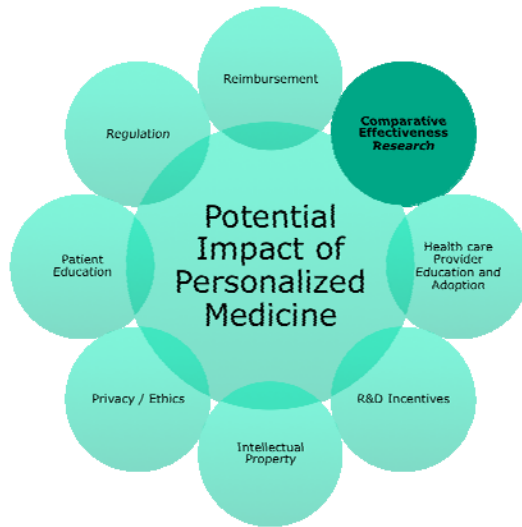


Comparative Effectiveness Research and Personalized Medicine

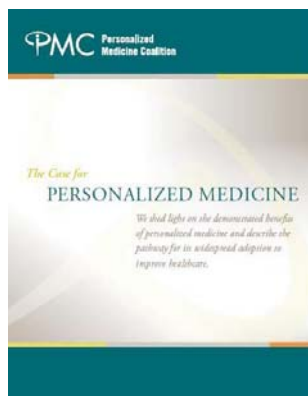
**Amy M. Miller, Ph.D.
Vice President, Public Policy
Personalized Medicine Coalition**

Personalized Medicine Coalition: Education and Advocacy

Addressing Public Policy Issues



Defining Personalized Medicine



“Personalized medicine refers to the tailoring of medical treatments to the individual characteristics of each patient ... Preventive or therapeutic interventions can then be concentrated on those who will benefit, sparing expense and side effects for those who will not.”

President's Council of Advisors on Science and Technology (PCAST), *Priorities for Personalized Medicine*, September 2008

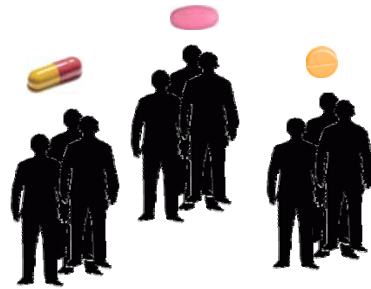
Defining Personalized Medicine

Current Practice



Trial and error

Personalized Medicine



The **right** treatment
for the **right** person
at the **right** time

What is Driving the Movement to Personalized Medicine?



The Science of New Drug Development

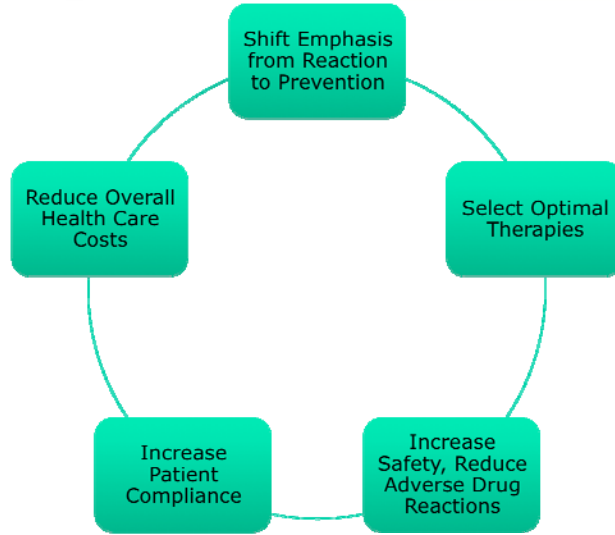


Faster Time to a Cure

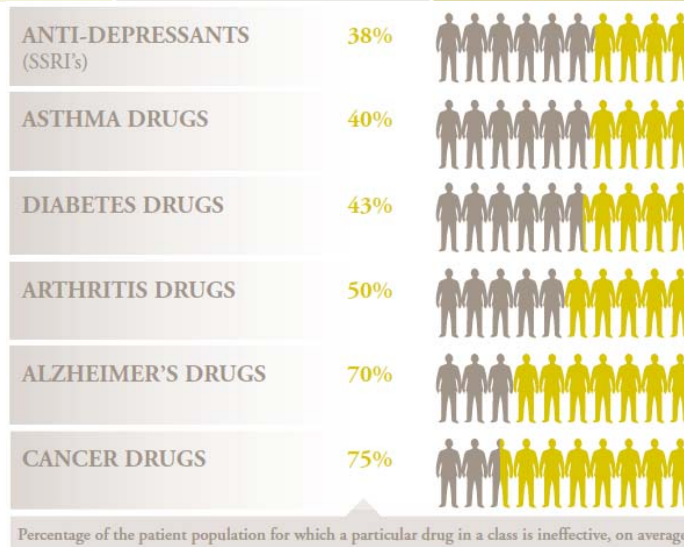


Cost-Effective Health Care

Potential Benefits of Personalized Medicine



Major Drugs Ineffective for Many



Ineffective Drugs Waste Money

<u>Type of Drug</u>	<u>Cost of Ineffectiveness in Health Care System</u>
Hypertension Drugs Ace Inhibitors	\$390 million – \$1.2 billion
Heart Failure Drugs Beta Blockers	\$345 million – \$575 million
Antidepressants SSRIs	\$2.3 billion – \$5.8 billion
Cholesterol Drugs Statins	\$3.8 billion – \$8.8 billion
Asthma Drugs Beta-2-agonists	\$560 million – \$1.0 billion

Personalized Medicine in the Clinic

Drug Dosing

- Coumadin/Warfarin Testing

Drug Avoidance

- Selzentry/Maraviroc

Drug Selection

- See PMC's Personalized Medicine Product Compendium

Erbitux® (cetuximab) <i>BRAF</i> Iressa® (gefitinib) Tarceva® (erlotinib) Vectibix® (panitumumab)	Colon cancer: A mutation in BRAF identifies 12-15% of metastatic colorectal cancer patients who fail to respond to TKI's. ¹ Non-mutated forms of <i>BRAF</i> and <i>KRAS</i> genes are required for response.
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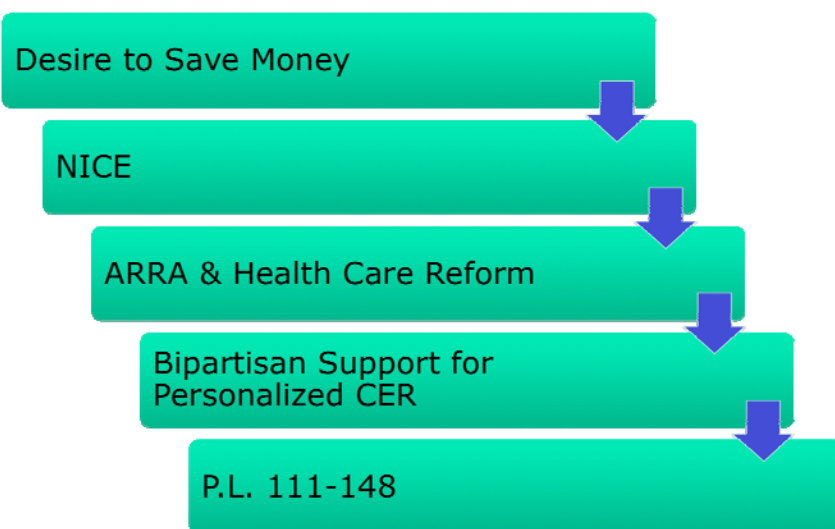
¹ Di Nicolantonio F, Martini M, Molinari F, et al. Wild-type BRAF is required for response to panitumumab or cetuximab in metastatic colorectal cancer. *J Clin Oncol.* 2008; 26:5705-12.

Comparative Effectiveness Research (CER)--

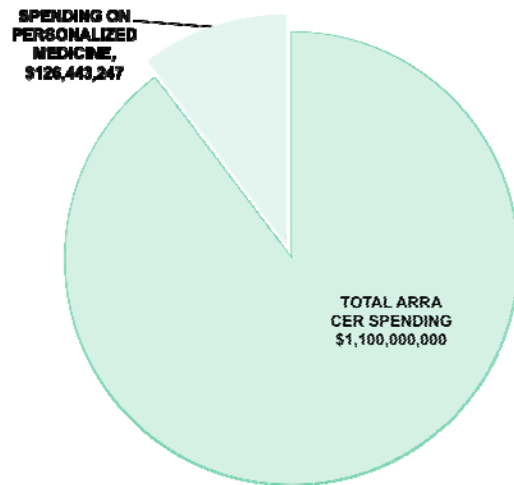


“[T]he generation and synthesis of evidence that compares the benefits and harms of alternative methods to prevent, diagnose, treat, and monitor a clinical condition or to improve the delivery of care.”
— Institute of Medicine, 2009

Brief History of Recent CER Policy



ARRA CER FUNDS SPENT ON PERSONALIZED MEDICINE



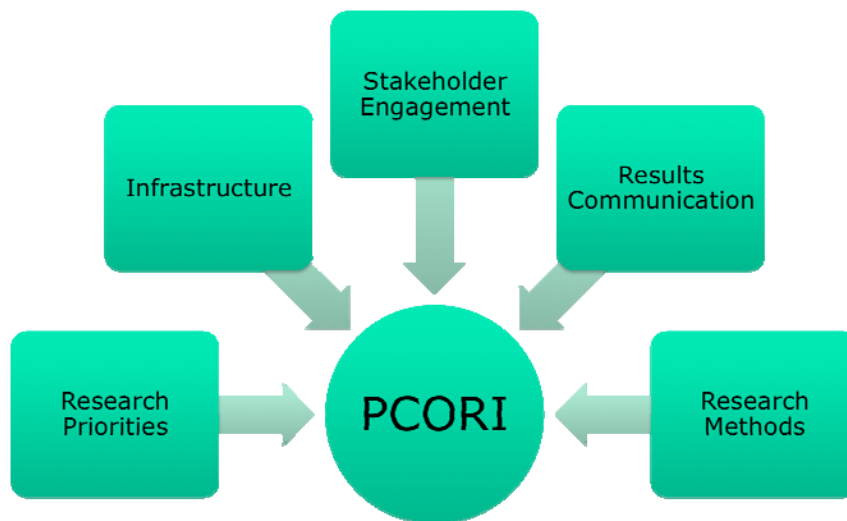
ARRA CER and Personalized Medicine

- Mostly infrastructure: caBIG ~100mil
- 24 of the 25 projects at the NIH
- 1 AHRQ
- Expect a shift to disease focus after infrastructure is built

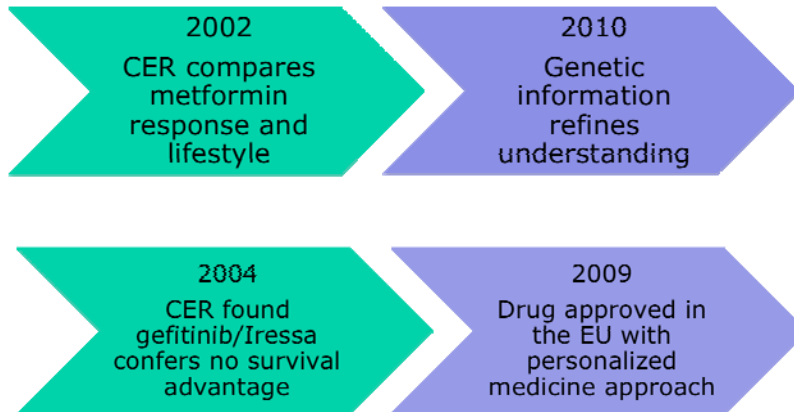
Patient Protection and Affordable Care Act (PPACA/health care reform)

“Comparative clinical effectiveness research means **research that evaluates and compares the patient health outcomes and benefits of two or more medical treatments or services.** Such treatment and services are defined broadly to include protocols for treatment, care management and delivery; procedures; diagnostic tools; medical devices; therapeutics; and any other strategies used to treat, diagnose or prevent illness or injury.” 42 U.S.C. 1181(a)(2)(A)-(B).

PPACA: Opportunity to Improve Care



CER Gets Personalized Over Time*



* A presentation by F. Collins, M.D., Ph.D., on Oct. 19, 2010 informed this slide

Personalized CER Moving Forward

Is
Avastin
Next?

Personalizing Comparative Effectiveness Research

Compare the Red Pill to the Blue Pill

- What if the blue pill doesn't work for you?

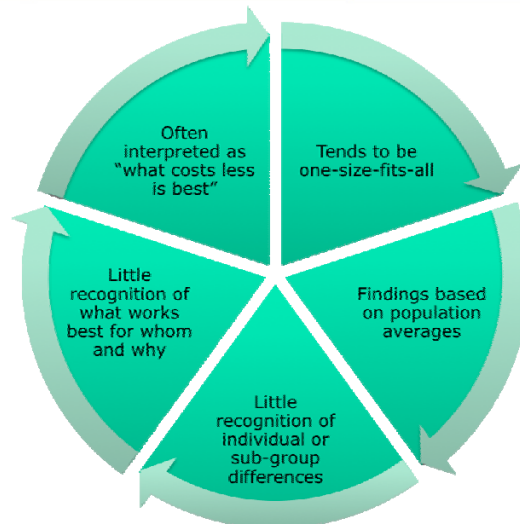
Traditional CER

- Lower quality of care
- Forestall progress

Personalized CER

- Focus on factors related to response
- Efficiencies improve care and save money

Traditional CER Can Impede Personalized Medicine



How to “Personalize” CER?

Understand Why

- Genotype all drug study participants
- Figure out drivers of treatment benefit

Methodology

- Accept alternatives to the RCTs (registries, observation studies, database analysis and systematic reviews)
- Build personalized medicine into them

Patient Centered Outcomes Research: PCORI’s Working Definition

- **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?”

PMC's CER Agenda

Work with PCORI to align personalized medicine with CER

Work with legislators to advance personalized medicine policies

Align regulation and reimbursement policies to allow for personalized medicine

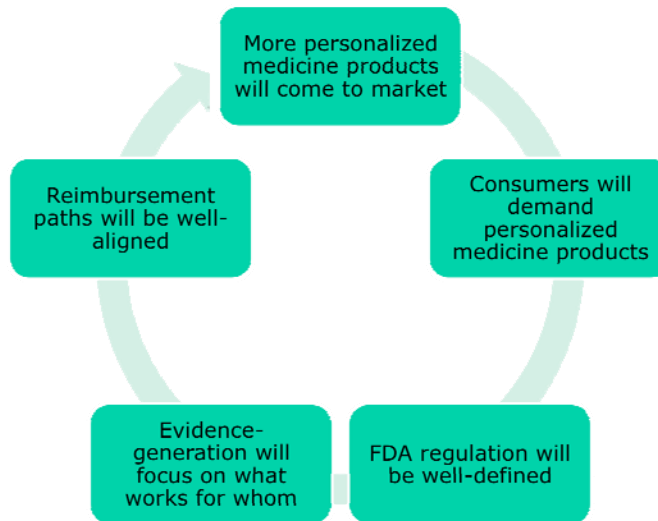
CER: Role of Payers?

Innovative partnerships with product developers will generate evidence

Cost-benefit will be evaluated across the system of care

Patient-centered focus will increase consumer satisfaction

Personalized Medicine in 10 years



What is the Future of CER Policy?

Patient Centered Outcomes Research Institute

- Board of Governors seated
- Methodology Committee seated
- Executive Director hired
- Definition of PCORI implies recognition of personalized medicine

Congress

- Intense pressure to reduce deficit by 1.2 Trillion
- Discretionary funding at risk (AHRQ, NIH)
- Structure of PCORI legislation makes complete de-funding unlikely

CER Resources



- National Pharmaceutical Council
 - CER Daily Newsfeed
- Partnership to Improve Patient Care
 - CER Inventory
- ECRI Institute
- Friends of Cancer Research
- AHRQ
- Age of Personalized Medicine Blog

Personalized Medicine Coalition



The Personalized Medicine Coalition, representing scientists, patients, providers and payers, promotes the understanding and adoption of personalized medicine concepts, services and products to benefit patients and the health system.

The Personalized Medicine Coalition

To learn more, visit:

www.PersonalizedMedicineCoalition.org