Comparative Effectiveness Research: The Role of the National Institutes of Health

Science in Pursuit of Knowledge to Improve Health

Michael S Lauer, MD
NHLBI
June 12, 2009

NIH and Comparative Effectiveness Research (CER)

I. NIH's Support for Landmark CER Research

II. The Many Definitions of CER

III. ARRA and CER

IV. The NIH CER Coordinating Committee

V. ARRA and CER: Opportunities and Challenges

Which Treatment is Best for Whom? High-Quality Evidence is Scarce

ORIGINAL CONTRIBUTION

Scientific Evidence Underlying the ACC/AHA Clinical Practice Guidelines

Pierluigi Tricoci, MD, MHS, PhD
Joseph M. Allen, MA
Judith M. Kramer, MD, MS
Robert M. Califf, MD
Sidney C. Smith Jr, MD

CLINICAL PRACTICE GUIDElines are systematically developed statements to assist practitioners with decisions about appropriate health care for spe-

Context The joint cardiovascular practice guidelines of the American College of Cardiology (ACC) and the American Heart Association (AHA) have become important documents for guiding cardiology practice and establishing benchmarks for quality of care.

Objective To describe the evolution of recommendations in ACC/AHA cardiovascular guidelines and the distribution of recommendations across classes of recommendations and levels of evidence.

Data Sources and Study Selection Data from all ACC/AHA practice guidelines issued from 1984 to September 2008 were abstracted by personnel in the ACC Science and Quality Division. Fifty-three guidelines on 22 topics, including a total of 7196 recommendations, were abstracted.

Tricoci P et al. JAMA 2009;301:831-41

Drug vs. drug

Screening vs. usual care


The NEW ENGLAND JOURNAL of MEDICINE

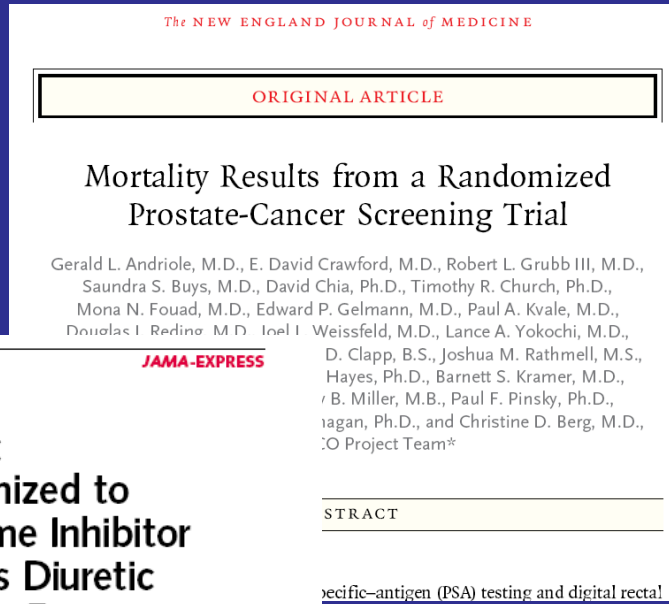
ESTABLISHED IN 1812 SEPTEMBER 22, 2005 VOL. 353 NO. 12

Effectiveness of Antipsychotic Drugs in Patients with Chronic Schizophrenia

Jeffrey A. Lieberman, M.D., T. Scott Stroup, M
Robert A. Rosenheck, M.D., Diana
Sonia M. Davis, Dr.P.H., Clarence E. Da
and John K. Hsiao, M.D., for the Clinical Antips

BACKGROUND

The relative effectiveness of second-generation (aty


The NEW ENGLAND JOURNAL of MEDICINE

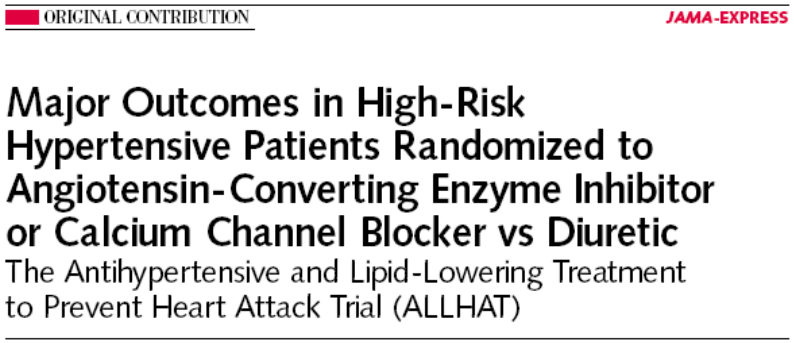
ORIGINAL ARTICLE

Mortality Results from a Randomized Prostate-Cancer Screening Trial

Gerald L. Andriole, M.D., E. David Crawford, M.D., Robert L. Grubb III, M.D.,
Saundra S. Buys, M.D., David Chia, Ph.D., Timothy R. Church, Ph.D.,
Mona N. Fouad, M.D., Edward P. Gelmann, M.D., Paul A. Kvale, M.D.,
Douglas J. Reding, M.D., Joel L. Weissfeld, M.D., Lance A. Yokochi, M.D.,
D. Clapp, B.S., Joshua M. Rathmell, M.S.,
Hayes, Ph.D., Barnett S. Kramer, M.D.,
B. Miller, M.B., Paul F. Pinsky, Ph.D.,
nagan, Ph.D., and Christine D. Berg, M.D.,
:O Project Team*

STRACT

pecific–antigen (PSA) testing and digital rectal


ORIGINAL CONTRIBUTION

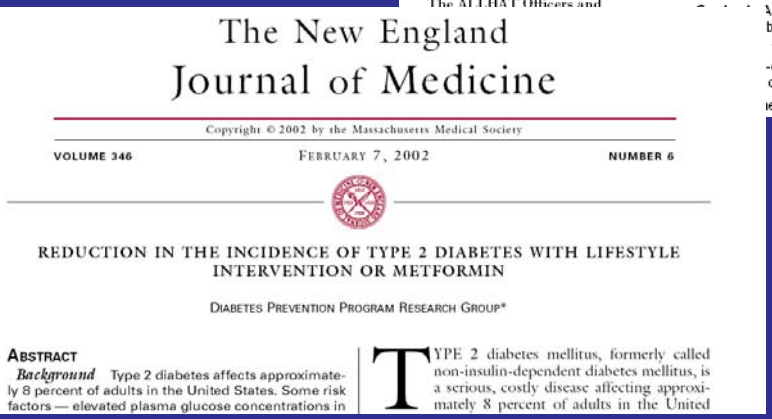
JAMA-EXPRESS

Major Outcomes in High-Risk Hypertensive Patients Randomized to Angiotensin-Converting Enzyme Inhibitor or Calcium Channel Blocker vs Diuretic

The Antihypertensive and Lipid-Lowering Treatment to Prevent Heart Attack Trial (ALLHAT)

The ALLHAT Officers and

Antihypertensive t
bidity and mortality
To determine whe
-converting enzym
or other cardiovasc
e Antihypertensive


The New England Journal of Medicine

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VOLUME 346 FEBRUARY 7, 2002 NUMBER 6

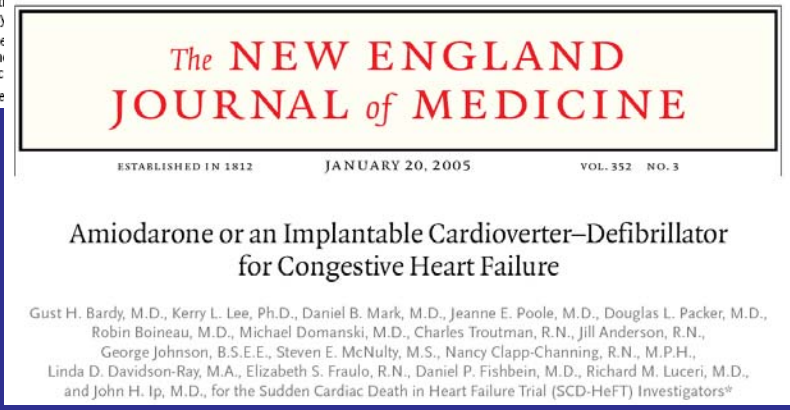
REDUCTION IN THE INCIDENCE OF TYPE 2 DIABETES WITH LIFESTYLE INTERVENTION OR METFORMIN

DIABETES PREVENTION PROGRAM RESEARCH GROUP*

ABSTRACT

Background Type 2 diabetes affects approximately 8 percent of adults in the United States. Some risk factors — elevated plasma glucose concentrations in

TYPE 2 diabetes mellitus, formerly called non-insulin-dependent diabetes mellitus, is a serious, costly disease affecting approximately 8 percent of adults in the United


The NEW ENGLAND JOURNAL of MEDICINE

ESTABLISHED IN 1812 JANUARY 20, 2005 VOL. 352 NO. 3

Amiodarone or an Implantable Cardioverter–Defibrillator for Congestive Heart Failure

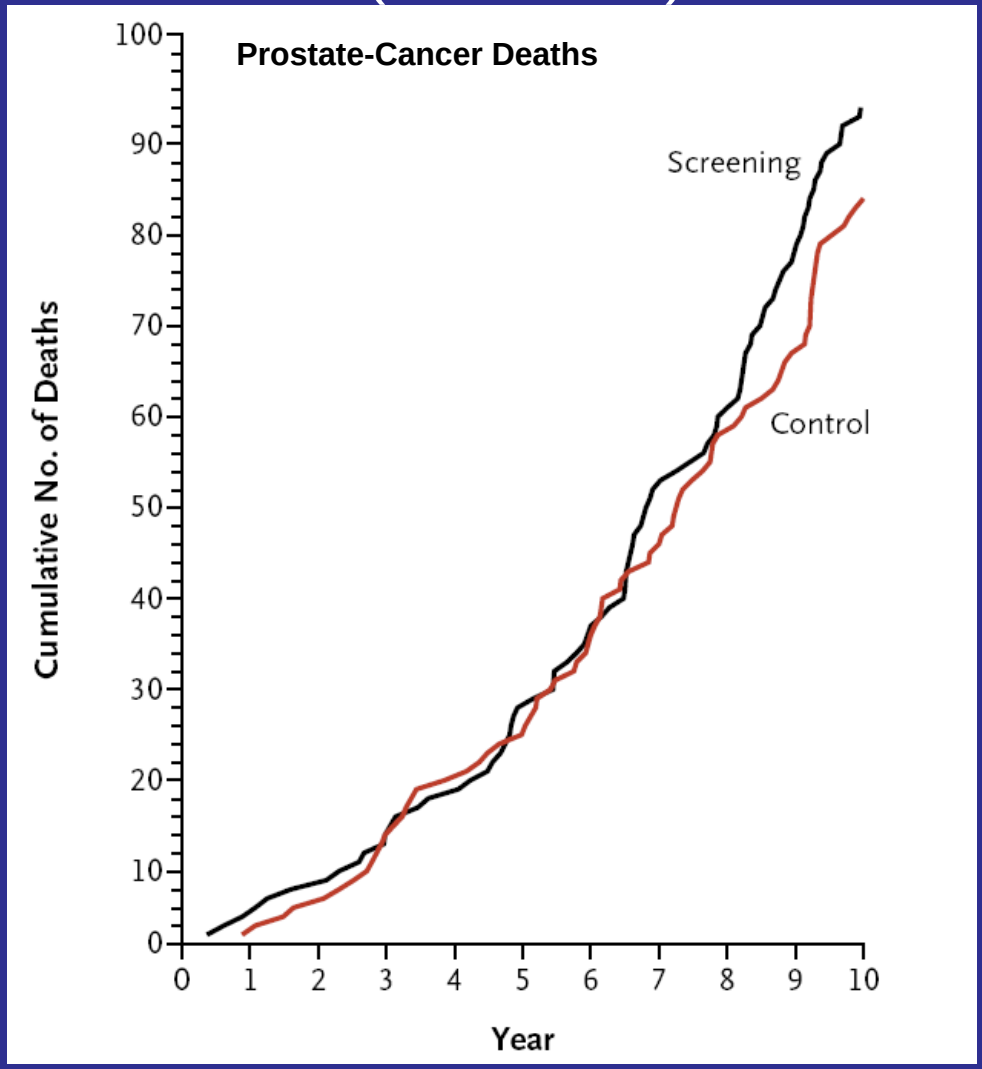
Gust H. Bardy, M.D., Kerry L. Lee, Ph.D., Daniel B. Mark, M.D., Jeanne E. Poole, M.D., Douglas L. Packer, M.D.,
Robin Boineau, M.D., Michael Domanski, M.D., Charles Troutman, R.N., Jill Anderson, R.N.,
George Johnson, B.S.E.E., Steven E. McNulty, M.S., Nancy Clapp-Channing, R.N., M.P.H.,
Linda D. Davidson-Ray, M.A., Elizabeth S. Fraulo, R.N., Daniel P. Fishbein, M.D., Richard M. Luceri, M.D.,
and John H. Ip, M.D., for the Sudden Cardiac Death in Heart Failure Trial (SCD-HeFT) Investigators*

Lifestyle vs. drug

Drug vs. device

Screening versus Usual Care: PLCO

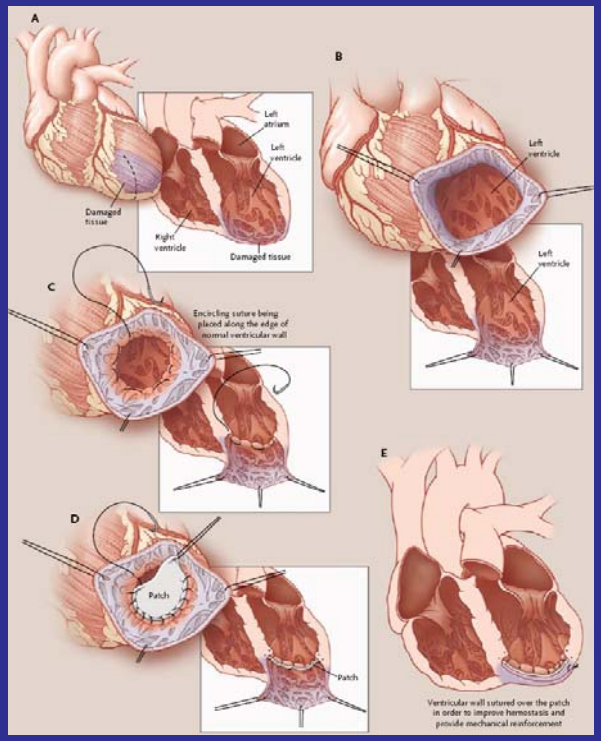
Screening PSA and digital rectal exam (N=76,693)

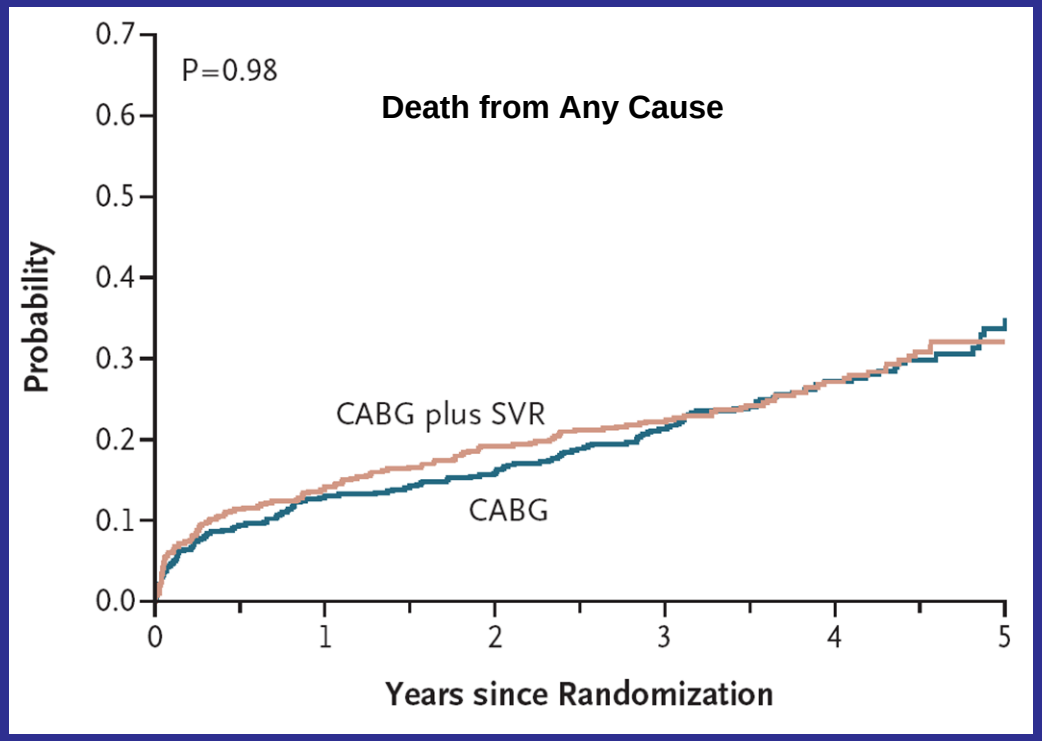


Andriole GL, et al. *N Engl J Med* 2009;360:1310-9

Surgery versus Surgery: STICH

LV reconstruction versus CABG only
(N=1000)

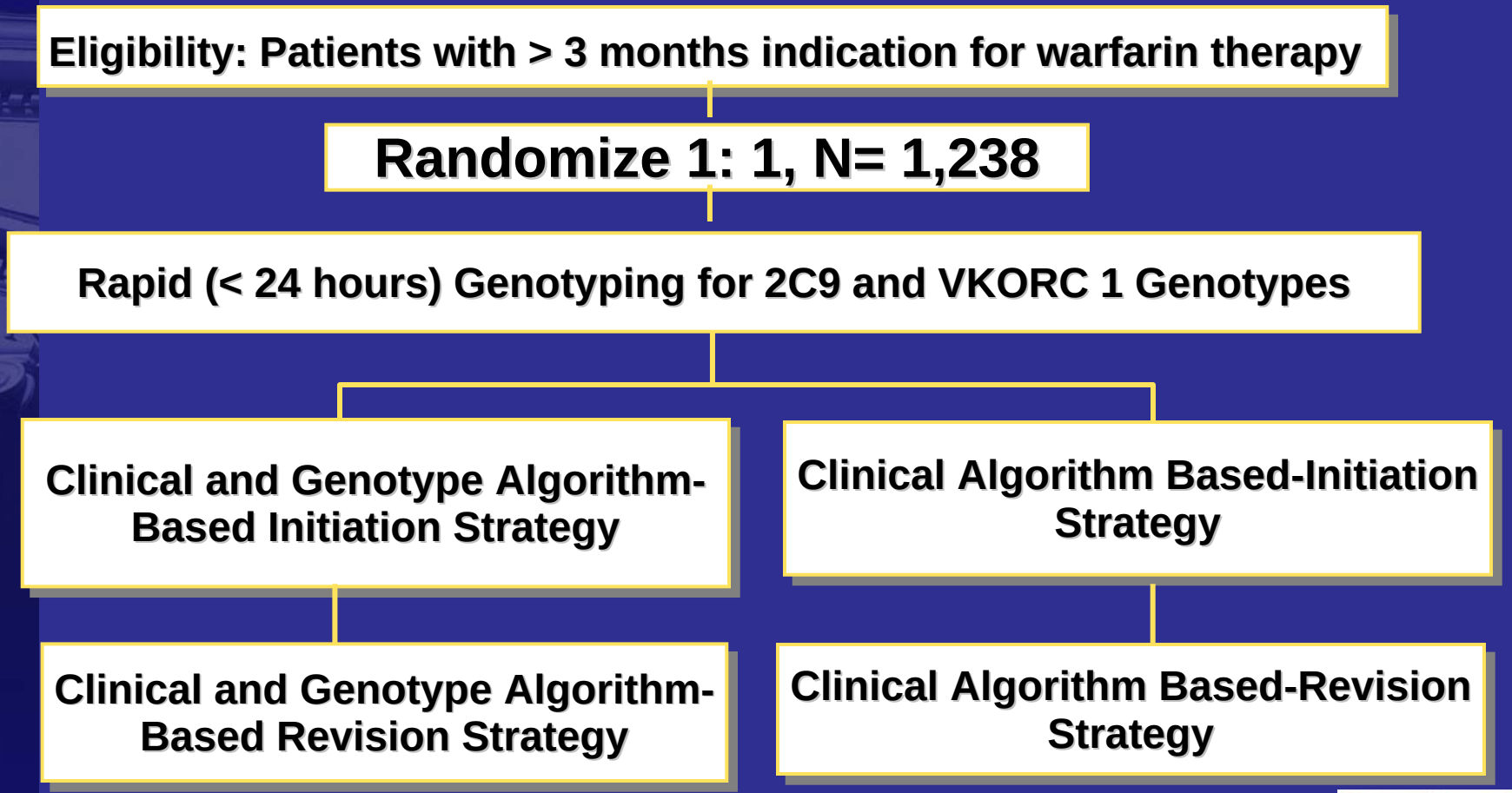




Jones RH, et al. *N Engl J Med.* 2009;360 (on line)
Eisen HJ, *N Engl J Med.* 2009; 360 (on line)

The Clarification of Optimal Anticoagulation Through Genetics (COAG) Trial

Trial Flowchart



http://coagstudy.org/

NIH CER Research Infrastructure:

- Clinical Trial Networks, Cooperative Groups, Disease Registries, HMO Clinical Research Networks, etc.
- NIH Consensus Development Program
- NLM National Center on Health Services Research
- CTSAs and community collaborations
- Post-market surveillance database with FDA
- Integrates CMS and SEER databases

Many Definitions of CER

CBO: “A rigorous evaluation of the impact of different options that are available for treating a given medical condition for a particular set of patients. Such a study may compare similar treatments, such as competing drugs, or it may analyze very different approaches.”

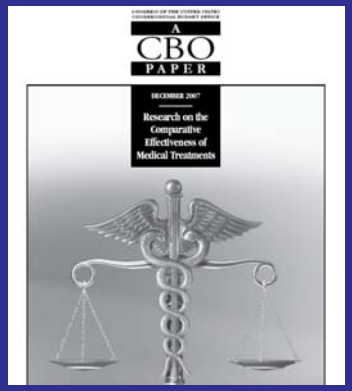
FCC: “Conduct and synthesis of systematic research comparing different interventions and strategies to prevent, diagnose, treat and monitor health conditions…to inform patients, providers, and decision-makers…about *which interventions are most effective for which patients under specific circumstances*.”

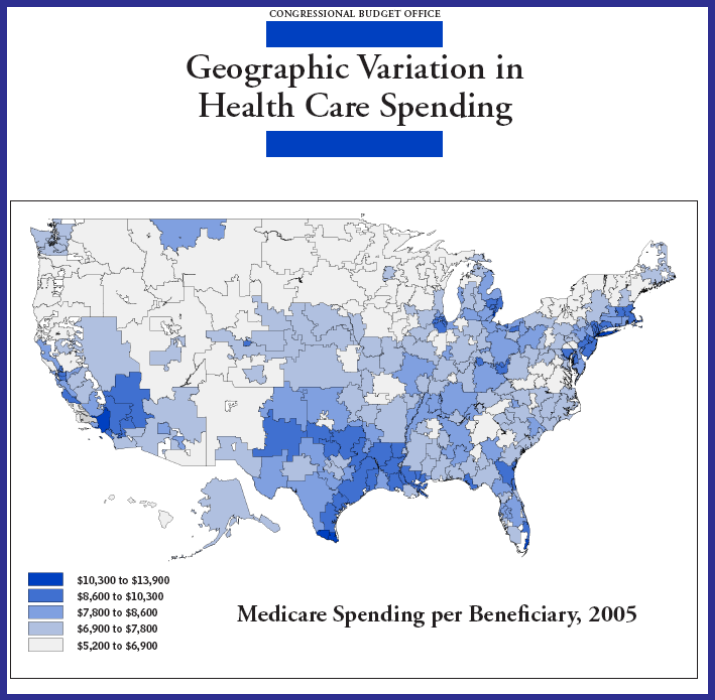
Common Themes Across Definitions

- Valid comparison
- Effectiveness (as opposed to efficacy)
  - "Real world"
  - Available options (i.e. not experimental)
  - "Real outcomes"
    - Length of life
    - Quality of life
    - Major clinical events (e.g. MI, CVA, hospitalization)
    - Costs

ARRA and CER: $1.1 billion

- NIH: $400 million
- AHRQ: $300 million
- HHS: $400 million





NIH is grateful to President Obama and to Congress for the opportunity for NIH to play its part in improving the nation's heath and economy.

http://www.opencongress.org/bill/111-h1/text

NIH CER Coordinating Committee

- Chairs: Drs. Betsy Nabel and Richard Hodes
- Best use of CER stimulus funds
- Collaboration with sister agencies
- Staging NIH CER portfolio analyses
- Communication and dissemination of CER findings
- Accelerating CER using existing mechanisms and new programs (e.g. Challenge Grants, GO Grants)
- Considering the agency's longer-term CER charge

NIH ARRA Interim Spending Plan

NIH plans to obligate $400M in ARRA support to advance CER in major activities including:

- Peer-reviewed meritorious grants
- Supplements to current research
- Challenge and Grand Opportunity Grants
- Contracts
- Funds will be awarded based on peer review, scientific opportunity and potential biomedical and public health impact

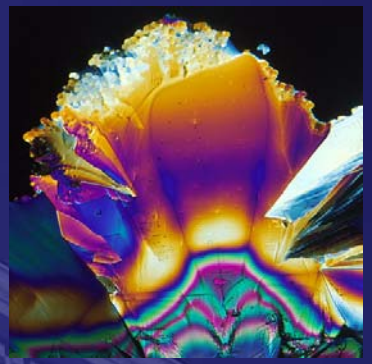
CER ARRA Challenges

- Rapid Timetable
- Unusual Two-Year Funding Mechanisms
- Political Context
- Economic Impact
- Inter-agency contexts
- Long-Term Effects of One-Time Bolus Infusion
- New Accountability, Budget Tracking Mechanisms
- Pressure on Review Functions

Stay Tuned!

The CER train is moving Fast






NIH

Transforming medicine and health through Comparative Effectiveness Research