Patient-Centered Outcomes Research Institute

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<th>About PCORI</th>
<th>Goal</th>
<th>Research Focus</th>
<th>DSF Projects Funded</th>
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| • Independent (non-governmental) | • Help patients compare healthcare options given their preferences | • Patient Engagement in research design and implementation  
• Comparative Effectiveness Research (CER) that compares ≥ two known treatments based on patient priorities  
• CER Methods and Infrastructure (PCORnet, electronic databases) | • Pipeline to Proposal Tier I ($15k, 1 yr)  
• Pipeline to Proposal Tier II ($25k, 1 yr)  
• Eugene Washington Engagement Award ($244k, 2 yrs) |
| • Non-profit |          |                |                     |
| • Appointed by Congress in 2010 |          |                |                     |
What is PCOR?

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?

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?

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

Traditional

Design – What should/can we measure?

Experiment/Measure

Analyze

PCOR

Design – What do patients want? (Sometimes this IS the project)
How can we measure that?
What are the current models missing?

Experiment/Measure

Analyze
What is CER?

**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
- Comparisons
- No patient reported data

Patient-Centered Outcomes Research
- Not comparative
- Patient reported outcomes

Subgroups

Linkedin Learning Slideshare, AcademyHealth: 2011 www.slideshare.net/academyhealth/matching-the-research-design-to-the-study-question
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

Small/disorganized patient groups

Disengaged/unprepared community

Patient concerns not prioritized

Few proven treatments

--> Nothing to compare/no CER

Few clinicians & researchers available to investigate

Little funding

Patient Centered Outcomes
## Progress so far

<table>
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<tr>
<th>Project</th>
<th>Focus</th>
<th>Results</th>
<th>Next Steps</th>
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<tbody>
<tr>
<td>DSF Family Network, 2016</td>
<td>Organizing the patient community</td>
<td>&gt;2000 registered families, accessible to researchers</td>
<td>Engage 5 US Regions</td>
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<tr>
<td>Caregiver Concerns Survey, 2016</td>
<td>Identifying caregiver concerns</td>
<td>Seizure control &gt;Communication &gt;Sibling Impact &gt;Cognition/Delay &gt;Behavior</td>
<td>PCOR Workshops at Conference and DoD</td>
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<tr>
<td>Pipeline to Proposal (P2P), Tier I ($15k)</td>
<td>Engaging stakeholders, prioritizing caregiver concerns</td>
<td>Categories of concern: SUDEP, Gait, Behavior, Nutrition/GI, Seizures/SE, Sleep</td>
<td>→P2P Tier II</td>
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<tr>
<td>P2P, Tier II ($25k)</td>
<td>What categories lend themselves to CER?</td>
<td>Tools to measure behavior are lacking, few proven treatments for CER</td>
<td>→Working Group/ Stakeholder Advisory Group</td>
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<td>Eugene Washington Engagement Award ($244k)</td>
<td>Preparing patient community and researchers for PCOR/CER</td>
<td>TBD based on: • SCC Meeting  • PCORI sessions at conference  • DoD Workshops  • Webinars</td>
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Panel and Q&A

Michelle Welborn, parent of Lily

Linda Laux, MD, Lurie Children’s Hospital

Anne Berg, PhD, Lurie Children’s Hospital
Conversation continues tomorrow...

- 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!