

Objective of Webinar #2: To inform our audience about Comparative Effectiveness Research approaches (CER), and in particular, **Practiced Based Evidence (PBE)**, an innovative kind of methodology that will be used to create, define, and analyze the data being collected in the CPRN registry/database

Definition of Comparative Effectiveness Research:

Comparative Effectiveness Research (CER) *is research that identifies what clinical and public health interventions work best for improving health.* This type of research has become synonymous with the term “patient-centered” research which means the research addresses the questions and concerns most relevant to patients.

In a CER study, interventions should, at a minimum, be compared on the basis of some health-related outcome measure. Examples of the types of interventions examined through CER research include diagnosis and treatment protocols, innovations in health care delivery, organization and financing, as well as public health interventions like awareness, lifestyle, diet, or environmental exposures.

**From the Harvard School of Public Health website section on Comparative Effectiveness Research Initiative

To begin the webinar Dr. Horn reviewed one of the most common and traditional types of research methodologies/approaches that can be used to conduct CER studies called Randomized Controlled Trials (RCTs):

1. **Randomized Control Trials (RCTs)** have long been considered the most accurate and reliable approach for conducting research. This approach to research allows the investigator to set up a study where they *proactively* try to control for variables that may influence the outcome or results of the study. They do this with the goal of understanding how, for example, a treatment may affect individuals in a study while minimizing the possibility that the results they see can be attributed to other factors.

Example-In CP if a researcher wanted to study how well botulinum toxin (ie. Botox) may improve range of motion, they would want to control for other variables that may influence the outcome of range of motion. They may have criteria for participation such as ensuring that participants do not receive other treatments during the study such as a muscle or tendon

release or another surgical procedure that may affect an individual's range of motion.

Next, Dr. Horn explained how Practiced Based Evidence (PBE) differs from RCTs and how this approach can be used to learn new information about CP. She used examples from other populations and conditions where this methodology has already been applied.

2. **Practiced Based Evidence (PBE)** is the kind of comparative effectiveness research methodology we will be using to create, define, and analyze the data collected from the participating CPRN sites that make up our clinical registries. In contrast with RCTs, PBE analyses control for confounding variables in the statistical analyses, *after*, rather than before the data have been collected. **This type of methodology is very inclusive compared to RCTs where there is a small segment within a population that is typically chosen to be studied.** This broad inclusion approach allows for discovering trends and outcomes that researchers may not have thought of on their own and also discover trends more quickly because of how much information is available to analyze.

Example comparing RCT to PBE approaches: Below is an example of a more narrowly defined study population that could be used to test a treatment with an RCT approach:

1. People ages 18-35
2. Who are ambulatory (GMFCS 1-3)
3. And have spastic forms of CP only

In contrast and just below, is a comparison to these proposed and more specifically defined RCT categories. In CER-PBE studies the data collection is very robust in the beginning:

1. People may be all ages
2. All movement abilities—i.e. both ambulatory and non-ambulatory
3. Will collect data on people with a variety of movement disorders or other conditions that may normally disqualify them from participating in an RCT where variables are being controlled at the beginning of the study.

Finally, Dr. Horn went over how both PBE and RCTs are important to advancing research.

We need both RCTs and PBE approaches. PBE research can help identify hypotheses and trends that can be studied further in an RCT. In one of the examples that Dr. Horn provided about PBE data collected and analyzed on the Traumatic Brain Injury (TBI) population, it was clear through data analysis that there were certain treatment protocols that were more effective than others in predicting long-term outcomes. Researchers may wish to investigate further which protocols work the best in a specific population by conducting an RCT.

Example of how a PBE finding can be used to conduct an RCT—remember variables are controlled at the beginning in an RCT methodology and this is done by having defined parameters for participation:

1. Select a very impaired group of patients following a TBI
2. Designate an age range that can be included in the study
3. Randomly assign participants two groups receiving different two types of rehabilitation protocols to determine which one works better.

These groups could be:

- A. Putting them in a harness to start walking on their first day in rehabilitation and continue with more complex activities during the remaining time in rehab.
- B. Or they could be assigned to usual therapy care such as starting with spending time on bed mobility and sitting in bed on the first day in rehabilitation, and then initiating more complex activities after the patients are successful 80% or more of the time with the less complex activities.

