Board of Governors Meeting

In-Person and via Teleconference/Webinar

May 13, 2019
9:00 AM – 1:00 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

9:00 AM	**Call to Order and Welcome**
9:00 – 9:10	**Consider for Approval:** Consent Agenda
9:10 – 10:00	**Executive Director's Report & Q2 Dashboard Review**
10:00 – 10:15	**Research Portfolio Exploration Series:** Portfolio Overview
10:15 – 11:15	**Research Portfolio Exploration Series:** Focus on Telehealth Portfolio
11:30 – 11:45	**Consider for Approval:** Funds to Support Implementation of the PCORnet Common Data Linkage Method
11:45 – 12:30	**Dissemination and Implementation Update:** Overview and Project Focus
12:30 – 1:00	**Public Comment Period**
1:00 PM	**Wrap up and Adjournment**

Consent Agenda Items

Grayson Norquist, MD, MPH

Chairperson, Board of Governors

Motion for Consent Agenda Items

That the Board approve:

- Minutes from the April 16, 2019 PCORI Board of Governors Meeting
- Amended Governance Committee Charter with provisions addressing:
  - The process by which a Vice-Chair is named, and
  - Voting by written consent on audit matters to be consistent with DC nonprofit law

Board Vote

Call for a Motion to:	• **Approve** each of the Motions on the Consent Agenda
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

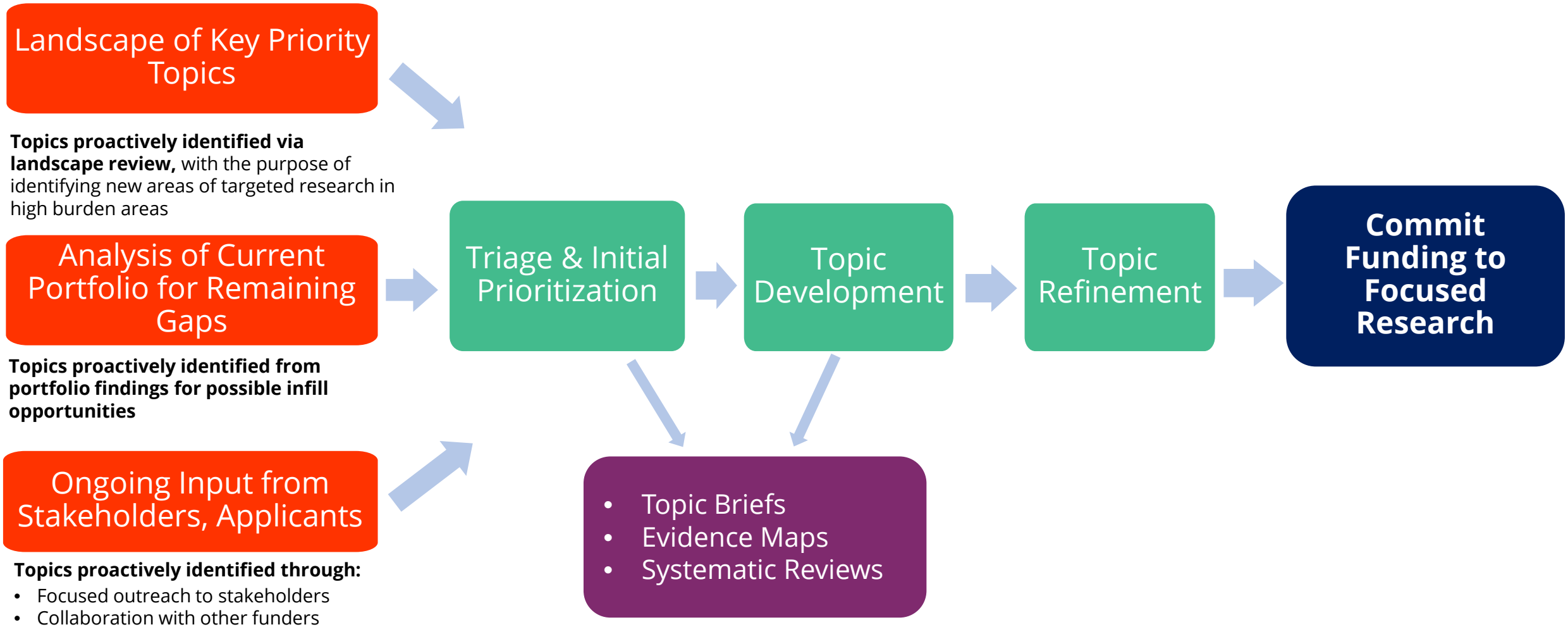
Joe Selby, MD, MPH

Executive Director

Upcoming Discussions for Future Board Meetings

- During the Board Planning Session in March, the Board began thinking about the year ahead with reauthorization on the horizon
- The Board identified several topics for discussion at upcoming board meetings over the course of the year to start planning for "PCORI 2.0" including:
  - Revitalizing, enhancing and making our stakeholder-driven topic generation process more transparent.
  - Exploring our research portfolios as one way to generate the next round of targeted research topics. ***This exploration series kicks off today with a look at the telehealth portfolio.***
  - Enhancing our focus and activities to promote worthy research findings with dissemination and implementation activities. ***This discussion begins today.***

PCORI's Topic Pipeline for Focused Research:
Process for Topic Capture, Generation, and Prioritization



Portfolio Exploration Series

A new, regular series in the coming year is an exploration of various portfolios of PCORI-funded projects.

With the series we'll be taking stock, delving into the current set of funded projects in PCORI's portfolio and beginning to think about how we can continue to shape it for research that matters most to patients and stakeholders

Topic Portfolios Ready to Explore

Telehealth (later today!)

Opioids

Multiple Sclerosis

Mental Health

Cancer

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Dashboard Review:
Second Quarter of FY-2019

Joe Selby, MD, MPH
Executive Director

PCORI Board of Governors Dashboard

Second Quarter FY-2019 (As of 3/31/2019)

Inputs

Process

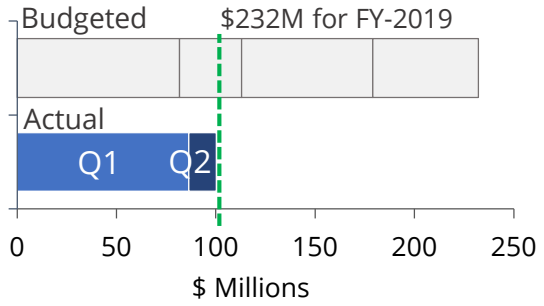
Outputs

Uptake

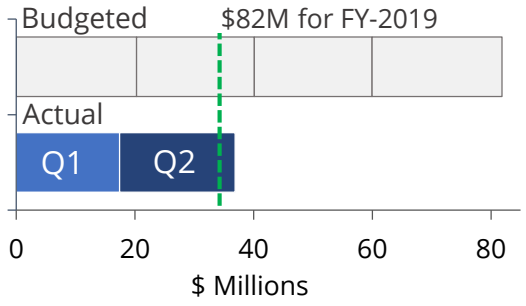
Use

Impact

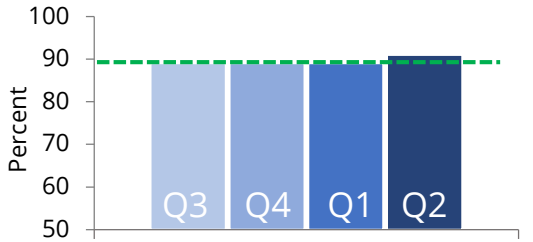
Funds Committed



Operational Expenses

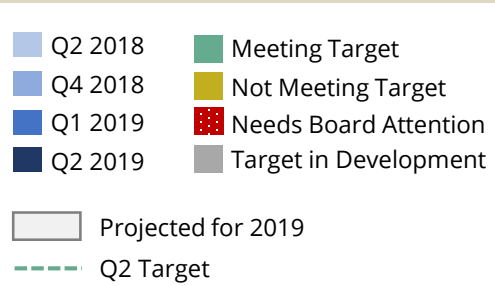


Research Project Performance

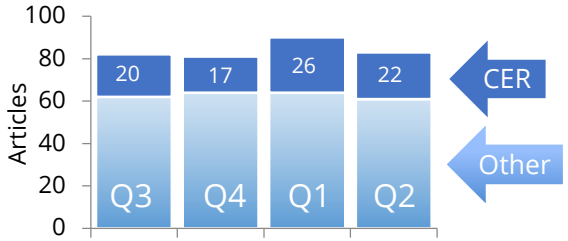


% of Research Projects On Track

Dashboard Key

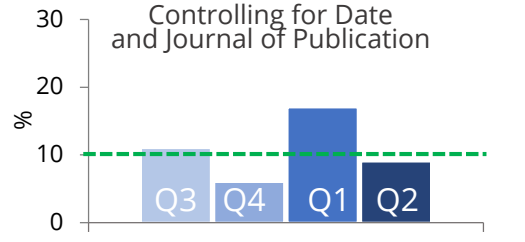


Results Published in Literature



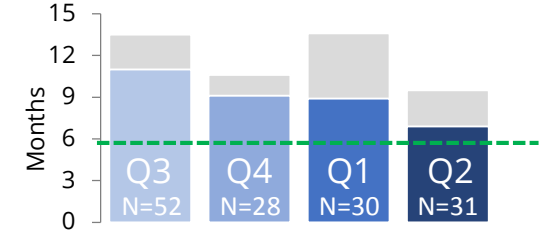
CER Results and Other Results Published in the Literature

Altmetric: Attention to PCORI Results



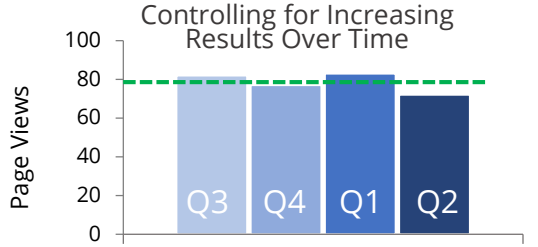
% of CER Results Publications in Top 10% of Attention

Speed of PCORI Peer Review



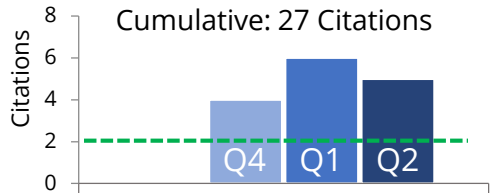
Median Time to Complete Peer Review (Grey = 75th Percentile)

Results Viewed on PCORI.org



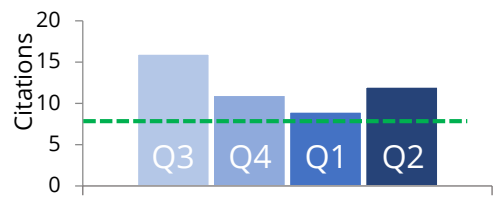
Average Pageviews of Results Posted to PCORI.org

Uptake into UpToDate®



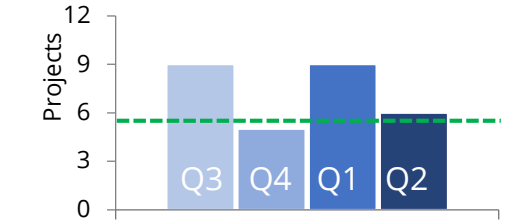
Citations in UpToDate® point-of-care decision tool

Other Examples of Uptake



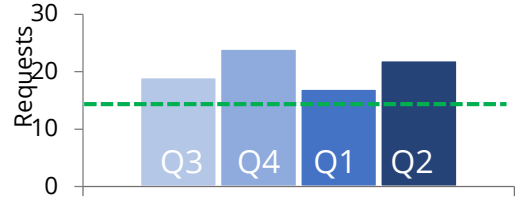
Citations in Systematic Reviews, Guidelines, & Policy Documents

New Studies Underway in PCORnet



New Studies Underway in PCORnet

Front Door Requests in PCORnet



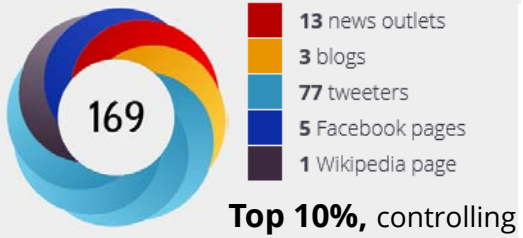
Front Door Requests Submitted to PCORnet

Goal 1: Increase Information for Health Decision-Making

For Women with Urinary Incontinence, Behavioral Therapies are More Effective than Drugs Alone

PCORI Study	**Study Title:** Nonsurgical Treatments for Urinary Incontinence in Women: A Systematic Review Update **Organization:** Memorandum of Understanding with Agency for Healthcare Research and Quality (AHRQ); Study conducted by Evidence-based Practice Center at Brown University
Results Publication	Balk EM, et al. **Pharmacologic and Nonpharmacologic Treatments for Urinary Incontinence in Women: A Systematic Review and Network Meta-analysis of Clinical Outcomes**. *Ann Intern Med.* Mar 2019.

Altmetric Score (Attention)



Top 10%, controlling for Journal and Date of Publication

Summary: Urinary incontinence (UI) can affect a woman's physical, psychological, and social well-being and can impose substantial lifestyle restrictions. Nonpharmacologic approaches to managing UI mostly aim to strengthen the pelvic floor, or change behaviors that influence bladder function. Pharmacologic treatments typically address bladder and urethral function

PCORI and AHRQ partnered to commission a systematic review update for **comparing the effectiveness and harms of pharmacologic and nonpharmacologic interventions to improve or cure stress, urgency, or mixed UI** in nonpregnant women

Reviewers included 84 randomized trials that evaluated 14 categories of interventions. Nonpharmacologic and pharmacologic interventions were more likely than no treatment to improve UI outcomes. **Behavioral therapy, alone or in combination with other interventions, is generally more effective than pharmacologic therapies alone** in treating both stress and urgency UI

> A reasonable approach is to **start with behavioral modifications.** If that's unsuccessful, then move on to medications or procedures. But this is a quality-of-life issue, not a life-threatening problem… **go through all the options and let the patient decide**.
>
> -Dr. Peter C. Jeppson, MD
> Senior Researcher, Urogynecologist
> University of New Mexico

Quote from: Nicholas Bakalar, "For Urinary Incontinence, Try Behavioral Treatments or Drugs, or Both," *The New York Times.* Mar 22, 2019. (link)

Goal 1: Increase Information for Health Decision-Making

Among Nonsurgical Treatments for UI, Behavioral Therapies Have Lowest Associated Risks

PCORI Study	**Study Title:** Nonsurgical Treatments for Urinary Incontinence in Women: A Systematic Review Update **Organization:** Memorandum of Understanding with Agency for Healthcare Research and Quality (AHRQ); Study conducted by Evidence-based Practice Center at Brown University
Results Publication	Balk EM, et al. **Adverse Events Associated with Nonsurgical Treatments for Urinary Incontinence in Women: a Systematic Review**. *J Gen Int Med*. May 2019.

Altmetric Score:

Too early to assess (published 5/7/19)

Summary: For women with Urinary Incontinence (UI), there are numerous nonsurgical treatments available, and each are associated with risk of adverse events (AEs). **This companion article reports on the harms associated with treatment options**

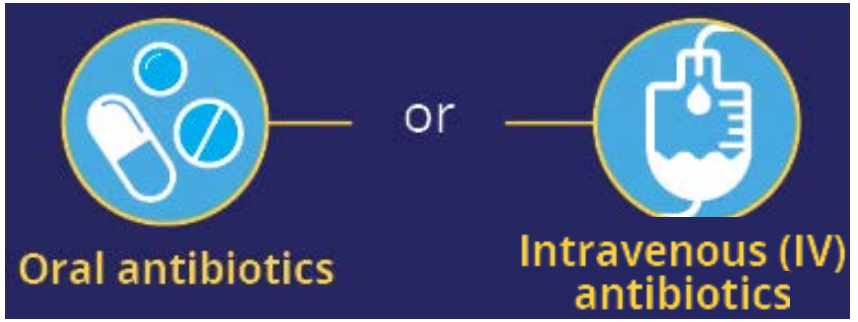
Nonpharmacologic Therapies: 52 studies reported on AEs in studies of nonpharmacological interventions. Behavioral therapies and neuromodulation have low risk of AEs

Pharmacologic Therapies: 102 studies reported on AEs in studies of pharmacological interventions. Anticholinergics and alpha agonists have high rates of dry mouth and constitutional effects such as fatigue and gastrointestinal complaints. BTX (botulinum toxin injection) is associated with urinary tract infections and voiding dysfunction. Periurethral bulking agents are associated with erosion and voiding dysfunction. These AE risks should be considered when selecting appropriate UI treatment options

> **The choice of which treatment option is best for a particular woman with UI will vary** depending on her symptoms, the severity of those symptoms, her history of prior treatments, treatment goals, preferences, and values regarding the types of treatments she is willing to undergo, and also the AE risks she is willing to assume.

Goal 2: Speed Uptake and Use of Information
Uptake of Results from PCORI-Funded Study into IDSA Guidelines

Results from a PCORI-funded study on antibiotic therapies for serious bacterial infections in children **were taken up into the 2018 Infectious Diseases Society of America (IDSA) Clinical Practice Guideline** for the Management of Outpatient Parenteral Antimicrobial Therapy (OPAT).



The study found that oral antibiotics are as effective as intravenous antibiotics, with fewer complications. Three CER results publications from the study were cited in the guideline update:

> "…There is mounting evidence that oral therapy can be substituted for OPAT without compromising cure rates. Safety is enhanced by avoiding OPAT-related complications for certain clinical conditions where OPAT has traditionally been the preferred treatment mode. **The evidence supporting this practice includes studies comparing oral vs parenteral therapy for osteomyelitis, perforated appendicitis, and complicated pneumonia**. As a result, ensuring that OPAT is only prescribed for patients where an equivalent oral therapy is not available is a high priority for pediatric ID specialists and pediatric antimicrobial stewardship programs."

Full Citation:
2018 Infectious Diseases Society of America Clinical Practice Guideline for the Management of Outpatient Parenteral Antimicrobial Therapy. *Clinical Infectious Diseases*, Volume 68, Issue 1, 1 January 2019, Pages e1–e35, https://doi.org/10.1093/cid/ciy745

Study Title: Comparative Effectiveness of Intravenous vs. Oral Antibiotic Therapy for Serious Bacterial Infections

Principal Investigator: Ron Keren, MD, MPH, The Children's Hospital of Philadelphia

Goal 3: Influence the Way Research is Done

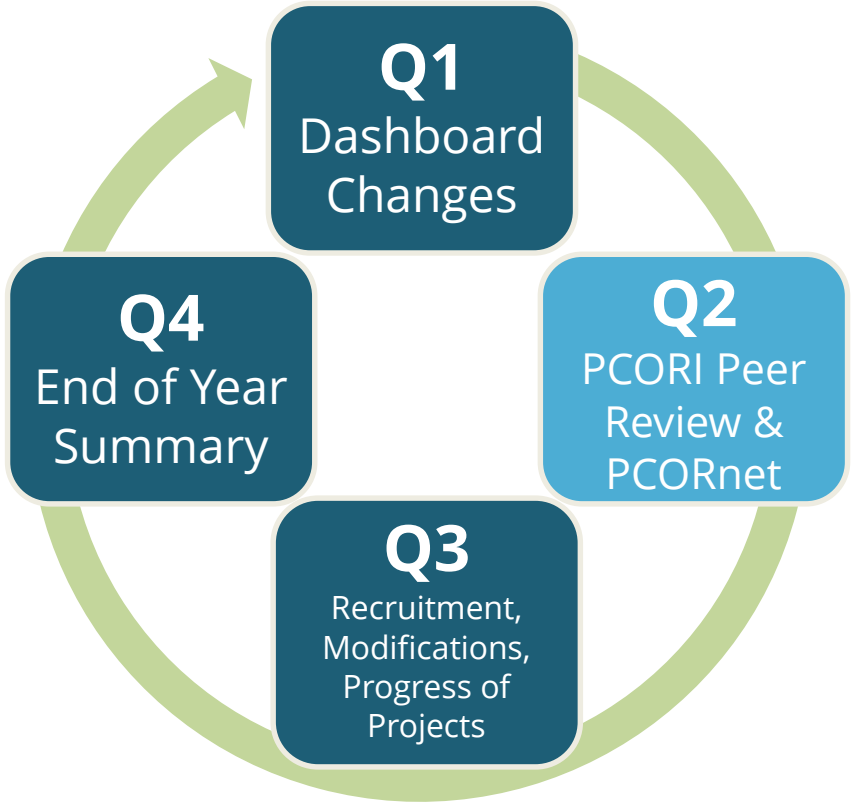
Publications on How Research is Done at PCORI

- Performed **self-assessment of practice and policies against Lancet recommendations** to benchmark our current practice and identify areas for improvement

Whitlock EP, Selby JV, Dunham KM, Fernandez A, Forsythe LP, Norquist G. Examining the role of funders in ensuring value and reducing waste in research: An organizational case-study of the Patient-Centered Outcomes Research Institute [version 1; peer review: awaiting peer review]. F1000Research 2019, 8:288

- Review of trial transparency policy among US noncommercial funders
  - 6 of 9 (**67%**) **of top US funders, including PCORI, have a publicly available written policy addressing clinical trial registration, summary results sharing, and individual patient data sharing**
  - Fewer US funders require specific actions (11%-56%) or monitor compliance (56%-67%)

Whitlock EP, Dunham KM, DiGioia K, Lazowick E, Gleason TC, Atkins D. Noncommercial US Funders' Policies on Trial Registration, Access to Summary Results, and Individual Patient Data Availability. *JAMA Network Open.*2019;2(1):e187498.



Q2 Focus on PCORI Peer Review

Peer Review's Stages of Review and Revision

Multiple checks ensure that the final report is clear, complete, and scientifically sound

Pre-Peer Review

- Pre-Review editor reviews for clarity & DFRR structure
- Program Officer reviews for completeness
- The PI may need to revise before peer review can start

External Peer Review

- External peer reviewers provide comments
- Associate editor sends a synthesis letter of peer review comments.
- PI responds to comments within 2 months
- Associate editor may request additional changes until they determine that the DFRR has met Peer Review expectations

PCORI's Final Review

- PCORI Director of Peer Review and Scientific Publications provides final review for clarity
- This step may repeat until the Director of Peer Review approves the report on behalf of PCORI

PCORI Peer Review Process and Public Release of Findings

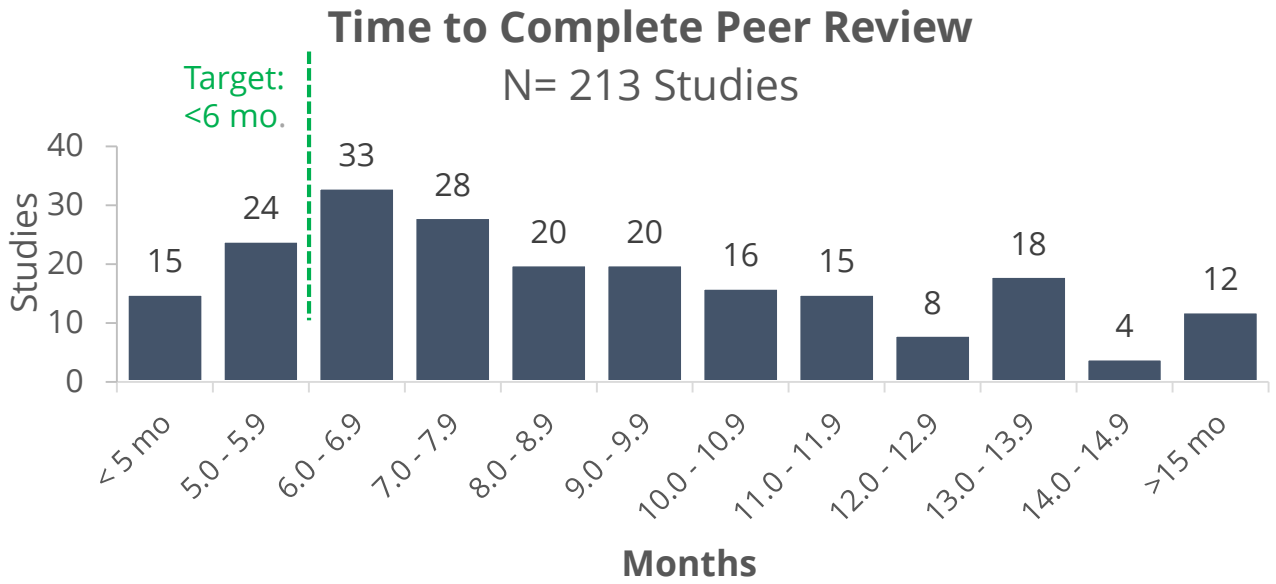
Studies in PCORI Peer Review Process or Completed PCORI Peer Review

N=285, as of Q2-19

*Does not include Pilots, Systematic Reviews, Infrastructure, or D&I awards
(50 Pilots and 4 Systematic Reviews are complete, with results posted to PCORI.org)

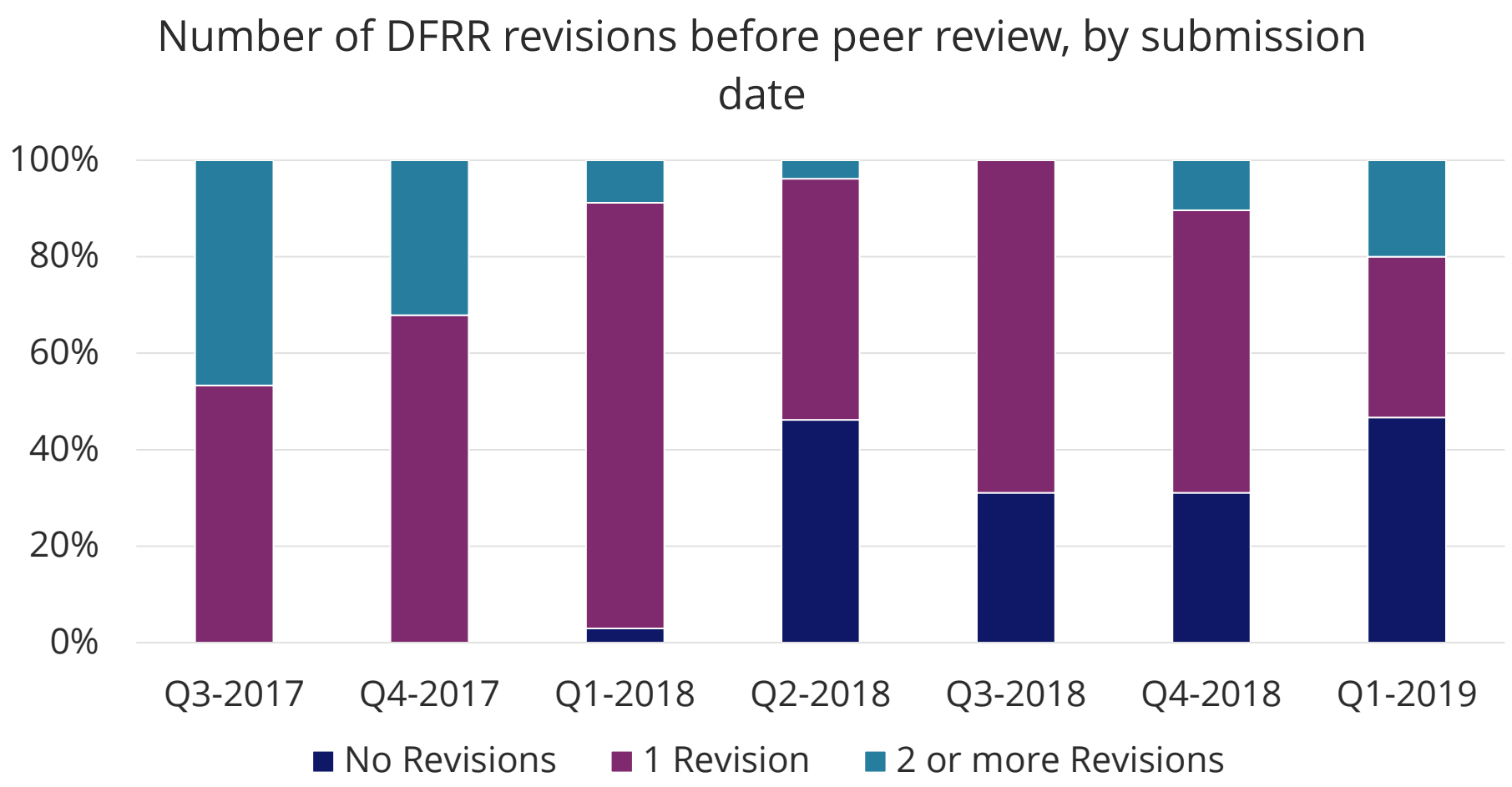
Speed of PCORI Peer Review Q2-19 Update

- **Target:** 6 months from acceptance of the Draft Final Research Report (DFRR) to acceptance of Final Research Report (FRR).
- **Time for PCORI Peer Review to-date:**
  - 213 completed as of Q2-19
    - Median time in Peer Review: 8.3 months (average 9.0 m)
    - Fastest to-date: 3.4 months; Slowest to-date: 19.8 months



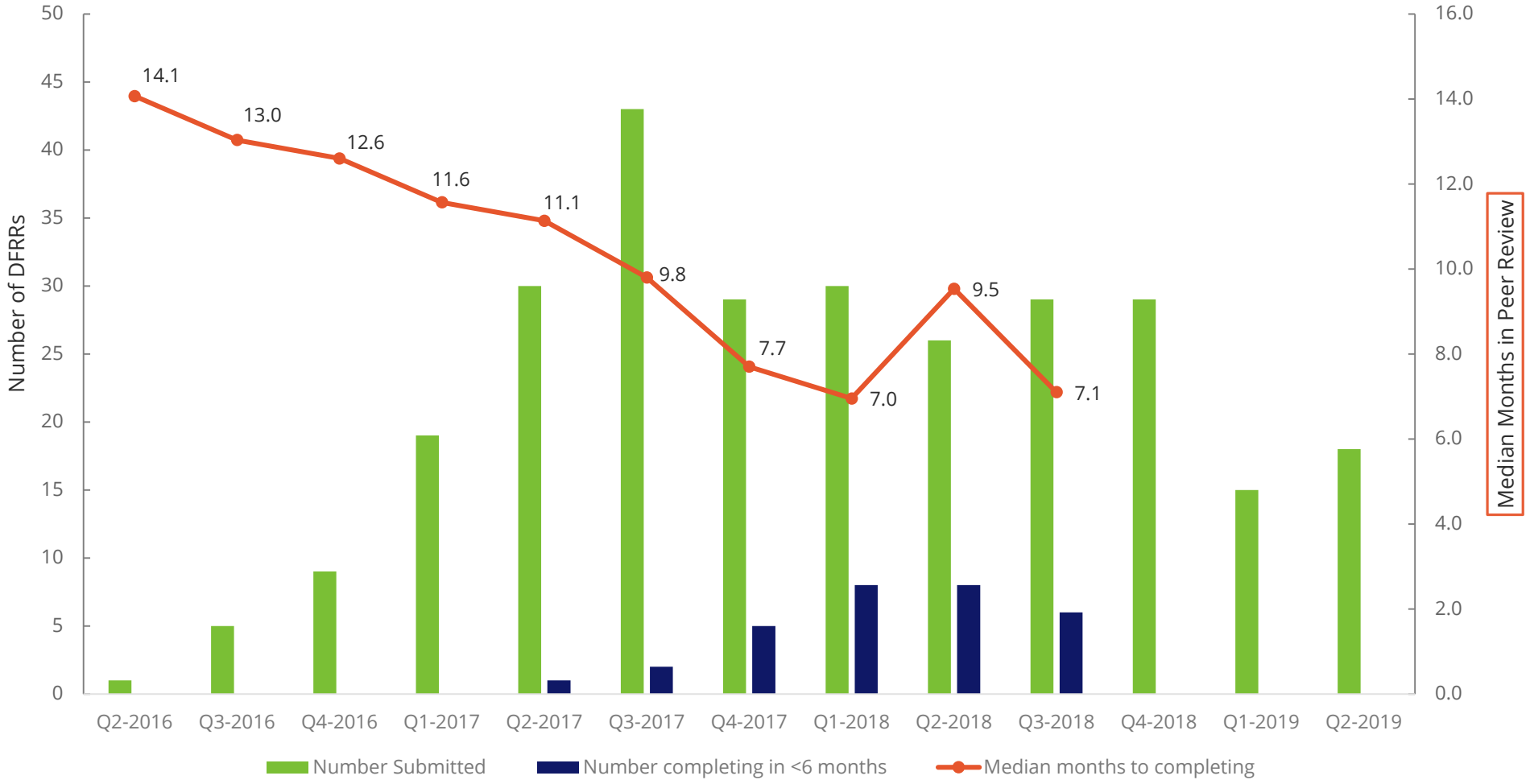
Pre-Peer Review: Clarity and Completeness

The pre-peer review phase was initiated because many DFRRs were coming in not following DFRR instructions, or poorly developed. This has changed with increased focus on the instructions and reminders from program staff. More reports are coming in ready for peer review – no need for revisions before going external reviewers (dark blue).



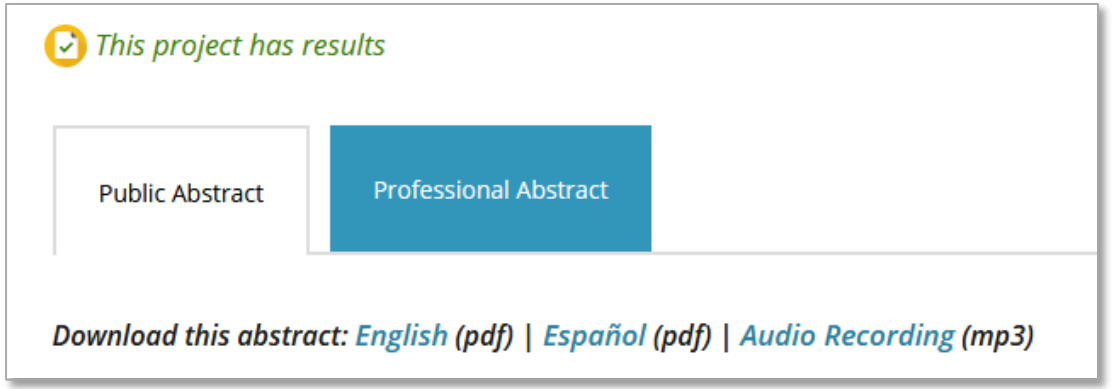
Timeliness of Peer Review: Cohort View
Trends Over Time

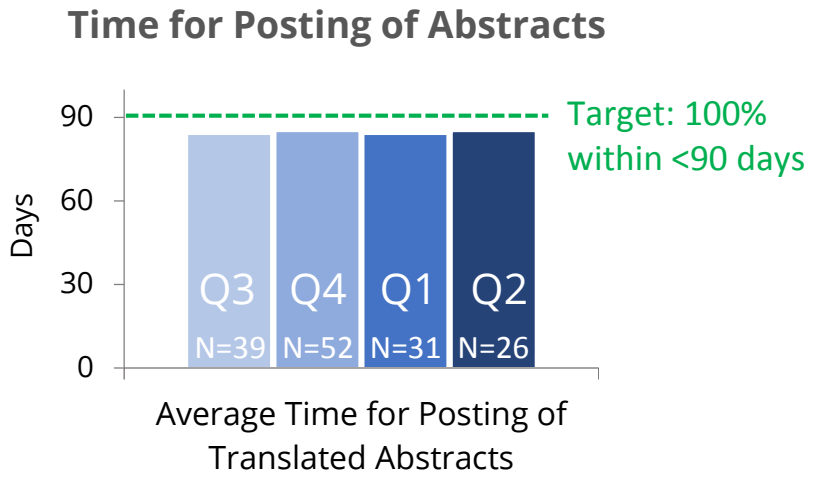
In 2 years, we have cut the median amount of time reports spend in peer review by half: 7 months from start to finish.



Speed of Posting Results to PCORI.org

- PCORI's authorizing law requires that we make research findings available no longer than 90 days after PCORI Peer Review is complete
- This includes the time it takes to translate findings into lay and scientific text
- **All abstracts to-date have been posted within 90 days of completion of PCORI Peer Review**
- Time for Posting of Abstracts:
  - 182 completed as of Q2-19
    - Median time to post: 86 days (average 84 days)



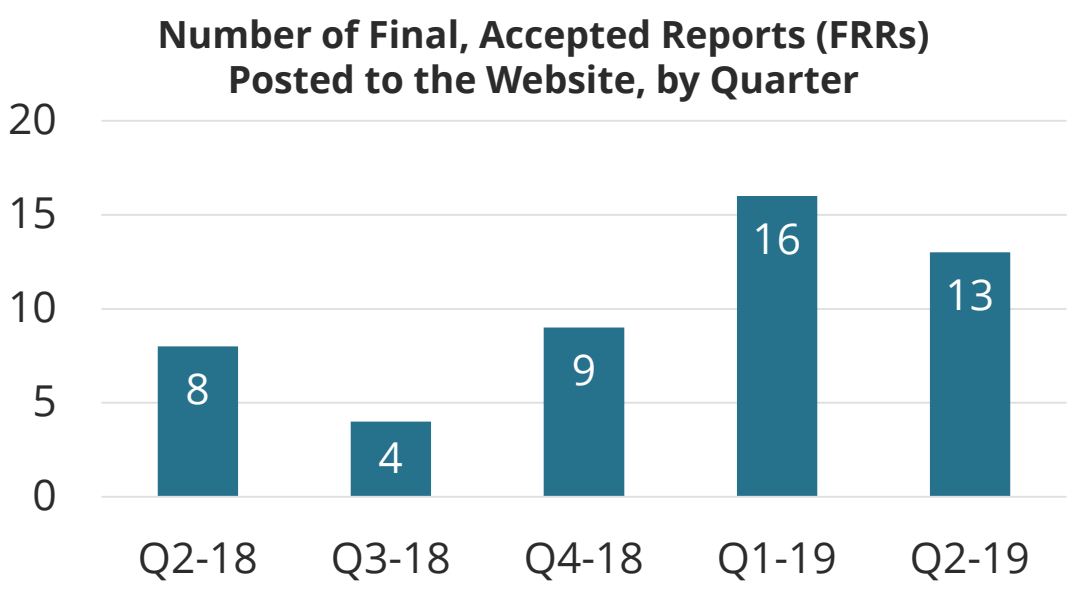


Comprehensive Final Research Reports Are Being Posted to the Website

The Final Research Report (FRR) is a comprehensive, full final report of the funded study, usually equivalent to the length and content of 3-5 journal articles.

- Up to 152 final reports may be posted by the end of FY-2019
- Average time from acceptance to posting: about 10 months
- The new FRR search function & presentation makes the reports easier to find

Coming soon:
Measures of attention to FRRs



Promoting Our Model of Peer Review in JAMA and Accompanying Blog Post

This Issue Views 4,198 | Citations 0 | Altmetric 23

Viewpoint

FREE

March 28, 2019

A Model for Public Access to Trustworthy and Comprehensive Reporting of Research

Marina Broitman, PhD[1]; Harold C. Sox, MD[1]; Jean Slutsky, PA, MSPH[1]

Author Affiliations | Article Information

JAMA. 2019;321(15):1453-1454. doi:10.1001/jama.2019.2807

PCORI's Commitment to Publicly Accessible Study Results Captured in JAMA Viewpoint

Date: March 28, 2019

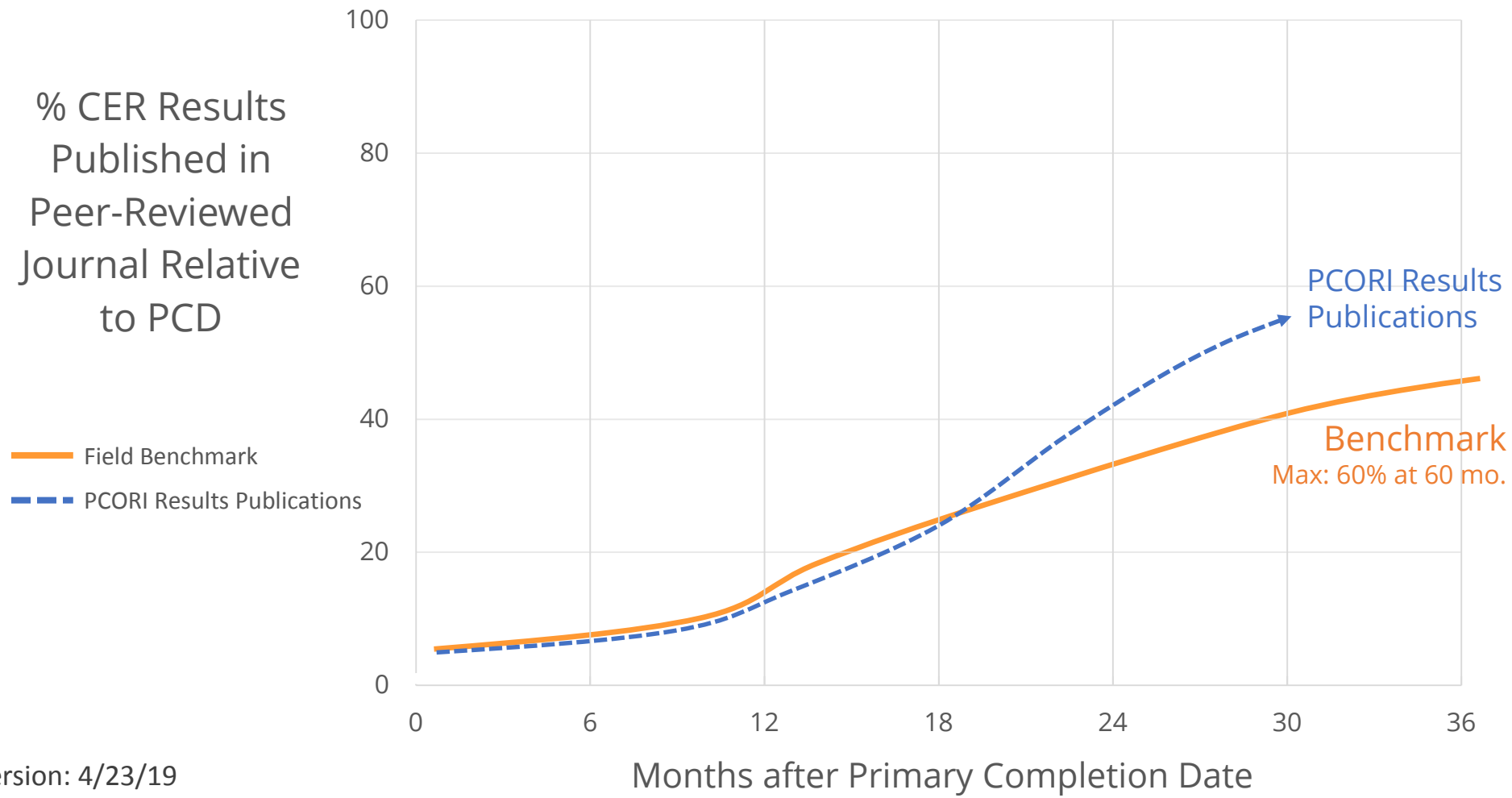
Blog Topics: Evaluating Our Work, Research

At PCORI, we're committed not only to funding research that can help patients and those who care for them make better-informed healthcare decisions, but to doing all we can to see that the results of those studies are made widely available. As we outline in a newly posted *JAMA Viewpoint*, PCORI's approach to this is unique not just among research funders but across the healthcare community more broadly.

Broitman M, Sox HC, Slutsky J. **A Model for Public Access to Trustworthy and comprehensive Reporting of Research**. *JAMA*. 2019;321(15):1453-1454. doi:10.1001/jama.2019.2807

Link to blog post

PCORI-Funded Trials Publish Their Results as Quickly or Faster Than Studies Typically Do

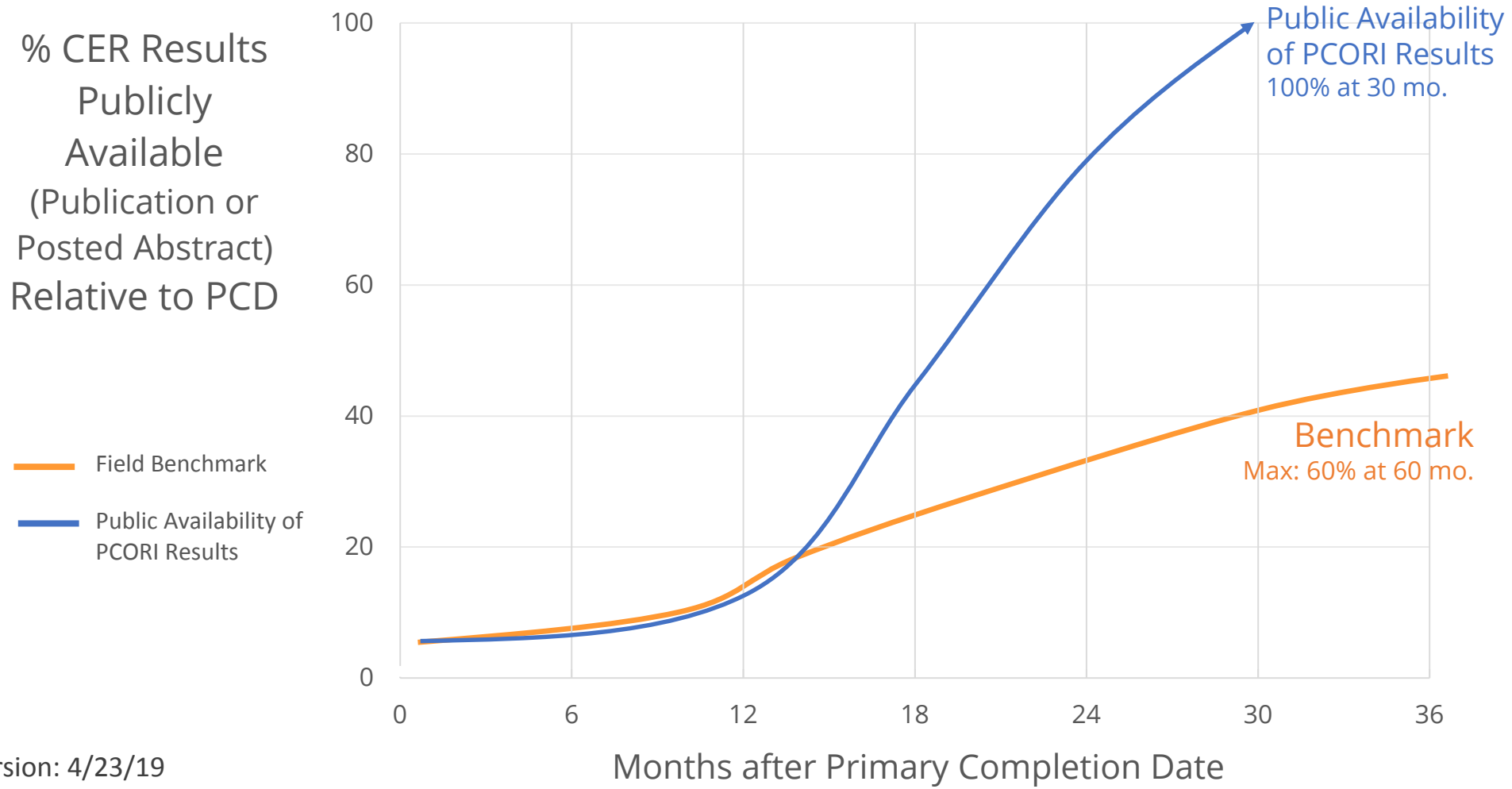


Benchmarks Based on:

- C Riveros, A Dechartres, et al. Timing and Completeness of Trial Results Posted at ClinicalTrials.gov and Published in Journals. Plos One, Dec 2013.
- R Chen, N Desai, H Krumholz, et al. Publication and Reporting of Clinical Trial Results: Cross Sectional Analysis Across Academic Medical Centers. BMJ, Jan 2016.

Version: 4/23/19
Cohort: PCORI's first 4 cycles (135 trials)

PCORI's Process Results in Complete and Earlier Availability of Results in the Public Domain



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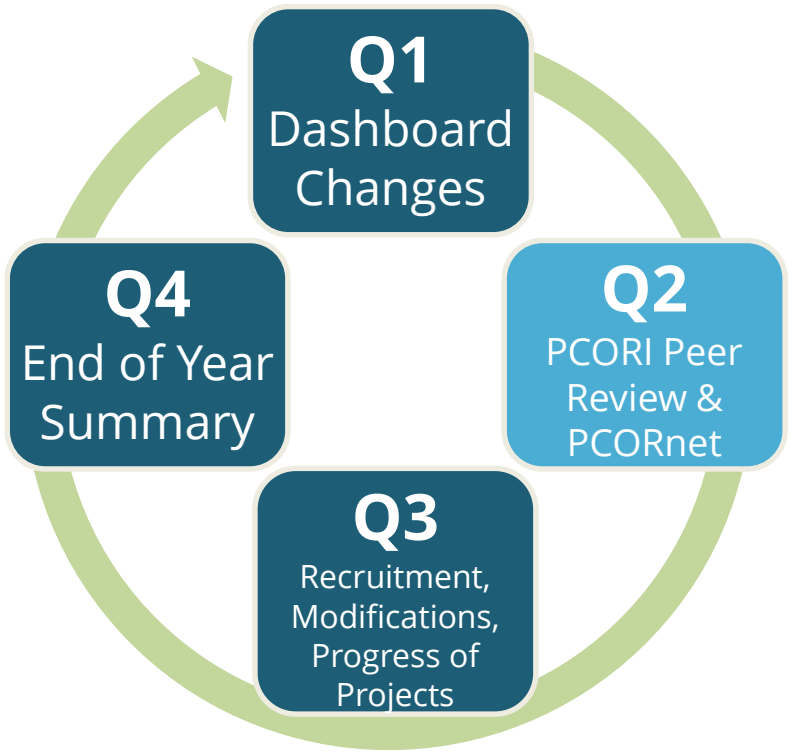


Q2 Focus on PCORnet

1) Data improvements

2) New projects and prospects

PCORnet 2.0
What Data are Available from CRNs (Most recent assessment)

- In 2019, PCORnet 2.0 **Clinical Research Networks (CRNs)** accessed 30.8 million patient records who had a least one diagnosis record.
- Response received from all 9 PCORnet 2.0 Clinical Research Networks (43 sites)

	Patients with at least one diagnosis record in the period 7/1/2017 - 6/30/2018	
	N	%
Number of Unique Patients	30,851,103	
By Age (N, % of patients)		
0-20	9,703,036	32%
21-44	8,010,121	26%
45-64	7,724,101	25%
65-74	3,391,699	11%
75+	2,433,714	8%
By Sex (N, % of patients)		
Male	13,335,325	43%
Female	17,512,824	57%
Other	2,884	0%
By Race (N, % of patients)		
Black or African American	5,119,514	16%
White	19,350,858	63%
Other	6,380,731	21%
By Hispanic (N, % of patients)		
Yes	4,338,870	14%
No	22,901,067	74%
Other	3,611,165	12%

PCORnet 2.0
What Data are Available from Health Plans?

- In 2019, PCORnet's two **Health Plan Research Networks (HPRNs)** accessed data on 24.6 million patients with a least one diagnosis record

	Patients with at least one diagnosis record in the period 7/1/2017 - 6/30/2018	
	N	%
Number of Unique Patients	24,574,856	
By Age (N, % of patients)		
0-20	5,316,011	22%
21-44	6,960,375	28%
45-64	7,547,717	31%
65-74	3,099,768	13%
75+	2,216,196	9%
By Sex (N, % of patients)		
Male	11,262,575	46%
Female	13,312,277	54%
Other	0	0%
By Race (N, % of patients)		
Black or African American	524,904	2%
White	2,634,055	11%
Other	21,415,897	87%
By Hispanic (N, % of patients)		
Yes	67,523	0%
No	3,262,523	13%
Other	21,244,810	86%

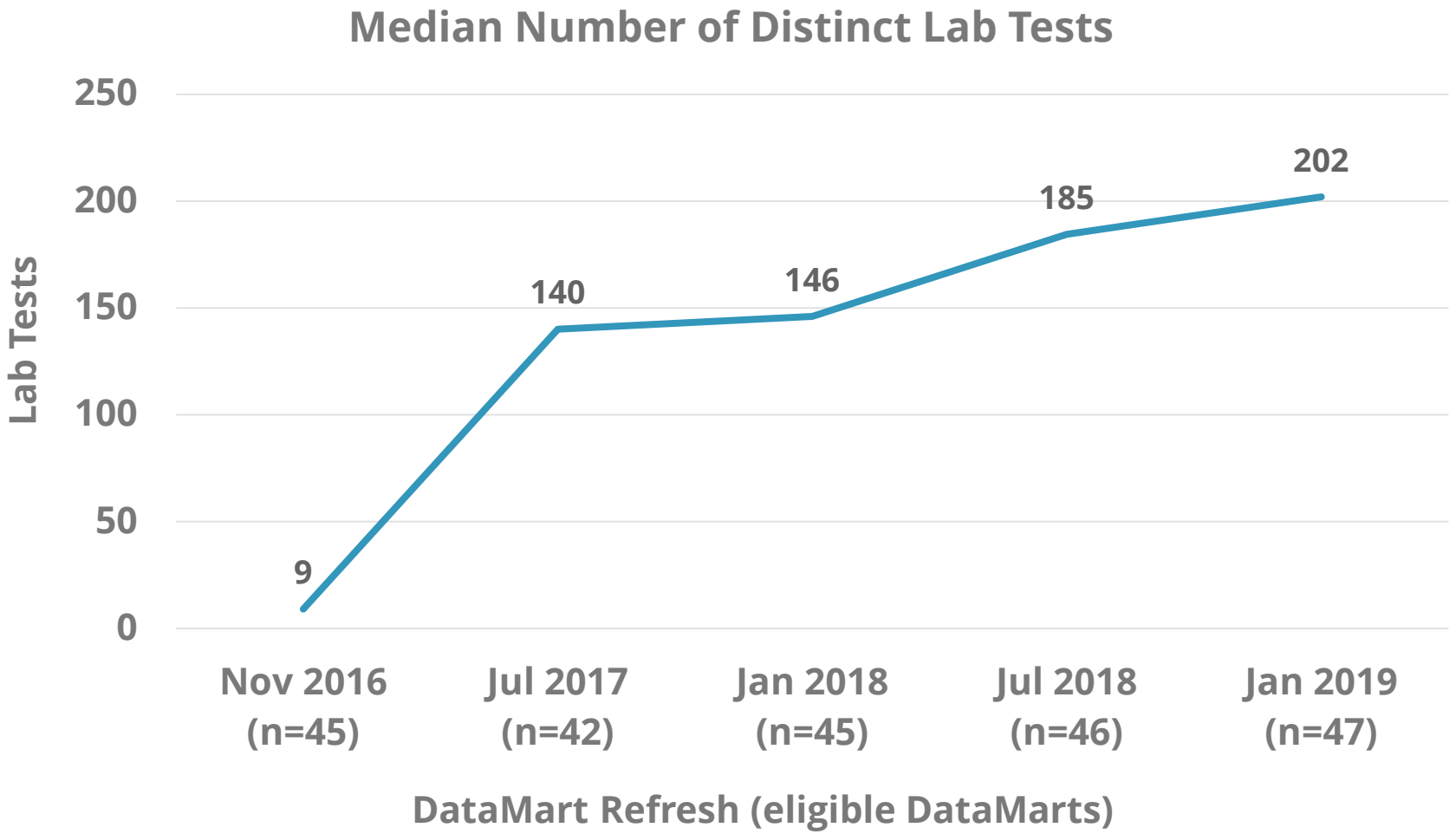
PCORnet 2.0
Data Available on Selected Conditions (CRNs)

	Anxiety		Alzheimer's		Breast Cancer		Asthma		Heart Failure		Heart Failure and Diabetes	
	N	%	N	%	N	%	N	%	N	%	N	%
Number of Unique Patients	2,625,750		99,409		1,736,474		290,267		689,730		295,007	
By Age (N, % of patients)												
0-20	473,676	18%	327	0%	749,134	43%	1,966	1%	10,500	2%	1,116	0%
21-44	881,623	33%	278	0%	397,115	23%	24,637	9%	42,998	6%	12,903	4%
45-64	798,653	30%	5,524	6%	370,224	21%	129,432	45%	203,432	30%	95,652	32%
65-74	287,998	11%	16,479	17%	142,416	8%	83,391	29%	172,685	25%	85,318	29%
75+	205,417	8%	77,542	78%	87,500	5%	56,473	20%	269,020	39%	104,737	36%
By Sex (N, % of patients)												
Male	885,267	34%	33,647	34%	718,827	41%	7,480	3%	349,582	51%	150,571	51%
Female	1,740,325	66%	65,751	66%	1,017,583	59%	282,766	97%	340,098	49%	144,421	49%
Other	95	0%	0	0%	22	0%	0	0%	13	0%	0	0%
By Race (N, % of patients)												
Black or African American	308,945	12%	12,225	12%	442,770	26%	37,201	13%	141,638	21%	70,375	24%
White	1,949,909	74%	70,475	71%	940,478	54%	218,144	75%	465,693	68%	185,632	63%
Other	366,896	14%	16,668	17%	353,226	20%	34,907	12%	82,388	12%	38,985	13%
By Hispanic (N, % of patients)												
Yes	276,898	11%	10,835	11%	291,435	17%	18,833	7%	47,096	7%	25,223	9%
No	2,116,159	81%	79,311	80%	1,291,711	74%	248,756	86%	582,003	84%	243,903	83%
Other	232,680	9%	9,200	9%	153,303	9%	22,634	8%	60,595	9%	25,848	9%

PCORnet 2.0
Data Available on Selected Conditions (HPRNs)

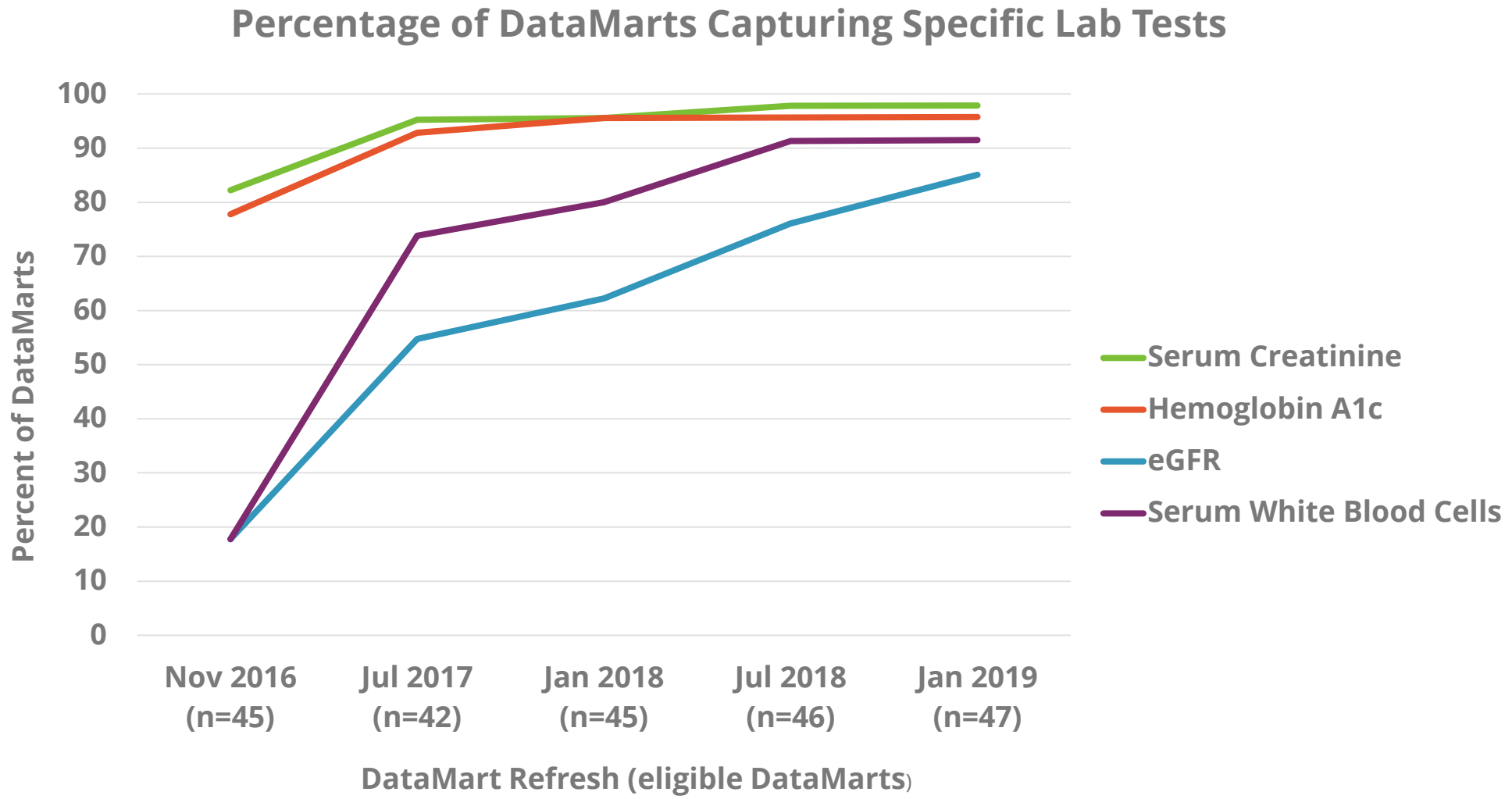
	Anxiety		Alzheimer's		Breast Cancer		Asthma		Heart Failure		Heart Failure and Diabetes	
	N	%	N	%	N	%	N	%	N	%	N	%
Number of Unique Patients	3,535,088		133,638		1,499,805		264,107		751,358		360,987	
By Age (N, % of patients)												
0-20	419,312	12%	18	0%	345,787	23%	66	0%	1,716	0%	45	0%
21-44	1,164,821	33%	171	0%	353,813	24%	14,173	5%	17,505	2%	5,246	2%
45-64	1,193,722	34%	4,361	3%	466,443	31%	112,224	43%	161,152	21%	82,142	23%
65-74	457,502	13%	20,543	15%	209,742	14%	78,331	30%	219,599	29%	121,878	34%
75+	341,864	10%	109,860	82%	137,285	9%	65,238	25%	363,874	48%	159,078	44%
By Sex (N, % of patients)												
Male	1,198,272	34%	47,813	36%	585,924	39%	2,237	1%	382,732	51%	191,875	53%
Female	2,336,815	66%	85,825	64%	913,881	61%	261,870	99%	368,626	49%	169,112	47%
Other	0	0%	0	0%	0	0%	0	0%	0	0%	0	0%
By Race (N, % of patients)												
Black or African American	72,277	2%	11,337	9%	48,411	3%	13,264	5%	83,737	11%	51,811	14%
White	561,104	16%	59,901	45%	184,030	12%	58,773	22%	313,458	42%	158,073	44%
Other	2,901,707	82%	62,400	47%	1,267,364	85%	192,070	73%	354,163	47%	151,103	42%
By Hispanic (N, % of patients)												
Yes	12,741	0%	1,786	1%	5,511	0%	1,030	0%	7,602	1%	4,720	1%
No	644,717	18%	72,867	55%	239,799	16%	74,000	28%	406,355	54%	215,673	60%
Other	2,877,630	81%	58,985	44%	1,254,495	84%	189,077	72%	337,401	45%	140,594	39%

PCORnet 2.0
Density of Lab Data



Eligible DataMarts: PCORnet 2.0 DataMarts which populate the LAB_RESULT_CM table

PCORnet 2.0
Completeness of Selected Lab Tests



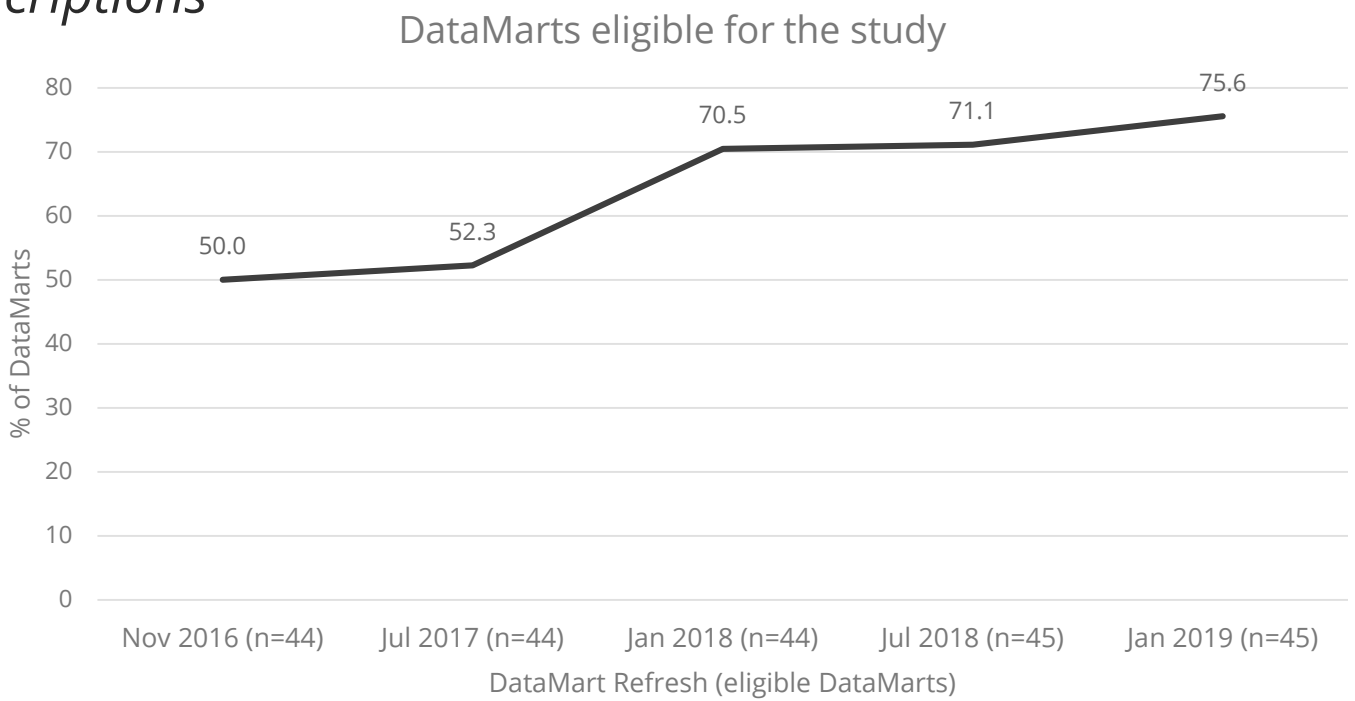
Eligible DataMarts: PCORnet 2.0 DataMarts which populate the LAB_RESULT_CM table

PCORnet 2.0

Availability of detailed prescribing data (medication orders)

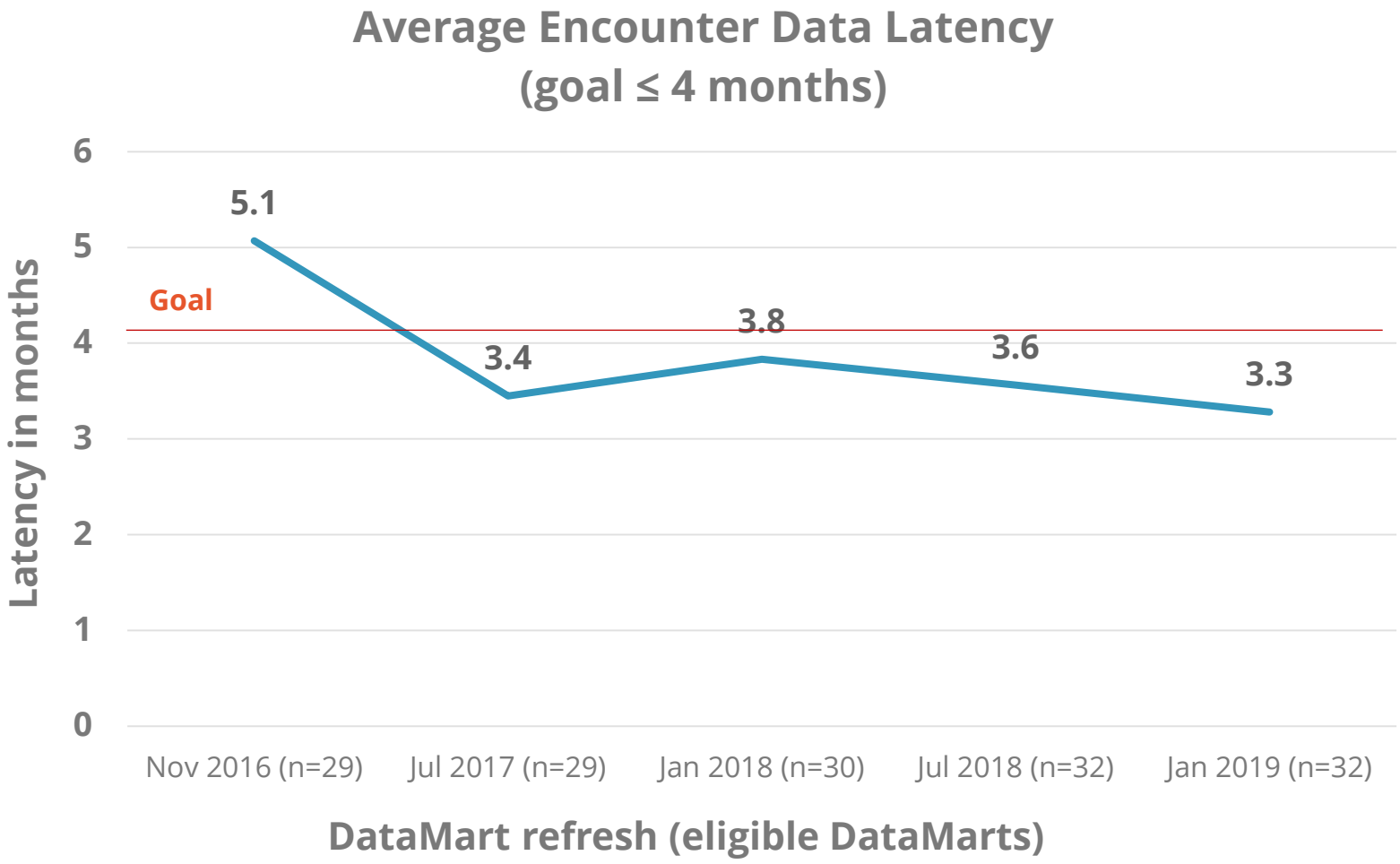
Use Case: A study to evaluate whether an approved **biosimilar** is interchangeable with its reference product.

DataMarts must capture the specific drug names that can distinguish a biosimilar from its reference product. Here we show the % of datamarts that capture specific product names for at least 80% of prescriptions



Eligible DataMarts: PCORnet 2.0 DataMarts which include inpatient, ambulatory, and/or Emergency Department encounters and do not use date obfuscation

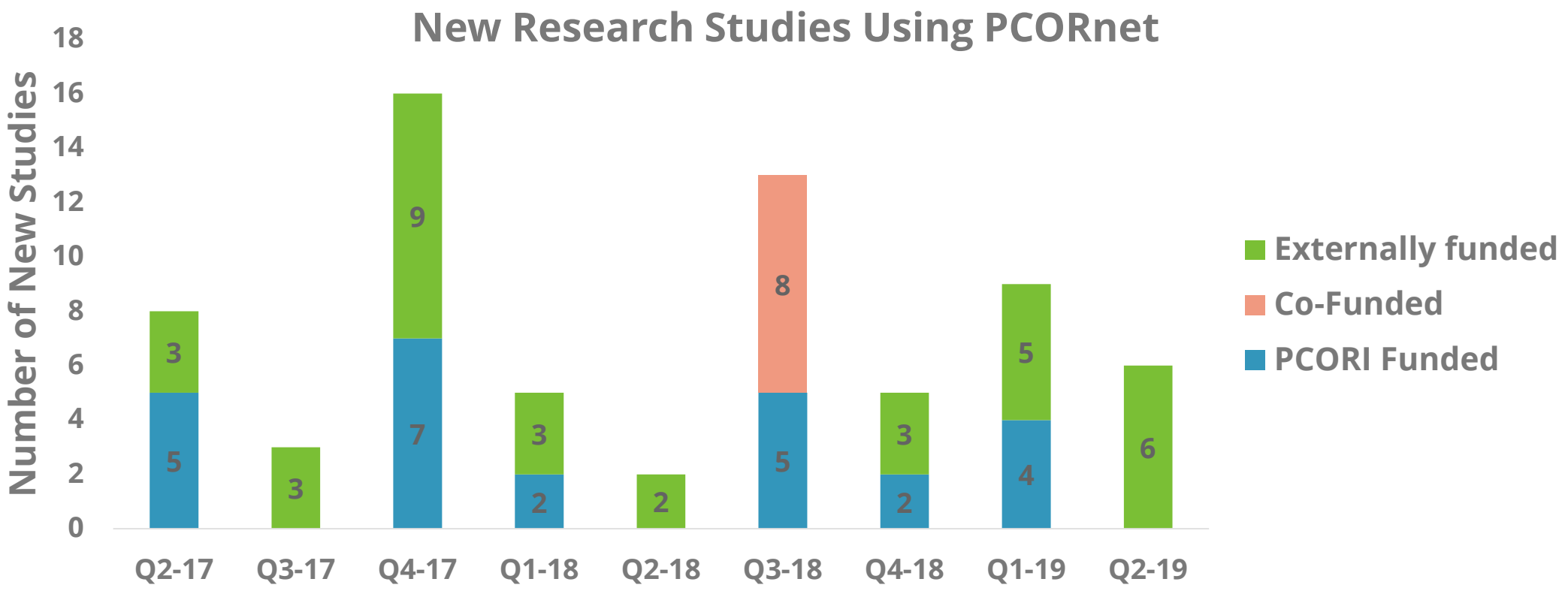
PCORnet 2.0
Availability of Recent Data



Eligible DataMarts: PCORnet 2.0 DataMarts which include inpatient, ambulatory, and/or Emergency Department encounters and do not use date obfuscation

PCORnet
Research Studies Using PCORnet

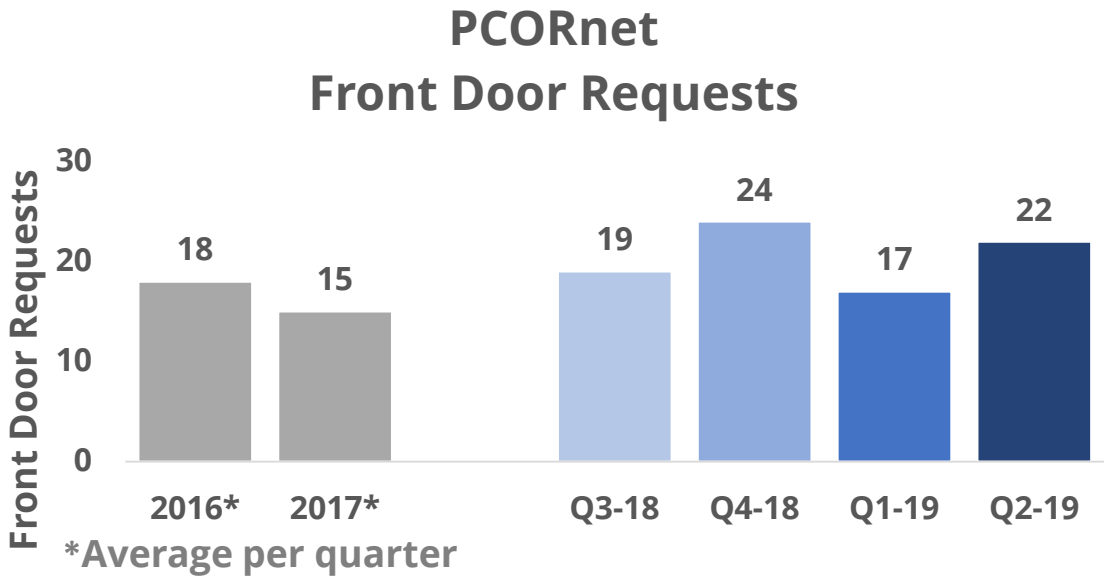
Many of the research studies using PCORnet are externally funded or co-funded. For Q2-19, **there are 6 new externally-funded studies in PCORnet**



PCORnet
Front Door Activity

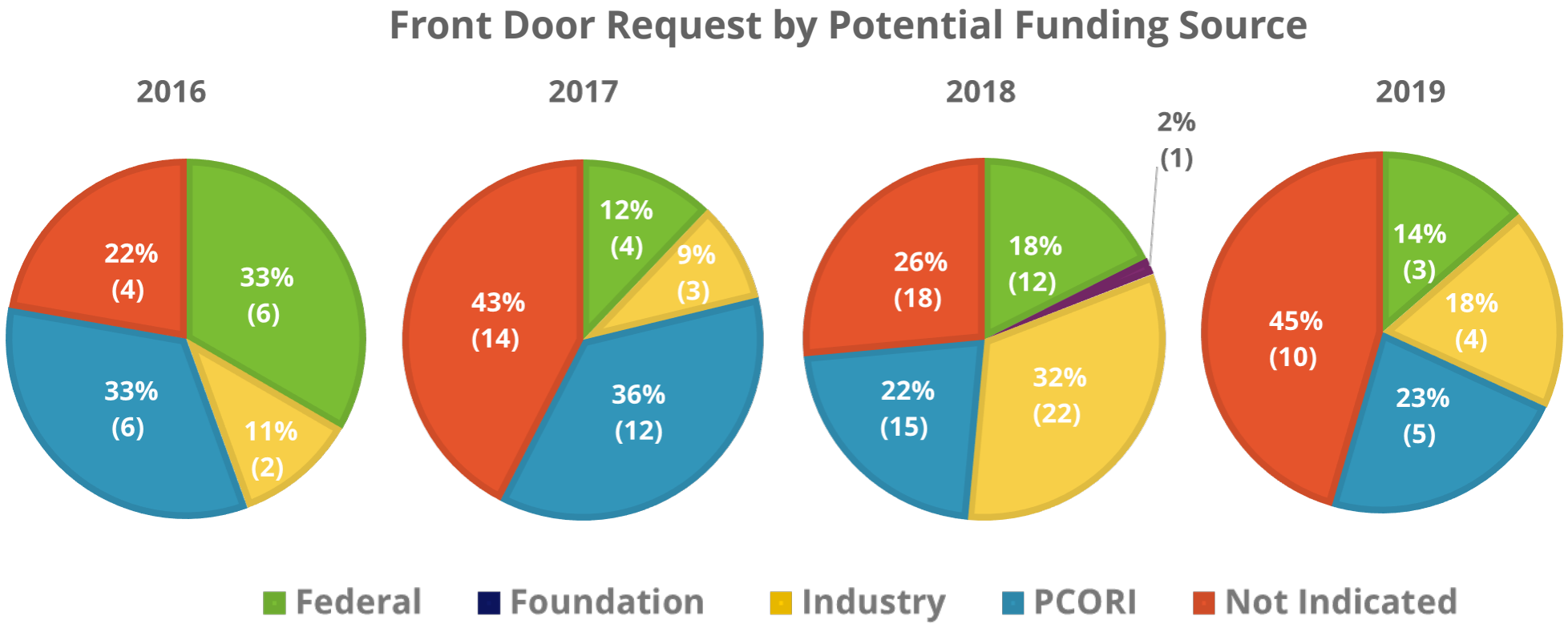
- **The Front Door is the access point** for potential investigators, patient groups, health systems, and sponsors to reach PCORnet resources and infrastructure. It is managed by the PCORnet Coordinating Center

- **Front Door Requests in PCORnet are being tracked**, including data network requests, network collaborator requests, study designation requests, and consultations



PCORnet
Inquiries from Potential Funders of Research

- Increasing diversity of funding sources pursued
- PCORI Partnerships to Conduct Research within PCORnet (PaCR) opportunity is increasing other funder's awareness of opportunities for co-funding



PCORnet
Research in Progress - INVESTED

INVESTED (INfluenza Vaccine to Effectively Stop CardioThoracic Events and Decompensated heart failure) INVESTED

- Randomized trial to compare high dose trivalent influenza vaccine versus standard dose quadrivalent vaccine in high-risk cardiovascular patients
- PCORnet sites leverage **electronic identification methods** and **cross-network collaborations** to enroll
- In Year 02, PCORnet had fewest sites (n=21), but **delivered most patients per site** (on average). And in Year 03, PCORnet sites tied for the **highest enrollment rate!**

Network	Number of Sites	YEAR 3 Enrollment	Daily Median Enrollment Rate Per Site
Canada	54	822	.09
Non-VA US	60	691	.07
PCORnet	25	306	.09
VA	33	445	.08
ALL	172	2264	.07

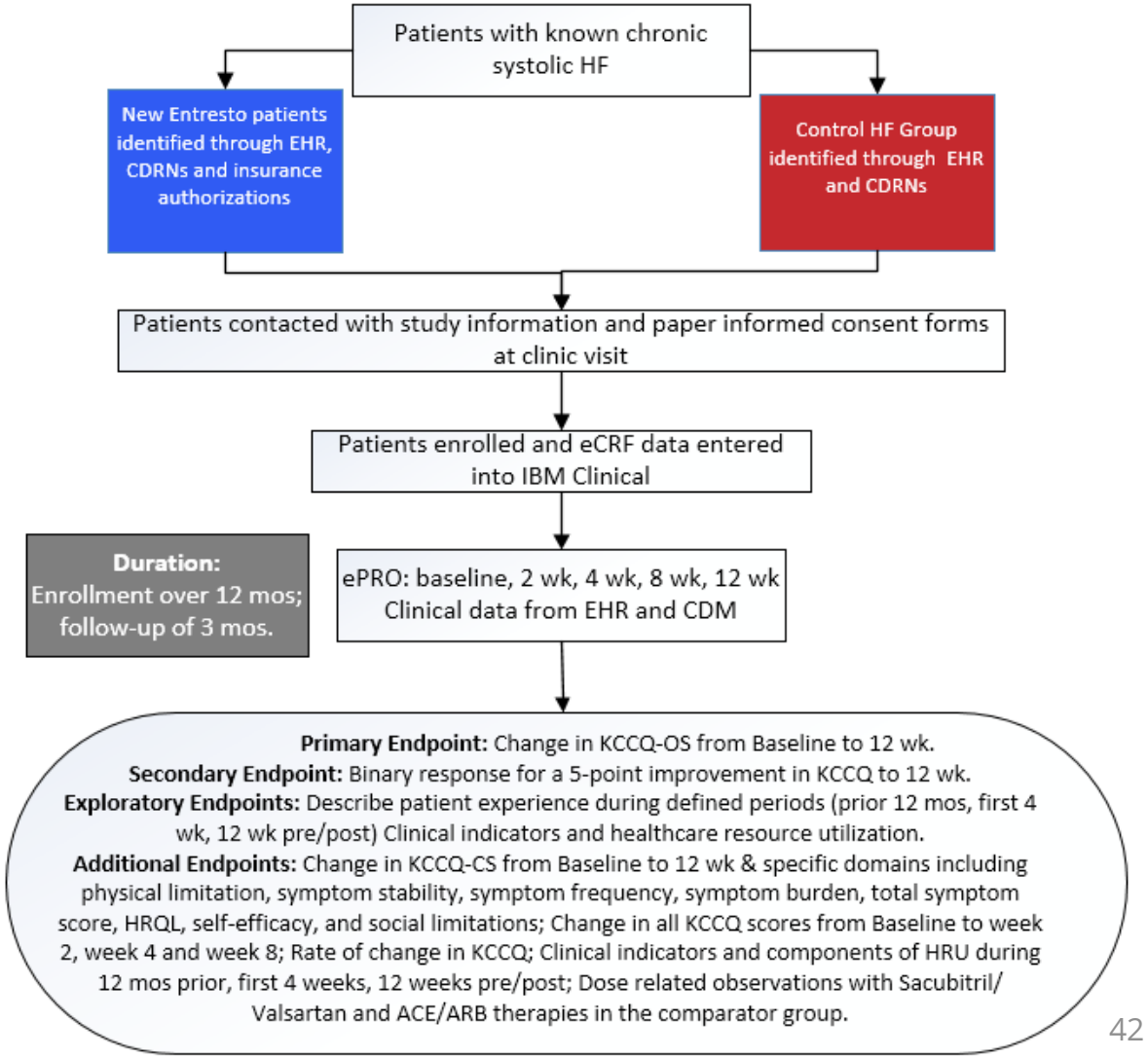
The INVESTED trial is funded by the National Heart, Lung and Blood Institute, ClinicalTrials.gov NCT02787044.

PCORnet
Research in Progress PROVIDE-HF

PROVIDE-HF (Patient Reported Outcomes inVestigation following Initiation of Drug therapy with Entresto (Sacubitril/Valsartan) in Heart Failure)

PROVIDE-HF

- A non-interventional prospective cohort study of 400 chronic HF patients.
- **Patient reported primary outcome** to determine impact on physical limitations, symptoms, self-efficacy, social interference, and quality of life.
- PCORnet enhanced recruitment techniques enabled this study to **complete enrollment ahead of schedule!** The final study query to capture outcomes scheduled for late summer 2019!

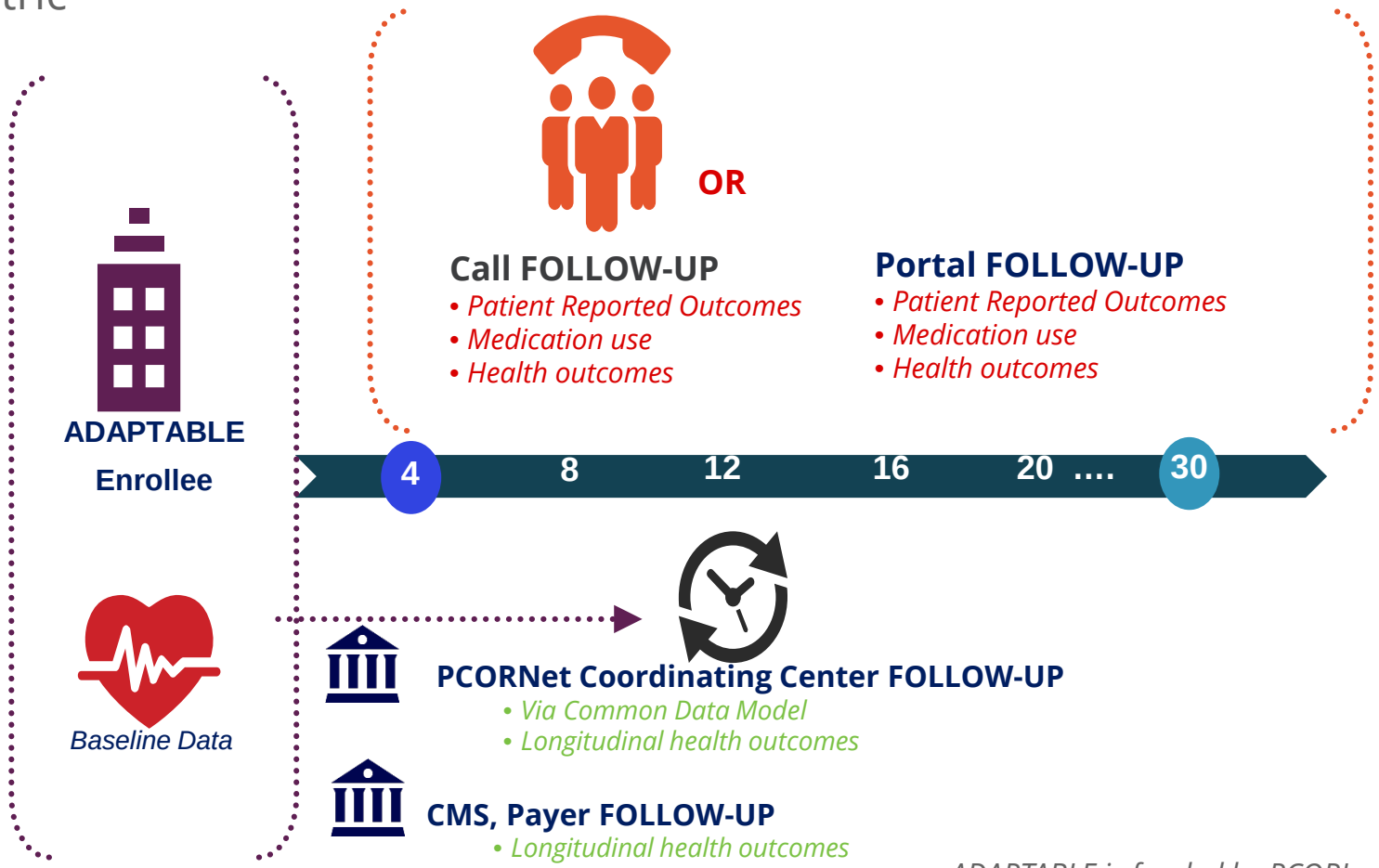


PROVIDE-HF is funded by Novartis

PCORnet
Research in Progress - ADAPTABLE

ADAPTABLE (Aspirin Dosing: A Patient-Centric Trial Assessing Benefits and Long-term Effectiveness)

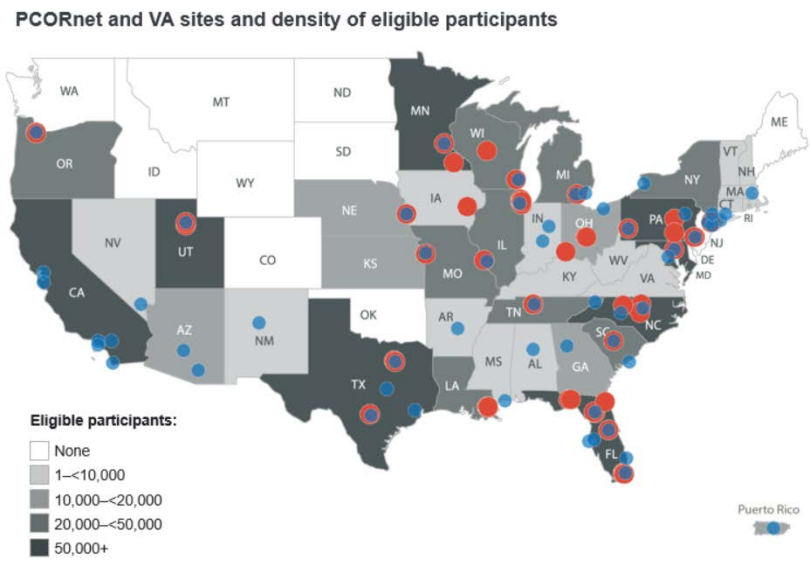
- PCORnet demonstration project to compare daily dose of 81mg or 325mg aspirin.
- PCORnet sites leverage the **Common Data Model** to identify patients and outcomes. Patient stakeholders (**ADAPTORS**) drive success!
- Enrollment rates far exceed other trials in this area. The project has achieved 92% of its enrollment goal to date (>14,000 of 15,000)!



ADAPTABLE is funded by PCORI
ClinicalTrials.gov NCT02697916

PCORnet
Select Future Opportunities for the Network

- PREVENTABLE (**PR**agmatic **EV**aluation of ev**ENT**s **A**nd **B**enefits of **L**ipid-lowering in old**E**r adults)
  - NIH (NIA, NHLBI, NINDS) application with budget of $60.5M submitted February 1st *(Review in May 2019)*
  - Response led by Mid-South (PIs: Alexander, Hernandez, Williamson, Ambrosius)
  - All 7 eligible PCORnet networks (~40 sites) participating*
  - Front Door prep-to-research query informed feasibility, approach, and site selection



AIM 1: Determine the role of a moderate-intensity statin in preventing dementia and prolonging disability-free survival in patients 75 years and older without clinically evident coronary heart disease, including those with frailty, impaired physical function, mild cognitive impairment, polypharmacy, and multi-morbidity.
AIM 2: Determine the role of moderate-intensity statin in preventing hospitalization for myocardial infarction/acute coronary syndrome, stroke, heart failure, revascularization or cardiovascular-related death, and preventing either mild cognitive impairment or dementia.
AIM 3: Test the safety and tolerability of statins in older adults and collect 17,000 bio-specimens to advance precision health.

*Neither PEDSnet nor ADVANCE have sufficient numbers of the eligible population

PCORI Board of Governors Dashboard

Second Quarter FY-2019 (As of 3/31/2019)

Inputs

Process

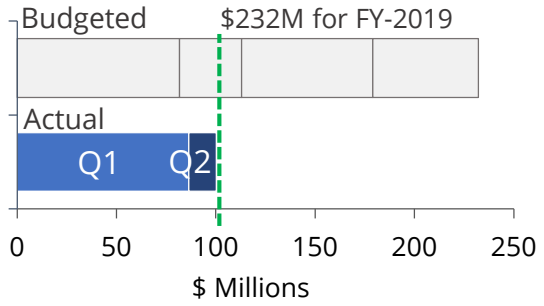
Outputs

Uptake

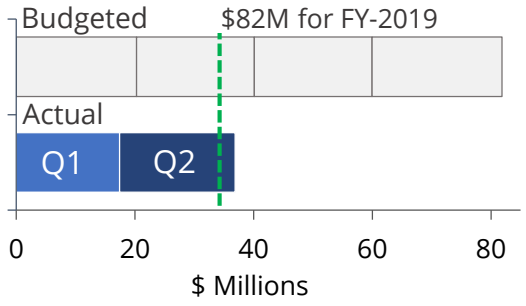
Use

Impact

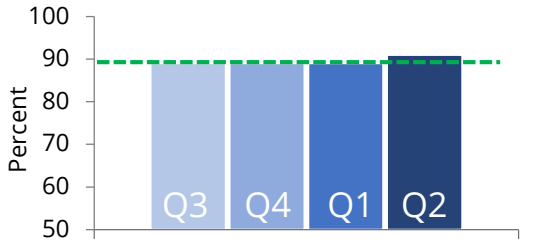
Funds Committed



Operational Expenses

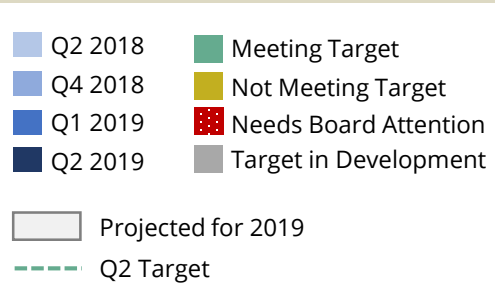


Research Project Performance

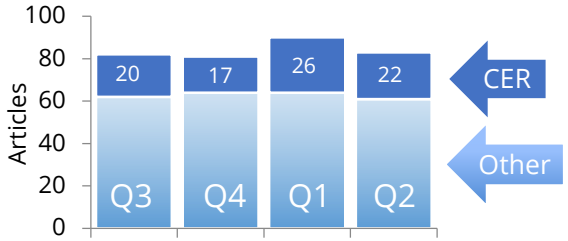


% of Research Projects On Track

Dashboard Key

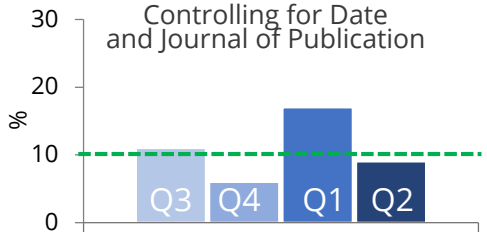


Results Published in Literature



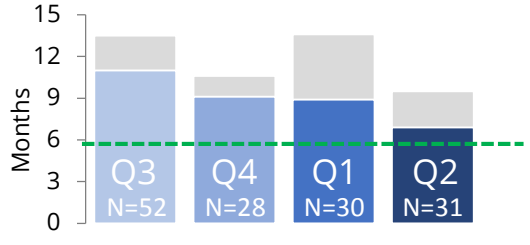
CER Results and Other Results Published in the Literature

Altmetric: Attention to PCORI Results



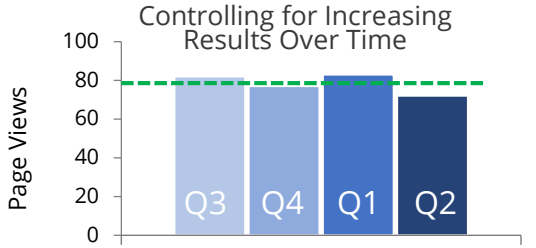
% of CER Results Publications in Top 10% of Attention

Speed of PCORI Peer Review



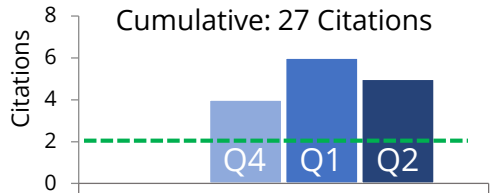
Median Time to Complete Peer Review (Grey = 75th Percentile)

Results Viewed on PCORI.org



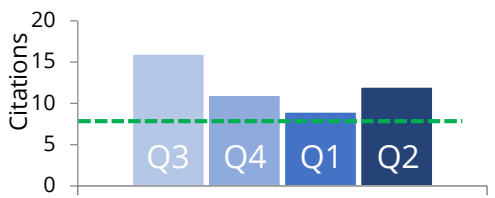
Average Pageviews of Results Posted to PCORI.org

Uptake into UpToDate®



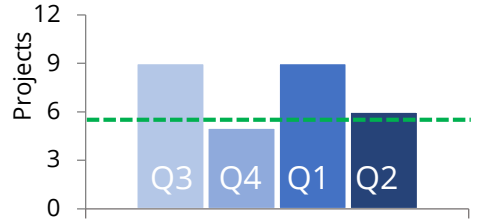
Citations in UpToDate® point-of-care decision tool

Other Examples of Uptake



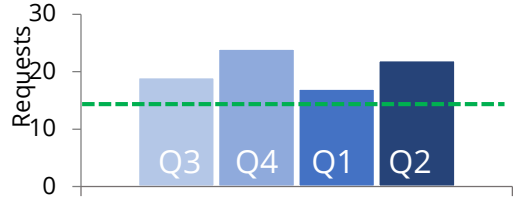
Citations in Systematic Reviews, Guidelines, & Policy Documents

New Studies Underway in PCORnet



New Studies Underway in PCORnet

Front Door Requests in PCORnet



Front Door Requests Submitted to PCORnet

Discussion Questions

- Do our FY-2019 Dashboard and associated background materials cover the topics that are most important for the Board to review?
- Do you have questions about our Q2 in-depth focus on PCORnet?
- Do you have questions about our Q2 in-depth focus on PCORI Peer Review?
- What questions do you have for our upcoming Q3-19 in-depth focus on Recruitment, Modifications, and Progress of Projects?

Research Portfolio Exploration Series:

Portfolio Overview

Joe Selby, MD, MPH

Executive Director

Exploring PCORI's Portfolio of CER Studies

- First, a *brief overview* of some basic dimensions of PCORI's CER portfolio
  - To set the *context*
  - Find out *what else you might like to know* about our portfolio
    - We will prepare these analyses for future meetings

- Then, an *in-depth look* at one of our topic portfolios – Telehealth
  - The first of many planned topic presentations, with others already in the queue
    - Opioids, Multiple Sclerosis, Mental Health, Cancer

- *What other topic portfolios would you like to learn about?*
  - We will prepare in-depth looks for future meetings

Overall Research Portfolio (including Methods Research)

21 cycles funding 622 studies for a total of $1.95B

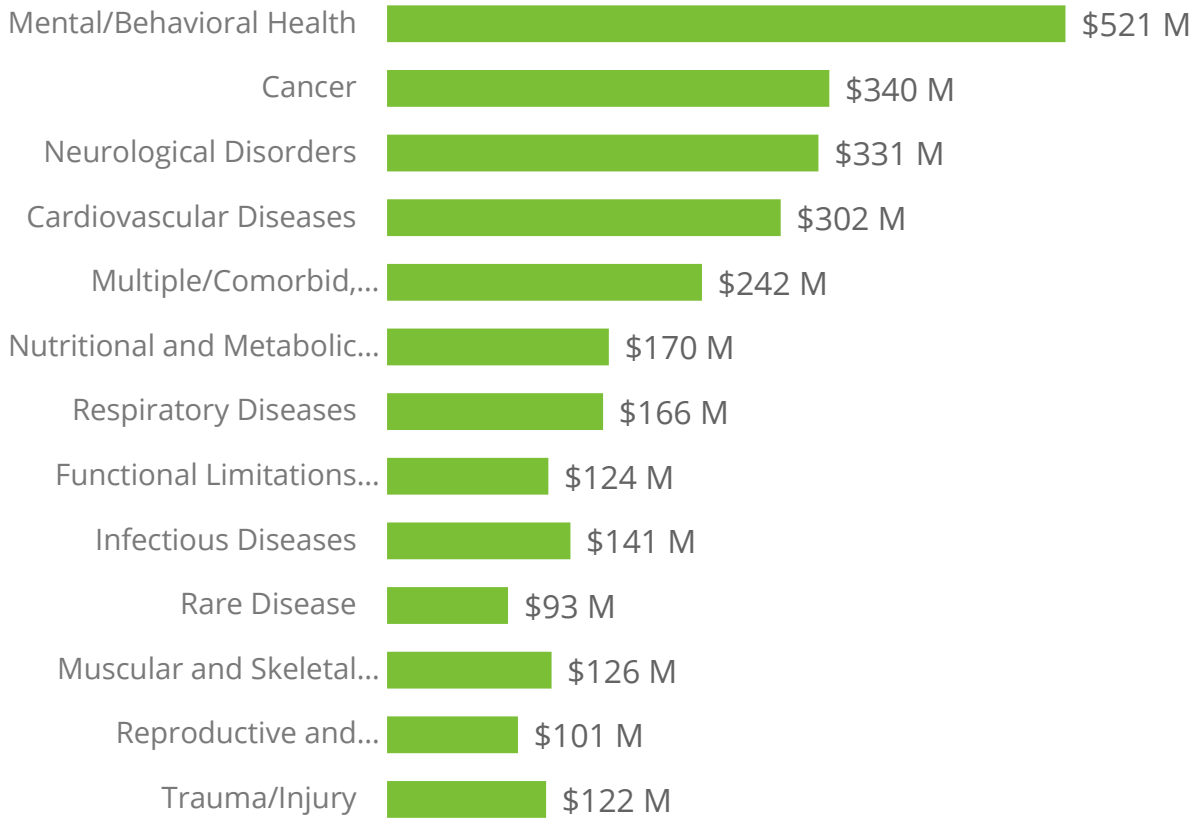
Broad PFAs	Pragmatic PFAs	Targeted PFAs
17 cycles	11 cycles	28 topics
493 studies	43 studies	86 studies
$902 M	$493 M	$557 M

Clinical Conditions in CER Studies

Number & Funding by Condition Category

By Number of Studies (n=464)

By Funding (Board-Approved $)

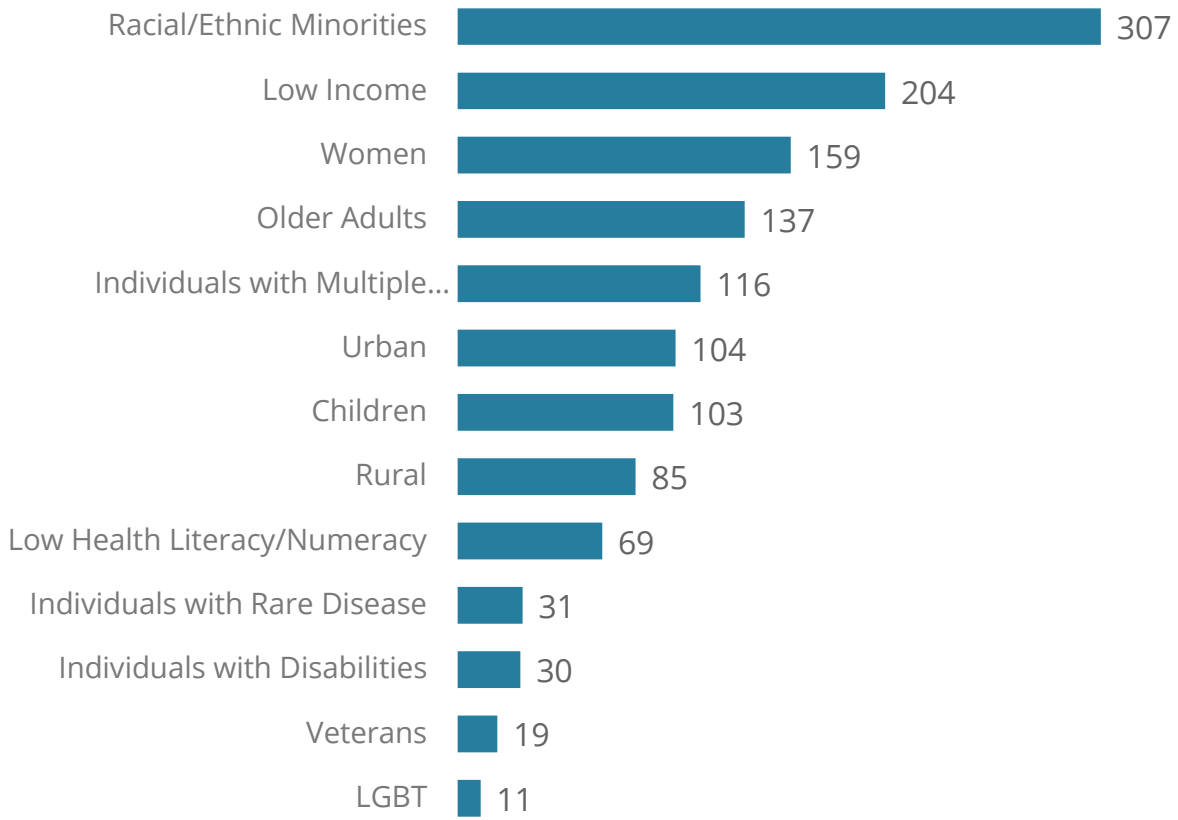


A study may be counted across more than one category

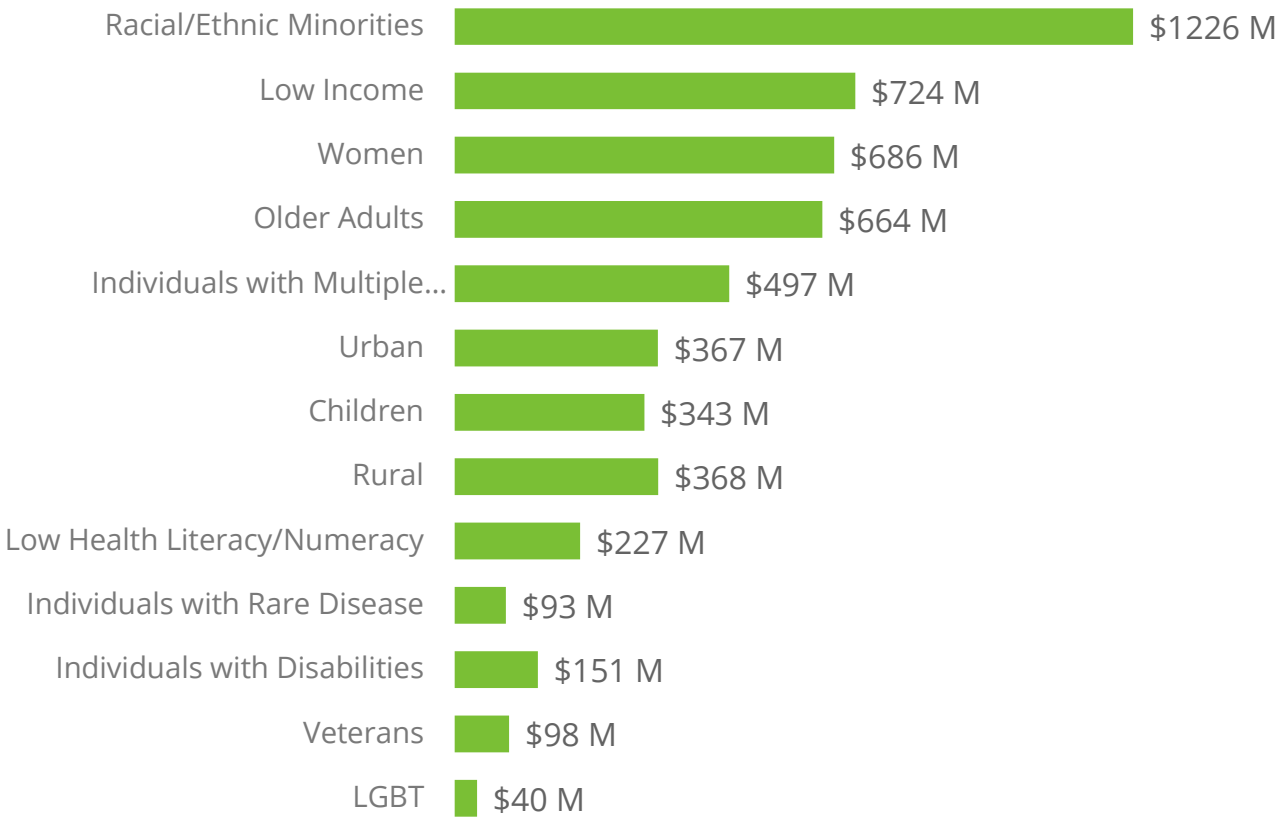
Populations of Interest in CER Studies

Number & Funding by Population

By Number of Studies (n=464)



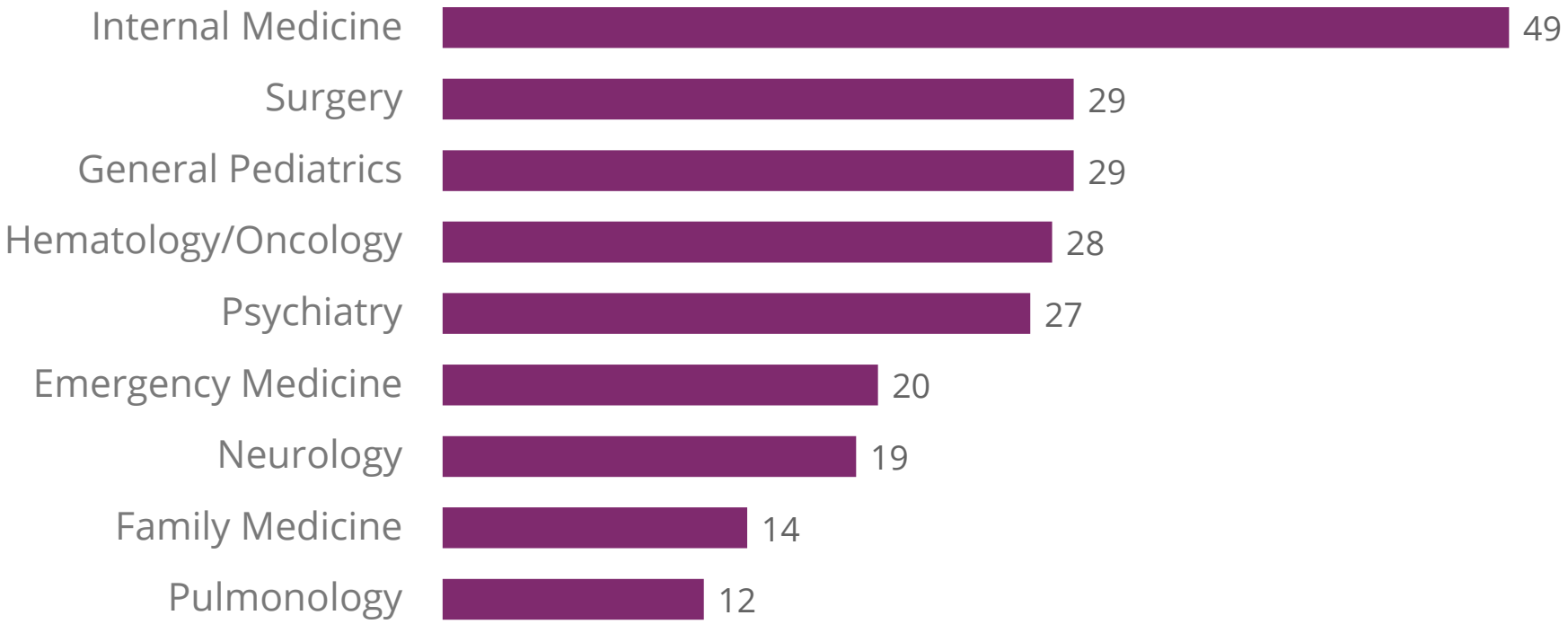
By Funding (Board Approved $)



A study may be counted across more than one category

Principal Investigators in CER Studies
Most Common Medical Specialties

Over half of PCORI CER studies are led by a principal investigator with a medical degree, including the following specialties:

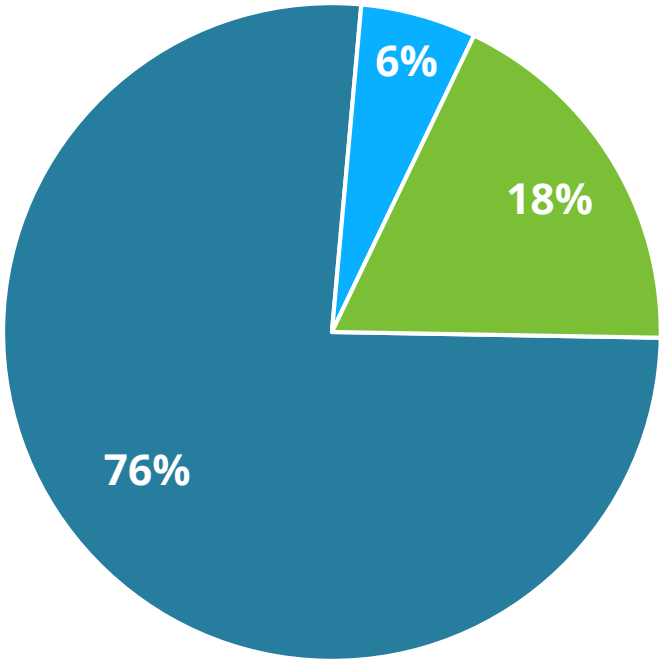


Number of principal investigators

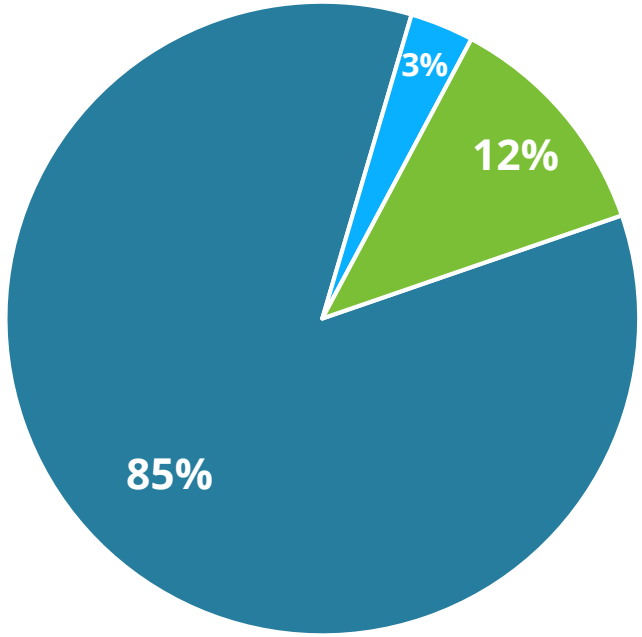
Lead PIs may be counted across more than one category. Chart shows specialties with 12 or more PIs.

CER Portfolio
CER Distribution by Study Design

- RCTs
- Quasi-Experimental
- Observational

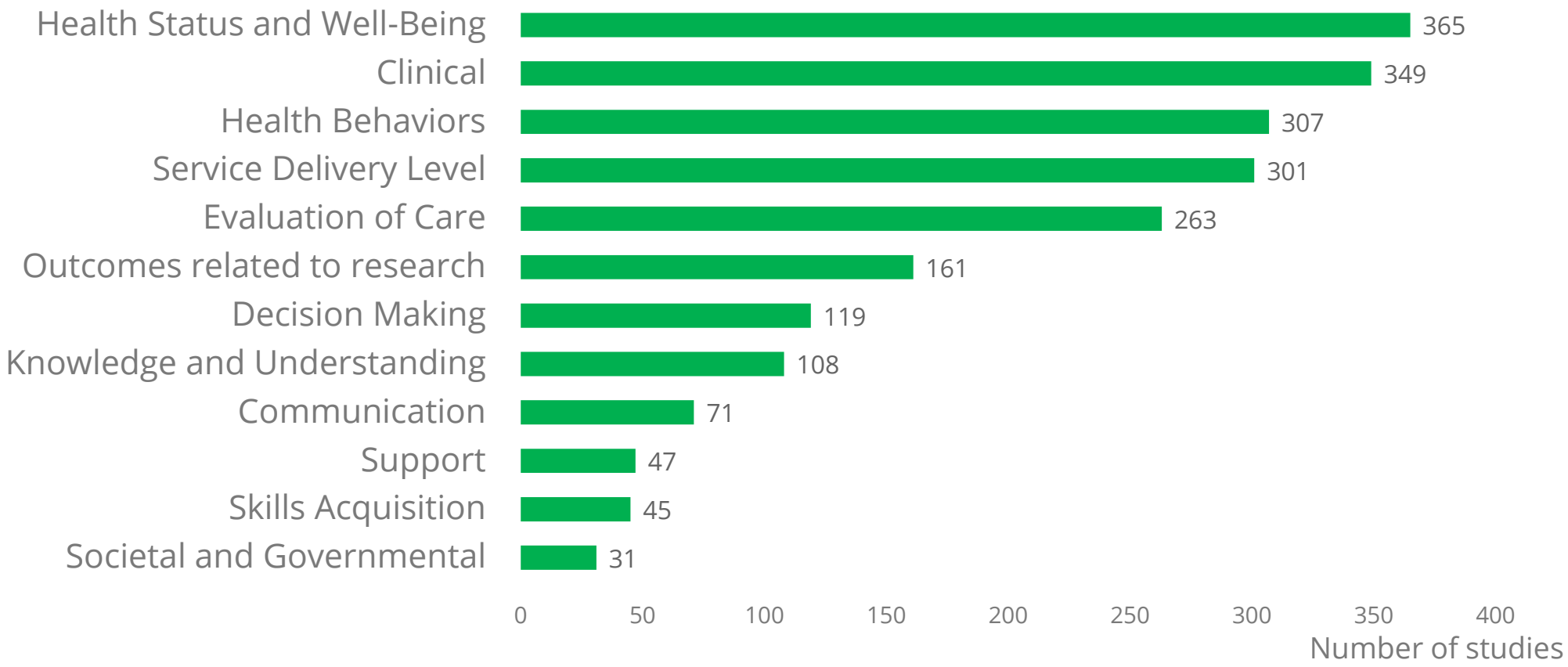


Study Design by
number of awards



Study Design by
Funding amount ($)
Total: $1.8B

CER Study Outcomes
Themes



Studies often measure outcomes across more than one theme.

PCORI Funds Research on High-Cost, High-Impact Health Conditions

Condition	Number of PCORI Studies	PCORI Investment
Stroke	15	$63 M
Heart Failure	15	$70 M
Hepatitis (Chronic Viral B and C)	8	$59 M
Chronic Obstructive Pulmonary Disease	17	$89 M
Schizophrenia/Other Psychotic Disorders	11	$22 M
Chronic Kidney Disease	18	$72 M
Asthma	16	$51 M
Atrial Fibrillation	4	$8 M
Alzheimer's Disease/Dementia	13	$78 M
Depression	47	$196 M
Cancer	85	$336 M
Ischemic Heart Disease	22	$108 M
Osteoporosis	1	$14 M
HIV/AIDS	9	$28 M
Arthritis	15	$50 M
Diabetes	32	$75 M
Hypertension	12	$54 M
Hyperlipidemia	7	$27 M
Autism Spectrum Disorders	2	$4 M

PCORI has funded **260 CER studies** examining what works best in care for the **19 highest cost conditions** in the United States^

^High-cost clinical conditions identified by the Centers for Medicare and Medicaid Services for 2015.
A study may be counted across more than one condition category.

Future Portfolio Exploration

- Thanks to the diligent work of teams that contribute to information services, we now have much *more information available* for each aspect of our funded research portfolio – for example, detail about the outcomes being studied in funded projects, as well as growing info an applications that were not funded

- *What questions do you have* about PCORI's portfolio of funded studies?

- What should we prepare to focus on in *future portfolio explorations*?

Overview of PCORI's Telehealth Portfolio:

Reflections of a Changing Delivery System

Penny Mohr, MA

Senior Advisor, Emerging Technology and Healthcare Delivery Innovation Research Initiatives

Overview

- Background
- Overview of the Telehealth Portfolio
- Putting Our Findings into Context
- Looking Ahead

- **Background**
- Overview of the Telehealth Portfolio
- Putting Our Findings into Context
- Looking Ahead

What is Telehealth?

Telehealth

- The use of medical information exchanged **from one site to another** via **electronic communications** to improve a patient's clinical health status. Voice only interactions are excluded

Telemedicine

- Telemedicine seeks to improve a patient's health by permitting **two-way**, **real time** or **asynchronous interactive** communication between the **patient, and the physician or practitioner** at the distant site. It allows health professionals to **evaluate, diagnose, and treat** patients at a distance

mHealth

- The use of **mobile and wireless devices** to improve health outcomes and healthcare services at a distance from the health care provider. Includes **unidirectional** communication (e.g., text message support)

Why PCORI Attracted Telehealth

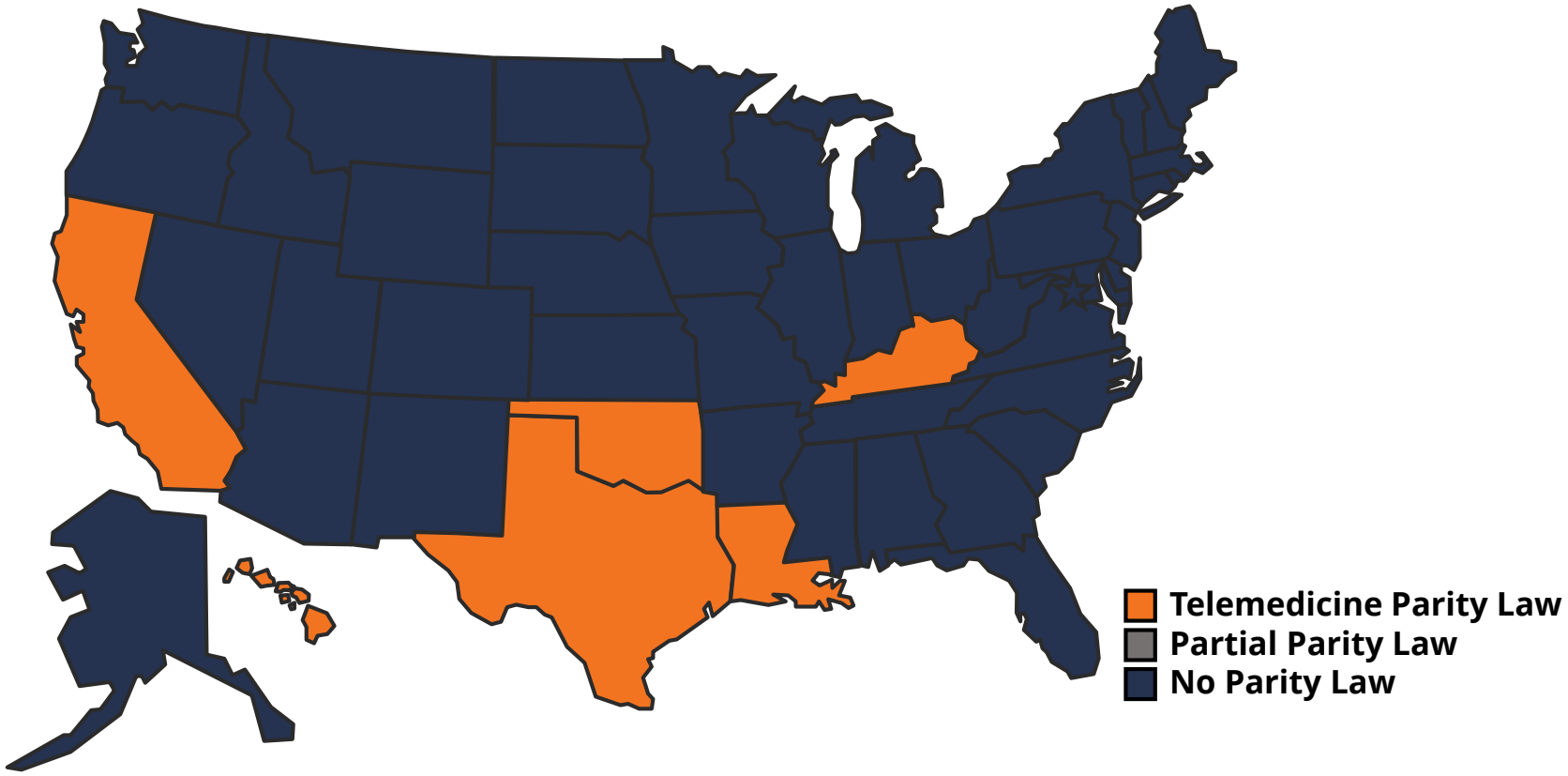
Research Gaps in Telehealth Reflect Our Mission

- **Robust Comparative Studies**
  - Head-to-head comparisons of functionality/integration
- **Measurement of Patient-Centered Outcomes**
  - Function, symptoms, and health-related quality of life.
- **Understanding Variation Across Diverse Populations and Settings.**
  - Addressing individual differences and barriers to implementation and dissemination
- **Engaging Stakeholders in the Design**
  - User-centered design can help surmount barriers to adoption and sustainability (integration into the workflow; acceptability to patients)

Telehealth Affords Patient-Centered Care

- **Delivery where and when care is needed**
- **Personalization of the interface**
  - Low health literacy /Limited English proficiency
  - Cultural preferences

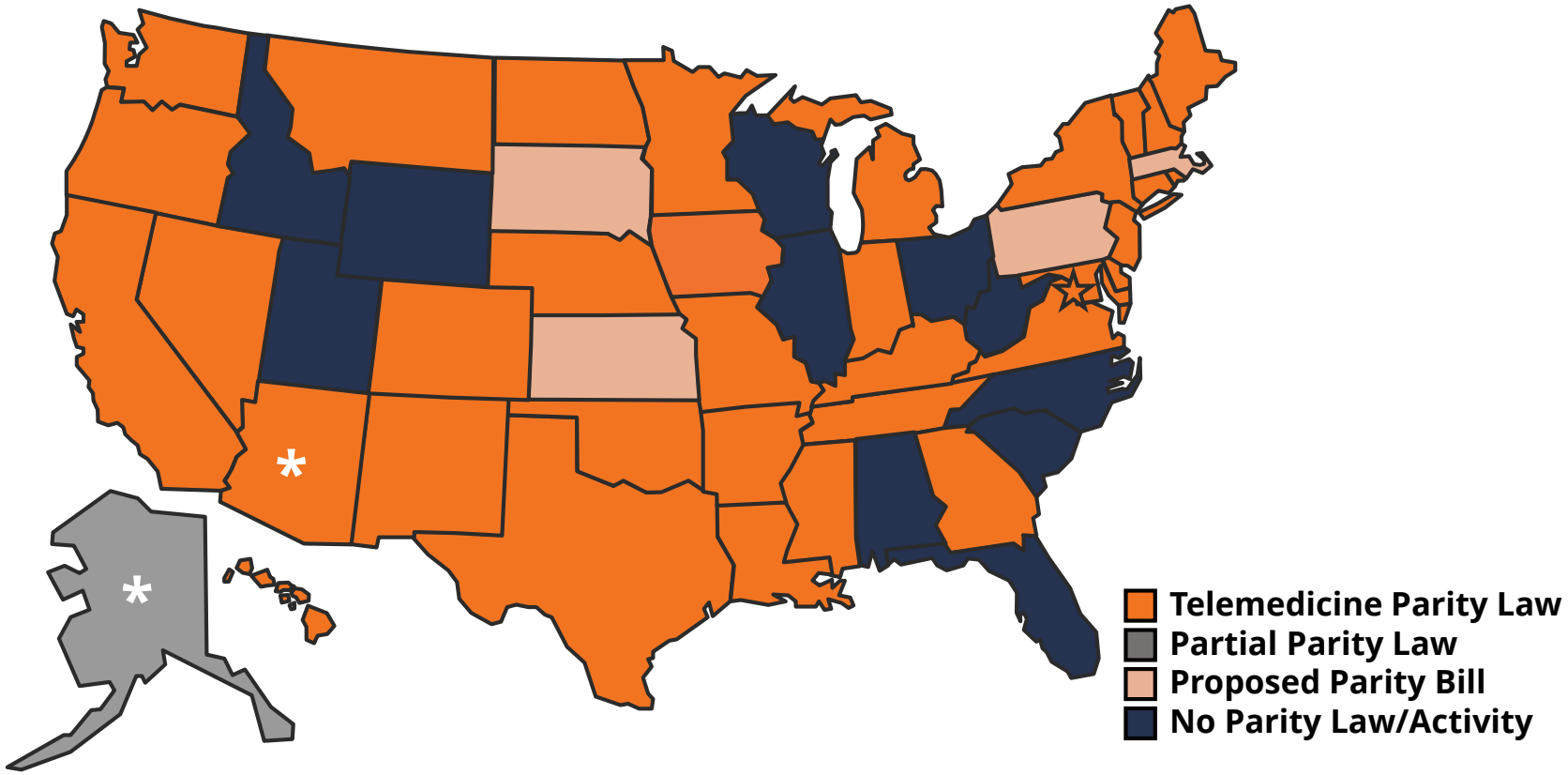
States with Parity Laws for Private Insurance Coverage of Telemedicine (2000)



States with the year of enactment: California (1996), Hawaii (1999), Kentucky (2000), Louisiana (1995), Oklahoma (1997), and Texas (1997)

AMERICAN TELEMEDICINE ASSOCIATION

States with Parity Laws for Private Insurance Coverage of Telemedicine (2018)



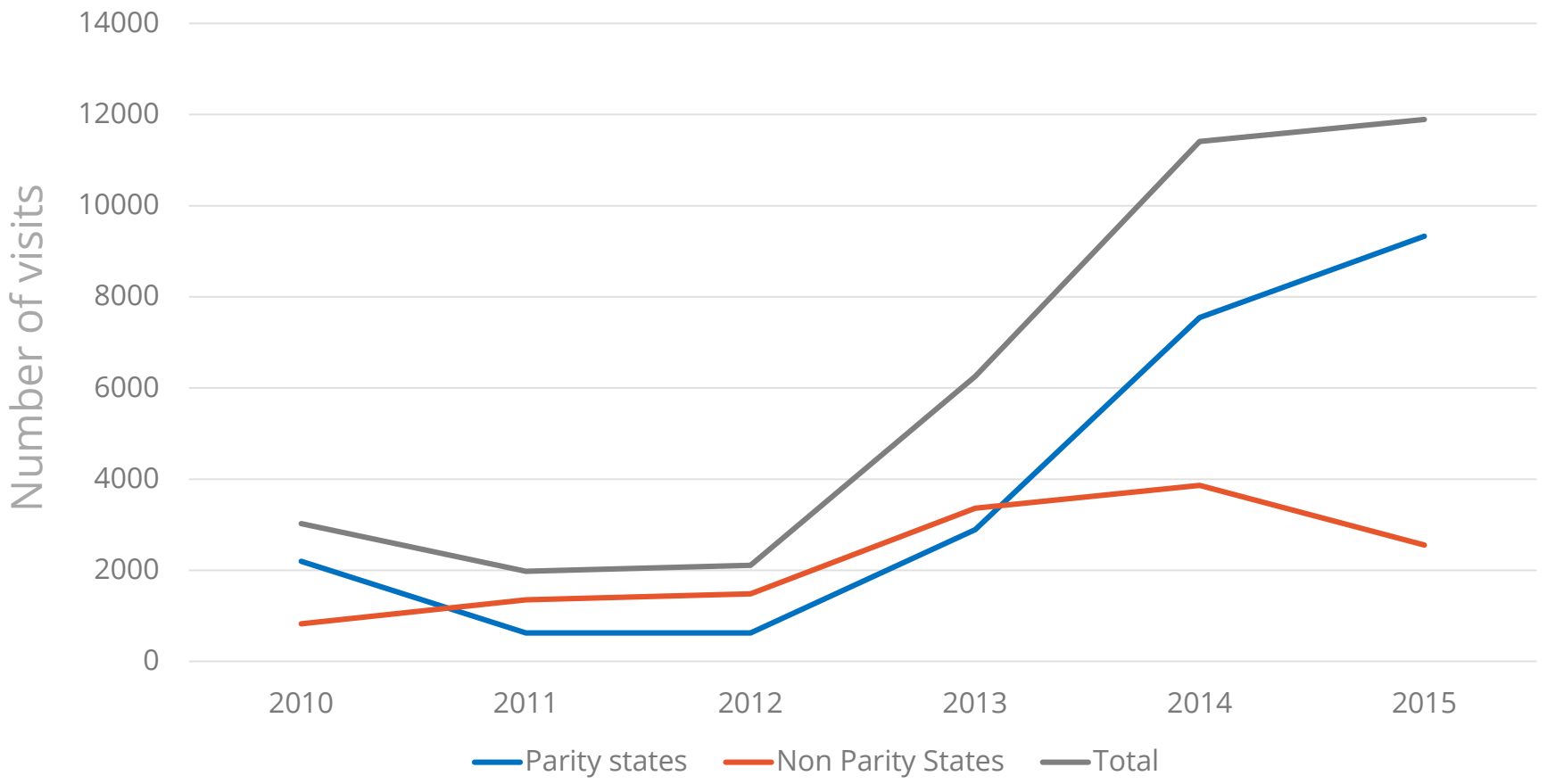
States with the year of enactment: Alaska (2016)*, Arizona (2013)*, Arkansas (2015), California (1996), Colorado (2001), Connecticut (2015), Delaware (2015), Georgia (2006), Hawaii (1999), Indiana (2015**), Iowa (2018),** Kentucky (2000), Louisiana (1995), Maine (2009), Maryland (2012), Michigan (2012), Minnesota (2015), Mississippi (2013), Missouri (2013), Montana (2013), **Nebraska (2017)**, Nevada (2015), New Hampshire (2009), **New Jersey (2017)**, New Mexico (2013), New York (2014), **North Dakota (2017)**, Oklahoma (1997), Oregon (2009), Rhode Island (2016), Tennessee (2014), Texas (1997), Vermont (2012), Virginia (2010), Washington (2015) and the District of Columbia (2013)

States with proposed legislation: In 2018, Kansas, Massachusetts, Pennsylvania, and South Dakota

**Coverage applies to certain health services.*

AMERICAN TELEMEDICINE ASSOCIATION

Growth in Outpatient Telehealth Services in Private Insurance



Source: Harvey JB et al. Utilization of Outpatient Telehealth Services in Parity and Non-Parity States, 2010-2015. Telemedicine and eHealth Published online 30 May 2018; https://doi.org/10.1089/tmj.2017.0265

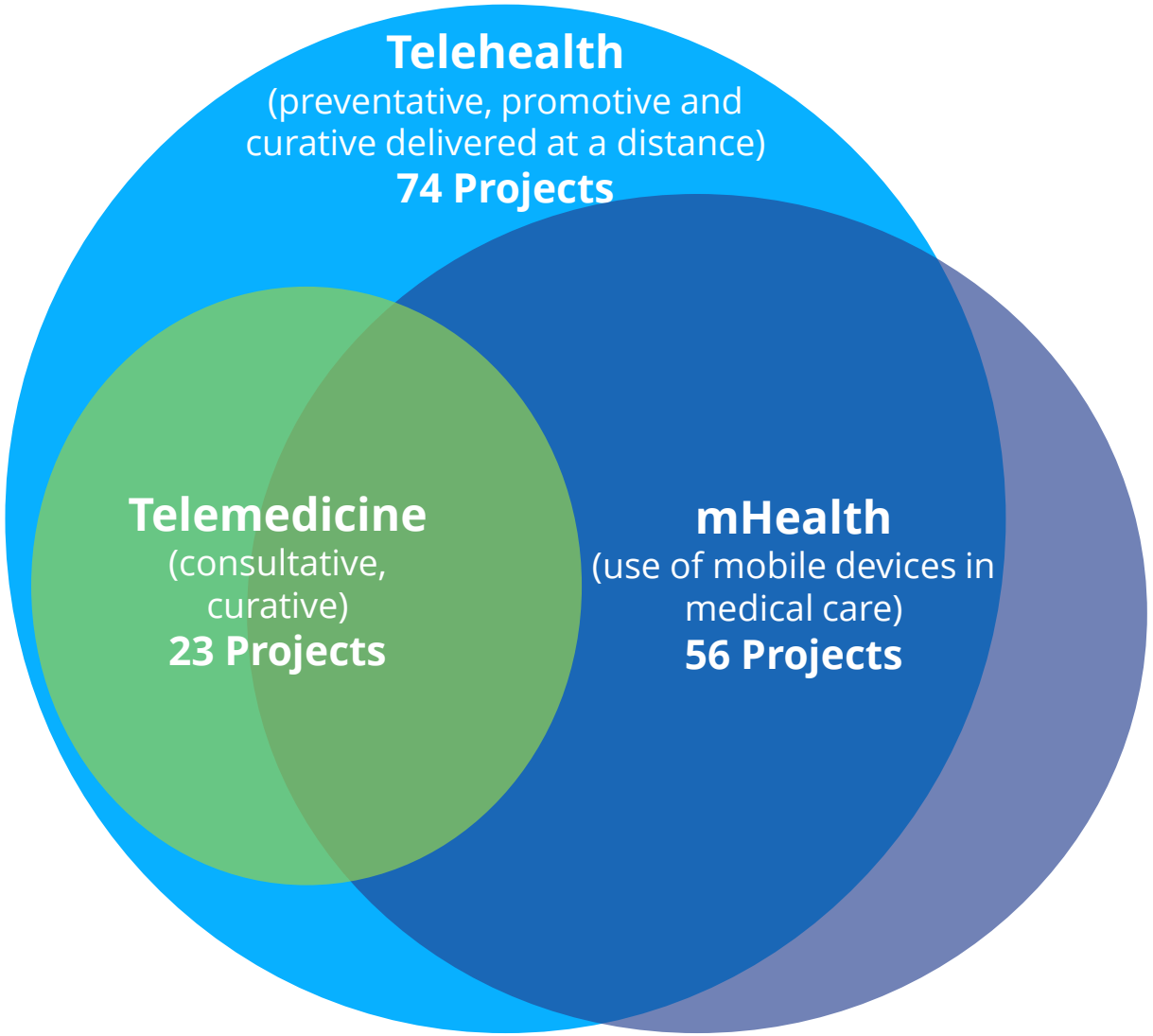
A Trend Towards Expanded Coverage

- Medicare Advantage Plans
  - Eliminates geographic restrictions
  - Allows payment for services in the home
- Physician Fee Schedule
  - Expands coverage for remote monitoring
  - Pays for Brief Virtual Visits
  - Expands coverage for inter-professional consults
- Expands coverage for addiction treatment

- All but one state pays for live video
  - Expansion by some states to eliminate originating site restrictions and include more specialties
- 11 states pay for store and forward
- 20 states pay for remote monitoring
- Allows remote prescribing for opioid treatment

- Background
- **Overview of the Telehealth Portfolio**
- Putting Our Findings into Context
- Looking Ahead

PCORI's Telehealth, Telemedicine, and mHealth Portfolio

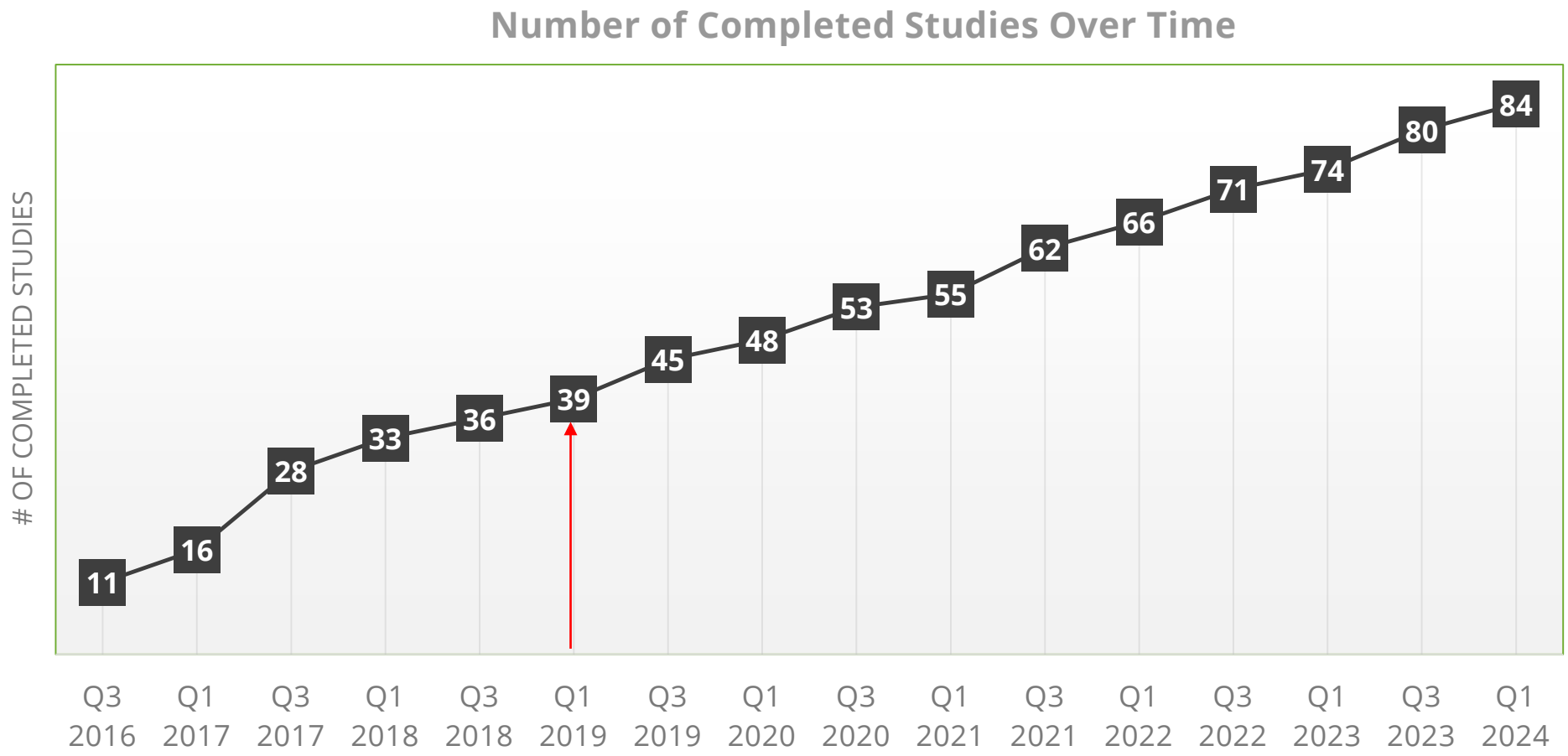


$352 MILLION SUPPORTING 84

COMPARATIVE CLINICAL EFFECTIVENESS RESEARCH STUDIES IN TELEHEALTH

As of April 2019
Projects may be classified as more than one type

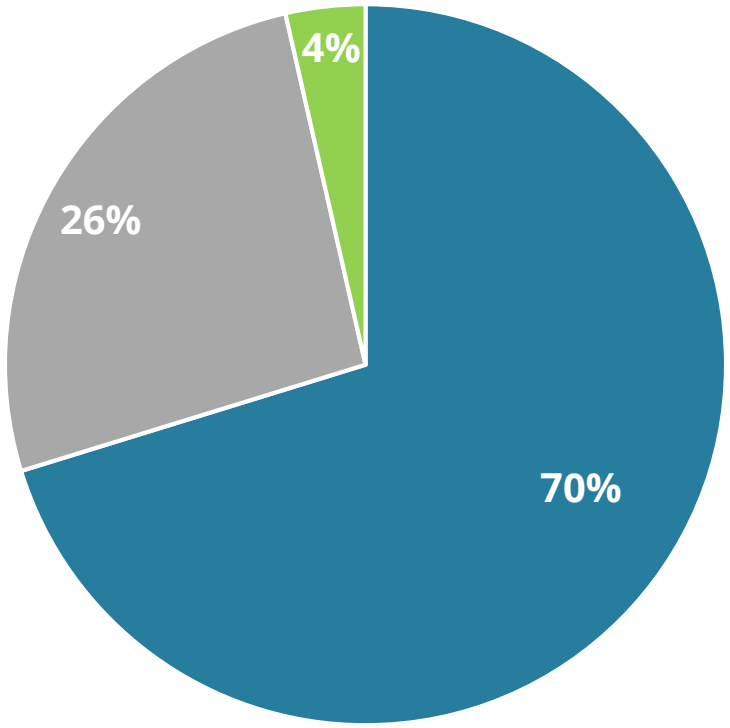
When Will Results from Telehealth Studies Likely Be Available?



Average duration of telehealth projects (N=84): **3.7 years**

Telehealth Projects by Study Design

Nearly all studies are RCTs

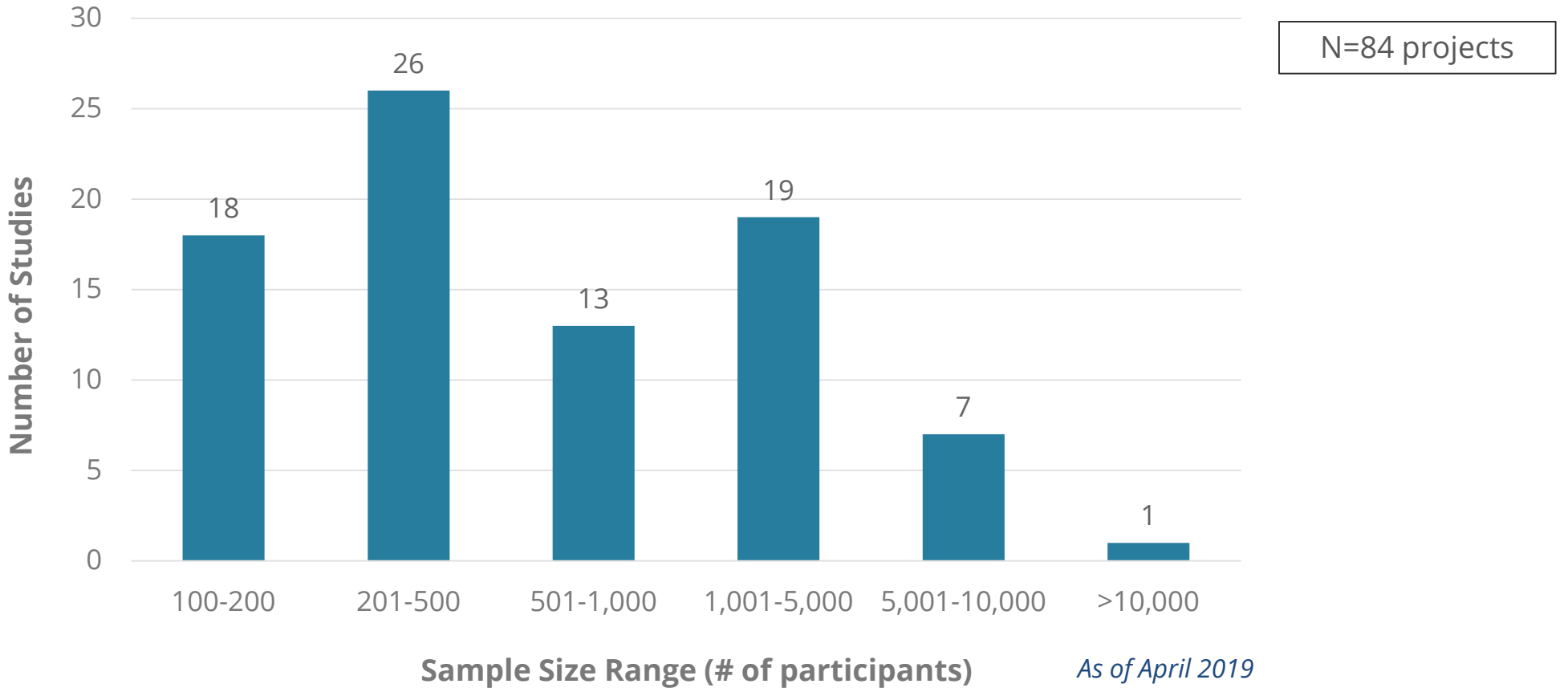


- Individual Randomized Controlled Trials (N=59)
- Cluster Randomized Controlled Trials (N=22)
- Observational Studies (N=3)

As of April 2019

Range of Sample Sizes Among Telehealth Projects

While most studies are moderate size, a significant number are very large



STUDY PROFILE

Comparative Effectiveness of Early Integrated Telehealth Versus In-Person Palliative Care for Patients with Advanced Lung Cancer

What This Study Does

- Compares the effectiveness of early integration of palliative care delivered via telemedicine versus in-person visits for advanced lung cancer patients living in remote areas

Design

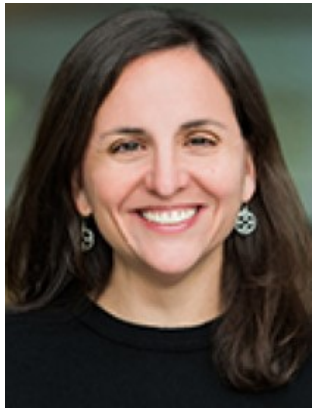
- Individual RCT (1,250 patients and 987 caregivers)
- 20 institutions in 17 states

Key Outcomes

- Quality of life, satisfaction with care, healthcare utilization

Engagement

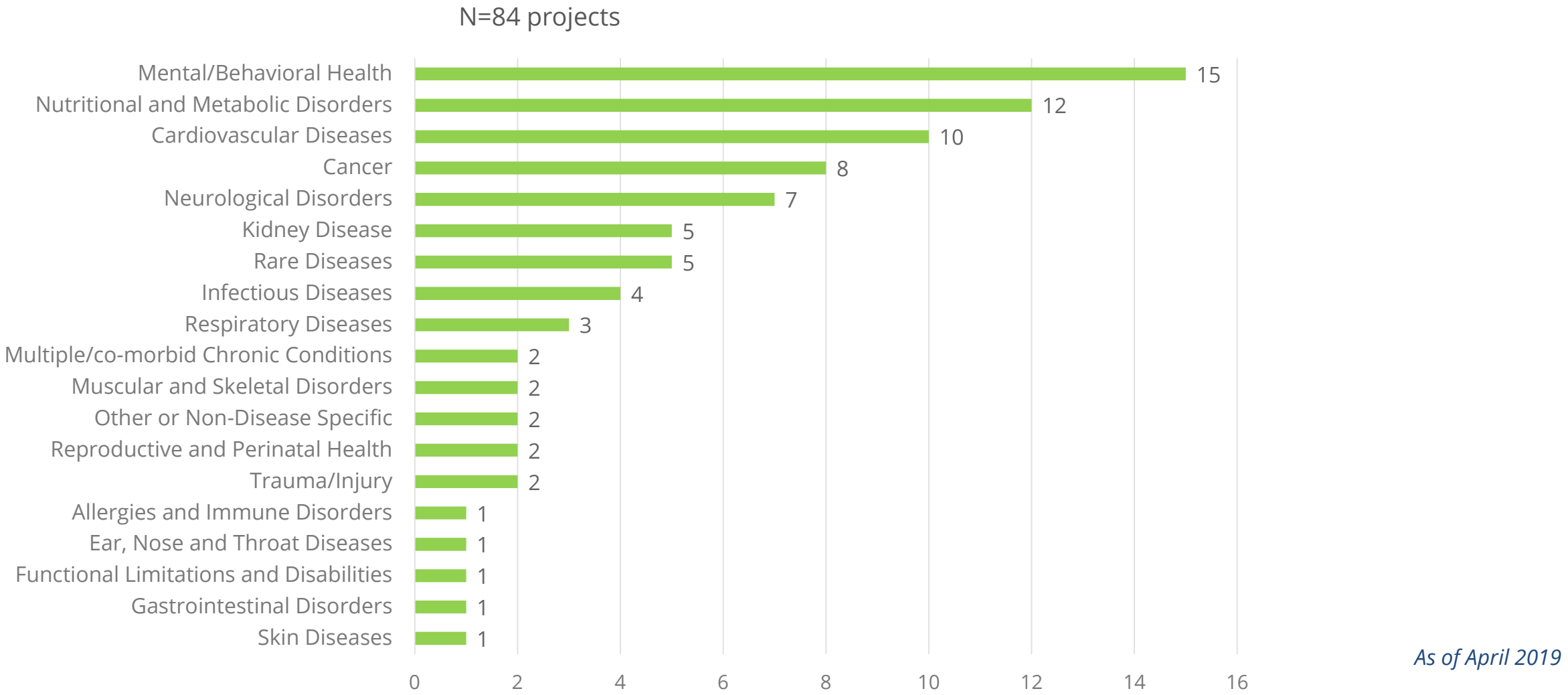
- Patients, caregivers, clinicians, telemedicine experts, health systems leaders, payers, and health policy experts will be engaged throughout this study



Jennifer Temel, MD
Massachusetts General Hospital

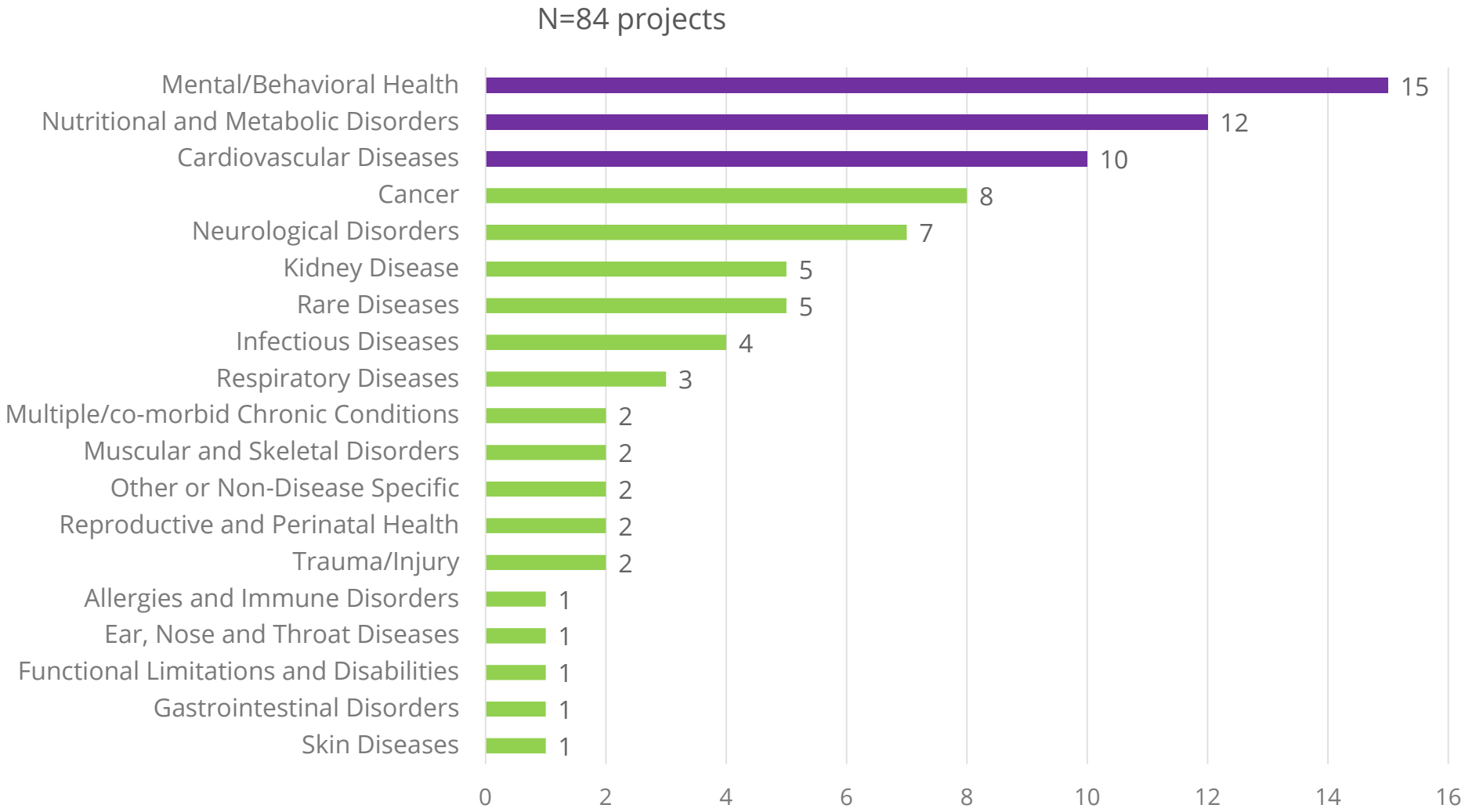
Palliative Care Targeted Funding Announcement, awarded August 2017

Number of Telehealth Projects by Primary Disease/Condition



As of April 2019

Number of Telehealth Projects by Primary Disease/Condition



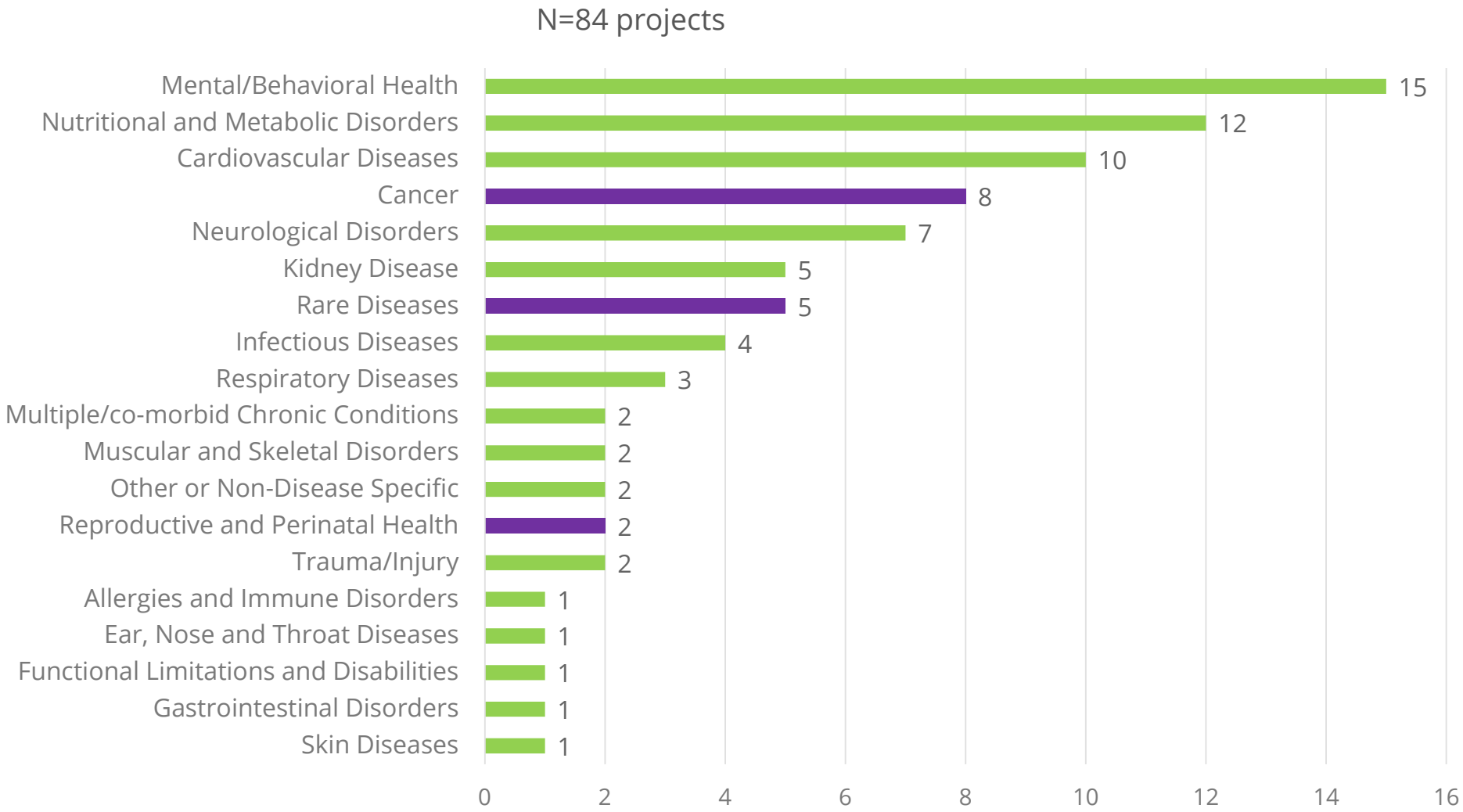
Mental and Behavioral Health

Nutritional and Metabolic Health

Cardiovascular Health

As of April 2019

Number of Telehealth Projects by Primary Disease/Condition



Cancer

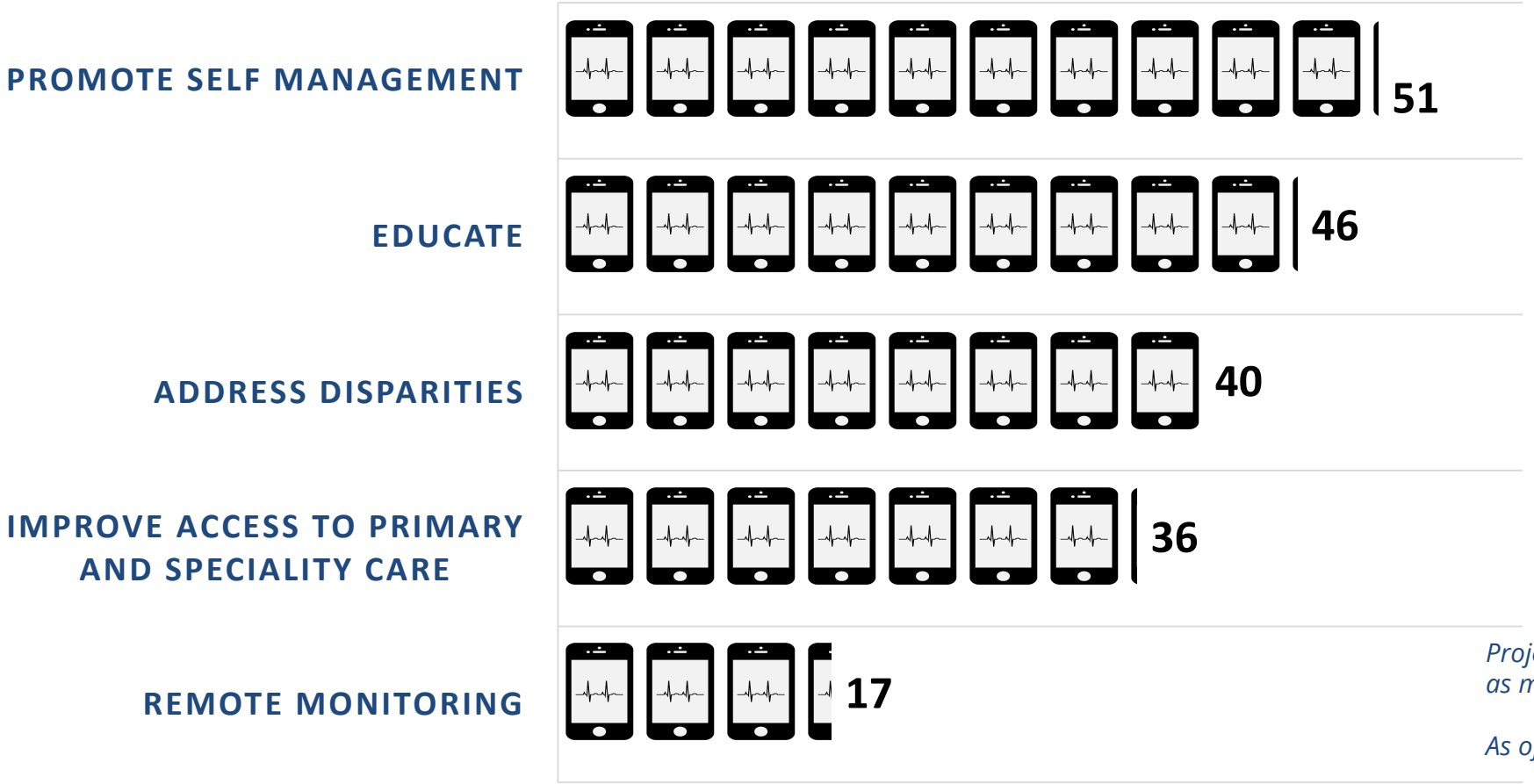
Rare Disease

Reproductive and Perinatal Health

As of April 2019

A Focus on Promoting Self-efficacy and Knowledge

Purpose of telehealth intervention



Projects may be classified as more than one type.

As of April 2019.

Framework adapted from American Telemedicine Association and Bashshur et al. The taxonomy of telemedicine. Telemedicine and eHealth. 2011 with stakeholder input.

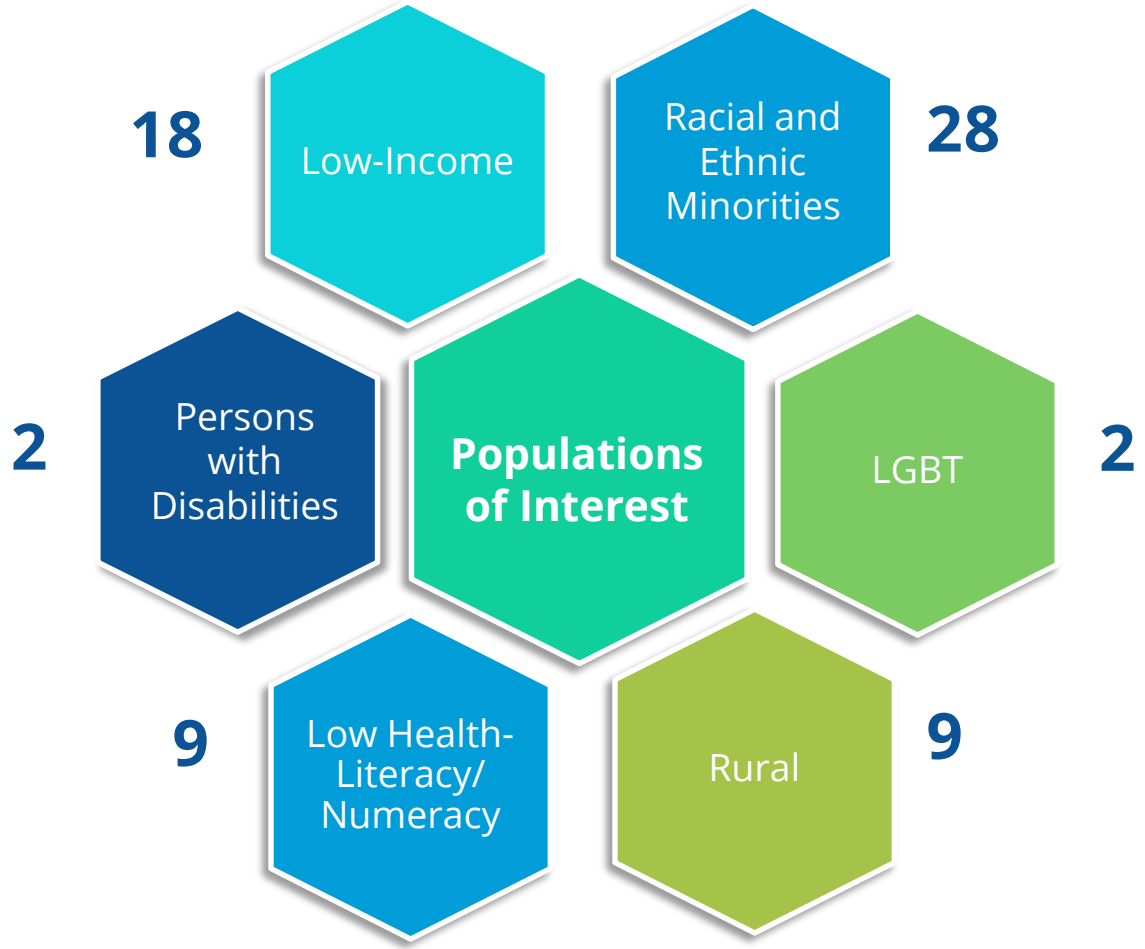
Our Telehealth Projects Target Underserved Populations

40
STUDIES

Are utilizing telehealth to address disparities by addressing at least 1 of the populations of interest

$173
MILLION

The total research investment towards telehealth studies that address disparities



N=40, as of April 2019.
Categories are not mutually exclusive

What Telehealth Modalities Are Included?

PCORI telehealth projects incorporate diverse modalities

Mobile phones or tablets
56 Studies
(22 use tailored messaging)

Remote monitoring through wireless devices
17 Studies

Store and forward
6 Studies

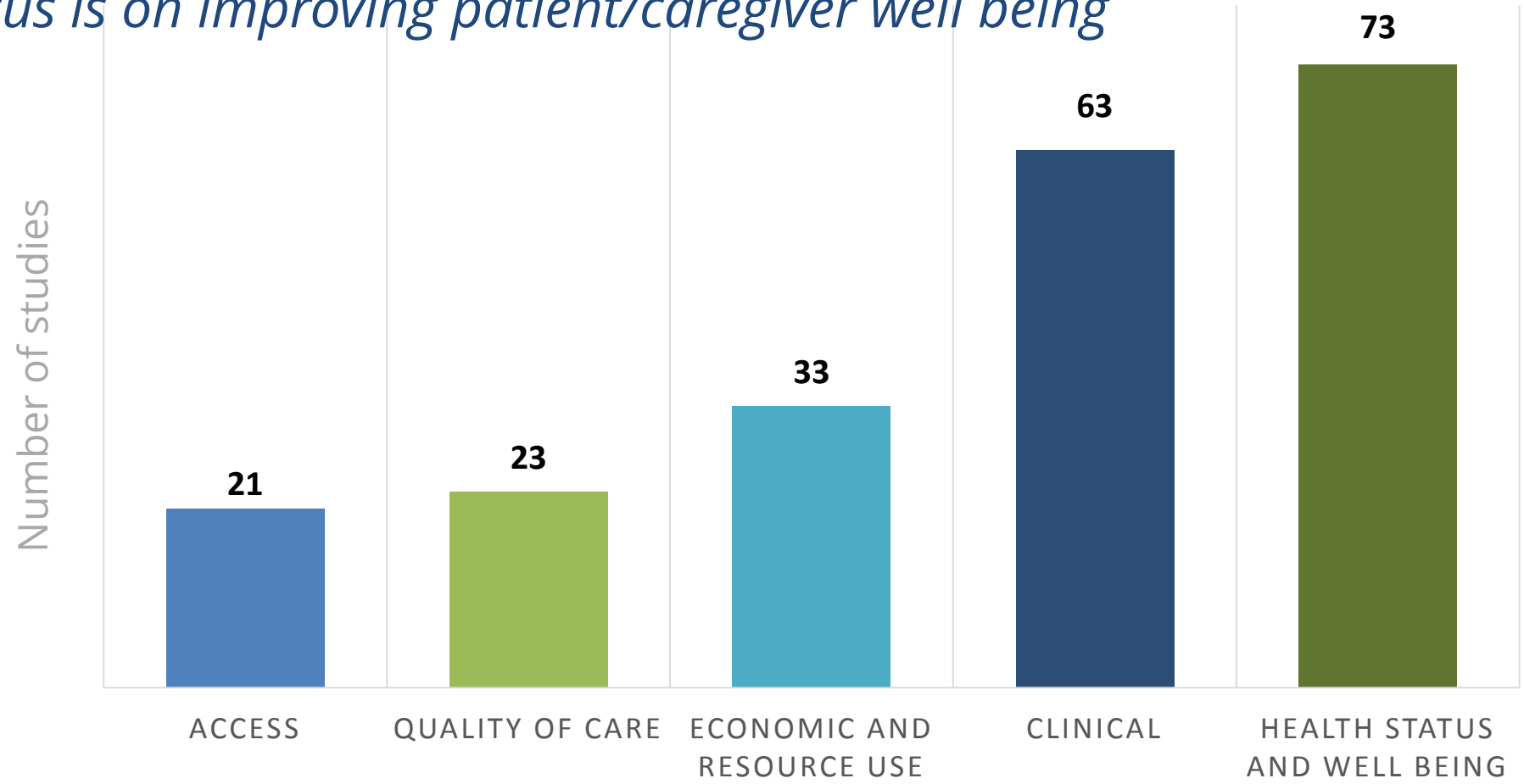
Interactive live video
31 Studies

Web portals (accessed through multiple devices)
62 Studies

Categories are not mutually exclusive

Outcomes for Telehealth Studies

The focus is on improving patient/caregiver well being



Projects may be classified as more than one type As of April 2019.

Framework adapted from: Edmunds et al. An Emergent Research and Policy Framework for Telehealth. *eGems* 2017; 5(2): available at: https://www.ncbi.nlm.nih.gov/pmc/articles/PMC5389433/

STUDY PROFILE

Using Technology to Deliver Multidisciplinary Care To Individuals With Parkinson's Disease In Their Home

What This Study Did

- Evaluated the feasibility, effectiveness, and satisfaction associated with tele-healthcare visits for patients with Parkinson's Disease

Design

- RCT (200 patients)
- 20 sites in 16 states

Key Findings

- Telehealth was feasible
- High levels of satisfaction
- No differences in quality of life, quality of care, or caregiver strain for telehealth versus control

Engagement

- Parkinson's Foundation, patient advisory board members, and steering committee

Earl Ray Dorsey, MD, MBA
University of Rochester, Rochester, NY

Dorsey et al., National randomized controlled trial of virtual house calls for Parkinson's Disease. Neurology. 2017; 89(11):1152-1161.

DISSEMINATION AND IMPLEMENTATION AWARD

A Virtual Care Model for Parkinson's Disease and Other Chronic Conditions

Study Aims

- Expand intervention to include multidisciplinary care and address comorbid conditions (anxiety, depression, dementia)
- Implement the intervention through a funded statewide (NY) telemedicine program that will provide care to 500+ individuals with Parkinson's disease

What This Study Does

- Expands reach to rural areas, racial/ethnic minorities, low income, elderly
- Builds national capacity (training neurologists and allied healthcare workers in telehealth and comorbid disease management)

Engagement

- Patients and caregivers, Parkinson Disease Care NY, Finger Lake Health Systems Agency, local home health care, nursing homes in upstate NY
- International Parkinson and Movement Disorders Society, American Academy of Neurology, American Telemedicine Association, West Health Institute, Michael J Fox Foundation/Parkinson's Action Network

Earl Ray Dorsey, MD, MBA
University of Rochester, Rochester, NY

PATIENT-POWERED RESEARCH NETWORK

Inflammatory Bowel Disease (IBD) Partners

Network Goals

- Patient engagement, community integration and education
- Healthcare decisionmaking
- Research opportunities
- Personal longitudinal health and healthcare tracking

Membership

- 18,261 adult patients with Crohn's disease and ulcerative colitis, collectively referred to as inflammatory bowel disease

mHealth Component

- Incorporation of data imported from mHealth apps and wearable devices
- Captures patient-generated data for research

IBD PARTNERS

The University of North Carolina at Chapel Hill

Crohn's & Colitis Foundation

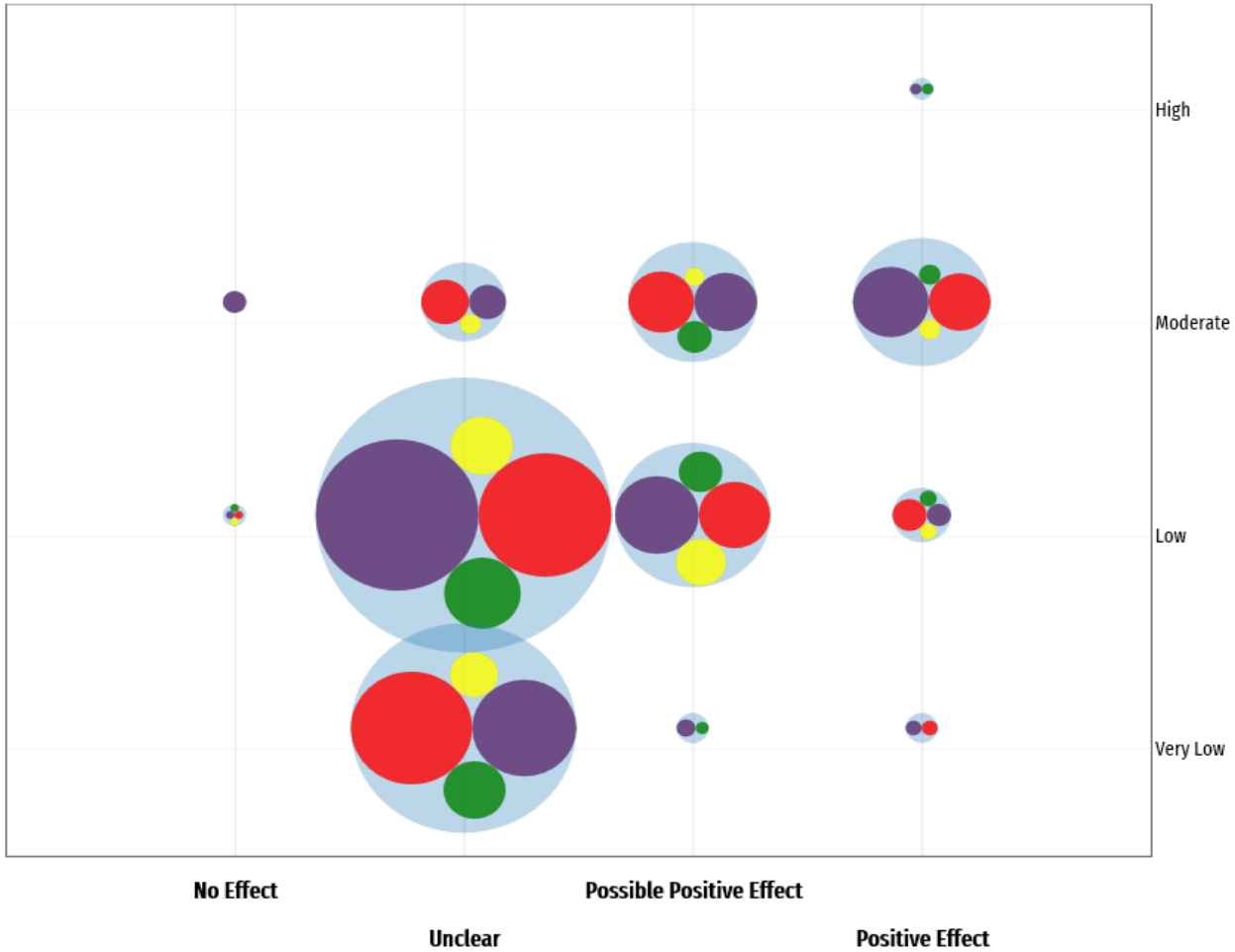
PI: Michael Kappelman, MD, MPH

Funded in 2014

- Background
- Overview of the Telehealth Portfolio
- **Putting Our Findings into Context**
- Looking Ahead

Evidence Map on mHealth for Self-Management of Chronic Disease

https://www.pcori.org/evidence-maps/results-strength-evidence-mHealth-systematic-reviews-1



482 Systematic Reviews Full Text Review
(2010 – 2017)

99 Included Assessing Strength of Evidence

19 PCORI Studies

Gaps being addressed:

- Vulnerable populations
- Pediatric populations
- Measuring patient-centered outcomes

Advancing the State of Evidence for Decisionmakers About Telehealth

PCORI Stakeholder Workshop (May 24, 2018)

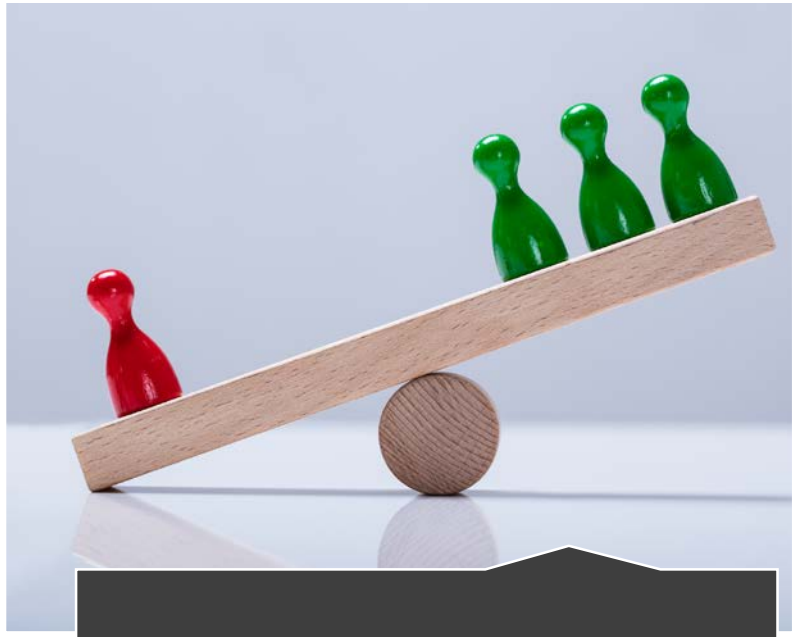
Key message: patient/clinician experience and context are critical

- Need to understand long-term adherence
  - Patient and provider experience that contributes to adoption/lack of interest and sustained use or discontinuation
  - Tracking use and outcomes beyond the study period
- Report out contextual factors that make the telehealth component work
  - Type of support personnel needed
  - How they interact with the care team
  - Type of training needed
  - Requirements for integration with the EHR

Included 22 participants representing patients, clinicians, payers, health systems, policymakers, and telehealth advocacy

Establishing Investigator Communities

Number of studies=36

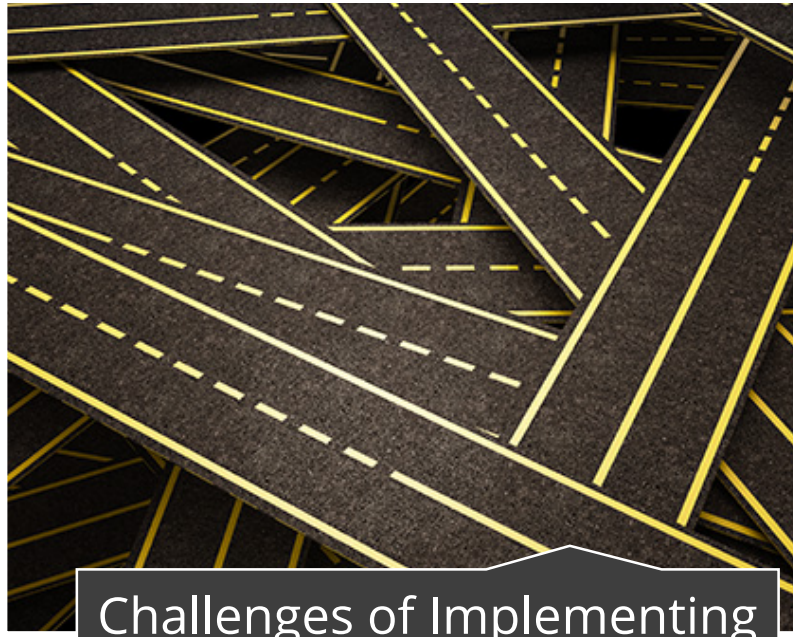


Addressing Disparities

Number of studies=29

mHealth for Self-Management of Chronic Disease

Number of studies=15



Challenges of Implementing Multi-site Telemedicine Trials

Engagement Award:
Community-Driven Evidence Dissemination

- Disseminating PCOR on Telehealth to State Health Policymakers to Maximize its Relevance & Impact
  - National Academy for State Health Policy (NASHP)
- NASHP is convening an interbranch, interagency workgroup of state health policy makers to disseminate PCOR findings on telehealth and explore in depth the policy and contextual issues affecting implementation
  - Create opportunities for policymakers to learn directly from their peers' experiences implementing telehealth
  - Share the necessary contextual information for policymakers to most effectively understand, and when appropriate, to implement actionable telehealth research
- Deliverables include an in-person convening at the NASHP 2019 Annual Conference, four posts on the NASHP blog, a topic brief, and a webinar
  - All promoted via NASHP's newsletter

- Background
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Remaining Gaps

- Head-to-head trials of mobile apps (Veazie et al., 2018)
- Cancer treatment (not symptom management) (Totten et al., 2016)
- Maternal and child health (Totten et al., 2016)
- Children and adolescents; Management of serious pediatric conditions (Totten et al., 2016; Reston et al., 2018)

Sources:

Reston et al. PCORI Evidence Map: The Impact of mHealth for Self-Management of Chronic Disease on Patient-Centered Outcomes. ECRI Institute–Penn Medicine Evidence Based Practice Center. 2018.

Totten et al. Telehealth: Mapping the evidence for patient outcomes from systematic reviews. Agency for Healthcare Research and Quality. 2016.

Veazie et al. Mobile applications of self-management of diabetes. Agency for Healthcare Research and Quality. 2018.

The Telehealth Research Synthesis Team

Cathy Gurgol
Anum Lakhia
Metti Duressa
Soknorntha Prum
Sindhura Gummi
Adam Bloom-Paicopolos

Heather Edwards
Emily Lazowick
Emily Russell
Jessica McCreary
Holly Ramsawh
Kristin Carman
Greg Martin
Bill Lawrence
Robert Treadway

Questions?

BREAK

We will return at 11:30 am ET

Join the conversation on Twitter via @PCORI

PCORnet: Implementing a Common Linkage Method

Kathleen Troeger, MPH
Chair, Research Transformation Committee

Maryan Zirkle, MD, MS, MA
Senior Program Officer

Identifying a Common Linkage Method in PCORnet
History & Evolution of Linkage

- **August 2016:** Long history of **seeking to obtain more complete data in PCORnet** given the enormous value add when several disparate sources of data can be linked together at the level of an individual participant in a secure and private way, called privacy-preserving record linkage (PPRL)

- **August 2018:** Over time PCORnet community realized the **need for more efficient, privacy-preserving approach to record linkage** in lieu of the many one-off linkages conducted on a project by project basis

- **October 2018:**
  - PCORI **Approved FY2019 Commitment Plan**: $10M for infrastructure to support integration and linkage of additional sources of data in PCORnet
  - PCORnet community set out to **identify a Common Linkage Method** (PPRL) that all partners use to perform their linkages

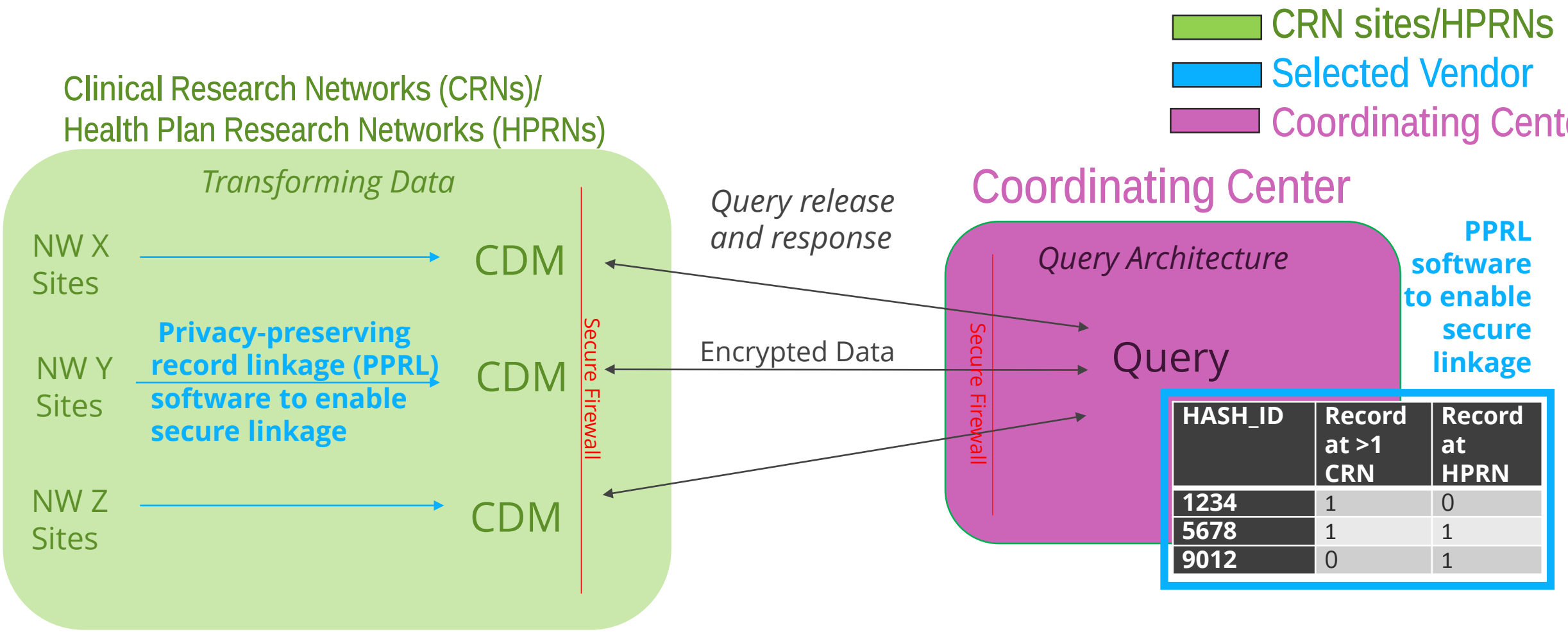
- **December 2018-March 2019:** A robust search and review process **identified a vendor and an approach** to supply the Common Linkage Method for all networks and all projects

- **April 2019:** The PCORI **RTC recommends that the Board approve the use of up to $2.0M in funds** to implement a Common Linkage Method in PCORnet

Implementing a Common Linkage Method in PCORnet:
Who plays a role in implementation of the linkage method and what will each entity do?

Networks/Sites	Selected Vendor	Coordinating Center (CC)
Clinical Research Network (CRN) Sites & Health Plan Research Networks (HPRNs) Expand/amend existing infrastructure: • Common Data Model (CDM) • IRB approvals • Data Sharing/Use Agreement Integration (DSUA)	Contract pending Remote implementation of software: • CRN/HPRNs & DCRI/HPHCI • Enables privacy –preserving record linkage • Functions take place behind each institutions firewall and encrypt all information necessary to ensure privacy Ongoing technical support	Duke (DCRI) & Harvard Pilgrim Healthcare Institute (HPHCI) Expand existing infrastructure and develop *new* approaches: • Queries • Query architecture

Implementing a Common Linkage Method in PCORnet:
Linkage in Action: Overlap Analysis



Implementing a Common Linkage Method in PCORnet: What is the estimated budget?

- **Approved FY2019 Commitment Plan** included the use of $10M to enhance the **integration and linkage of additional sources of data in PCORnet** and/or support network expansion
- **Implementation of a Common Linkage Method** in PCORnet would significantly **increase the capacity to readily link disparate sources of data** (e.g., claims data) for use in future PCORnet studies
- Request approval for **up to $2.0M** in funds to support the activities needed to **implement a Common Linkage Method in PCORnet**

APPROVED
FY2019 PCORnet Commitment Plan

Activity	Amount
FY2019 Health Plans and/or Additional Data Networks	$10.0M
Common Linkage Method Implementation	**(up to $2.0M)**
FY2019 Health Plan and/or Additional Data Networks (Balance)	$8.0M

Board Vote

Call for a Motion to:	• **Approve** up to $2 million to fund infrastructure development for implementation of a common linkage method in PCORnet.
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

Dissemination & Implementation of Findings from PCORI-Funded Research

Personalizing Treatment: Realizing the Benefits of Diabetes Risk Prediction

Jean Slutsky, PA, MSPH

Chief Engagement and Dissemination Officer

John Cuddeback, MD, PhD

Chief Medical Informatics Officer
American Medical Group Association

PCORI's Mandate

"The purpose of the Institute is to assist patients, clinicians, purchasers, and policy-makers in making informed health decisions by advancing the quality and relevance of evidence concerning the manner in which diseases, disorders, and other health conditions can effectively and appropriately be prevented, diagnosed, treated, monitored, and managed ***through research*** *and evidence synthesis...*

... ***and the dissemination of research findings*** *with respect to the relative health outcomes, clinical effectiveness, and appropriateness of the medical treatments, services..."*

— from PCORI's authorizing legislation

PCORI's Dissemination & Implementation Program

- The D&I Program is charged with heightening awareness of the results of PCORI-funded research, and with **advancing efforts to put these findings into practice to improve healthcare delivery and health outcomes**.

  - Transparent reporting and public release of findings
  - Increasing awareness of these findings among the "right people"
  - Promoting systems for doctors, patients, and others to use the evidence to help with real life decisions and improve care

Snapshot of Funded D&I Awards

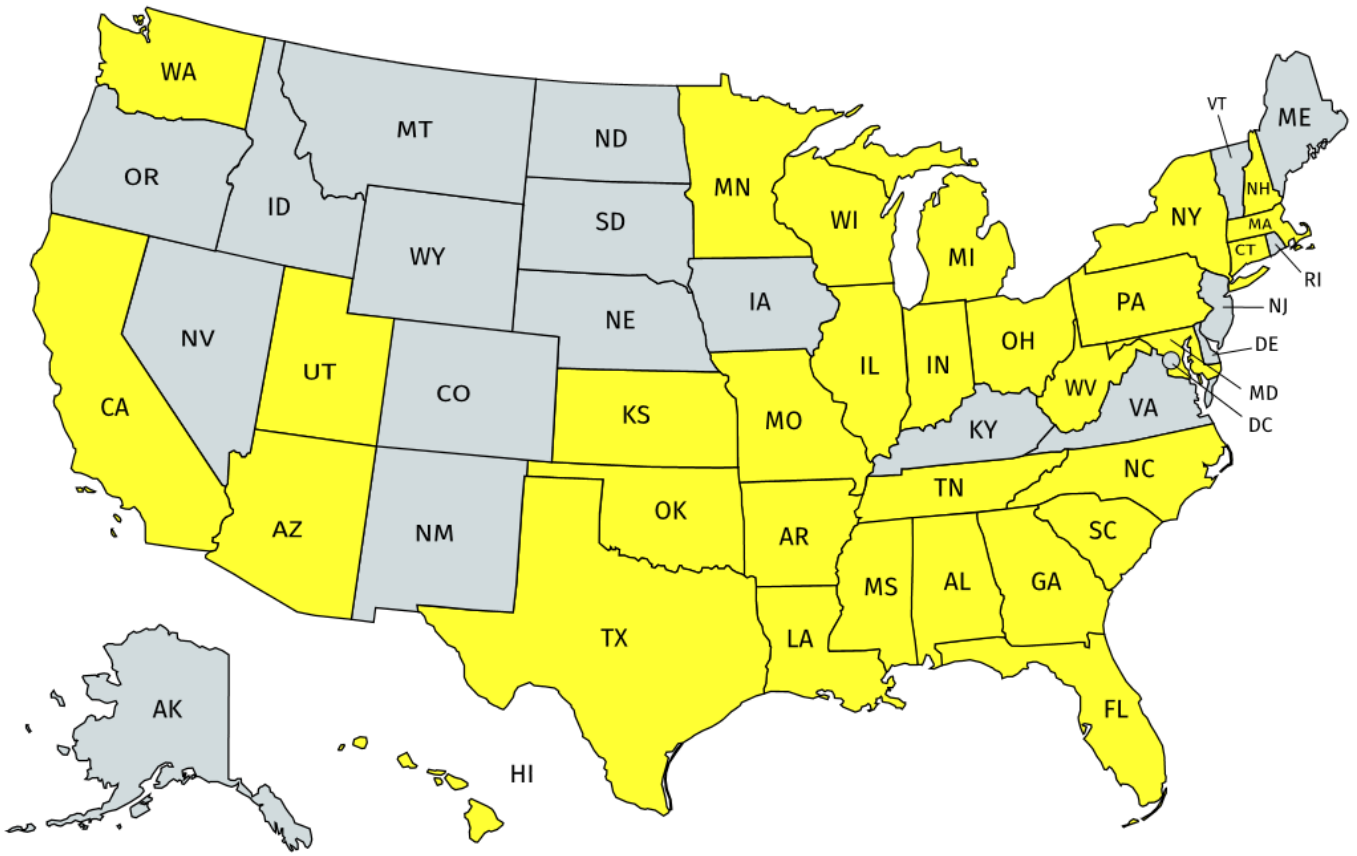
Completed Funding Cycles: 10

Funded Awards: 25

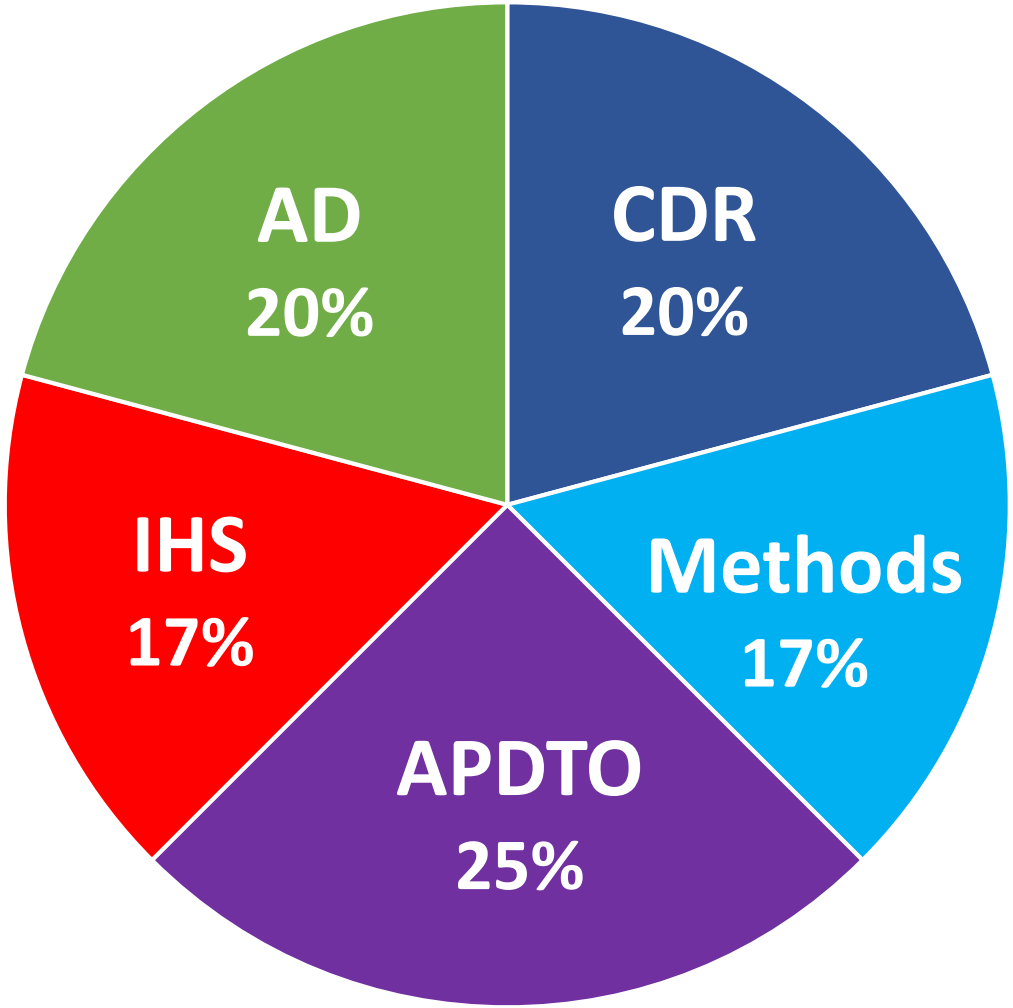
Total PCORI investment: $29M

Project budgets: $500K to $2.2M

Implementation Sites: in **32 states**



D&I Awards by Priority Area for the Original PCORI-Funded Research



- Communication and Dissemination Research
- Addressing Disparities
- Methods/Pilots
- Assessment, Prevention, Diagnosis, and Treatment Options
- Improving Healthcare Systems

PCORI D&I Program Funding Initiatives

Goal: To facilitate the uptake and integration of evidence from PCORI-funded studies into real-world practice, in the context of other relevant evidence

- **Limited Competition: Implementation of PCORI-Funded PCOR Results PFA**
  - Provides PCORI investigator teams the opportunity to propose next steps to put their findings into practice (up to $1M in direct costs per project)
- **Implementation of Effective Shared Decision Making Approaches in Practice Settings PFA**
  - Promotes the implementation and systematic uptake of shared decision making in practice settings (up to $1.5M in total costs per project)
- **Implementation of Findings from PCORI's Major Research Investments PFA**
  - Provides a broad applicant pool the opportunity to propose strategies to put evidence from specific, high-priority PCORI initiatives into practice, in the context of related evidence (up to $2.5M in total costs per project)

Improving Diabetes Prevention Based on Predicted Benefits of Treatment
(PI: David Kent, MD; Tufts Medical Center)

PCORI STUDY:

- Analyzed individual patient data from 32 studies, including the 2002 Diabetes Prevention Program (DPP) Study, to see how treatments affected different groups of people.

thebmj

BMJ 2015;350:h454 doi: 10.1136/bmj.h454 (Published 19 February 2015) Page 1 of 10

RESEARCH

Improving diabetes prevention with benefit based tailored treatment: risk based reanalysis of Diabetes Prevention Program

OPEN ACCESS

Jeremy B Sussman *research scientist*[1] *assistant professor*[2], David M Kent *professor of medicine and director*[3], Jason P Nelson *statistician*[3], Rodney A Hayward *professor of medicine*[1 2 4]

[1]Department of Veterans Affairs Center for Clinical Management Research, Ann Arbor, MI 48109-2800, USA; [2]Division of General Internal Medicine, University of Michigan, NCRC, 2800 Plymouth Road, Building 16/343E, Ann Arbor; [3]Predictive Analytics and Comparative Effectiveness Center, Tufts Medical Center, 35 Kneeland Street, Boston, MA 02111, USA; [4]RWJ Foundation Clinical Scholars Program, University of Michigan, 2800 Plymouth Road, NCRC B10-G016, Ann Arbor

Improving Diabetes Prevention Based on Predicted Benefits of Treatment
(PI: David Kent, MD; Tufts Medical Center)

Pre-diabetes affects ~84M people in the United States.

In practice-based screening, for every person with a result in the diabetes range, 6 people are identified with pre-diabetes.

STUDY FOUND:

- Risk of developing diabetes varied dramatically across patients.
- Low-risk patients showed little benefit from intensive lifestyle modification or taking metformin; high-risk patients showed significant benefit from these interventions.

D&I PROJECT: (in progress)

- Incorporate the prediction model into clinical workflow in the EHR, so it can be used in shared decisions
- Partner with the American Medical Group Association to implement the EHR tool in 50 clinic sites at Mercy (St. Louis) and Premier Medical Associates (Pittsburgh)

Personalized Treatment: Realizing the Benefits of Diabetes Risk Prediction

John Cuddeback, MD, PhD

Project Partner

Chief Medical Informatics Officer
American Medical Group Association (AMGA)

Personalized Treatment: Realizing the Benefits of Diabetes Risk Prediction

Tufts Medical Center

Predictive Analytics and Comparative Effectiveness (PACE) Center

Boston

David Kent, MD, MS
Director, PACE Center
Prof. of Medicine, Neurology, Clinical & Translational Sci.
Director, Graduate Program in Clinical & Translational Sci.

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Carolyn Koenig, MD
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Med. Dir., Care Management
Mercy East Community

Todd Stewart, MD
VP, Clinical Integrated Solutions
Clinical Informatics
Mercy Technology Services

AMGA

Alexandria, VA

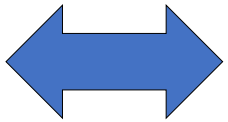
Elizabeth Ciemins, PhD, MPH
Sr. Director, Research & Analytics

Jill Powelson, RN, DrPH, MBA
Sr. Director, Clinical Translation

Richard Stempniewicz
Chief Technology Officer

American Medical Group Association

Advocate



Educate
Innovate

Volume → Value

Align payment incentives
with population health

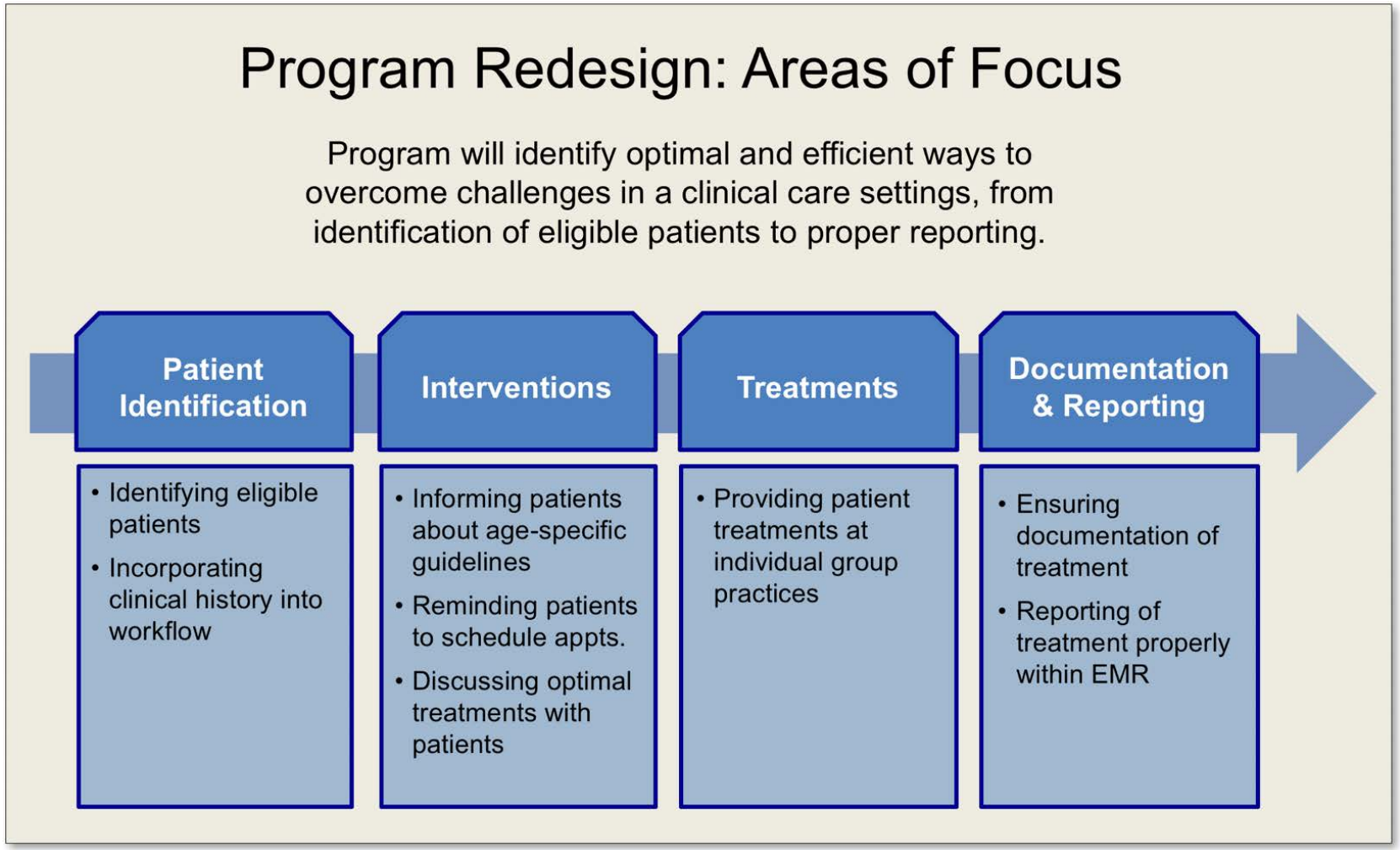
Help members redesign
their delivery systems
to manage population health

AMGA – 501(c)(6) non-profit trade association

AMGA Foundation – 501(c)(3)

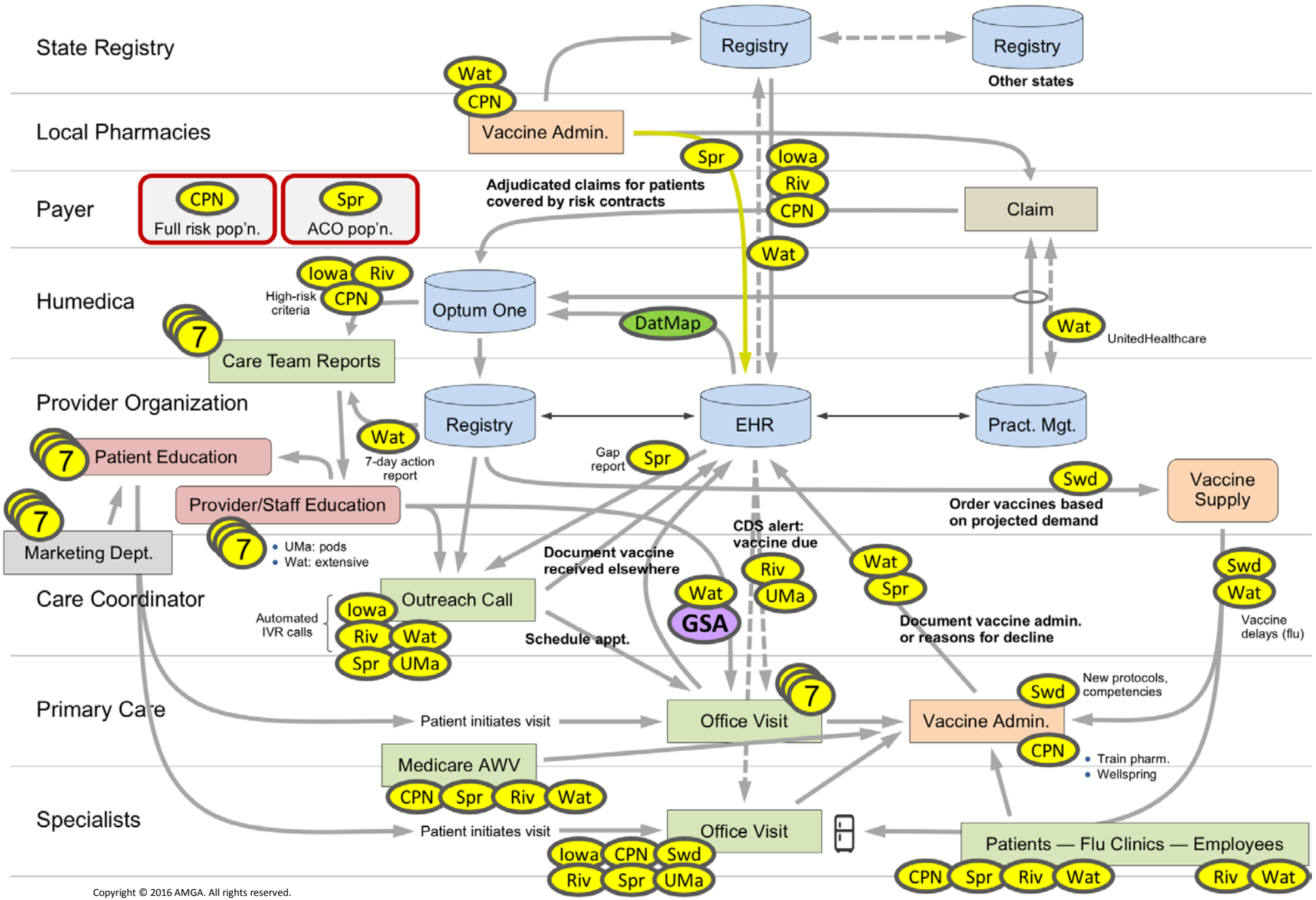
Some AMGA Members

Translation Is a Simple, Linear Process (Not!)

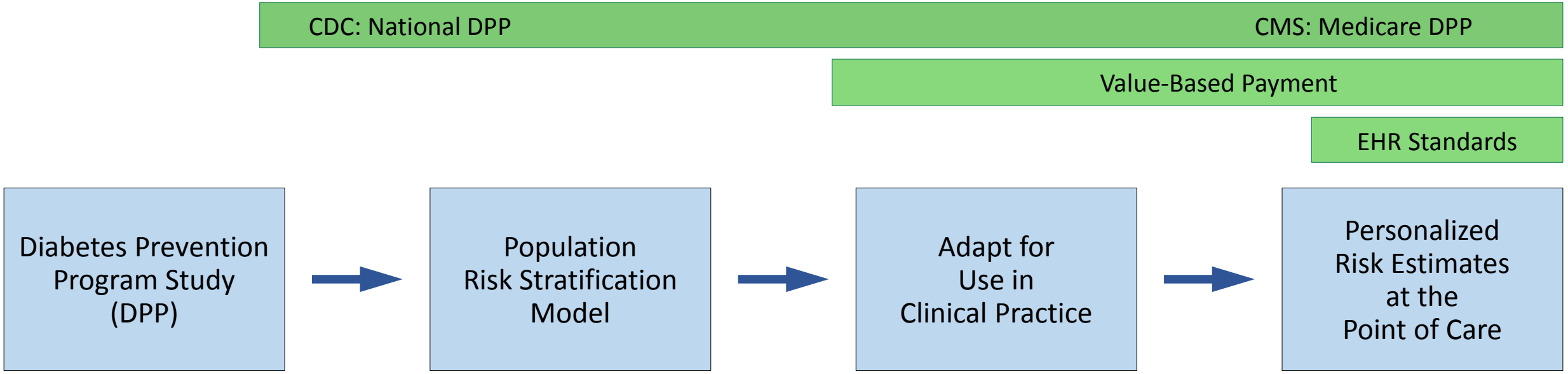


Immunization Data/Process Flow

version 0.98
12/8/2015



Overview



Landmark clinical trial, 1996–2001

3,234 adults with BMI ≥ 24 and impaired fasting glucose

Incidence of diabetes at 3 years:

- Placebo 29%
- Taking metformin 22
- Intensive lifestyle 14

PCORI methods study: Heterogeneity of treatment effect

David Kent, PI – Tufts PACE Center

Reanalyzed 32 clinical trials—can we determine, using data available at the beginning of the trial, which patients will experience the greatest benefit?

PCORI D&I study: patient and provider focus groups

- Would this be useful?
- How to present the data?

Redevelop model for real-world EHR data, at OptumLabs

- Missing data
- Fasting blood glucose values not labeled as fasting

Clinical decision support (CDS) features of EHRs—designed for simple conditional logic

Emerging EHR interoperability standards enable cloud-hosted predictive models

Strong business case – avert or delay onset of type 2 diabetes

AMGA Foundation: National Diabetes Campaign

Together2Goal®

Improve care for
1 million people with
type 2 diabetes
by 2021

AMGA Foundation: National Diabetes Campaign

Together2Goal®

Results after Year 2

763,000 patients, aged 18–79, with improved care

- 2/3 with net improvement in control on campaign measures
- 1/3 have new Dx—identified through screening

319,000 additional patients with sustained control on the bundle measure

Year 2 ended 2018 Q1

AMGA Foundation: National Diabetes Campaign

Together 2 Goal Launch: Member Survey

Which planks will you adopt?

Which will you not adopt?

31% said they *wouldn't* focus on screening.

They are already overwhelmed by
the number of patients with type 2 diabetes

...let alone prediabetes!

What Is Prediabetes?

Elevated blood sugar, but not high enough to indicate diabetes

- Fasting plasma glucose 100–125 mg/dL
- HbA1c 5.7–6.4 percent
- Oral glucose tolerance test, 2 hr 140–199 mg/dL

Elevated risk of progression to diabetes over 3 years—from DPP study

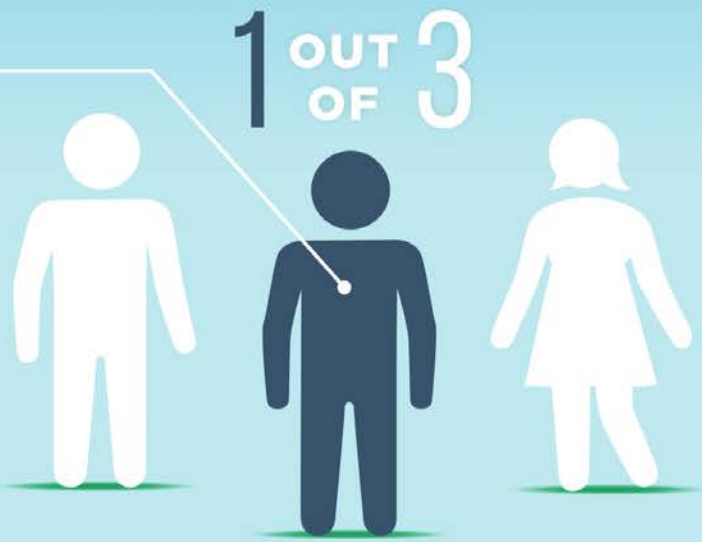
- No intervention (placebo) 28.9% over 3 years

PREDIABETES

COULD IT BE YOU?

84.1 MILLION

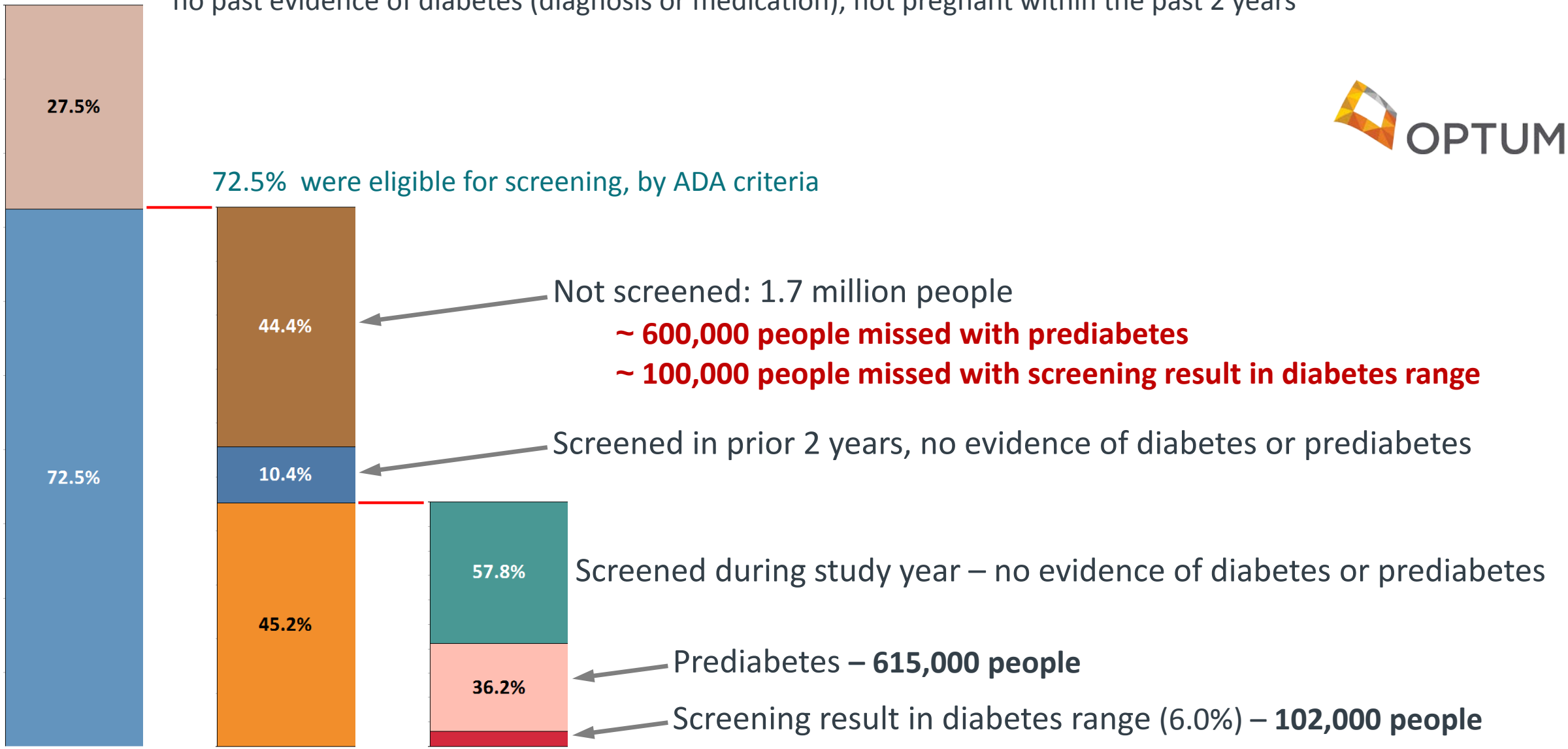
84.1 million American adults — more than 1 out of 3 — have prediabetes



9 OUT OF 10 people with prediabetes don't know they have it

5.1 million patients, 18–75, across 23 AMGA member organizations using Optum population health analytics

≥ 1 ambulatory visit with a PCP, endocrinologist, cardiologist, or nephrologist, July 2016 – June 2017, no past evidence of diabetes (diagnosis or medication), not pregnant within the past 2 years



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• Fasting plasma glucose	100–125 mg/dL
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Elevated risk of progression to diabetes over 3 years—from DPP study

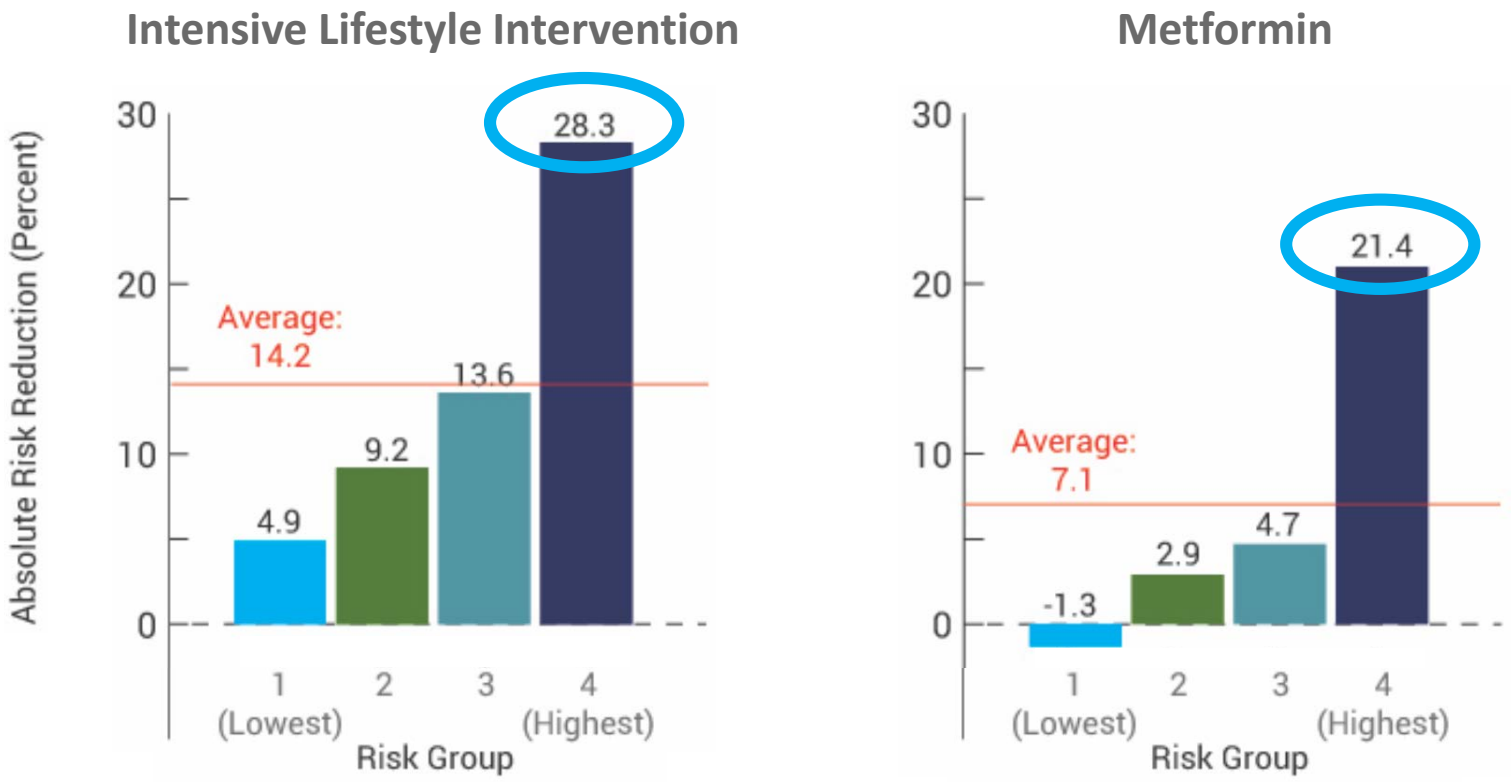
• No intervention (placebo)	28.9% over 3 years
• Taking metformin	**absolute risk reduction: 7.1%**
• Intensive lifestyle intervention	**absolute risk reduction: 14.2%**

Heterogeneity of Treatment Effect

Quartiles of Risk

Overall risk		29%
4	High	45%
3	Moderate	20
2	Moderate	12
1	Low	7

Absolute Risk Reduction Seen in DPP Study



http://www.pcori.org/research-in-action/moving-beyond-averages

Will a Predictive Model Be Useful?

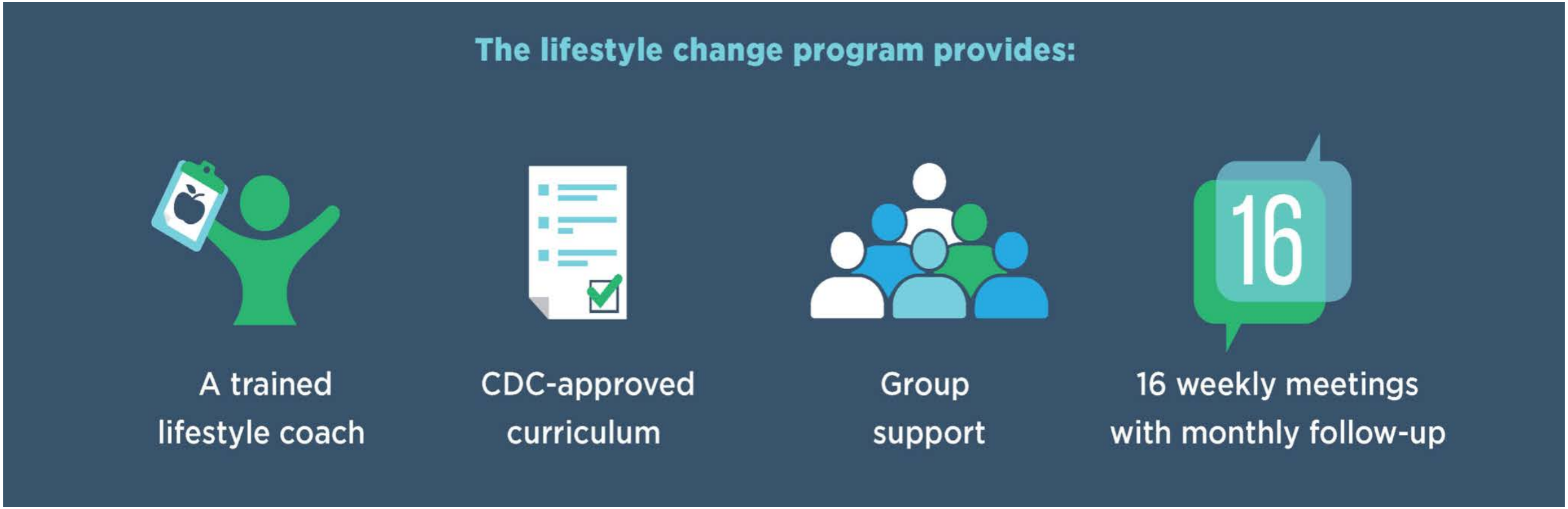
Patient focus groups

- Most had family members with type 2 diabetes
- Intensely interested in their own personal risk for developing diabetes
- Quoted the ages at which family members were diagnosed

Provider focus groups

- Want to support/encourage patients—especially for lifestyle program
- Feel overwhelmed—need to prioritize

DPP Lifestyle Intervention



Supervised physical activity sessions
Individualized—"toolbox" of adherence strategies
Materials and strategies tailored to address ethnic diversity

Goal is 7% weight loss
Motivational techniques, "restarts"
Medicare now pays for the program

Will a Predictive Model Be Useful?

Patient focus groups

- Most had family members with type 2 diabetes
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- Quoted the ages at which family members were diagnosed

Provider focus groups

- Want to support/encourage patients—especially for lifestyle program
- Feel overwhelmed—need to prioritize
- Multi-variable model is a better predictor than any individual parameter
  - In the lowest-risk quartile, about 15% of patients have A1c ≥ 6.0
  - In the highest-risk quartile, more than 25% of patients have A1c < 6.0

Are the Data Elements Available in the EHR?

Model from DPP Study Data

- HbA1c
- Fasting glucose
- Triglycerides
- History of elevated glucose
- Height
- Waist circumference
- Waist:hip ratio

Adapted Model for Use in EHR

- HbA1c
- Fasting glucose
- Triglycerides
- Age
- Gender
- Race
- BMI
- Smoking status
- Systolic blood pressure
- Hypertension diagnosis
- HDL cholesterol (“good cholesterol”)

Both have pre-diabetes. Who is at greatest risk for diabetes?

- 38-year-old female
- BMI 34
- HbA1c 5.8
- Systolic BP 153
- HDL 28
- Smoker?

HIGH RISK

- 44-year-old male
- BMI 22
- HbA1c 6.3
- Systolic BP 121
- HDL 100
- Former Smoker

LOW RISK

Predictive model results as displayed in EHR at Premier Medical Associates

Interpretation: Low Risk Patient

Predicted Risk of Type 2 Diabetes at 3 Years	Treatment	Relative Risk Reduction (RRR)	Number Needed to Treat (NNT)
5.47 %	Usual Care	Reference	N/A
4.38 %	Metformin	20%	91.4
2.30 %	DPP Lifestyle	58%	31.5

Add to Chart

Interpretation: High Risk Patient

Predicted Risk of Type 2 Diabetes at 3 Years	Treatment	Relative Risk Reduction (RRR)	Number Needed to Treat (NNT)
55.7 %	Usual Care	Reference	N/A
24.5 %	Metformin	56%	4
23.4 %	DPP Lifestyle	58%	4

Add to Chart

PREMIER MEDICAL ASSOCIATES

Premier Medical Associates: May 2018 – Jan 2019

Of the 670 high-risk patients…

11 were On Metformin before 5/1/2018 (6%)

134 were Started on Metformin after 5/1/2018 (19%)

0 were Referred to a DPP before 5/1/2018 (0%)

297 were Referred to a DPP after 5/1/2018 (44%)

Premier Medical Associates: May 2018 – Jan 2019

Risk Level	Action Taken
High	65%
Moderate	18%
Low	4%

During the project, 87 patients were identified as having diabetes, through timely screening

Potential for Cost Savings

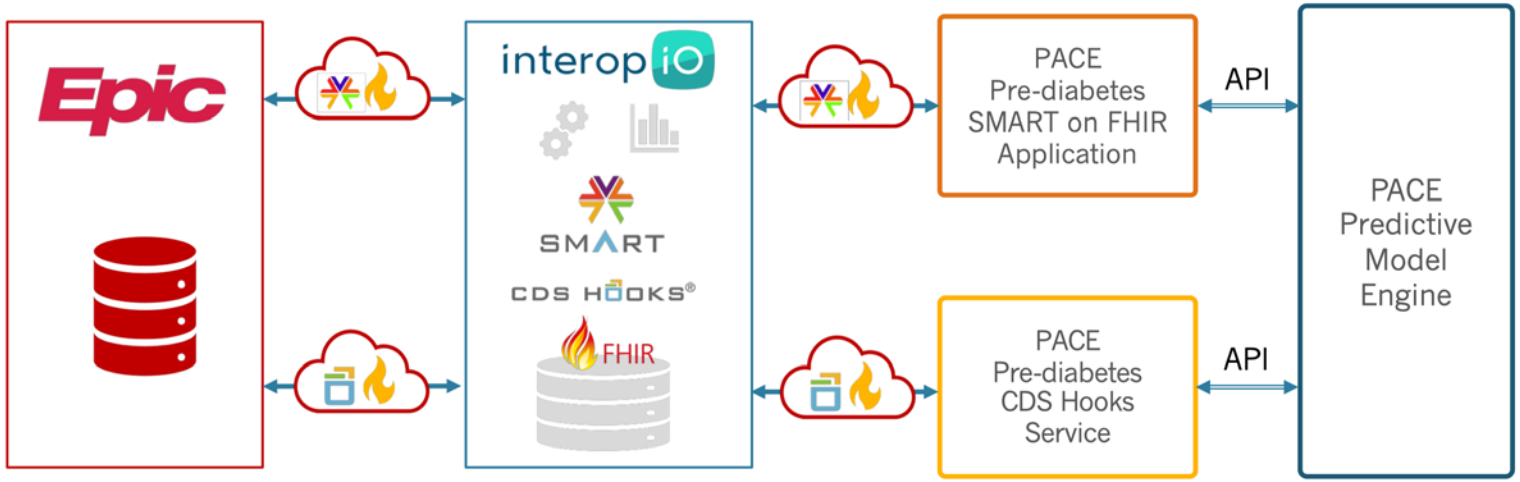
- Intermountain insurance plan saves $3,500 per person per year that development of diabetes is averted or delayed
  - $17,500 if diabetes is delayed for 5 years
- CMS Office of the Actuary estimates a savings of $2,650 over 15 months for Medicare beneficiaries participating in a DPP lifestyle program
- Cost of a DPP lifestyle program ~$600

https://www.ama-assn.org/delivering-care/diabetes/intermountains-prediabetes-effort-signs-10000-plus-patients

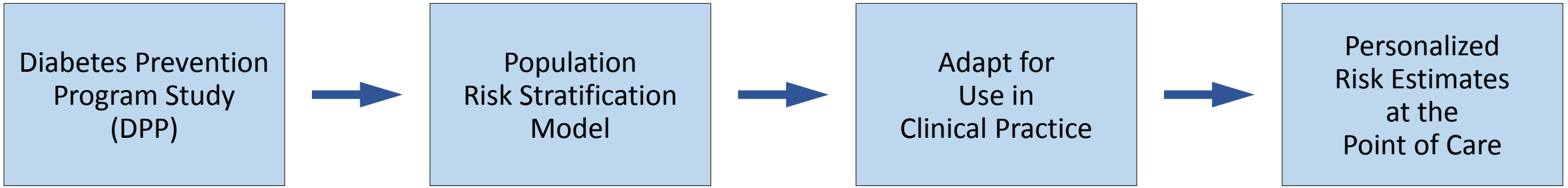
Khan T, Tsipas S, Wozniak G. Medical Care Expenditures for Individuals with Prediabetes: The Potential Cost Savings in Reducing the Risk of Developing Diabetes. *Population Health Management.* 2017;*20*(5), 389-396. DOI: 10.1089/pop.2016.0134

Personalized Risk Estimates at the Point of Care

- Premier Medical Associates: Calculator add-in for Allscripts EHR
- Mercy
  - Preliminary build using EHR's native clinical decision support logic—manual data entry
  - Alternative to building and validating full model in EHR—cloud-hosted version, using open interoperability standards



Personalized Risk Estimates at the Point of Care



Precision medicine without waiting for genomics

Public Comment Period

Kristin Carman, MA, PhD

Director, Public and Patient Engagement

Wrap Up and Adjournment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