



# Patient-Centered Outcomes Research

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# PCORI

## Patient-Centered Outcomes Research Institute

About PCORI	Goal	Research Focus	DSF Projects Funded
<ul style="list-style-type: none"><li>• Independent (non-governmental)</li><li>• Non-profit</li><li>• Appointed by Congress in 2010</li></ul>	<ul style="list-style-type: none"><li>• Help patients compare healthcare options given their preferences</li></ul>	<ul style="list-style-type: none"><li>• Patient Engagement in research design and implementation</li><li>• Comparative Effectiveness Research (CER) that compares <math>\geq</math> two known treatments based on patient priorities</li><li>• CER Methods and Infrastructure (PCORnet, electronic databases)</li></ul>	<ul style="list-style-type: none"><li>• Pipeline to Proposal Tier I (\$15k, 1 yr)</li><li>• Pipeline to Proposal Tier II (\$25k, 1 yr)</li><li>• Eugene Washington Engagement Award (\$244k, 2 yrs)</li></ul>

# What is PCOR?

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**Patient-Centered Outcomes Research (PCOR):** Research that helps patients/caregivers communicate and make informed health care decisions, allowing their voices to be heard in assessing the value of health care options.

## Examples

Given two particular medications approved for Dravet syndrome, which is best for my child at this point in time?

What types of behavioral therapies work best in Dravet syndrome?

What are the optimal components of a good transition of care program?

# What is PCOR?

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## Patient-Centered Outcomes Research

- Assesses the benefits and harms of treatments to inform decision making
- Looks at comparisons and outcomes that matter to patients, such as symptoms and quality of life
- Is inclusive of an individual's preferences and needs
- Incorporates a wide variety of settings and diversity of participants to address individual differences and barriers
- Investigates optimizing outcomes while addressing the burdens to patients

# Why PCOR?

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PCORI wants to see research that is designed to answer the questions patients have because:

- This is what is most important to patients
- These projects are more likely to succeed because patients want to be involved

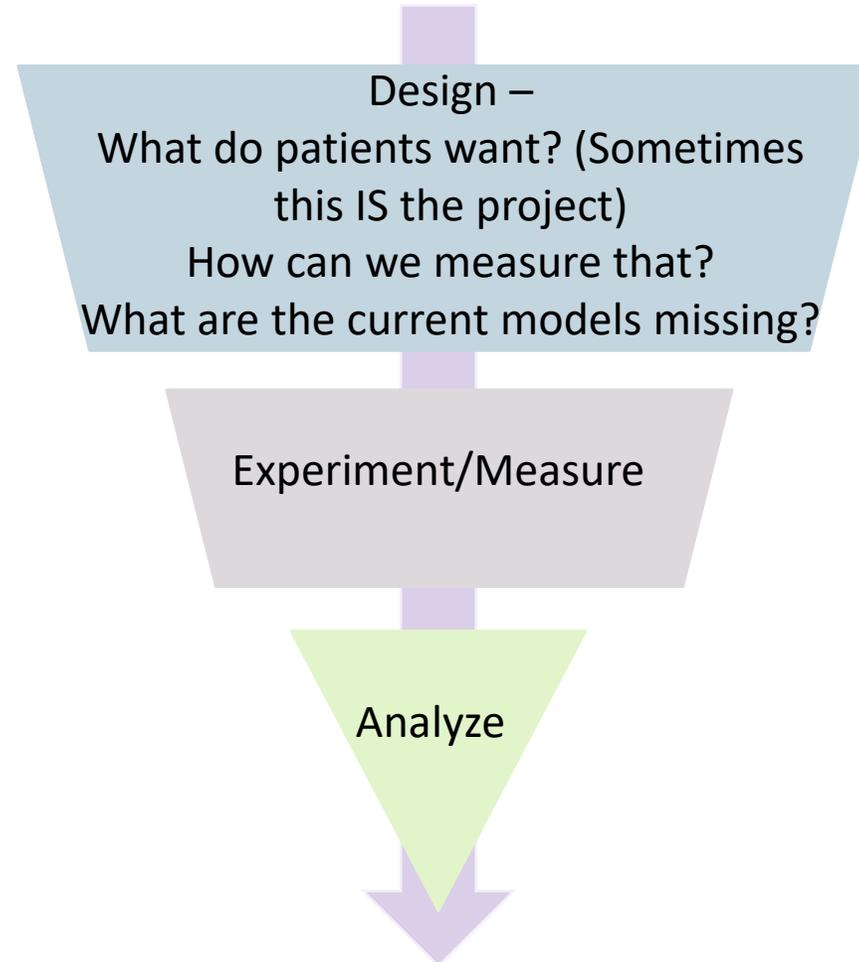
# PCOR vs. Traditional Research

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## Traditional



## PCOR



# What is CER?

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**Comparative Effectiveness Research (CER)** = The *direct comparison* of existing health care interventions to determine which work best for which patients and which pose the greatest benefits and harms

## Examples

Given two particular medications approved for Dravet syndrome, which is best for my child at this point in time?

~~What types of behavioral therapies work best in Dravet syndrome?~~  
Does ABA or Cognitive Behavioral Therapy work better in patients ages 5-10 with Dravet syndrome?

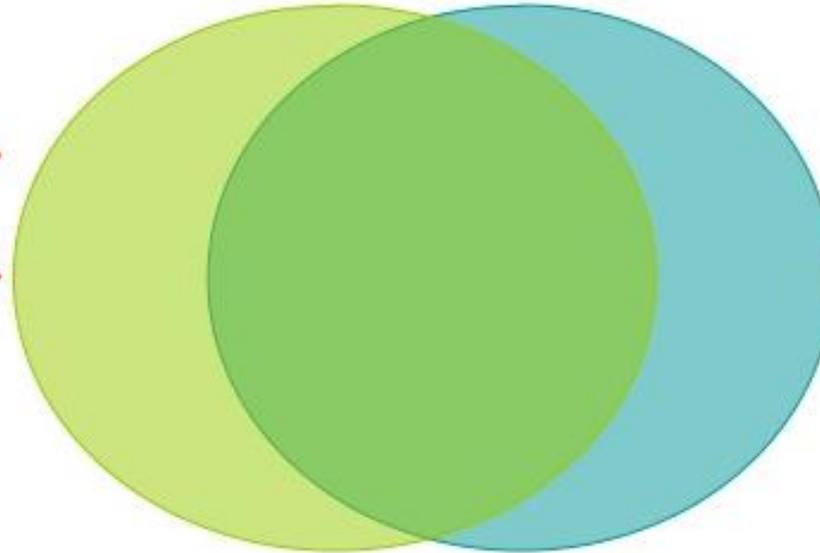
~~What are the optimal components of a good transition of care program?~~  
Which existing transition of care program works best in DS?

# CER vs. PCOR: Is there a difference?

**Comparative Effectiveness Research**

**Patient-Centered Outcomes Research**

Comparisons →  
No patient reported data →



← Not comparative  
← Patient reported outcomes

Subgroups

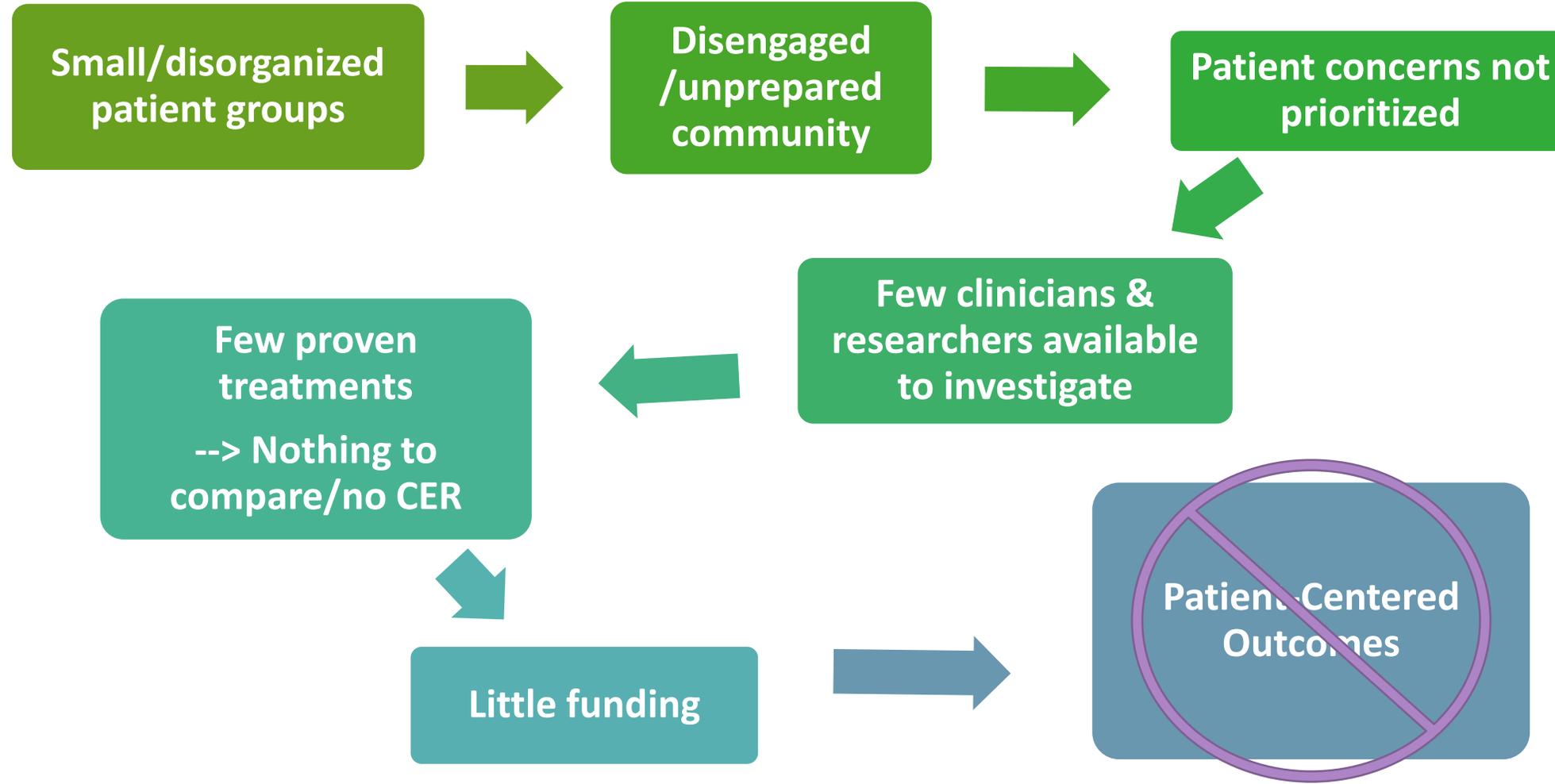
# CER in Dravet syndrome

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Some of the (CER) research questions that were developed:

- Which is better to improve sleep – melatonin or clonidine?
- How can we improve the duration of sleep?
- What is the best approach to improving nutrition?
- Is ABA Therapy or medical management more effective for addressing behavior?
- What is the best way to manage status epilepticus?
- What is the best way to talk to families about the risk of SUDEP?
- What is the best management for constipation?
- Does increasing L-Dopamine help with gait?

# Challenges in Rare Diseases



# Progress so far

Project	Focus	Results	Next Steps
DSF Family Network, 2016	Organizing the patient community	>2000 registered families, accessible to researchers	Engage 5 US Regions
Caregiver Concerns Survey, 2016	Identifying caregiver concerns	Seizure control >Communication >Sibling Impact >Cognition/Delay >Behavior	PCOR Workshops at Conference and DoD
Pipeline to Proposal (P2P), Tier I (\$15k)	Engaging stakeholders, prioritizing caregiver concerns	Categories of concern: SUDEP, Gait, Behavior, Nutrition/GI, Seizures/SE, Sleep	→P2P Tier II
P2P, Tier II (\$25k)	What categories lend themselves to CER?	Tools to measure behavior are lacking, few proven treatments for CER	→Working Group/ Stakeholder Advisory Group
Eugene Washington Engagement Award (\$244k)	Preparing patient community and researchers for PCOR/CER	TBD based on: <ul style="list-style-type: none"> <li>• SCC Meeting</li> <li>• PCORI sessions at conference</li> <li>• DoD Workshops</li> <li>• Webinars</li> </ul>	

# Panel and Q&A

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Michelle Welborn, parent of Lily

Linda Laux, MD, Lurie Children's Hospital

Anne Berg, PhD, Lurie Children's Hospital

# Conversation continues tomorrow...

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- Sunday morning session is 2 hours long, filled mainly with discussion groups broken up by age of patient.
- Attendees will receive \$35 gift cards for participation in Sunday's session.

Thank you!

