The National Comparative Effectiveness Summit

Welcome and Overview: The Role of Government in CER

October 13, 2011
Washington, DC

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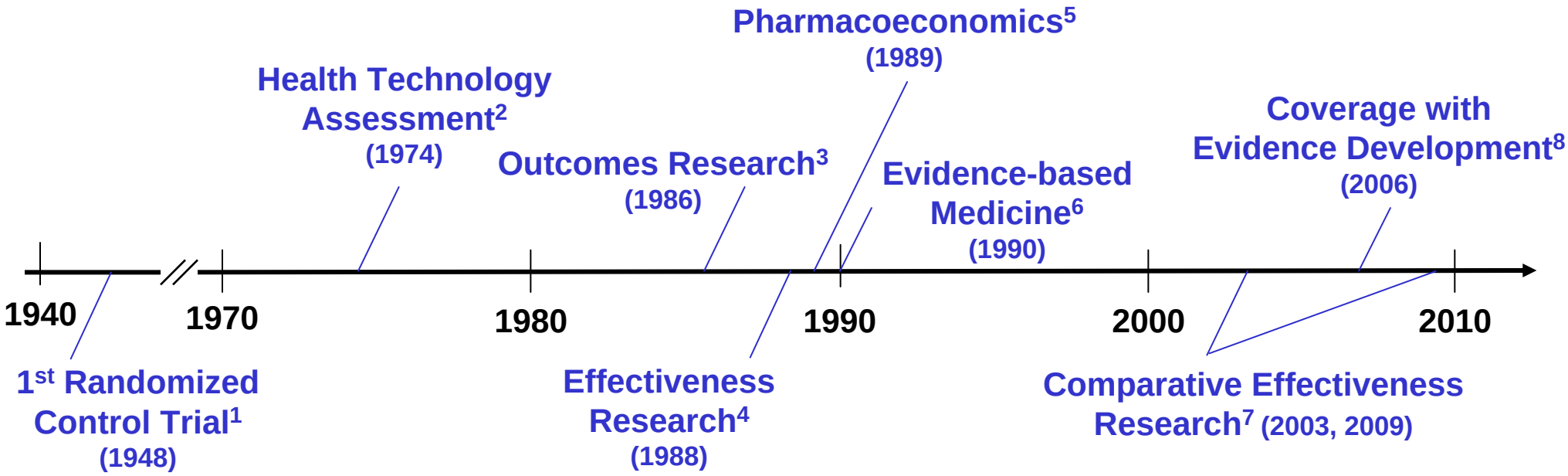
Center for Comparative Effectiveness Research

Getting to CER: Evolved from Attributes of Other Forms of Inquiry



[1] RCT of streptomycin for pulmonary tuberculosis, sponsored by Medical Research Council (UK): 1948.

[2] Origin of TA (not focused on health) in 1965: US Congressman Daddario; first "experimental" HTA by National Academy of Engineering in 1969 (multiphasic screening); Office of Technology Assessment published first HTA in 1974

[3] Patient Outcomes Assessment Research Program (later, PORTs) initiated by NCHSR (later renamed AHCPR; now AHRQ) in 1986 ("promote research with respect to patient outcomes of selected medical treatments and surgical procedures for the purpose of assessing their appropriateness, necessity and effectiveness ")

[4] HCFA (later renamed CMS) Effectiveness Initiative: 1988

[5] Early published appearance of "pharmacoeconomics": Bootman et al. 1989

[6] "Evidence-based": Eddy 1990; "Evidence-based medicine": Guyatt et al. 1992

[7] Medicare Prescription Drug, Improvement, and Modernization Act of 2003 (MMA) specifies AHRQ role in "comparative clinical effectiveness"; American Recovery and Reinvestment Act of 2009 (ARRA) authorizes major national investment in CER

[8] CMS draft guidance in 2005; formalized in 2006. Medicare and other payers began linking coverage to clinical research in 1990s

CER in the American Recovery and Reinvestment Act of 2009 (ARRA)

- **Provided $1.1 billion, to be obligated by Sept. 30, 2010**

  $300 M - Agency for Healthcare Research and Quality

  $400 M - National Institutes of Health

  $400 M - Secretary of Health and Human Services

- **Designated two groups to provide recommendations on national CER priorities and other advice by June 30, 2009:**
  - Federal Coordinating Council for CER: strengthen national capacity for CER
  - Institute of Medicine: 100 CER priorities

FEDERAL COORDINATING COUNCIL FOR
COMPARATIVE EFFECTIVENESS RESEARCH

REPORT TO
THE PRESIDENT
AND
THE CONGRESS

JUNE 30, 2009

US DEPARTMENT OF HEALTH AND HUMAN SERVICES

FCCCER Members

Appendix D. COUNCIL LIST AND STAFF SUPPORT

1. Carolyn Clancy, MD	AHRQ
2. Peter Delaney, PhD, LCSW-C	SAMHSA
3. Ezekiel Emanuel, MD, PhD	OMB
4. Jesse Goodman, MD, MPH	FDA
5. Garth Graham, MD, MPH	Office of Minority Health
6. Anne Haddix, PhD	CDC
7. Deborah Hopson, PhD, RN	HRSA
8. David Hunt, MD	ONC
9. Michael Kilpatrick, MD	Dept of Defense
10. Joel Kupersmith, MD	Dept of VA
11. Michael Marge, Ed.D.	Office of Disability
12. Elizabeth Nabel, MD	NIH
13. James Scanlon, PhD	ASPE
14. Neera Tanden, JD	Office of the Secretary
15. Tom Valuck, MD, MHSA, JD	CMS

Executive Director: Patrick Conway, MD, MSc
Deputy Executive Director: Cecilia Rivera Casale, PhD

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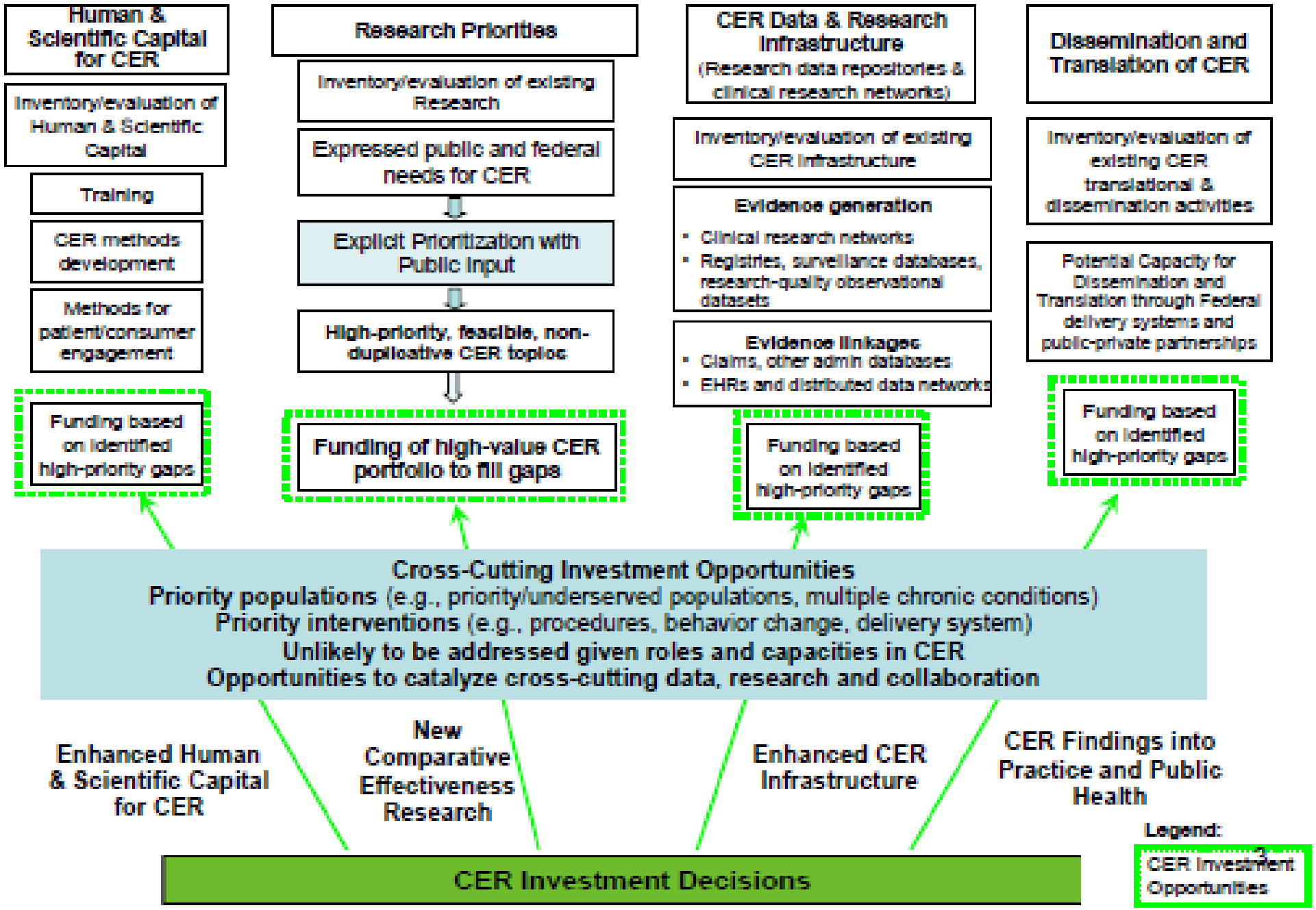
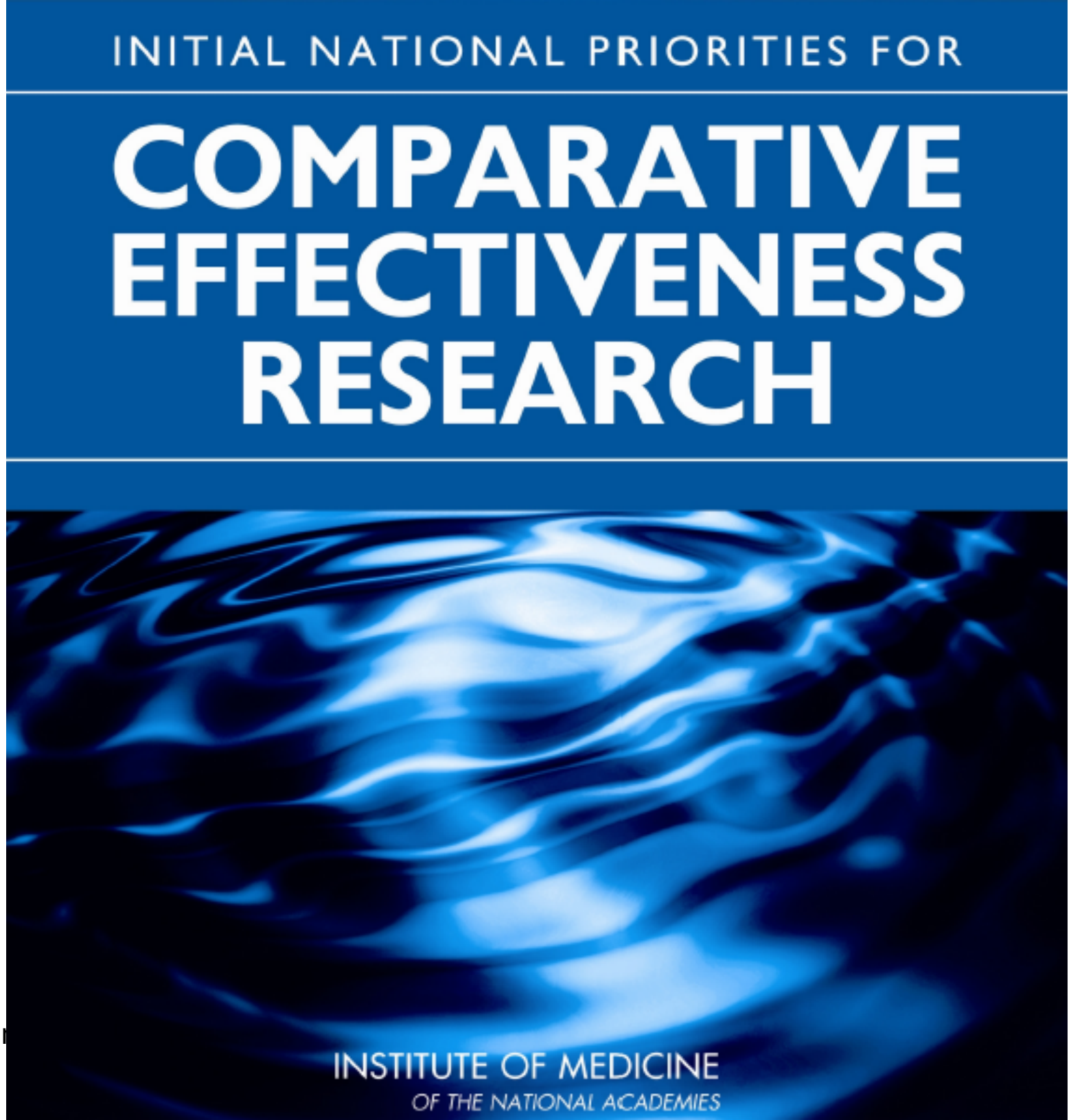
Using the CER Strategic Framework for Inventory and Investment Decisions



Source: FCCCER. Report to The President and The Congress. June 2009.


INITIAL NATIONAL PRIORITIES FOR
COMPARATIVE EFFECTIVENESS RESEARCH
INSTITUTE OF MEDICINE
OF THE NATIONAL ACADEMIES

LIST OF PRIORITY CER TOPICS

TABLE S-1 Final List of Priority Topics, by Quartile Ratings
**display within quartile does not indicate priority rank—topics are listed alphabetically by primary research area*

First Quartile (listed alphabetically by primary research area)	
CAD	Compare the effectiveness of treatment strategies for atrial fibrillation including surgery, catheter ablation, and pharmacologic treatment.
DIS	Compare the effectiveness of the different treatments (e.g., assistive listening devices, cochlear implants, electric-acoustic devices, habilitation and rehabilitation methods [auditory/oral, sign language, and total communication]) for hearing loss in children and adults, especially individuals with diverse cultural, language, medical, and developmental backgrounds.
ENDO	Compare the effectiveness of primary prevention methods, such as exercise and balance training, versus clinical treatments in preventing falls in older adults at varying degrees of risk.
GI	Compare the effectiveness of upper endoscopy utilization and frequency for patients with gastroesophageal reflux disease on morbidity, quality of life, and diagnosis of esophageal adenocarcinoma.
HCDS	Compare the effectiveness of dissemination and translation techniques to facilitate the use of CER by patients, clinicians, payers, and others.
HCDS	Compare the effectiveness of comprehensive care coordination programs, such as the medical home, and usual care in managing children and adults with severe chronic disease, especially in populations with known health disparities.

Main Attributes of CER

- Direct ("head-to-head") comparisons of alternative interventions (rather than comparison with placebo or indirect comparisons)
- Applies to all types of technologies/interventions
- Effectiveness (in realistic health care settings) rather than efficacy (in ideal circumstances)
- Emphasizes health care outcomes (e.g., morbidity, mortality, symptoms, quality of life (QoL), adverse events) rather than intermediate/surrogate endpoints
- Draws on variety of complementary methods, tools
- Enables subgroup analyses to yield findings for about different responses in particular patient groups
- Emphasis on priority diseases and priority populations
- No (US) consensus on role of economics; value for money

CER Methods "Toolkit" (Evolving)

Clinical Trials

- Randomized clinical trials
- Practical (pragmatic) clinical trials
- Other non-randomized controlled trials
- Adaptive clinical trials and other trial designs
- Other, e.g., randomized consent, regression discontinuity, combined single-subject ("n of 1") trials

Observational Studies (prospective or retrospective)

- Population-based longitudinal cohort studies
- Patient registries
- Claims databases
- Clinical data networks
- Electronic health record data analyses
- Post-marketing surveillance (passive and active)

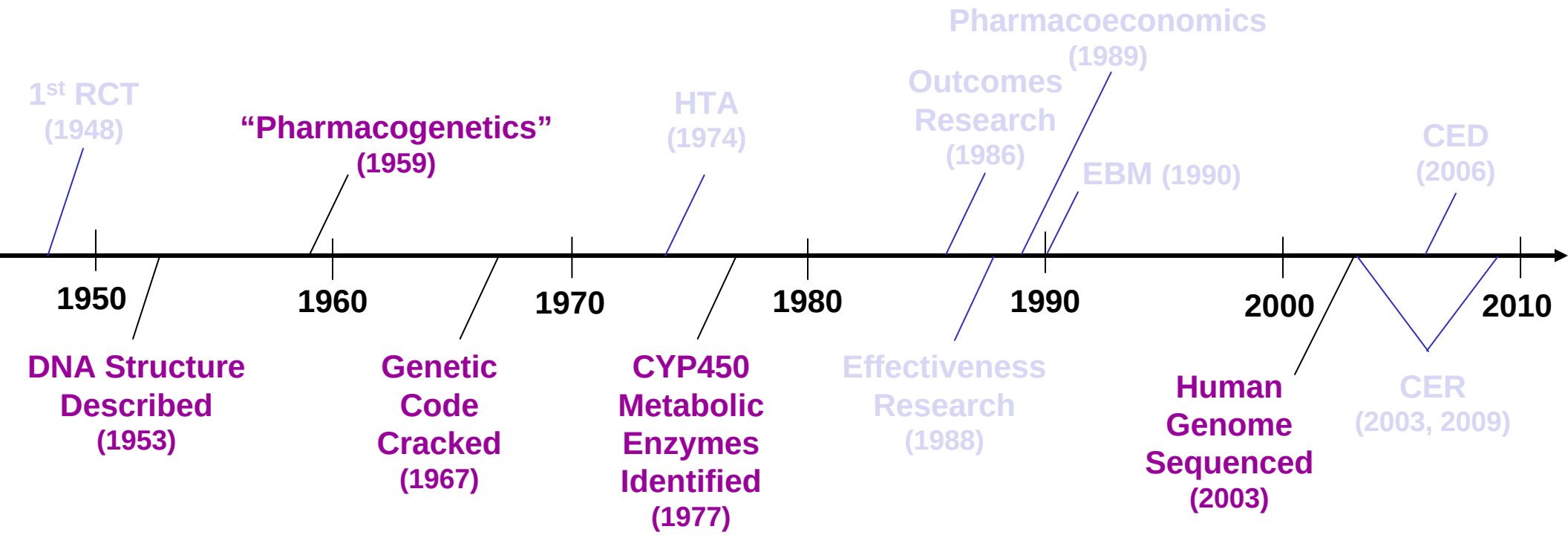
Syntheses of Existing Evidence

- Systematic reviews (comparative effectiveness reviews)
- Meta-analyses
- Modeling

What is Personalized Medicine?

- Personalized medicine (PM) is the tailoring of medical care to the particular traits (or circumstances or other characteristics) of a patient that influence response to a heath care intervention. These may include genetic, sociodemographic, clinical, behavioral, environmental, and other personal traits, as well as personal preferences. PM does not refer to the creation of interventions that are unique to a patient, but the ability to classify patients into subpopulations that differ in their responses to particular interventions.

Getting to Personalized Medicine



FCCCER on PM

- ***"In addition, comparative effectiveness should complement the trend in medicine to develop personalized medicine—the ability to customize a drug and dose based on individual patient and disease characteristics. One of the advantages of large comparative effectiveness studies is the power to investigate effects at the sub-group level that often cannot be determined in a randomized trial. This power needs to be harnessed so personalized medicine and comparative effectiveness complement each other."***

Source: Federal Coordinating Council for Comparative Effectiveness Research. Report to the President and Congress. U.S. Department of Health and Human Services, June 30, 2009.

Patient-Centered Outcomes Research Institute

- **Established by Patient Protection and Affordable Care Act**
- **Private, non-profit organization that is not "an agency or establishment of the U.S. Government."**
- **Identify research priorities; establish, implement research agenda**
- **21-member Board of Governors, including Directors of AHRQ and NIH; 19 members appointed (9-23-10) by Comptroller General**
- **Funded through combination of appropriations + transfers from the Medicare Federal Hospital Insurance and Federal Supplementary Medical Insurance Trust Funds, and transfers from health insurance and self-insured health plans**
- **Limitations on PCORI's and the Secretary's ability to use PCORI research findings for coverage and reimbursement**
  - Cannot "mandate coverage, reimbursement, or other policies for any public or private payer"
  - Government may use findings in coverage "if such use is through an iterative and transparent process which includes public comment and considers the effect on subpopulations," subject to other constraints

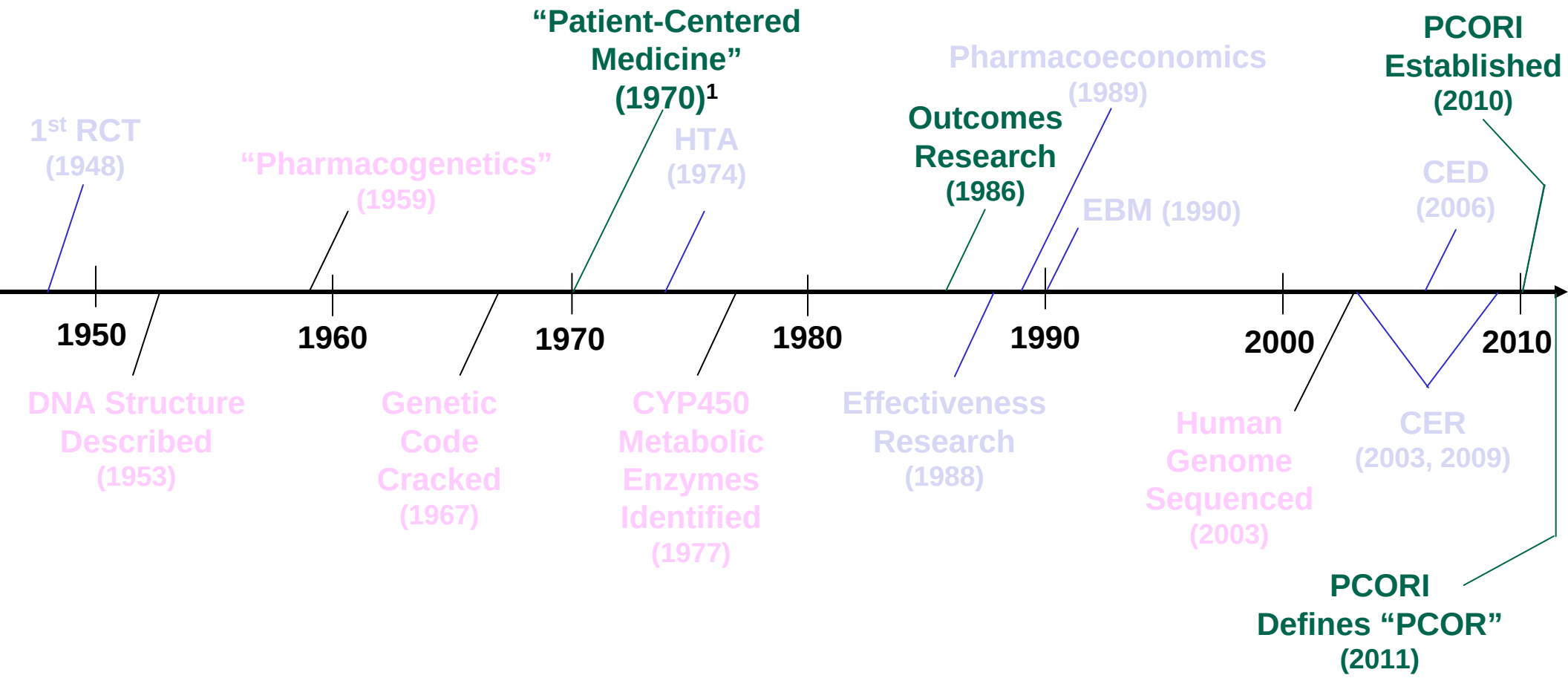
What Is Patient-Centered Outcomes Research?

Patient-centered outcomes research (PCOR) helps people make informed health care decisions and allows their voice to be heard in assessing the value of health care options. This research answers patient-focused questions:

- Given my personal characteristics, conditions and preferences, what should I expect will happen to me?
- What are my options and what are the benefits and harms of those options?
- What can I do to improve the outcomes that are most important to me?
- How can the health care system improve my chances of achieving the outcomes I prefer?

Source: Patient-Centered Outcomes Research Institute. Working definition of patient-centered outcomes research. July 2011.

Getting to PCOR



[1] Balint M, Hunt J, Joyce D, et al. Treatment or Diagnosis: A Study of Repeat Prescriptions in General Practice. Philadelphia, PA: JB Lippincott; 1970.

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