QIBA-NIBIB Alliance

2010-2015
Congratulations on five successful years

The U.S. Precision Medicine Initiative
[William Heetderks, MD, PhD]
[Associate Director, NIBIB]
[May 6, 2015]
“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

www.nih.gov/precisionmedicine
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

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**Precision Medicine Initiative: Timing is Everything**

<table>
<thead>
<tr>
<th></th>
<th>Ten Years Ago</th>
<th>Now – 2014 (most recent data)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Cost of sequencing a human genome</td>
<td>$22,000,000</td>
<td>$1000 - $5000</td>
</tr>
<tr>
<td>Amount of Time to Sequence a Human Genome</td>
<td>2 years</td>
<td>&lt;1 day</td>
</tr>
<tr>
<td>Number of smart phones in the United States</td>
<td>1 million (&lt;2%)</td>
<td>160 million (58%)</td>
</tr>
<tr>
<td>EHR Adoption, (% providers)</td>
<td>20-30%</td>
<td>&gt;90%</td>
</tr>
<tr>
<td>Computing Power</td>
<td>n</td>
<td>n x 16</td>
</tr>
<tr>
<td>QIBA</td>
<td>no</td>
<td>9 active biomarker committees</td>
</tr>
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</table>
PMI Proposed Support: FY16

<table>
<thead>
<tr>
<th>Agency</th>
<th>$ Million</th>
</tr>
</thead>
<tbody>
<tr>
<td>NIH</td>
<td></td>
</tr>
<tr>
<td>• Cancer</td>
<td>$200</td>
</tr>
<tr>
<td>• Cohort</td>
<td>$70</td>
</tr>
<tr>
<td></td>
<td>$130</td>
</tr>
<tr>
<td>FDA</td>
<td>$10</td>
</tr>
<tr>
<td>ONC</td>
<td>$5</td>
</tr>
<tr>
<td>TOTAL</td>
<td>$215</td>
</tr>
</tbody>
</table>

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, imaging, 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

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**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
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)