Board of Governors Meeting

In-Person and via Teleconference/Webinar

October 29, 2018
1:00 PM – 5:00 PM ET

pcori
PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE

Welcome and Introductions

Grayson Norquist, MD, MSPH

Chairperson, Board of Governors

Joe Selby, MD, MPH

Executive Director

Agenda

1:00 PM	**Call to Order and Welcome**
1:00 – 1:05	**Consider for Approval:** Minutes of the September 18, 2018 Board Meeting
1:05 – 2:00	**Executive Director's Report**
2:00 – 3:15	**Transforming Research:** • The Future of Health Research • PCORnet – ADAPTABLE Update
3:30 – 4:00	**Consider for Approval:** FY2019 Funding Commitment Plan
4:00 – 4:30	**Strategic Plan Update:** Efforts to Address Emerging Interventions
4:30 – 5:00	**Public Comment**
5:00 PM	**Wrap up and Adjournment**

Welcome New PCORI Board of Governors Members

Kara Ayers, PhD
Assistant Professor, Department of Pediatrics, University of Cincinnati

Jennifer DeVoe, MD, DPhil
Professor and Chair, Department of Family Medicine, Oregon Health and Science University

Christopher Friese, PhD, RN
Elizabeth Tone Hosmer Professor of Nursing, University of Michigan

Trent Haywood, MD, JD
Senior Vice President, Office of Clinical Affairs and Chief Medical Officer, Blue Cross Blue Shield Association

Michael Herndon, DO
Chief Medical Officer, Oklahoma Health Care Authority

Michelle McMurry-Heath, MD, PhD
Worldwide Vice President for Global Regulatory Affairs, International Clinical Evidence and Strategic Operations for Medical Device Companies, Johnson & Johnson

Janet Woodcock, MD
Director, Center for Drug Evaluation and Research, Food and Drug Administration

Board Vote

Call for a Motion to:	• **Approve** the Minutes of the September 18, 2018 Board Meeting
Call for the Motion to be Seconded:	• **Second** the Motion • If further discussion, may propose an **Amendment** to the Motion or an **Alternative** Motion
Voice Vote:	• Vote to **Approve** the **Final** Motion • Ask for votes in favor, opposed, and abstentions

Executive Director's Report

Joe Selby, MD, MPH

Executive Director

New Vice Chairperson Named to PCORI Board of Governors

Christine Goertz, DC, PhD

Named by Government Accountability Office as new Vice Chairperson of the PCORI Board of Governors

2018 Annual Meeting

From Evidence to Impact: Putting What Works Into Action

Details

October 31 – November 2
Marriott Wardman Park Hotel
Washington, DC

Focus

Spotlight results from dozens of PCORI-funded patient-centered clinical effectiveness research studies and explore opportunities to **use this evidence** in ways that **patients and other healthcare stakeholders** find most useful

Agenda

Two **keynotes**, five **plenaries,** and nine **breakout sessions**, **workshops**, and **poster session**

Sessions

Range of sessions focusing on topics such as making **health care more efficient by making it more patient-centered, interpreting "negative studies";** addressing the opioid epidemic and improving pain management; and the **impact of engagement** on research

Attendees

Expecting 1000+ members of the PCORI Community to attend including researchers, patients, caregivers, clinicians, employers, insurers, staff & others

AHRQ and PCORI Announce Learning Health System Traineeship

- The jointly funded five-year, $40 million initiative between PCORI and the Agency for Healthcare Research and Quality (AHRQ) is designed to train the next generation of PCOR professionals to lead research within institutional training programs
- The 11 LHS Centers of Excellence funded under this initiative will produce the next generation of LHS researchers to conduct PCOR and implement the results to improve quality of care and patient outcomes
- 40 trainees in year one, estimated 92 over the 5 years

LHS K12 Mentored Career Development Program

The LHS Centers of Excellence are

- **A Chicago Center of Excellence in Learning Health Systems Research Training (ACCELERAT)**
  Northwestern University, Chicago, Ill.
- **CATALyST: Consortium for Applied Training to Advance the Learning Health System with Scholars/Trainees**
  Kaiser Permanente Washington Health Research Institute, Seattle, Wash.
- **Learning Health System Scholar Program at Vanderbilt**
  Vanderbilt University, Nashville, Tenn.
- **Learning Health System (LITI- PCORLHS)**
  Indiana University School of Medicine, Indianapolis, Ind.
- **Leveraging Infrastructure to Train Investigators in Patient-Centered Outcomes Research in the Minnesota Learning Health System Mentored Career Development Program (MN-LHS)**
  University of Minnesota, Minneapolis, Minn.
- **NW Center of Excellence & K12 in Patient Centered Learning Health Systems Science**
  Oregon Health and Science University, Portland, Ore.
- **PEDSnet Scholars: A Training Program for Pediatric Learning Health System Researchers**
  Children's Hospital of Philadelphia, Philadelphia, Pa.
- **Stakeholder-Partnered Implementation Research and Innovation Translation (SPIRIT) program**
  University of California, Los Angeles, Los Angeles, Calif.
- **The Center of Excellence in Promoting LHS Operations and Research at Einstein/Montefiore (EXPLORE)**
  Albert Einstein College of Medicine, Bronx, N.Y.
- **Transforming the Generation and Adoption of PCOR into Practice (T-GAPP)**
  University of Pennsylvania, Philadelphia, Pa.
- **University of California, San Francisco Learning Health System K12 Career Development Program**
  University of California, San Francisco, San Francisco, Calif.

Recent Recognitions

- PCORI has received recognition for its major investments in palliative care research by two stakeholder organizations, the Coalition to Transform Advanced Illness Care (C-TAC) and the American Academy of Hospice and Palliative Medicine (AAHPM)

- **2018 Champion's Award** presented by C-TAC, and **AAHPM Presidential Citation Award** presented by AAHPM, recognize PCORI's leadership and significant contributions to support and develop the research field of advanced illness care

PCORI's Palliative Care Portfolio **Snapshot**

- **2012-present: Funded 28 investigator initiated studies (> $75M)**
  - Models of care delivery (n=7)
  - Communication and shared decision making (n=12)
  - Symptom management and/or reducing caregiver burden (n=9)
- **2017: Funding Announcement: "Community-Based Palliative Care Delivery for Adult Patients with Advanced Illnesses and their Caregivers"**
  - ~$74M, 3 Advance Care Planning studies, 4 Models of Care studies
- **2018: Funding Announcement: "Symptom Management for Patients with Advanced Illness"**
  - Funds committed: $25M; 1 award in round one, second round ongoing

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Dashboard Review

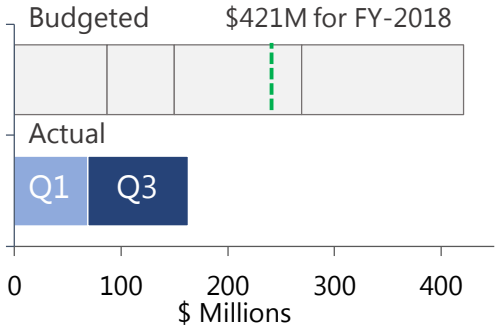
Third Quarter of FY-2018

PCORI Board of Governors Dashboard
Third Quarter FY-2018 (As of 6/30/2018)

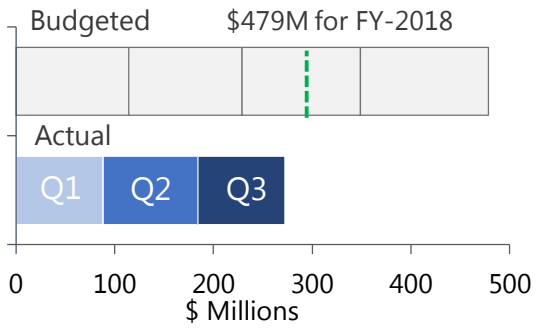
Dashboard Key

- Projected
- Q3 Target
- Meeting Target
- Off Target
- Needs Board Attention
- Target in Development
- Q4 2017
- Q1 2018
- Q2 2018
- Q3 2018

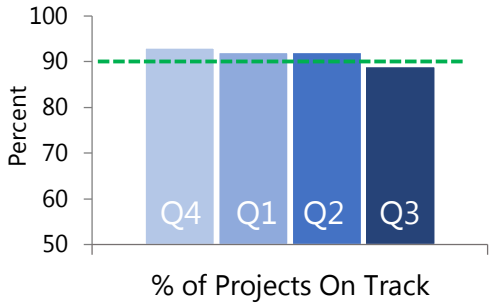
Funds Committed to Research



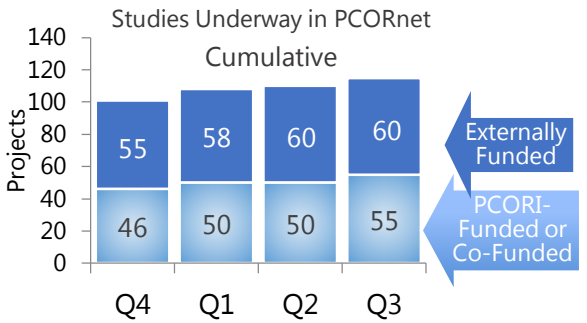
Expenses



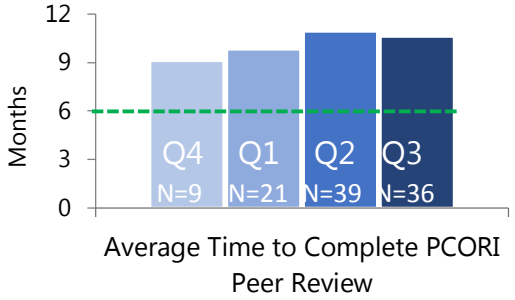
Project Performance



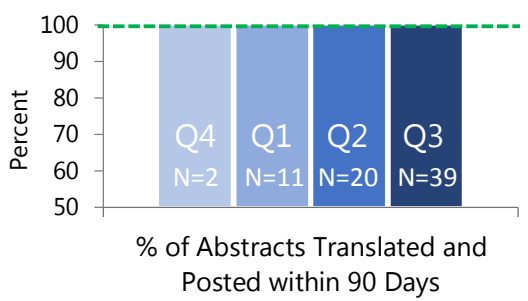
Research Using PCORnet



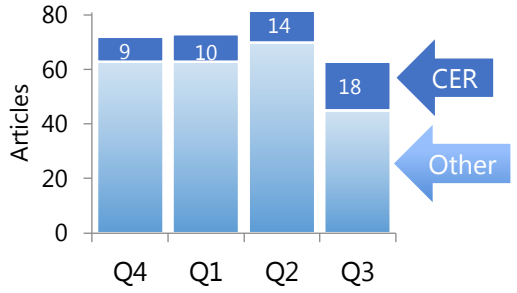
Speed of PCORI Peer Review



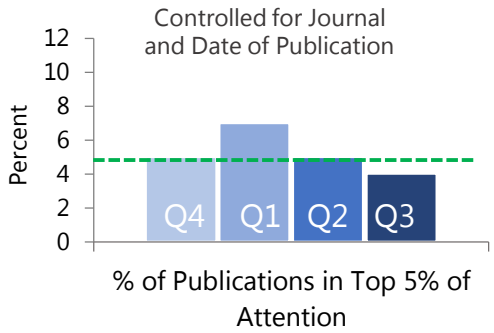
PCORI Abstracts Posted to Website



Results Published in Literature



Altmetric: Attention to PCORI Research



Featured Results on PCORI's Strategic Goals

Results on Goal 1

Increasing Information

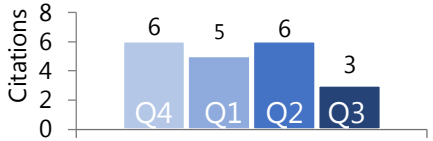
Results Highlight: A PCORI-funded study of treatments for serious mental illness compared a smartphone-based vs. a clinic-based intervention: participants in the smartphone arm were more likely to start treatment and remain engaged at 3 months.

Results on Goal 2

Speeding Uptake

As of Q3, results from PCORI studies have been cited 25 times in evidence-based clinical recommendations

Uptake Highlight: Results from a PCORI-funded study of community health workers supporting veterans were selected for an implementation award at the 2018 Veterans Health Administration Shark Tank Competition



Early Uptake of Findings (citations in evidence-based clinical recommendations)

Results on Goal 3

Influencing Research

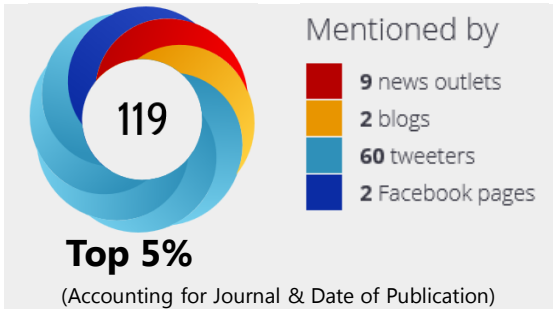
PCORI is credited with influencing a new approach to engagement of patients, knowledge users, and scientists in reviewing applications from competitive research calls at the Ontario Ministry of Health and Long-Term Care (MOHLTC)

Goal 1: Results of PCORI-Funded Research

Smartphone-Based Treatment Can Improve Access for People with Serious Mental Illness

PCORI Study	**Study Title:** Comparing Mobile Health and Clinic-Based Self-Management Interventions for Serious Mental Illness: Patient Engagement, Satisfaction, and Outcomes **Principal Investigator:** Dror Ben-Zeev, PhD, University of Washington
Results Publication	Ben-Zeev D, Brian RM, Jonathan G, et al. **Mobile Health (mHealth) Versus Clinic-Based Group Intervention for People With Serious Mental Illness: A Randomized Controlled Trial.** *Psychiatr Serv. May 2018.*

Altmetric Score (Attention)



Summary: Mobile health approaches that use smartphones to deliver interventions can help improve access to care for people with serious mental illness (SMI). This three-month randomized trial compared a smartphone-delivered intervention vs. a traditional, clinic-based peer-led group intervention

Both interventions produced significant gains among people with SMI who were mostly from racial minority groups. Participants in the **smartphone based intervention group were significantly more likely to start treatment (90% versus 58%)** and have continued engagement after 8-weeks. The two groups had similar rates of clinical outcomes and treatment satisfaction, and similar rates of continued engagement at 12-weeks

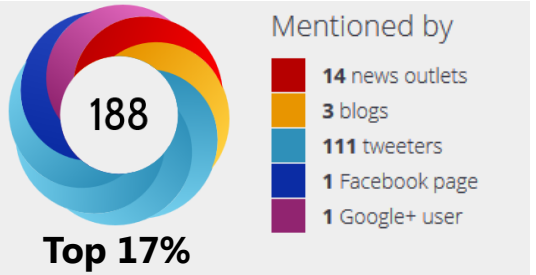
> This study shows that we may lose close to half of our patients when we ask them to come to a clinic. ***This mobile approach was not only more accessible to them, clinically it was as helpful as group sessions*** conducted in person.
>
> -Dr. Dror Ben-Zeev, Principal Investigator
> University of Washington News Release

Goal 1: Results of PCORI-Funded Research

Less-Frequent Colorectal Cancer Surveillance Does not Compromise Long-Term Outcomes

PCORI Study	**Study Title:** Patient-Centered, Risk-Stratified Surveillance After Curative Resection of Colorectal Cancer **Principal Investigator:** George J. Chang, MD, MS, The Alliance for Clinical Trials in Oncology Foundation
Results Publication	Snyder RA, Chung-Yuan H, Cuddy A, et al. **Association Between Intensity of Posttreatment Surveillance Testing and Detection of Recurrence in Patients With Colorectal Cancer.** *JAMA*. May 2018.

Altmetric Score (Attention)



Summary: For patients who have surgery to remove colorectal tumors, reoccurrence is a major concern. This retrospective cohort study compared healthcare facilities that do more-frequent (every 6 months) to those that do less-frequent (at 12 and 36 months) surveillance

The study found that facilities that do more-frequent surveillance did not detect recurring cancers any sooner than those that do less-frequent surveillance. They also found **no link between the intensity of surveillance and overall survival, suggesting more-frequent surveillance provided no health benefit**

This article was published in *JAMA* alongside the results of a randomized trial on frequency of colorectal cancer surveillance, and reported similar results. Both studies found that lower frequency surveillance does not compromise long-term outcomes

> In addition to added costs, unnecessary testing in cancer patients can lead to treatment toxicity, increased patient anxiety, and the potential for false positives, which can lead to patient harm… The data argue that in many cases, ***a less-intensive surveillance may be a better approach for patients***.
>
> -Dr. George Chang, Principal Investigator
> MD Anderson News Release

Goal 2: Speed Uptake and Use of Information

Project Awarded to Implement Findings from a PCORI-funded Study

Summary: In an effort to facilitate standardization across the VA healthcare system, VA's Diffusion of Excellence Initiative, the VHA Shark Tank Competition, identifies promising field-developed practices for implementation at chosen site(s), and some practices are later selected for national rollout across the VA healthcare system

A PCORI-funded study found that a standardized community health worker program (IMPaCT) was effective at improving quality of care and reducing hospitalizations among veterans with chronic illness. The **IMPaCT intervention was awarded a 2018 Diffusion of Excellence award**, **and will be implemented within the Iowa City Health Care System**. There is a plan in development for how **the VA will evaluate the impact of the practice** before further implementation

PCORI Study title: Effectiveness of Collaborative Goal-Setting Versus IMPaCT Community Health Worker Support for Improving Chronic Disease Outcomes

Principal Investigator: Judith A. Long, MD, University of Pennsylvania

Diffusion of EXCELLENCE
Diffusing Best Practices Across VHA

Goal 3: Influence Research

PCORI Stakeholder-Engaged Approaches Adopted by Other Funders

PCORI participated in and informed a new approach undertaken by the Ontario Ministry of Health and Long-Term Care (MOHLTC) to engage patients alongside knowledge users and scientists in reviewing applications from a competitive research call

The MOHLTC identified an opportunity to move towards collaborative research that would accelerate patient-oriented health system learning by driving greater connectivity between researchers, patients and health system partners

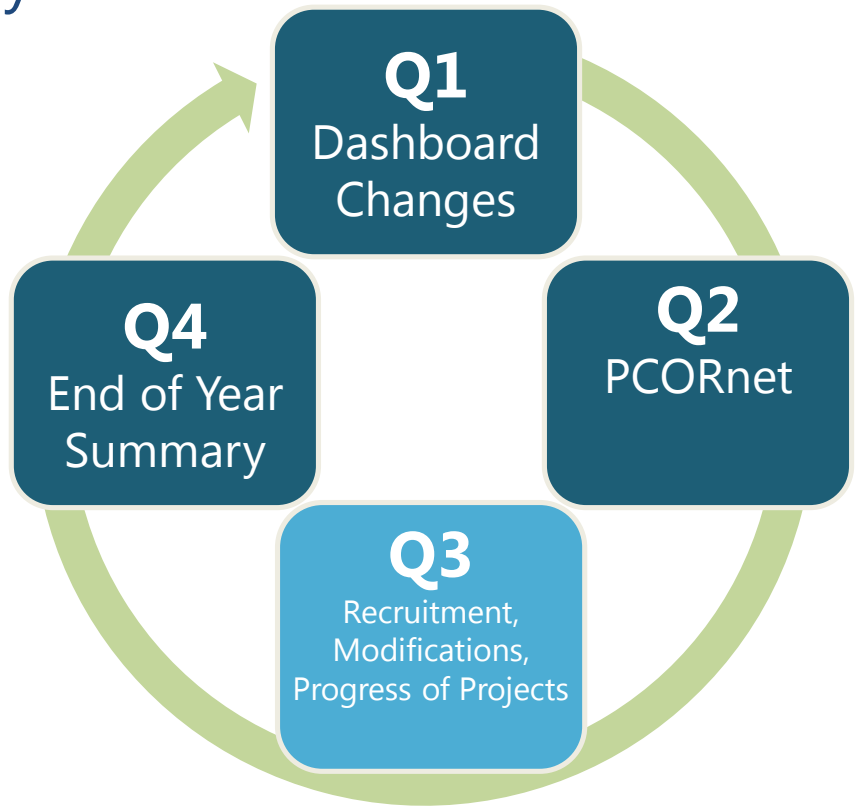
- Sought process-oriented guidance as well as reviewer training and evaluation resources from PCORI with regards to integrating patients and other stakeholders in the competitive research call adjudication process
  - **Restructured review panel** first launched with the targeted call for patient-oriented research in mental health and addictions
  - Health System Research Fund (HSRF) competitive research calls now use a **more comprehensive multi-stakeholder review process** that brings together scientific peer-reviewers with people with lived experience and health system knowledge users who respond to complementary review criteria

> *… It's been a really positive experience …We are planning to repeat the process again in our next research call.*

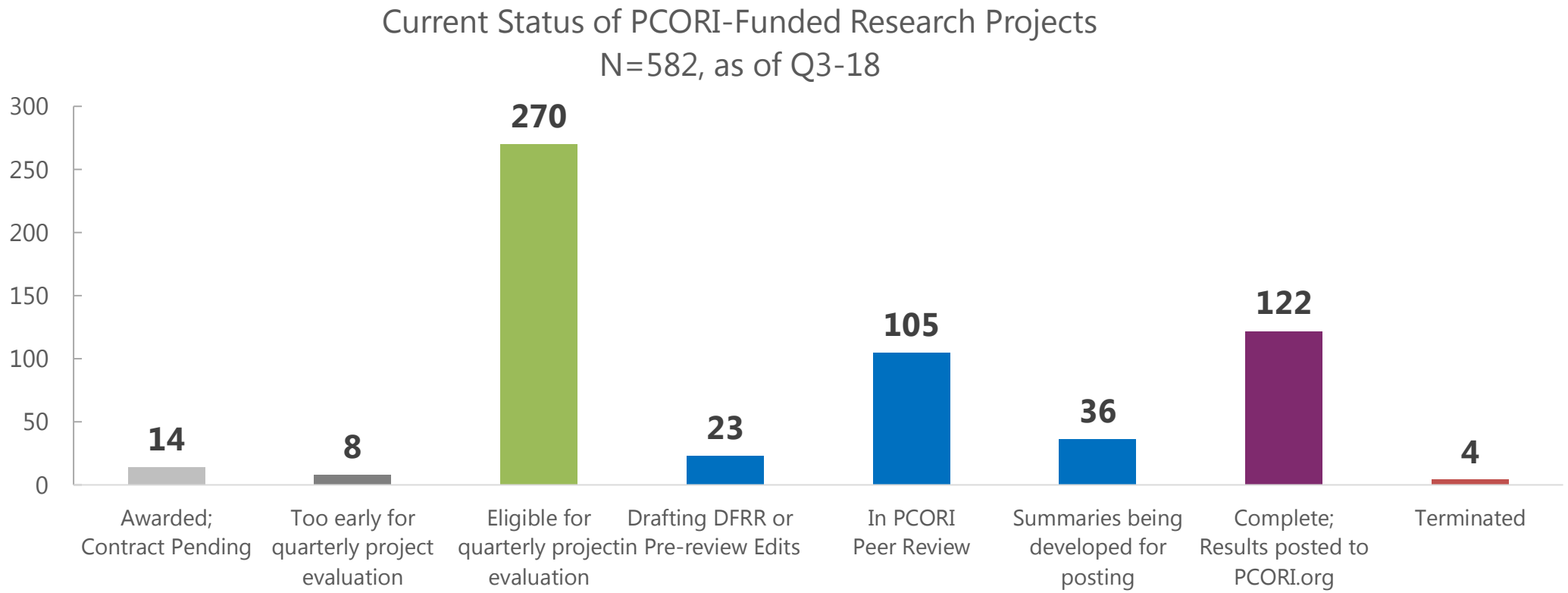
[Erika Runions MacNeil; Lisa Brown; Anne Hayes; *Ontario Ministry of Health and Long-Term Care* [July 2018]]

Our Q3 quarterly focus is on the progress of projects with an in-depth focus on study recruitment

- In addition to focusing on any Dashboard items that are noteworthy, off target, or in need of attention, we provide a consistent in-depth focus in each quarter for items that are top priority



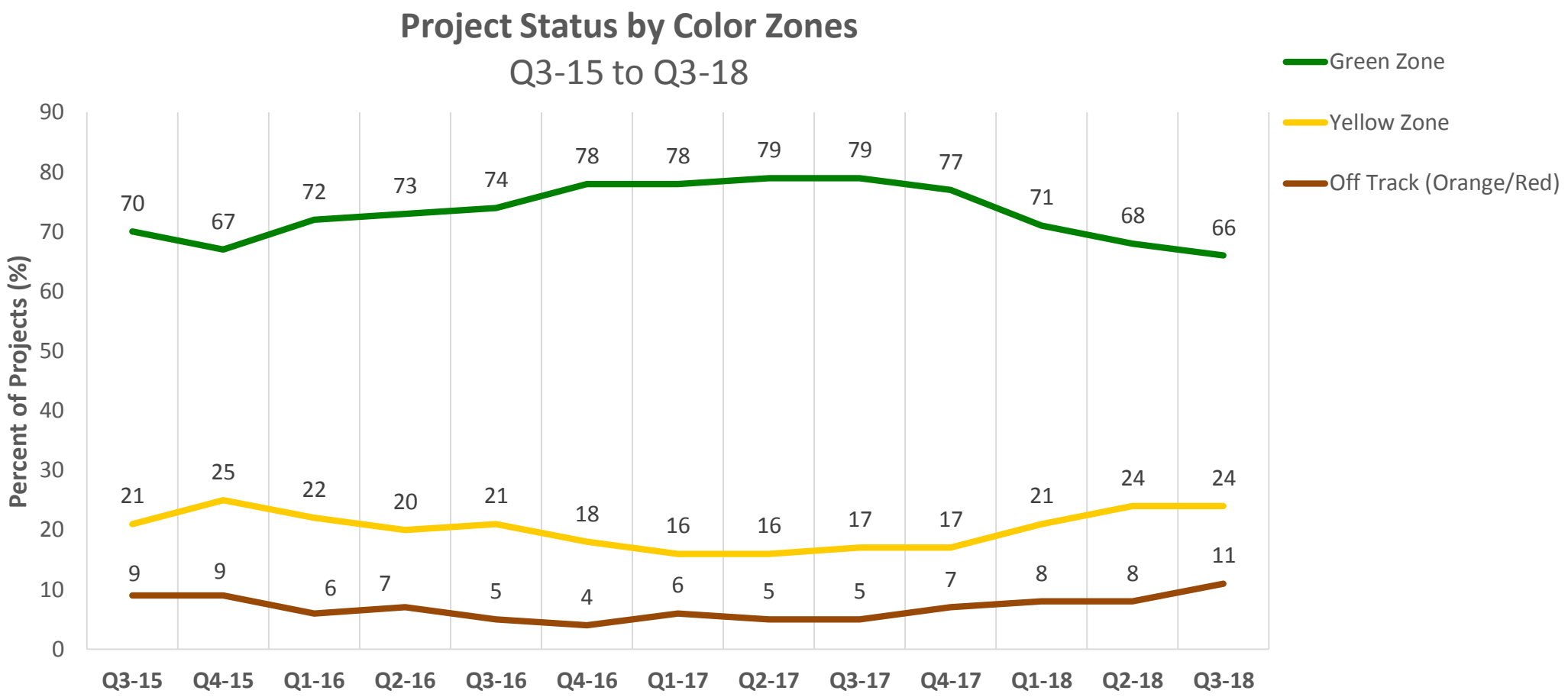
Half of our funded research projects are entering PCORI Peer Review or have been completed, with their results posted to PCORI.org



*Does not include Infrastructure or D&I awards
*Does not include studies awarded in Q3-2018

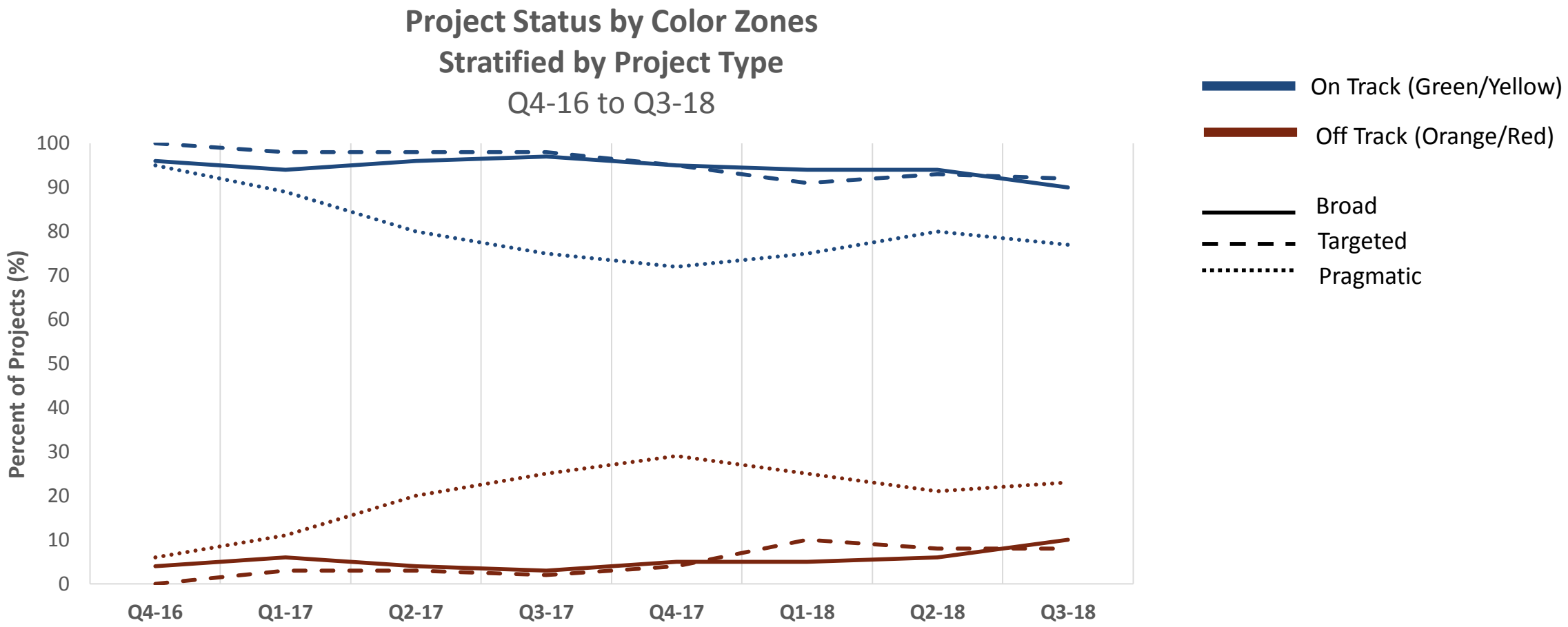
The majority of our funded research projects are on track, but the proportion off track has increased slightly over the past year – we continue to closely monitor all projects

We are monitoring trends and shifts in project status



Pragmatic Clinical Studies, which tend to be larger, longer, and more complex, are more likely to be off track

We are monitoring trends and shifts in project status



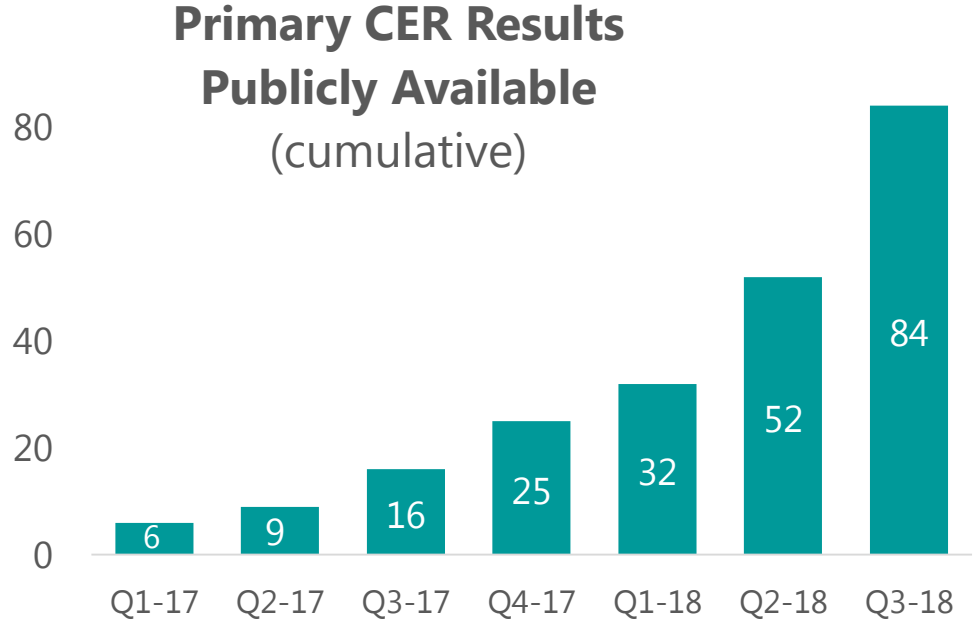
The primary results of over 100 PCORI-funded CER studies are available to the public and many more are coming soon

As of Q3-18, **84 CER Studies have their primary results peer-reviewed and publicly available,** and this number is steadily increasing

- **Primary results** are results that report on a comparison of clinical approaches using the pre-specified primary outcome(s). Also commonly referred to as primary publications, or Public Disclosure of Results (PDOR).
- Primary results can be **made publicly available** by being published in a peer-reviewed journal, and/or by completing the PCORI Peer Review Process and having their abstracts posted to PCORI.org

Of the 84 CER studies with primary results:

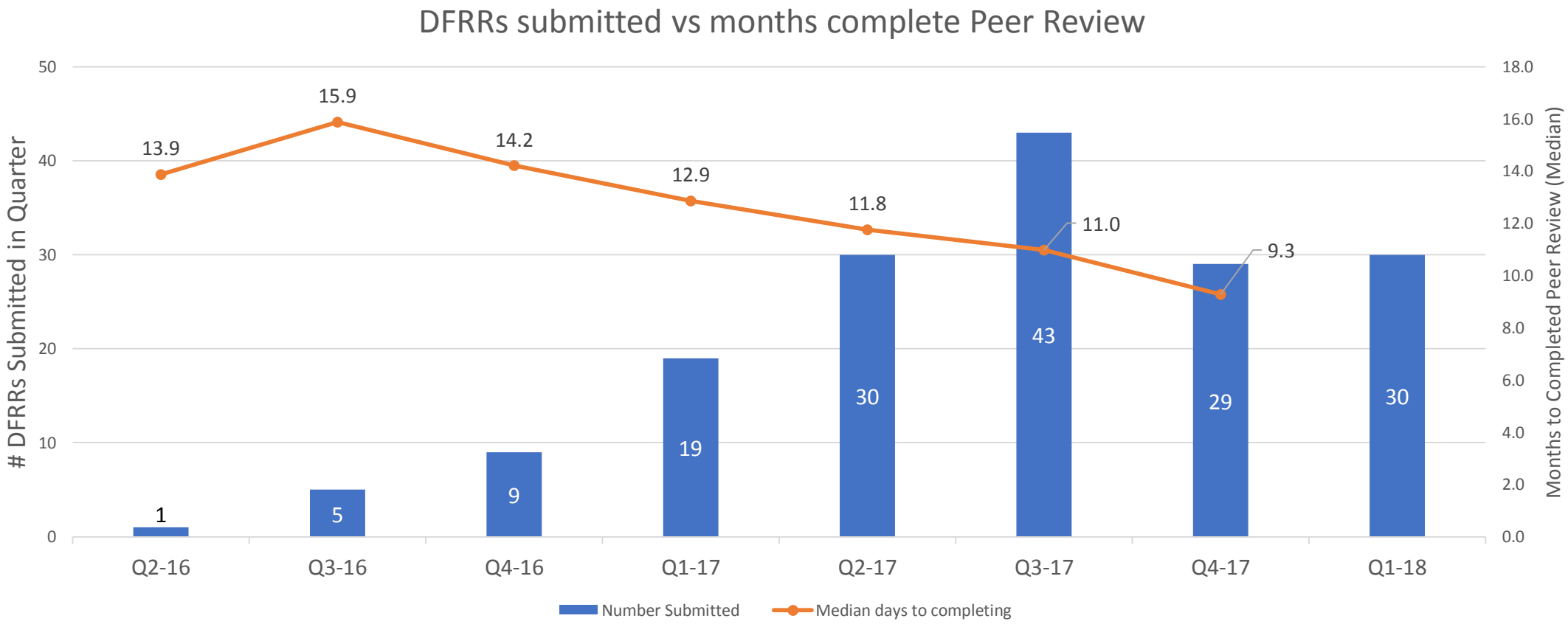
- 46 (55%) were first made available via publications
- 38 (45%) were first made available via PCORI.org



Update:
As of 10/24/18, **123 CER studies** have publicly available primary results, and at least 2 more results papers are in-press

Due to experience and process improvements, the speed of PCORI Peer Review is steadily increasing

Improvements in median time to complete Peer Review can be seen when grouping studies by quarter of DFRR submission (cohort view).



Early results from our initial cohorts indicate that PCORI-funded studies are doing better on recruitment than available benchmarks

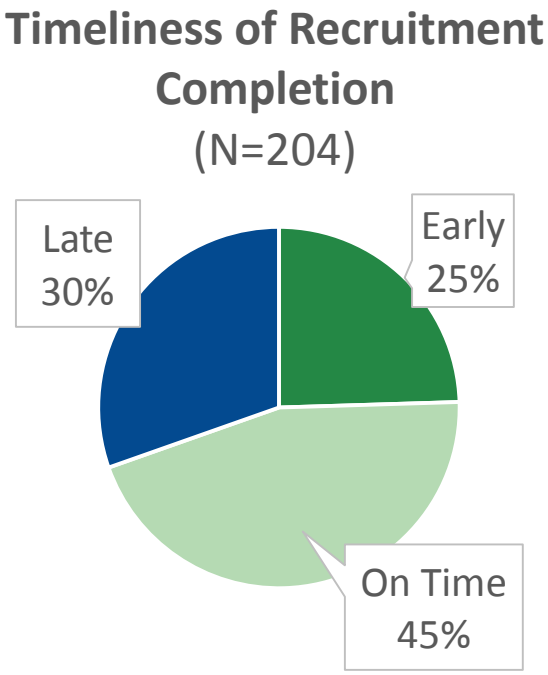
Through literature searches and working with other funders, we identified ***points of reference for research projects:***

Primary Reference: Tufts Center for the Study of Drug Development. **89% of Trials Meet Enrollment, but Timelines Slip, Half of Sites Under-enroll.** *Tufts CSFDD Impact Reports.* January/February 2013, Vol. 15 No. 1.

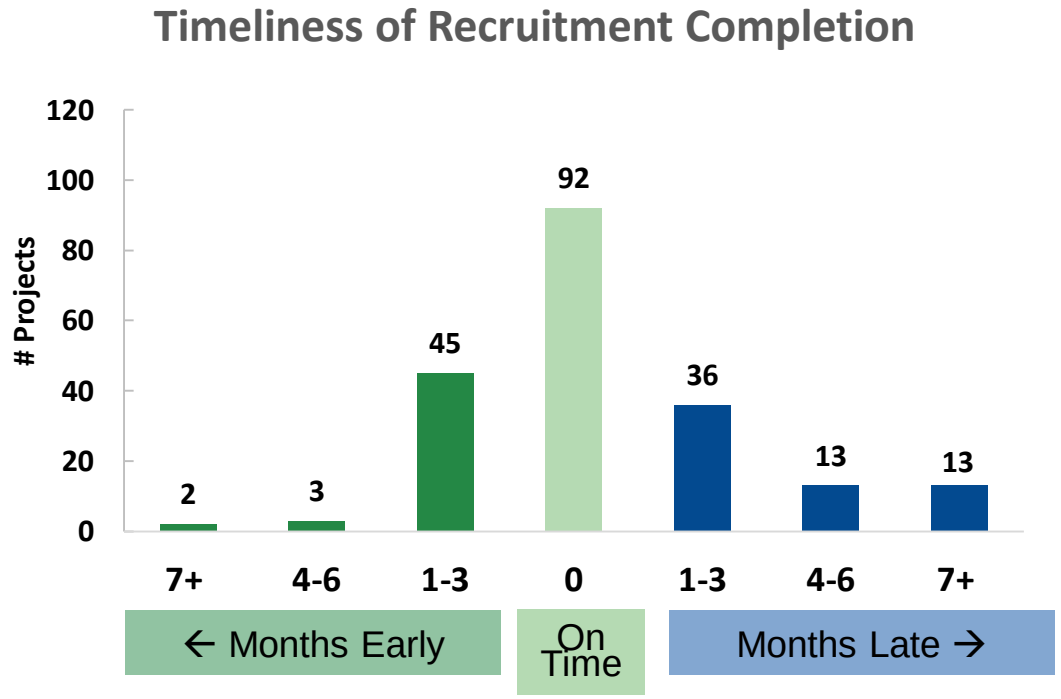
Recruitment Status of PCORI Projects as of Q3-18

366 Projects: 75% Broad, 10% PCS, 15% Targeted

Most PCORI-funded studies complete recruitment early or on time, and majority of those completed late take 3 or fewer additional months to complete it



70% Completed on time or early



Many PCORI-funded studies require modest extensions of their recruitment timelines

To what extent do studies need to modify recruitment timeline to complete recruitment?

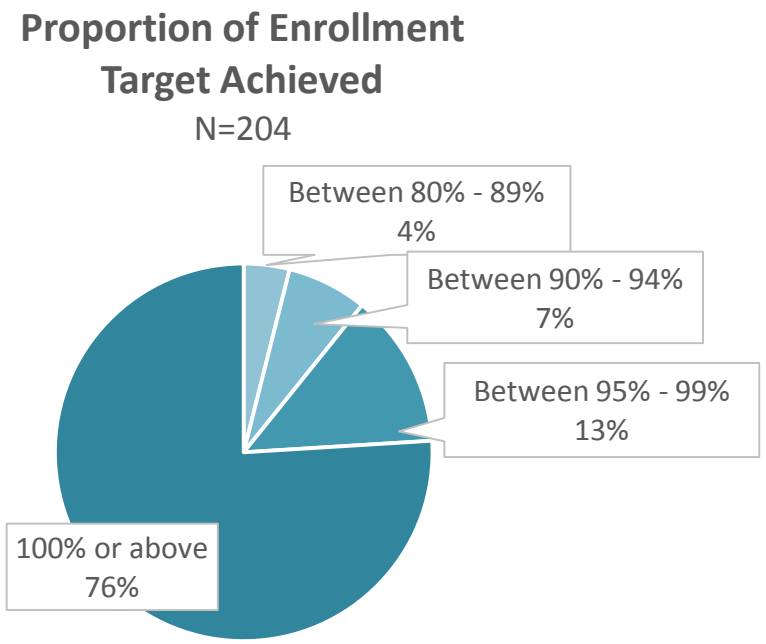
82/204 (40%) of studies that have completed recruitment have extended planned recruitment time by >1 month

- Median recruitment extension: 6.1 months (Average 7.1 m)
- 7% (N=6) did not need the extension- completed on time with original timeline
- 73% (N=60) completed recruitment on time with new deadline
- 20% (N=16) completed recruitment late, even with new deadline

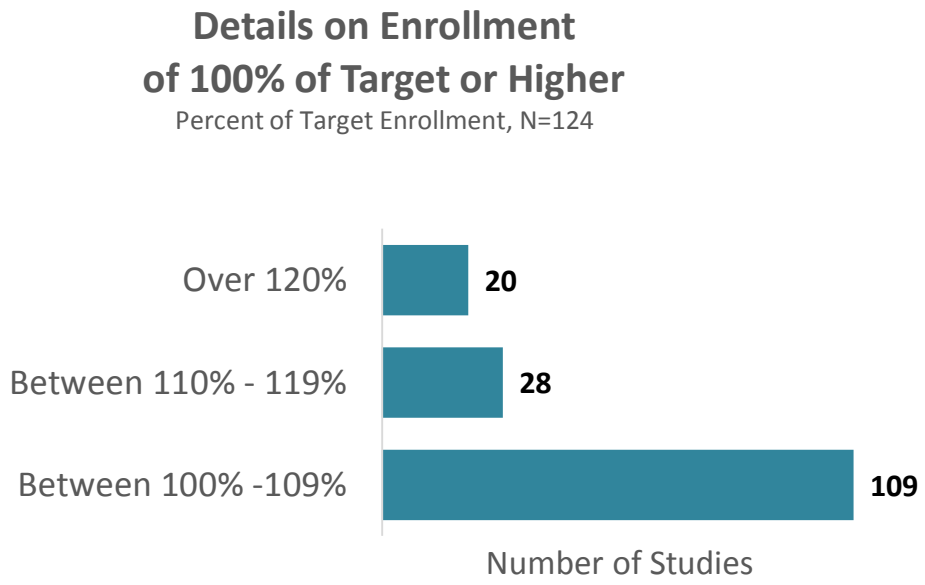
Among all completed studies, the average time to complete recruitment was 138% of the original planned recruitment timeline (median 110%)

PCORI Average: 138% of planned enrollment time, vs. Point of Reference: 194% (Tufts 2013)

Almost all PCORI-funded studies achieve at least 90% of their enrollment targets, and more than half of them meet or exceed it



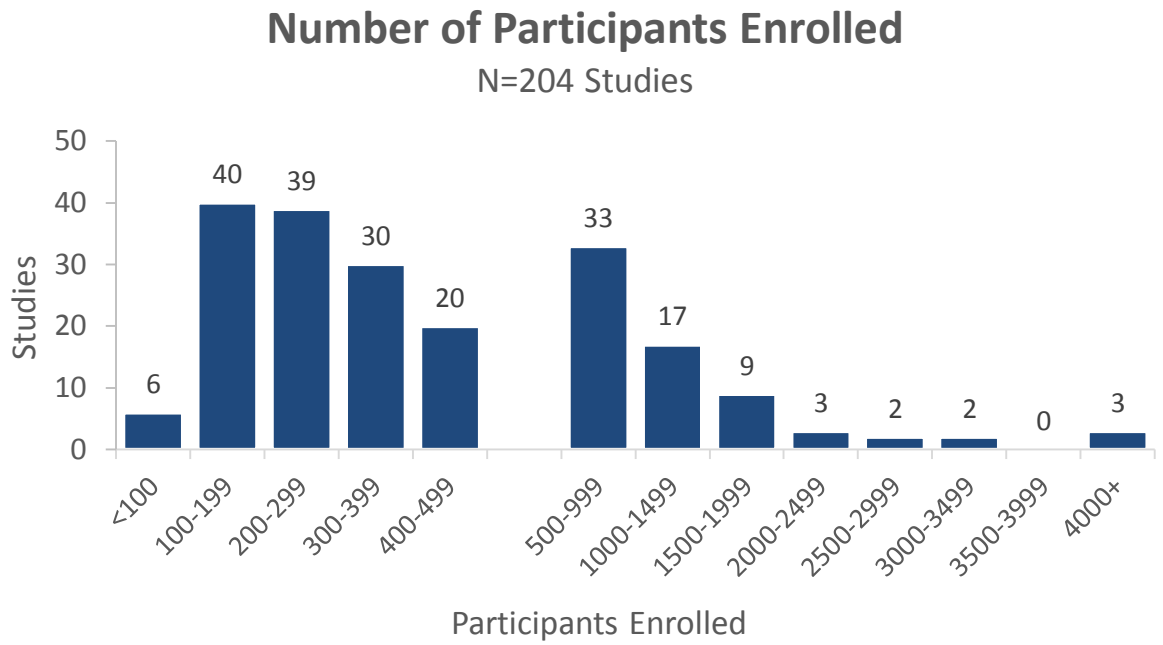
96% achieved >90% of enrollment target



10% of PCORI studies that have completed recruitment achieved >120% of enrollment target

PCORI: 72% met or exceeded enrollment, vs. Point of Reference: 52% (Tufts 2013)

Most PCORI-funded studies that have completed enrollment to date enrolled between 100 and 1,000 participants



Median: 348 participants per study
Average: 656 participants per study

- 18% Enrolled more than 1000 participants

PCORI Average: 656 Enrolled vs. Point of Reference: Average 622 Enrolled (Tufts 2013)

PCORI Board of Governors Dashboard
Third Quarter FY-2018 (As of 6/30/2018)

Dashboard Key

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- Off Target
- Needs Board Attention
- Target in Development
- Q4 2017
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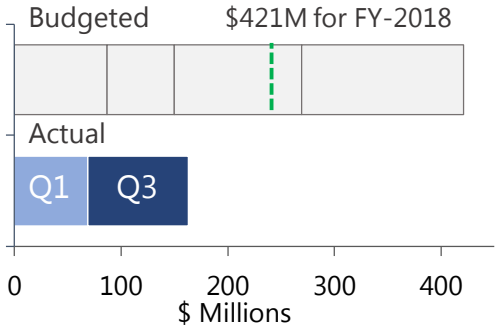
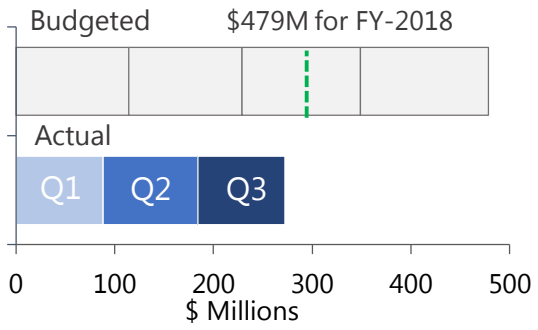
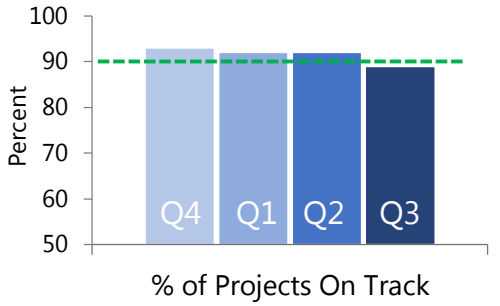
Funds Committed to Research



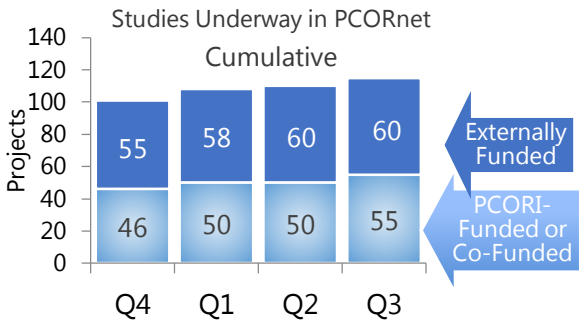
Expenses



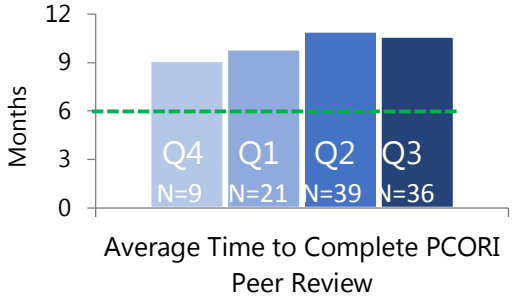
Project Performance



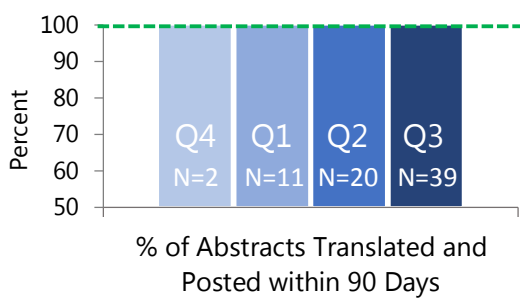
Research Using PCORnet



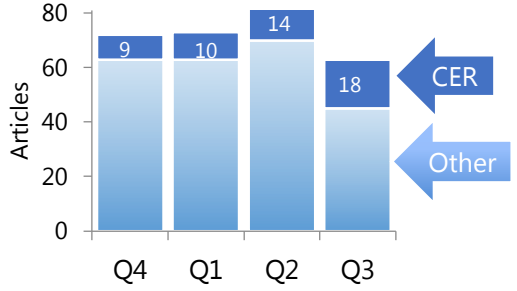
Speed of PCORI Peer Review



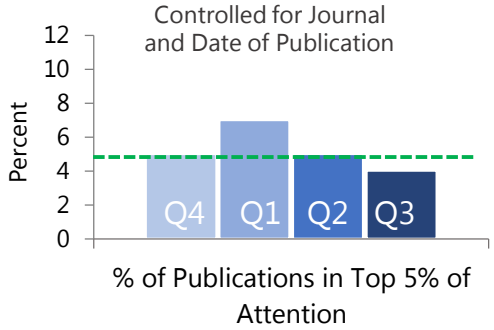
PCORI Abstracts Posted to Website



Results Published in Literature



Altmetric: Attention to PCORI Research



Featured Results on PCORI's Strategic Goals

Results on Goal 1

Increasing Information

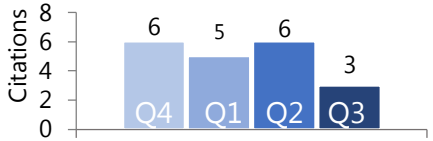
Results Highlight: A PCORI-funded study of treatments for serious mental illness compared a smartphone-based vs. a clinic-based intervention: participants in the smartphone arm were more likely to start treatment and remain engaged at 3 months.

Results on Goal 2

Speeding Uptake

As of Q3, results from PCORI studies have been cited 25 times in evidence-based clinical recommendations

Uptake Highlight: Results from a PCORI-funded study of community health workers supporting veterans were selected for an implementation award at the 2018 Veterans Health Administration Shark Tank Competition



Early Uptake of Findings (citations in evidence-based clinical recommendations)

Results on Goal 3

Influencing Research

PCORI is credited with influencing a new approach to engagement of patients, knowledge users, and scientists in reviewing applications from competitive research calls at the Ontario Ministry of Health and Long-Term Care (MOHLTC)

Agenda

1:00 PM	**Call to Order and Welcome**
1:00 – 1:05	**Consider for Approval:** Minutes of the September 18, 2018 Board Meeting
1:05 – 2:00	**Executive Director's Report**
2:00 – 3:15	**Transforming Research:** • The Future of Health Research • PCORnet – ADAPTABLE Update
3:30 – 4:00	**Consider for Approval:** FY2019 Funding Commitment Plan
4:00 – 4:30	**Strategic Plan Update:** Efforts to Address Emerging Interventions
4:30 – 5:00	**Public Comment**
5:00 PM	**Wrap up and Adjournment**

The Future of Health Research

Robert M. Califf, MD, MACC

Vice Chancellor for Health Data Science,
Duke University

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HEALTH DATA IN ACTION

ROME IS BURNING…

Robert M Califf MD
Vice Chancellor for Health Data Science
Duke University
Advisor, Verily Life Sciences
Chair, PCRF Board

PCORI Board Presentation
October 29th, 2018

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LET THEM EAT CAKE…

Robert M Califf MD
Vice Chancellor for Health Data Science
Duke University
Advisor, Verily Life Sciences
Chair, PCRF Board

PCORI Board Presentation
October 29th, 2018

LEARNING HEALTHCARE SYSTEMS: WE ARE AT THE FRONT OF THE WAVE

Robert M Califf MD

Vice Chancellor for Health Data Science

Duke University

Advisor, Verily Life Sciences

PCORI Board Presentation

October 29th, 2018

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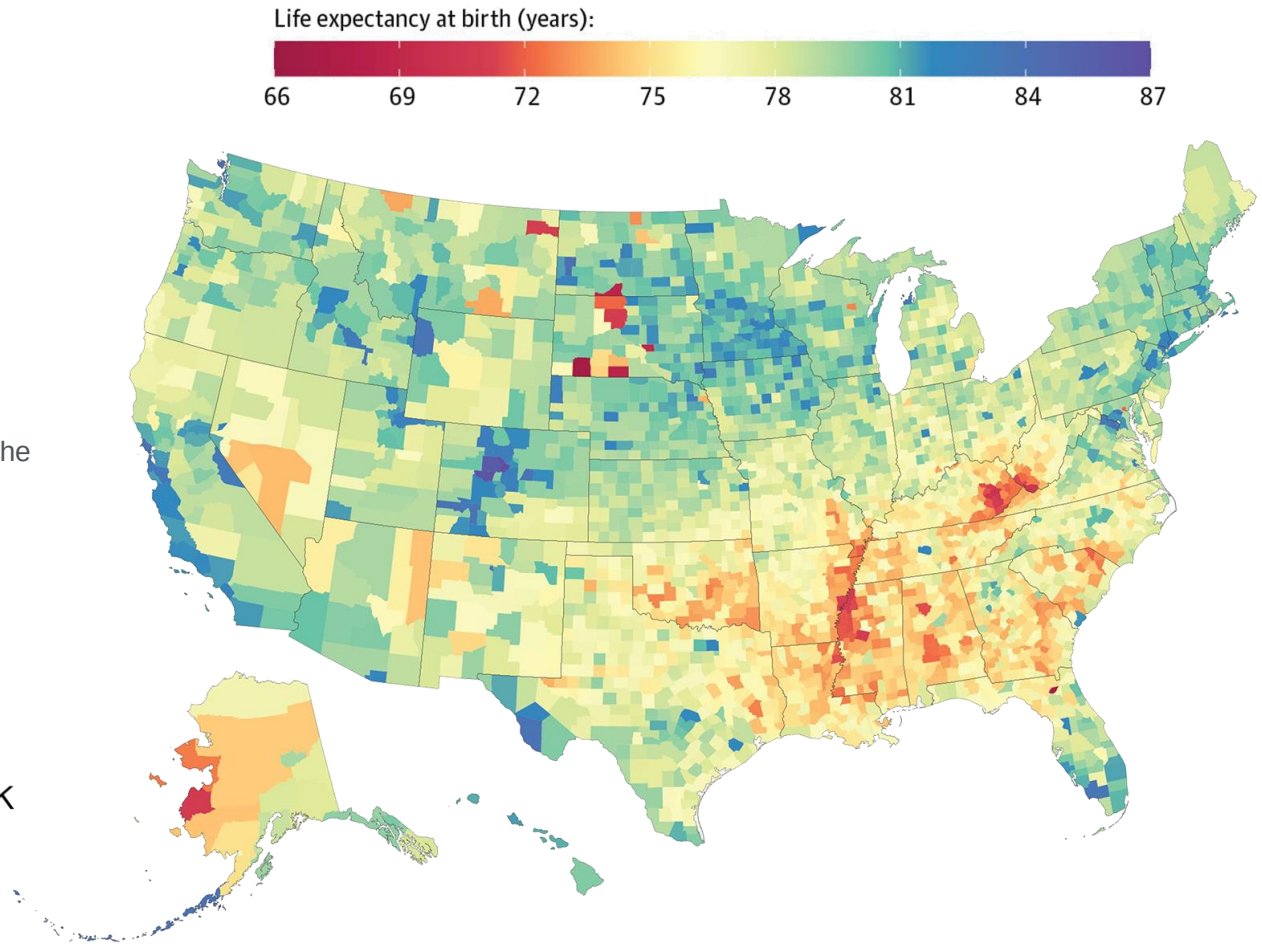
LIFE EXPECTANCY AT BIRTH BY COUNTY, 2014



- Counties in South Dakota and North Dakota had the lowest life expectancy, and counties along the lower half of the Mississippi, in eastern Kentucky, and southwestern West Virginia also had very low life expectancy compared with the rest of the country. Counties in central Colorado had the highest life expectancies.

The JAMA Network

Dwyer-Lindgren L, et al. Inequalities in life expectancy among US counties, 1980 to 2014 - temporal trends and key drivers. JAMA Intern Med. 2017;177:1003-11. doi:10.1001/jamainternmed.2017.0918

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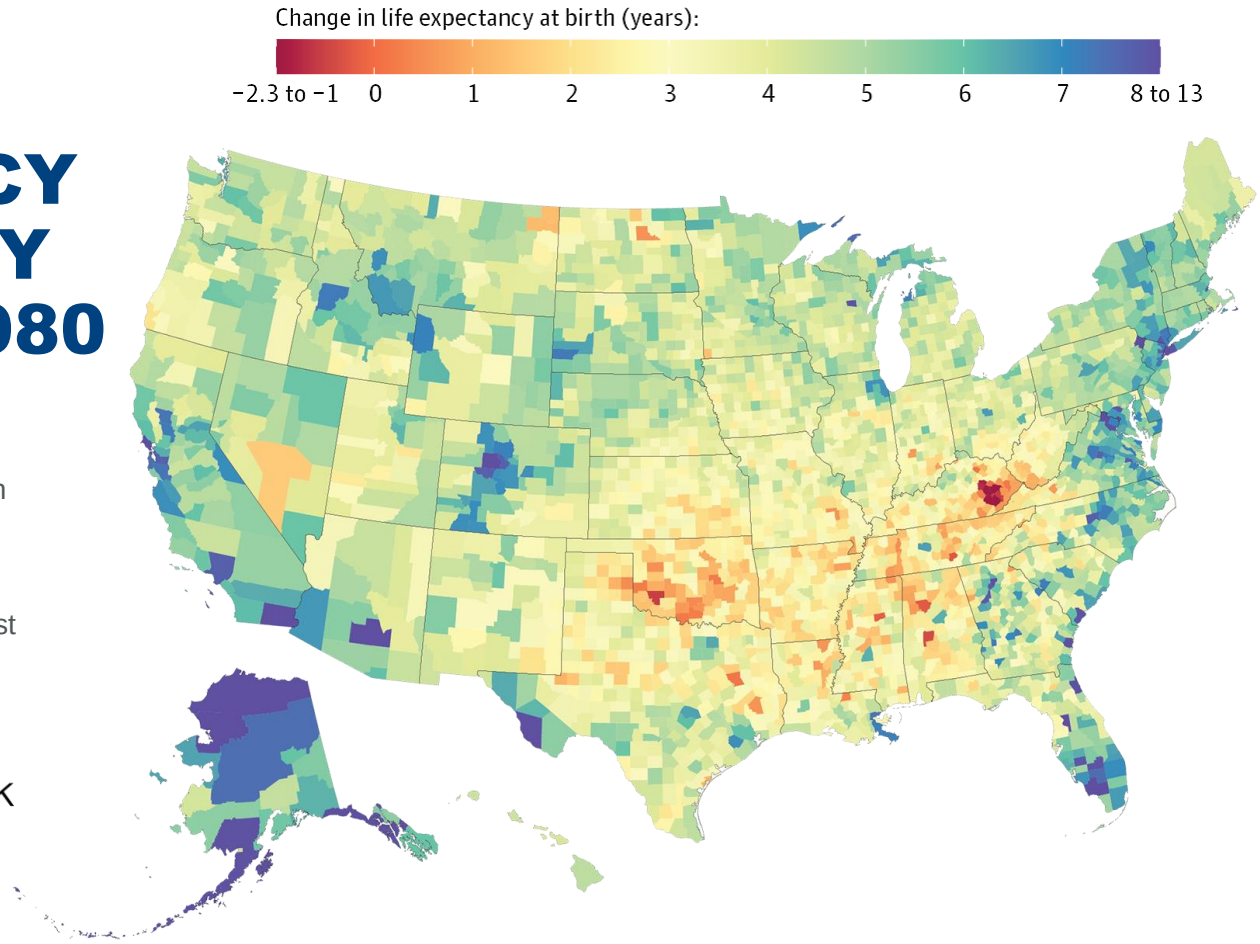
CHANGE IN LIFE EXPECTANCY AT BIRTH BY COUNTY, 1980 TO 2014

- Compared with the national average, counties in central Colorado, Alaska, and along both coasts experienced larger increases in life expectancy between 1980 and 2014, while some southern counties in states stretching from Oklahoma to West Virginia saw little, if any, improvement over this same period.



The JAMA Network

Dwyer-Lindgren L, et al. Inequalities in life expectancy among US counties, 1980 to 2014 - temporal trends and key drivers. JAMA Intern Med. 2017;177:1003-11. doi:10.1001/jamainternmed.2017.0918

From: **Inequalities in Life Expectancy Among US Counties, 1980 to 2014Temporal Trends and Key Drivers**

JAMA Intern Med. Published online May 08, 2017. doi:10.1001/jamainternmed.2017.0918

Table 1. Variables Included in the Regression Analysis With Summary Statistics and Bivariate Regression Results

Variable	Summary Statistics, Mean (SD) [Range]	Bivariate Regression Results	
		Coefficient (SE)	R^2
Socioeconomic and race/Ethnicity factors			
Population below the poverty line, %	16.3 (6.4) [3.1-62.0]	−0.24 (0.005)	0.47
Median household income, log $	10.6 (0.2) [9.8-11.6]	6.06 (0.130)	0.41
Graduates, age ≥25 y, %			
High school	83.7 (7.2) [46.3-98.6]	0.20 (0.004)	0.42
College	19.2 (8.6) [4.2-72.0]	0.15 (0.004)	0.34
Unemployment rate, age ≥16 y, %	9.1 (3.2) [2.1-27.4]	−0.29 (0.011)	0.18
Black population, %	9.4 (14.7) [0-85.8]	−0.07 (0.002)	0.24
American Indian, Native Alaskan, and Native Hawaiian population, %	2.3 (7.9) [0-97.2]	−0.06 (0.005)	0.04
Hispanic population, %	8.1 (13.1) [0-95.9]	0.02 (0.003)	0.01
Behavioral and metabolic risk factors, %			
Obesity prevalence, age ≥20 y	37.0 (4.3) [18.0-52.0]	−0.39 (0.006)	0.54
No leisure-time physical activity prevalence, age ≥20 y	27.0 (5.2) [11.7-47.2]	−0.34 (0.005)	0.62
Cigarette smoking prevalence, age ≥18 y	24.7 (4.1) [7.7-42.1]	−0.40 (0.007)	0.54
Hypertension prevalence, age ≥30 y	39.5 (3.6) [27.9-56.4]	−0.49 (0.007)	0.62
Diabetes prevalence, age ≥20 y	14.0 (2.4) [8.1-25.5]	−0.72 (0.011)	0.59
Health care factors			
Insured population, age <65 y, %	81.7 (5.7) [57.3-96.7]	0.15 (0.007)	0.14
Quality index	70.1 (11.5) [0-100]	0.10 (0.003)	0.28
Physicians per 1000 population, No.	1.1 (1.0) [0-4.4]	0.53 (0.039)	0.06

Abbreviation: SE, standard error.

Table Title:

Variables Included in the Regression Analysis With Summary Statistics and Bivariate Regression Results

Increases in Rates of Premature Death, Cardiovascular Death and Drug Deaths

The premature death rate has increased for the third straight year, along with **increases in rates of cardiovascular and drug deaths.**

PREMATURE DEATHS

+3%

Since 2015

DRUG DEATHS

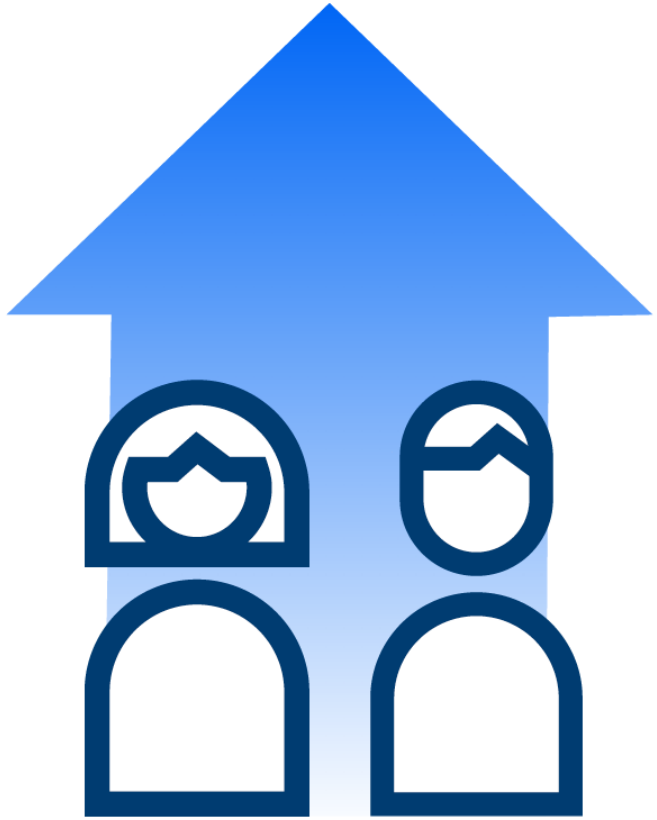
+7%

In the past year

CARDIOVASCULAR DEATHS

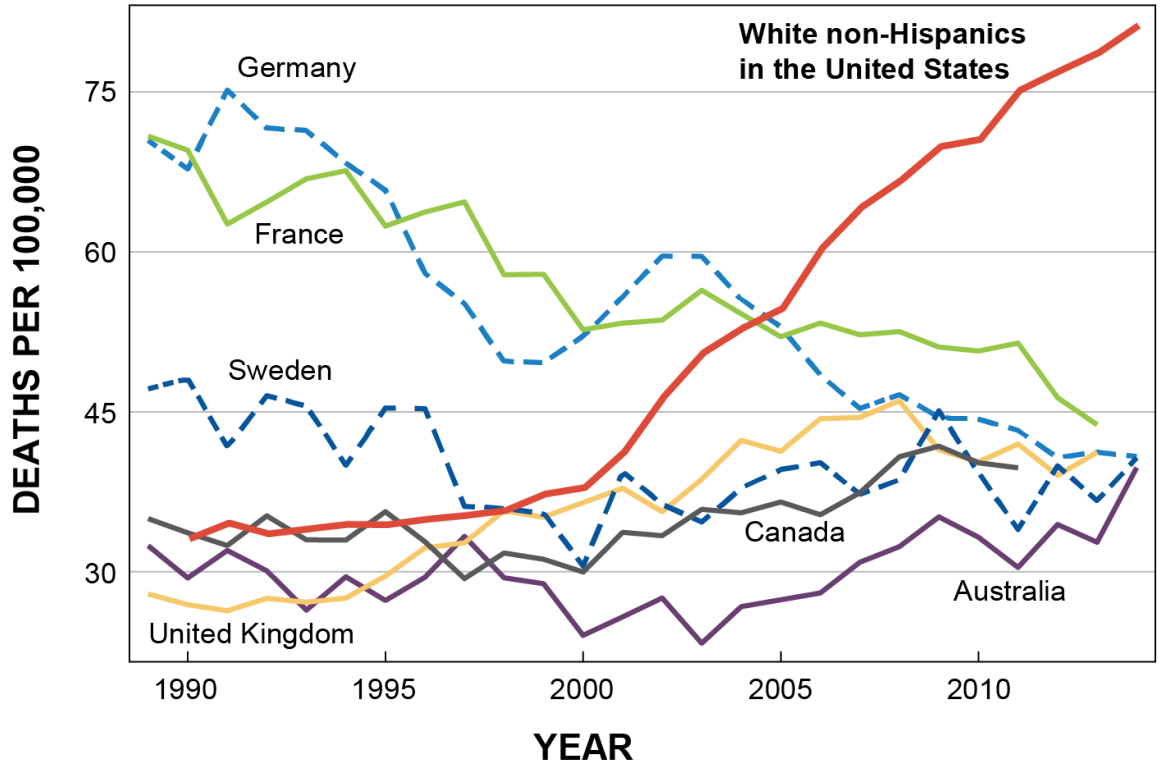
+2%

Since 2015



Midlife mortality from “deaths of despair” across countries

Men and women ages 50–54, deaths by drugs, alcohol, and suicide, 1989–2014

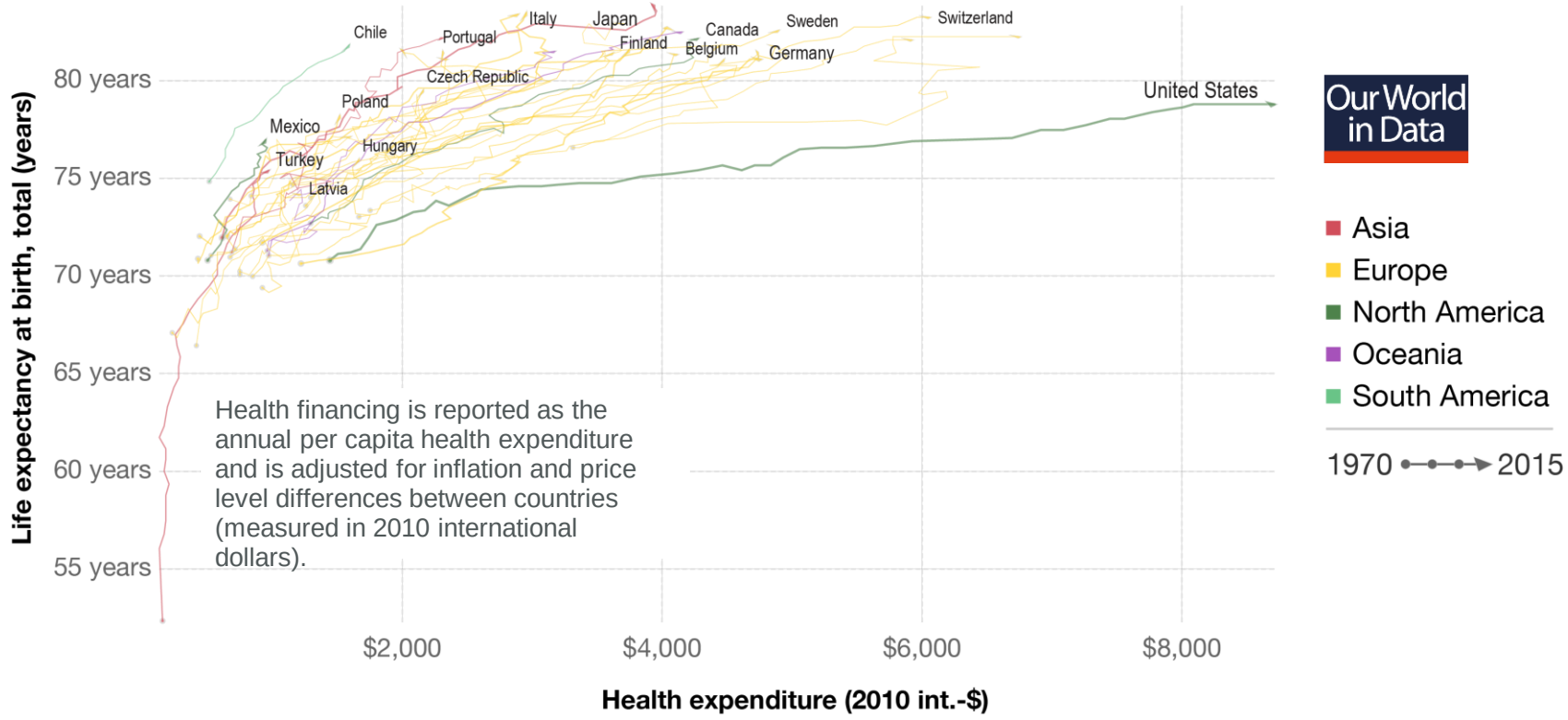


Source: “Mortality and morbidity in the 21st century” by Anne Case and Angus Deaton, Brookings Papers on Economic Activity, Spring 2017.

Life expectancy at birth (years) in 18 high income countries for women and men during 2010-16 and 1990-2015.

Jessica Y Ho, and Arun S Hendi BMJ 2018;362:bmj.k2562

Life expectancy vs. health expenditure, 1970 to 2015



Source: World Bank—WDI, Health Expenditure and Financing – OECDstat (2017)
OurWorldinData.org/the-link-between-life-expectancy-and-health-spending-us-focus • CC BY-SA

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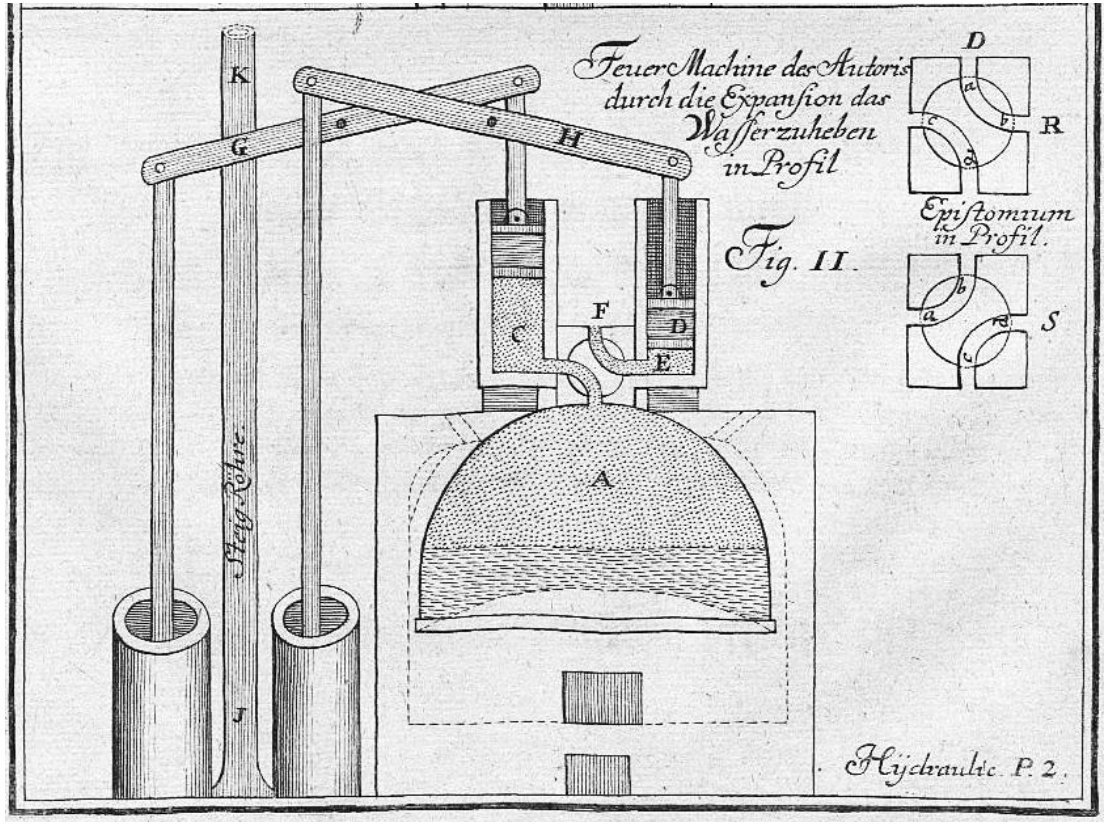
Four industrial revolutions

FIRST
Water and steam power mechanize production.

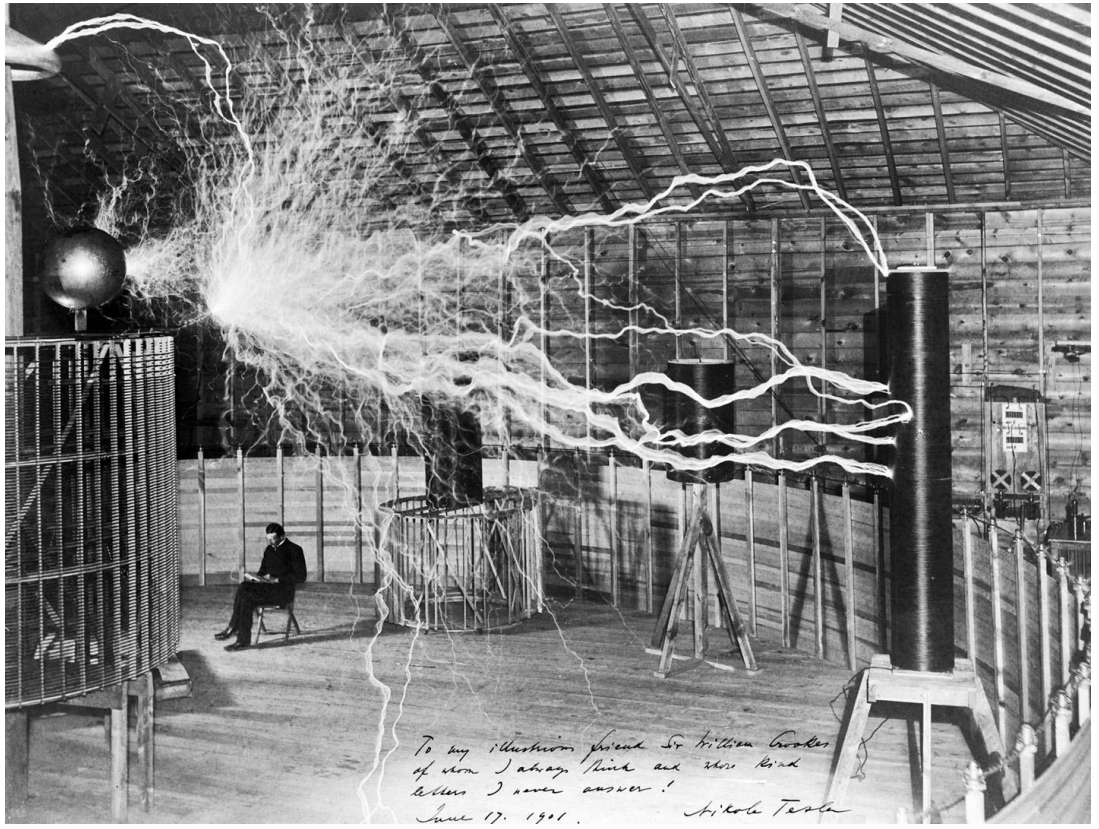


Jacob Leupold, Steam Engine, in *Theatri Machinarum Hydraulicarum II* (1720)

Four industrial revolutions

SECOND
Electric power creates mass production.

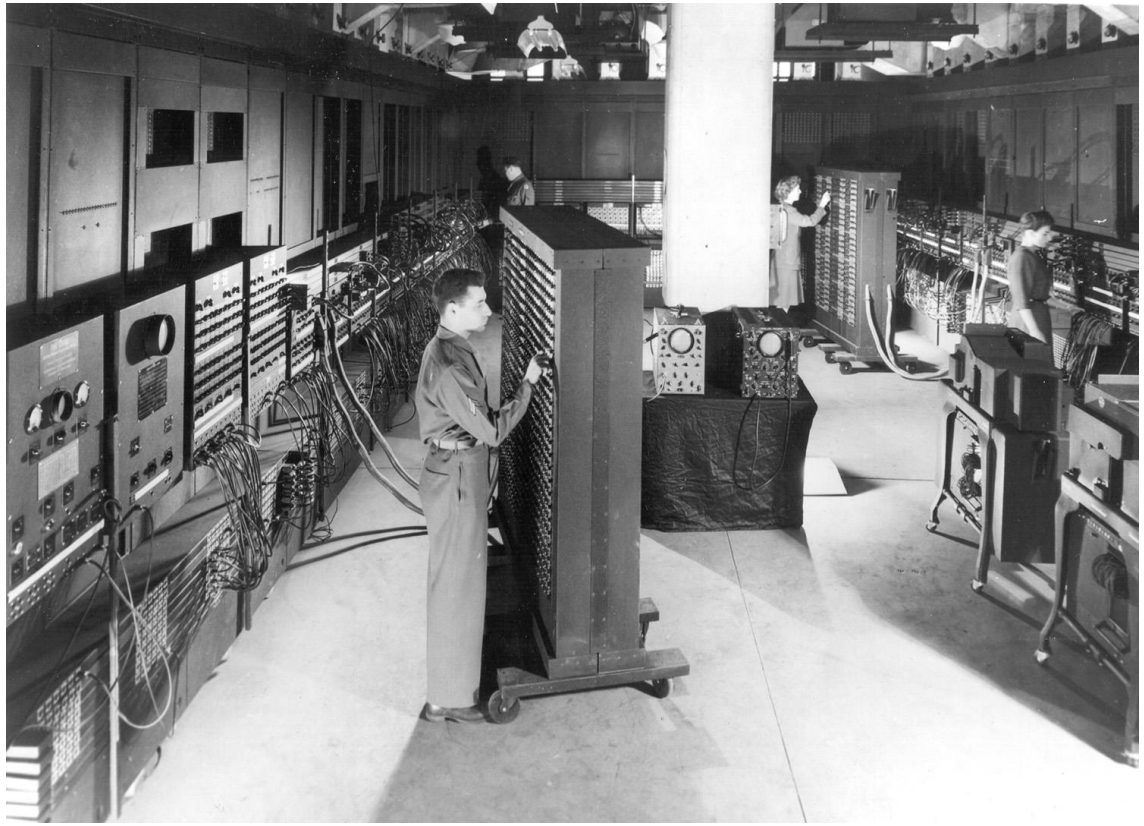
2



Photographer: Dickenson V. Alley, CC BY 4.0, https://commons.wikimedia.org/w/index.php?curid=36367226

Four industrial revolutions

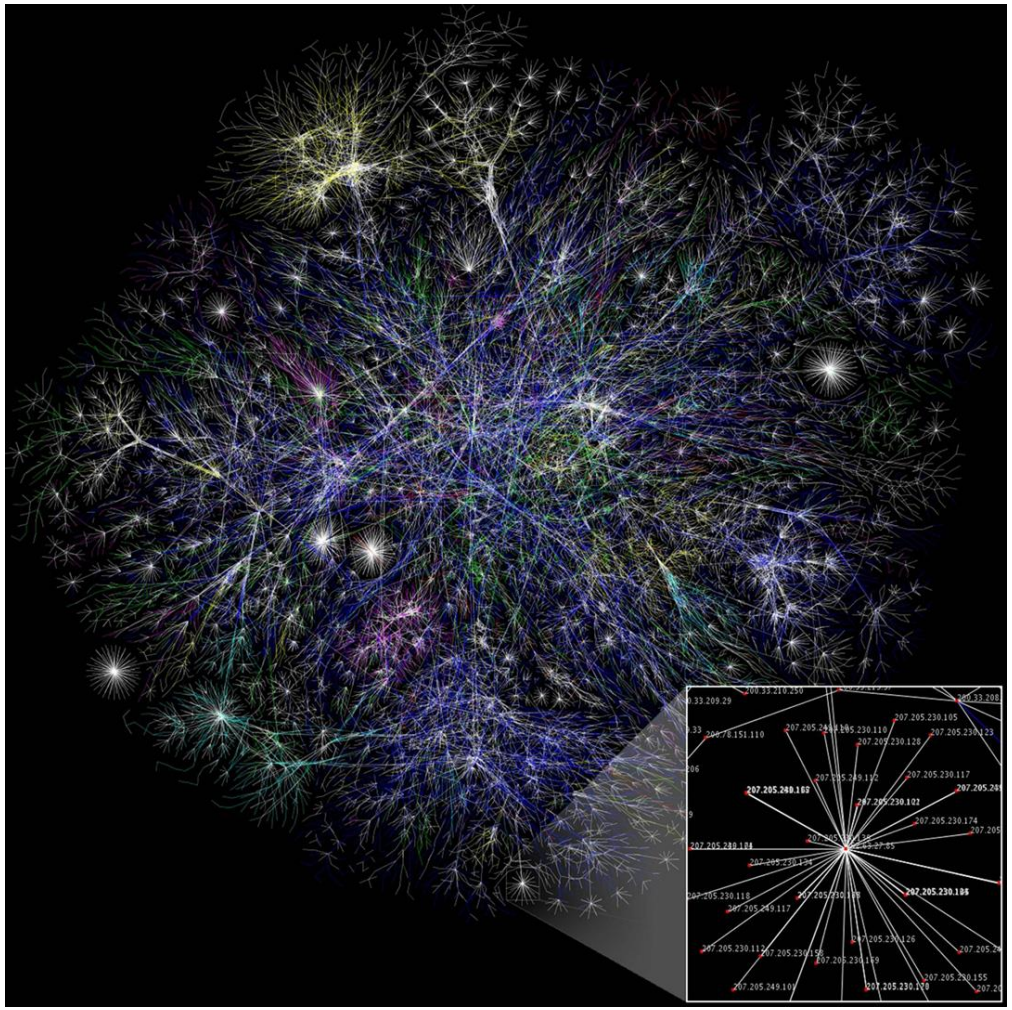
THIRD
Electronics and information technology automate production.



ENIAC digital computer. Unidentified U.S. Army photographer. Public Domain, https://commons.wikimedia.org/w/index.php?curid=978770

Four industrial revolutions

FOURTH
The digital revolution—characterized by a fusion of technologies—blurs the lines between physical, digital, and biological spheres.



Opte Project. Internet map. https://commons.wikimedia.org/wiki/File:Internet_map_1024.jpg

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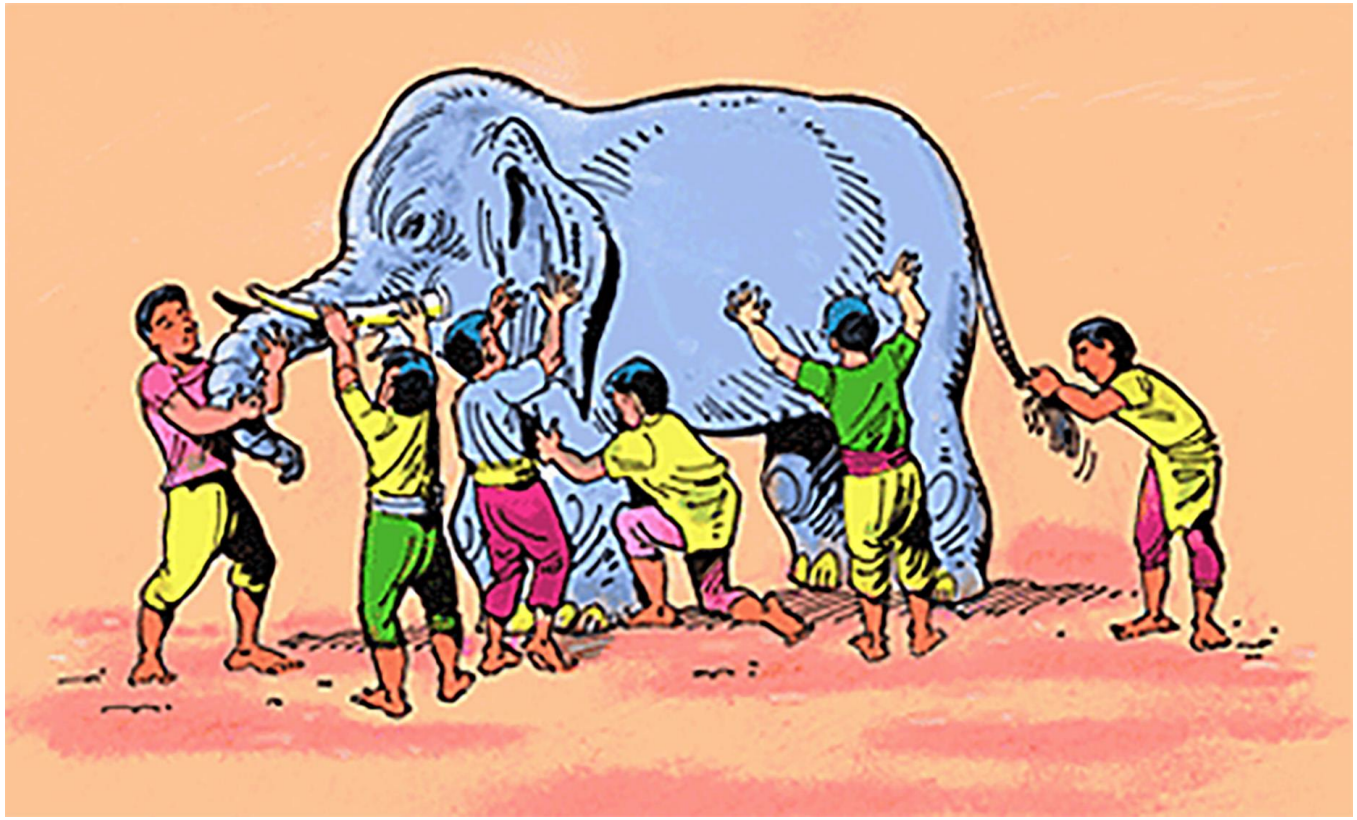

Project Baseline

WE'VE MAPPED THE WORLD. NOW LET'S MAP HUMAN HEALTH.

www.projectbaseline.com

verily

Duke University School of Medicine

Stanford MEDICINE

Google

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Big Challenges in Biomedicine

- **Lack of significant information over time dimension**
  - Measurements to assess biology and human health are made periodically in visits to healthcare or for research
- **Missing systems biology**
  - When developing concepts of human biology or drug development we make limited measurements focused on specific mechanisms —**we look "under the lamppost"**
- **Missing the opportunity to measure interactions of biology, sociology, environment and decision-making that could enable optimization of individualized and population health**
  - Although we know that health and disease are the product of the interactions of genes, multiple derivative biological systems, environment, social context and personal decisions, we tend to look at one part of the time

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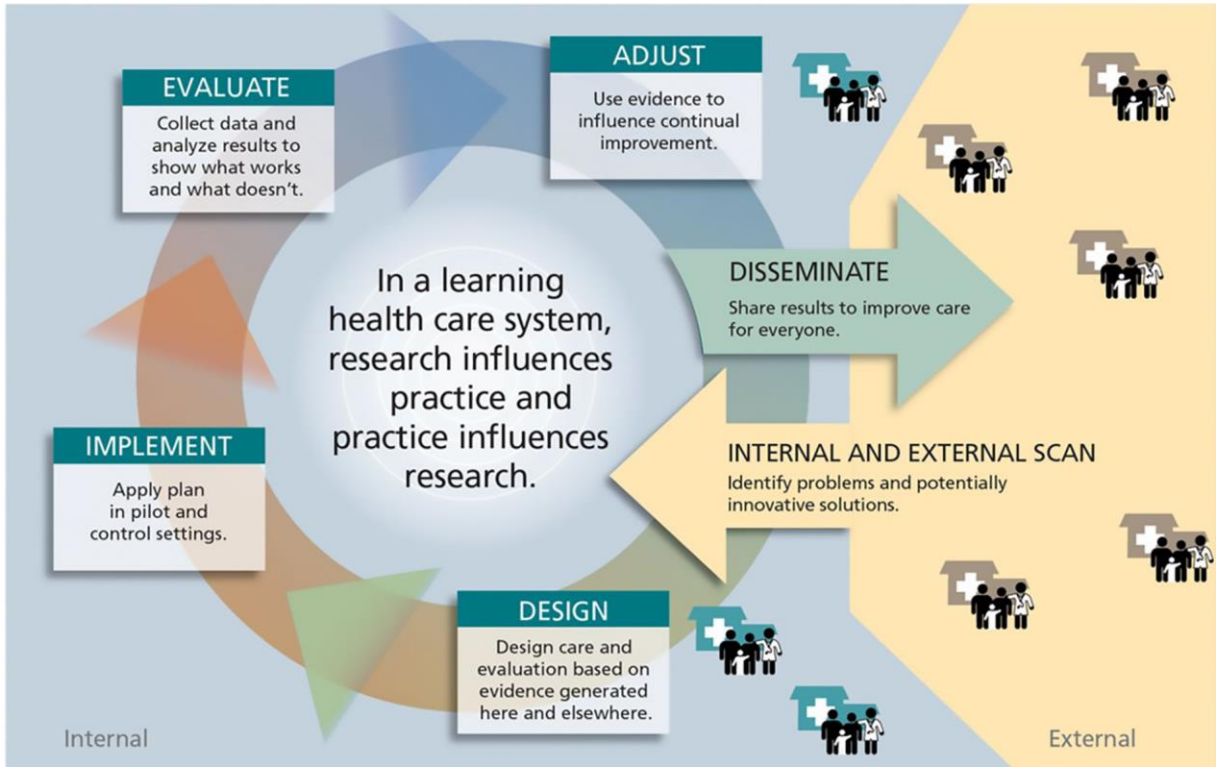
Learning health care systems



www.fda.gov

CONTINUOUS MONITORING THROUGH PASSIVE SENSORS

Study watch

Investigational wrist-worn sensor for continuous recording of physiological and environmental data

App

Mobile interface for self-reported and passive data acquisitions

Sleep sensor

Commercially available, placed under mattress to passively monitor multiple physiologic data parameters

Study hub

Safely sends device data to secure, encrypted Baseline database

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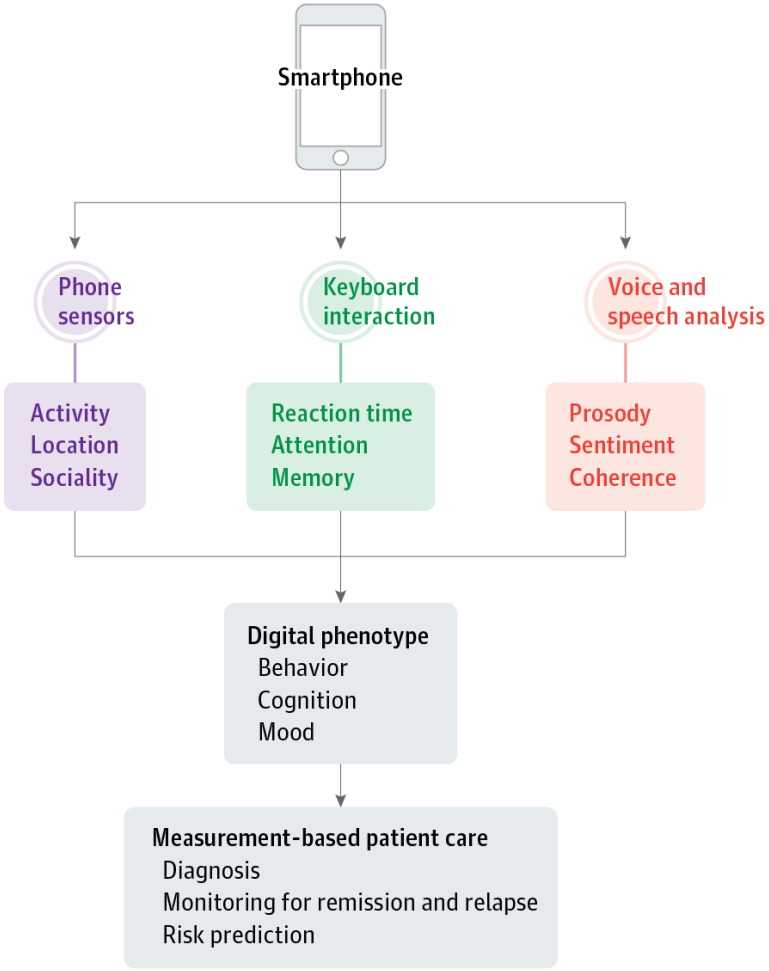
The process of digital phenotyping

Digital phenotyping involves collecting sensor, keyboard, and voice and speech data from smartphones to measure behavior, cognition, and mood.

The JAMA Network



Insel TR. Digital phenotyping technology for a new science of behavior. JAMA. 2017;318:1215-16. doi:10.1001/jama.2017.11295

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HEALTH DATA IN ACTION

MAPPING HEALTH

By Marie Lynn Miranda, Jeffrey Ferranti, Benjamin Strauss, Brian Neelon, and Robert M. Califf

DOI: 10.1377/hlthaff.2012.1199
HEALTH AFFAIRS 32,
NO. 9 (2013): 1608–1615

Geographic Health Information Systems: A Platform To Support The 'Triple Aim'

Marie Lynn Miranda (mlmiranda@umich.edu) is dean of and a professor in the School of Natural Resources and Environment and is a professor in the Department of Pediatrics, University of Michigan, in Ann Arbor.

Jeffrey Ferranti is the chief medical information officer and vice president for clinical informatics, and an assistant professor in newborn critical care, at Duke University Medical Center, in Durham, North Carolina.

Benjamin Strauss is an associate in research at the Nicholas School of the Environment, Duke University.

Brian Neelon is a statistician at the Nicholas School of the Environment, Duke University.

Robert M. Califf is vice chancellor for clinical and translational research and a professor of medicine in the Division of Cardiology, Duke University Medical Center.

ABSTRACT **Despite the rapid growth of electronic health data, most data systems do not connect individual patient records to data sets from outside the health care delivery system. These isolated data systems cannot support efforts to recognize or address how the physical and environmental context of each patient influences health choices and health outcomes. In this article we describe how a geographic health information system in Durham, North Carolina, links health system and social and environmental data via shared geography to provide a multidimensional understanding of individual and community health status and vulnerabilities. Geographic health information systems can be useful in supporting the Institute for Healthcare Improvement's Triple Aim Initiative to improve the experience of care, improve the health of populations, and reduce per capita costs of health care. A geographic health information system can also provide a comprehensive information base for community health assessment and intervention for accountable care that includes the entire population of a geographic area.**

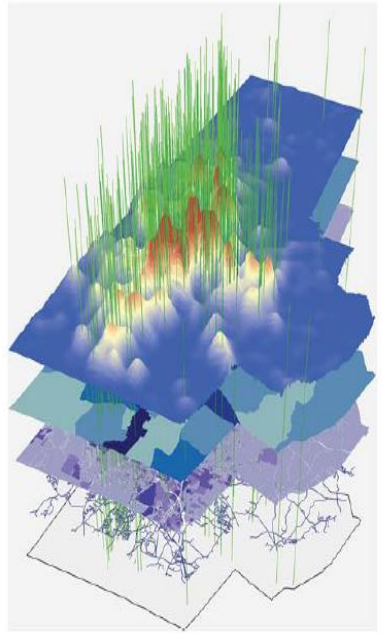
Donald Berwick and colleagues' influential 2008 *Health Affairs* article, "The Triple Aim: Care, Health, and Cost," describes a conceptual framework developed by the Institute for Healthcare Improvement for improving the US health care system.[1] In the Triple Aim, the institute has identified three aims that must be simultaneously pursued: improve the experience of care, improve the health of populations, and reduce per capita costs of health care. In this article we introduce and describe information technology designed to support health systems and communities in achieving the Triple Aim. We demonstrate how this technology can be used to assess the health of hood lead exposure; a health services application to better manage patient flow to emergency departments (EDs); and a clinical population health application designed to care for people with diabetes at the individual, neighborhood, and county levels.

The Triple Aim has been used by a number of health systems as a conceptual framework for designing health system improvement programs.[2–4] The abundant electronic health data that are accumulating are highly relevant to managing population health and developing new insights.[5] Until recently, however, these data have been dispersed across many locations, with little integration.[6,7] As integrated health systems are becoming more widespread, these data are being

MAPPING HEALTH

EXHIBIT 1

Example Of Geographic Health Information Systems (GHIS) For Mapping The Terrain Of Diabetes In Durham County, North Carolina



SOURCE Duke Health Technology Solutions Decision Support Repository (DSR), using information on boundaries and streets layers from the US Census Bureau Geography Division, census 2010; and tax-parcel data from the Durham County Tax Assessor. NOTE The elements of this GHIS map are ex-


1 in 20
Google searches are health related
Google

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WHY DEPRESSION?

DEPRESSION IS HIGHLY PREVALENT

300M

people suffer from depression globally, WHO has declared it a leading cause of disability [WHO]

MANY PEOPLE DON'T GET TREATMENT

50%

of people with depression in the US did not get any treatment [JAMA]

TREATMENT IS OFTEN DELAYED

7 YRS

average time from onset to treatment in the US [JAMA]

TREATMENT IS EFFECTIVE

70%

of patients can improve, often in a matter of weeks [NIMH]

Google has the reach, scale and technology to help

PRODUCT OVERVIEW: What is PHQ-9?

PHQ-9 is a Patient Health Questionnaire, with 9 questions, that is used to measure depression severity

PATIENT HEALTH QUESTIONNAIRE-9				
Over the last 2 weeks, how often have you been bothered by any of the following problems?	**Not at all**	**Several days**	**More than half the days**	**Nearly every day**
1. Little interest or pleasure in doing things	0	1	2	3
2. Feeling down, depressed, or hopeless	0	1	2	3
3. Trouble falling or staying asleep, or sleeping too much	0	1	2	3
4. Feeling tired or having little energy	0	1	2	3
5. Poor appetite or overeating	0	1	2	3
6. Feeling bad about yourself — or that you are a failure or have let yourself or your family down	0	1	2	3
7. Trouble concentrating on things, such as reading the newspaper or watching television	0	1	2	3
8. Moving or speaking so slowly that other people could have noticed? Or the opposite — being so fidgety or restless that you have been moving around a lot more than usual	0	1	2	3
9. Thoughts that you would be better off dead or of hurting yourself in some way	0	1	2	3

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Parachute use to prevent death and major trauma related to gravitational challenge: systematic review of randomised controlled trials

Gordon C S Smith, Jill P Pell

Parachutes reduce the risk of injury after gravitational challenge, but their effectiveness has not been proved with randomised controlled trials

BMJ 2003

PCORnet® embodies a “network of networks” that harnesses the power of partnerships

9 Clinical Research Networks (**CRNs**) + 2 Health Plan Research Networks (**HPRNs**) + Patient Partners + 1 Coordinating Center = A national infrastructure for people-centered clinical research

pcornet®

NIH Collaboratory *Rethinking Clinical Trials®*

Health Care Systems Research Collaboratory

NIH Health Care Systems Research Collaboratory

Enabling pragmatic clinical trials embedded in health care systems

The Collaboratory Story

Initiated through the NIH Common Fund in 2012

Goal: Strengthen the national capacity to implement cost-effective large-scale research studies that engage health care delivery organizations as research partners

Vision: Support the design and execution of innovative pragmatic clinical trial Demonstration Projects to establish best practices and proof of concept

NIH Collaboratory *Rethinking Clinical Trials®*
Health Care Systems Research Collaboratory

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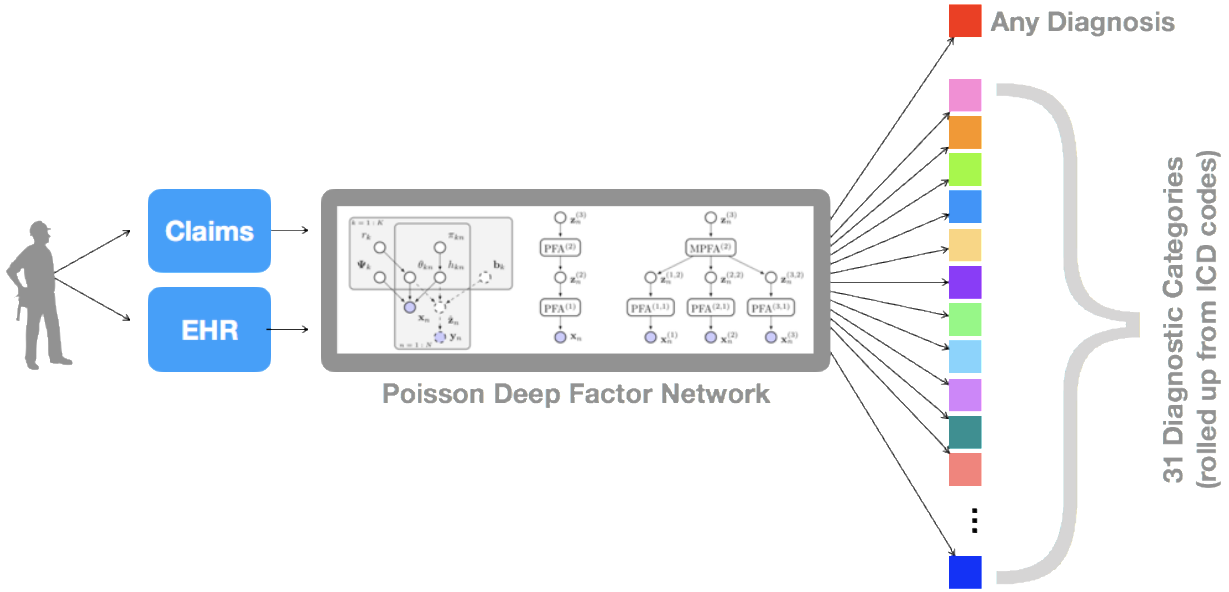
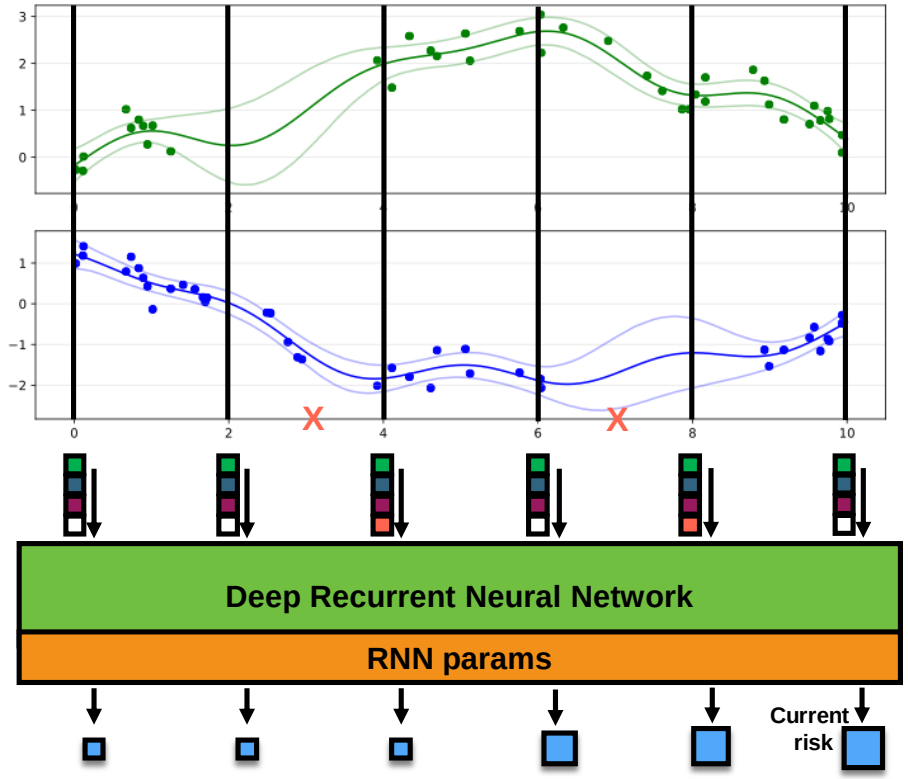
Admission Risk Prediction Model



Expressed as absolute 6-month probability and percentile risk (e.g. 73% and 87th %-ile)

Model Schematic

: Lab 1
: Lab 2
: Baseline
: Medication

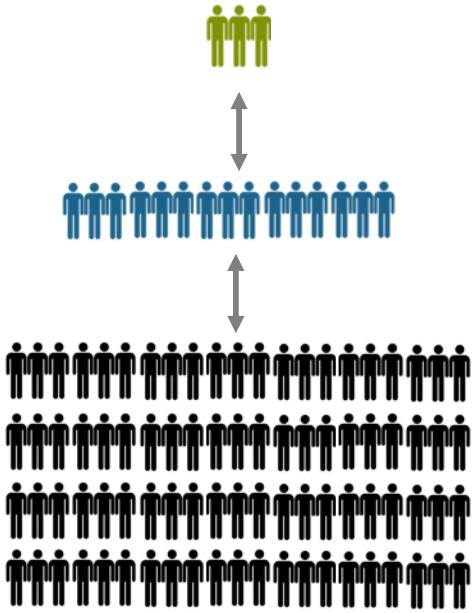


Multitask Gaussian Process

Deep Learning Model

6
5

Our Vision



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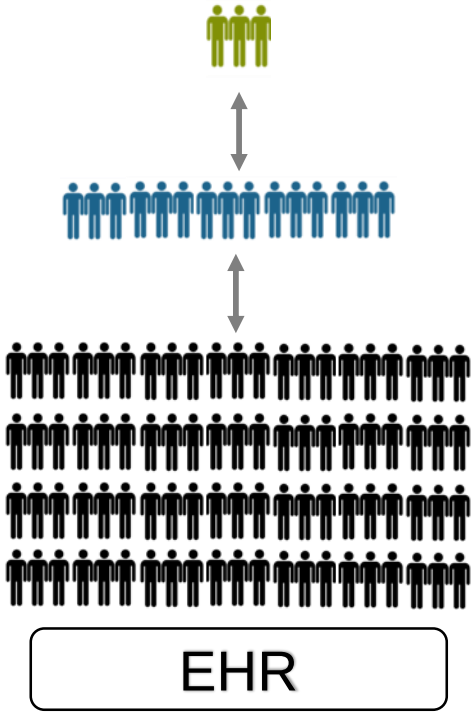
Our Vision



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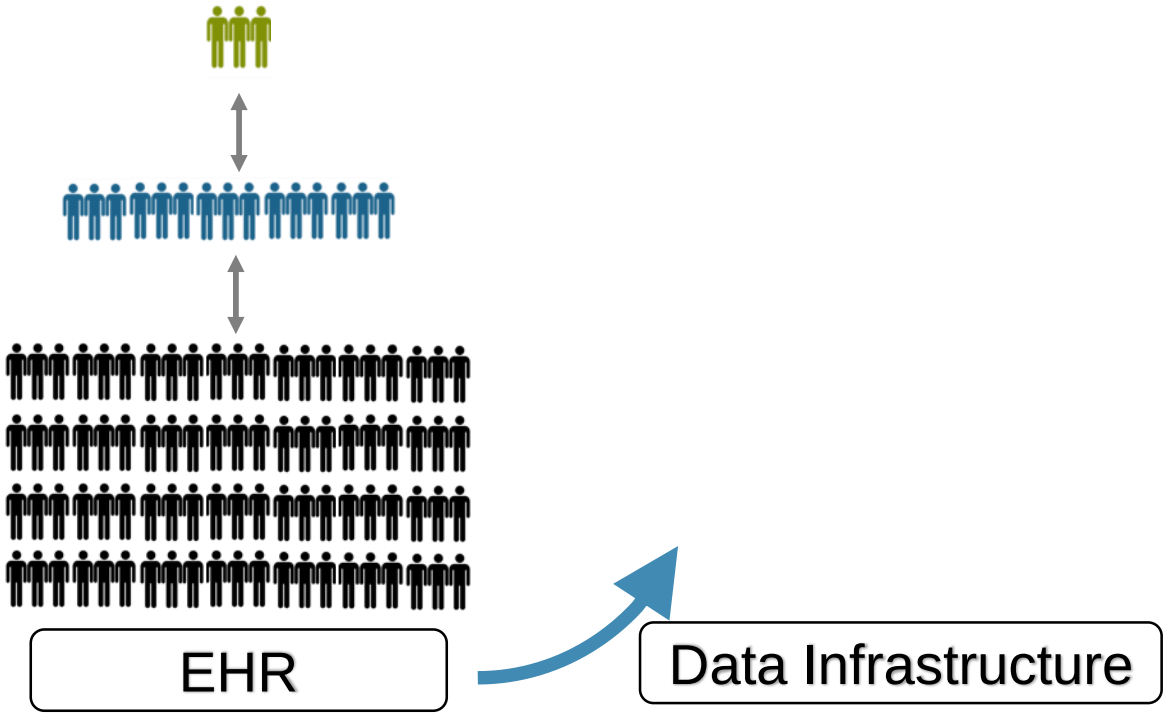
Our Vision



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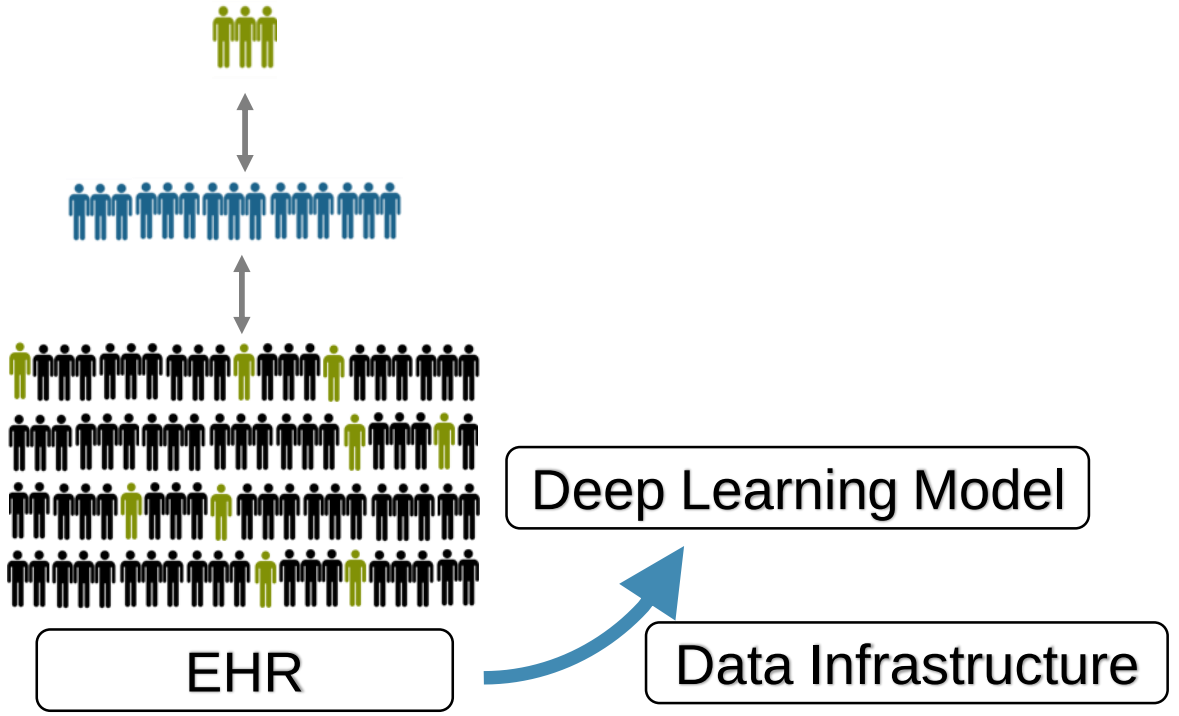
Our Vision



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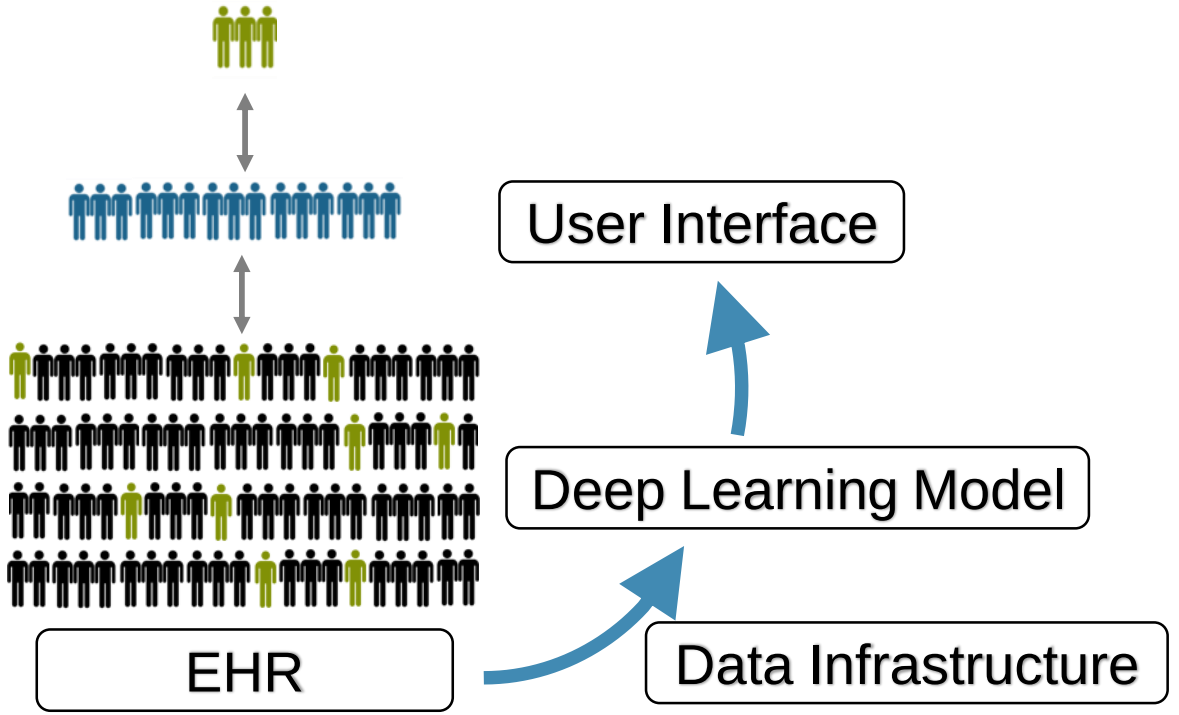
Our Vision



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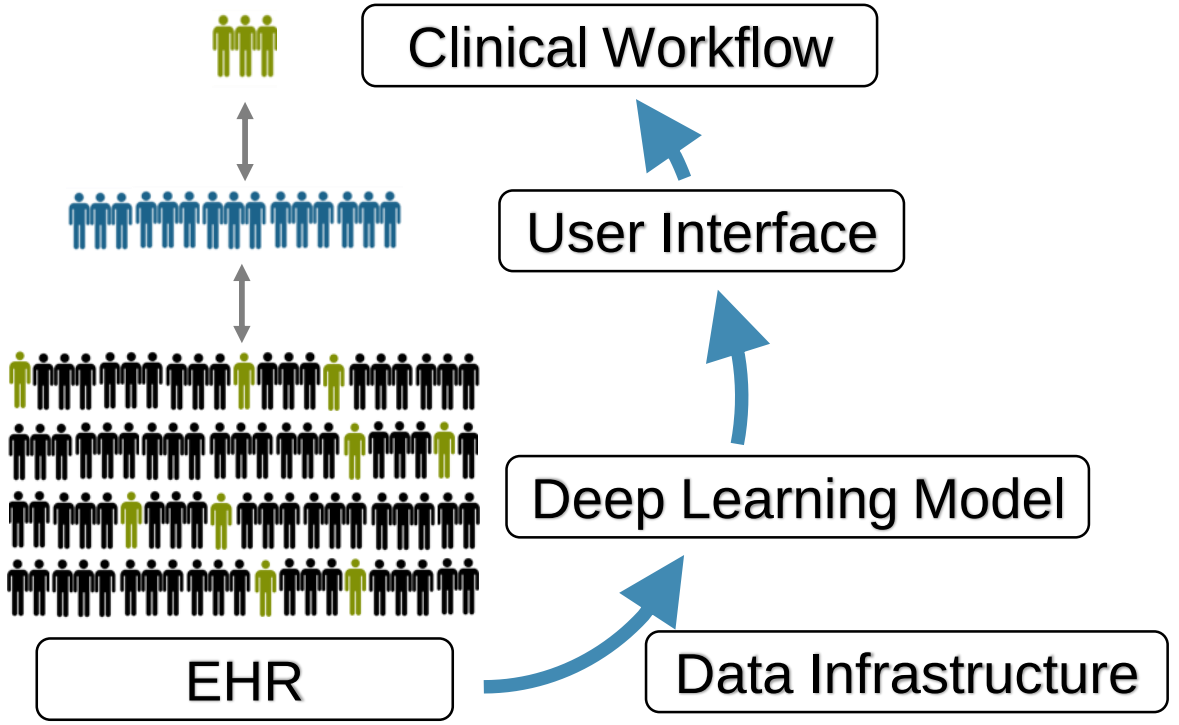
Our Vision



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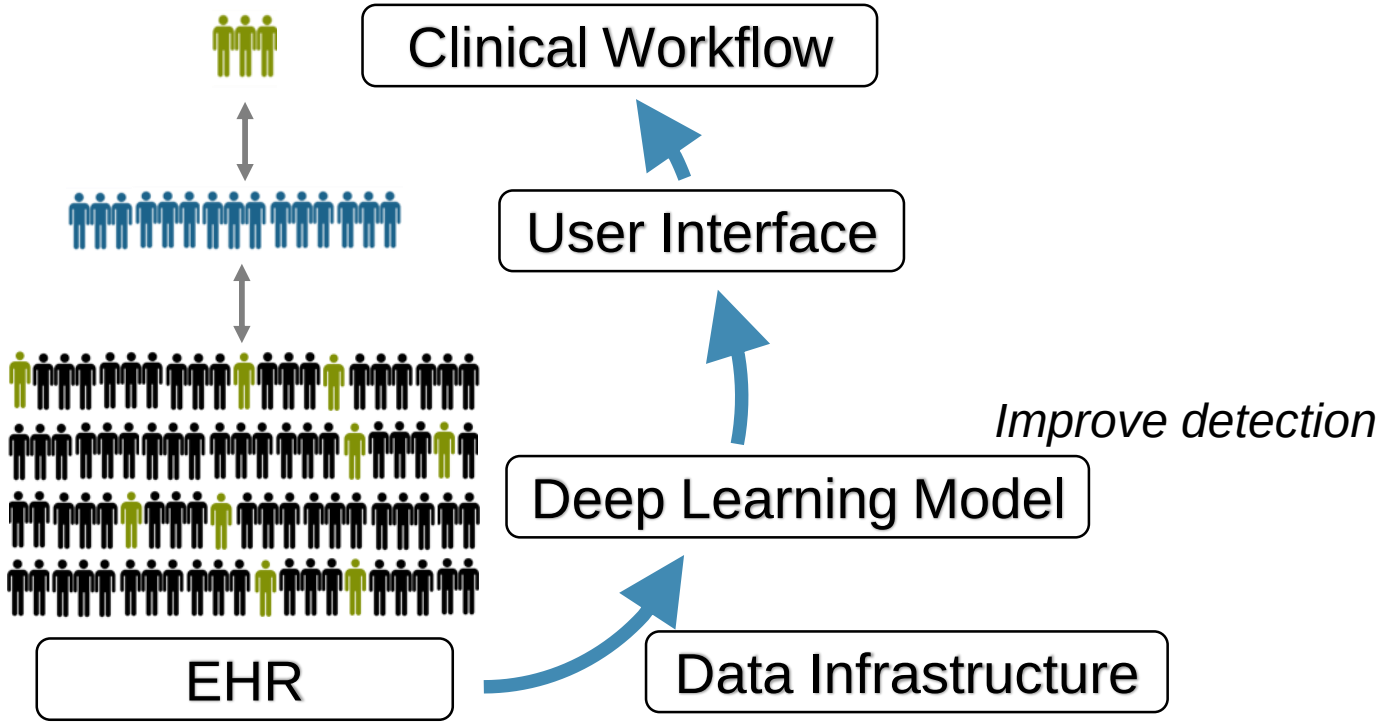
Our Vision



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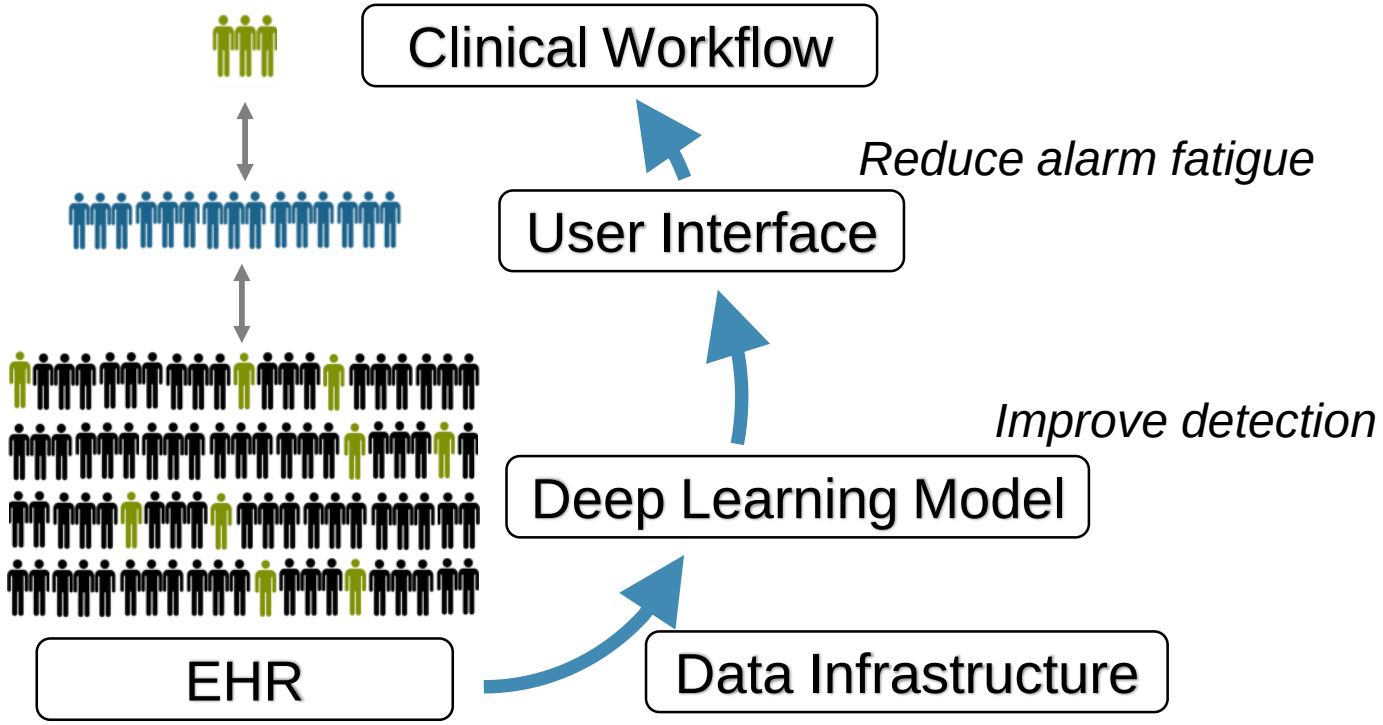
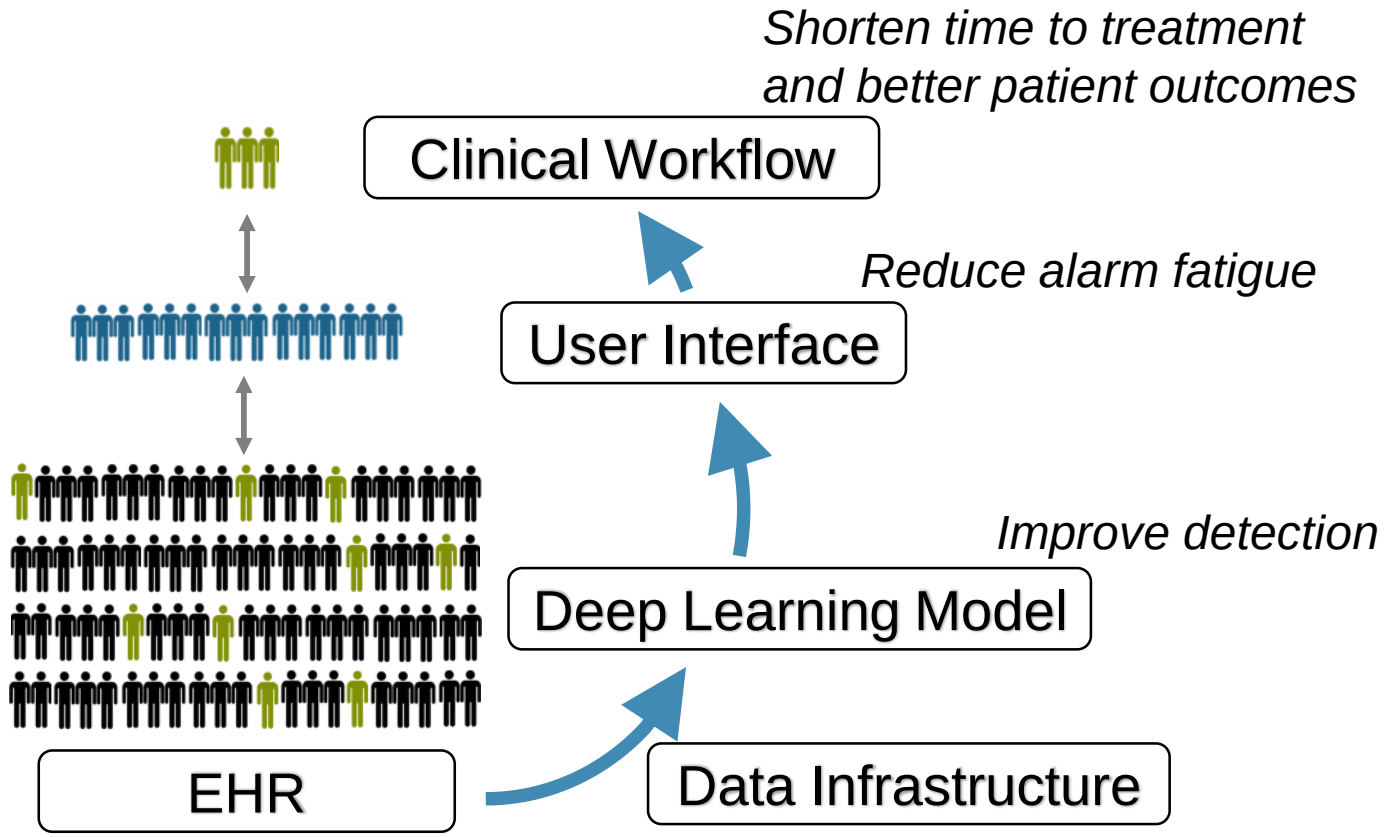
2

Our Vision



7
3

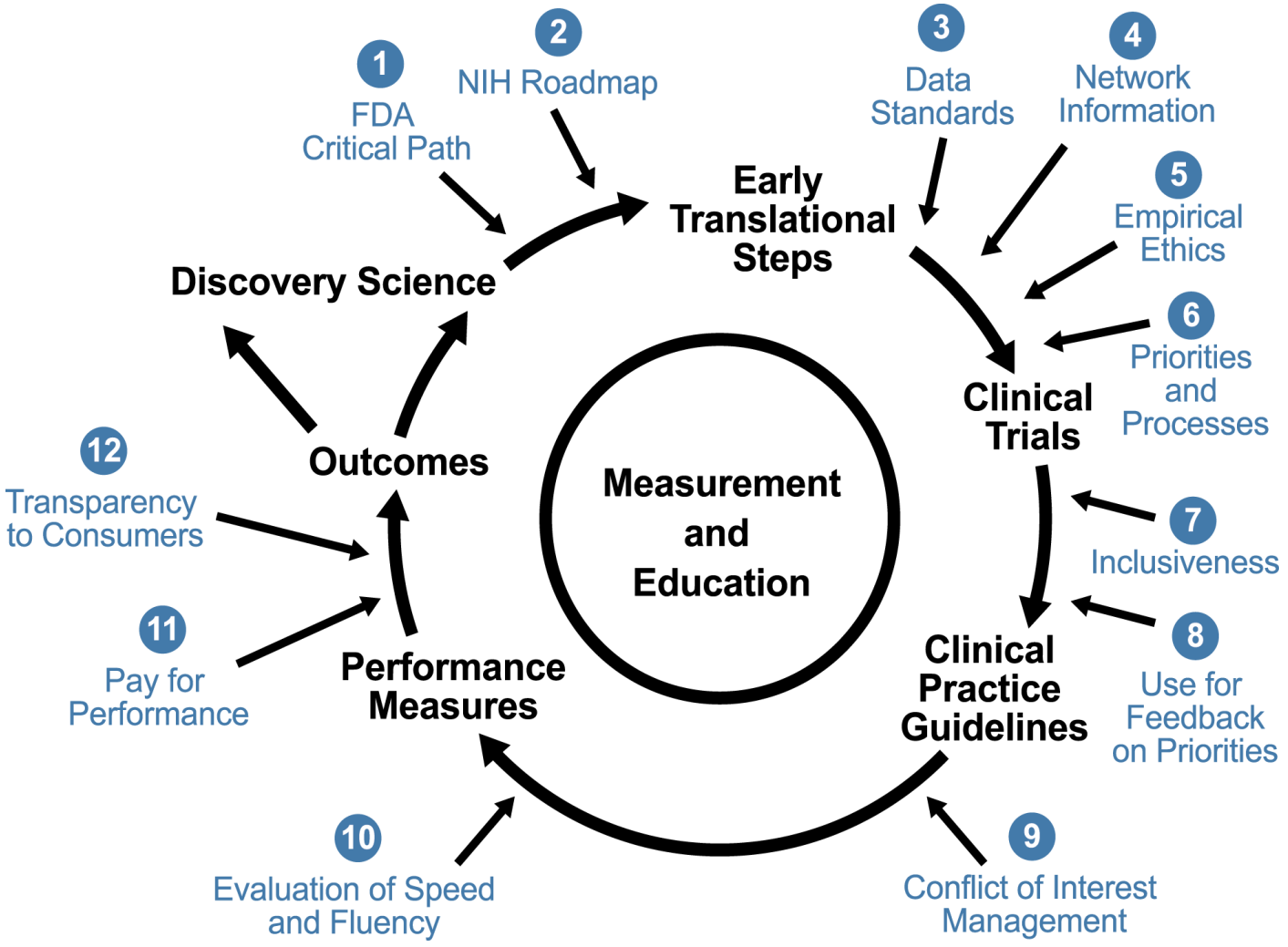
Our Vision



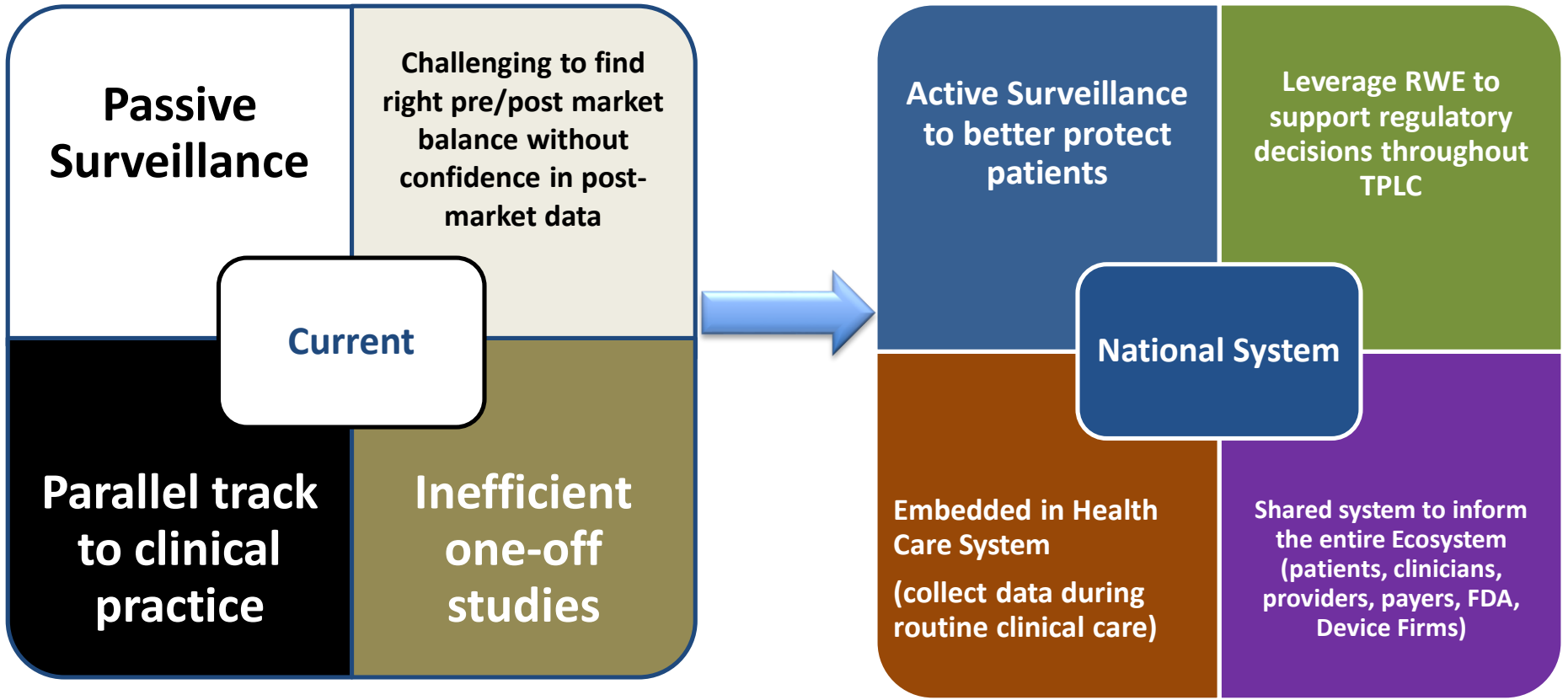
7
4

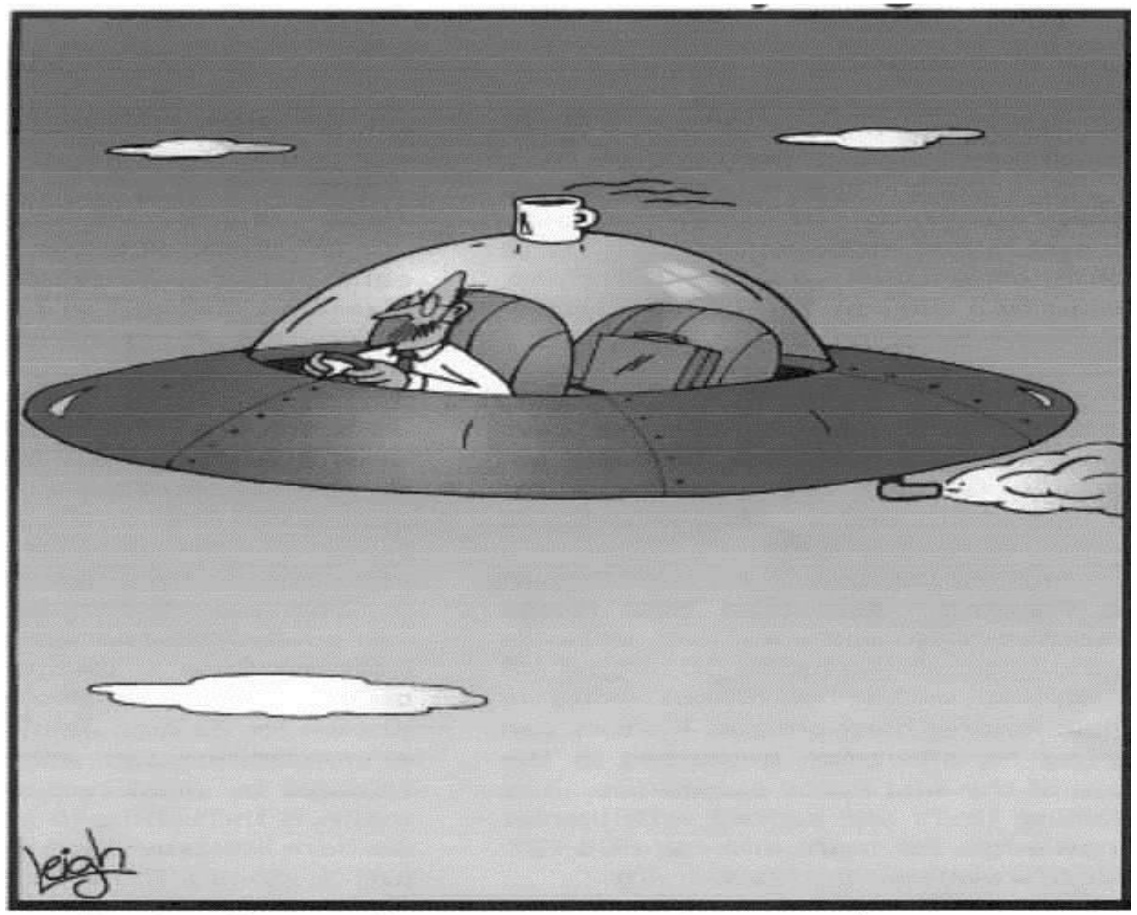
Duke | FORGE HEALTH DATA IN ACTION

Generating Evidence to Inform Decisions



National System Paradigm Shift



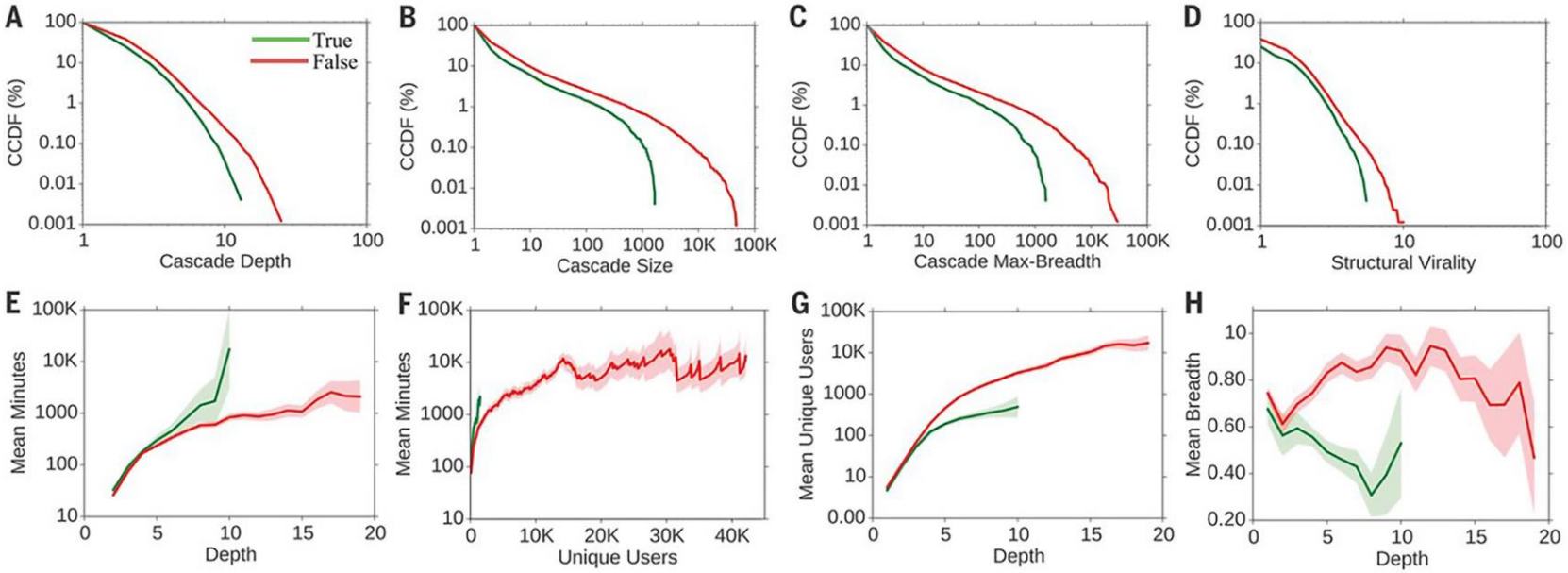


Technology advances; people stay the same.

Krista Kennell / Stone / Catwalker / Shutterstock / The Atlantic
https://www.theatlantic.com/technology/archive/2018/03/largest-study-ever-fake-news-mit-twitter/555104/

Duke | FORGE
HEALTH DATA IN ACTION

Complementary cumulative distribution functions (CCDFs) of true and false rumor cascades



Vosoughi S, et al. Science. 2018;359:1146-51.

Science
AAAS

TABLE 1 – Proportions of Polarized and Antivaccine Tweets by User Type: July 14,2014 - September 26, 2017

User Type	Polarized, %	Antivaccine, %
Assorted users, bot score, %		
<20	31	35
20-80	39***	60***
>80	26	49*,a
Unknown	37*,a	62***
Known bots and trolls		
NBC Russian trolls	20*,a	47
Content polluters	38	60***
Fake followers	0	NA
Traditional spambots	3***	0
Social spambots	18**	56*,a
Sophisticated bots	28	44
Congressional list Russian trolls	39	48

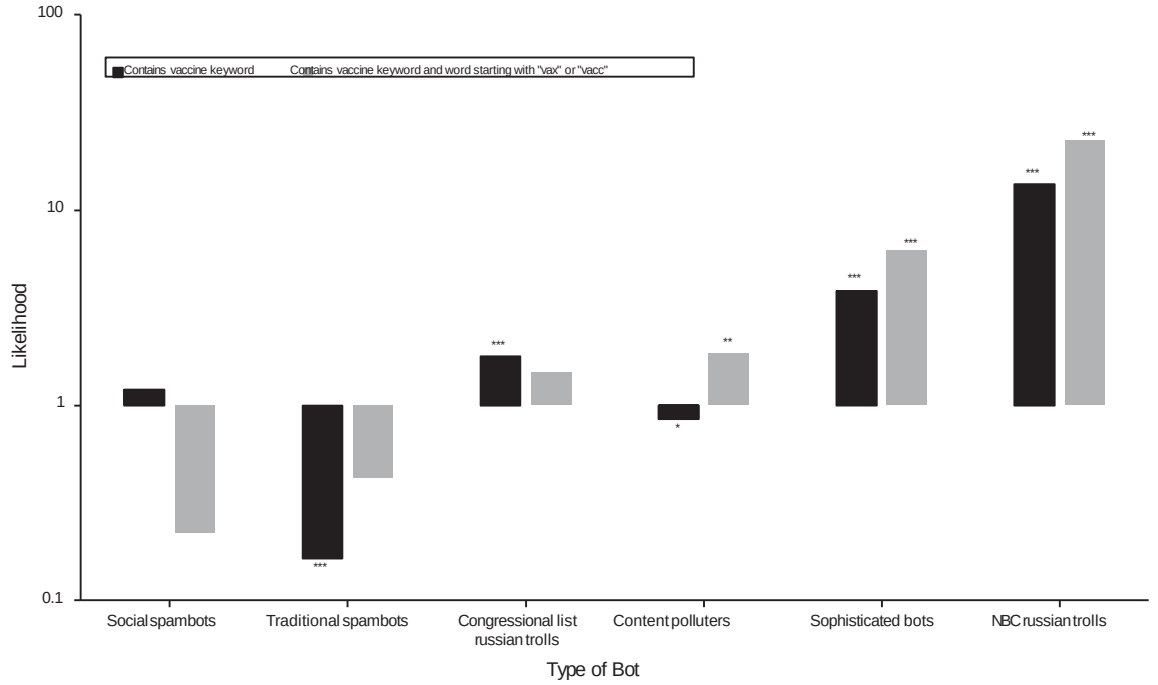
Note. NA = not applicable because of insufficient data; NBC =National Broadcasting Network. A statistically significant result indicates that a certain type of account posts polarized or antivaccine tweets at a rate that differs significantly from that of accounts with bot scores < 20% (likely humans). Polarized proportion is the ratio of all nonneutral tweets to all tweets. Antivaccine proportion is the ratio of antivaccine tweets to polarized tweets. Raw counts are shown in Table E (available as a supplement to the online version of this article at http://www.ajph.org).

a. No longer significant after controlling for multiple comparisons using the Holm–Bonferroni procedure

**P < .05; **P < .01; ***P < .001.*

Source: Broniatowski et al., "Weaponized Health Communication: Twitter Bots and Russian Trolls Amplify the Vaccine Debate" Am J Public Health. Published online ahead of print August 23, 2018: e1–e7. doi:10.2105/AJPH.2018.304567, Table 1

FIGURE 1—Bots' Likelihood of Tweeting About Vaccines Compared With Average Twitter Users: July 14, 2014–September 26, 2017



Note. NBC = National Broadcasting Network. All results remained significant after controlling for multiple comparisons using the Holm–Bonferroni procedure. Raw counts are given in Table B (available as a supplement to the online version of this article at http://www.ajph.org).

*$P < .05$; **$P < .01$; ***$P < .001$.

Source: Broniatowski et al., "Weaponized Health Communication: Twitter Bots and Russian Trolls Amplify the Vaccine Debate" Am J Public Health. Published online ahead of print August 23, 2018: e1–e7. doi:10.2105/AJPH.2018.304567, Table 1

Policy efforts underpinning RWE push

Cures provisions (Sec. 3022)

- Requires FDA to establish a program to evaluate the potential use of real world evidence to:
  - Help support the approval of new indications for an approved drug
  - Help support or satisfy post approval study requirements

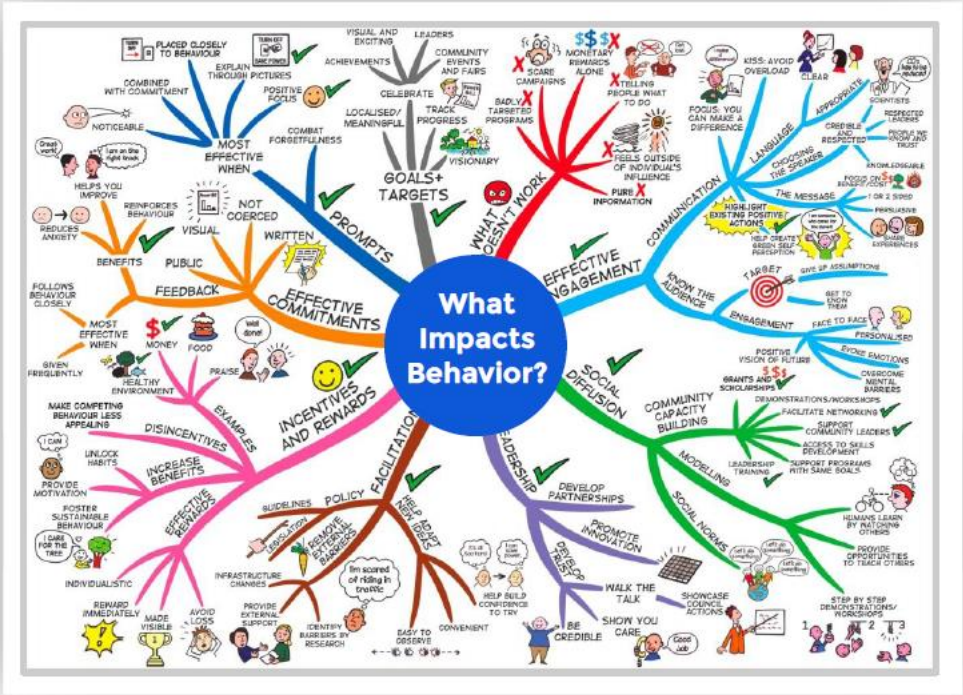
PDUFA RWE provisions

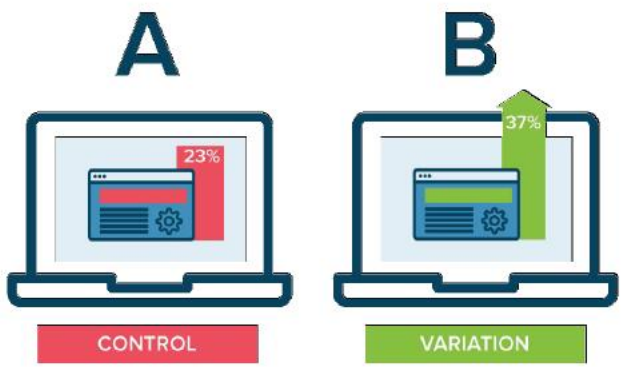
- Tracks with Cures Act
- Requires FDA to establish a program to evaluate the potential use of real world evidence to:
  - Help support the approval of new indications for an approved drug
  - Help support or satisfy post approval study requirements

Reinforcing of a Learning Health Care System:

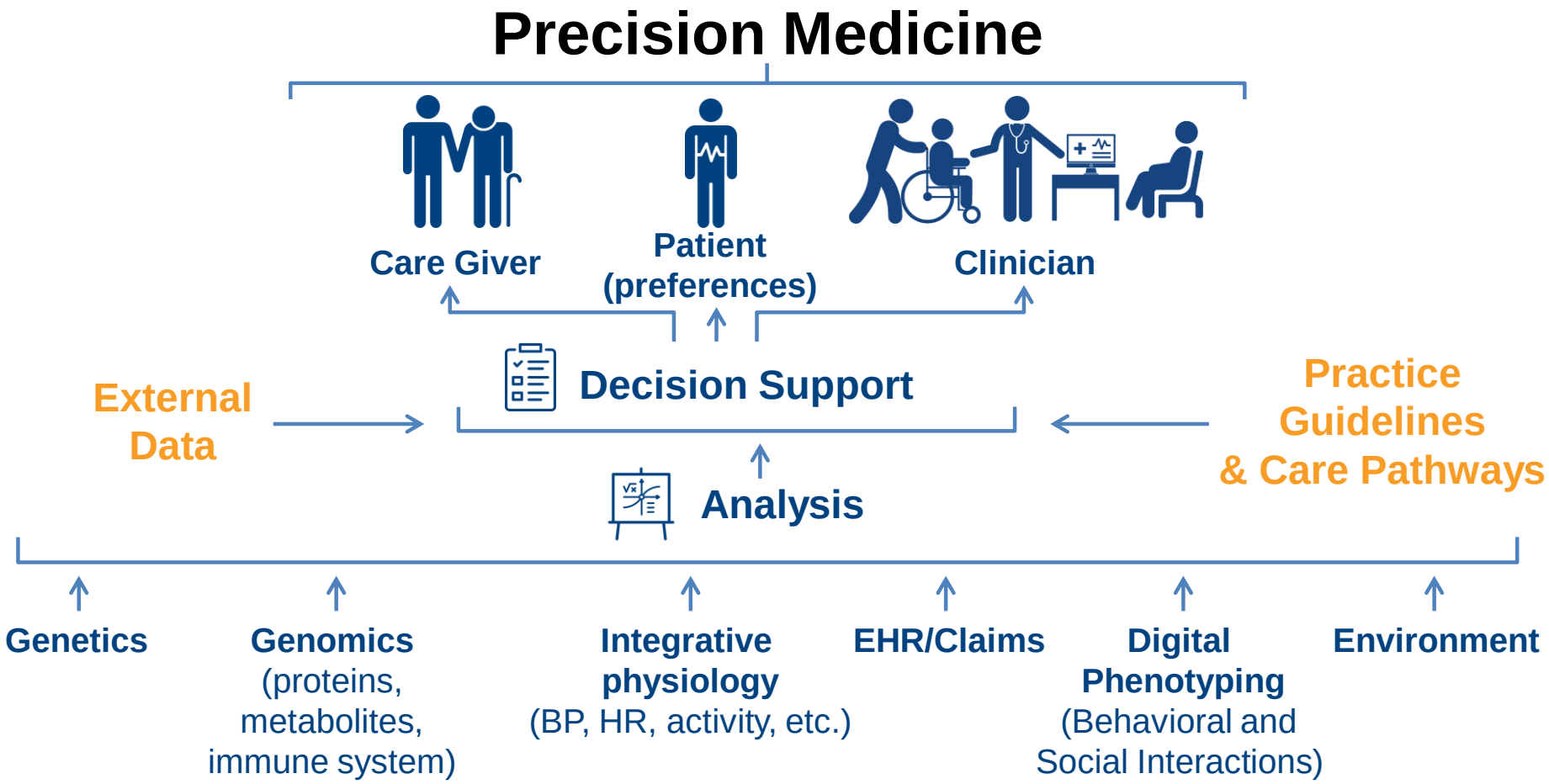
- Doesn't change approval standards, rather it better supports and enables use of data and evidence on outcomes that are hard to get from traditional RCTs (e.g., outcomes that are too costly, too small populations with particular clinical features, too long follow-up needed, diff impact in diff clinical settings, etc.)
- Learning from real-world patient experiences can support better informed health care decision-making by a range of stakeholders

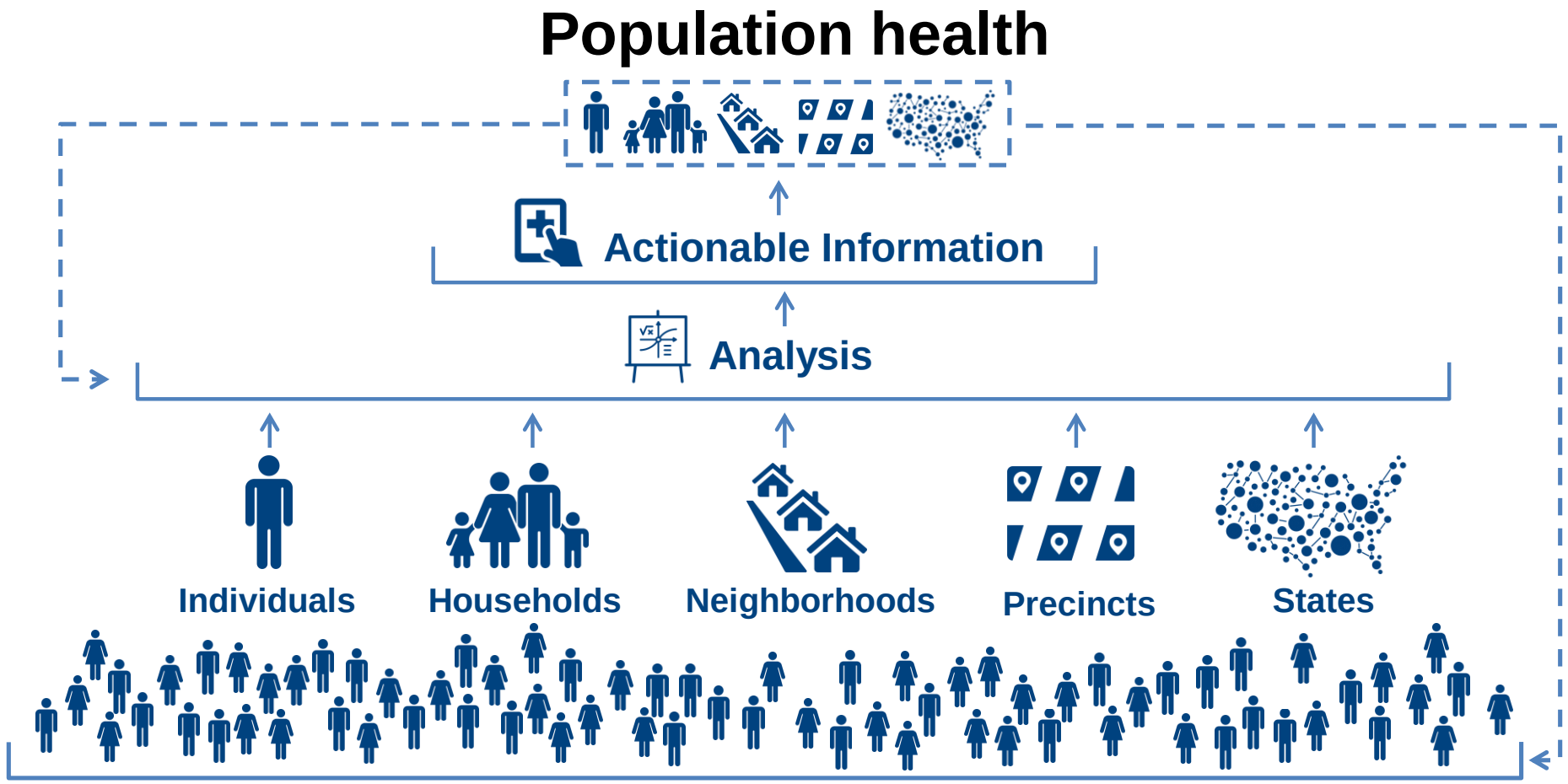
Data Activation and Testing Outcomes





Verily |


Precision Medicine
Care Giver
Patient
(preferences)
Clinician
External
Data
Decision Support
Practice
Guidelines
& Care Pathways
Analysis
Genetics
Genomics
(proteins,
metabolites,
immune system)
Integrative
physiology
(BP, HR, activity, etc.)
EHR/Claims
Digital
Phenotyping
(Behavioral and
Social Interactions)
Environment


Population health
Actionable Information
Analysis
Individuals
Households
Neighborhoods
Precincts
States

PCORnet – ADAPTABLE Update

Adrian Hernandez, MD, MHS, and Elizabeth Shenkman, PhD

Co-Principal Investigators

ADAPTABLE Key Aims

- To compare the effectiveness and safety of two daily doses of aspirin (81 mg and 325 mg) in reducing major cardiovascular events (death, myocardial infarction, stroke) in patients with a history of coronary artery disease

- To develop, refine, and evaluate the infrastructure for PCORnet to conduct multiple comparative-effectiveness trials in the future

Pragmatic Clinical Trials Roadmap:
Engagement, Electronic Health Data and Embedded Delivery

Pre-study

- Assess sites' use of EHR to facilitate research
- Usability of inclusion and exclusion criteria
- Refine protocol
- Community interaction profiles with health system
- Feasibility analysis
- Recruitment plan

Study setup

- Utilize EHR to identify local participants
- Embed encounter instructions and site content into EHR
- Pre-consent & study specific consent
- Alert clinician about trial
- Model outcomes

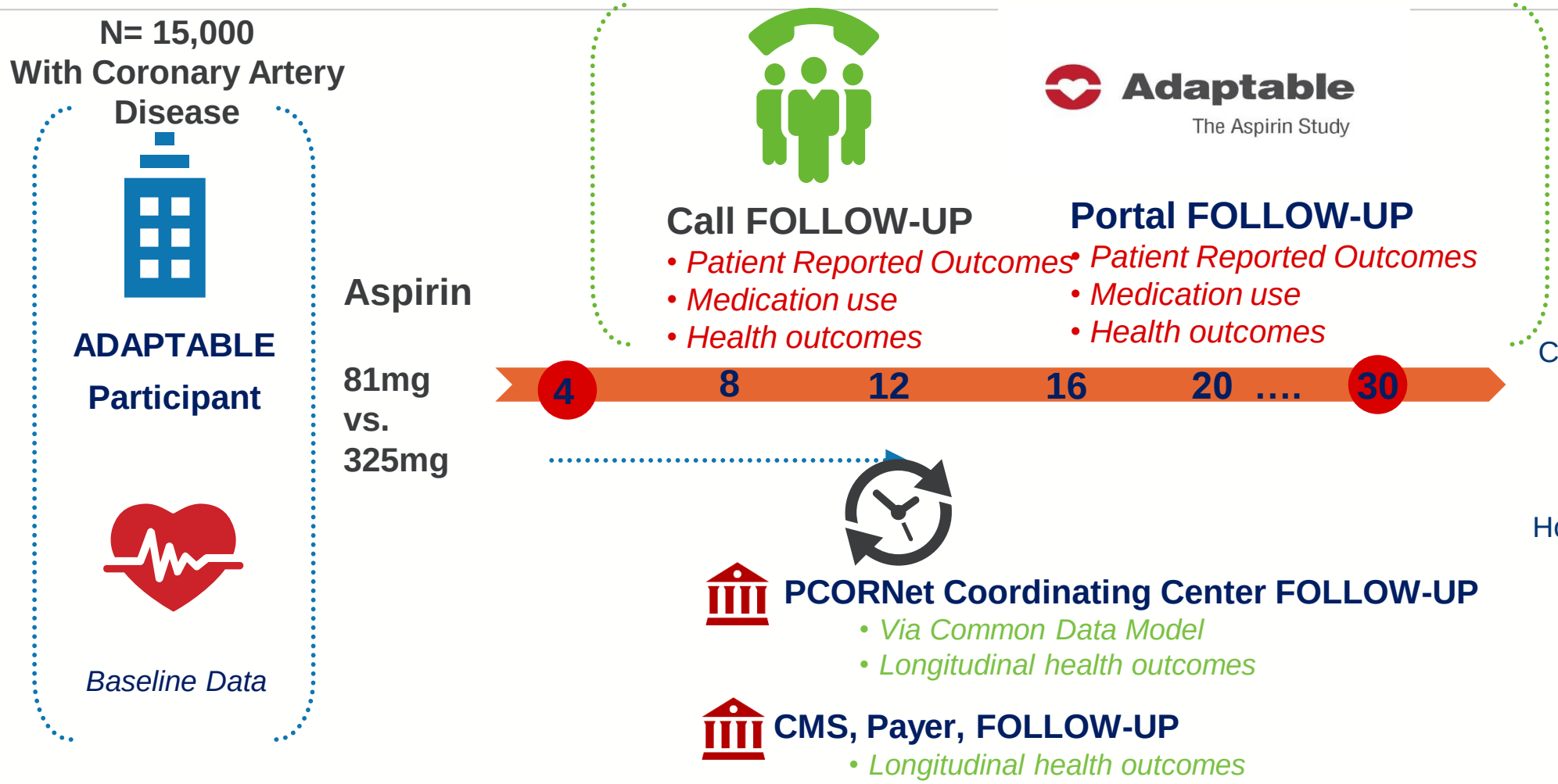
Recruitment

- E-consent with comprehension questions
- Incorporate screening criteria into EHR for
  - Scheduling patients
  - Contacting patients
  - Recruiting patients
- Alert clinician of patient eligibility
- EHR Health Portals
  - Patient opt in/out for types of studies

Study conduct

- Trials specific data capture from care delivery
- Auto-populated CRFs fields from EHR
- Extract data to facilitate work of study coordinator
- Query data to identify events
- Participant retention and education
- Return of results

ADAPTABLE Study Design: eScreening, eEnrollment and eFollow-up



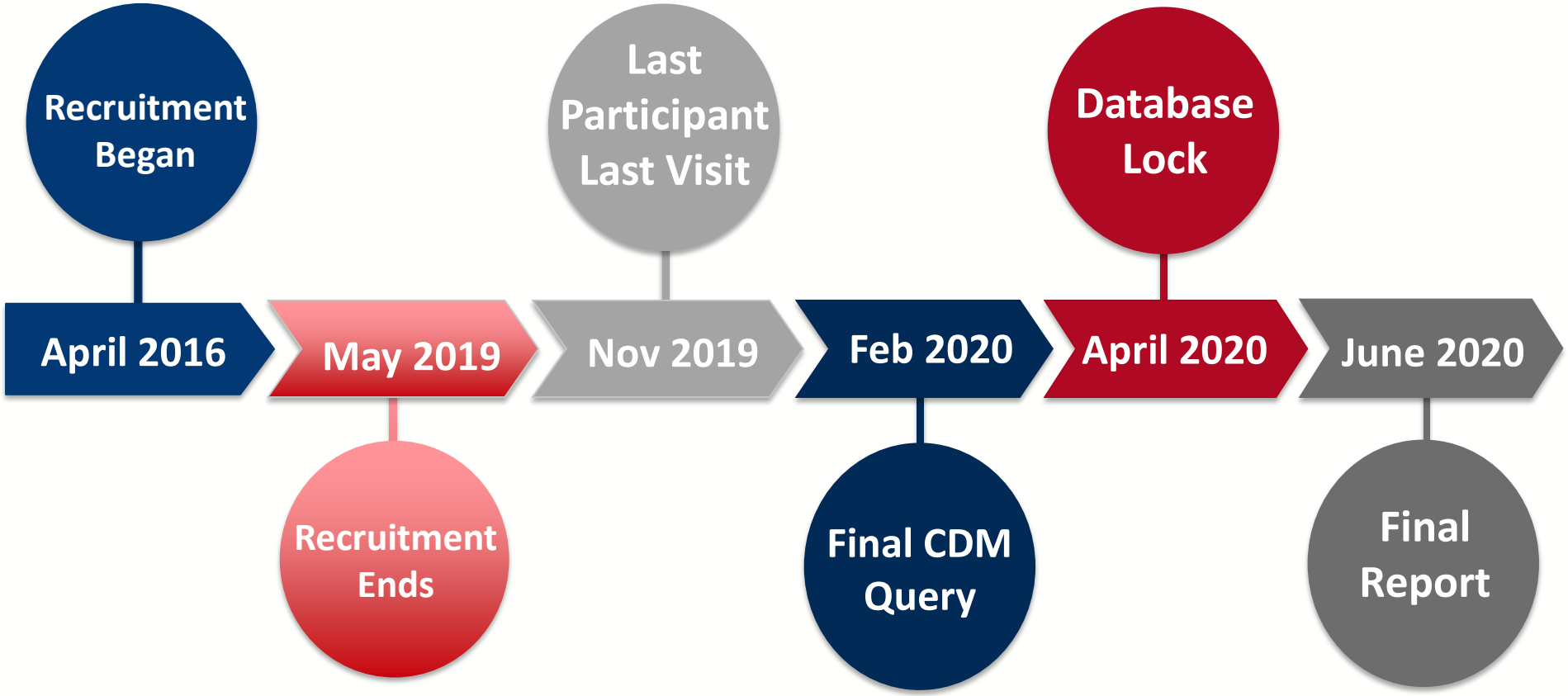
Primary endpoint:
Composite of all-cause mortality, hospitalization for MI, or hospitalization for stroke

Primary safety endpoint:
Hospitalization for major bleeding

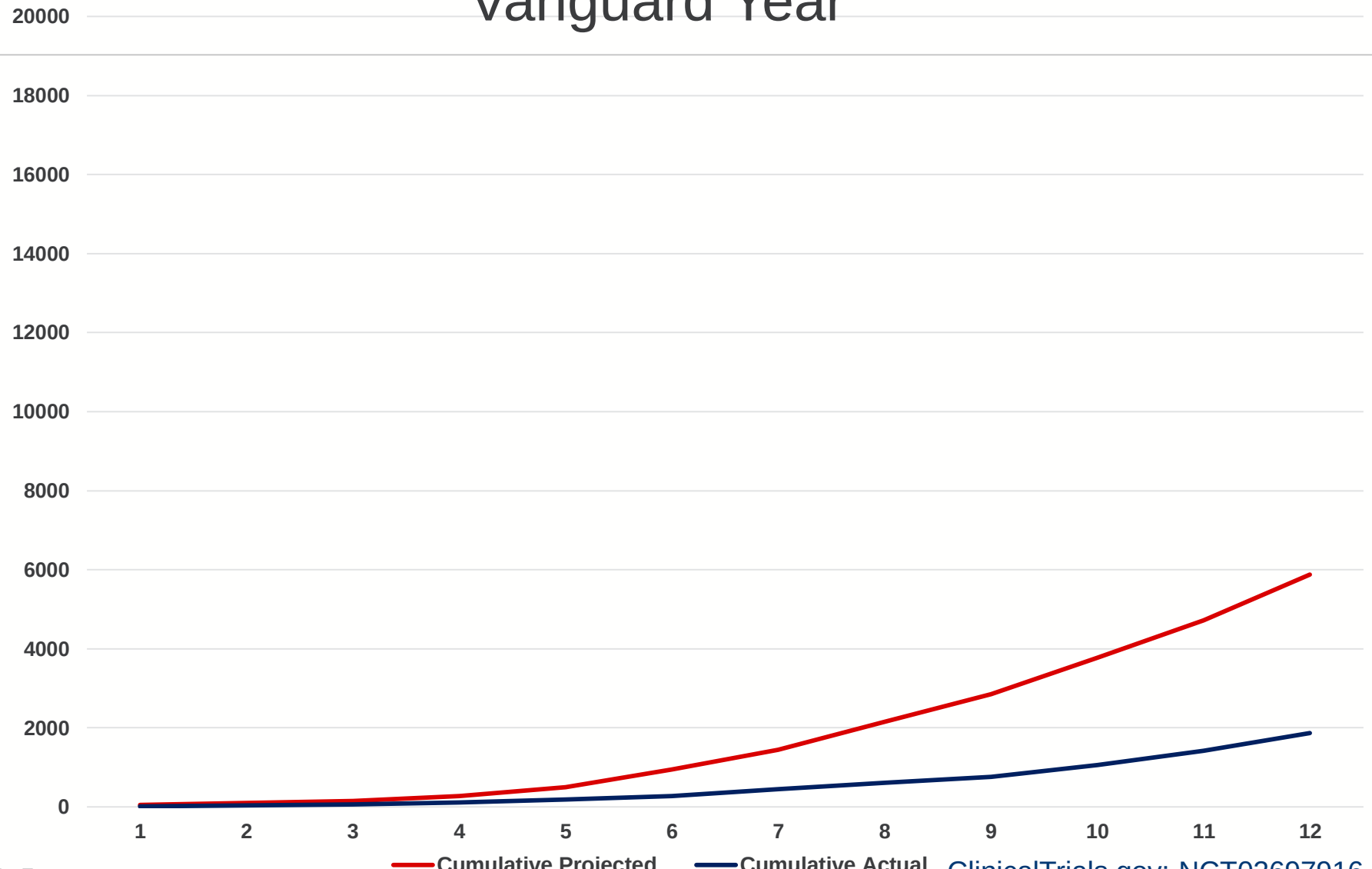
Traditional Trials vs. ADAPTABLE

	Traditional	ADAPTABLE
Incl/Excl criteria reviewed	Sample via CRA visit	Common Data Model
Representative cohort	Narrow	Broad
Consent	Facilitated	Patient-directed
Comprehension tested	No	Yes
Format	Paper	e-consent
Data collection	Patient-reported	Patient-reported
	Site-recorded	CDM
Source documents	Only seen by site	Received via CDM
Endpoint adjudication	Yes	CDM, EHR data
Patient involvement	Participants only	Protocol design, committee, analyses, dissemination

ADAPTABLE Timeline



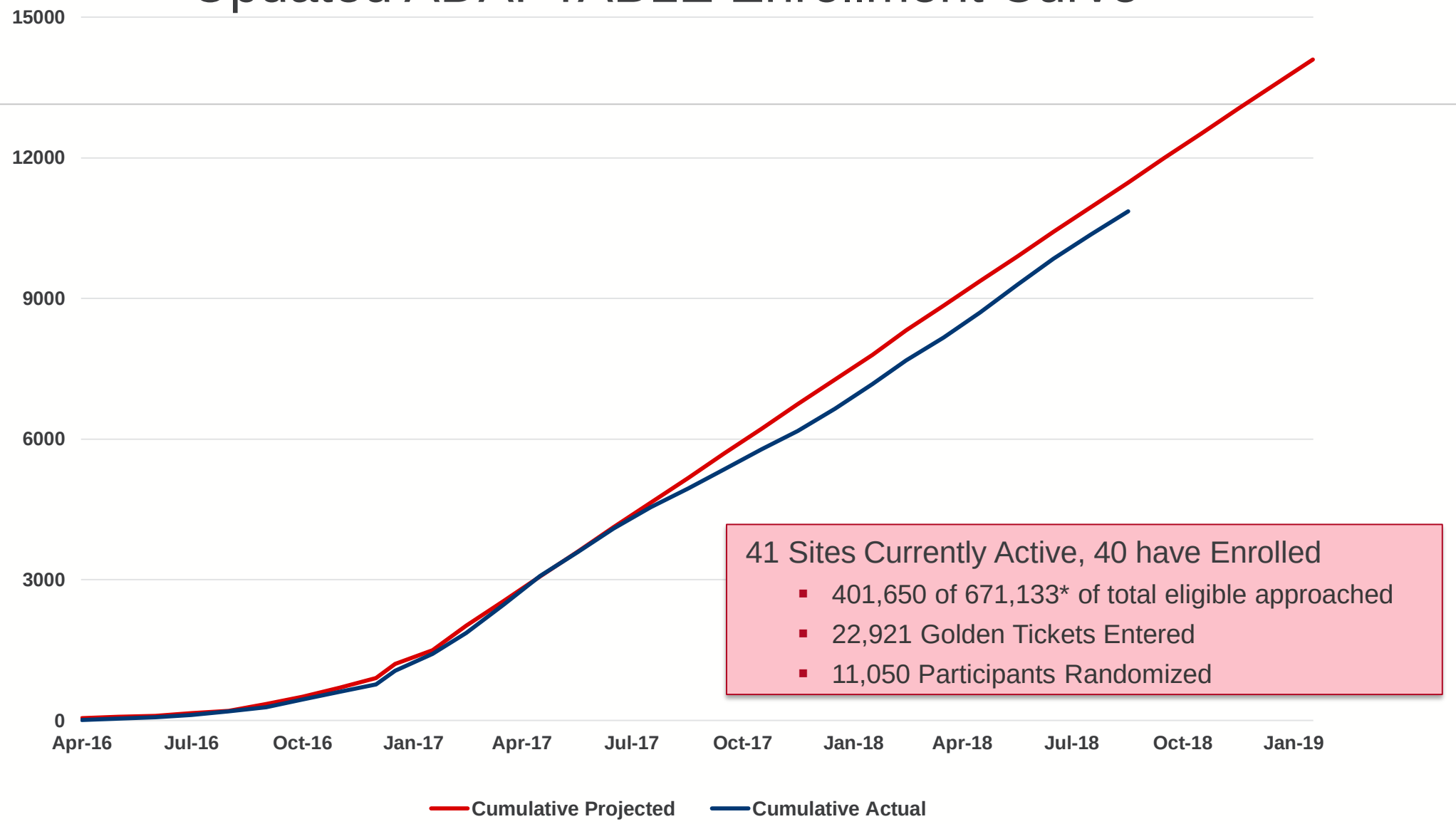
ADAPTABLE Enrollment
Vanguard Year



Adaptable

ClinicalTrials.gov: NCT02697916

Updated ADAPTABLE Enrollment Curve*



41 Sites Currently Active, 40 have Enrolled

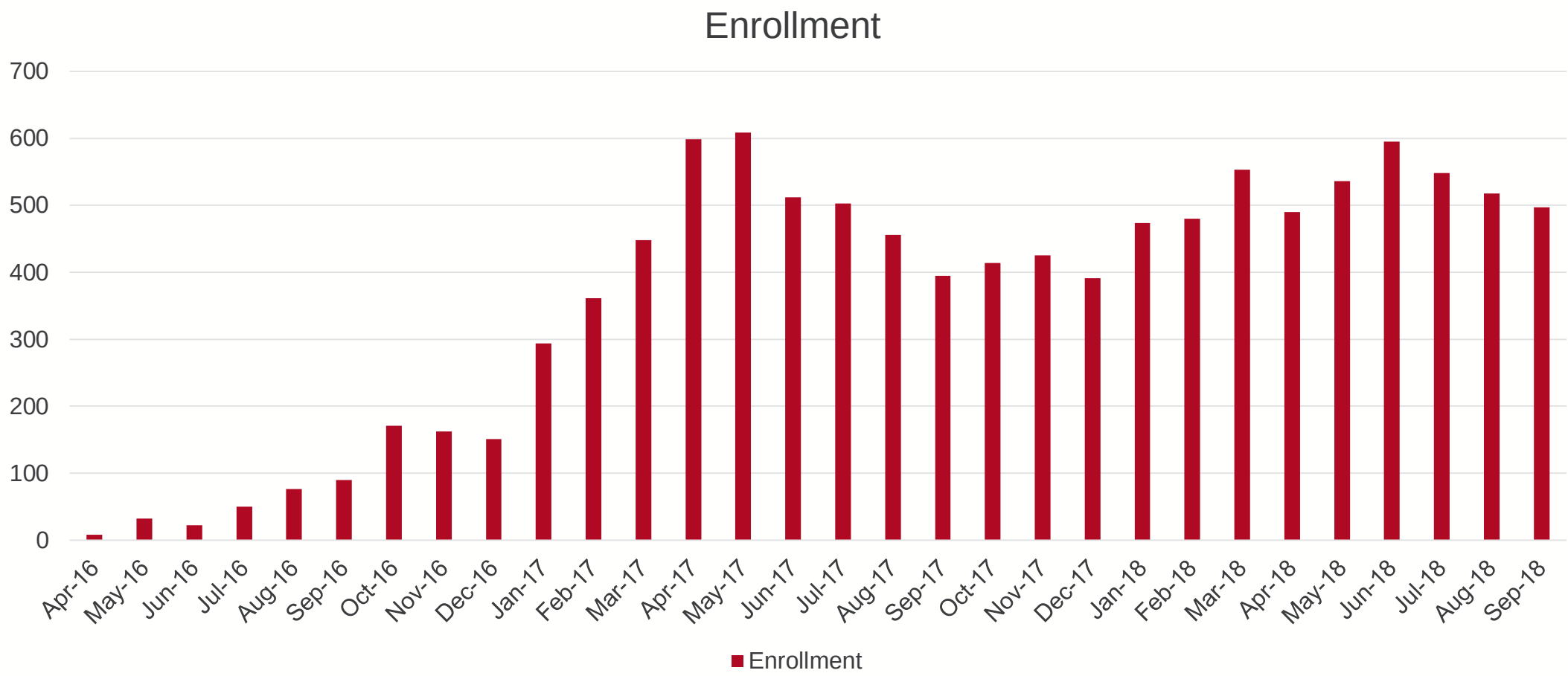
- 401,650 of 671,133* of total eligible approached
- 22,921 Golden Tickets Entered
- 11,050 Participants Randomized

Adaptable

*Modified projections based on vanguard phase

ClinicalTrials.gov: NCT02697916

ADAPTABLE Enrollment Rate (Participants/Month)



Average monthly enrollment for last 6 months= 530/month
Average needed to enroll 15K by May 2019= 525/month

Adaptable

Network Enrollment Average
Enrollment Average

CDRN	First Site Activated	Started Enrollment	Total Enrolled	Enrollment Rate/Month
MidSouth	4/18/2016	April-16	3,942	131.4
LHSNet	12/1/2017	December-17	865	86.5
GPC	7/18/2016	August-16	1,690	65
PaTH	7/18/2016	August-16	1,279	49.2
HealthCore	9/27/2017	November-17	358	32.5
NYC	11/1/2016	November-16	710	30.8
OneFlorida	11/1/2016	November-16	593	25.8
REACHnet	4/19/2016	April-16	773	25.8
CAPriCORN	8/30/2016	September-16	516	20.6
pScanner	11/7/2016	November-16	131	5.7

Demographics of Enrolled Participants Internet vs. Non-Internet

	Total (n 10,360)	Internet *(n 8,780)*	Non-Internet *(n 1,580)*	Expected
Gender				
Male	68.8%	70.2%	61.1%	
Female	31.2%	29.8%	38.9%	30-40%
Race				
American Indian/ Alaska Native	0.7%	0.6%	1.7%	
Asian	1.0%	0.9%	1.2%	
Black/ African American	9.2%	5.9%	27.7%	12.1%
White	76.0%	79.7%	55.6%	
Multi-Race	0.9%	0.9%	1.0%	
Other	2.8%	1.7%	8.5%	
Unknown	9.3%	10.2%	4.3%	
Ethnicity				
Hispanic	3.3%	2.2%	9.3%	12.3%
Non-hispanic	85.3%	85.1%	86.3%	
Unknown	11.4%	12.7%	4.4%	
Age				
< 45	1.8%	2.0%	0.8%	
45-54	8.5%	9.0%	5.6%	
55-64	24.9%	25.1%	23.5%	
65-74	45.4%	46.1%	41.6%	
>75	19.3%	17.7%	28.4%	10% >75

Withdrawn Consent & Limited Participation

	Randomized	%WDC	% Limited Participation*
Total	**10,360**	1.7%	1.1%

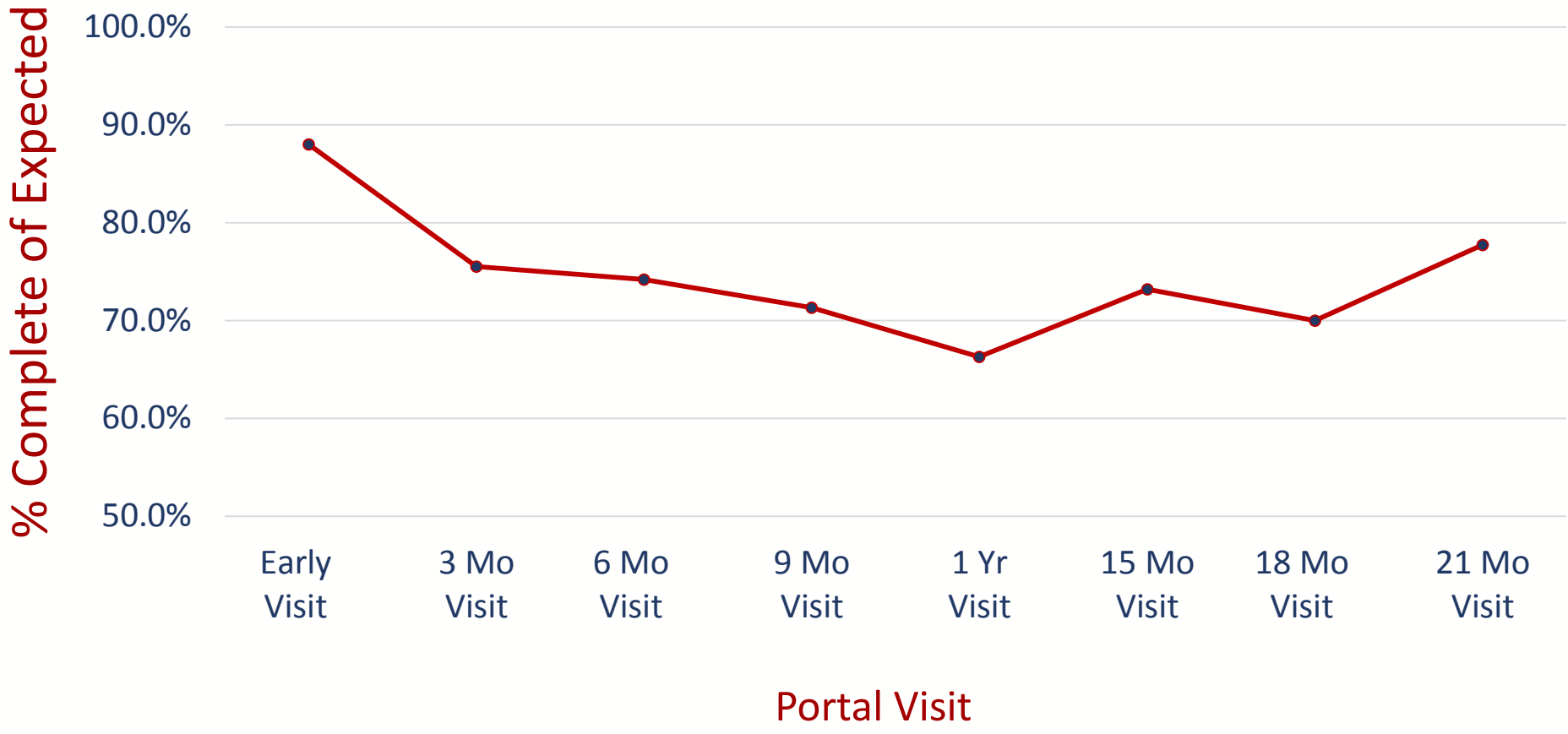
- **Median time to WDC: 97 Days**

  Range (0,587)

- **Most common reasons reported for changing participation level:**
  - #1 Medication/Health Issues
  - #2 Privacy Concerns

*Limited participation: may use electronic records or limited contact

Visit Completion Rates



ADAPTABLE Call Center for Complete Outcomes:

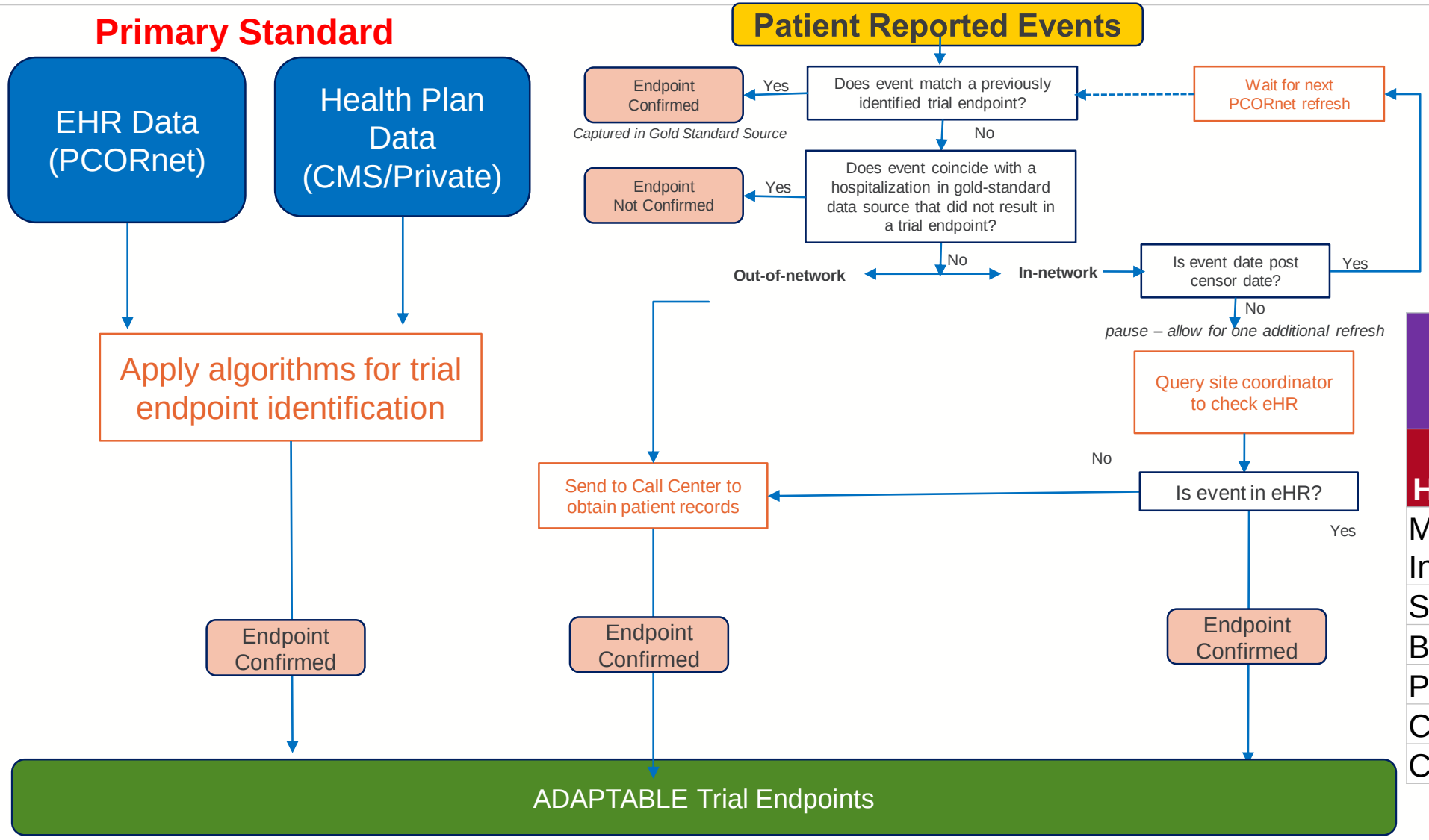
Portal Missing
- 51% complete (430/845)
- 3% partial/incomplete (25/845)
- 46% not done (390/845)

Non-internet partiicipants:
- 86% complete (2680/3113)
- 2% partial/incomplete (62/3113)

12% not done (317/3113)

*Based on visits not participants

ADAPTABLE Endpoints
Understanding Outcomes and Reconciliation



Patient Reported Event Reconciliation	
Reason for Hospitalization	**Confirmation Rate**
Myocardial Infarction:	53.6%
Stroke:	40.0%
Bleeding:	64.7%
PCI:	66.1%
CABG:	56.3%
Chest Pain:	13.5%

What Have We Learned from Adaptable?

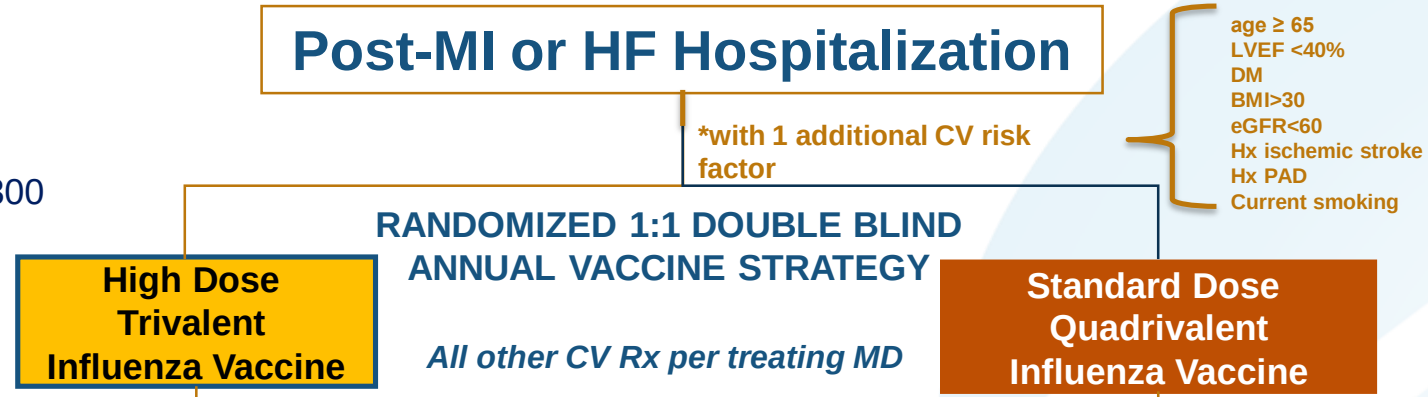
Successes	Lessons Learned
E-approach of ~ 400,000	E-Identification and e-approach
Continued recruitment of ~500/month	Multi-touch approach
Integration of health plan research Networks	Harmonization of endpoints
Majority of follow-up virtually	Validation of endpoints
Challenges	**Improvement for Future Studies:**
Migration of health encounters	Test different portal engagement models
Inconsistent self-reporting of events	Understand preferences for follow-up
Transitions from PCORnet 1.0 -> 2.0	Increase cross- system data linkage

What's Next?

Other Ongoing Pragmatic Trial Examples

INVESTED

A pragmatic trial to compare outcomes of high versus low dose influenza vaccine in 9,300 high--risk CV patients



Duration
3 Influenza Seasons
+ Vanguard Season

Primary EP
Death or Cardiopulmonary Hospitalization

INVESTED

In Year 02, PCORnet had fewest sites (n=21), but delivered most patients per site (on average)

Year 03 is starting now and as of October 4th, 22 of 25 are activated for enrollment

Y2 enrollment ended January 31, 2018

- **TOTAL enrollment = 2,626 new**

Network	Number Enrolled (new)	Enrollment/Site
Non- VA U.S. (67 sites)	1,037	15.5
Canada (51 sites)	705	13.8
VA (34 sites)	491	14.4
PCORnet (21 sites)	393	18.7

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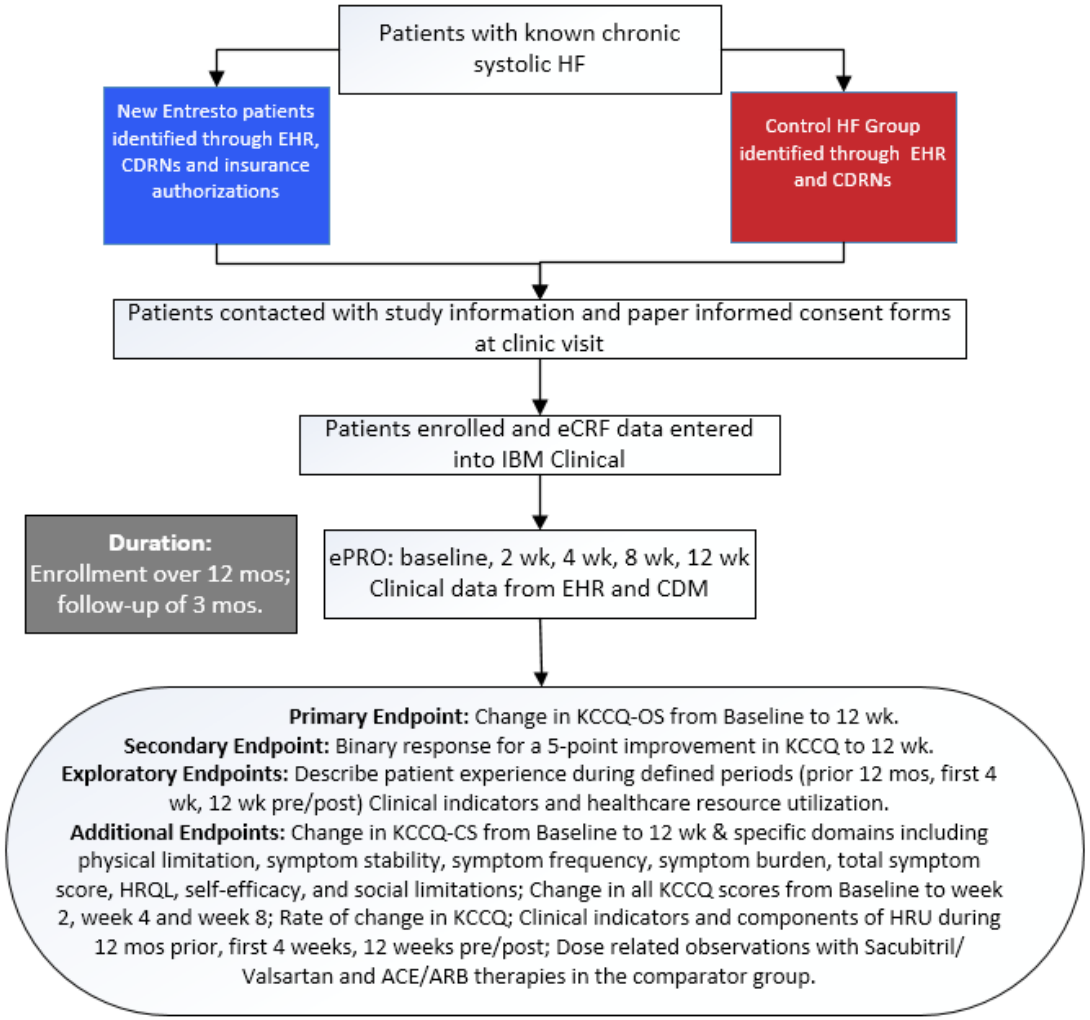
PROVIDE-HF

A prospective cohort study of approximately 400 chronic heart failure patients. One group will be identified who were initiated on sacubitril/valsartan (Entresto) and compared against group of similar HF patients.

Patient reported primary outcome to determine impact on physical limitations, symptoms, self-efficacy, social interference, and quality of life.



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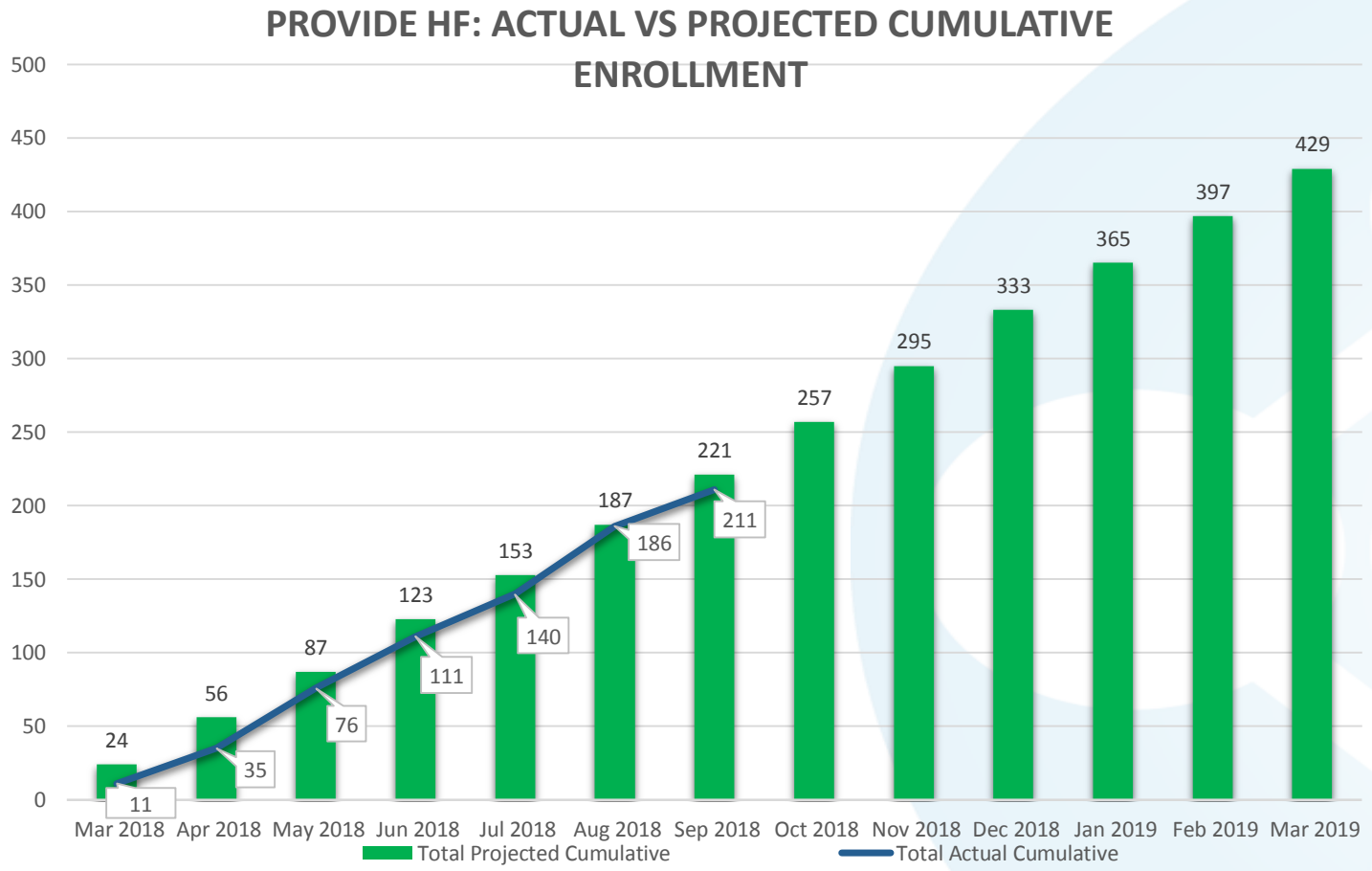
PROVIDE-HF

As of September 25th, have enrolled 214 people (54% of goal) across 18 PCORnet sites.

Enrollment continues through March, 2019.



Future Opportunities

- Pragmatic Clinical Trials
  - Engaged network
  - Research ready data to design studies
  - Recruitment methods better compared with historical standards
  - Sponsor interest due to changing landscape (21st Century Cures Act, PDUFA)
- Development of Real World Evidence
  - Prospective, observational
  - Patient reported outcomes
- Implementation of Evidence into Practice
  - Engaged health systems
  - Research ready data for surveillance and reporting

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Future Opportunities – Case Example

- In August 2018, NIH released a RFP to determine effects of statins in older adults without cardiovascular disease
  - 18,000 adults aged 75+
  - Primary outcome is death or new onset of dementia or disability
- $38.8 million over 7 years
  - 10 Cores, organized around a CCC and DCC
- PCORnet is organizing a multi-network response
  - 7 Clinical Research Networks Participating + VA Network
  - All CRNs and patient partner included in planning workgroup (and eventual Steering Committee)

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BREAK

We will return at 3:30 pm ET

Join the conversation on Twitter via @PCORI

Proposed FY2019 Funding Commitment Plan

Joe Selby, MD, MPH
Executive Director

Proposed FY2019 Commitment Plan
Based on Revenues Expected through FY2019

- The Board approved our FY2019 Budget for ***Expenses*** at its last meeting in September
  - Budget includes Expenses on Awards and Operating Expenses
  - FY2019 Expenses largely reflect *commitments already made* in previous years
- Today the Board considers our proposed plan for ***Commitments*** to Awards in FY2019
  - For a large portion of the commitments planned for FY2019, the activities that will result in them began in FY2018 – for example, funding announcements made in FY2018 with LOIs, applications, merit review, or selection committee in process
  - *Major commitments, such as research funding slates, will come to the Board for final approval* – smaller commitments, such as Engagement Awards, follow a different Board-approved process and are summarized for the Board quarterly
- The plan provides ***guiderails for FY2019 commitments*** to awards
  - Actual commitments depend on the particulars of each funding announcement
  - *Significant departures from the plan would come back to the Board for approval*

Commitment Plan Overview

Category – (subcategories in white)	Through FY2018 ($M)	FY2019 ($M)	FY2020 ($M)	FY2021 ($M)	Total ($M)	Total (%)
Engagement Total	**88**	**21**			**109**	**4%**
Research Total	**1,845**	**255**	**11**		**2111**	**77%**
Research in Programs (includes Methods)	1,750	246	11		2,007	73%
Research in PCORnet	95	9			104	4%
Dissemination Total	**28**	**30**	**30**	**34**	**122**	**4%**
Infrastructure Total	**376**	**25**	**6**	**6**	**413**	**15%**
PCORnet	364	19			383	14%
Workforce	12	6	6	6	30	1%
Total ($M)	**2,337**	**331**	**47**	**40**	**2,755**	
Total (%)	**85%**	**12%**	**2%**	**1%**		**100%**

Engagement and Dissemination

Engagement Commitments	Through FY2018 ($M)	FY2019 ($M)	FY2020 ($M)	FY2021 ($M)	Total
Eugene Washington Engagement Awards	78	21			**99**
Pipeline to Proposal Awards	10				**10**
Total	**88**	**21**			**109**

Dissemination Commitments	Through FY2018 ($M)	FY2019 ($M)	FY2020 ($M)	FY2021 ($M)	Total
Public Reporting	10	7	7	7	**31**
Horizon Scanning		2	3	3	**8**
Dissemination & Implementation Awards	18	21	20	24	**83**
Total	**28**	**30**	**30**	**34**	**122**

Research in Programs

Research Commitments	Through FY2018 ($M)	FY2019 ($M)	FY2020 ($M)	FY2021 ($M)	Total
Pilots	31				**31**
Broad PFAs	815	124			**939**
PCS PFAs	450	37			**487**
Targeted PFAs	451	72			**523**
Other Research Funding (systematic review updates, evidence maps, and other)	3	13	11		**27**
Total	**1,750**	**246**	**11**		**2,007**

PCORnet and Workforce Development

Research in PCORnet Commitments	Through FY2018 ($M)	FY2019 ($M)	FY2020 ($M)	FY2021 ($M)	Total ($)
Research in PCORnet	**95**	**9**			**104**

PCORnet Infrastructure Commitments	Through FY2018 ($M)	FY2019 ($M)	FY2020 ($M)	FY2021 ($M)	Total ($)
PCORnet	323	4			327
PCRF	41	15			56
Total	**364**	**19**			**383**

Workforce Commitments	Through FY2018 ($M)	FY2019 ($M)	FY2020 ($M)	FY2021 ($M)	Total ($)
AHRQ – PCORI LHS Traineeship	**12**	**6**	**6**	**6**	**30**

Commitment Plan Overview

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Total ($M)	**2,337**	**331**	**47**	**40**	**2,755**	
Total (%)	**85%**	**12%**	**2%**	**1%**		**100%**

Board Vote

Call for a Motion to:	• **Approve** the Proposed FY2019 Funding Commitment Plan
Call for the Motion to be Seconded:	• **Second** the Motion • If further discussion, may propose an **Amendment** to the Motion or an **Alternative** Motion
Roll Call Vote:	• Vote to **Approve** the **Final** Motion • Ask for votes in favor, opposed, and abstentions

Strategic Plan Update: Efforts to Address Emerging Interventions

Jean Slutsky, PA, MSPH
Chief Engagement & Dissemination Officer

Progress Report on Updating PCORI's Strategic Plan

#1: Balance Our Efforts Across 3 Main Lines of Work (funding research, dissemination and implementation, influencing how research is done) – but keep focus squarely on Research

#2: Address issues related to assessment of new high-cost technologies – new products

#3: Expand our options for supporting CER for health decision-making

#4: Continue to work toward greater specificity in all PCORI solicitations and more focus in research portfolio

#5: Greater Emphasis on Dissemination Including Shared Decision-Making

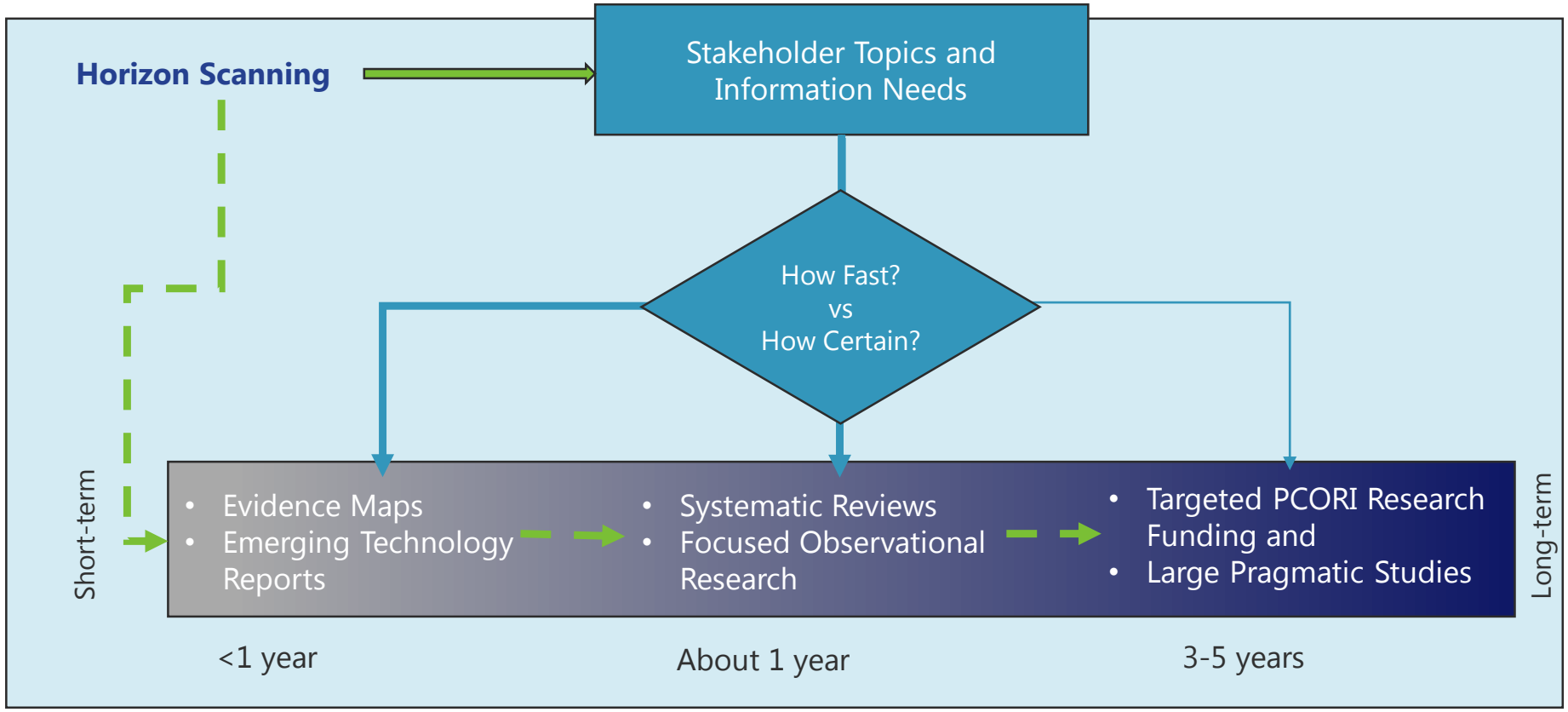
#6: Find ways to include and use PCORnet in organizational activities

#7: Re-visit our National Priorities and Research Agenda, with public engagement

Patients and Other Stakeholders Decisional Needs

- PCORI regularly convenes stakeholders to understand their decisional needs
- Several themes have coalesced
  - Emerging technologies and therapeutics are increasingly creating pressing information needs
  - Knowing what might be on the horizon can help frame decision making and research priorities
  - While long-term clinical studies are important, shorter-term research products would be very helpful
  - Understanding the context and “state of the science” around emerging technologies and therapeutics is a useful adjunct to short-term decision making

PCORI's Efforts to Create Shorter-Term Research Products to Meet Decision Maker Needs



Focused Rapid-Cycle Research Products

- **PCORI is establishing a Healthcare Horizon Scanning Program by 12/2018**
  - Horizon scanning identifies and monitors target technologies and therapeutics in healthcare and serves to identify key research questions
- **PCORI is commissioning emerging technology and therapeutics briefs**
  - The briefs are intended to meet information needs and inform more definitive research; our first, on gene therapies (CRISPR/Cas9 and CAR-T) is due in December, 2018
- **PCORI is commissioning updated and novel systematic reviews and evidence maps**
  - Reviews support decision-making and stakeholder efforts to develop or refine clinical guidelines and shared decision making tools and research prioritization
- **Rapid Cycle Research Products**
  - Access to data in common format across a number of health systems, clinics, and patient groups, to be able to analyze the data
- **PCORI continues to develop evidence updates and shared decision making tools**
  - These products promote uptake of evidence from these activities, as well as PCORI-funded CER

Horizon Scanning

- Goal: Identify and monitor target technologies and therapeutics in healthcare
  - Forecast which target technologies:
    - Will be high impact (e.g., clinical care, care delivery, outcomes, costs)?
    - Will be disruptive?
- Purpose: Help payers, patients, clinicians, researchers, and PCORI identify and monitor important new therapeutics and technologies before they enter the market
- Use: Identify key research questions that PCORI can address as the technologies enter the market

Reports on Emerging Technologies and Therapeutics

- Goal: Provide timely information for patients, clinicians, and payers on new drugs /devices (e.g., from horizon scan reports to evidence summaries, evidence mapping, and more in-depth evidence syntheses, as appropriate)
- Purpose: "Pre-PCORI research" evidence summaries intended both to meet information needs and to inform more definitive research
  - Develop with stakeholder input
  - Include patient-centered outcomes, healthcare delivery disruptions, and comparisons that need to be addressed in order to measure value
  - Identify on-going studies, including patient populations, settings, etc.
  - Discuss regulatory and policy environment
- Use: Provide clarity about available evidence and areas of uncertainty for decision makers, identify further research priorities and development that would better determine the technology's value, inform other investments

Inaugural Emerging Technology Report- Gene Therapies

- Provide an Evidence Map and Landscape Review of Gene Therapies to include:
  - Chimeric Antigen Receptor T-Cell (CAR-T) Therapies
  - Gene Modifications including CRISPR/Cas9 or similar processes
- Nominated by public and private payers
- Informed by stakeholder interviews
- Landscape review to evaluate what conditions may have relevant therapies in the next 5 years that could be considered high-impact
- Key-informant interviews for issues that may not be clear on review (e.g., potential for adverse events, durability of effect)

Rapid-Cycle Projects

- **Goal:** To provide focused studies using PCORnet Collaborative Research Groups to answer useful questions for stakeholders
- **Purpose:** Through access to data in common format across a number of health systems, clinics, and patient groups, to be able to analyze the data to answer questions such as:
  - Patterns of utilization
  - Monitoring for safety signals (e.g., potential adverse events)
  - Surveillance
- **Use:** Provide data to better understand current care patterns, to inform policy decision making and future research

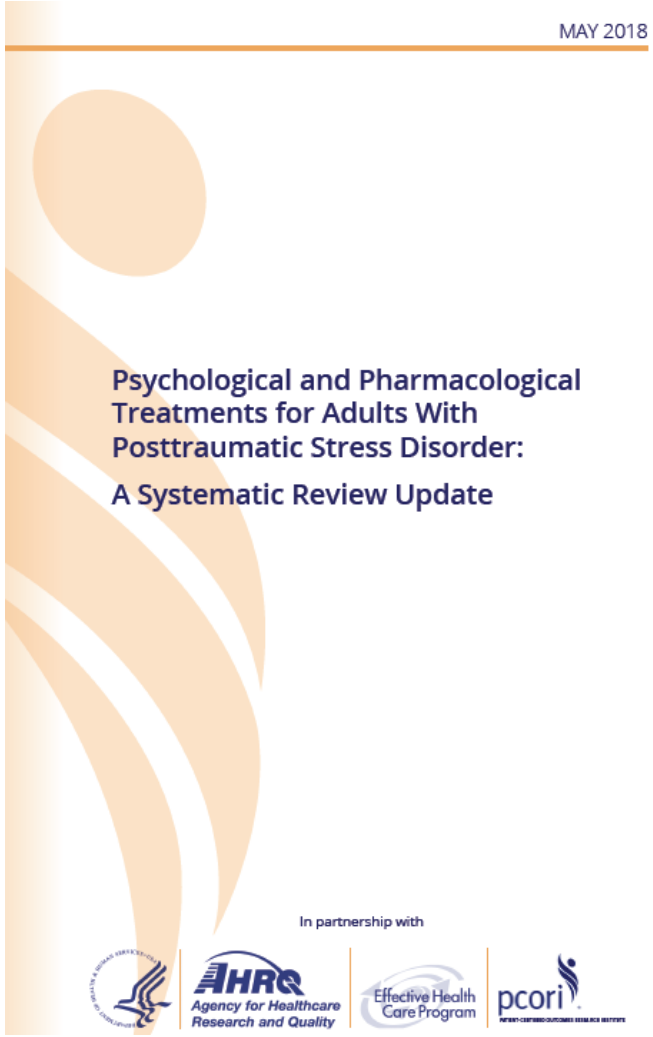
Topic Area	Question	Project Completed
PCSK9	Trends and current prevalence of use of PCSK9 Inhibitors in persons who are eligible for statin therapy	April 2018
Cancer	Patterns of use of molecular and genetic biomarkers/targeted cancer treatment for new onset solid tumors	August 2018
Hep C	Do pts with Hep C who are prescribed newer direct acting antiviral meds experience higher rates of adverse events than pts with Hep C who are untreated?	Nov 2018
T2DM	Trends in use of newer agents in treatment of T2D and the potential for outcomes studies	Dec 2018
Opioids	Evaluate and Demonstrate the Fitness for Use of PCORnet for surveillance of the opioid epidemic	March 2019

Evidence Maps

- Goal: Inform clinical and research decision making through systematically searching research evidence, and displaying the findings in an accessible, usually visual, format
- Purpose: Develop an easily understandable visual representation of the availability of research evidence that helps patients, clinicians, researchers, and policy makers understand
  - How much research has been done (and sometimes what that research shows)
  - What questions research HASN'T answered (evidence gaps)
- Use: To help with evidence-informed decision making, and to highlight and prioritize evidence gaps for future research

Systematic Reviews

- Goal: To comprehensively synthesize research evidence so that patients, clinicians, and policy makers have the best available evidence on care interventions
- Purpose: To understand whether and how conclusively the body of research evidence supports (or does not support) the effectiveness of health care interventions
- Use: decision making about care by patients, clinicians, policy-makers; identifying research gaps



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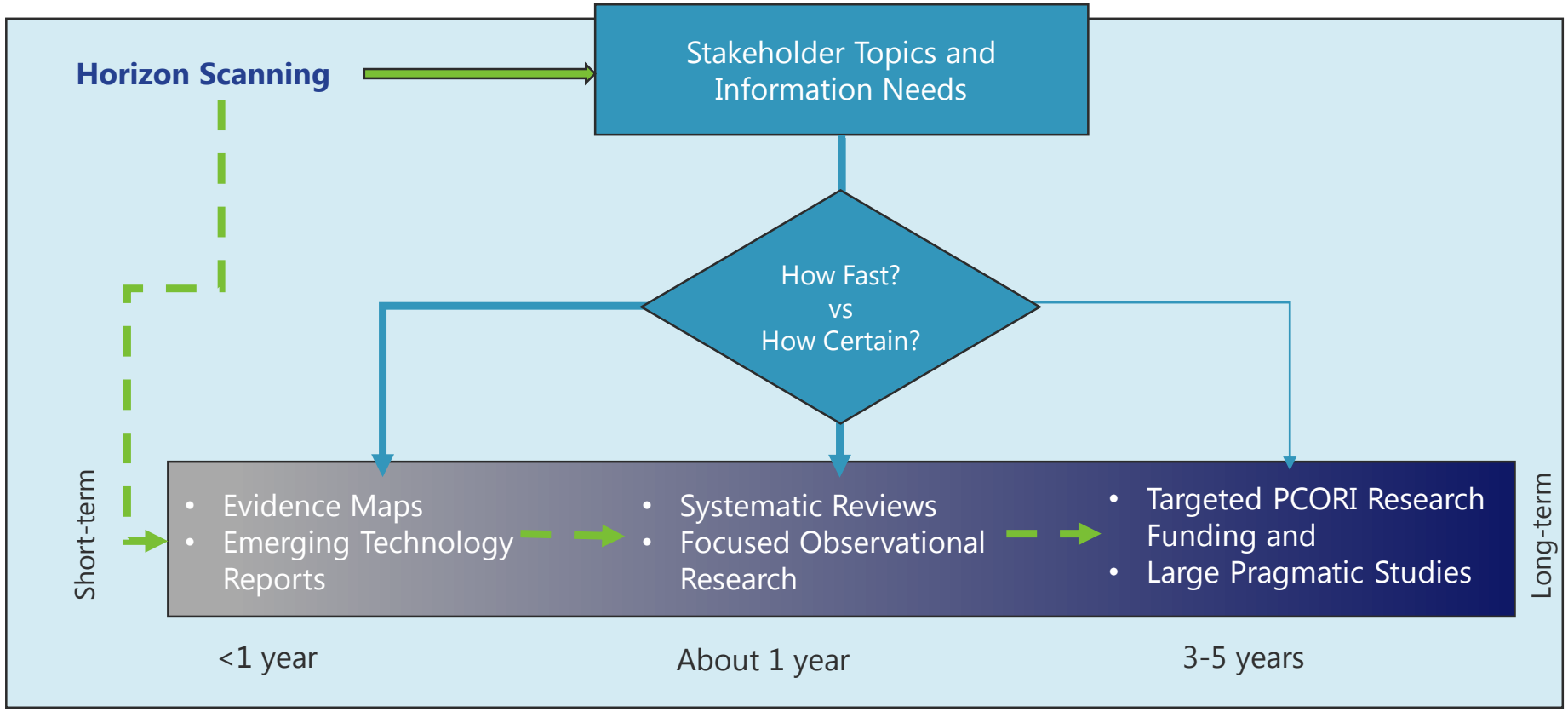

Public Comment Period

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Wrap Up and Adjournment


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