Autoimmune Conditions

PCORI Overview & Portfolio Highlights

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pcori® PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE

Get To Know PCORI®

PCORI®

- Nonprofit that funds patient-centered comparative clinical effectiveness research (CER)
- Widely acknowledged as a leader in driving U.S. clinical research to become more patient-centered
- Provides funding for CER, engagement in research, dissemination and implementation and research infrastructure projects

Our Mission

Our Mission

Help people make better-informed healthcare decisions, and improve healthcare delivery and outcomes, by producing and promoting high-integrity, evidence-based information that comes from research guided by patients, caregivers, and the broader healthcare community

Multiple Funding Mechanisms

Types of PCORI-Funded Projects

CER Awards

- Fund studies comparing two or more treatments, services or health practices

Dissemination & Implementation Awards

- Fund projects to deliver the evidence to those who can use it

Engagement Awards

- Fund projects to bring more patients, caregivers, clinicians and communities into the health research process

CER Funding Opportunities: Broad and Focused

Broad

- Solicits CER proposals on any topic generated by CER community
- Aligned with PCORI's National Priorities for Health

Topic-Focused

- Solicits CER proposals aligned to the Topic Themes
- Emphasize patient-centered needs and creative approaches to close evidence gaps

PCORI & Patient Centeredness

Comparative Clinical Effectiveness Research

Research Team

Researchers and Stakeholders

Patient-Centered Outcomes Research

Stakeholders Include

- Patient and Family Members or Other Unpaid Caregivers of Patients
- Community Based Organizations
- Researchers
- Clinicians
- Hospital and Health Systems
- Training Institutions
- Purchasers
- Payers
- Policy Makers
- Industry

Patient-Centered Outcomes Research Institute

Engagement Makes Meaningful Differences

Engagement Influences…

- Study conceptualization, execution, materials and dissemination products
- Carrying out study tasks
- Engagement design and practice
- Researchers' understanding of patients, clinicians, & health care organizations

Engagement Influences all Aspects of CER Projects

- Research focus
- Research design
- Intervention tailoring/delivery
- Recruitment/retention
- Data collection/measures
- Data analysis
- Dissemination

Research Done Differently®

Online Guidance | PDF | FAQs

https://www.pcori.org/engagement-research/engagement-resources/foundational-expectations

Patient-Centered Burdens and Economic Outcomes (PCBEOs)

- PCORI encourages its funded researchers to capture the full range of outcomes in CER, including PCBEOs
- Include often-overlooked financial impacts that can create barriers to care
  - Examples: Lost wages, transportation expenses, childcare costs
- Available Resources:
  - PCBEO Landscape report with practical examples
  - Technical support documents providing research guidance

https://www.pcori.org/research-related-projects/about-our-research/patient-centered-burdens-and-economic-outcomes

Autoimmune Conditions

Highlights from the PCORI Portfolio

Psoriasis

CER Evidence that Matters

Comparing Two Ways to Provide Phototherapy to Treat Psoriasis

PROJECT INFORMATION

A Pragmatic Trial of Home versus Office-Based Narrowband Ultraviolet B Phototherapy for the Treatment of Psoriasis — The LITE Study

Principal Investigator
Joel Gelfand, MD, MS

Organization
University of Pennsylvania

Top 5% for journal & age
Data as of February 10, 2025

- Office-based phototherapy treatment involves office visits 3 times per week for 12 weeks, which presents significant barriers to treatment completion.
- This randomized controlled trial of 783 participants found that home-based phototherapy was as effective as office-based phototherapy.
  - Patients in both groups achieved clear/almost clear skin and similar improvements in quality of life.
  - Patients receiving home-based phototherapy had better treatment adherence and experienced lower burden of indirect costs.

Gelfand JM, Armstrong AW, Lim HW, et al. *Home- vs Office-Based Narrowband UV-B Phototherapy for Patients With Psoriasis: The LITE Randomized Clinical Trial*. *JAMA Dermatol*. 2024 Sep 25:e243897. [link to project page]

CER Evidence that Matters

Comparing Two Ways to Provide Phototherapy to Treat Psoriasis

- Patients receiving home-based phototherapy had better treatment adherence and experienced lower burden of indirect costs:
  - Time: patients in the in-office arm spent an average of 50 minutes driving to and from each treatment
  - Money: Costs associated with transportation to and from in-office visit as well as copays in office (average across sample of about $17.50 per visit)

- Important consideration:
  - Access beyond research study: those in the study received the device at no cost

Takeaway: home-based therapy was as effective as in-office, with the benefit of saving both time and money for participants

Gelfand JM, Armstrong AW, Lim HW, et al. *Home- vs Office-Based Narrowband UV-B Phototherapy for Patients With Psoriasis: The LITE Randomized Clinical Trial*. *JAMA Dermatol*. 2024 Sep 25:e243897. [link to project page]

Multiple Sclerosis

Goals of Treatments for MS

MS has no cure, but patients and clinicians have clear treatment goals

- **Address acute relapses**
  - Generally with corticosteroids
- **Managing symptoms**
  - Fatigue
  - Difficulty walking
  - Memory or attention problems
  - Bladder problems
  - Numbness, tingling, pain
- **Modify the disease course to prevent disability**
  - Focused on altering the natural history of MS

Patient Experience of Fatigue in MS

"MS fatigue is like hitting a wall or having a door slam shut on you … My entire focus is on how tired I am and what I cannot do."

Patient Partner, PCORI-funded COMBO-MS Study

"I often tell myself, 'How can one human being be this tired and continue to live?' "

Patient with MS

"I really, really just feel fatigued. Just mentally, you're tired. Physically, you're tired. Emotionally, you're tired, and you just don't wanna do anything, and even sitting there and just being an empty vessel, you're still tired."

Patient with MS

www.pcori.org/Nourbakhsh359 and www.pcori.org/BraleyKratz474

CER Evidence that Matters

Managing Multiple Sclerosis-Related Fatigue

TRUIMPHANT-MS Study

- Randomized controlled trial including 141 adults with MS and fatigue in Maryland and California
- Average age was 47, most patients had relapsing-remitting MS
- Randomized to 1 of 4 treatment sequences (6 weeks each treatment)
  - Amantadine, methylphenidate, modafinil, placebo
- Outcomes: Fatigue, sleepiness, quality of life
- Patients with MS, Clinicians, and advocates informed the study

COMBO-MS Study

- Randomized controlled trial of 336 adults with MS and fatigue in Michigan and Washington
- Average age 49, most patients had relapsing-remitting MS
- Randomized to telephone-based cognitive behavioral therapy, modafinil, or a combination of both over 12 weeks
- Outcomes: Fatigue, adverse events
- Patients with MS, clinicians, health insurers and national and regional advocacy organizations informed the study

https://www.pcori.org/evidence-updates/managing-multiple-sclerosis-related-fatigue

CER Evidence that Matters
Managing Multiple Sclerosis-Related Fatigue

TRIUMPHANT-MS Study

The TRIUMPHANT-MS study collected MFIS scores at baseline and again after five weeks of treatment with each medicine.

Outcome/Measure	Finding
Fatigue (MFIS)	MFIS scores* were no different than placebo across all medicines (p=0.20).
Quality of life (Quality of Life in Neurological Disorders [Neuro-QoL] fatigue item bank)	No treatments improved fatigue-related quality of life (p=0.42).
Daytime sleepiness (Epworth Sleepiness Scale)	No treatments improved daytime sleepiness (p=0.071).
Adverse events	A higher percentage of patients reported adverse events while taking amantadine (39%), methylphenidate (40%), and modafinil (40%) than when taking the placebo (31%). The most commonly reported nervous system or psychiatric adverse events included headaches, insomnia, lightheadedness and dizziness, and anxiety. The most common gastrointestinal adverse events were nausea, dry mouth and constipation.

*Mean values for the MFIS total scores were collected at the highest tolerated dose in each medication period.

COMBO-MS Study

The COMBO-MS study collected MFIS scores at baseline and again 12 weeks later.

Outcome/Measure	COMBO-MS
Fatigue (MFIS)	Reductions in MFIS scores* did not differ by treatment. Of note, all three treatments were associated with statistically and clinically significant (i.e., >10 points) reductions in total MFIS scores.
Adverse events	A total of 0, 27, and 32 adverse events were determined to be related to treatment in the CBT, modafinil and combination therapy groups, respectively. The most commonly reported adverse events were anxiety, headache and insomnia (related to modafinil and combination therapy only).

* Lower MFIS total scores are indicative of improved symptoms.

Key Takeaways

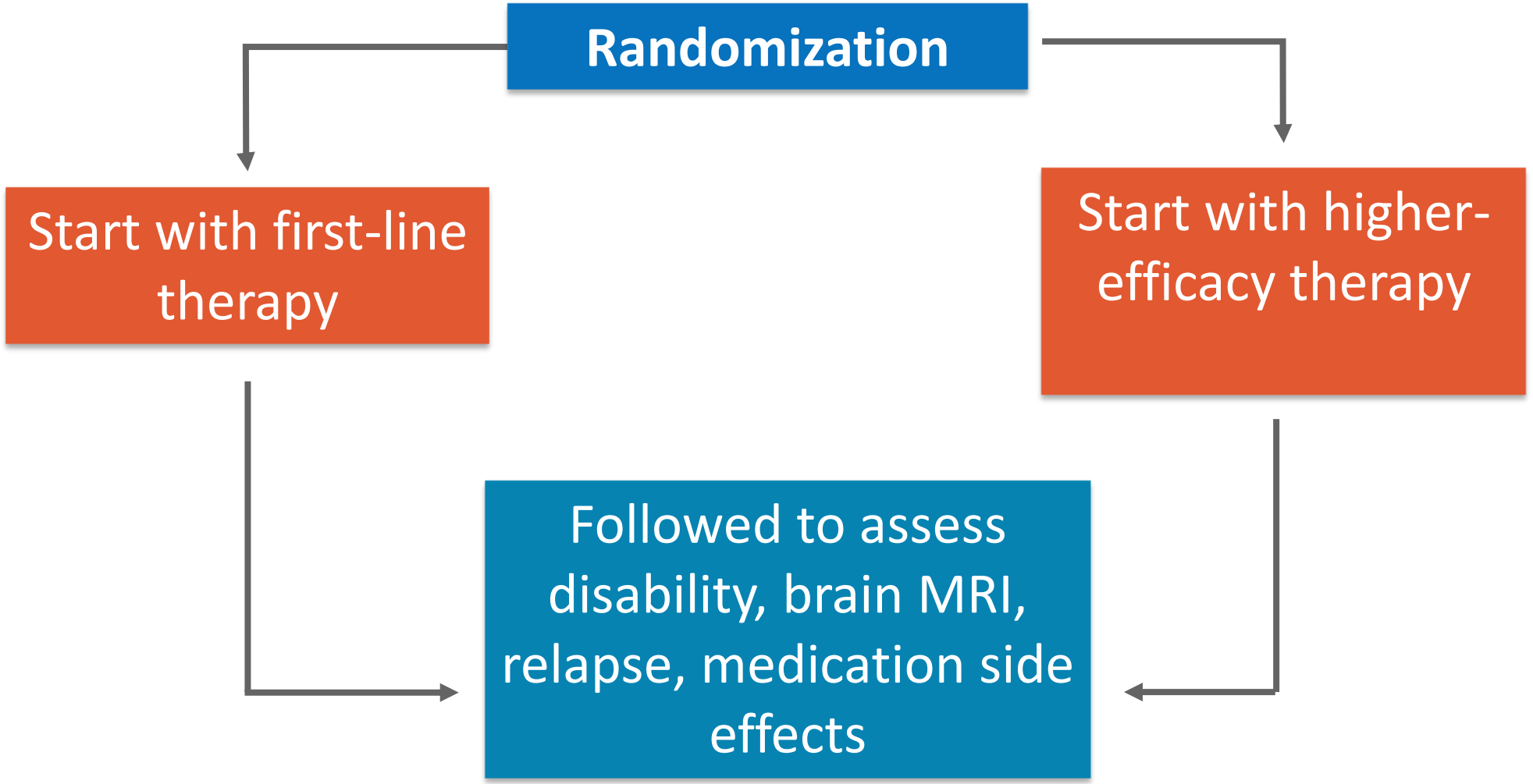
- Triumphant-MS: Medications were not superior to placebo in improving fatigue. More patients experienced adverse events when taking one of the medications than when taking placebo.
- Combo-MS: telephone-based CBT, modafinil, and combination therapy each reduced the intensity of MS-related fatigue about the same. Overall low adverse event rate, but those who used modafinil and combination therapy reported more adverse events than CBT alone.

https://www.pcori.org/evidence-updates/managing-multiple-sclerosis-related-fatigue

Another Key Decisional Dilemma for Newly-Diagnosed Patients with MS

- In the early stages of MS, should patients take a **standard, first-line agent** or should they start with a **higher-efficacy medication** to reduce relapses, and prevent disease progression and disability?
- It is not known if **starting** with standard, first-line medicine and then **escalating** to other therapy as needed is better for preventing long-term MS disability than starting with aggressive medicines

Two PCORI trials addressing this key Question



Looking to the Future: Two MS Studies Addressing a Key Question in MS Care

Do higher efficacy medicines work better than first-line medicine for preventing long-term MS disability?

- Two complementary trials
  - Harmonized endpoints
    - Total brain volume
    - Disability
    - Fatigue, quality of life
- Examining heterogeneity of treatment effects (what works best for whom)
  - Demographic characteristics
  - Level of baseline disease activity
  - Risk for disability
- Current Status: enrollment complete; collecting follow-up data – anticipate results in 2026-2027

TREAT-MS

Examining Whether Early Aggressive Therapy Can Prevent or Delay Disability in People with Multiple Sclerosis

Ellen Mowry, MD
Scott Newsome, DO
Johns Hopkins University

DELIVER-MS

Comparing Two Approaches to Treat Relapsing-Remitting Multiple Sclerosis

Daniel Ontaneda, MD
Nikolaos Evangelou, MD, Dphil
Cleveland Clinic Foundation

Rheumatoid Arthritis

CER Evidence that Matters
Rheumatoid Arthritis Treatment

PROJECT INFORMATION

Comparing Stopping Versus Continuing Use of Tumor Necrosis Factor Inhibitors in Adults with Rheumatoid Arthritis in Remission

Principal Investigator
Florina Constantinescu, MD, PhD

Organization
Georgetown University

- This randomized controlled noninferiority trial included 102 participants with RA across 12 rheumatology centers who had sustained remission for at least 6 months and receiving standard remission maintenance randomized to placebo (discontinue TNFi) vs. TNFi on relapse free survival.
- The Data Safety Monitoring Board stopped the study early:
  - The difference in relapse between discontinuing and continuing a TNFi exceeded the noninferiority margin
  - Joint damage progressed more among patients who continued their TNFi than it did among patients who discontinued a TNFi ($p<0.03$); however, this difference was small and not considered clinically meaningful.
  - The two groups did not differ significantly in functional ability.

https://www.pcori.org/research-results/2014/comparing-stopping-versus-continuing-use-tumor-necrosis-factor-inhibitors-adults-rheumatoid-arthritis-remission-stara-study

Looking to the Future: RA Study in Progress

PROJECT INFORMATION

Comparing Two Ways to Treat Patients with Rheumatoid Arthritis

Principal Investigator
Jasvinder Singh, MD, MPH

Organization
University of Alabama at Birmingham

- This randomized controlled trial will assess whether switching/adding a targeted synthetic disease-modifying anti-rheumatic drugs (tsDMARD) is superior to switching/adding a non-TNF-biologic in patients with active RA
  - Choosing between these options is a common and difficult choice for patients and providers.
  - Comparative effectiveness of these treatment options for improvement in patient-reported outcomes (PRO) is lacking.
- Multicenter, 12-month, 2-arm, open-label trial
- 924 adults with a diagnosis of active RA despite treatment with a TNFi-biologic
- Outcomes: functional ability secondary: fatigue, work productivity, sleep, treatment satisfaction
- Status: Recruiting, anticipate results in 2027

https://www.pcori.org/research-results/2020/comparing-two-ways-treat-patients-rheumatoid-arthritis-ra-pro-pragmatic-trial-ra-propr

Inflammatory Bowel Disease

Looking to the Future: IBD Study in Progress

PROJECT INFORMATION

Comparing Two Ways to Reduce Flare-Ups among People with Inflammatory Bowel Disease -- The QUOTIENT Study

Principal Investigator
Siddharth Singh, MD, MS

Organization
University of California San Diego

- This randomized controlled trial compares the effectiveness, burden, and safety of switching to an alternative targeted immunomodulator (TIM) to treat to a target of endoscopic remission vs. continuing the index TIM among 406 asymptomatic adult patients with Crohn's Disease or Ulcerative Colitis who are in symptomatic remission but have ongoing moderate-to-severe endoscopic inflammation.
- **Comparator(s):** Switching to an alternative TIM vs. continuing the index TIM
- **Outcomes:**
  - *Primary:* symptomatic relapse based on patient-reported outcomes, b) IBD-related hospitalization, c) IBD-related surgery, and/or d) structural complications due to IBD
  - *Select Secondary:* Total time spent in remission, health-related quality of life, fatigue, IBD-related disability, treatment burden, treatment satisfaction, patients' self-reported annual out-of-pocket costs, treatment-related adverse events
- **Length of Follow-up:** Quarterly assessments for 2 years, primary outcome assessed at 2 years
- **Status**: Recruiting, anticipate results in 2028

https://www.pcori.org/research-results/2021/comparing-two-ways-reduce-flare-ups-among-people-inflammatory-bowel-disease-quotient-study

In Summary…

- Those living with autoimmune conditions are often managing periods of high symptom burden, and for those that are progressive, increasing levels of functional impairment and disability
- Patients (with their clinicians) are often faced with complex treatment decisions, often in the absence of sufficient evidence to make an informed decision about the best choice given their individual circumstances
- These conditions have a substantive impact across many facets of daily life (productivity, financial cost, time, well-being)
- Research generating evidence to inform what works best for whom across important outcome domains is critical to supporting achievement of optimal health and well-being for all

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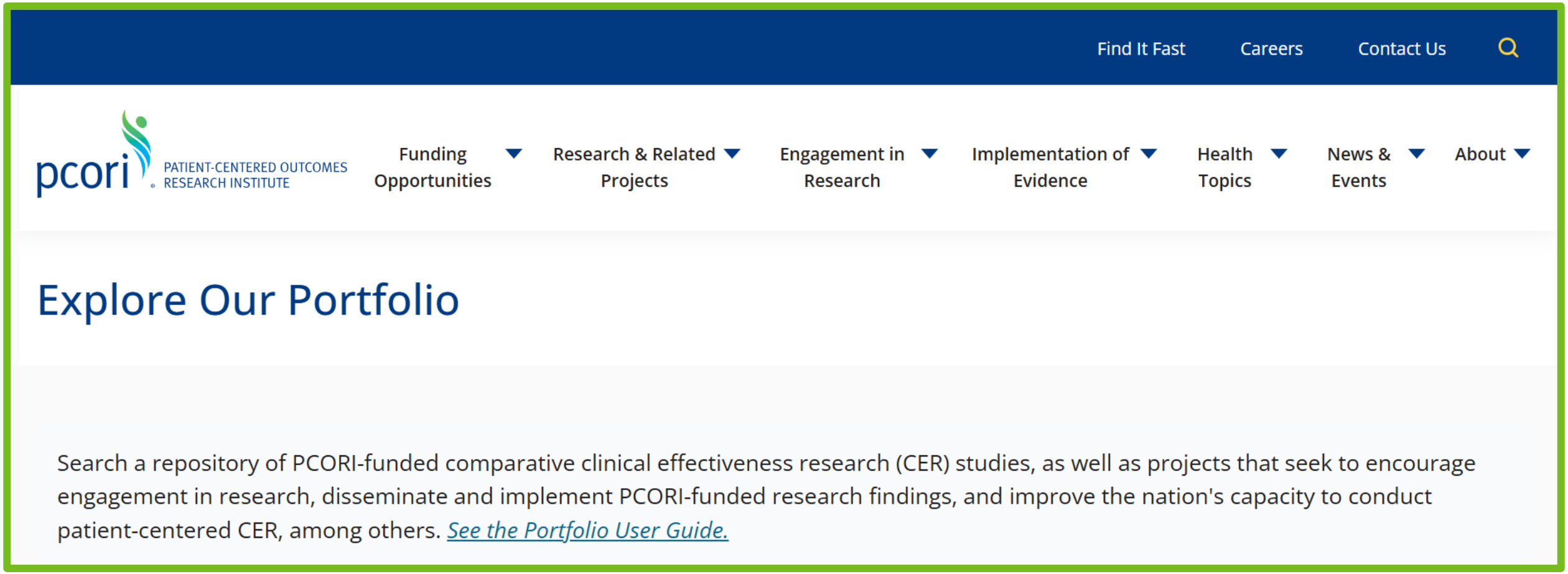
Searchable Repository of PCORI-Funded Studies



https://www.pcori.org/explore-our-portfolio

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  - Comparing Two Approaches to Treat Relapsing-Remitting Multiple Sclerosis -- the DELIVER-MS Study | PCORI
- Rheumatoid Arthritis

  Complete
  - https://www.pcori.org/research-results/2014/comparing-stopping-versus-continuing-use-tumor-necrosis-factor-inhibitors-adults-rheumatoid-arthritis-remission-stara-study

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