

The U.S. Precision Medicine Initiative

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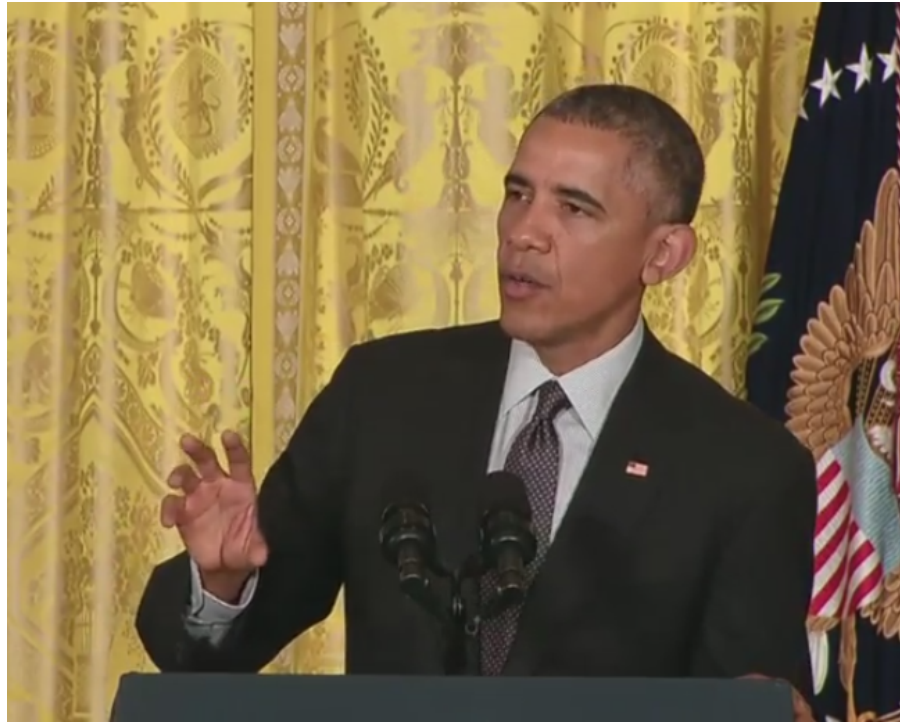
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Precision Medicine Initiative: Timing is Everything

	Ten Years Ago	Now – 2014 (most recent data)
Cost of sequencing a human genome	\$22,000,000	\$1000 - \$5000
Amount of Time to Sequence a Human Genome	2 years	<1 day
Number of smart phones in the United States	1 million (<2%)	160 million (58%)
EHR Adoption, (% providers)	20-30%	>90%
Computing Power	n	n x 16



“And that’s why we’re here today. Because something called precision medicine ... gives us one of the greatest opportunities for new medical breakthroughs that we have ever seen.”

President Barack Obama
January 30, 2015



The NEW ENGLAND JOURNAL *of* MEDICINE

January 30, 2015

Perspective

A New Initiative on Precision Medicine

Francis S. Collins, M.D., Ph.D., and Harold Varmus, M.D.

“Tonight, I’m launching a new Precision Medicine Initiative to bring us closer to curing diseases like cancer and diabetes — and to give all of us access to the personalized information we need to keep ourselves and our families healthier.”

— President Barack Obama, State of the Union Address, January 20, 2015

The proposed initiative has two main components: a near-term focus on cancers and a longer-term aim to generate knowledge applicable to the whole range of health and disease. Both components are now within our reach because of advances in basic research, including molecular biology, genomics, and bioinformatics. Furthermore, the initiative

www.nih.gov/precisionmedicine

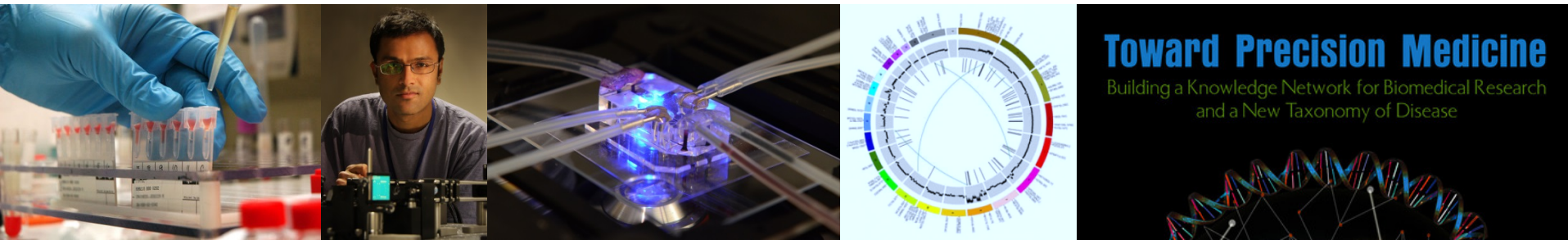
Precision Medicine

Concept is not new

- Consider prescription eyeglasses, blood transfusions...
- Prospects for broader application raised by recent advances in basic research, technology development, genomics, proteomics, metabolomics, EHRs, Big Data, mHealth, etc.
- Reinforced by 2011 National Research Council report

What is needed **now**

- Development of rigorous research program to provide scientific evidence needed to turn concept into reality
- Recruitment of the best and brightest from multiple disciplines to join the team



Toward Precision Medicine

Building a Knowledge Network for Biomedical Research
and a New Taxonomy of Disease

Precision Medicine Initiative (PMI)

Vision: Build a broad research program to encourage creative approaches to precision medicine, test them rigorously, and, ultimately, use them to build the evidence base needed to guide clinical practice.

- **Near Term:** apply the tenets of precision medicine to a major health threat – cancer
- **Longer Term:** generate the knowledge base necessary to move precision medicine into virtually all areas of health and disease

PMI: Policy and Privacy Prerequisites

To develop a new research and care model, PMI will:

- **Engage Stakeholders:** White House, HHS, other federal agencies will solicit input from patient groups, bioethicists, technologists, privacy experts, civil liberties advocates, etc.
- **Modernize Regulations:** Food and Drug Administration (FDA) to review regulatory landscape, support changes to advance precision medicine and protect public health
- **Safeguard Privacy:** Office of the National Coordinator for Health Information Technology (ONC) will develop interoperability standards, enable secure data exchange



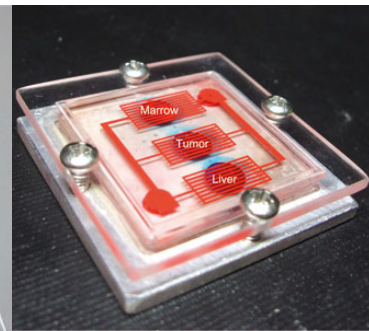
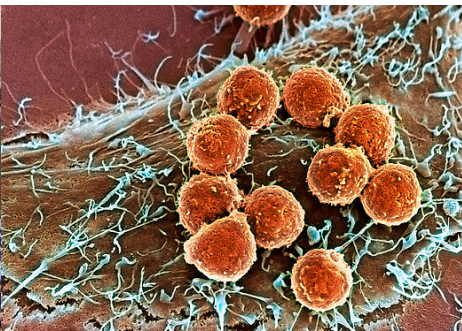
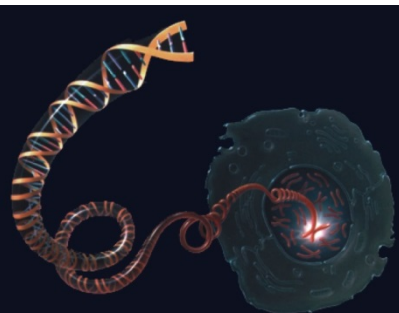
PMI Proposed Support: FY16

Agency	\$ Million
NIH <ul style="list-style-type: none">• <i>Cancer</i>• <i>Cohort</i>	\$200 <ul style="list-style-type: none">\$70\$130
FDA	\$10
ONC	\$5
TOTAL	\$215

PMI: Near Term

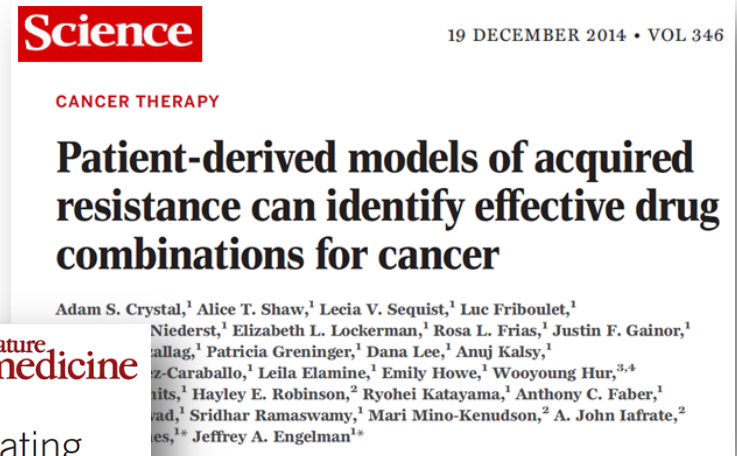
Apply tenets of precision medicine to **cancer**

- Use NCI clinical trials as models
 - NCI-Match: solid tumors, lymphomas (multi-drug, multi-arm)
 - Lung-MAP: squamous cell lung cancer (multi-drug, multi-arm, randomized)
- Identify new cancer subtypes, therapeutic targets
- Test precision therapies, with private sector partners
 - Wide spectrum of adult and pediatric cancers
 - Early stage to advanced disease



PMI and Cancer Research: Possible Near-Term Applications

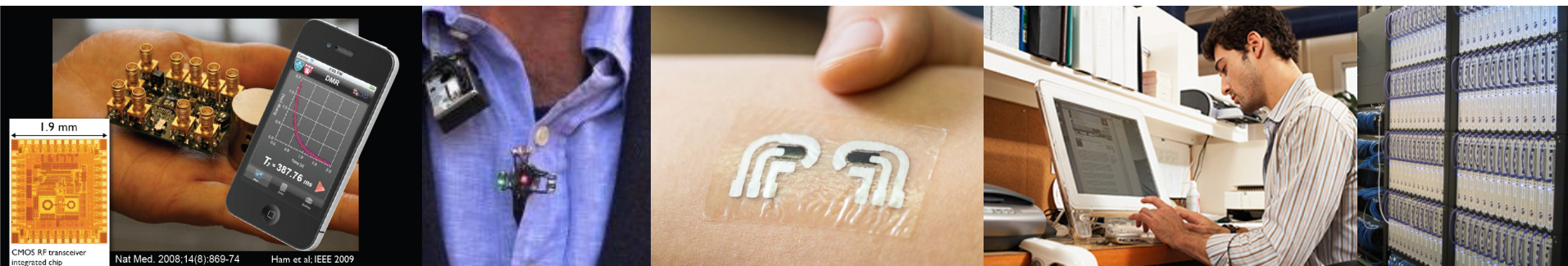
- Evaluating use of “liquid biopsies” for non-invasive detection of tumor response
- Understanding, counteracting development of resistance to targeted therapies
- Determining prospects of combination targeted therapy based on individual tumor genome analysis



PMI: Longer Term

Generate knowledge base needed to move precision medicine into **the whole range of health and disease**

- To reach this goal, the Initiative will support research to:
 - Create new approaches for detecting, measuring, analyzing a wide array of biomedical variables: molecular, genomic, cellular, clinical, behavioral, physiological, and environmental
 - Test these approaches in small, pilot studies
 - Utilize the most promising approaches in greater numbers of people over longer periods of time to collect data of great value to both researchers and participants



PMI: National Research Cohort

- Will comprise:
 - >1 million U.S. volunteers
 - Numerous existing cohorts (many funded by NIH)
 - New volunteers
- Participants will be:
 - Centrally involved in design, implementation
 - Able to share genomic data, lifestyle information, biological samples – all linked to their electronic health records
- Will forge new model for scientific research that emphasizes:
 - Engaged participants
 - Open, responsible data sharing with privacy protections



National Research Cohort: Possible Uses

- Facilitate research on therapeutic safety/efficacy/metabolism
- Study resilience by finding people who should be ill but aren't
- Uncover therapeutic targets by identifying rare variants protective against disease
- Provide unbiased quantitative determination of risk
- Offer powerful test bed for:
 - Incorporating patient-reported outcomes
 - Improving utility of EHRs
 - Evaluating wide array of mHealth applications





Patient Partnerships



EHRs



Technologies



Genomics



Data Science

Advisory Committee to the NIH Director

Precision Medicine Initiative Working Group

Co-Chairs:

Richard Lifton, MD, PhD, Yale Univ School of Medicine, New Haven, CT

Bray Patrick–Lake, MFS, Duke Univ, Durham, NC

Kathy Hudson, PhD, National Institutes of Health

Members:

- **Esteban Gonzalez Burchard, MD, MPH**
University of California, San Francisco
- **Tony Coles, MD, MPH**
Yumanity Therapeutics, Cambridge, MA
- **Rory Collins, FMedSci**
University of Oxford, UK
- **Andrew Conrad, PhD**
Google X, Mountain View, CA
- **Josh Denny, MD**
Vanderbilt University, Nashville
- **Susan Desmond–Hellmann, MD, MPH**
Gates Foundation, Seattle
- **Eric Dishman**
Intel, Santa Clara, CA
- **Kathy Giusti, MBA**
Multiple Myeloma Res Foundation, Norwalk, CT
- **Sekar Kathiresan, MD**
Harvard Medical School, Boston
- **Sachin Kheterpal, MD, MBA**
University of Michigan Medical School, Ann Arbor
- **Shiriki Kumanyika, PhD, MPH**
Perelman School of Medicine, Philadelphia
- **Spero M. Manson, PhD**
University of Colorado, Denver
- **P. Pearl O’Rourke, MD**
Partners Health Care System, Inc., Boston
- **Richard Platt, MD**
Harvard Pilgrim Health Care Institute, Boston
- **Jay Shendure, MD, PhD**
University of Washington, Seattle
- **Sue Siegel**
GE Ventures & Healthymagination, Menlo Park, CA

Looking to the Future

- Advisory Committee to the NIH Director Working Group
 - Public workshops to inform report in September 2015
- NIH coordinating with White House, FDA, other agencies
- Seeking input from:
 - Potential participants
 - Leaders of current cohorts
 - mHealth technology developers
 - EHR developers
 - Potential international partners
- **Oct. 1, 2015—PMI launched!** (pending FY2016 appropriations)



Precision Medicine Is Already Working To Cure Americans: Success Stories



William Elder Jr.



Emily Whitehead



Elana Simon



Melanie Nix



Hugh and
Beatrice Rienhoff



Kareem
Abdul-Jabbar



Noah and
Alexis Beery

PRECISION MEDICINE INITIATIVE



Precision Medicine Initiative

- [What are the near-term goals?](#)
- [What are the longer-term goals?](#)
- [How is it different?](#)
- [Who will participate?](#)
- [NIH Workshop](#)

Precision Medicine Initiative

Far too many diseases do not have a proven means of prevention or effective treatments. We must gain better insights into the biology of these diseases to make a difference for the millions of Americans who suffer from them. Precision medicine is an emerging approach for disease treatment and prevention that takes into account individual variability in genes, environment, and lifestyle for each person. While significant advances in precision medicine have been made for select cancers, the practice is not currently in use for most diseases. Many efforts are underway to help make precision medicine the norm rather than the exception. To accelerate the pace, President Obama has now unveiled the Precision Medicine Initiative – a bold new enterprise to revolutionize medicine and generate the scientific evidence needed to move the concept of precision medicine into every day clinical practice.



The Precision Medicine Initiative: Infographic
[View larger](#) (PDF - 163KB)

Email Updates

To sign up for updates please enter your e-mail address.



Related Links

- [NEJM Perspective: A New Initiative on Precision Medicine](#)
- [White House Precision Medicine Web Page](#)
- [White House Fact Sheet: President Obama's Precision Medicine Initiative](#)
- [Precision Medicine Initiative and Cancer Research](#)
- [Storify: The Precision Medicine Initiative](#)

Questions?

