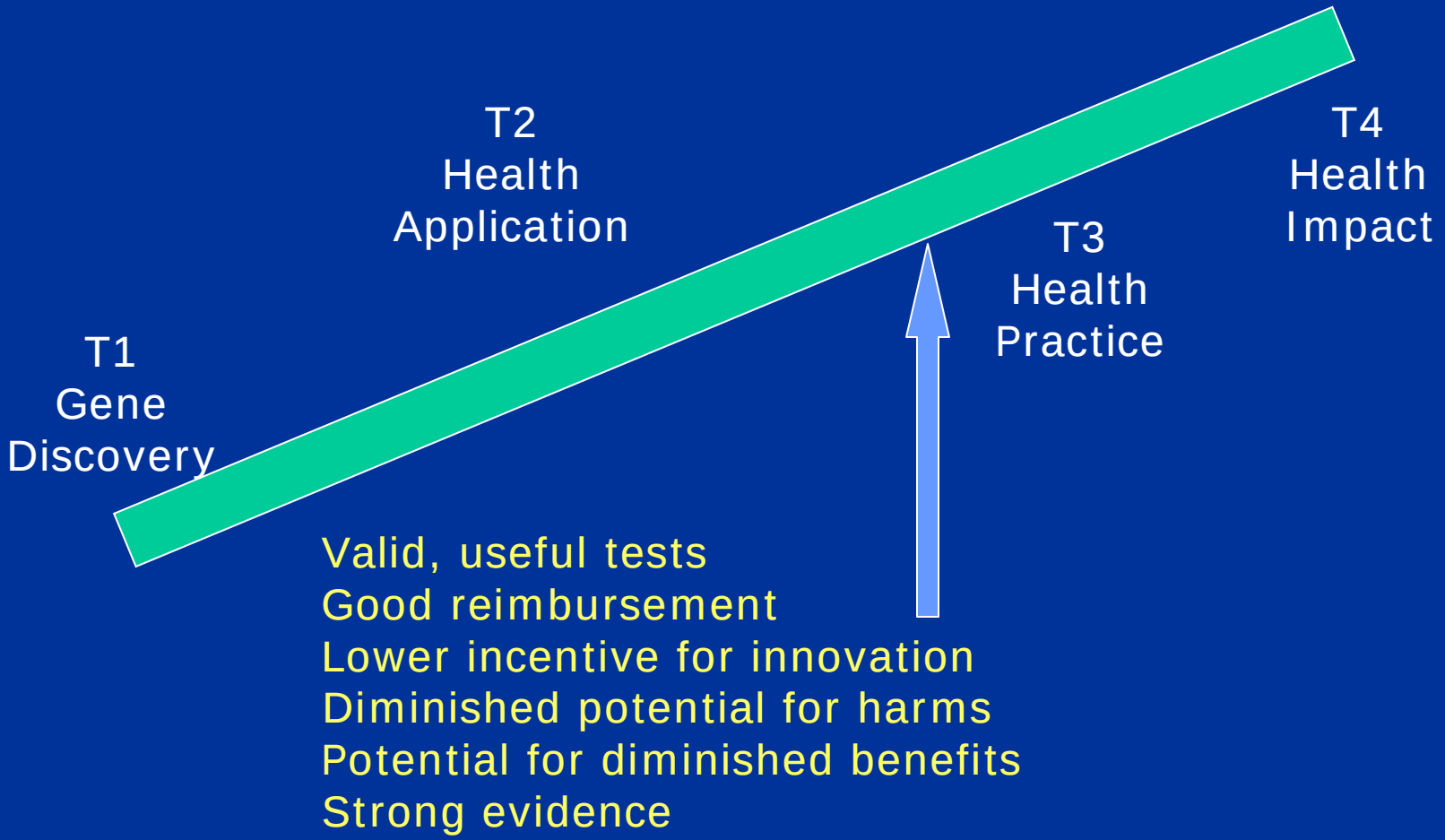
How High Should the Evidence Bar Be?

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

Raising the Evidentiary Threshold for Translation into Practice



Decision Factor Matrix

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

Decision Factor Matrix
(Straw Man for Discussion Only)

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IOM Priorities Quartile 3

- To compare the effectiveness of biomarker information (including genetic information) with standard care in motivating behavior change and improving clinical outcomes
  - Few projects funded specifically address these critical utility issues
  - Possibly more in AHRQ
  - Role for the Secretary?

Coordination of Activities

- Standardized data representation and storage
- Opportunities to share findings across projects
  - Impact of genomics in a condition (i.e. Psoriasis) with associate risks for another condition (i.e. CAD)
- Role of Secretary?

CER Funding by HHS Secretary

- Secretary's funding decisions unknown at this time
  - Secretary was required to send operating plans to Congress in July and November 2009 concerning funding decisions by OS, NIH, and AHRQ for fiscal years 2009 and 2010
  - report not publicly available

Potential Legislation Concerning CER

- Health care reform bills passed in House and Senate in late 2009 call for more CER funding
  - Both House and Senate bill indicate that studies should take into account “genetic and molecular subtypes”
  - Status now unclear

Potential Next Steps for Task Force

- Establish evidentiary standards for use of genomic tests
  - Outline considerations for adjusting evidentiary bar
  - Identify other entities (e.g. GAPPnet) who are addressing this issue
- Creation of inventory of genomic CER projects with identification and prioritization of gaps in genomic CER agenda
  - Letter to Secretary with suggestions of how to spend ARRA CER monies to address gaps
  - Special attention to HCDS-I
- Workshop for June meeting
- Monitor health IT issues through this task force or genomic data sharing group
  - Meaningful use rules
- Dissolve task force
- Other