

Pragmatic Research Initiatives

PCORI Perspective

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NIH Collaboratory Steering Committee Meeting

April 20, 2022



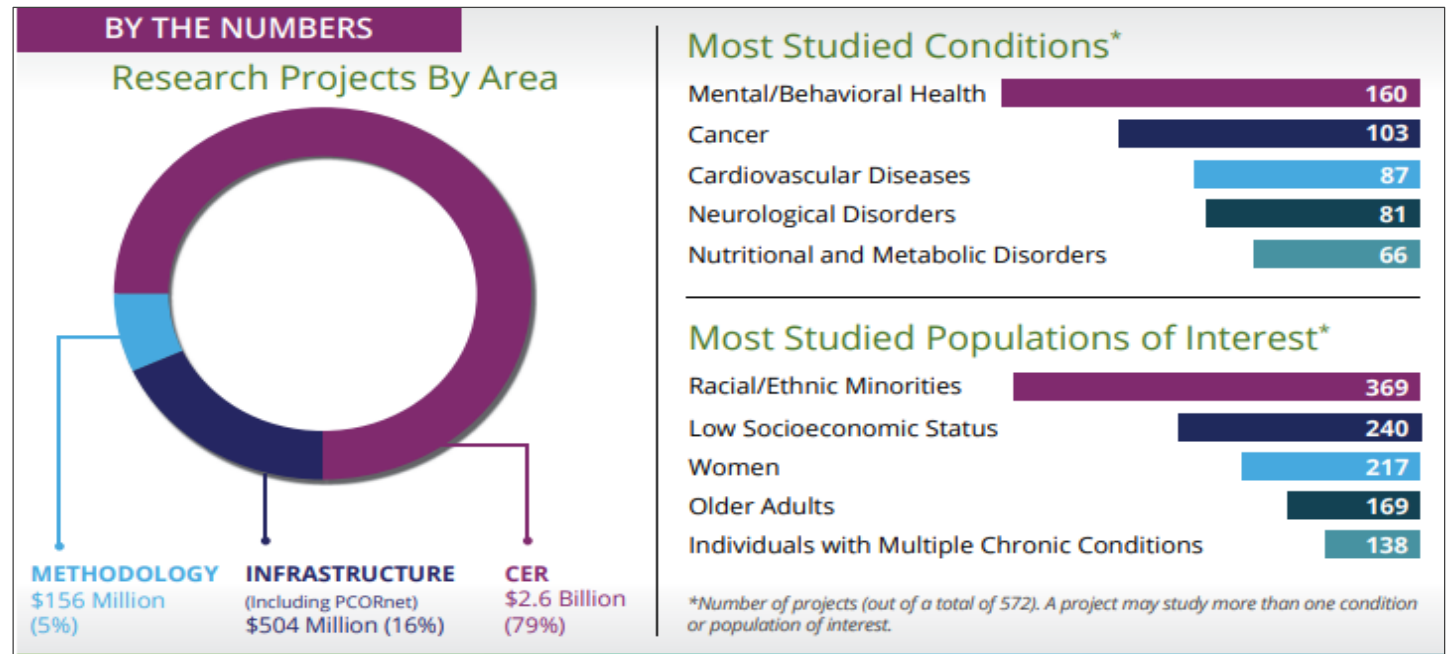
Patient-Centered Outcomes Research Institute – PCORI



- Non-profit, governed by a diverse Board of stakeholders = patients, caregivers, payers, providers, health systems, and others
- Supports patient-centered, comparative clinical effectiveness research (CER) via research projects, methods and infrastructure development, and engagement
- To address compelling clinical choices among effective or widely-used interventions, overall and by subgroups

To date:

- > 1900 funded research and related projects
- Awards > \$3.4 billion



- All PCORI research projects are on the pragmatic continuum in these ways
 - Enable decision-making about options to treat, diagnose, prevent, palliate, or improve health and health care
 - Done under typical, real-world conditions – patients, care partners, providers, settings, and care delivery
- Variety of types
 - Individual and cluster-randomized trials from \$3M - \$22 million direct costs
 - Clinical and health systems interventions
 - Embedded in real-world health care with varying levels of reliance on measures obtained from electronic health records due to frequent use of patient-reported outcomes (QoL, function)

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A Randomized Trial Comparing Antibiotics with Appendectomy for Appendicitis

The CODA Collaborative*

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Spinal Anesthesia or General Anesthesia for Hip Surgery in Older Adults

M.D. Neuman, R. Feng, J.L. Carson, L.J. Gaskins, D. Dillane, D.I. Sessler, F. Sieber, J. Magaziner, E.R. Marcantonio, S. Mehta, D. Menio, S. Ayad, T. Stone, S. Papp, E.S. Schwenk, N. Elkassabany, M. Marshall, J.D. Jaffe, C. Luke, B. Sharma, S. Azim, R.A. Hymes, K.-J. Chin, R. Sheppard, B. Perlman, J. Sappenfield, E. Hauck, M.A. Hoefl, M. Giska, Y. Ranganath, T. Tedore, S. Choi, J. Li, M.K. Kwofie, A. Nader, R.D. Sanders, B.F.S. Allen, K. Vlassakov, S. Kates, L.A. Fleisher, J. Dattilo, A. Tierney, A.J. Stephens-Shields, and S.S. Ellenberg, for the REGAIN Investigators*

ABSTRACT

BACKGROUND

The effects of spinal anesthesia as compared with general anesthesia on the ability to walk in older adults undergoing surgery for hip fracture have not been well studied.

METHODS

We conducted a pragmatic, randomized superiority trial to evaluate spinal anes-

The authors' full names, academic degrees, and affiliations are listed in the Appendix. Dr. Neuman can be contacted at neumanm@penmedicine.upenn.edu or at the Department of Anesthesiology and Critical Care, University of Pennsylv-

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Comparative Effectiveness of Aspirin Dosing in Cardiovascular Disease

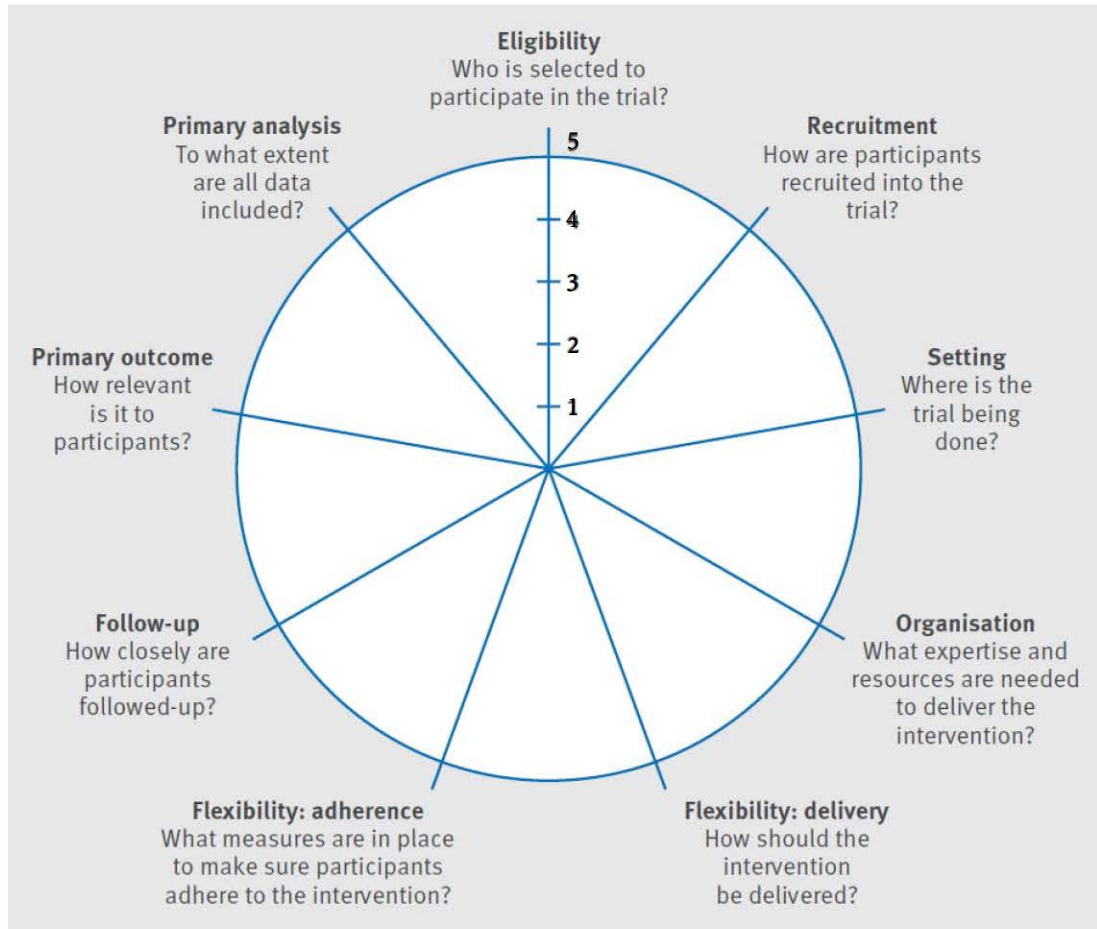
W.S. Jones, H. Mulder, L.M. Wruck, M.J. Pencina, S. Kripalani, D. Muñoz, D.L. Crenshaw, M.B. Effron, R.N. Re, K. Gupta, R.D. Anderson, C.J. Pepine, E.M. Handberg, B.R. Manning, S.K. Jain, S. Girotra, D. Riley, D.A. DeWalt, J. Whittle, Y.H. Goldberg, V.L. Roger, R. Hess, C.P. Benziger, P. Farrehi, L. Zhou, D.E. Ford, K. Haynes, J.J. VanWormer, K.U. Knowlton, J.L. Kraschnewski, T.S. Polonsky, D.J. Fintel, F.S. Ahmad, J.C. McClay,

The NEW ENGLAND JOURNAL of MEDICINE

ORIGINAL ARTICLE

Reliever-Triggered Inhaled Glucocorticoid in Black and Latinx Adults with Asthma

E. Israel, J.-C. Cardet, J.K. Carroll, A.L. Fuhlbrigge, L. She, F.W. Rockhold, N.E. Maher, M. Fagan, V.E. Forth, B.P. Yawn, P. Arias Hernandez, J.M. Kruse, B.K. Manning, J. Rodriguez-Louis, J.B. Shields, B. Ericson, A.D. Colon-Moya, S. Madison, T. Coyne-Beasley, G.M. Hammer, B.M. Kaplan, C.S. Rand, J. Robles, O. Thompson, M.E. Wechsler, J.P. Wisnivesky, M.D. McKee, S.P. Jariwala, E. Jerschow, P.J. Busse, D.C. Kaelber, S. Nazario, M.L. Hernandez, A.J. Apter, K.-L. Chang, V. Pinto-Plata, P.M. Stranges, L.P. Hurley, J. Trevor, T.B. Casale, G. Chupp, I.L. Riley, K. Shenoy, M. Pasarica, R.A. Calderon-Candelario, H. Tapp, A. Baydur, and W.D. Pace



The PRagmatic-Explanatory Continuum Indicator Summary 2 (PRECIS-2) wheel.

In theory, there is no difference between theory and practice.

In practice, there is.

Yogi Berra

Lessons Learned in Practice – Value of Engagement Prework



- PCORI experience: engaged relationships with diverse stakeholders are valuable throughout
 - Salient research question for the involved communities
 - Identification of important outcome measures
 - Understanding and insights about the environment and context for carrying out research
 - Available capacity and resources (e.g., advanced monitoring)
 - Constraints of time, space, personnel, equipment – especially in non-academic centers
 - Facilitate/improve dialogue, communications, and trust-building, particularly among under-served populations and racial/ethnic minorities (e.g., clarity and idiomatic nomenclature)
- PCORI supports engagement prework
 - Engagement Funding Opportunities
 - Feasibility testing phase (18 mo, ≤\$2M) of Phased Large Awards in Comparative Effectiveness Research (PLACER)

* Examples

Lessons Learned in Practice

Value of Data Resources



Value of electronic health data and other data resources

- Identify/characterize populations and practices for interventions
 - Candidate participants
 - Range of usual care practices (frequency of visits, types of providers, labs, imaging, etc.) and what are common/core practices
- Minimize research operations and intrusion into practice
 - Can allow direct outreach and recruitment of participants (attentive to representativeness with comparison of enrolled to candidate eligible participants)
 - Outcomes capture

Well-known data challenges

- Data access, variability across sites, and periodic disruptions with mergers, updates

Investigator Feedback from PCORI's Largest Pragmatic Awards



- Welcome opportunity to share challenges, lessons learned (via formal and informal “Learning Networks”)
- Management of variability/flexibility/fidelity of operations is most challenging aspect of pragmatic, multi-site research
 - Local interpretations of “pragmatic” may compromise adherence
 - Need to establish clear “guardrails” of required & discretionary work (prework can help)
- Central IRB debates
- Navigating acceptance of randomized treatment assignments
 - Explanations using various media, guided by stakeholder advisors
 - For irreversible choices (e.g. surgery), some exploration of clinician equipoise panels

Pandemic Challenges and Opportunities



Need for flexible and rapid adaptations from in-person operations

- eConsent, virtual visits, remote data collection

Opportunities

- Enlarge reach geographically and diversify participation by clinicians and patients
- Ease access, burdens of participation, and data collection

Attention and vigilance needed to virtual methods do not compromise equity, inclusivity, credible and trusted relationships

Collaboration of researchers, funding organizations, and industry around COVID a worthy model to achieve rapid advancement of science and health

Thank you

 www.pcori.org

 @pcori

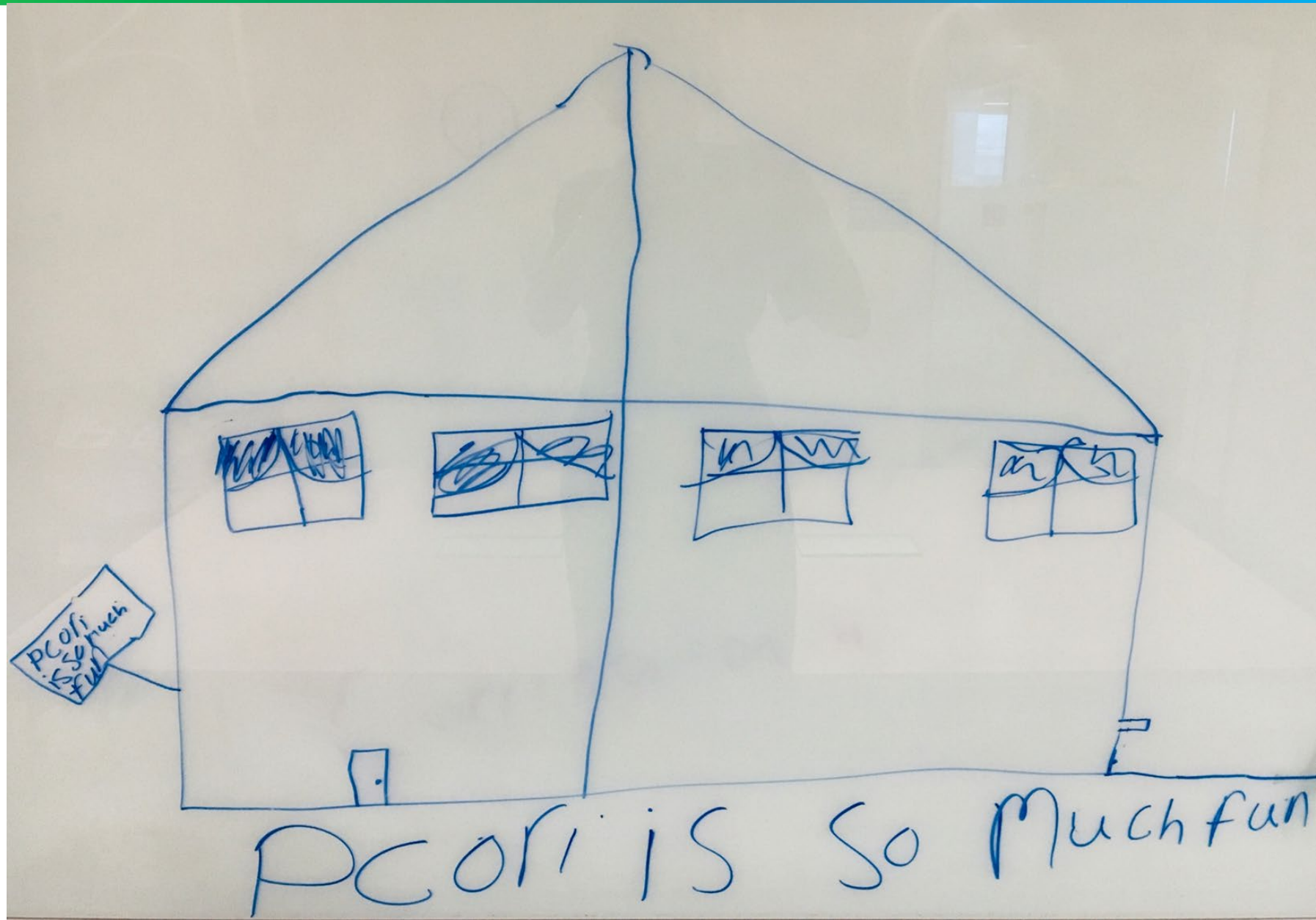
 [/PCORInstitute](https://www.facebook.com/PCORInstitute)

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Engagement Resources to Support Multi-Stakeholder Research



[Building Effective Multi-Stakeholder Research Teams](#)

For new and experienced researchers and stakeholder partners, offers **practical guidance and resources to support multi-stakeholder teams** in effectively working together.

- [Research Fundamentals](#) (for partners)
- [PCORI Engagement Rubric](#)
- [Compensation Framework](#)
- [Budgeting for Engagement Activities](#)
- [Engagement Plan \(Updated Template\)](#)
- [Engagement Tool and Resource Repository](#)
- [Engagement in Health Research Literature Explorer](#)
- [Guide for Engaging Research Partners about Data and Analytics](#)

Upcoming PCORI Funding Opportunities



PCORI Funding Opportunities

Broad Pragmatic Studies (next cycle opens May 3, 2022 Cycle 3 on Sept 7)

- Up to 5 years, \$10 million direct costs

Phased Large Awards in Comparative Effectiveness Research (opens Sept 7)

- Feasibility phase: Up to 18 months, \$2 million direct costs
- Full-scale continuation: Up to 5 years, \$20 million direct cost