Systematic Review: Management Strategies for Infantile Epilepsy

A PCORI Virtual Multi-Stakeholder Workshop

October 8, 2020

PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE

Welcome

And thank you for participating!

Agenda

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- Housekeeping
- Introductions
- Background and Your Perspective
- Prepared Comments
- Moderated Discussion
- Summary and Closing Remarks

Housekeeping

- Participants’ lines are live – please mute your line when you are not speaking
- Today’s conversation is being recorded and will be posted to the PCORI website
- During the Prepared Comment period, we will take stakeholder comments in the order indicated
- If you wish to speak during the Moderated Discussion period, please indicate that you have a comment in the chat box
- Please introduce yourself when you begin to speak
- Additionally, comments and questions from participants may be submitted via the chat window

Introductions

Introductions

Today's PCORI Representatives:

- Bill Lawrence, MD, MS, Senior Clinical Advisor, Engagement, Office of the Chief Engagement and Dissemination Officer
- Jennie Dalton Bowen, MPH, Program Officer, Research Synthesis and New Technology

Stakeholder Outreach Coordinator:

- Tara Lucian, MPH, Program Associate, Public and Patient Engagement

About PCORI

PCORI helps people make informed healthcare decisions, and improves 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.**

Our Strategic Goals:

- Increase quantity, quality, and timeliness of useful, trustworthy research information available to support health decisions
- Speed the implementation and use of patient-centered outcomes research evidence
- Influence research funded by others to be more patient-centered

Today’s Participants

American Epilepsy Society
Clinician, Researcher

Bridge the Gap
Patient advocacy

Children’s Hospital Colorado
Clinician

DEE-P Connections
Caregiver

Dravet Syndrome Foundation
Caregiver

Epilepsy Foundation of Minnesota
Caregiver, Patient advocacy

Epilepsy Foundation of San Diego
Caregiver

International Foundation for CDKL5 Research
Patient advocacy

LGS Foundation
Researcher

National Association of Pediatric Nurse Practitioners
Clinician

Pediatric Epilepsy Research Foundation
Research funder

TESS Research Foundation
Caregiver

Tuberous Sclerosis Alliance
Patient advocacy

University of Colorado
Clinician

University of Utah
Clinician

Wishes for Elliott
Caregiver

Background and Your Perspective

Background

PCORI is partnering with the **Agency for Healthcare Research and Quality (AHRQ)** to develop a systematic evidence review on strategies for managing epilepsy in infants and young children (0-3), a topic nominated by the **American Epilepsy Society (AES)**.

Goals

- Support the possible development of an evidence-based guideline
- Summarize existing research to support parents and clinicians in treatment decisions
- Identify key areas for future clinical studies

What is a systematic review?

- A systematic review is a way to summarize what existing research says about a given topic.
- They are conducted using rigorous methods.
- The results can have many potential uses:
    - Informing clinical guidelines
    - Helping patients and clinicians with treatment decisions
    - Identifying areas for future research
    - Support policy initiatives

Your Perspective

A systematic review is a powerful tool. We want it to be as useful as possible to patients, caregivers, clinicians, and decision makers.

We need your personal and professional expertise to do this.

Proposed Systematic Review Questions

Key Questions

1. What is the effectiveness and comparative effectiveness of pharmacologic treatments for infantile epilepsy?
2. What is the effectiveness and comparative effectiveness of non-pharmacologic treatments for infantile epilepsy (i.e. resective or palliative surgeries; brain stimulation therapies; and dietary therapies), including comparisons to other non-pharmacologic and/or pharmacologic therapies?
3. What are the harms or comparative harms of treatments for infantile epilepsy?

*The official draft Key Questions, PICOTS, and analytic framework are posted on AHRQ's website:
https://effectivehealthcare.ahrq.gov/products/management-infantile-epilepsy/draft-key-questions

Population, Outcomes, Study Design

Population	• Children, birth to 3 years • Focal or generalized epilepsy • Subpopulations: baseline seizure severity/ frequency, history of previous treatment	Excludes • Febrile seizures • West Syndrome/infantile spasms • Seizures not attributed to epilepsy
Outcomes	• Sudden Unexpected Death in Epilepsy (SUDEP) • Time to seizure remission or reduction • Increase or decrease in medication • Neurodevelopmental milestones met • Functional performance (e.g. school) • Adverse events (infection, new neurological deficits, surgical complications, irritability, somnolence, dizziness, drug toxicity, etc.)	• All-cause mortality • Seizure freedom • Remission • Quality of life • General health status • Social function • Behavioral function • Cognitive function
Study Design	Randomized controlled trials (RCT), controlled trials (CTs), cohorts, case-control studies, observational designs including pre-post and post-only	

Prepared Comments

Order of Prepared Comments Representative & Organization

William D. Gaillard, MD
American Epilepsy Society
President

Kevin Chapman, MD, FAES, FACNS
University of Colorado at Denver
Professor of Pediatrics and Neurology

Erin Fecske, DNP, CPNP
National Association of Pediatric Nurse Practitioners
Epilepsy Nurse Practitioner

Moderated Discussion

Quick reminders:
- Use the chat to indicate you have a question or comment
- Introduce yourself when you begin to speak

General

- Do these key questions capture the most crucial issues for parents/caregivers? Are there any important issues overlooked or neglected by these questions?
- In what ways do these questions capture the decisional dilemmas you face in caring for infants and young children with epilepsy? In what ways do they not?
- What should the authors of this systematic review consider as they prepare their research protocol?

Population

- How comfortable are you with extrapolating findings from older children to inform treatment of infantile epilepsy?
- Is it clinically appropriate to combine infants and young children with epilepsy (ages 0-3) into one study population for the purpose of a systematic review?
- Do you recommend changes to the inclusion/exclusion criteria for Population in the PICOTS table below?

Includes	Excludes
• Children, birth to 3 years • Focal or generalized epilepsy • Subpopulations: baseline seizure severity/ frequency, history of previous treatment	• Febrile seizures • West Syndrome/infantile spasms • Seizures not attributed to epilepsy

Treatments

- What is your perspective on the use of nonmedication treatments in this age group, including dietary therapies (e.g., ketogenic diets and variants) and surgery?
- What factors do you consider as a caregiver or a clinician when agreeing upon a course of treatment?
- What is the importance of stratifying treatment efficacy questions by seizure type, syndrome and/or etiology?
- Are there other issues related to treatment that we have not discussed?

Outcomes

- How do you measure the success of treatment?
- Which outcomes are most important to you?
- How do inequities and disparities in care affect treatment options and outcomes in infantile epilepsy?
- Are there other issues related to outcomes that we have not discussed?

- Seizure freedom
- Remission
- Time to seizure remission or reduction
- All-cause mortality
- Sudden Unexpected Death in Epilepsy (SUDEP)
- Increase or decrease in medication
- Quality of life
- Social function
- Behavioral function
- Cognitive function
- General health status
- Neurodevelopmental milestones met
- Functional performance (e.g. school)
- Adverse events (infection, new neurological deficits, surgical complications, irritability, somnolence, dizziness, drug toxicity, etc.)

Contextual Questions

1. What are the parental preferences for treatment options for infantile epilepsy?

2. What are the harms or comparative harms of not treating infantile epilepsy?

Trade-offs

- What are the harms or potential harms of treating (and not treating) infantile epilepsy that you are most concerned about?
- How do you balance the potential harms and benefits when weighing treatment options for the child or recommending a course of care?
- Are there other items we haven’t discussed that you consider when making treatment decisions?

Research Landscape

- What important research on infantile epilepsy is underway that you expect will be published in the next year or two?
- Are there emerging treatments or trends in treatments of which you are aware?
- What is the most needed clinical research on infantile epilepsy?

Summary and Closing Remarks

Contact Information

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Thank you!