

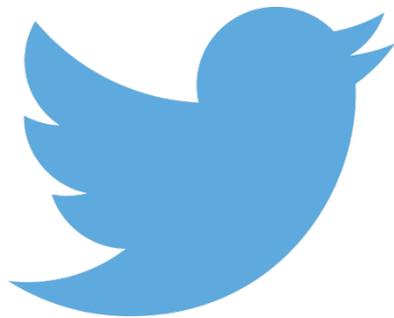
**The Precision Medicine Initiative®**  
**Cohort Program:**  
*An Update*

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Josie Briggs, NICHD Council  
January 21 , 2016

# Follow PMI Cohort Program

[www.nih.gov/precision-medicine-initiative-cohort-program](http://www.nih.gov/precision-medicine-initiative-cohort-program)



#PMINetwork

@NCCIH\_Josie

# Precision Medicine Initiative® Mission

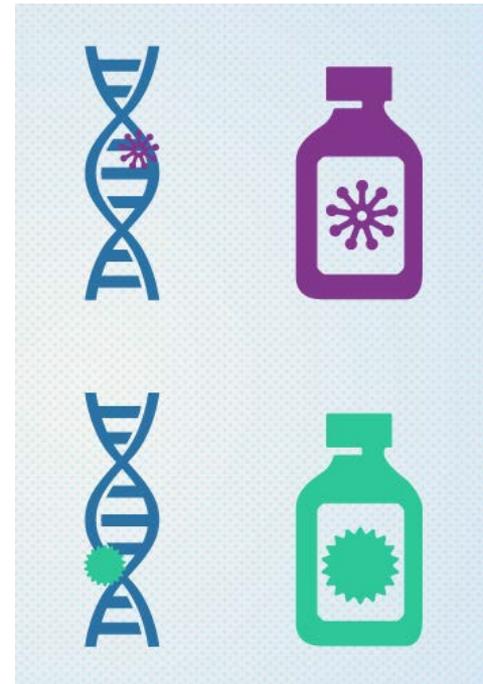
To enable a new era of medicine through research, technology, and policies that empower patients, researchers, and providers to work together toward development of individualized treatments.

# Precision Medicine Initiative®

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

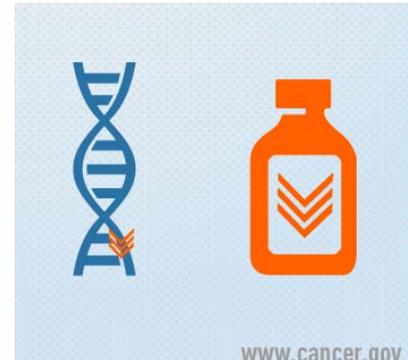
# Promise of PMI: New Treatments

- PMI will result in more therapies like Zelboraf™
  - Targeted to 60% of metastatic melanoma patients whose cancer contains a V600E mutation in the BRAF gene
  - doubled the survival rates in this group
- Kalydeco™ treats cystic fibrosis (CF)
  - Targeted to mutations responsible for 5% of all CF cases
  - The first CF therapy to treat the underlying cause of CF rather than just treating its symptoms
- Benefits of precision medicine can be scaled up to help more people



# Promise of PMI: Pharmacogenomics

- Combine EHR and genetic information to “crowdsource” what Rx works for which patient.
  - **DNA Chip** of all known variants of pharmacogenomic relevance
  - **Provide information** to both patients and clinicians
  - **LOINC** from EHR for Rx – what Rx is written
  - **Pharmacy records** – what Rx is filled
  - **Mine data** for common genetic variants
  - **Learn** what works & what doesn't
  - **Mount an interventional trial** if no one knows if the gene/drug info is really linked
  - **Dramatically expand** existing knowledge
    - FDA counts over one hundred labels with genetic info
- Apply to widely used therapeutics
  - (e.g. aspirin resistance for heart attacks)





## PARTICIPANT PARTNERSHIPS



## EHRs



## TECHNOLOGIES



## GENOMICS



## DATA SCIENCE

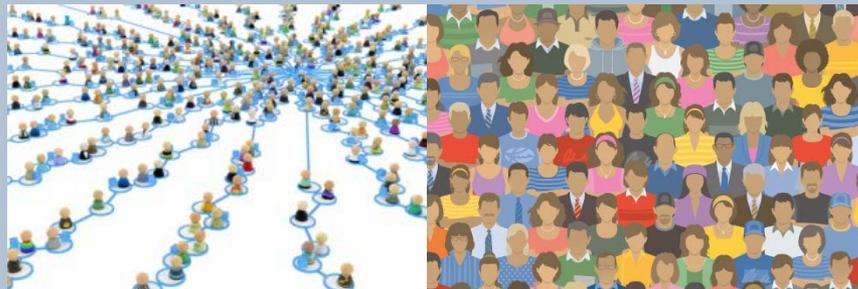
# PARTICIPANTS AT THE CENTER

Long ago, U.S. business learned the benefits of constantly soliciting advice from workers on the shop floor by studying the startling success of the Japanese automobile industry.

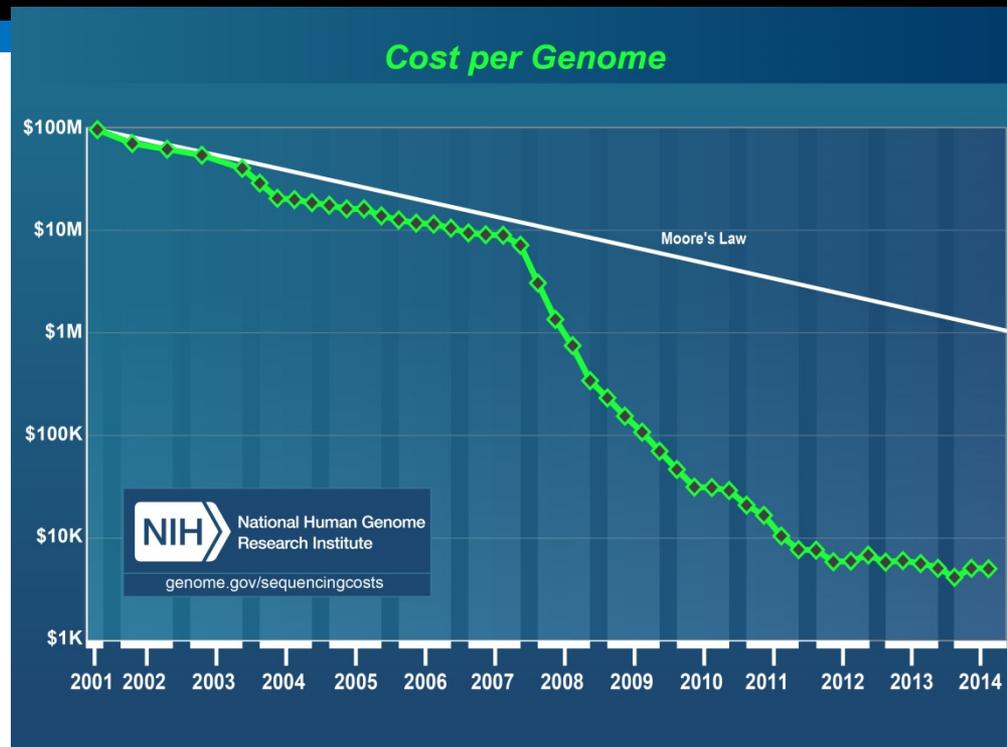
- Bruce Albert
- Science lead editorial
- January 15, 2016

# PARTICIPANTS AT THE CENTER

- Participants engaged in design and oversight
- Participant demands that data sharing include sharing data with THEM
- Participants pushing new consent models
- User centered approach that puts the participant first
- Partnership replacing paternalism



# DNA SEQUENCING POWER



- Human genome can now be sequenced in less than a day for around \$1000-5000
- Whole-exome or whole-genome sequencing of one million Americans or more is now feasible

# mHEALTH ADVANCES

Mobile devices can track increasing amounts of health information

- Blood pressure, pulse rate, connect with devices such as inhalers and spirometers
- Mt. Sinai Asthma Mobile Health Study, together with Apple Healthkit measures:

Symptoms

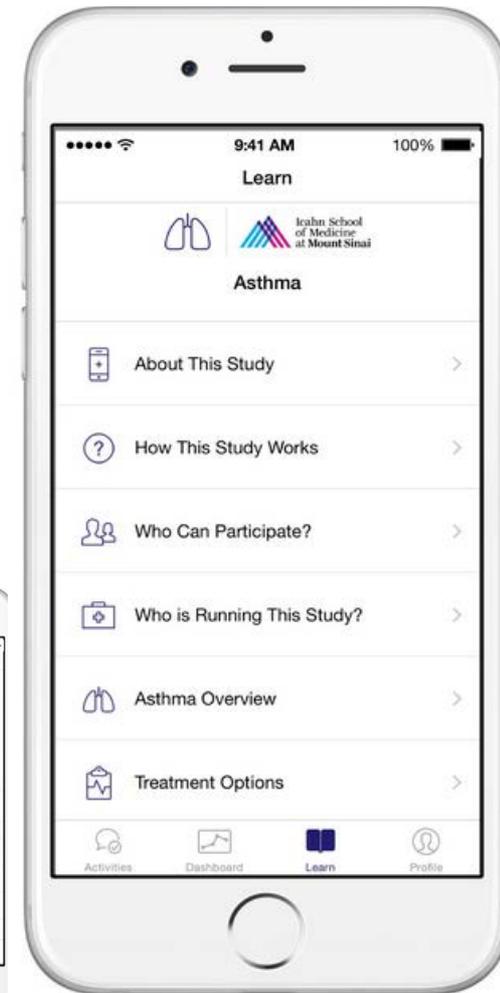
Daily activities

Environmental triggers

Peak expiratory flow

Medications

Health events



# ELECTRONIC HEALTH RECORDS (EHR)

- Now widely adopted
- Offers unique tools for researchers and data mining
- Research use requires agreements on data syntax, semantics, transmission methods, etc with multiple organizations who hold the EHRs



# ELECTRONIC HEALTH RECORDS (EHR)

- Can we put people, not institutions, in control of EHR data sharing?
- Blue-button technology promises to allow patients to download information from their electronic health record
- Promise not yet achieved but perhaps can be accelerated by leadership, resources, and patient demand
- Data could be centralized- truly enabling data science



# 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

# PMI Core Values

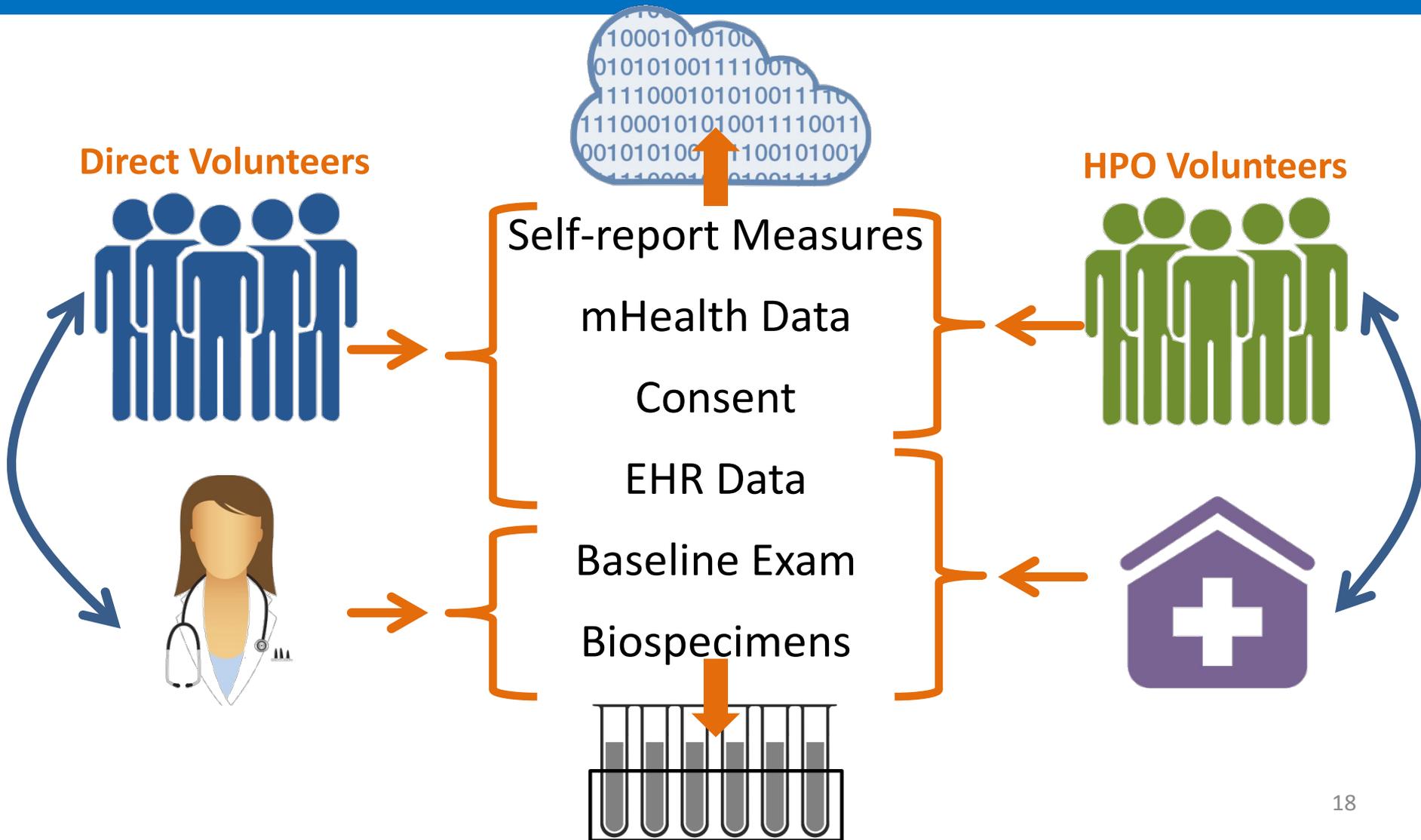
1. Participation is open to interested individuals
2. Representing the rich diversity of America is essential
3. Participants are partners in all phases of the cohort program
4. Participants have access to study information and data about themselves
5. Data can be accessed broadly for research purposes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

# Initial Core Data Set

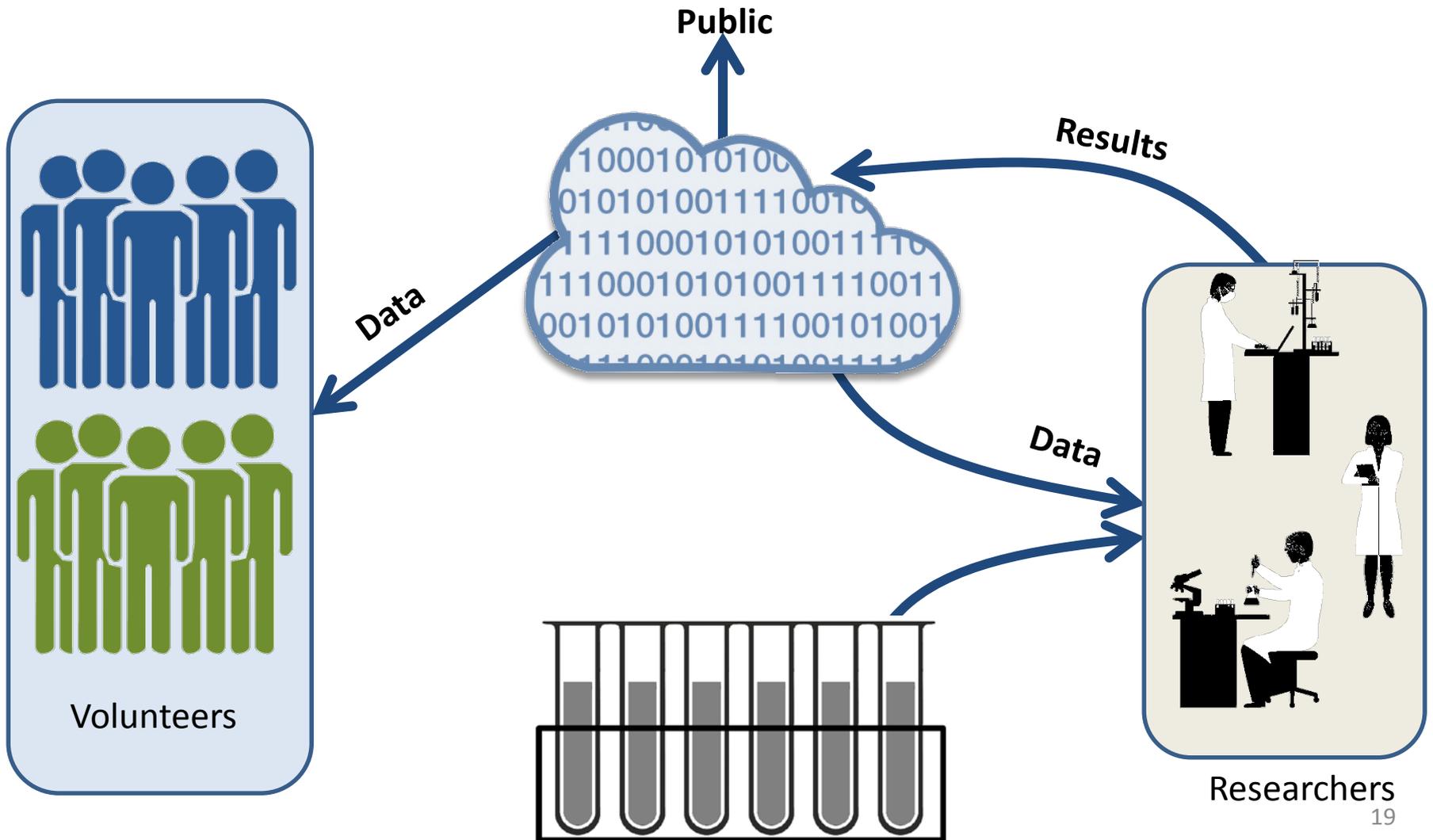
- Centrally collected and stored in a Coordinating Center
- Align with other data sets when possible
- Leverage existing data standards and common data models when possible

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

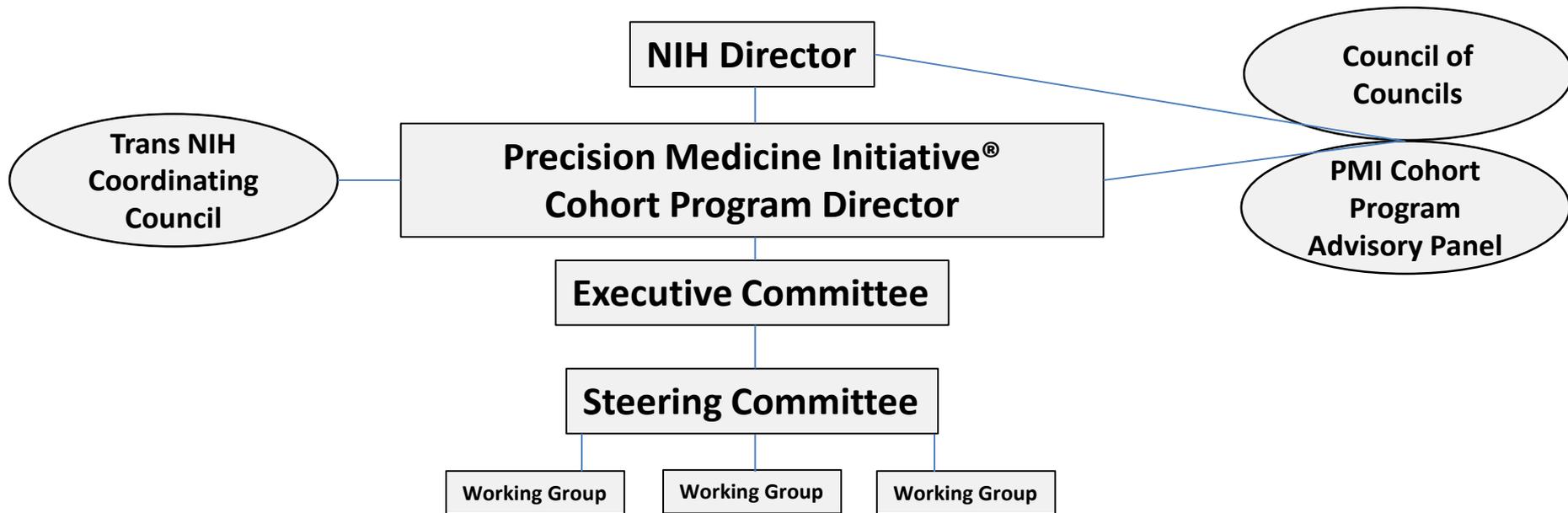


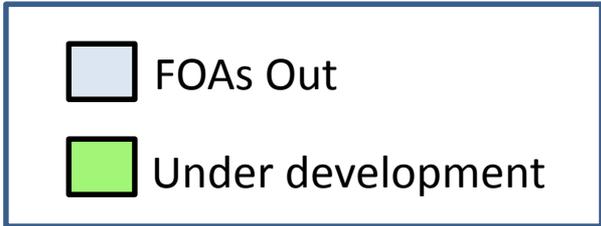
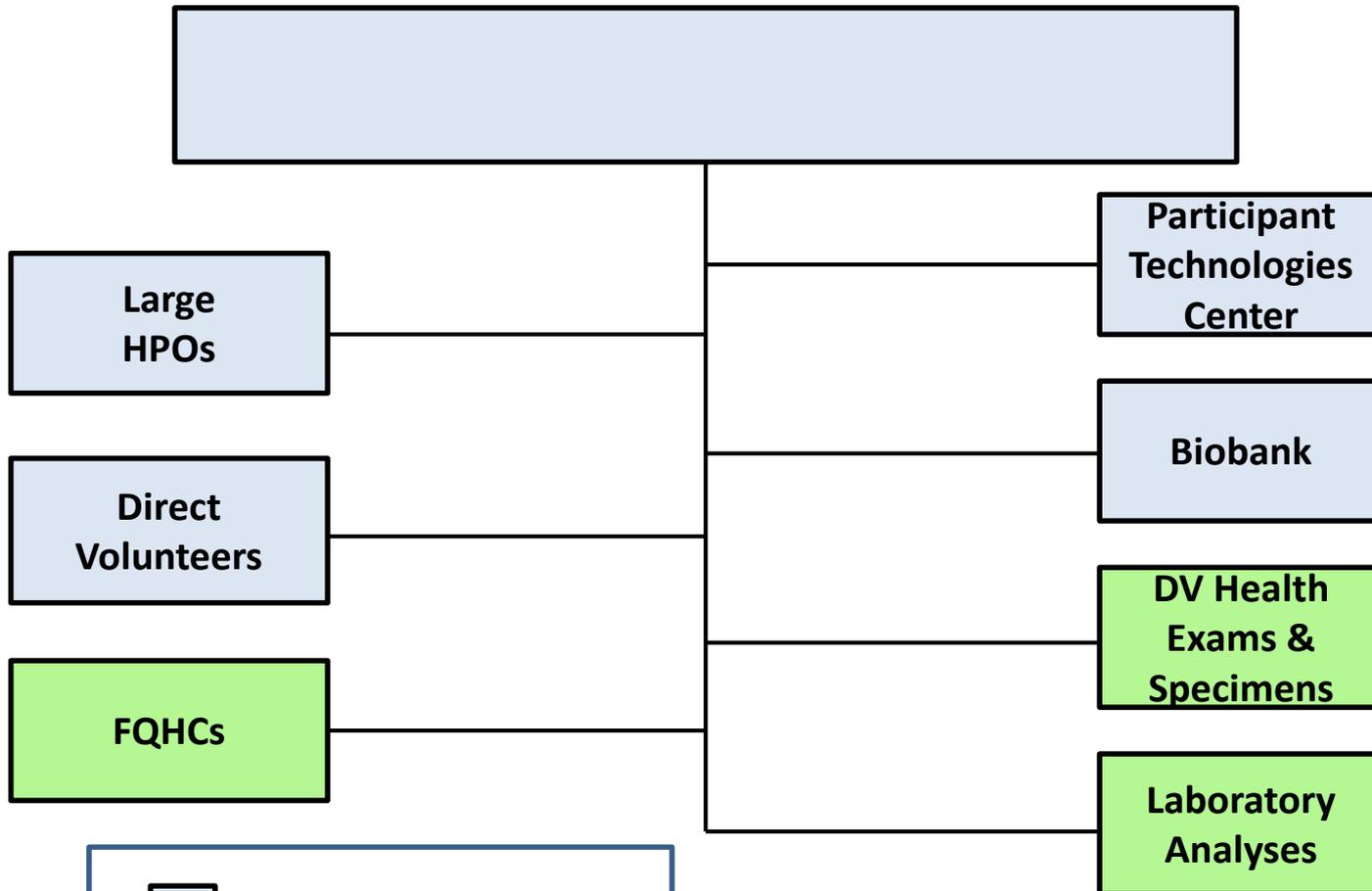
# Information Flow Out



# Precision Medicine Initiative®

## Cohort Program Governance



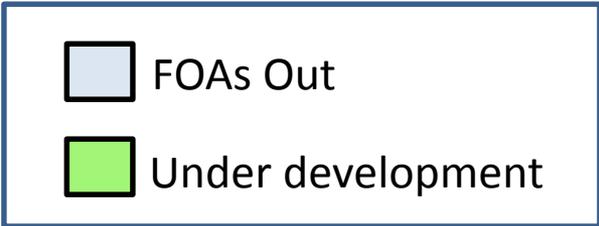
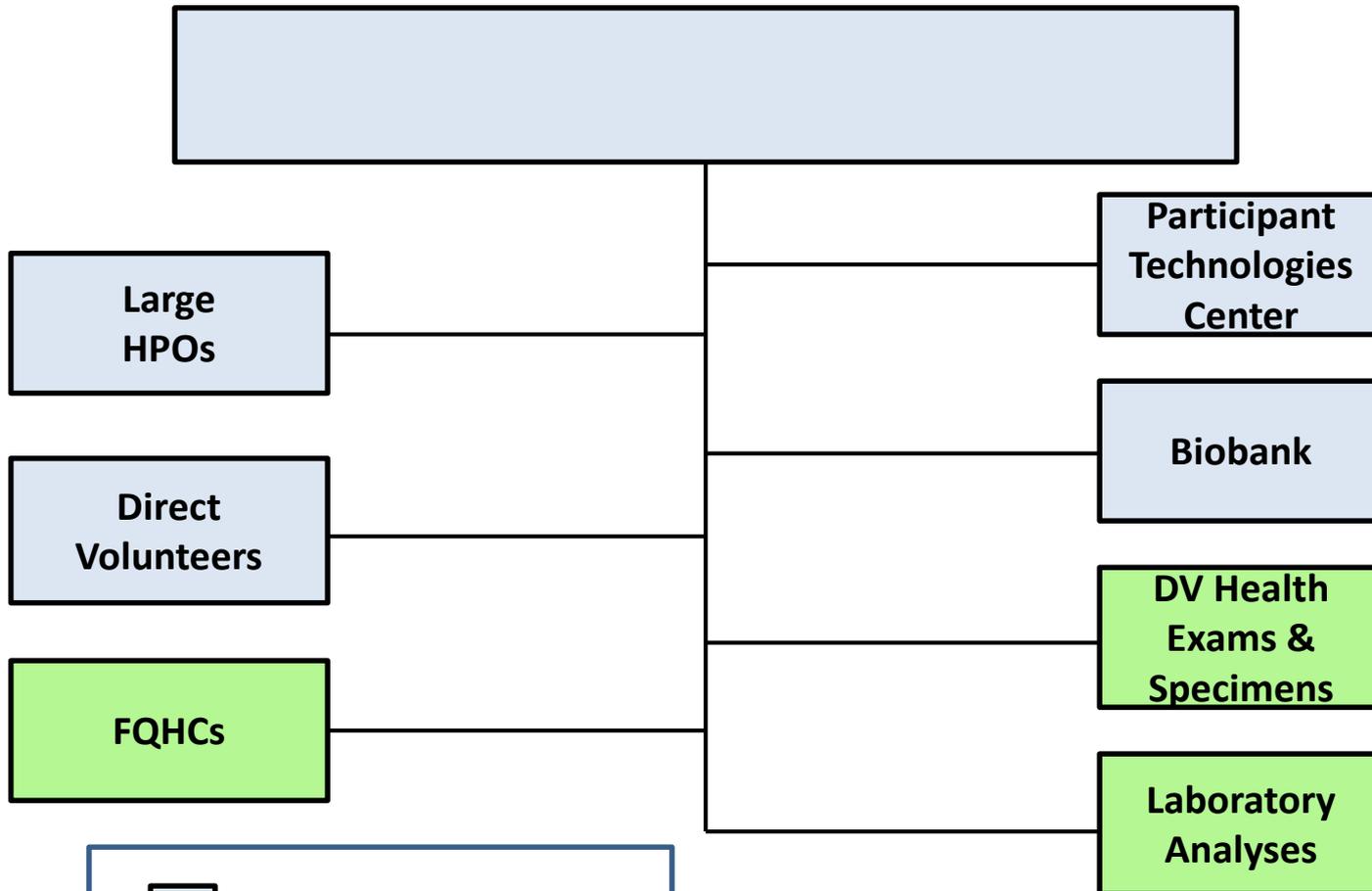


# Projected Enrollments

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
<b>TOTALS</b>	<b>~79,000</b>	<b>397,000</b>	<b>801,000</b>	<b>1,098,000</b>

# 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



# 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"><li>• <i>PMI for Oncology</i></li><li>• <i>PMI Cohort Program</i></li></ul>	\$200 \$70 \$130
Food and Drug Administration	\$10
Office of the National Coordinator for Health Information Technology	\$5
TOTAL	\$215

# Thank you!

