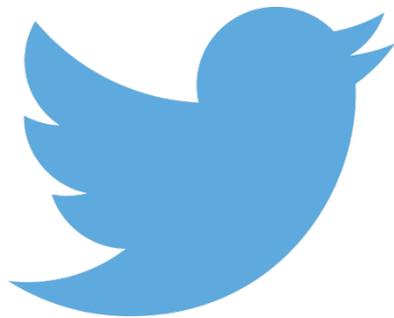


The Precision Medicine Initiative®
Cohort Program:
An Update

Josie Briggs and Kathy Hudson
December 10, 2015

Follow PMI Cohort Program

www.nih.gov/precision-medicine-initiative-cohort-program



#PMINetwork

@NCCIH_Josie

@KathyHudsonNIH

Precision Medicine Initiative® Mission

To enable a new era of medicine through research, technology, and policies that empower patients, researchers, and providers to work together toward development of individualized treatments.

Precision Medicine Initiative®

- Precision medicine is much bigger than PMI
- PMI has many components
 - Cohort
 - Cancer
 - FDA
 - ONC
 - Etc.

Timeline

January 2015	President launches Precision Medicine Initiative®
March 2015	NIH names ACD PMI Working Group
September 2015	ACD receives and approves Working Group Report
November 2015	First implementation steps
December 2015	Report to ACD on PMI progress



Assembling the PMI Cohort

- One million or more volunteers
 - Broadly reflect the diversity of the U.S., not statistically representative
 - Strong focus on underrepresented groups
- Longitudinal cohort, with continuing interactions, recontactable for secondary studies
- Two methods of recruitment
 - Direct volunteers - anyone can sign up
 - Healthcare provider organizations
- Estimated 3-4 years to reach one million

Wealth of Scientific Opportunities

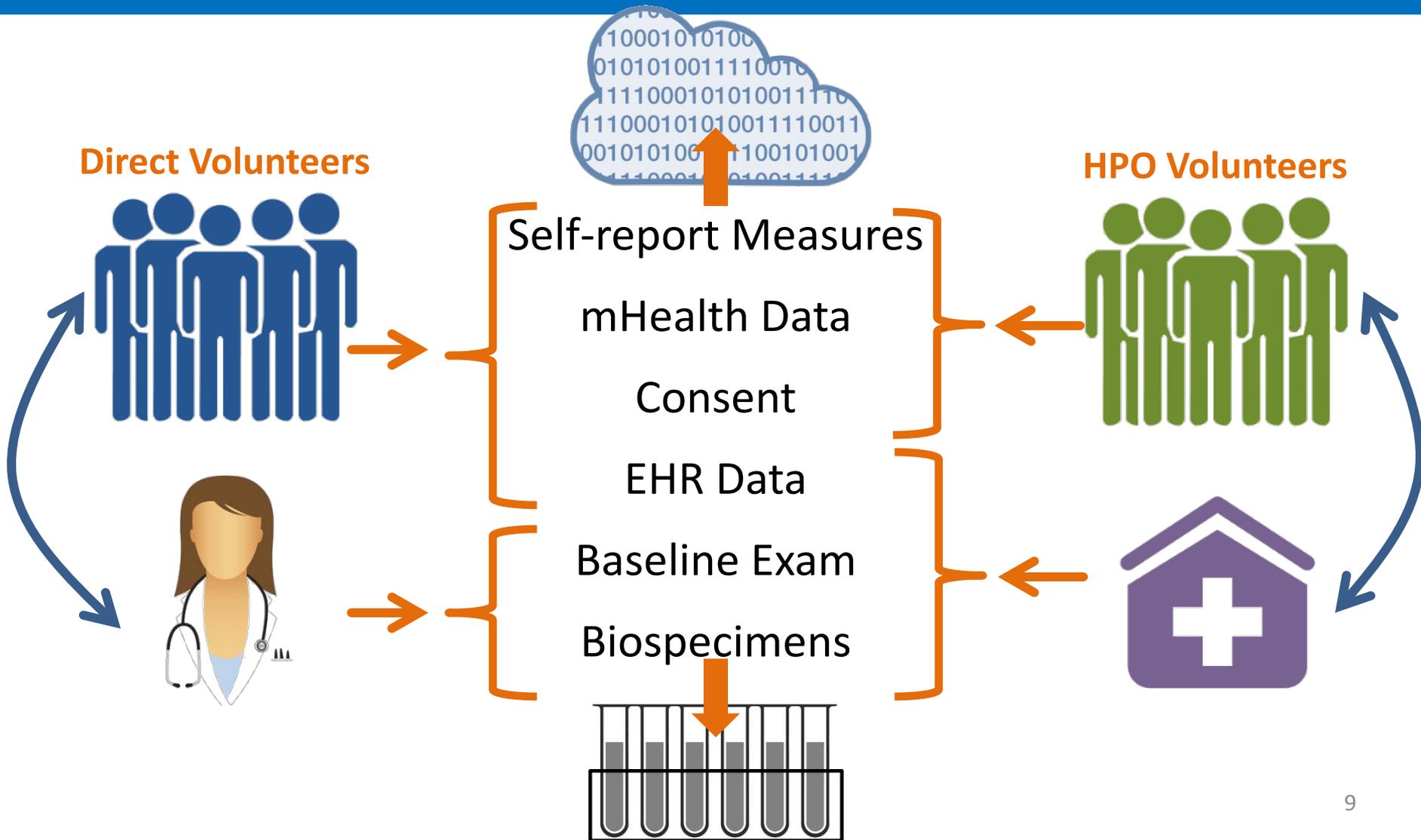
- Develop quantitative estimates of risk for a range of diseases by integrating environmental exposures and genetic factors
- Identify the causes of individual variation in response to commonly used therapeutics (pharmacogenomics)
- Discover biological markers that signal increased or decreased risk of developing common diseases
- Understand and address causes of health disparities
- 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

Initial Core Data Set

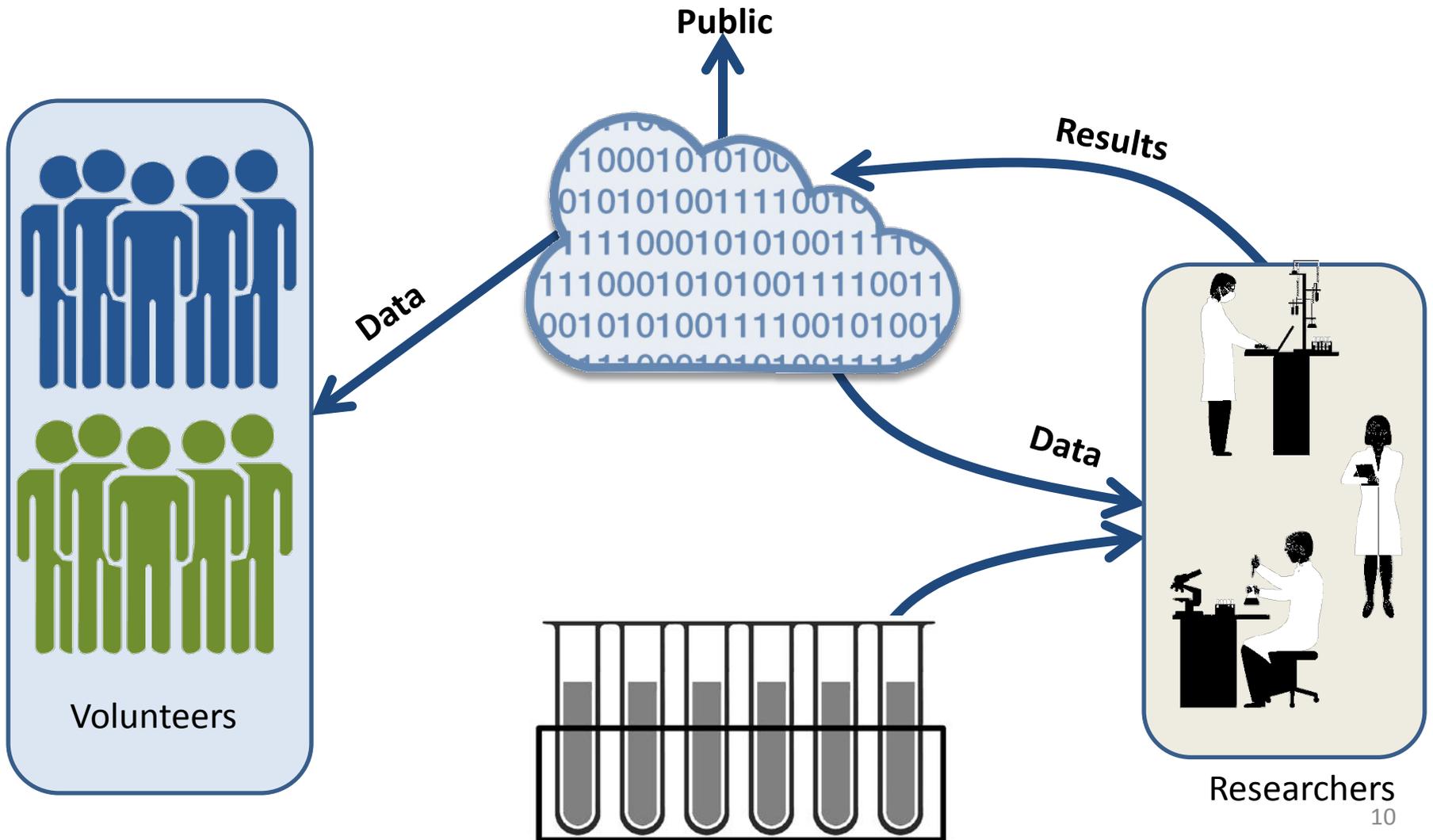
- Centrally collected and stored in a Coordinating Center
- Align with other data sets when possible
- Leverage existing data standards and common data models when possible

Data Source	Data Provided
Self report measures	Diet, substance use, self-report of disease and symptoms (e.g., cognitive or mood assessment)
Baseline health exam	Vitals (e.g., pulse, blood pressure, height, weight), medical history, physical exam
Structured clinical data (EHR)	ICD and CPT codes, medication history, select laboratory results, vitals, encounter records
Biospecimens	Blood sample
mHealth data	Passively-collected data (e.g., location, movement, social connections) from smartphones, wearable sensor data (activity, hours and quality of sleep, time sedentary).

Information Flow In



Information Flow Out



FNIH Survey of public opinion on a large US cohort study

- 79% agree cohort probably/definitely should be done
- 54% would probably/definitely participate in the cohort
- What motivates participation?
 - 82% interested in receiving results of study
 - 62% wish to help advance health research
- 71% said participants should be partners with researchers

PMI Core Values

1. Participation is open to interested individuals
2. Representing the rich diversity of America is essential
3. Participants are partners in all phases of the cohort program
4. Participants have access to study information and data about themselves
5. Data can be accessed broadly for research purposes
6. Adherence to the PMI privacy principles and forthcoming security framework
7. PMI is a catalyst for progressive research programs and policies

ACD PMI Working Group Report

September 17, 2015

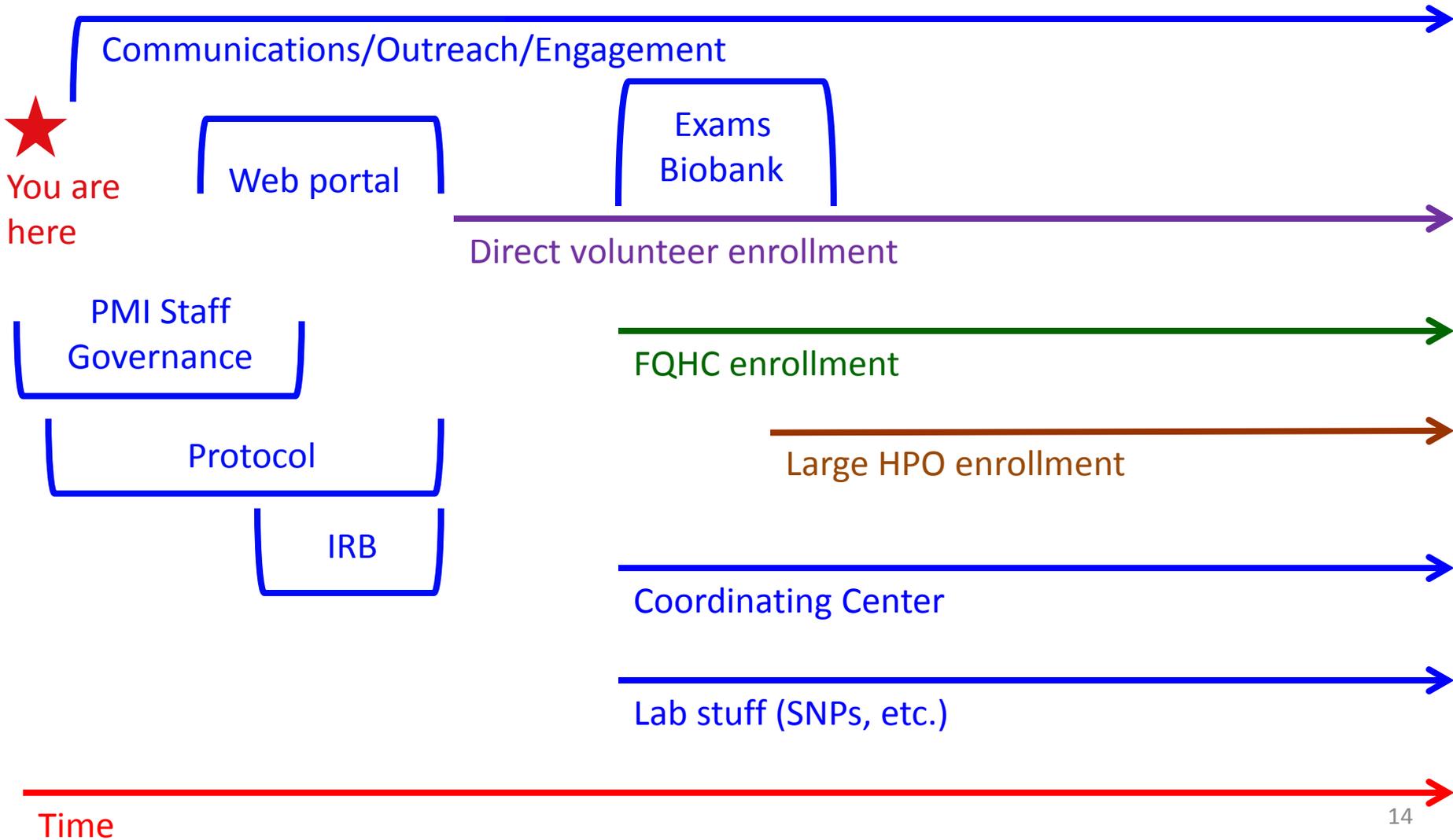


The Precision Medicine Initiative Cohort Program – Building a Research Foundation for 21st Century Medicine

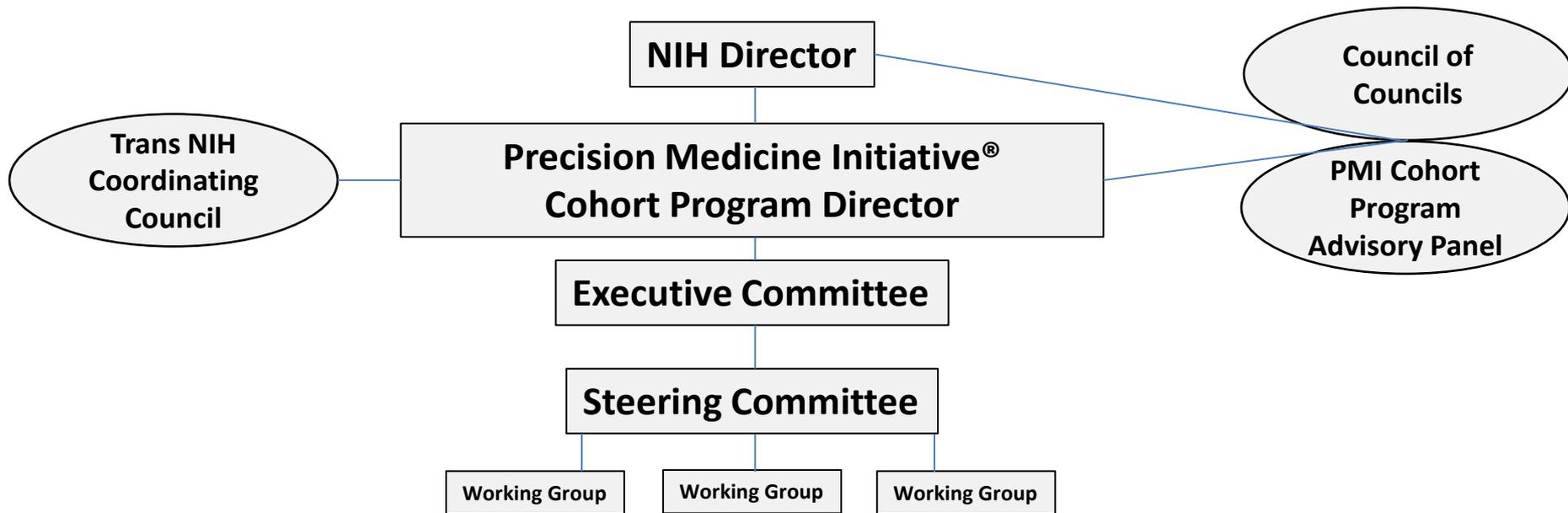
Precision Medicine Initiative (PMI) Working Group Report to the
Advisory Committee to the Director, NIH

September 17, 2015

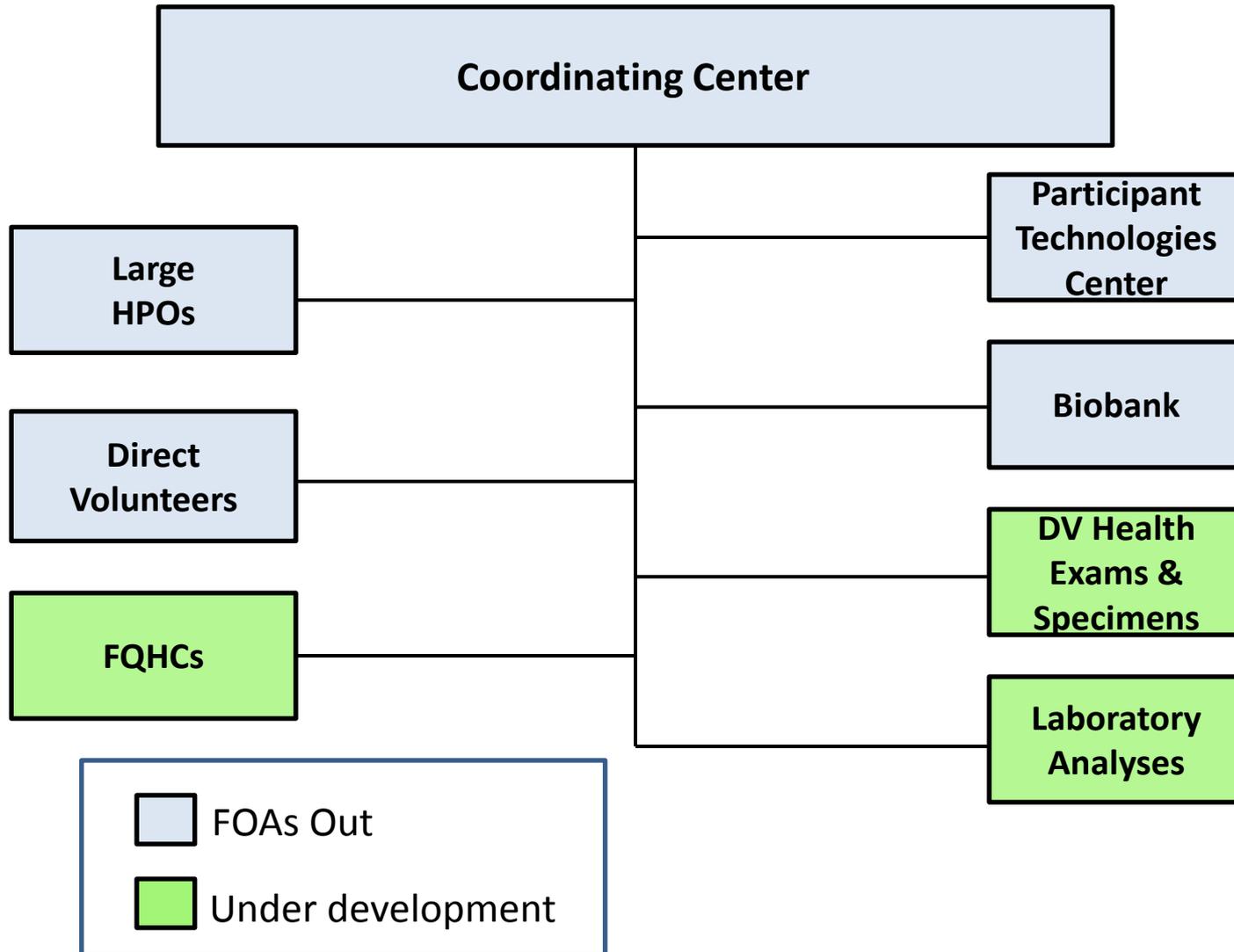
Initial Implementation Ideas



Precision Medicine Initiative® Cohort Program Governance



PMI Cohort Program



Projected Enrollments

Entry point to cohort	Estimated Cumulative Enrollments per Calendar Year			
	2016	2017	2018	2019
HPOs	28,000	196,000	448,000	595,000
Direct volunteers	50,000	150,000	252,000	352,000
FQHCs	<1,000	51,000	101,000	151,000
TOTALS	~79,000	397,000	801,000	1,098,000

PMI Implementation Progress: November 17

1. National search for PMI Cohort Director – closes 12/24
2. Appointment of PMI Cohort Program Advisory Panel
3. Two Other Transaction award announcements
 - Direct Volunteer Pilot
 - Communications and Engagement
4. Four funding announcements for cooperative agreements
 - Healthcare Provider Organizations
 - Coordinating Center
 - Biobank
 - Participant Technologies Center

PMI Cohort Program Advisory Panel

Lon Cardon, Ph.D.

GlaxoSmithKline

Alta Charo, J.D.

University of Wisconsin

Tony Coles, M.D., M.P.H.

Yumanity Therapeutics

Rory Collins, FRS

University of Oxford

Eric Dishman

Intel

Alejandra Gepp, M.A.

National Council of La Raza

Sachin Kheterpal, M.D., M.B.A.

University of Michigan

Marie Lynn Miranda, Ph.D.

Rice University

Bray Patrick-Lake, M.F.S.

Duke University

Dara Richardson-Heron, M.D.

YWCA

Gregory Simon, M.D., M.P.H.

Group Health Research Institute

Sharon Terry, M.A.

Genetic Alliance

David Williams, Ph.D., M.P.H.

Harvard University

Coordinating Center: Administrative Core

- **Scientific & Administrative leadership**
 - Co-chair Steering and Executive Committees
 - Ensure effective transition from pilot phase
 - Coordinate core protocol development
 - Monitor enrollment, retention, and protocol implementation from both DV and HPO participants
- **Direct Volunteer Operations**
 - Point-of-contact for direct volunteers
 - Schedule & track biospecimens and physical evaluations
- **Healthcare Provider Organization (HPO)-related Operations**
 - Ensure effective protocol implementation and consistent patient engagement strategies

Coordinating Center: Data Core

- **General functions**
 - Develop & maintain all shared scientific and management data
 - Develop and oversee implementation of a common data model
 - Establish standards and implement processes for federated data
 - Establish and implement standards for RUID, consent preferences, self-report, clinical & biospecimen data, return of results
 - Oversee all aspects of data security
 - Oversee all aspects of participant privacy protection
- **Health IT Specific Operations**
 - Provide effective interfaces that facilitate integration of data from health IT records both from HPOs and from DV (Blue Button) records

Coordinating Center: Research Support Core

- Establish and oversee secure computing environment
- Define analytical capabilities for data core
- Develop software tools & algorithms for datasets
- Provide all needed researcher-focused services
- PoC for all users at all levels of sophistication to design and implement studies using the PMI Cohort Program datasets and technical issues
- Provide interface to future –omics lab services
- Oversee the development, analysis and quality assessment of cohort-wide lab analysis

Healthcare Provider Organization Enrollment Centers (UG3/UH3)

- Establish structures to enroll participants, including family members and meeting diversity targets
- Establish effective local participant engagement, monitor participant enrollment and retention:
 - UG3: >10K expected enrollees
 - UH3: >35K expected enrollees/yr
- Conduct baseline physical evaluation on all enrolled participants
- Collect baseline biospecimens on all enrolled participants; legacy biospecimens will not be used
- Establish methods to capture complete health care information of all enrolled participants, both ongoing and when possible legacy data
- Develop methods to transmit health care information to CC in standardized format, meeting interoperability standards across the consortium
 - Standards for EHR capture and representation of family health history
 - SNOMED CT and HL7 Version 3

Participant Technologies Center (U24)

- Develop, upgrade mobile applications developed in pilot phase for DV enrollment, supporting their use for entire cohort
- Provide parallel platforms for non-smartphone users (e.g., feature phones, web site)
- Provide scientific leadership and technical expertise for use of mHEALTH technologies across the cohort
 - Develop, pilot and implement use across the cohort of data acquisition from a wide array of potential participant technologies,
 - Devices should include participants own devices, novel sensors and wearable devices
- Test emerging technologies for study deployment, validate, and co-calibrate emerging technologies with existing technologies to ensure continuity of trend data over time

Biobank (U24)

- Provide biospecimen collection kits and mailers
- Receive, process, store, and distribute:
 - Phase 1: receive saliva or blood
 - Phase 2: plasma, serum, RBCs, buffy coats, urine, DNA
- Establish automation of specimen aliquoting, DNA extraction initially; Transition to automated specimen retrieval systems, when it is cost effective
- Set up information systems for sample tracking, coordinating RUIDs with CC as well as other PMI Cohort Program sites
- Establish robust QA and QC, CLIA processes

Direct Volunteers Pilot Studies (OT)

- Develop and test innovative methods and technologies for data collection and management, and participant engagement
 - Website to engage potential volunteers
 - Participant interface optimized to keep participants engaged and return information
 - Pilot expansion of recruitment to family members
 - Data structures ensure the secure collection and sharing
 - Approaches for biospecimen collection

Communication Support (OT)

- Support communication efforts for the PMI research programs at NIH, with particular emphasis on the PMI Cohort Program
 - Communications planning, message and visual identity development
 - Collection and analysis of evaluation metrics.
 - Outreach through a variety of strategies and platforms

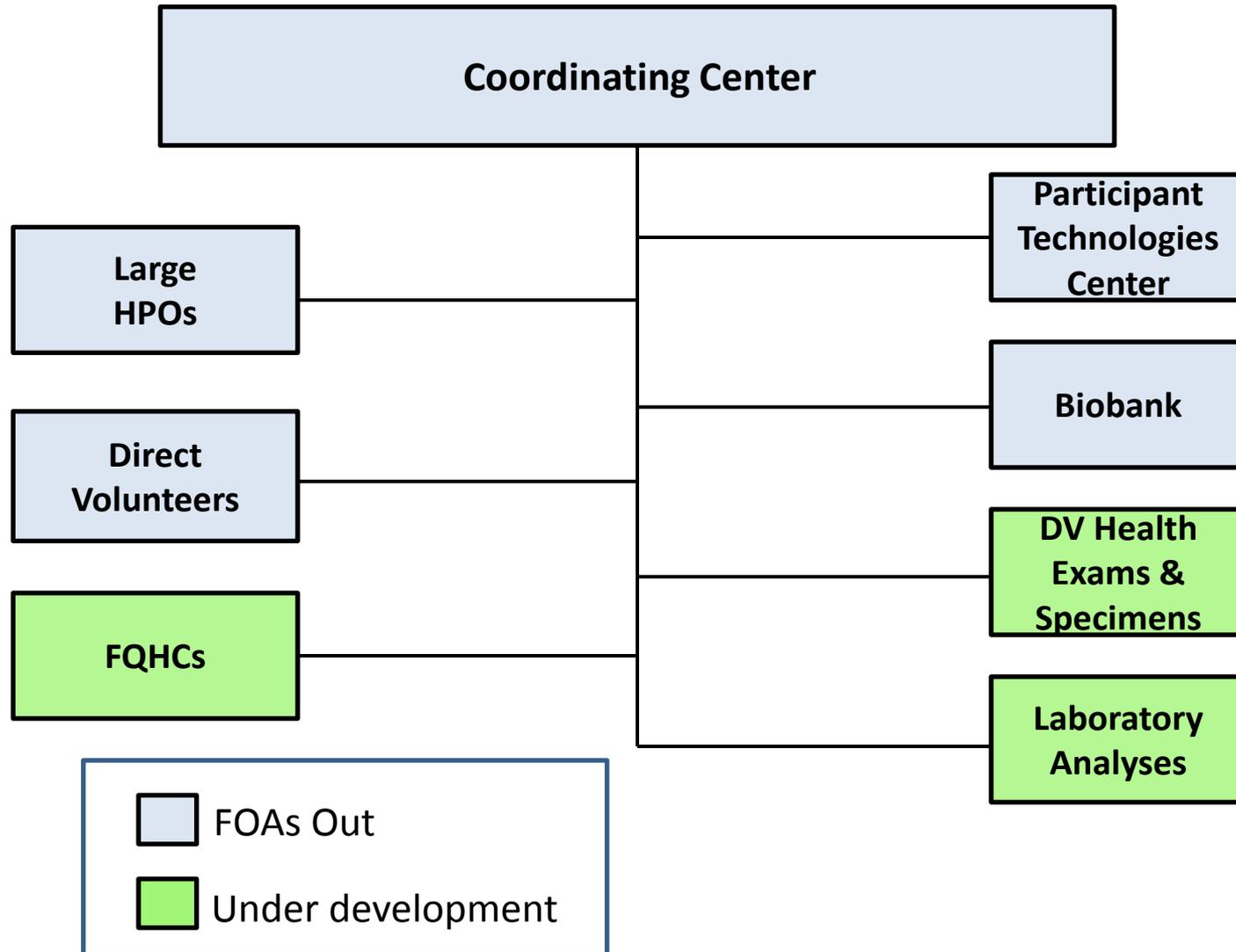
Other Transaction Authority

- NASA, NHS, DARPA, BARDA, etc. have authority
- At NIH, only NHLBI, Common Fund, and NCATS
- Designed to obtain cutting edge technology, often from non-traditional sources, and to allow a high degree of flexibility
- Procurement and grant laws, regulations and policies do not apply

PMI Cohort Program Funding Opportunities

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

PMI Cohort Program



Timeline

2016

Oct Nov Dec Jan Feb Mar Apr May Jun Jul Aug Sep Oct Nov Dec

FOA

Award

Pilot testing

Transition to CC

Direct Volunteer Pilot Phase

FOAs

Applications

Awards

Integrate DV Pilot

Coordinating Center

HPOs, Biobank, Participant Technologies

Explore

Pilot sites

Expansion

FQHCs

Proposed FY16 Budget for PMI

Agency	\$ Million
National Institutes of Health <ul style="list-style-type: none">• <i>PMI for Oncology</i>• <i>PMI Cohort Program</i>	\$200 \$70 \$130
Food and Drug Administration	\$10
Office of the National Coordinator for Health Information Technology	\$5
TOTAL	\$215

Thank you!

