

# Welcome and Introductions

**Joe Selby, MD, MPH,  
Executive Director**

March 25, 2016



PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE

# About Us

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- An independent research institute authorized by Congress in 2010 and governed by a 21-member Board of Governors representing the entire healthcare community
- Funds comparative clinical effectiveness research (CER) that engages patients and other stakeholders throughout the research process
- Seeks answers to real-world questions about what works best for patients based on their circumstances and concerns



# PCORI's Research Agenda is Driven by Stakeholders' Needs

“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...**”

The Institute shall **identify national priorities** for research, taking into account factors of disease incidence, prevalence, and burden in the United States (with emphasis on chronic conditions), **gaps in evidence in terms of clinical outcomes, practice variations and health disparities in terms of delivery and outcomes of care**, the potential for new evidence to improve patient health, well-being, and the quality of care...



# Engagement as a Path to Useful, High-Quality Research

**Topic Selection  
and Research  
Prioritization**



**Proposal Review;  
Design and  
Conduct of Research**



**Evaluation**



**Dissemination  
and Implementation**



# Why Engage?



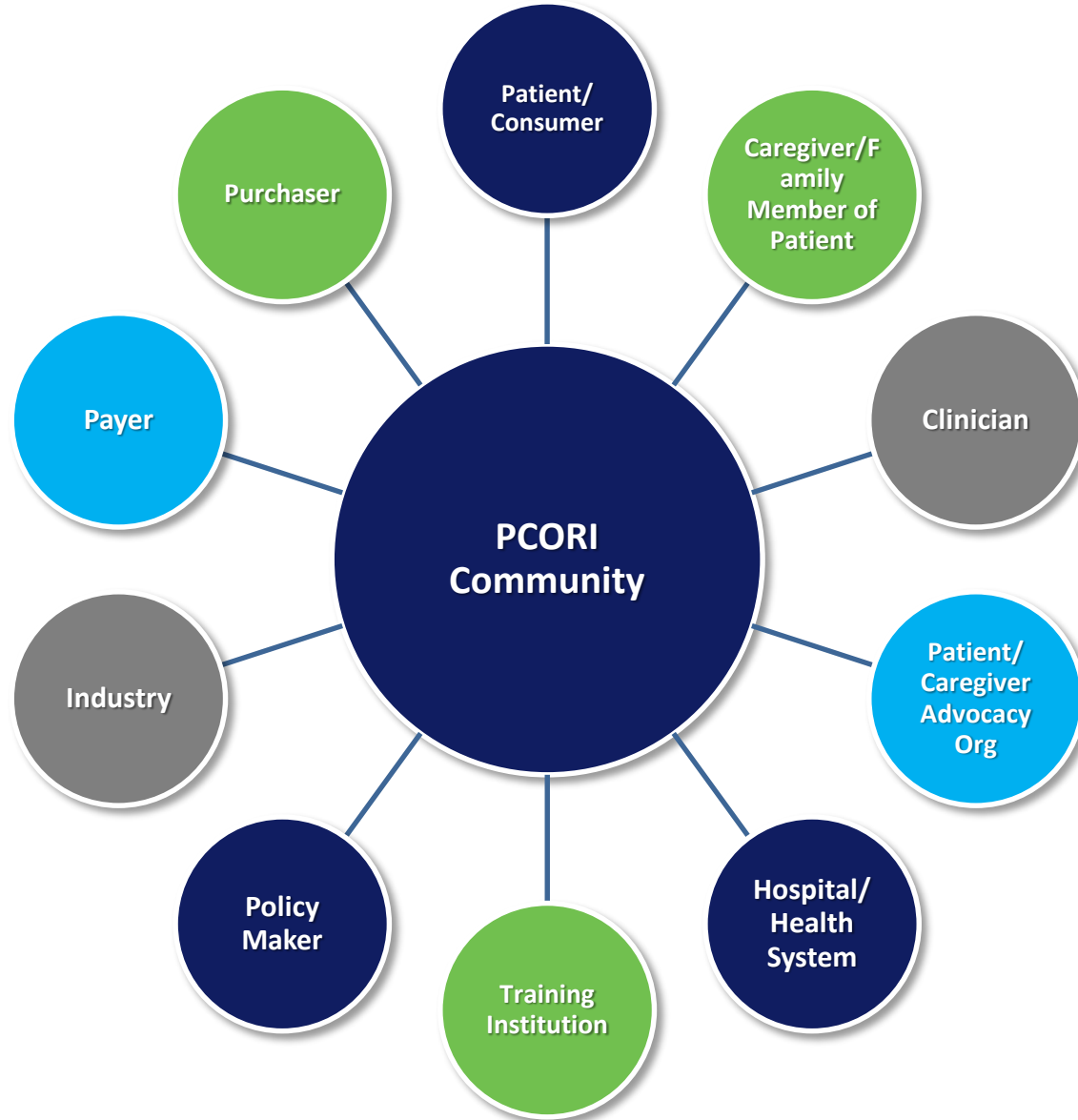
To influence research to be patient-centered, relevant, and useful

To establish trust and a sense of legitimacy in research findings

To encourage successful uptake and use of research results



# Who Are Our Stakeholders?



# Complete List of PCORI's Funded Pragmatic Clinical Studies

Cycle	Title
Fall 2014	Integrating Patient-Centered Exercise Coaching into Primary Care to <b>Reduce Fragility Fracture</b>
Fall 2014	Mobility: Improving Patient-Centered Outcomes Among Overweight and <b>Obese Youth with Bipolar Spectrum Disorders</b> Treated with Second-Generation Antipsychotics
Fall 2014	Pragmatic Randomized Trial of Proton vs. Photon <b>Therapy for</b> Patients with Stage II or III <b>Breast Cancer</b>
Fall 2014	A Practical Intervention to Improve Patient-Centered Outcomes after <b>Hip Fractures</b> Among Older Adults (Regain Trial)
Fall 2014	Anti-TNF Monotherapy versus Combination Therapy with Low Dose Methotrexate in <b>Pediatric Crohn's Disease</b>
Spring 2014 PCS	Early Supported Discharge for Improving <b>Functional Outcomes After Stroke</b>
Spring 2014 PCS	Enabling a Paradigm Shift: A Preference-Tolerant RCT of Personalized vs. Annual <b>Screening for Breast Cancer</b>
Spring 2014 PCS	Pragmatic Trial of More versus Less Intensive Strategies for Active <b>Surveillance of Patients with Small Pulmonary Nodules</b>
Spring 2014 PCS	Targeted interventions to <b>Prevent Chronic Low Back Pain</b> in High Risk Patients: A Multi-Site Pragmatic RCT
Spring 2014 PCS	A Pragmatic Trial to Improve <b>Colony Stimulating Factor Use in Cancer</b>
Winter 2015	<b>Integrating Behavioral Health and Primary Care</b>
Winter 2015	Comparative Effectiveness of <b>Pulmonary Embolism Prevention after Hip and Knee Replacement</b> (PEPPER): Balancing Safety and Effectiveness
Winter 2015	Comparing <b>Outcomes of Drugs and Appendectomy</b> (CODA)
Winter 2015	Integrated Versus Referral Care for <b>Complex Psychiatric Disorders in Rural FQHCs</b>



# PCORI's Funded Targeted Studies

Award Date	Topic	PFA Title	Number of Projects
12/17/2013	<b>Asthma</b>	Treatment Options for African Americans and Hispanics/Latinos with Uncontrolled Asthma	8
6/4/2014	<b>Fall Prevention</b>	Clinical Trial of a Multifactorial Fall Injury Prevention Strategy in Older Persons	1 <i>MOU with NIH</i>
9/30/2014	<b>Fibroids</b>	PCOR for Treatment Options in Uterine Fibroids: Developing a Prospective Multi-Center Practice-based Clinical Registry (P50)	1 <i>MOU with AHRQ</i>
9/30/2014	<b>Care Transitions</b>	The Effectiveness of Transitional Care	1
9/30/2014	<b>Obesity Treatment</b>	Obesity Treatment Options Set in Primary Care for Underserved Populations	2
5/4/2015	<b>Aspirin (PCORnet)</b>	Optimal Maintenance Aspirin Dose for Patients with Coronary Artery Disease (PCORnet Demo)	1
8/18/2015	<b>Obesity (PCORnet)</b>	Obesity Observational Research Initiative (PCORnet Demo)	2
9/28/2015	<b>Hypertension</b>	Testing Multi-Level Interventions to Improve Blood Pressure Control in Minority Racial/Ethnic, Low Socioeconomic Status, and/or Rural Populations	2 <i>MOU with NIH</i>
9/28/2015	<b>Hepatitis C</b>	Clinical Management of Hepatitis C Infection	2



# How We Select Research Topics: Approach One

- **Investigator-Initiated Approach**
  - Aligned with our national priorities
  - Topic identified by research team in collaboration with stakeholders
  - PCORI's first funding stream
    - 321 studies in progress; \$554M awarded to-date

Assessment of  
Prevention, Diagnosis,  
and Treatment Options

Communication and  
Dissemination Research

Improving Health  
Systems

Addressing Disparities

Accelerating Patient-  
Centered Outcomes  
Research and  
Methodological  
Research

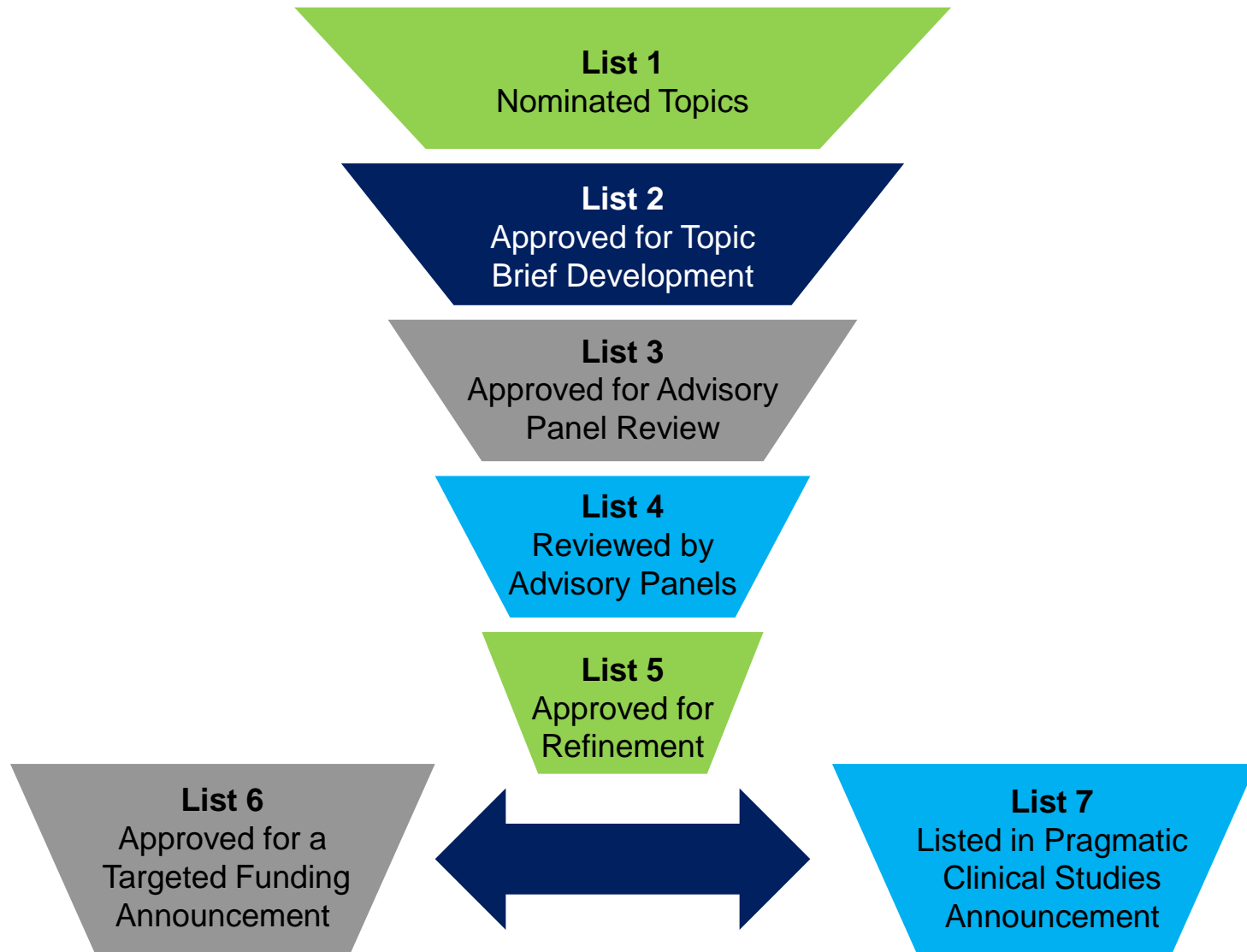


# How We Select Research Topics: Approach Two

- **Patient- and Other Stakeholder- Initiated Approach**
  - Designed for targeted PCORI Funding Announcements (PFAs) and priority topics in Pragmatic Clinical Studies (PCS) PFAs
  - Allows us to focus dedicated resources on high-priority topics
  - Topics submitted to PCORI directly from patients and other stakeholders
    - Approximately 2,000 topics submitted to-date
    - Pathway and topic status available on our website
  - 20 targeted studies on 8 topics; \$183M awarded
  - 14 PCS studies; \$177M awarded



# Topic Prioritization Pathway



# Topic Prioritization Pathway: January 2016

## Under Consideration

### Endorsed for Refinement (List 5)

- Asthma
- Chronic low back pain
- Community-acquired pneumonia
- Diabetes
- Mental health integration
- Palliative care
- Decreasing opioid initiation for chronic pain
- Sickle cell disease

### Reviewed by Advisory Panels (List 4)

- Antimicrobial resistance
- Adolescents and alcohol abuse
- Autism Spectrum Disorders- risk assessment
- Care coordination
- Cognitive Impairment
- Communication
- Coronary artery disease- statins
- Dementia
- Genetic testing for rare disease
- Glaucoma
- Health IT & evidence-based treatment
- High cholesterol/PCSK9 inhibitors
- Implantable cardiac defibrillators
- Links btw providers & community
- Neck pain
- NOACs for stroke prevention
- Orthopedic surgery
- Post acute care transitions
- Role of spacers in asthma
- Weight maintenance & reduction

## Approved Topics

### Targeted PFA Released (List 6)

- Chronic pain/Long term opioid therapy
- Multiple sclerosis
- NOACs for blood clots
- Treatment-resistant depression

### Priority Topics for Pragmatic Clinical Studies PFA (List 7)

- Autism Spectrum Disorders- behavioral analysis
- Care transitions
- Chronic pain management
- Dental caries
- Diabetes
- End-stage renal disease
- Medication management
- Migraine
- Osteoarthritis
- Pelvic floor dysfunction
- Pre-term birth & low birth weight
- Substance abuse – Tobacco cessation
- Suicide prevention
- Traumatic brain injury

## Funded Topics

### Funded via Targeted PFA

- CAD-Aspirin dose
- Care transitions
- Falls in elderly
- Hepatitis C- New therapies
- Hypertension
- Obesity in diverse populations
- Severe asthma in African Americans & Hispanics
- Uterine fibroids

### Funded via Pragmatic Clinical Studies PFA

- Bipolar disorder
- Breast ductal carcinoma in situ
- Crohn's disease- Biologics
- Chronic back pain
- Hip fracture
- Mental health & primary care
- Particle beam therapy
- Pulmonary nodules & CT surveillance



# The National Patient-Centered Clinical Research Network (PCORnet)



- Create a large, highly representative, national patient-centered clinical research network, focused on conducting randomized and observational comparative studies.
- Support a learning US healthcare system, allowing large-scale research to be conducted with enhanced accuracy and efficiency within real-world care delivery systems.

## **11 Clinical Data Research Networks (CDRNs)**

- Health system-based networks, such as hospital systems
- \$76.8 million awarded

## **18 Patient-Powered Research Networks (PPRNs)**

- Patients with a single condition form a research network
- \$16.8 million awarded

## **Coordinating Center**

- Provides technical and logistical assistance under the direction of a steering committee and PCORI staff



# Nursing in PCORI's Portfolio

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**Evelyn P. Whitlock, MD, MPH**  
**Chief Science Officer**

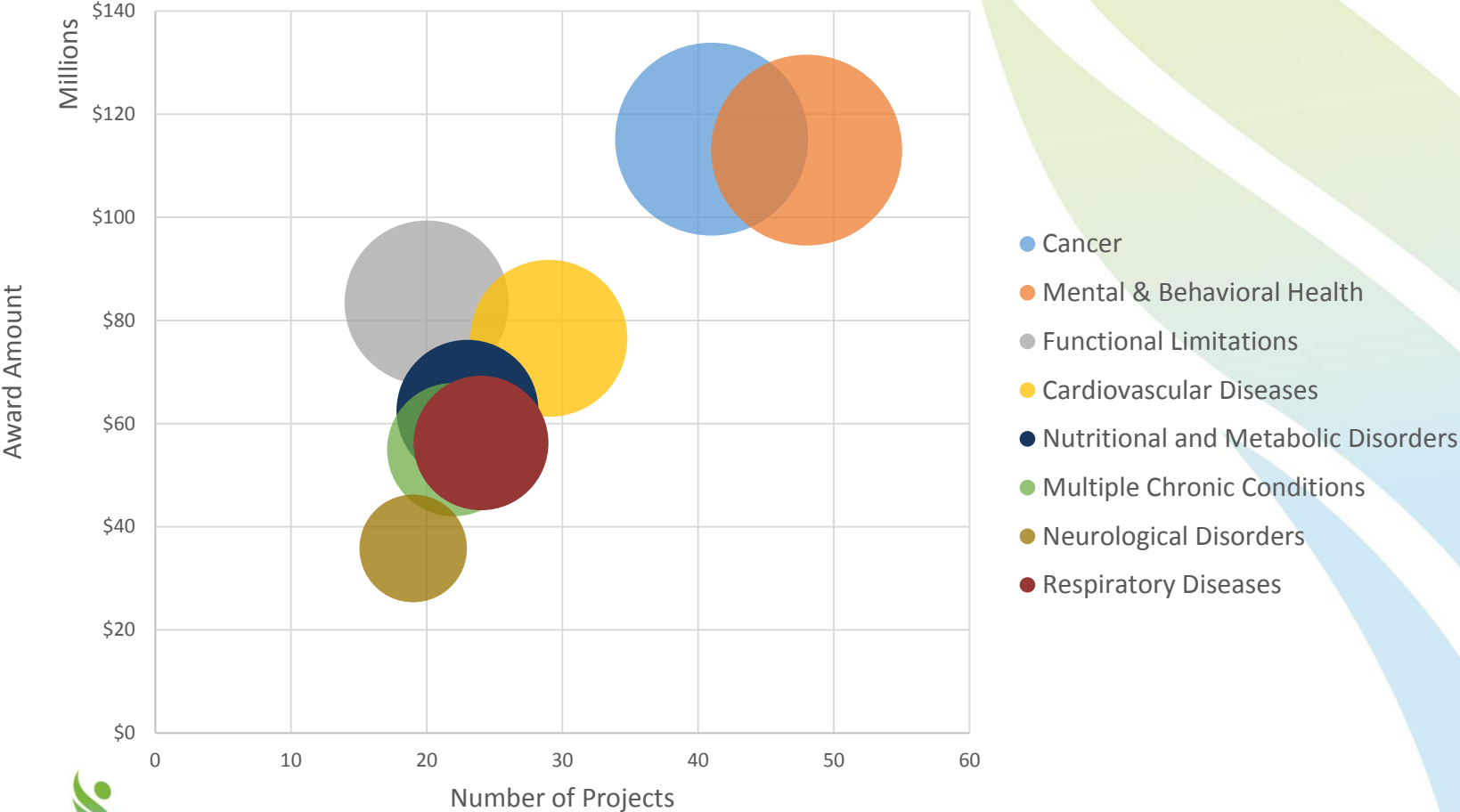
March 25, 2016



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# Most Common Clinical Conditions in PCORI's Portfolio By Number of Projects and Investment

N = 252



# Nursing Through the Years



# Overview

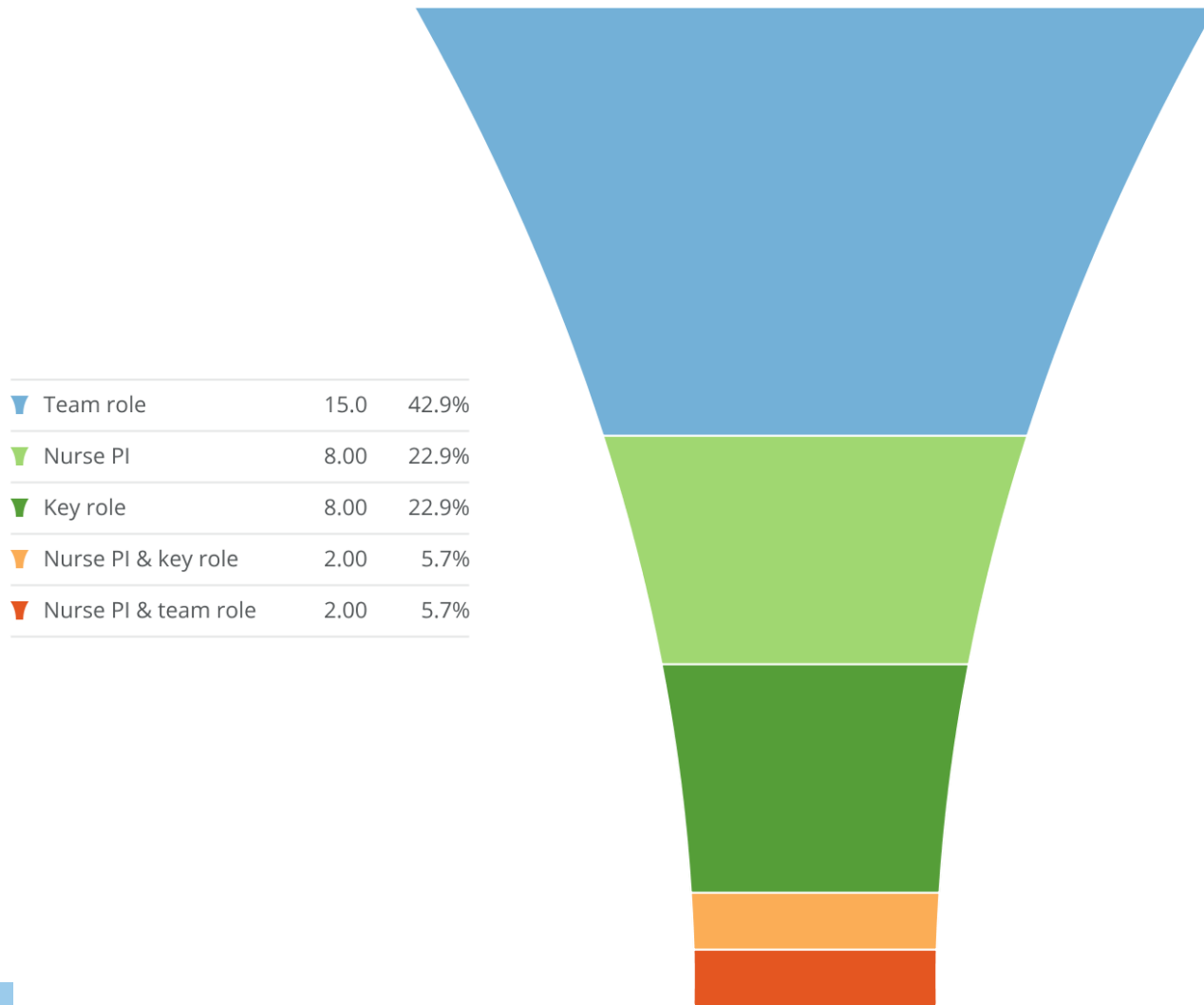
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**PCORI's portfolio includes 35 projects with explicit roles for nurses, based on research plans. Roles are categorized as the following\***

- Nurse as Principal Investigator
- Nurse(s) in key roles in the projects, such as delivering the care intervention
- Nurse(s) in team roles, such as being named as members of the multidisciplinary care team in projects

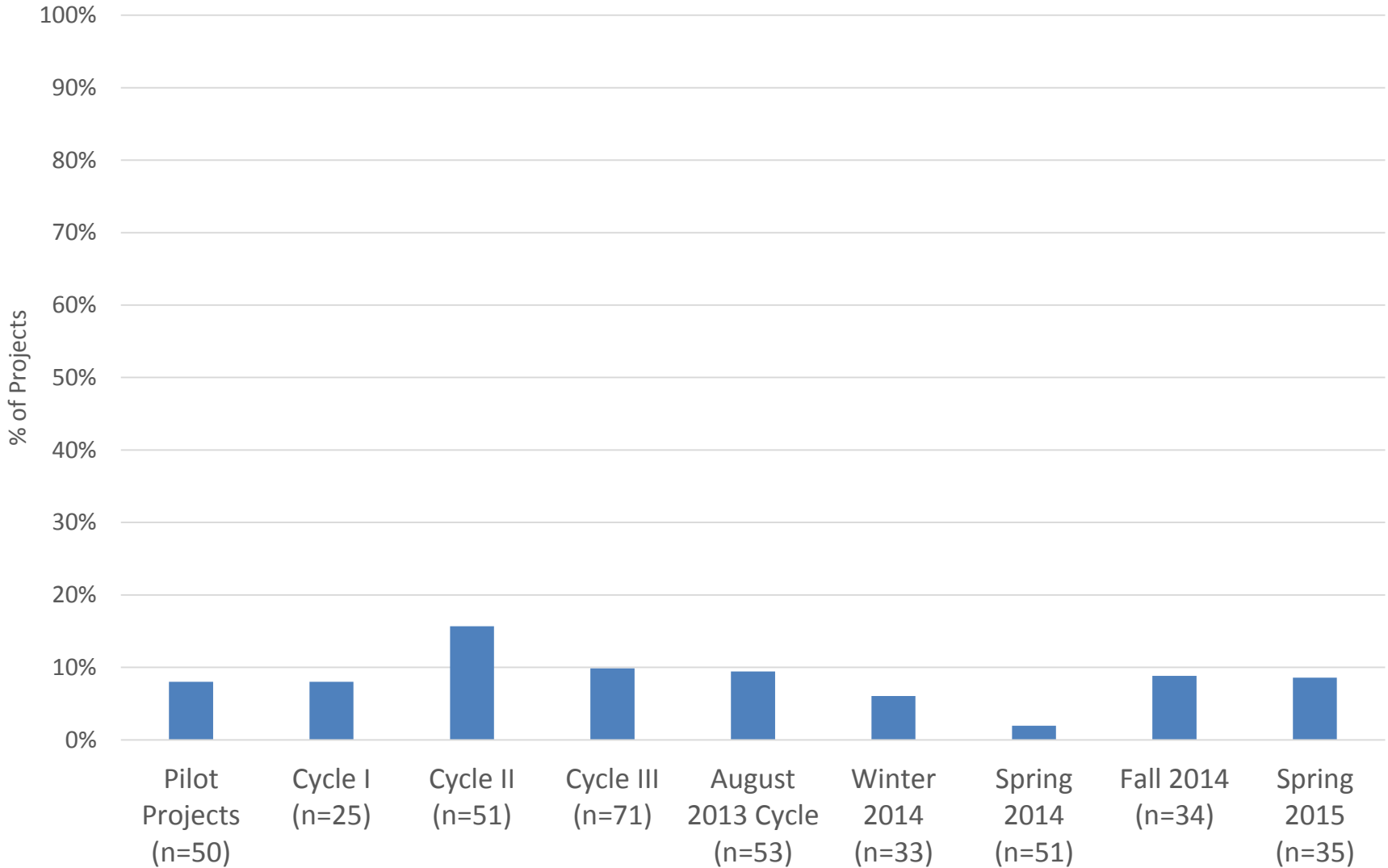


# The most common role for Nurses within the 35 projects is member of a care team.

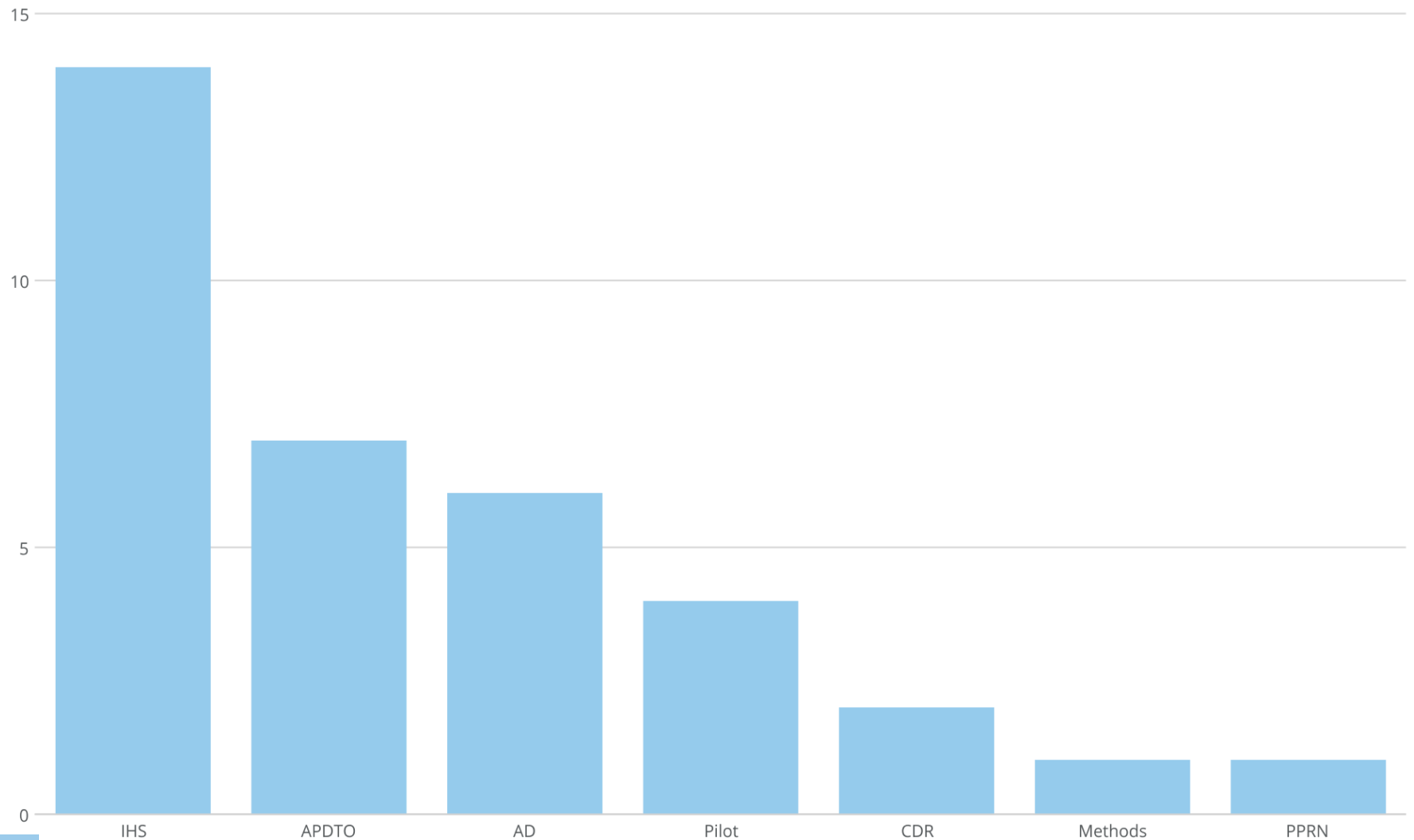


# Nursing Projects Funded Across the Years

Proportion of Nursing Projects per Cycle



# Most Funded Nursing Projects Have Come Through Our Improving Health Systems Funding Announcements





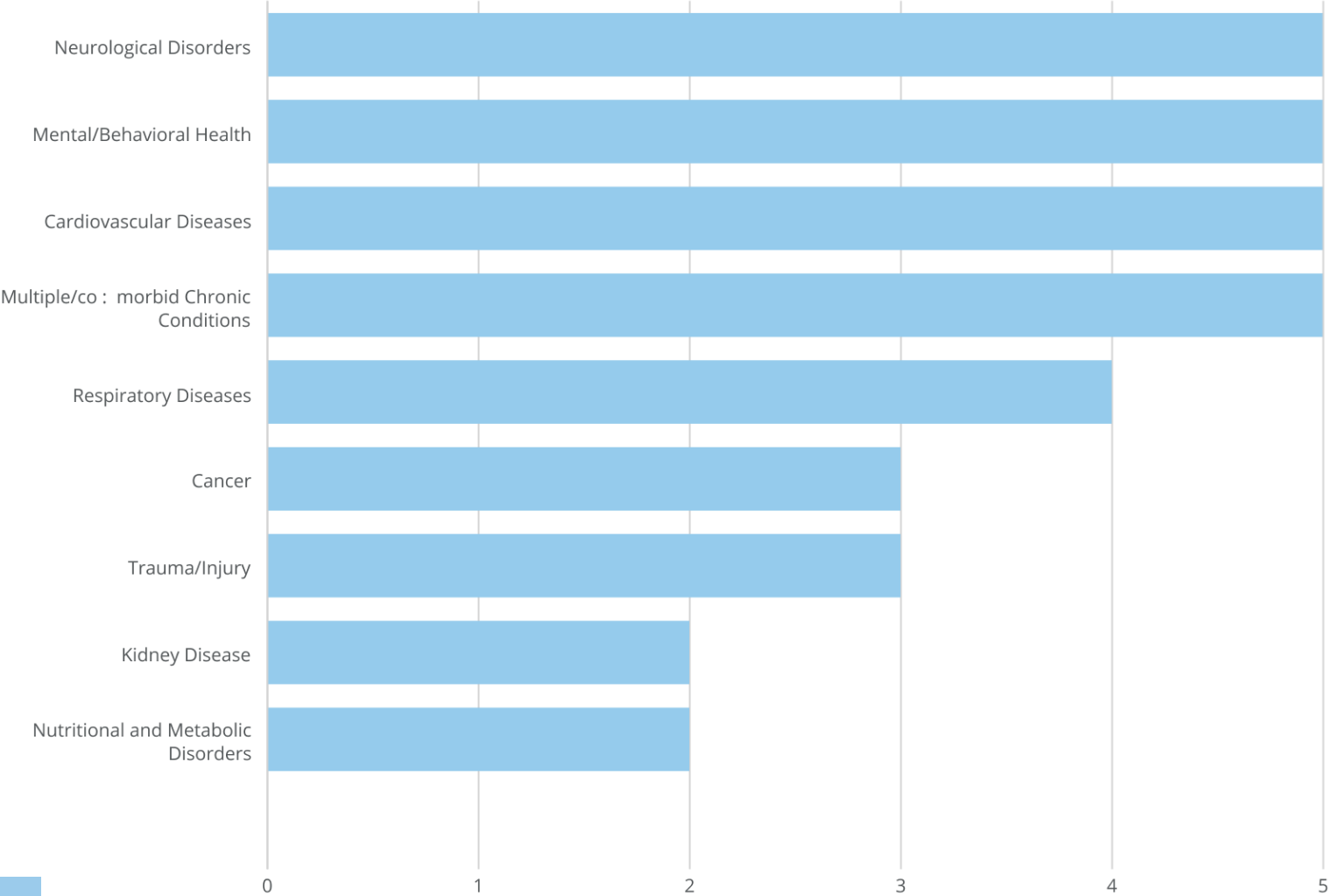
# Overview

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The PCORI taxonomy dataset currently includes 26 of these 35 nursing projects, allowing for more advanced analyses of this subset of projects.

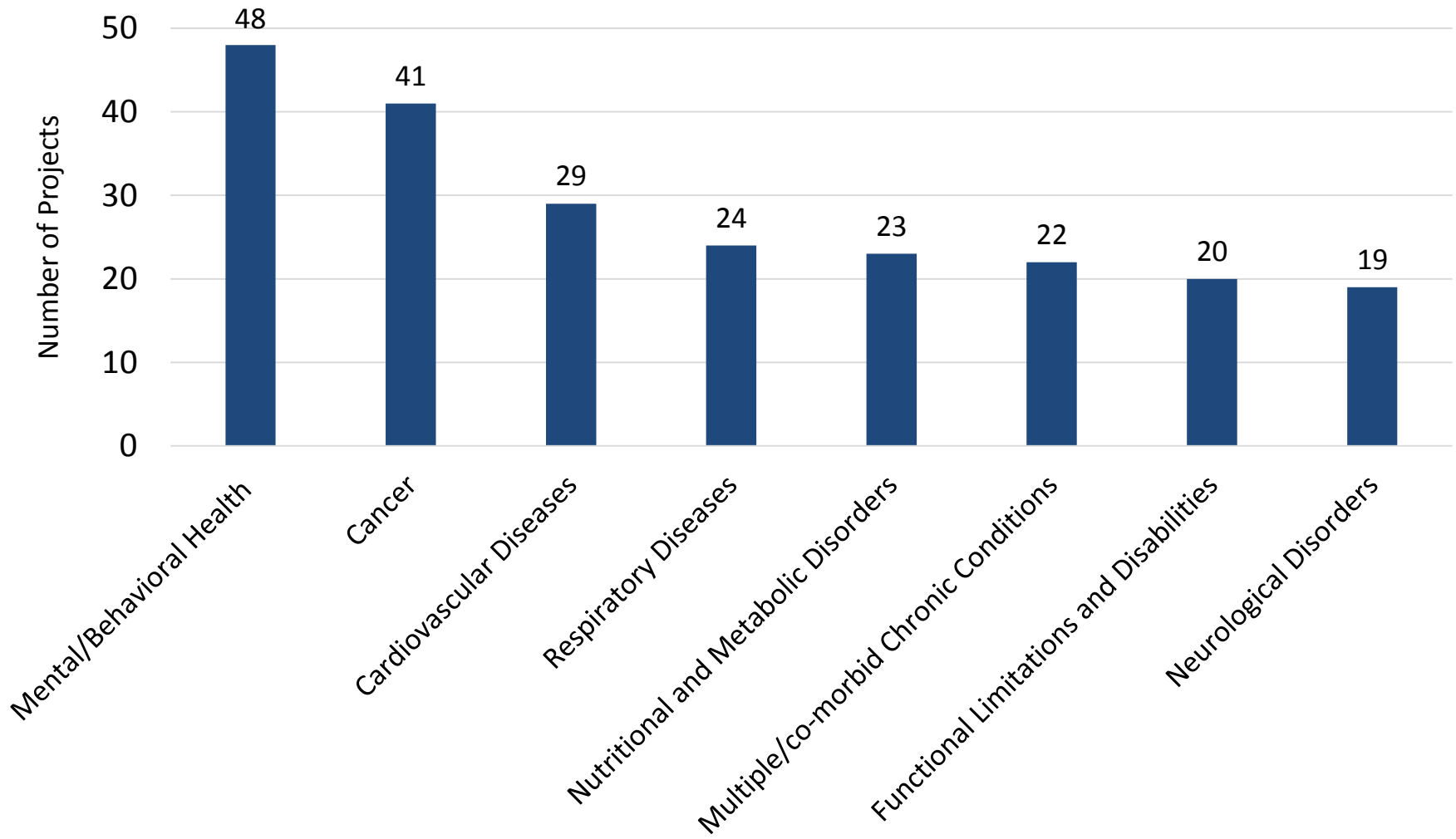


# Clinical Conditions in Nurse-involved Projects

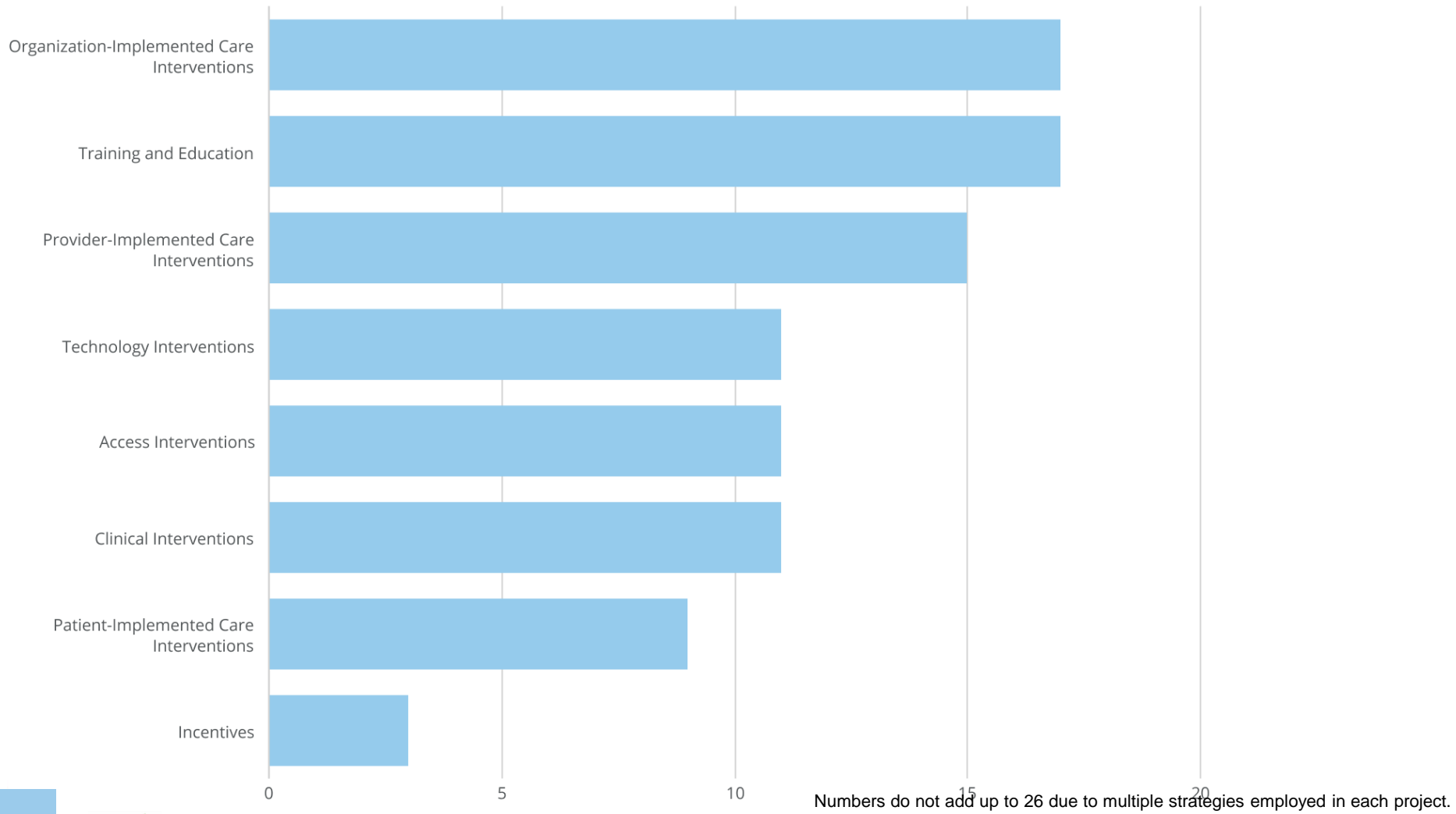


# Most Common Clinical Conditions in PCORI's Portfolio

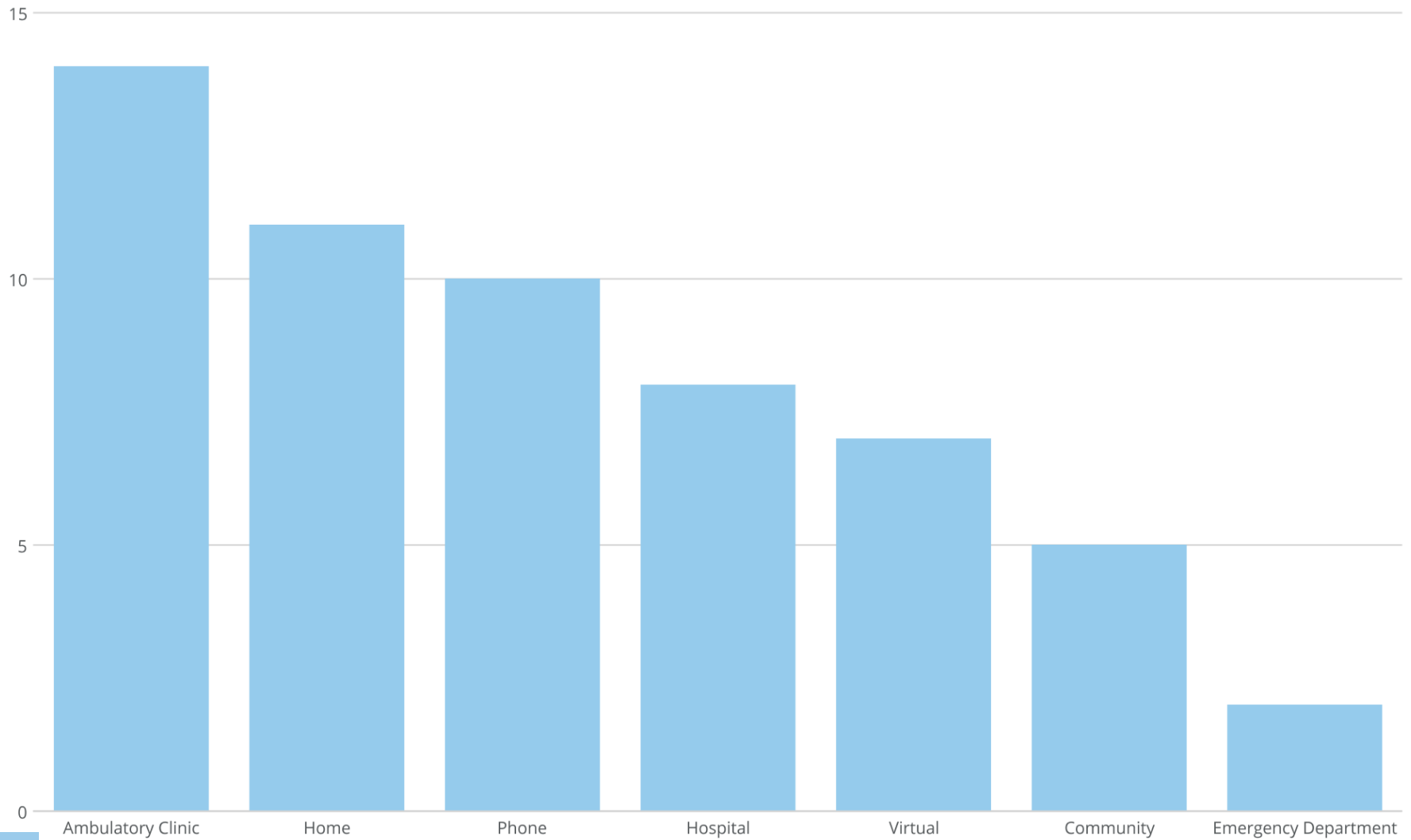
N = 252



# Intervention Strategies

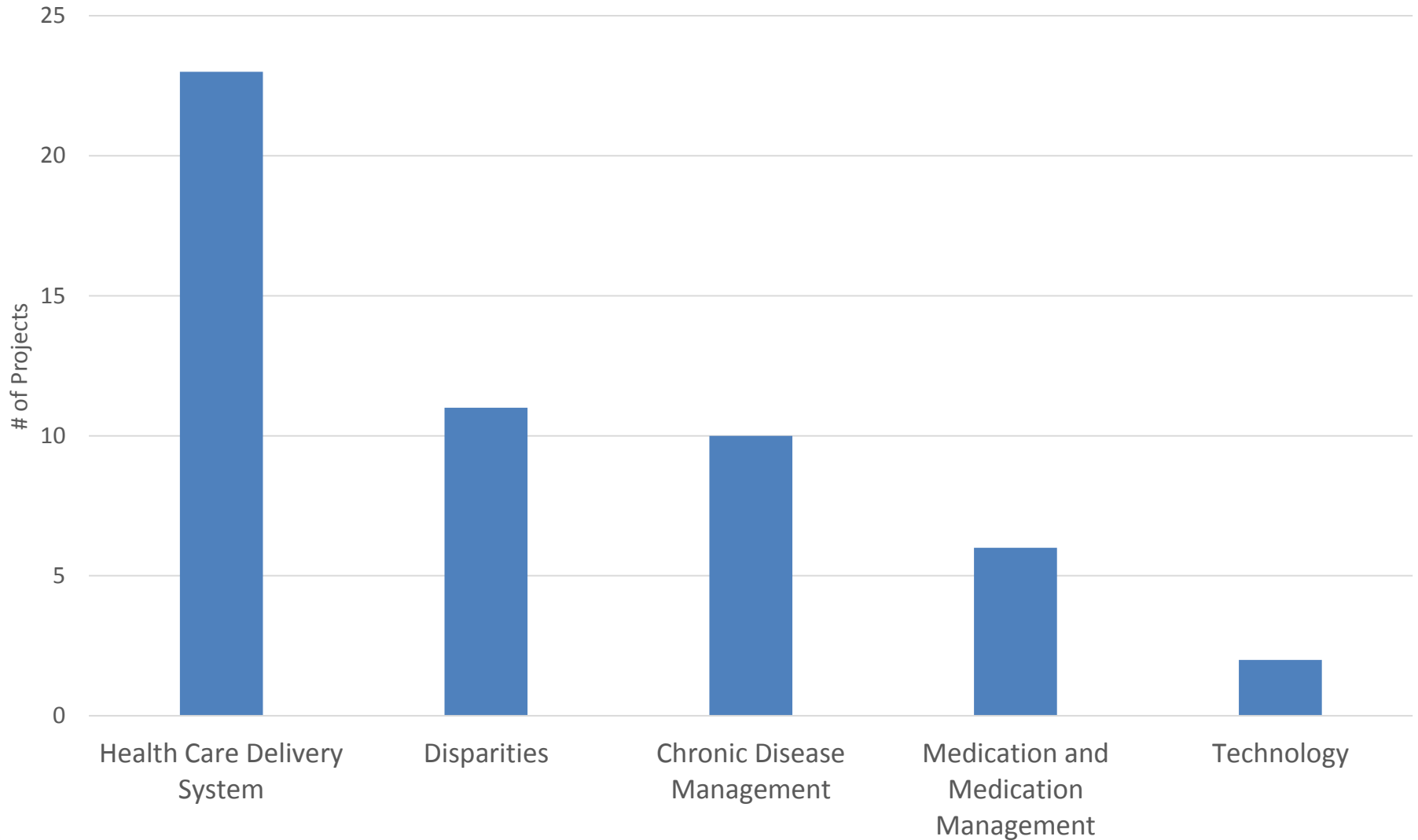


# Intervention Setting



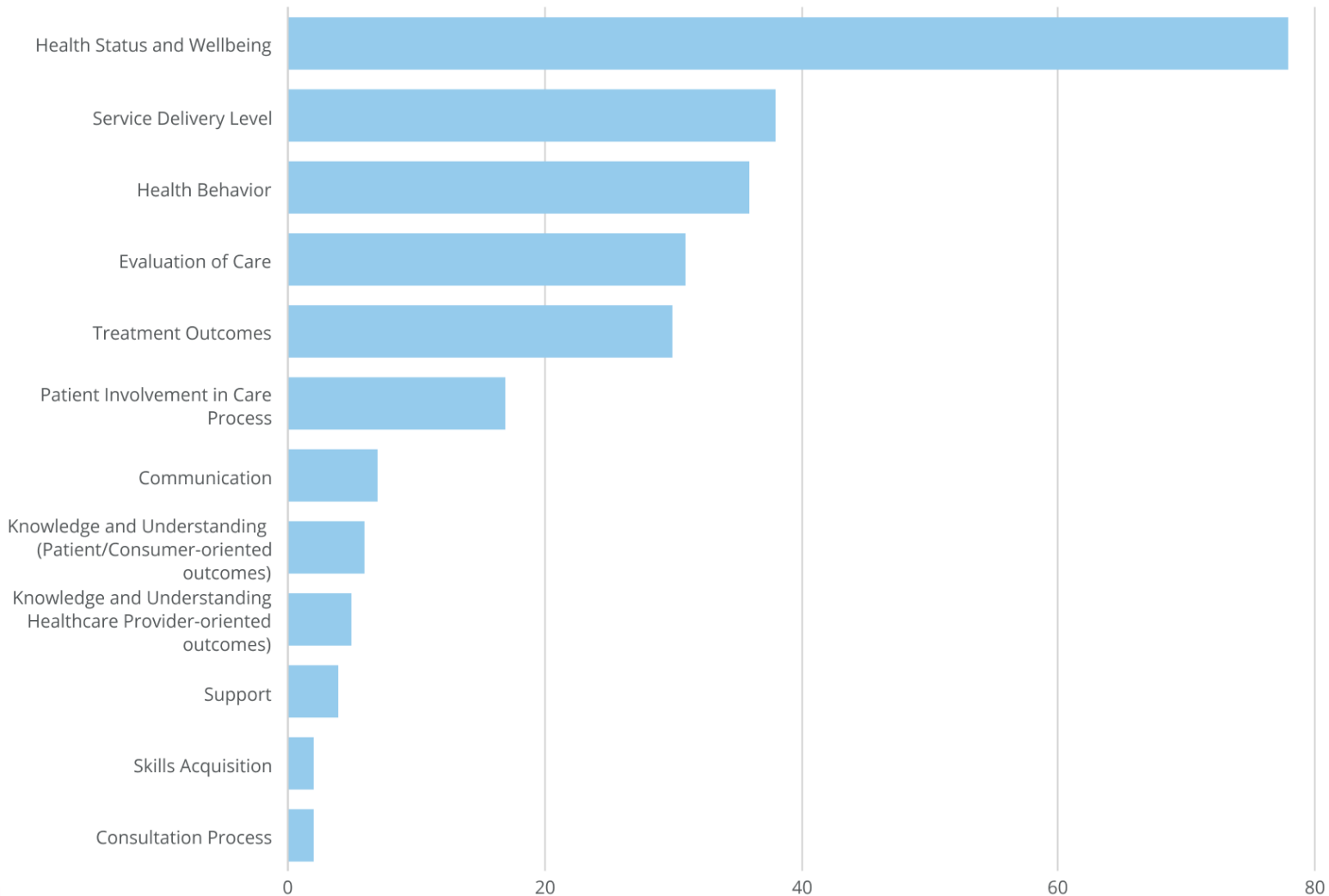
Numbers do not add up to 26 due to multiple settings in each project.

# Health Service Focus



Numbers do not add up to 26 due to multiple health services foci in each project.

# Outcomes Themes



Numbers do not add up to 26 due to multiple outcomes measured in each project.

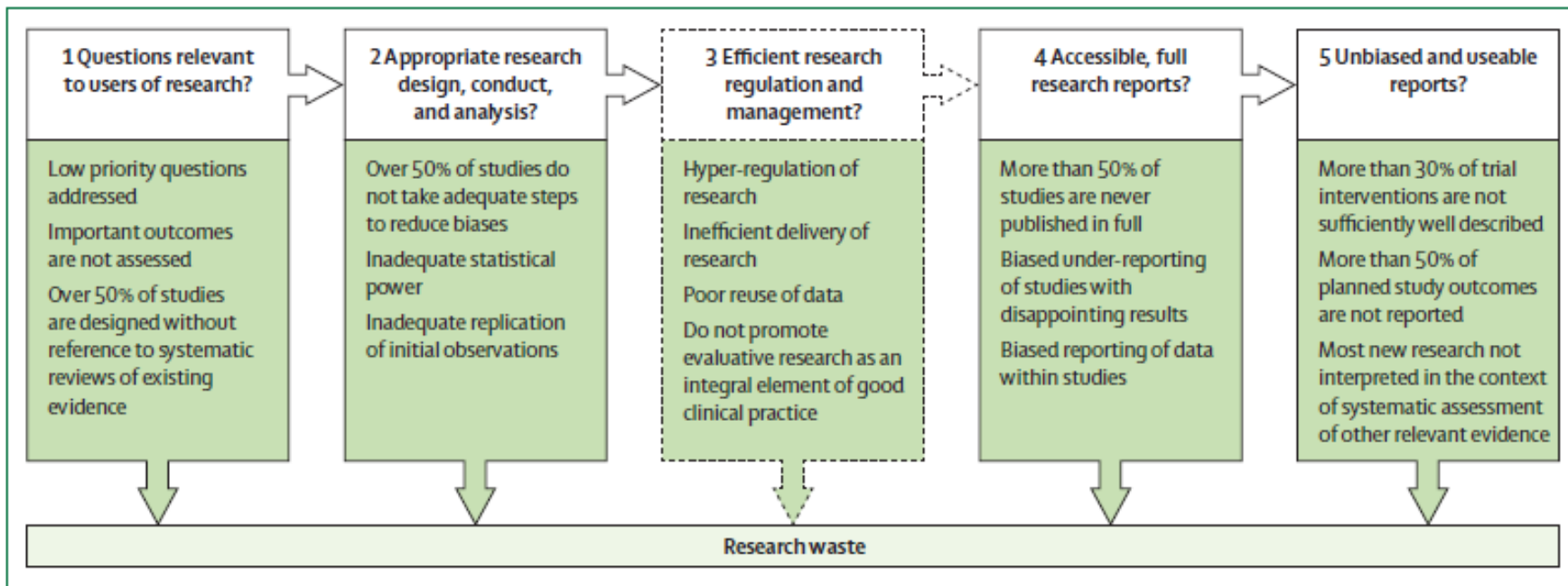
# Looking Forward



# Increasing Value and Reducing Waste

- 2009 estimate: 85% of research funding is avoidably wasted

## Stages in research production that lead to waste. Moher et al.



THE LANCET

Avoidable waste in the production and reporting of research evidence. Chalmers I, Glasziou, P. *Lancet* 2009; **374**: 86-89.

Increasing value and reducing waste in biomedical research: who's listening? Moher D, et al. *Lancet* 2015; **Online**: Sept.28



# PCORI's Approach Aligns

- 17 recommendations to reduce waste and increase value across 5 stages of research production

1. Questions relevant to end users

- Users engaged
- In-process research and processes online

2. Appropriate design, conduct and analysis

- Methodology Standards and training
- COI policies

3. Efficient research regulation and management

- Efficiency of recruitment and retention
- Human subjects protections matched to risks

4. Accessible, full reports

- Peer review and public availability of full reports

5. Unbiased and useable reports

- Infrastructure to support good reporting and archiving



# Areas for Future Scientific Focus

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- **Learning from What We've Done**
  - Engagement in the research enterprise
- **Making Additional Targeted Investments**
  - Stakeholder-driven research priorities
  - Achieving a “critical mass” of funded research
  - Continued emphasis on reducing disparities
- **Investigating Supports for the clinical encounter**
  - Effective communication of evidence (including reducing low-value care)
  - Systems support for all clinician activities and patient encounters
  - Appropriately rigorous methodologies in clinical research
- **Launching our Evidence synthesis portfolio**
  - Targeted approaches/differences in treatment response
    - Individual-participant data meta-analyses, predictive analytics in trials
  - Confirmation and contextualization of research results



# Thank You



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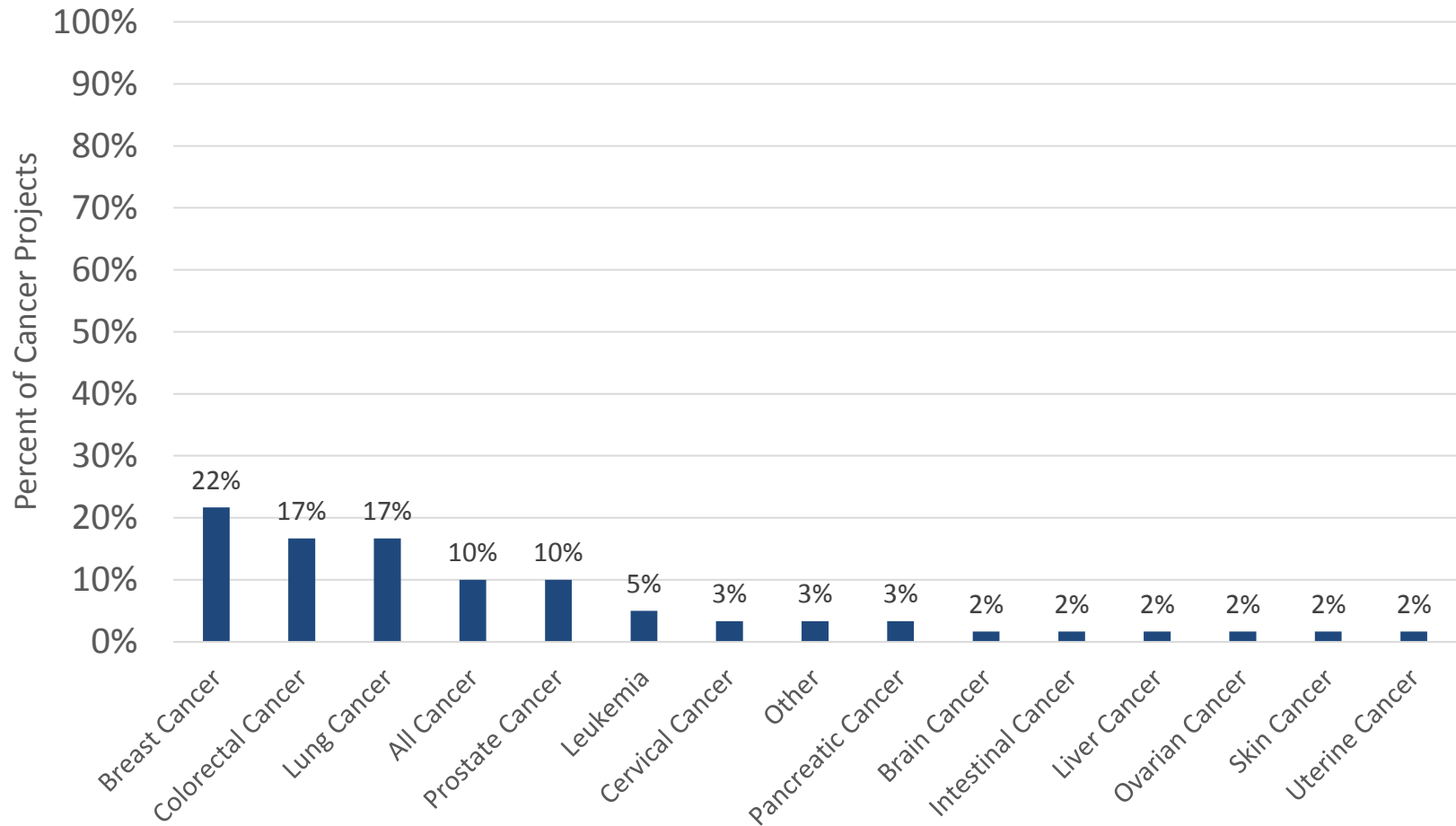
# Appendix

## Clinical Sub-conditions in the PCORI Portfolio



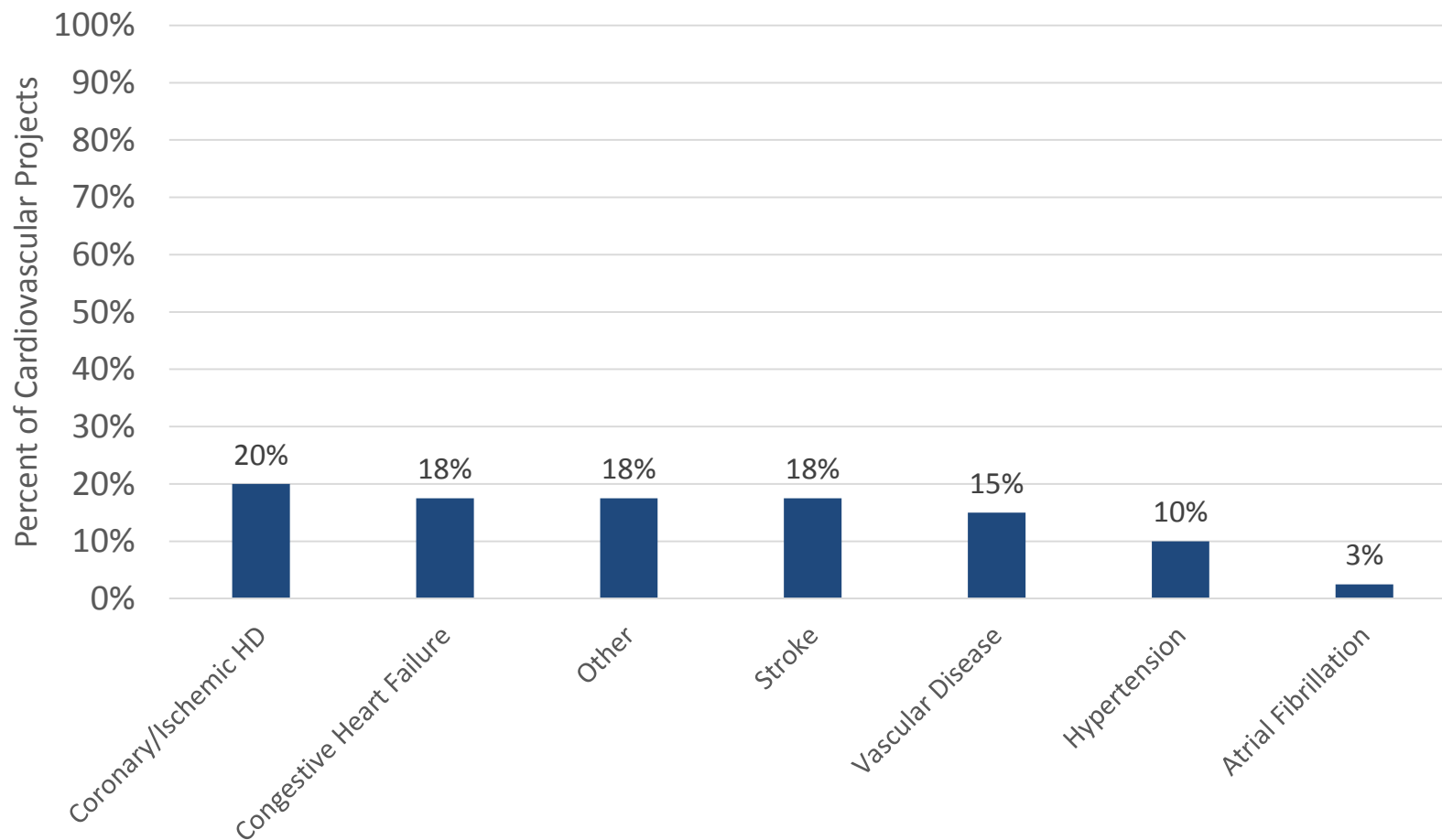
# Cancer

N = 60



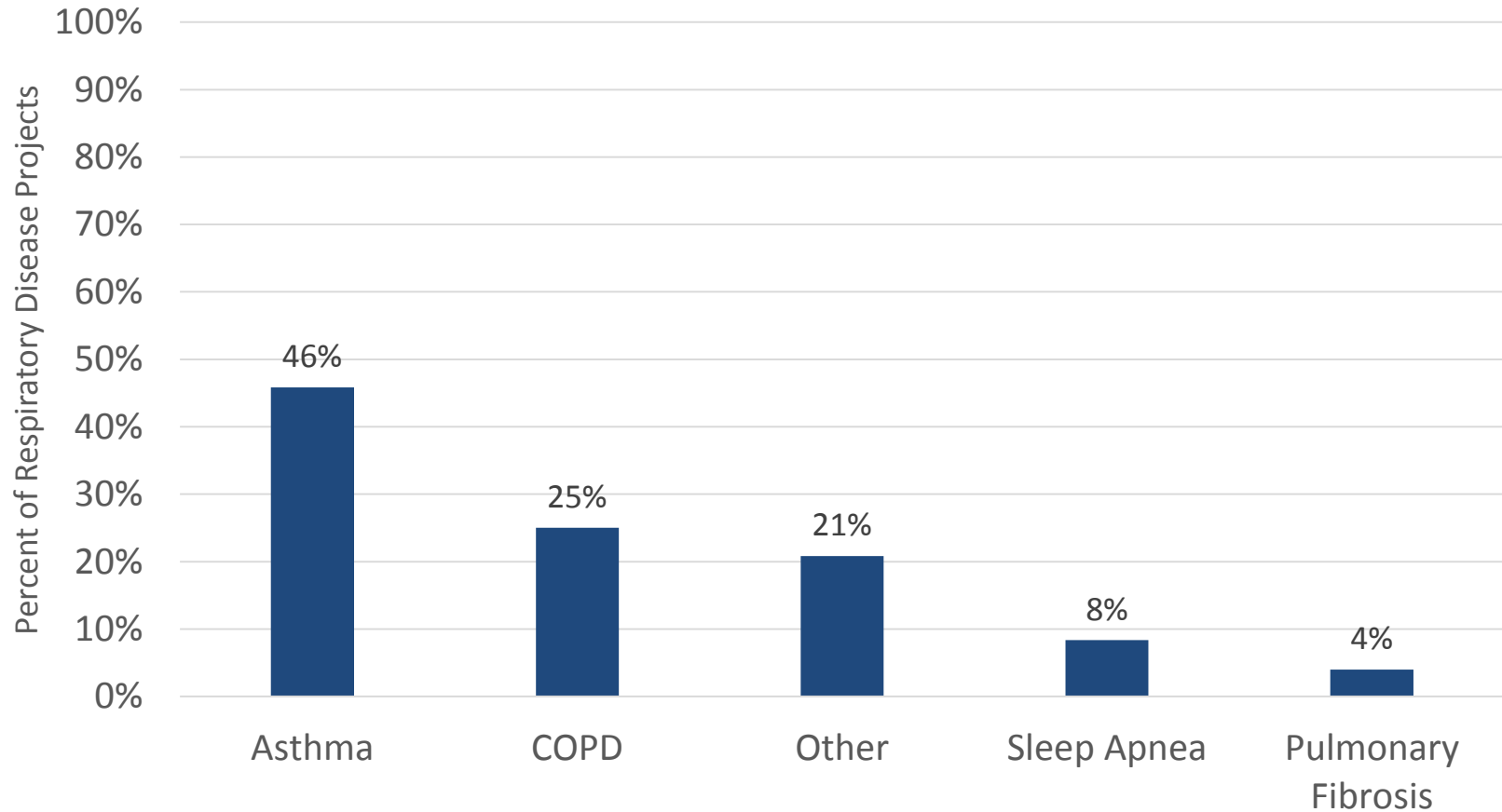
# Cardiovascular

N = 40



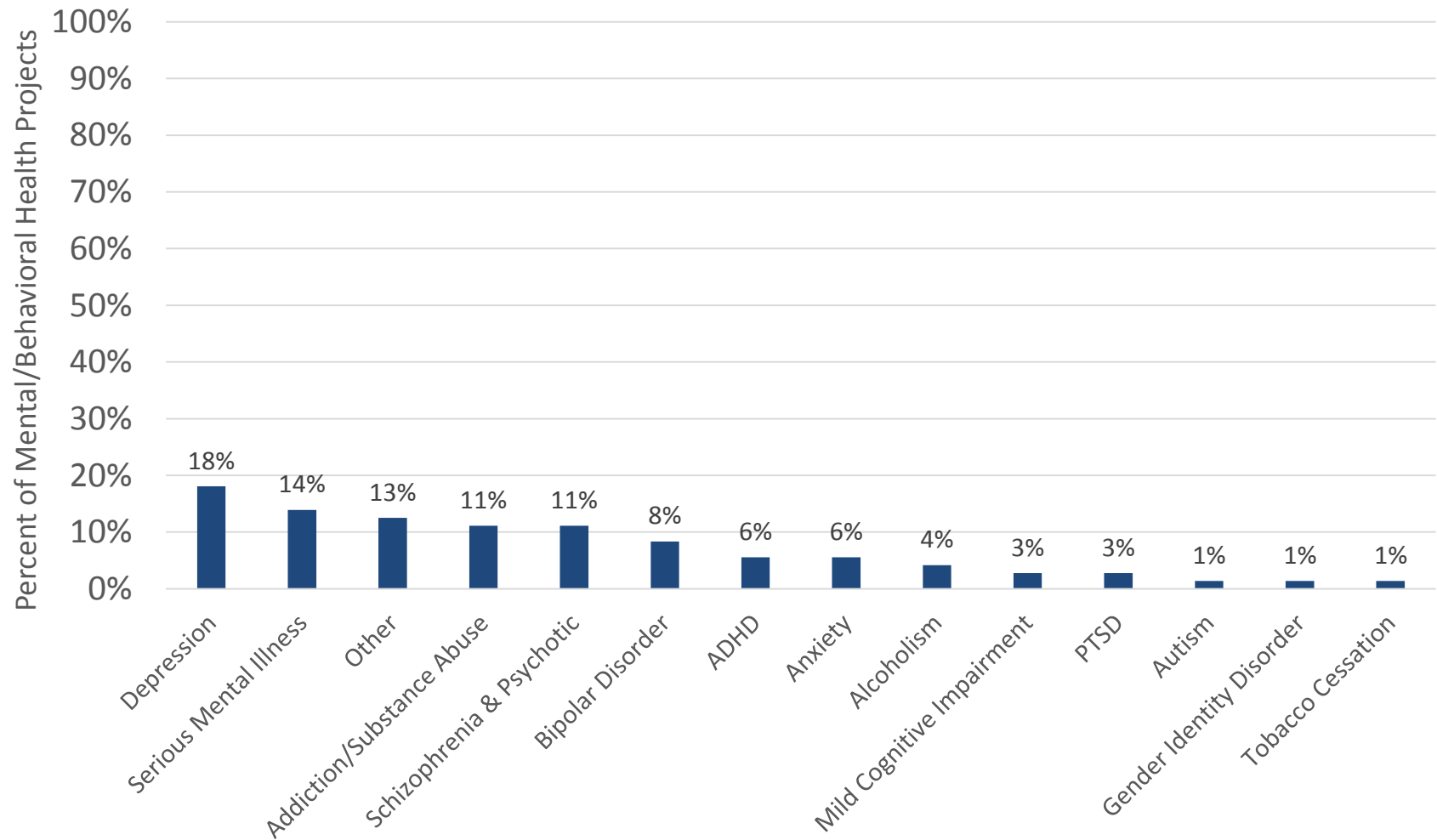
# Respiratory Diseases

N = 25



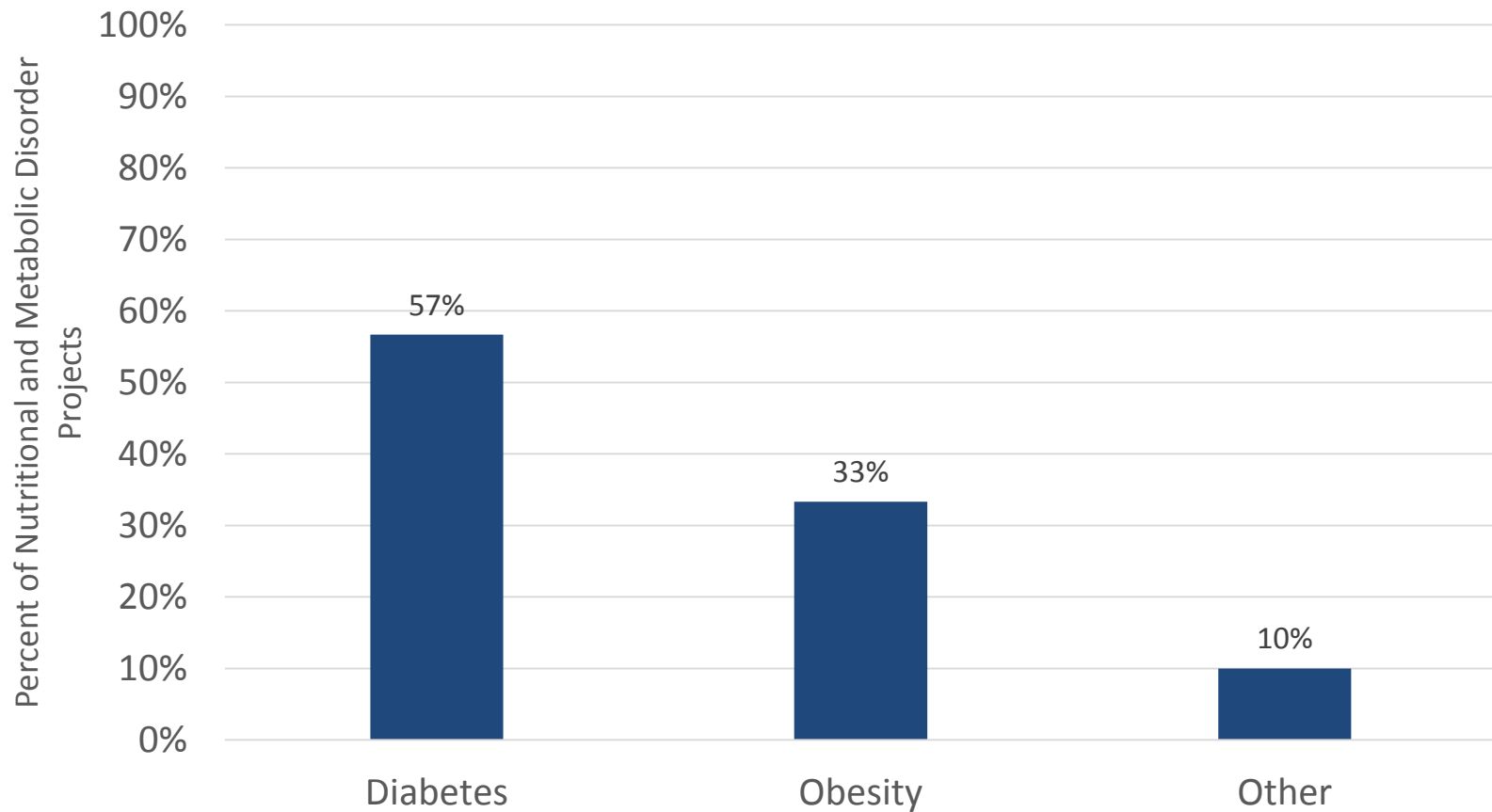
# Mental/Behavioral Health

N = 72



# Nutritional and Metabolic Disorders

N = 30



# Neurological Disorders

N = 19

