Glut1 Deficiency Foundation

research ready SERIES

BUILDING A STRONG patient centered RESEARCH FOUNDATION

Overview of Modules:

Module 1: Developing Research Questions

Module 2: Designing the Research Study

Module 3: Designing Patient Centered Consent and Study Protocols

Module 4: Sampling, Recruiting, and Retaining Study Participants

Module 5: Understanding and Sharing Research Findings

Module 1

Developing Research Questions

Key Questions:

- What are patient centered outcomes research and comparative effectiveness research?
- How are research studies created?
- How is a patient centered outcomes research study created?
- What are the stakeholder roles in patient centered outcomes research?

Decisions:

- What is the recommended treatment for Glut1 Deficiency?
- Are there options besides the ketogenic diet?
- Which treatment works best for patients like me or my child?
- What version of the ketogenic diet, or what ratio, works best?
- Which medications work best to help with seizures or movements?
- Should I use medications to help with focus or attention issues?
- How long should I stay on a ketogenic diet?

but... what if we don't have the information we need to make informed decisions?

When research is patient centered, it can provide the information we need to make good decisions about healthcare.

This module is designed to help all stakeholders learn about how patient-centered research is created.

Who are stakeholders?

(Slido)

Patient Centered- CER stakeholders can be...

- patients
- parents
- caregivers
- community members
- clinicians
- researchers
- policymakers
- others interested in shaping healthcare

What do they do?

They help identify research topics, review proposals, plan and participate in studies, and share results.

Patient centered - CER

focuses on answering questions that matter to stakeholders

- patients and those who care for them
- healthcare providers
- healthcare advocates
- others concerned with being able to make the best healthcare choices

you are a stakeholder!

research

an organized process designed to learn more about a problem or answer questions

TRADITIONAL

researchers come up with the research questions, study design, and collect data from research participants/patients

Patient Centered - CER

patients and caregivers are equal members of the team and help guide the study from idea to results

research collects *data* using an organized approach

- interviews
- patient medical records
- surveys
- registries and natural history studies

data

is the language of research!

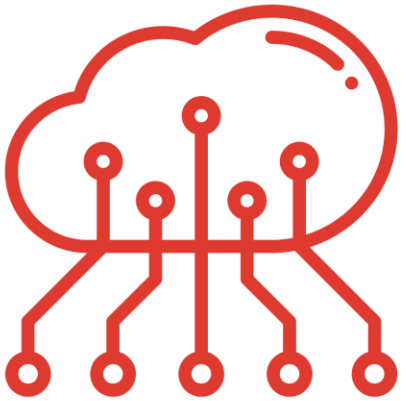
data is analyzed using a

scientific method

Scientific research only uses methods that have been tested and proven to provide trustworthy, reliable answers.

many different kinds of

studies



collect data and analyze it

Each study has its own purpose.

outcomes = measurable results

positive and negative

examples

- Did symptoms improve?

  Which ones? How much?
- What were the side effects?
- Did my trips to the hospital or doctor decrease?
- Did my school or work absences decrease?
- Did I have more energy?

What type of questions do you have that might be addressed by patient centered outcomes research?

What are the things that matter most to

Ketogenic Diet Examples:

- Which version of the diet works best for people like me?
- Do some versions work better or faster than others?
- How long does it take to start making me feel better?
- Are there medications that work the same or better?

different stakeholders might have
different questions they want answered

1st step in developing research questions

SYSTEMATIC REVIEW

look at the evidence and what research has already found out so we can determine what we already know

- Has this been studied before?
- Is there information out there already?
- How was it collected and analyzed?
- Who was in the study?
- How long did the study last?
- What did the study measure?
- Was the method trustworthy and reliable?
- What were the results?
- Were the questions answered?
- Were the answers the same?

2nd *step* in developing research questions

IDENTIFY RESEARCH GAPS

what still needs to be studied, studied more, or in a different way

examples

- questions around age, race, sex, ethnicity and the role they play
- effects in people with more than one health problem
- effect over time
- benefits and harms
- treatments that haven't been studied
- how treatments compare

what we KNOW already

what we WANT to know

what we DON'T KNOW

what we should *focus* on in our study

- what questions still don't have enough evidence to help answer them
- consider how the gaps relate to questions patients and caregivers most want answered

How do we *know* what matters most to patient and families?

We have to ask, and we have to listen!

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developing the study questions

To create a PCOR study comparing treatments researchers make a series of decisions called PICOTS:

- P population
- I intervention
- C comparator
- O outcomes
- T timeframe
- S setting

PICOTS are a brief overview of the essential characteristics of a study

P is for *population*

- **P** population
- I intervention
- C comparator
- O outcomes
- T timeframe
- S setting

Who?

- people of a certain age
- people with certain symptoms
- people with a certain variant or type of variant
- people with other diseases or conditions

researchers need to keep in mind that what might be best for one group might not be best for another

I is for *intervention*

P population
I intervention
C comparator
O outcomes
T timeframe
S setting

making a change – or intervening - in order to study the outcome of what has been changed

What?

- a new treatment
- a therapy
- medical device
- activity
- procedure

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C is for *comparator*

P population
I intervention
C comparator
O outcomes
T timeframe
S setting

comparing one intervention to another

- a new treatment
- the usual standard treatment
- sometimes a placebo
- which one works better?
- who for?

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O is for *outcomes*

P population
I intervention
C comparator
O outcomes
T timeframe
S setting

the results

measuring the benefits or harms of each treatment

- How do we know if it worked?
- What should we measure?
- How will we measure it?
- Who is it important to?

T is for *timeframe*

- P population
- I intervention
- C comparator
- O outcomes
- **T timeframe**
- S setting

how long?

- How long will the study last?
- How long should people get the treatment?
- How long after should follow up happen?
- Short term and long term considerations.
- Results may change over time.

S is for setting

- P population
- I intervention
- C comparator
- O outcomes
- T timeframe
- S setting

where?

- Patients in rural areas vs. urban
- Study patients in one city or many?
- Hospital setting or at home?
- Will telemedicine work?

Research team now has a complete set of questions based on PICOTS:

P population

I intervention

C comparator

O outcomes

T timeframe

S setting

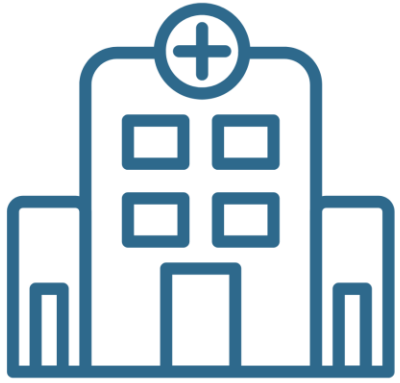
Stakeholders contributing to a patient centered outcomes research study can contribute to each element of PICOTS.

What are stakeholder roles in Patient Centered - CER?

DIVERSE STAKEHOLDERS → COMMON QUESTIONS

patients
and families

healthcare
providers



hospitals,
payors, others

case study

A NEW TREATMENT FOR GLUT1 DEFICIENCY

What are some questions you might have when making decisions about this treatment?

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questions

P population

I intervention

C comparator

O outcomes

T timeframe

S setting

Population

Will the information apply to people like me?

Do the options work for everyone, or only certain people?

Intervention

What is the treatment?

How do I take it?

What do I have to do?

Is it easy to get?

Do I need special training?

Comparators

How is the option different?

Does it work as well as a ketogenic diet?

Is administration easier than the diet?

Which one impacts my life the most/least?

Outcomes

How does it affect symptoms?

What are the side effects?

Does it improve my life?

Am I able to manage this treatment?

Do benefits outweigh risks?

Timeframe

How long do I have to take the treatment?

Does it work at each stage of life?

Does effectiveness change over time?

How long do any side effects last?

Setting

Do I have the option available to me where I live?

As a clinician, do I have this option to offer my patients?

Do I have to begin this treatment in the hospital or can I do it at home?

If I want to continue, where will I be able to get it?

research

QUESTIONS IDENTIFIED

PLAN THE STUDY

CONDUCT THE STUDY

SHARE THE RESULTS

Stakeholders play a role at every stage of

PATIENT CENTERED COMPARATIVE CLINICAL EFFECTIVENESS RESEARCH

IMPROVE LIVES

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