Quantitative Imaging Biomarker Alliance

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QIBA Queries

• How might current NIH Initiatives benefit from QIBA Biomarker Development?
  • Plan activities to align with the NIH goals
  • Make a case to potential future funders

• Translation to Clinical Research & Practice?
NIH INITIATIVES

Precision Medicine

Cancer Moonshot

Human Placenta

Human Connectome

BRAIN
NIH INITIATIVES

Precision Medicine

Cancer Moonshot
Precision Medicine Initiative

The challenges ...

• Many diseases lack effective prevention strategies, diagnostics, or treatments
  • Options fail to consider key differences among individuals: genes, lifestyle, environment

• Research findings take too long to be implemented into clinical practice

• Need to look beyond the genome

• Participants in biomedical research often treated as “subjects,” not partners
Scientific Opportunities in the PMI Cohort Program

• Develop quantitative estimates of risk for a range of diseases by integrating environmental exposures, genetic factors and gene-environment interactions
• Identify the causes of individual variation in response to commonly used therapeutics (pharmacogenomics)
• Discover biological markers that signal increased or decreased risk of developing common diseases
• Use mobile health (mHealth) technologies to correlate activity, physiological measures and environmental exposures with health outcomes
• Develop new disease classifications and relationships
• Empower study participants with data and information to improve their own health
• Create a platform to enable trials of targeted therapies
<table>
<thead>
<tr>
<th>Date</th>
<th>Event Description</th>
</tr>
</thead>
<tbody>
<tr>
<td>January 2015</td>
<td>President launches Precision Medicine Initiative®</td>
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<tr>
<td>March 2015</td>
<td>NIH names ACD PMI Working Group</td>
</tr>
<tr>
<td>September 2015</td>
<td>ACD receives and approves PMI Working Group Report</td>
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<tr>
<td>November 2015</td>
<td>6 funding opportunities issued</td>
</tr>
<tr>
<td>December 2015</td>
<td>PMI Cohort Program Advisory Panel convened</td>
</tr>
<tr>
<td>January 2016</td>
<td>Search for Director closed</td>
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<tr>
<td>January 2016</td>
<td>1st &amp; 2nd level review for pilots</td>
</tr>
<tr>
<td>February 2016</td>
<td>Pilot awards made</td>
</tr>
</tbody>
</table>
# PMI Cohort Program Funding Opportunities

<table>
<thead>
<tr>
<th>Title / Type</th>
<th>Year 1</th>
<th>Number of awards</th>
<th>Project Period</th>
<th>Application</th>
<th>Award</th>
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</thead>
<tbody>
<tr>
<td>Direct Volunteers Pilot Studies (OT)</td>
<td>TBD</td>
<td>1</td>
<td>1 yr</td>
<td>December 22, 2015</td>
<td>February 2016</td>
</tr>
<tr>
<td>Communication Support for the Precision Medicine Initiative Research Programs (OT)</td>
<td>TBD</td>
<td>1</td>
<td>2 yrs</td>
<td>December 22, 2015</td>
<td>February 2016</td>
</tr>
<tr>
<td>PMI Cohort Program Biobank (U24)</td>
<td>$15 M</td>
<td>1</td>
<td>5 yrs</td>
<td>February 4, 2016</td>
<td>June 2016</td>
</tr>
<tr>
<td>PMI Cohort Program Coordinating Center (U2C)</td>
<td>$21 M</td>
<td>1</td>
<td>5 yrs</td>
<td>February 17, 2016</td>
<td>July 2016</td>
</tr>
<tr>
<td>PMI Cohort Program Healthcare Provider Organization Enrollment Centers (UG3/UH3)</td>
<td>$28 M</td>
<td>≤7</td>
<td>5 yrs</td>
<td>February 17, 2016</td>
<td>July 2016</td>
</tr>
<tr>
<td>PMI Cohort Program Participant Technologies Center (U24)</td>
<td>$8 M</td>
<td>1</td>
<td>5 yrs</td>
<td>February 17, 2016</td>
<td>July 2016</td>
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</tbody>
</table>
What will the PMI Cohort Program have accomplished by Dec. 2016?

- Direct volunteer recruitment and engagement strategies pilot completed
- Pilot results used to design and launch scale up of the direct volunteer program
- Strong partnerships with 5-7 major Healthcare Provider Organizations
- Successful implementation of test recruitment sites in 5 FQHCs
- ~79,000/1M engaged participants fully consented and enrolled in the Cohort
- Collection of biospecimens from at least 25,000
- Sync4Science FHIR method pilot complete
- Functioning data platform to allow collection of different types of data
  - Secure environment accessible to researchers
  - Participants see info about themselves according to their preferences
- 8-10 research studies using cohort data underway
Interventions to prevent, diagnose, or treat a disease (e.g., cancer), based on a molecular and/or mechanistic understanding of the causes, pathogenesis, and/or pathology of the disease.

Where the individual characteristics of the patient are sufficiently distinct, interventions can be concentrated on those who will benefit, sparing expense and side effects for those who will not.
Components of PMI-Oncology

- *Developing and expanding clinical therapy trials* in precision oncology
- Improving predictive oncology: by overcoming drug resistance, determining effective combination targeted therapy, and advancing immunotherapy
- Sharpen our ability to diagnose cancer at its earliest stages, when it is usually most treatable
- Building a national cancer knowledge system that integrates cancer genomic information, clinical information, and laboratory model information
US Mortality Rates have Declined: All Cancer Sites Combined 1992–2011

Mortality data source: National Center for Health Statistics (NCHS)
Long-Term Mortality Trends 2002-2011, by Cancer Site

**Men**
- Liver & IBD: 26%
- Soft Tissue inc. Heart: 8%
- Pancreas: 3%
- Melanoma: 3%
- Bladder: 0%
- Decreasing: Brain & ONS (4), Oral Cavity (5), Esophagus (5), Kidney (8), Leukemia (9), Myeloma (11), All Sites (18), Non-Hodgkin Lymphoma (23), Larynx (25), Lung & Bronchus (26), Colon & Rectum (30), Stomach (31), Prostate (33)
- Increasing: Liver & IBD (13), Corpus & Uterus (4)

**Women**
- Liver & IBD: 19%
- Corpus & Uterus: 10%
- Pancreas: 4%
- Decreasing: Bladder (4), Kidney (9), Brain & ONS (9), Leukemia (11), Gallbladder (12), Lung & Bronchus (12), Cervix (13), All Sites (14), Esophagus (15), Myeloma (16), Breast (19), Oral Cavity (20), Ovary (20), Stomach (27), Colon & Rectum (29), Non-Hodgkin Lymphoma (31)
- Increasing: Liver & IBD (13), Corpus & Uterus (4)

**Percent Change 2002–2011**
NIH Cancer Moonshot

• Supports 7 areas for cutting edge research opportunities
  • Prevention & Cancer Vaccine development
  • **Early Cancer Detection** - evaluation of minimally invasive screening assays
  • Cancer Immunotherapy & Combination Therapy
  • Genomic Analysis of Tumor & Surrounding Cells
  • Enhanced Data Sharing
  • **FDA - Oncology Center of Excellence** - combining skills of regulatory scientists and reviewers with expertise in drugs, biologics, and devices.
  • **Pediatric Cancer**

AND

• **VP’s Exceptional Opportunities in Cancer Research Fund** - focused on high-risk, high-return research identified by the collaborative work and new ideas stimulated by the research community
Human Placenta Project

Overarching goal: Understand human placental development, structure, and function in real time

- Improve current methods and develop new technologies for real-time assessment of human placental development and function across gestation
- Apply these approaches in normal and abnormal human pregnancies
- Develop and evaluate non-invasive markers of placental dysfunction for prediction of adverse pregnancy outcomes
- Understand the contributions of placental development to long-term human health and disease
- Develop interventions to prevent abnormal human placental development, and hence improve pregnancy outcome
Three RFAs in FY15

• Novel Tools to Assess Human Placental Structure and Function (R01, R21)

For development of new technology, or novel application of current technology, for studying the placenta in vivo with the ultimate goal of human use across pregnancy. Objective is to push beyond existing paradigms.

• Developing Paradigm-Shifting Innovations for in vivo Human Placental Assessment in Response to Environmental Influences (U01 Cooperative Agreement)

• Formation of interdisciplinary teams to design and develop new or next-generation placental imaging and assessment technologies and methods that will increase our capability to assess human placental structure and function safely in vivo throughout gestation
• Explore the impact of environmental influences on placental structure and function across pregnancy
**Distribution of FY15 Projects**

- **MRI (9)**
- **US (6)**
- **Blood Analysis (4)**
- **NIRS (1)**

**Near Infrared Resonance Spectroscopy**
(assessment of hemodynamic alterations)

- **Exosomes, Lipids, miRNA, IncRNA**

- **19 awards ~$46M**
- **16/19 in Imaging**

Focus on collaboration across disciplines and development of synergy
Two RFAs newly published for FY17

• Assessing Human Placental Development and Function Using Existing Data
  • RFA- HD-17-004 (R01)
  • RFA- HD-17-005 (R21)

Evaluation of human placental development and function across pregnancy through the application of assessment technologies to existing data.
3rd Human Placenta Project Meeting

• Explore how to incorporate novel technology into the HPP
• Focus on imaging, bioinformatics, and technology
• Breakout groups, poster session, and technology demonstrations
• Co-sponsored by NICHD and NIBIB
• April 14-15, 2016 in Bethesda, Maryland
QIBA Biomarkers: Translation to Clinical Research

CT Volumetry/Cancer – NCI, NINDS, NIDDK, NHLBI, NICHD

FDG-PET (SUV)/Cancer – NCI, NINDS, NIDDK, NHLBI, NICHD

Dynamic Contrast Enhancement MRI/Cancer – NCI, NINDS, NIDDK, NHLBI, NICHD

CT Lung Density Biomarker – NCI, NHLBI

FDG-PET/Amyloid Biomarker – NINDS

fMRI Biomarker – NINDS, NICHD

MR Elastography Biomarker – NCI, NIDDK

Perfusion/Diffusion/Flow MRI Biomarker – NINDS, NCI, NICHD

SPECT Biomarker – NCI, NHLBI

US Shear Wave-Speed/Volume Blood Flow Biomarkers – NCI, NIDDK, NICHD
Interagency Working Group, Medical Imaging
Meeting on Nov 5th 2015, at NIH

• External speakers:
  • Ms. Renee Cruea (ARR)
  • Dr. Richard Ehman (RSNA)
  • Dr. James Thrall (IS3R)
  • Dr. Mitchell Schnall (ACR)

• Discussion:
  ✓ Data sharing, quantitative imaging, machine-assisted image interpretation / “deep learning”
  ✓ Need for a pathway for validation of biomarkers for diseases; the importance of measurement standards

• IWGMI members considered topics for potential focus efforts to include Quantitative Imaging (near term) and Deep Learning (intermediate/longer term)

Member Agencies: NIH, NIST, NSF, FDA, DOE, DOD, NASA, FBI, VA, CMS, OMB, OSTP, & Treasury
NIH Partnerships

1. Interagency Working Group on Medical Imaging (NIBIB/NIST)
   1. Academia (11/5/15)
   2. Imaging-device Industry (3/24/16)
2. QIN (NCI): focusing on imaging-derived quantitative measurements of responses to drugs and/or radiation therapy, and/or image-guided interventions (IGI)
3. Medical Device Innovation Consortium (MDIC Board has ex officio NIH, FDA & CMS representatives)
4. Trans-NIH/FDA Medical Device Interest Group
THANK YOU