Using Patient-Centered Outcomes Research In Practice:

Building Capacity for PA Dissemination

Faculty

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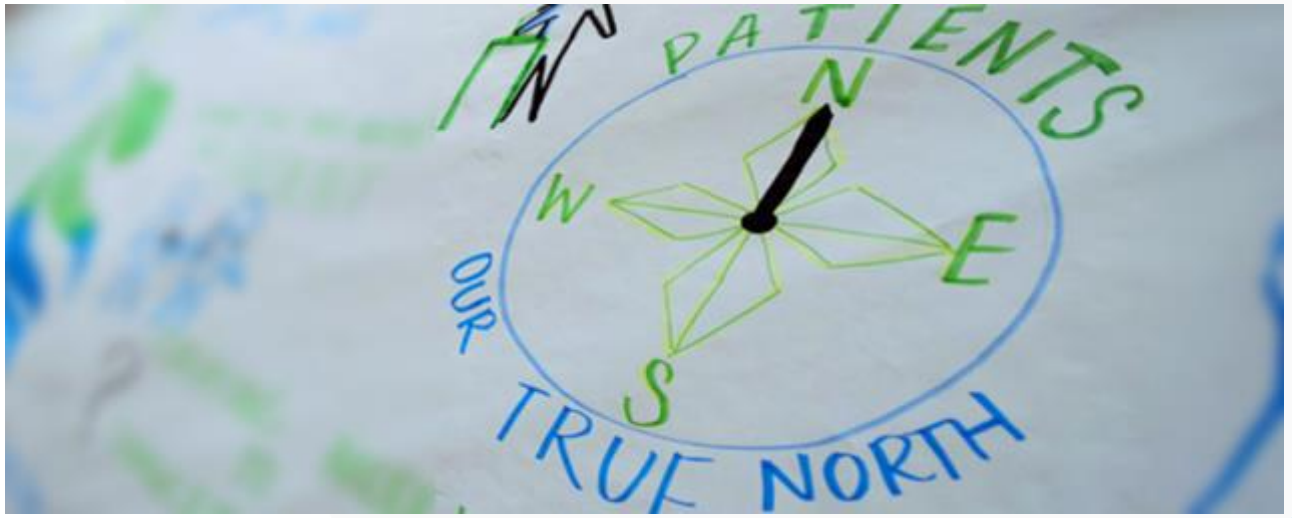
Learning Objectives

At the conclusion of this activity, participants should be able to:

- Define patient centered outcomes research (PCOR) and comparative effectiveness research (CER)
- Compare and contrast how PCOR and CER research designs differ to traditional research designs
- Access and assess PCOR and CER relevant to the patients under their care
- Incorporate PCOR and CER results into patient care activities

About Patient-Centered Outcomes Research Institute (PCORI)



- An independent research institute authorized by Congress in 2010 and governed by a 23-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 Broad and Complex Mandate

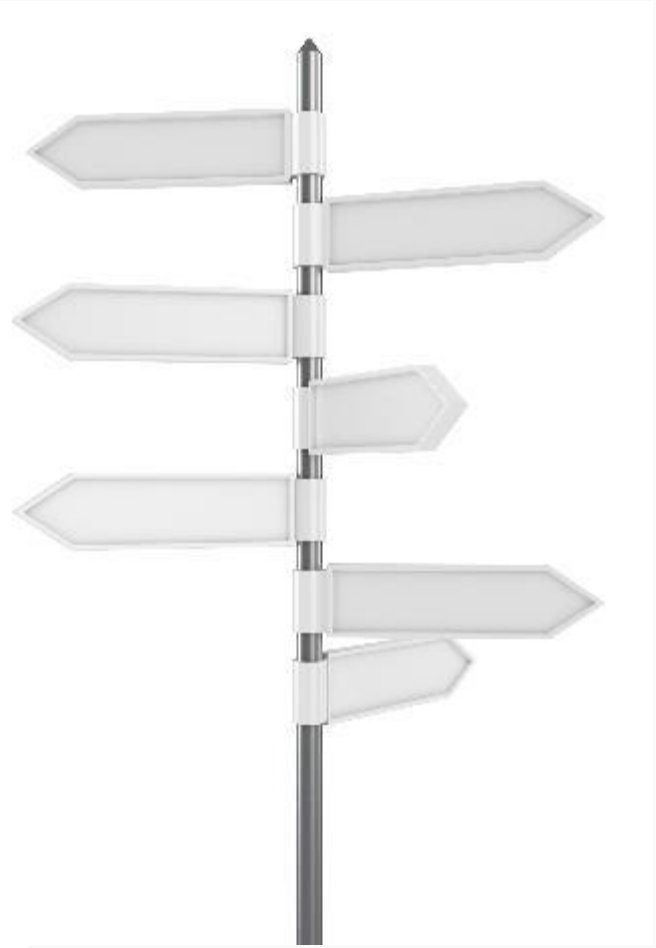
"The purpose of the Institute is to **assist patients, clinicians, purchasers, and policy-makers in making informed health decisions** by advancing the quality and relevance of evidence concerning the manner in which diseases, disorders, and other health conditions can effectively and appropriately be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

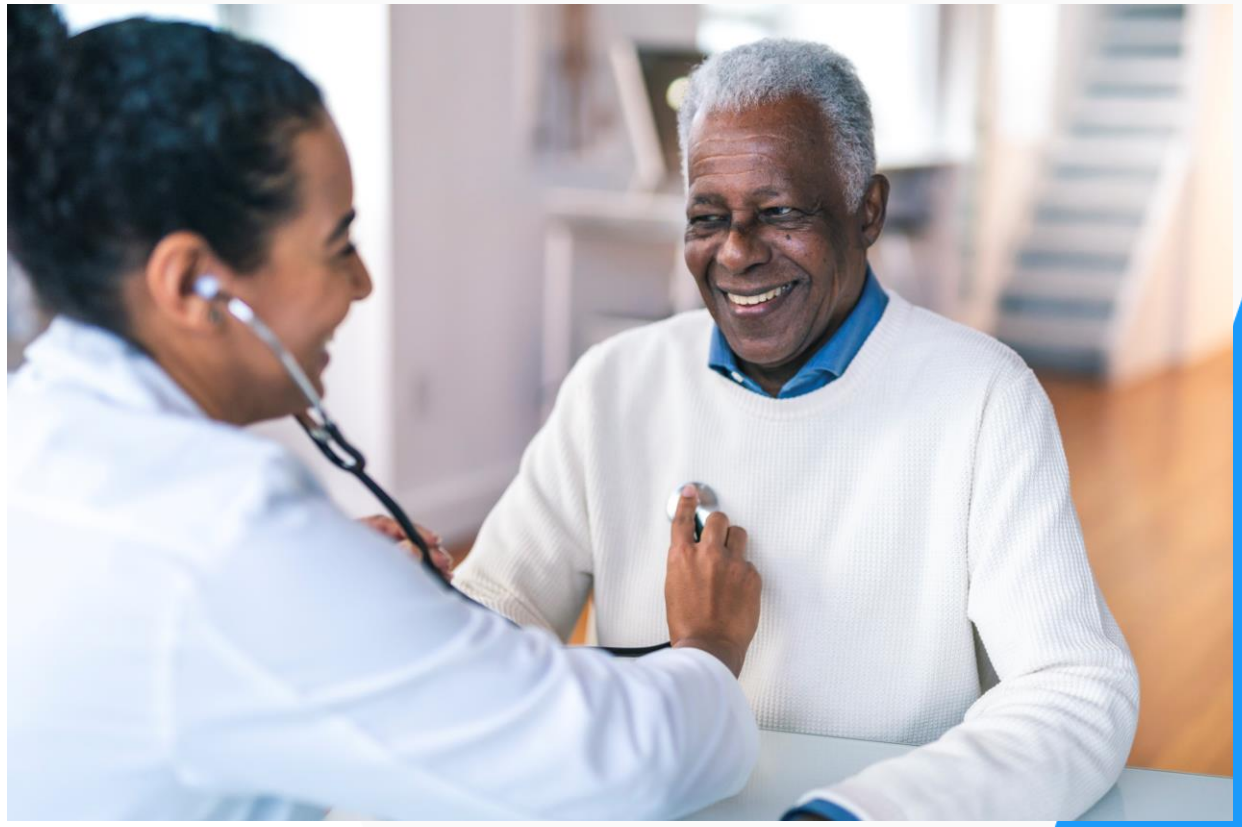
Why is PCORI's Work Needed?

- For all the advances it produces, traditional healthcare research has not answered many questions patients face
- People want to know which preventive, diagnostic, or treatment option is best for them
- Patients and their clinicians need information they can understand and use



How is PCORI Research Different?

- We aim to produce evidence that can be easily **applied in real-world settings**
- We focus on **answering questions most important to patients** and those who care for them
- We **engage** patients, **caregivers**, clinicians, insurers, employers, and other stakeholders throughout the research process



PCORI Funds Comparative Clinical Effectiveness Research(CER)

- Generates and synthesizes evidence comparing benefits and harms of at least two different methods to prevent, diagnose, treat, and monitor a clinical condition or improve care delivery
- Measures benefits in real-world populations
- Describes results in subgroups of people
- Helps consumers, clinicians, purchasers, and policy makers make informed decisions that will improve care for individuals and populations
- Informs a specific clinical or policy decision

Note: We do not fund cost-effectiveness research

Adapted from *Initial National Priorities for Comparative Effectiveness Research*, Institute of Medicine of the National Academies

PCORI Fund Patient-Centered Outcomes Research (PCOR)

PCOR is a relatively new form of CER that…

- Considers patients' needs and preferences, and the outcomes most important to them
- Investigates what works, for whom, under what circumstances
- Helps patients and other healthcare stakeholders make better-informed decisions about health and healthcare options

Implementing PCOR – Outcomes Important to Patients

The PA's Path to Patient-Centered Care

Traditional Health “Care” Approach

Disease Focused

- Has the patient had the appropriate initial or ongoing screening/surveillance?
- Is the patient on guideline directed medications?
- Which medications have the most benefit?
- Is the patient eligible for invasive interventions?
- Has the patient received counseling on diet and lifestyle?
- Has the patient seen or been referred to appropriate specialists?
- Has the patient been scheduled for follow-up?

Patient Focused - PCOR

- Given my personal characteristics, conditions, and preferences, what should I expect to happen to me?
- What are my options? What are the potential benefits and harms of those options?
- What can I do to improve the outcomes that are most important to me?
- How can clinicians and the healthcare systems they work in help me make the best decisions about my health and health care?

Diabetes Mellitus – Why We Treat

Medical Rationale

387 million people across the world have diabetes, 90% of those are Type II and lead to significant complications, medical sequelae

- Reduce or Avoid patient-level development of co-morbid conditions: cardiovascular events, nephropathy, neuropathy, retinopathy
- Follow guidelines-directed care
- Improve population and community health

Patient Concerns

- How will/can I afford the foods I am recommended to eat? Will I like the foods? Are they available at the stores I frequent?
- What foods should I eat? How do I prepare/cook healthy foods?
- Can I afford the medications?
- What side effects will I experience from medications?
- Will the medications harm me?
- What problems might occur if I don't treat? Will this shorten my life? Will I lose limbs or nerve feelings?
- Will treatment be inconvenient?

Reviewing the Evidence for Treatment: Traditional Medical Literature Scan

How does the SGLT2i class compare to GLP-1RA for reduction of A1c and major adverse cardiovascular events (MACE)?

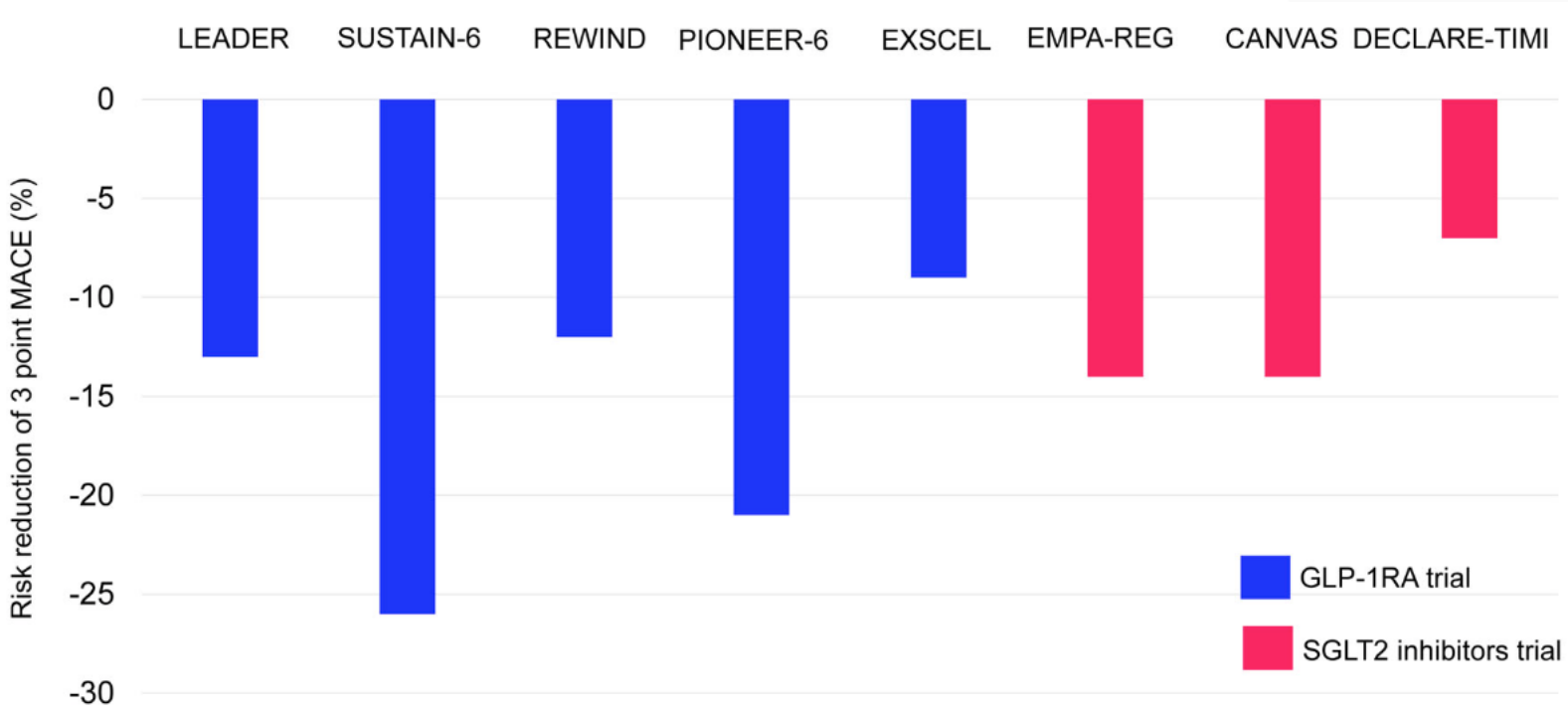


Figure 1. *The risk reduction in three-point MACE in GLP-1RA and SGLT2i trials. MACE: major adverse cardiovascular events; GLP-1RA: glucagon-like peptide 1 receptor agonists; SGLT2i: sodium-glucose cotransporter 2 inhibitors.*

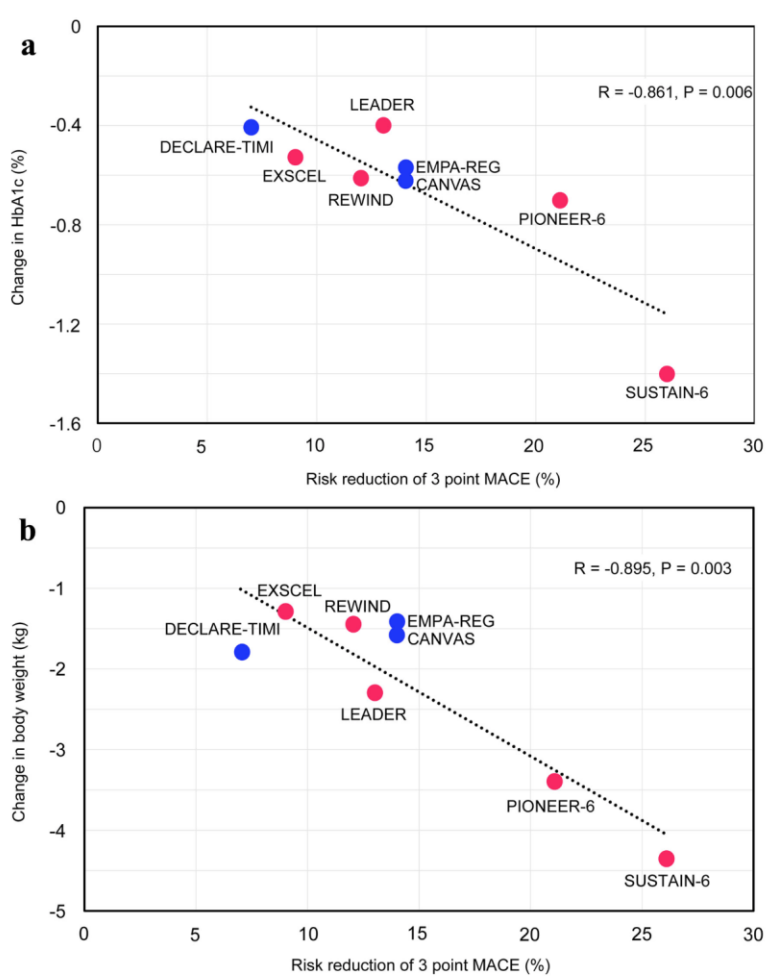


Figure 2. *The correlation between the risk reduction of three-point MACE between changes in HbA1c (a) and body weight (b). MACE: major adverse cardiovascular events.*

Yanal et al. Cardio Res 2023.

Reviewing the Evidence for Treatment:
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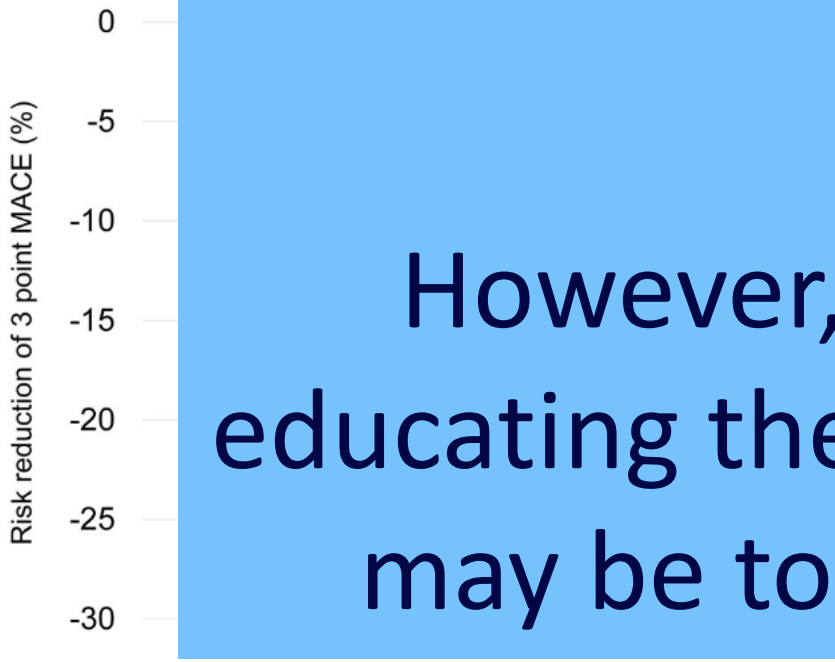
How d CE)?

This data may help the PA to understand the impact of these therapies on A1c and MACE.

However, may not translate well to educating the patient and addressing what may be top concerns for the patient.



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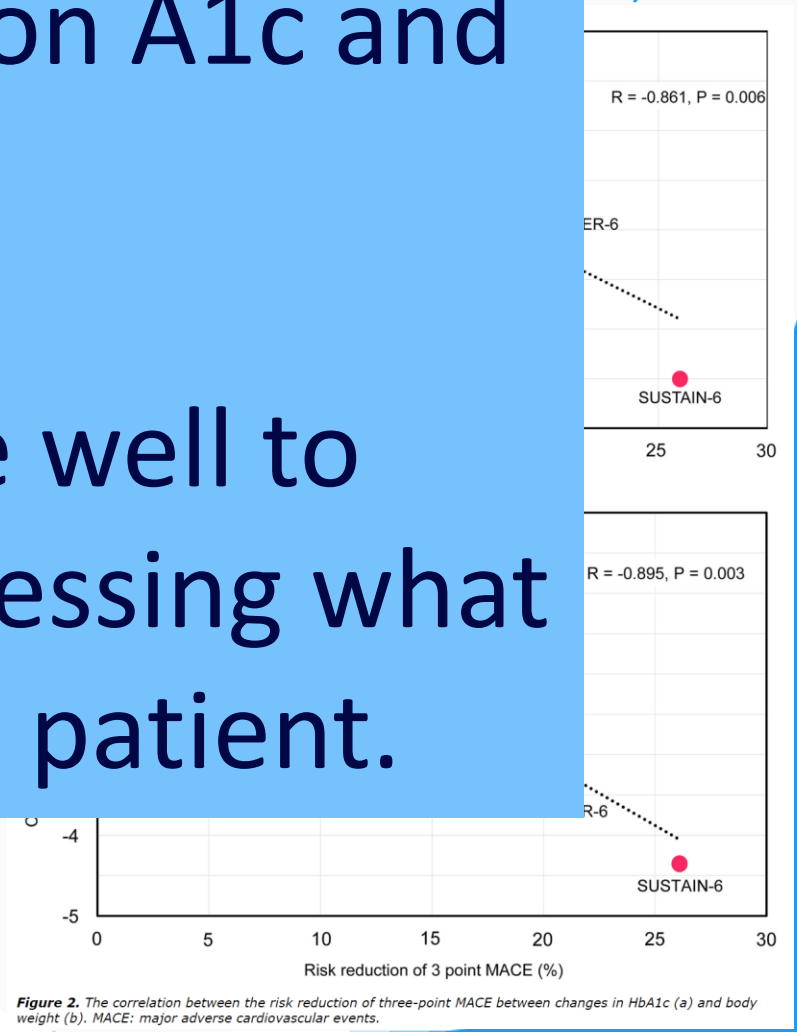


Figure 2. *The correlation between the risk reduction of three-point MACE between changes in HbA1c (a) and body weight (b). MACE: major adverse cardiovascular events.*

Pragmatic Diabetes Treatment Question

Will daily glucose monitoring lead to lower glucose levels in patients with diabetes and not using insulin?

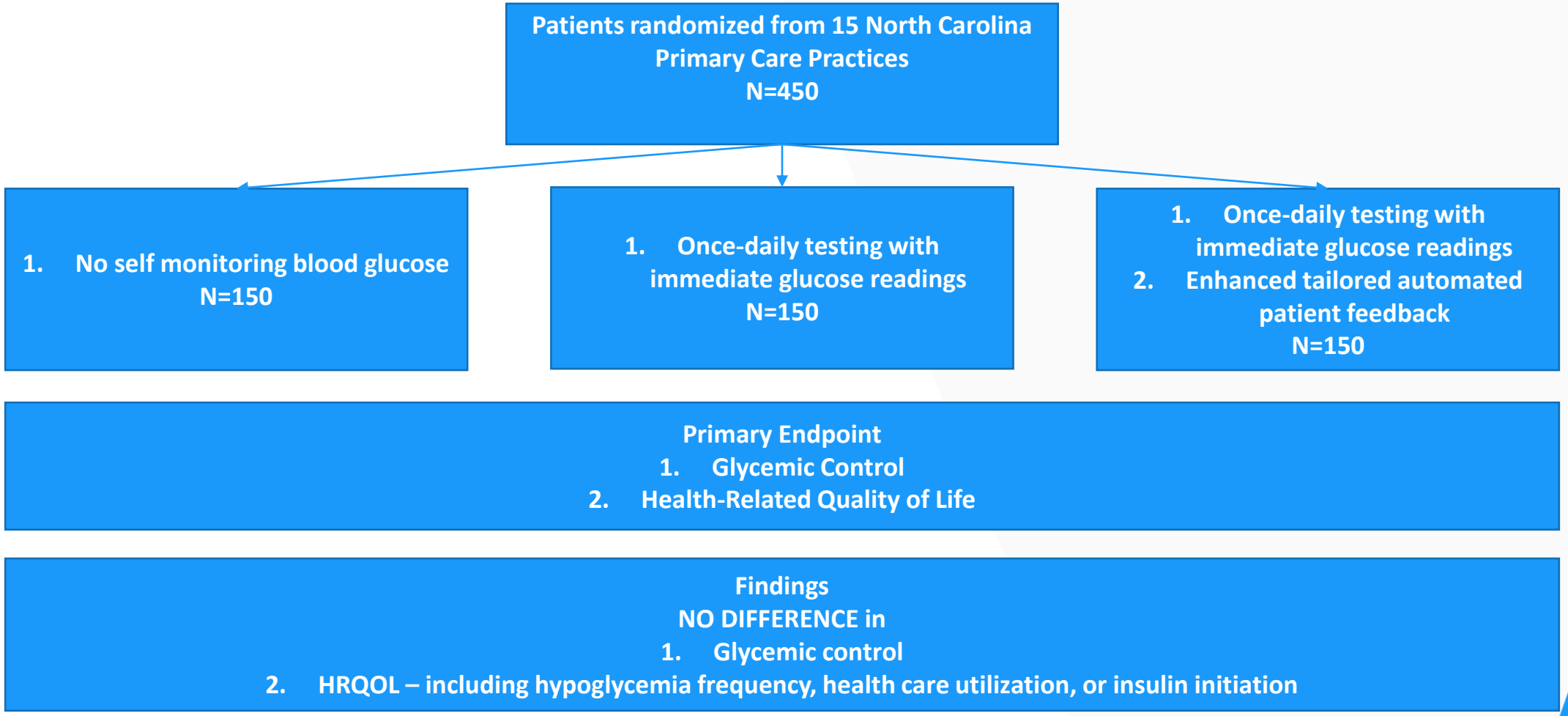
Potential Benefit

- Knowledge of glucose levels may help direct diet and inform medication titration/addition, and provide additional value to A1c obtained every 3-6 months
- Improve patient DM self-efficacy (confidence in managing their own chronic illness), DM-related quality of life, DM treatment satisfaction, patient-provider communication
- Lower hypoglycemia frequency and health care utilization

Patient Burden

- Cost of purchasing glucometer and recurring costs of purchase and replacement of testing strips and sharps
- Remembering to complete self-monitoring of blood glucose daily
- Understand results and know what to do based on the results (e.g., nothing, change diet intake, increase medications, etc.)

The Monitor Trial



Young et al. JAMA Intern Med. 2017 Jul; 177(7): 920–929.

Translating the Data to Patient-Centered Care

Patient: “I have diabetes. Does this mean I have to test my blood sugar everyday?”

Clinician: “Not all patients with the diagnosis of diabetes benefit from daily glucose monitoring. In fact, a study of those not on insulin found that daily monitoring did not improve sugar control, add any benefit to quality of life, or decrease health care use.”

Provide Patient Education From PCORI

Blood Sugar Testing to Manage Type 2 Diabetes in Patients Who Don’t Need Insulin (pcori.org)

Young et al. JAMA Intern Med. 2017 Jul; 177(7): 920–929.

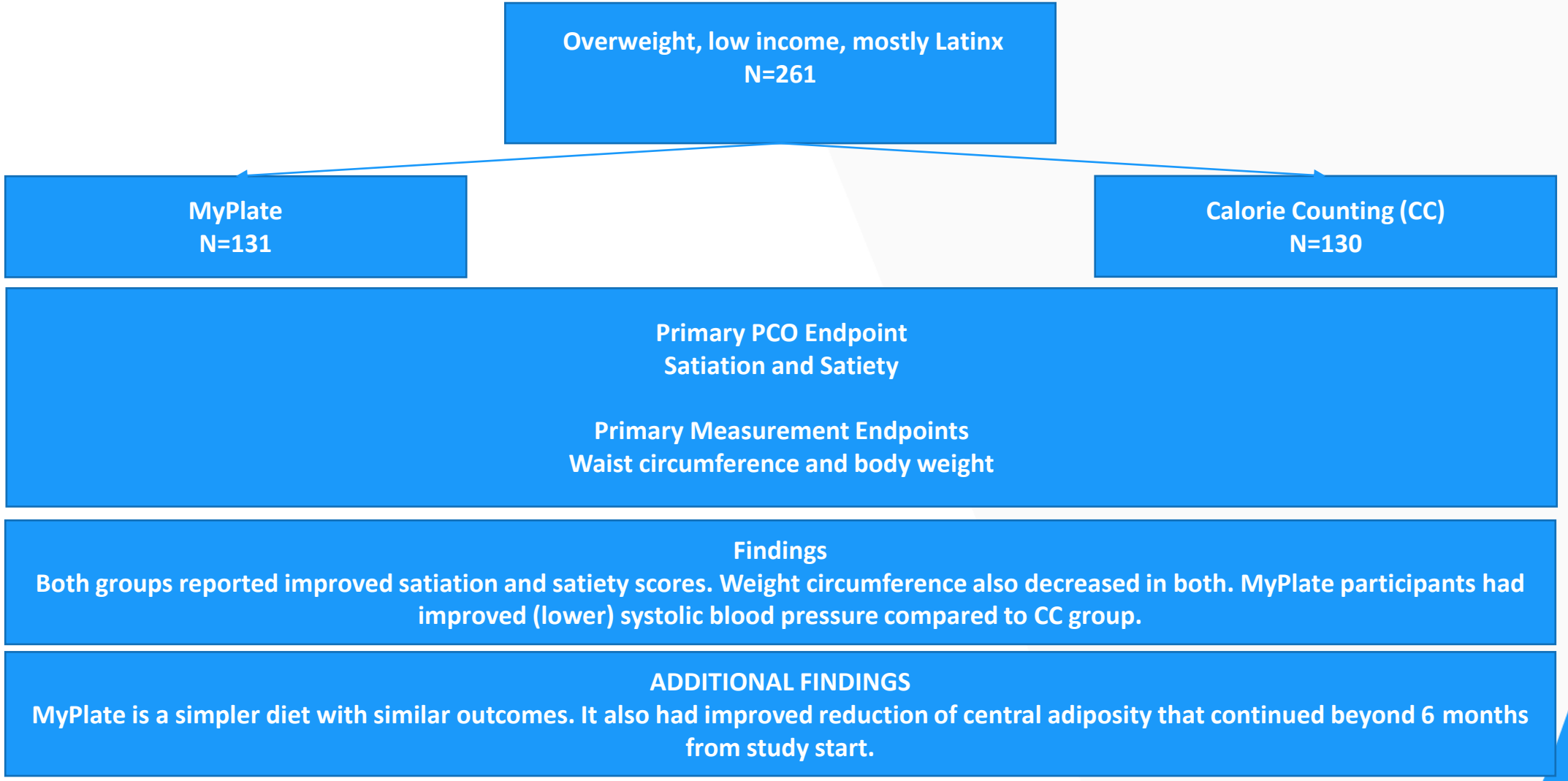
Diets: Which Evidence-Based Diet Works Best?

- We recommend “healthy lifestyle” to our patients which includes diet and activity for many different types of chronic diseases or to reduce risk. However, with so many evidence-based diets, which one works best for which disease, patient population?

Example of Recommended Diets:

- Dietary Approaches to Stop Hypertension (DASH)
- Mediterranean Diet
- MyPlate
- Centers for Disease Control and Prevention National Diabetes Prevention Program’s Calorie Counting (CC)

Comparative Effectiveness Trial - Diets



McCarthy et al. Ann Fam Med 2023;21:213-219.

Translating the Data to Patient-Centered Care

Clinician: “Following a healthy diet is important for reducing your longer-term risk for disease, improve your overall health, and may help with weight control.”

Patient: “Will it be hard to follow? What one lowers my weight more?”

Clinician: “A study of two diets, MyPlate and Calorie Counting showed they both reduce weight. However, the MyPlate diet is a simpler one to follow and may provide more central (abdominal) circumference (size) reduction.”

Discuss Results of PCORI Funded CER Trial

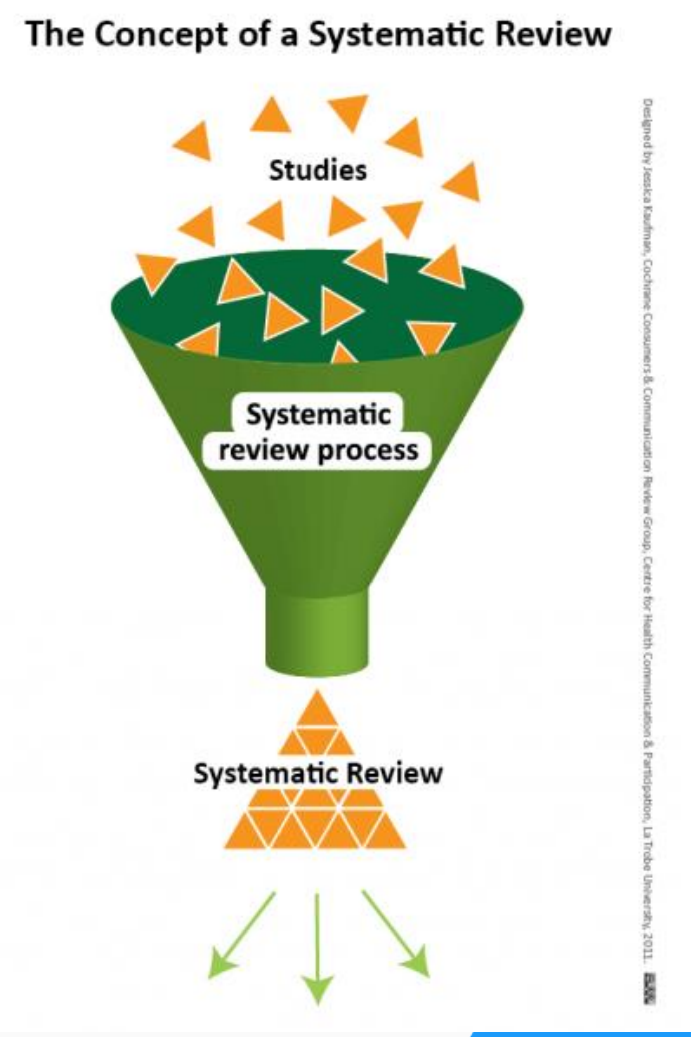
Randomized Comparative Effectiveness Trial of 2 Federally Recommended Strategies to Reduce Excess Body Fat in Overweight, Low-Income Patients: MyPlate.gov vs Calorie Counting | Annals of Family Medicine (annfammed.org)

McCarthy et al. Ann Fam Med 2023;21:213-219.

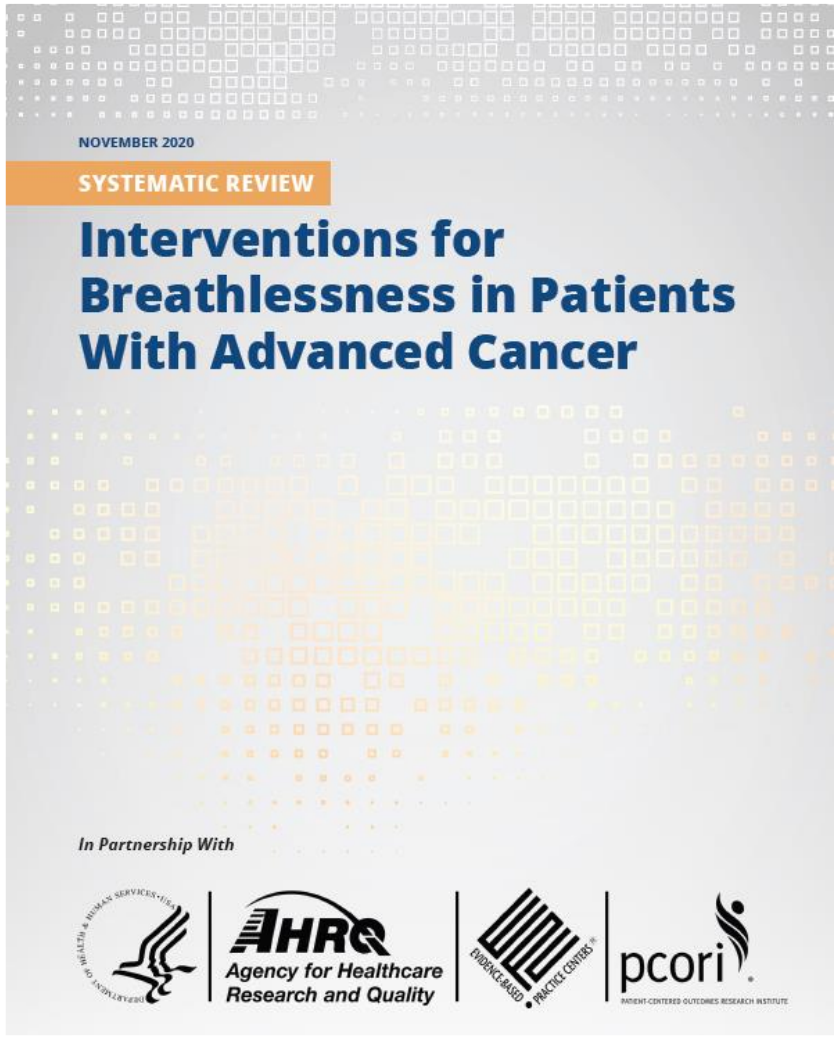
Getting Research Into Practice

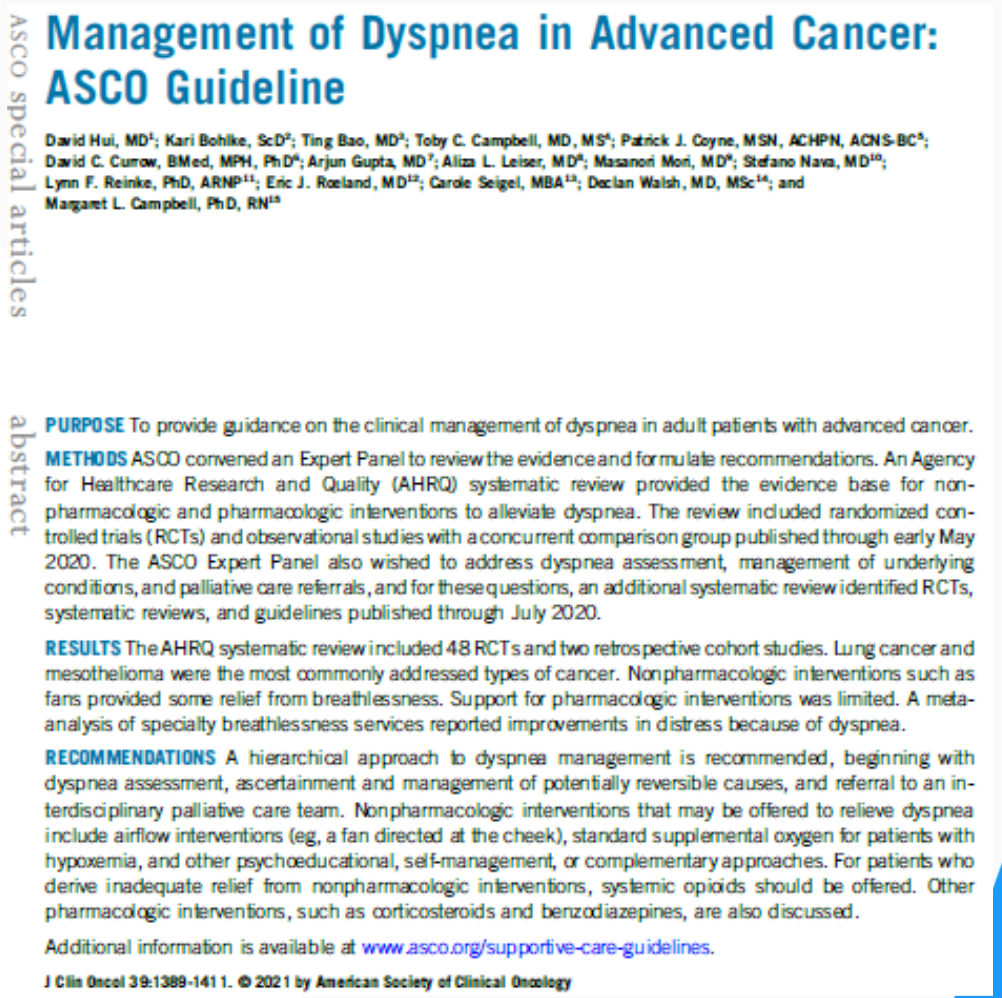
PCORI-Funded AHRQ Systematic Reviews

- **Systematic Reviews** use methodologically rigorous methods to produce a synthesis of the evidence
- **Primary Use:** Inform guidelines and evidence-based clinical practice
- **Nominating Partners:** Guideline writing organizations
- **Agency for Healthcare and Research (AHRQ)/PCORI Collaboration**



From Evidence Synthesis to Evidence-Based Guidelines





asco special articles

Management of Dyspnea in Advanced Cancer: ASCO Guideline

David Hui, MD[1]; Kari Bohlke, ScD[2]; Ting Bao, MD[3]; Toby C. Campbell, MD, MS[4]; Patrick J. Coyne, MSN, ACHPN, ACNS-BC[5]; David C. Currow, BMed, MPH, PhD[6]; Arjun Gupta, MD[7]; Aliza L. Leiser, MD[8]; Masanori Mori, MD[9]; Stefano Nava, MD[10]; Lynn F. Reinke, PhD, ARNP[11]; Eric J. Roeland, MD[12]; Carole Seigel, MBA[13]; Declan Walsh, MD, MSc[14]; and Margaret L. Campbell, PhD, RN[15]

abstract

PURPOSE To provide guidance on the clinical management of dyspnea in adult patients with advanced cancer.

METHODS ASCO convened an Expert Panel to review the evidence and formulate recommendations. An Agency for Healthcare Research and Quality (AHRQ) systematic review provided the evidence base for nonpharmacologic and pharmacologic interventions to alleviate dyspnea. The review included randomized controlled trials (RCTs) and observational studies with a concurrent comparison group published through early May 2020. The ASCO Expert Panel also wished to address dyspnea assessment, management of underlying conditions, and palliative care referrals, and for these questions, an additional systematic review identified RCTs, systematic reviews, and guidelines published through July 2020.

RESULTS The AHRQ systematic review included 48 RCTs and two retrospective cohort studies. Lung cancer and mesothelioma were the most commonly addressed types of cancer. Nonpharmacologic interventions such as fans provided some relief from breathlessness. Support for pharmacologic interventions was limited. A meta-analysis of specialty breathlessness services reported improvements in distress because of dyspnea.

RECOMMENDATIONS A hierarchical approach to dyspnea management is recommended, beginning with dyspnea assessment, ascertainment and management of potentially reversible causes, and referral to an interdisciplinary palliative care team. Nonpharmacologic interventions that may be offered to relieve dyspnea include airflow interventions (eg, a fan directed at the cheek), standard supplemental oxygen for patients with hypoxemia, and other psychoeducational, self-management, or complementary approaches. For patients who derive inadequate relief from nonpharmacologic interventions, systemic opioids should be offered. Other pharmacologic interventions, such as corticosteroids and benzodiazepines, are also discussed.

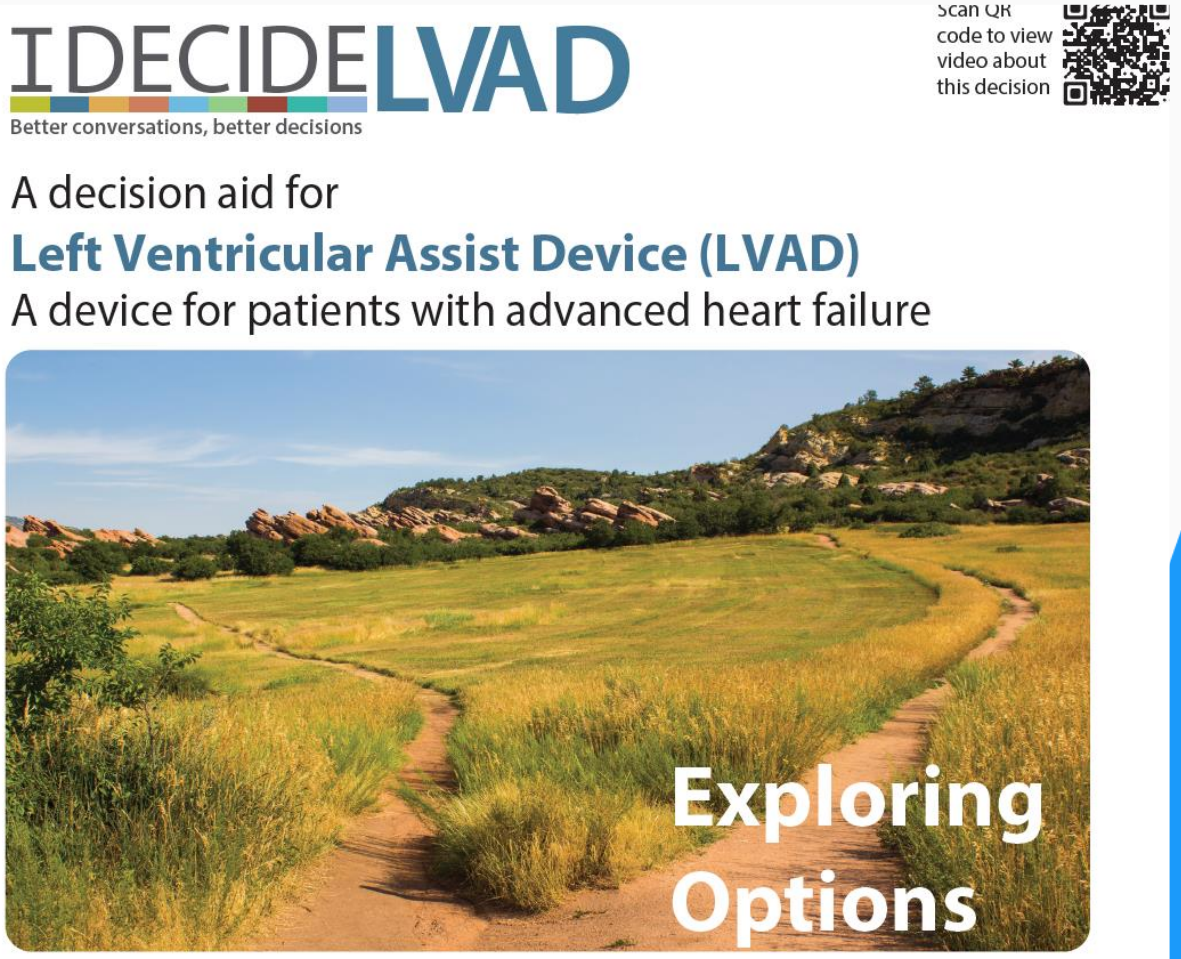
Additional information is available at www.asco.org/supportive-care-guidelines.

J Clin Oncol 39:1389-1411. © 2021 by American Society of Clinical Oncology

Source: https://ascopubs.org/doi/10.1200/JCO.20.03465

Shared Decision Making (SDM)

. . .where clinicians and patients share the best available evidence when faced with the task of making decisions, and where patients are supported to consider options, to achieve informed preferences *(Elwyn, et al. BMJ. 2010; 341: c5146.)*



Source: https://patientdecisionaid.org/lvad/

Providing Evidence and Supporting Decisions

JAMA | Original Investigation

Association Between Radiation Therapy, Surgery, or Observation for Localized Prostate Cancer and Patient-Reported Outcomes After 3 Years

Daniel A. Barocas, MD, MPH; JoAnn Alvarez, MA; Matthew J. Resnick, MD, MPH; Tatsuki Koyama, PhD; Karen E. Hoffman, MD, MHSc, MPH; Mark D. Tyson, MD; Ralph Conwill, BS; Dan McCollum, BS; Matthew R. Cooperberg, MD, MPH; Michael Goodman, MD, MPH; Sheldon Greenfield, MD; Ann S. Hamilton, PhD, MA; Mia Hashibe, PhD, MPH; Sherrie H. Kaplan, PhD, MS, MPH; Lisa E. Paddock, PhD, MPH; Antoinette M. Stroup, PhD; Xiao-Cheng Wu, MD, MPH; David F. Penson, MD, MPH

JAMA Internal Medicine | Original Investigation

Effectiveness of an Intervention Supporting Shared Decision Making for Destination Therapy Left Ventricular Assist Device
The DECIDE-LVAD Randomized Clinical Trial

Larry A. Allen, MD, MHS; Colleen K. McIlvennan, DNP, ANP; Jocelyn S. Thompson, MA; Shannon M. Dunlay, MD, MS; Shane J. LaRue, MD, MPHS; Eldrin F. Lewis, MD, MPH; Chetan B. Patel, MD; Laura Blue, DNP, ANP; Diane L. Fairclough, PhD; Erin C. Leister, MS; Russell E. Glasgow, PhD; Joseph C. Cleveland Jr., MD; Clifford Phillips; Vicie Baldridge; Mary Norine Walsh, MD; Daniel D. Matlock, MD, MPH

Getting SDM Into Practice

Implementation Project (Implementation of Effective Shared Decision Making Approaches PFA)	In progress; Recruitment not applicable

Improving Shared Decision Making for Men with Prostate Cancer That Has Not Spread

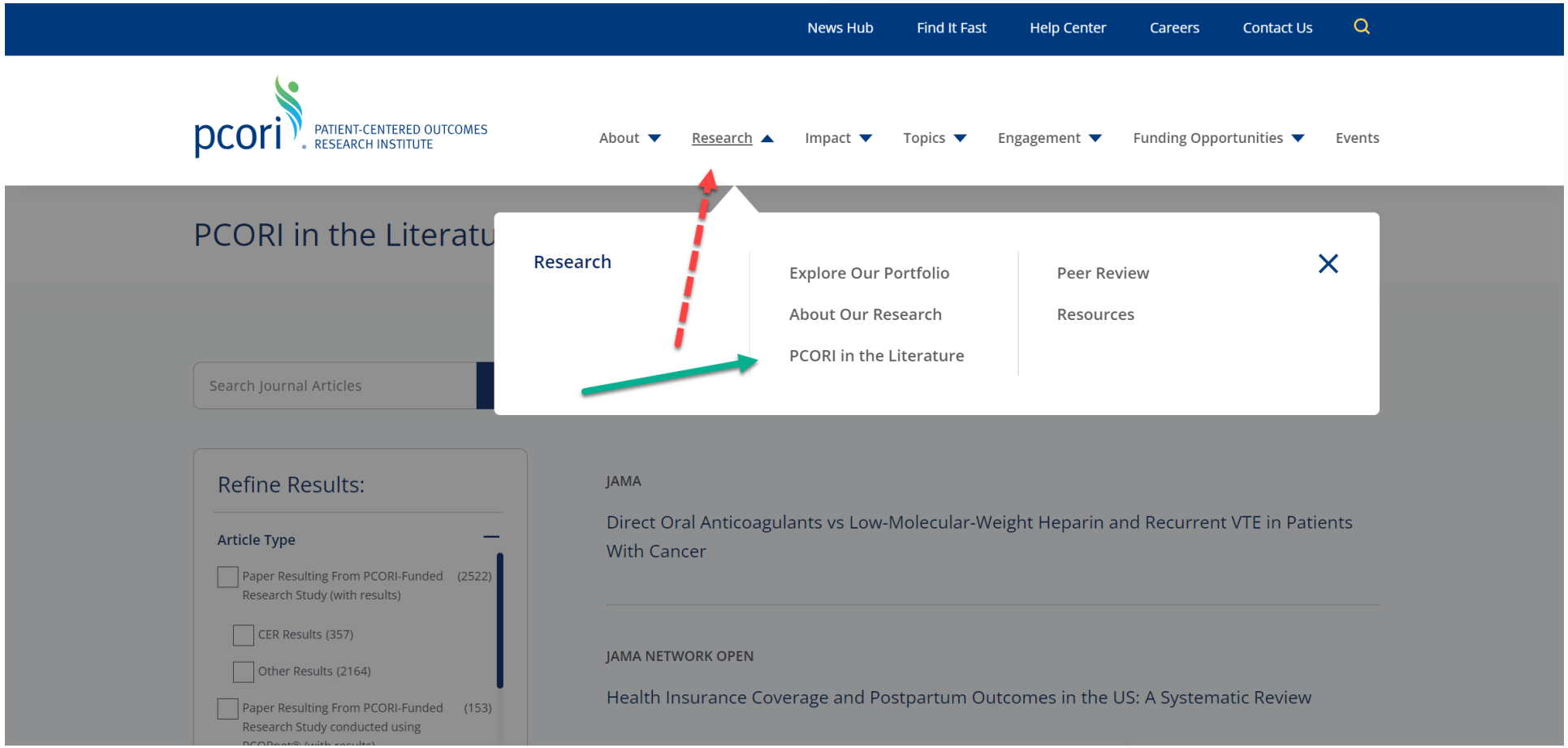
Implementation Project (Implementation of Effective Shared Decision Making Approaches PFA)	In progress; Recruitment not applicable

Expanding a Shared Decision Making Program for Patients Considering LVAD Treatment

https://www.pcori.org/research-results/2018/improving-shared-decision-making-men-early-stage-prostate-cancer

Finding Data to Support Implementation of Patient-Centered Approaches, Shared Decision Making

[PCORI in the Literature | PCORI]



Dissemination and Implementation (D&I) of Results

- We are committed to extending the pathway from PCORI-funded research through dissemination and implementation – to assure that research findings are used to improve health care and health
  - Enhancing awareness of evidence useful to people and organizations as they make health decisions
  - Speeding the integration of this evidence into practice

Evidence Updates

pcori | OAC Obesity Action Coalition

SEPTEMBER 2021

EVIDENCE UPDATE

Comparing Two Common Types of Weight Loss Surgery

Weight loss surgery, also called bariatric surgery, helps people with obesity lose weight. A recent study compared the benefits and harms of two common types of weight loss surgery.

Findings

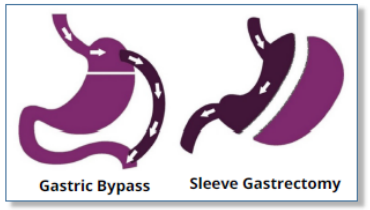
A PCORI-funded study found that people lost more weight with gastric bypass than with sleeve. But more people who had gastric bypass needed additional surgeries and hospitalizations than those who had sleeve.

Among patients with type 2 diabetes, about the same number of people no longer had to take medicine for their diabetes with both surgeries. However, more people with sleeve had their diabetes return.

Some adults with obesity are not able to lose weight with diet and exercise alone. Surgery can help people lose weight and improve problems related to obesity, like diabetes. But surgery can also cause harm.

Two of the most common types of weight loss surgery are:

- **Roux-en-Y gastric bypass, or gastric bypass.** In this surgery, a surgeon uses part of the stomach to create a pouch that is attached to the small intestine. Instead of food going into the person's stomach, it goes to the pouch and then into the small intestine.
- **Sleeve gastrectomy, or sleeve.** In this surgery, a surgeon removes a large part of the stomach. This turns the stomach into a narrow tube, or sleeve.



The study also included a third type of surgery called adjustable gastric banding, or lap band. This update does not include findings related to lap band because many weight loss surgery centers no longer perform this surgery.

pcori | OAC Obesity Action Coalition

SEPTEMBER 2021

EVIDENCE UPDATE

Comparing the Benefits and Harms of Bariatric Procedures

A recent study documented differences in the benefits and harms of two common types of bariatric surgery. The findings can help clinicians and patients work together to make informed decisions regarding patient care.[1, 2]

Obesity is associated with a range of comorbidities including type 2 diabetes mellitus (T2DM). Bariatric surgery may be a viable treatment for patients with a body mass index (BMI) of 35 or greater who are unable to lose weight through diet and exercise alone. However, outcomes vary across procedures.

A PCORI-funded study compared the benefits and harms of the two most common types of weight loss surgery: Roux-en-Y gastric bypass (RYGB) and sleeve gastrectomy (SG). The study included a third type of surgery, adjustable gastric banding. Because this surgery is no longer commonly used, the results are not included in this Evidence Update.

Findings

A PCORI-funded study found that both procedures resulted in weight loss and remission of T2DM for the majority of patients. Overall, RYGB had better total weight loss results and a more significant impact on increasing T2DM remission and improving glycemic control compared with SG. However, RYGB was also associated with a higher rate of adverse outcomes such as additional abdominal surgeries and rehospitalization.

Comparing the Benefits

Weight Loss

Patients in the RYGB cohort saw a higher percentage of total weight loss over a five-year period following surgery than patients in the SG cohort.[3] Patients in both cohorts experienced some level of weight gain after their initial weight loss.

Average Percent of Total Weight Loss Following Surgery (Compared with Pre-Surgery Weight)	RYGB	SG
Average weight lost in first year	31%	25%
Average weight lost at five years	26%	19%

Type 2 Diabetes Outcomes

RYGB and SG resulted in clinically comparable T2DM remission rates throughout the five-year period following surgery.[4] Remission is defined as HbA1c under 6.5% after six months without a diabetes medication prescription. Most T2DM remission occurred within two years of surgery. The risk of relapse was 25% lower for patients who had RYGB compared with patients who had SG.

Percent of Cohort Experiencing Type 2 Diabetes Remission and Relapse	RYGB	SG
T2DM remission during five-year period post-surgery	86%	84%
T2DM relapse during five-year period post-surgery	33%	42%

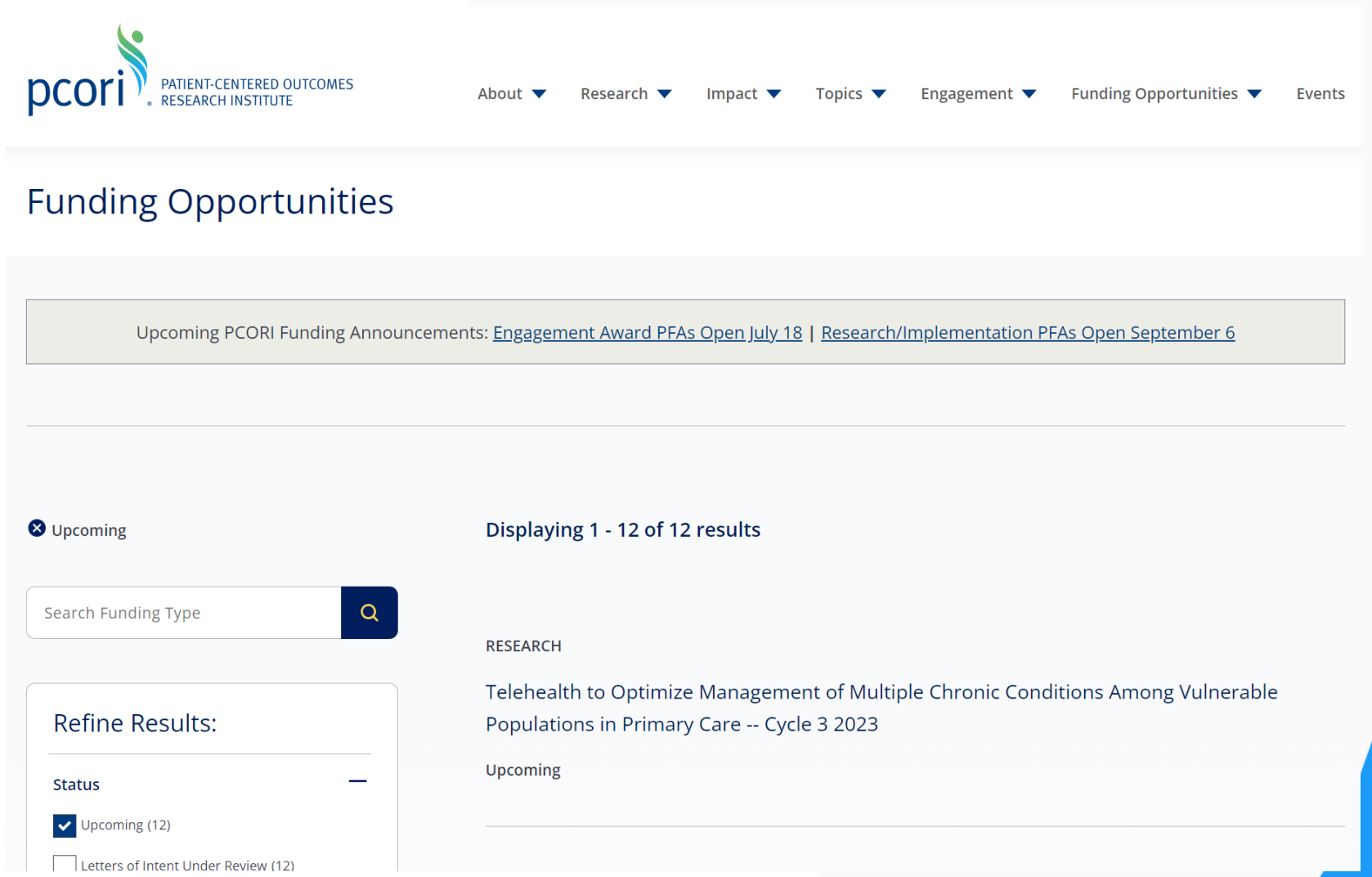
https://www.pcori.org/impact/evidence-updates

Interested in PCOR? How to Get Involved

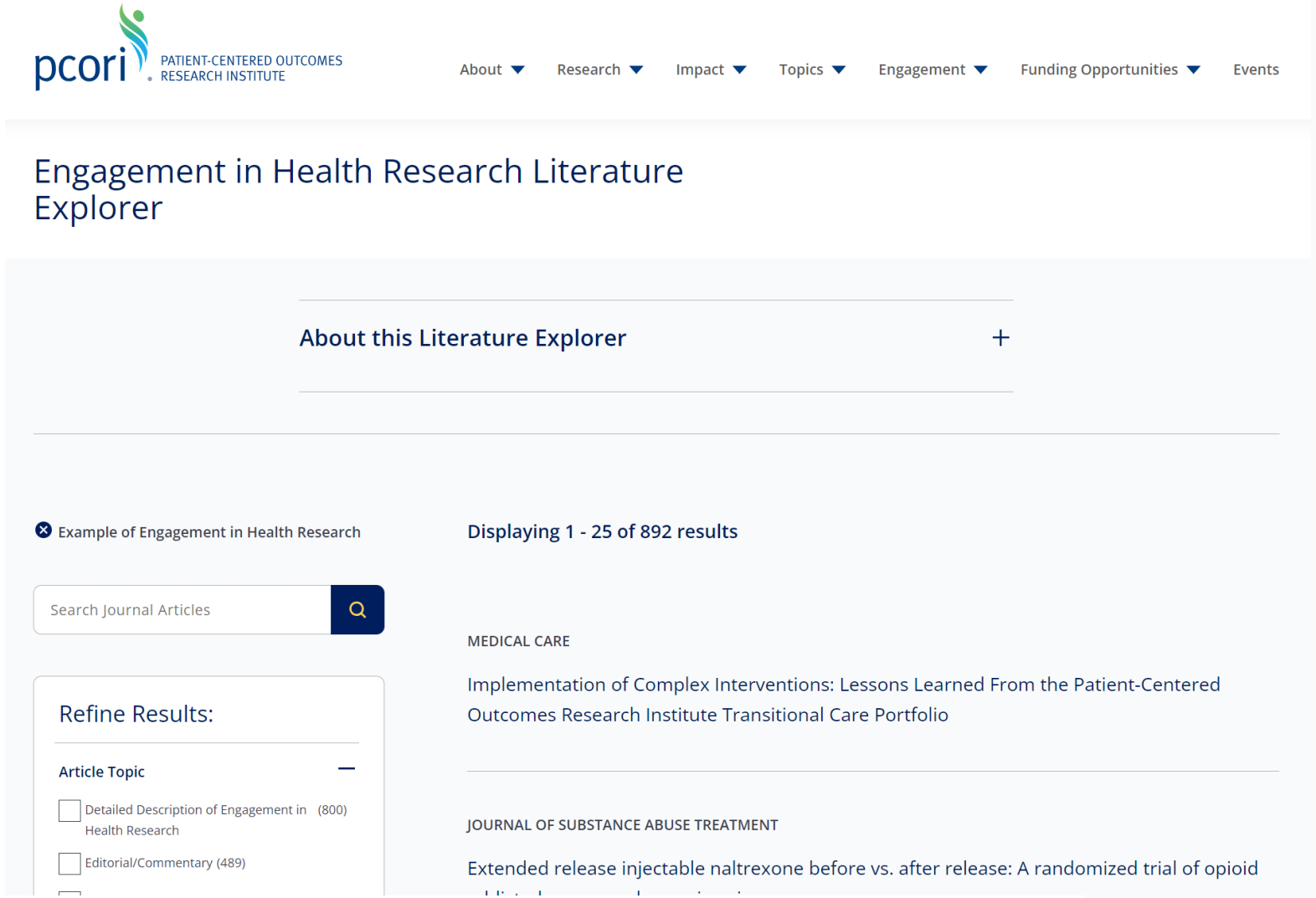
PCORI website

www.pcori.org

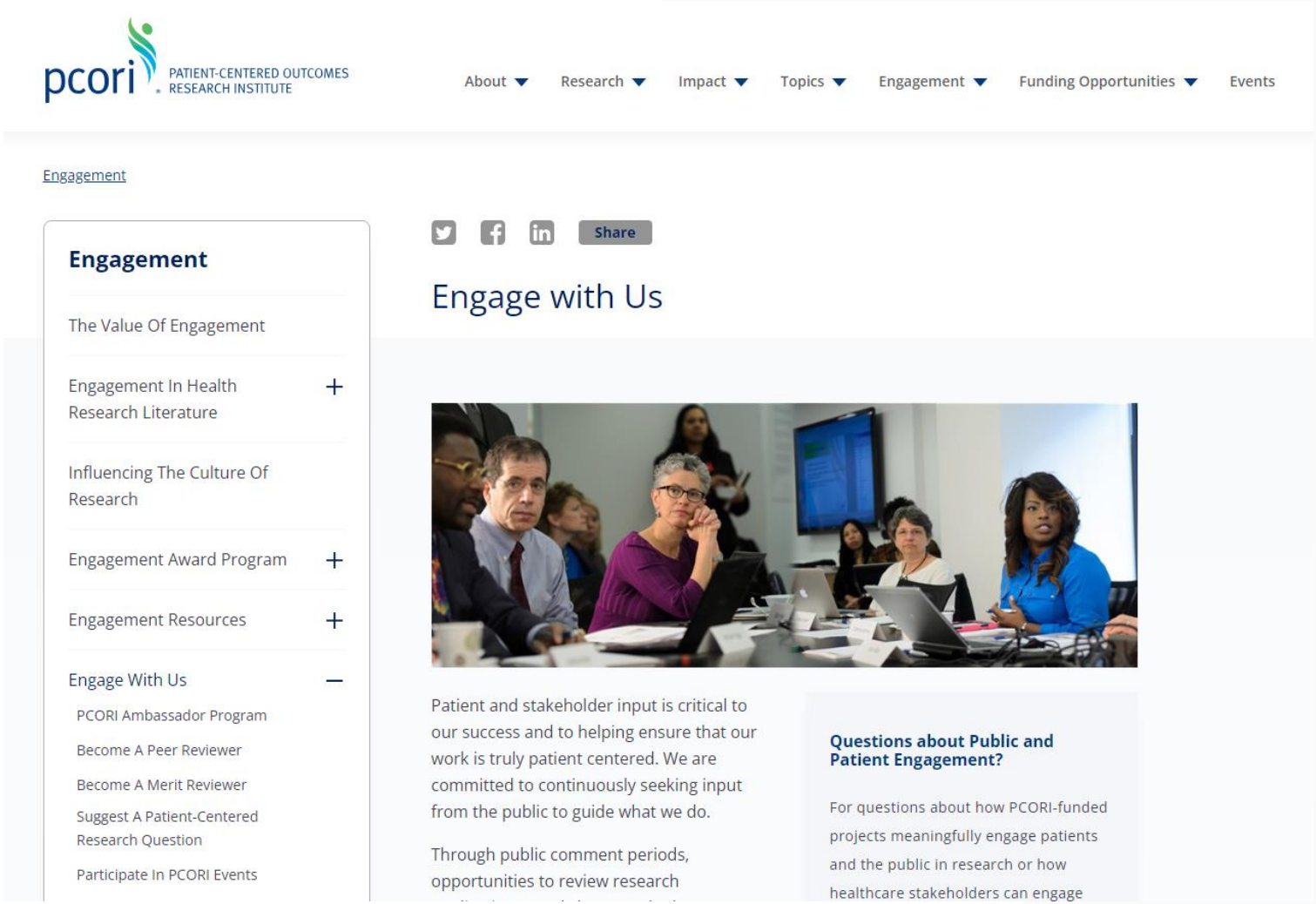
for research funding opportunities



Getting involved: Engagement in the Research Literature



Getting Involved With PCORI

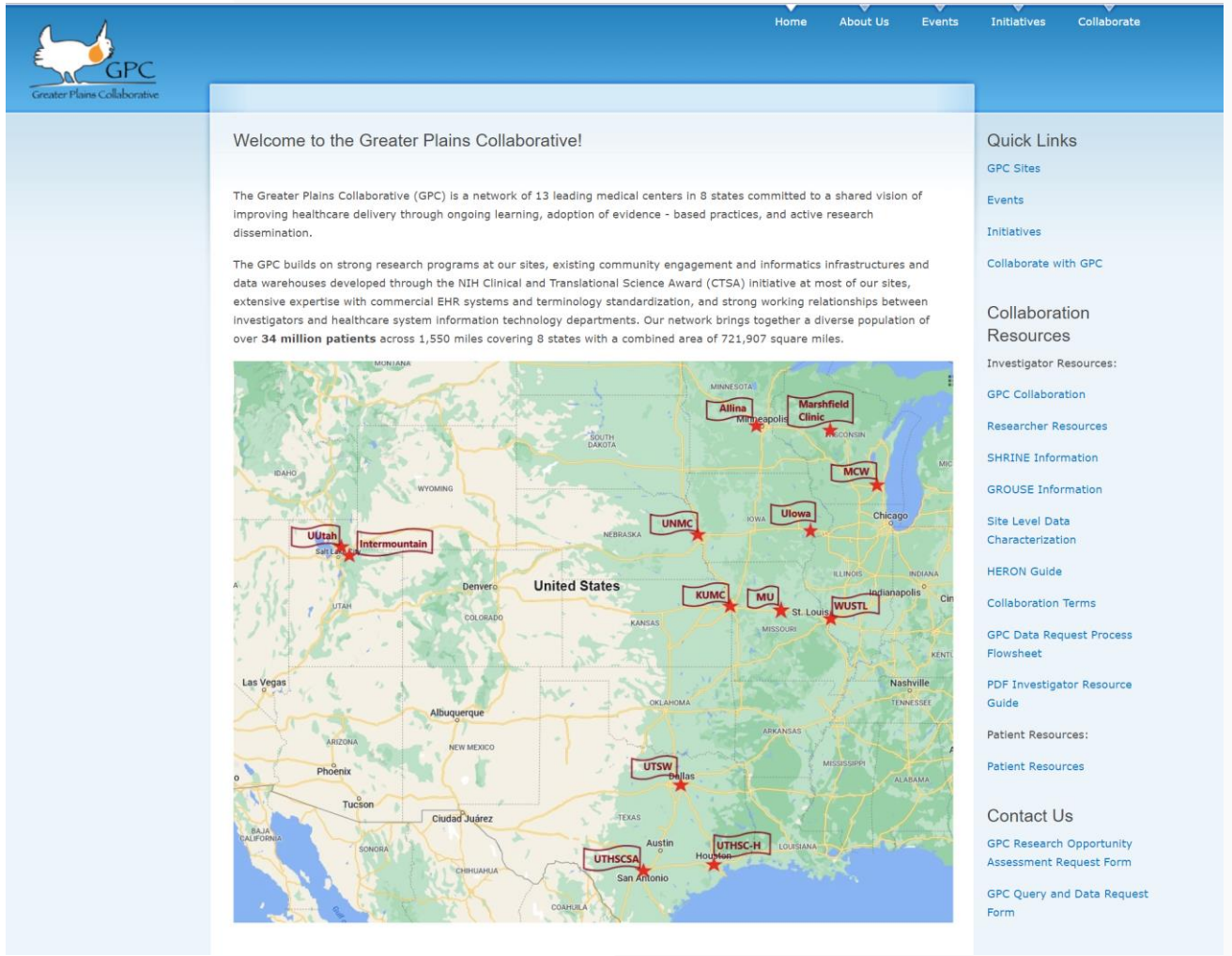


PA Engagement

I am a Patient Engagement Officer (PEO) for the Intermountain site of the Greater Plains Collaborative (GPC) – gpcnetwork.org

GPC represents a network of 13 medical centers (see picture)

This collaborative allows for aggregation of patient data (34 million patients), to research disease, therapies, disparities, etc. and compare effectiveness of therapies and approaches



Summary

- PCOR/CER aims to generate effectiveness information focusing on informing decisions
  - Take known and existing therapies and understand which are more applicable and effective under a patient-centered lens
  - Intentionally invite patient perspectives into study designs, treatment choices, and including outcomes that are most important to them
- Getting research into practice:
  - Getting the evidence into the hands of those who need it (In a form they understand!)
  - Intervention to speed the adoption of the evidence into practice

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