Practice-Based Evidence Study Design

A Systematic Approach to Physical Therapy Documentation for Comparative Effectiveness Research

Susan D. Horn, PhD
shorn@clinicalcor.com
Institute for Clinical Outcomes Research
699 E. South Temple, Suite 300
Salt Lake City, Utah 84102-1282
801-466-5595 (T) 801-466-6685 (F)

Presentation Overview

• Brief description of PBE, a practice-based evidence approach to comparative effectiveness of treatments, and how it compares with other study methodologies, particularly RCTs
• PBE examples of comparative effectiveness findings about physical therapy activities and processes for stroke rehabilitation
• How to implement PBE documentation in daily workflow

Practice-Based Evidence Study Design

Improve/Standardize:

- Process Factors
  - Management Strategies
  - Interventions
  - Medications

Control for:

- Patient Factors
  - Psychosocial/demographic Factors
  - Disease(s)
  - Severity of Disease(s)
  - Physiologic signs and symptoms
  - Genetic Information
  - Multiple Points in Time

Measure:

- Outcomes
  - Clinical
  - Health Status
  - Functional
  - Cost/LOS/Encounters

Why Practice-Based Evidence?

PBE is a validated, timely, cost-effective method to assess comparative effectiveness of specific interventions: devices, drugs, treatment processes

• PBE studies compare effectiveness of interventions used in clinical practice by many providers, overcoming limitations of RCTs
• More detailed patient, process, and outcome evaluation than is possible with traditional registries
• Useful when RCT is impractical or unethical

7 Signature Features of PBE Studies

1. Hypotheses can be focused or broad
2. Consider all interventions to determine relative contribution of each
3. Minimal patient selection criteria maximize generalizability and external validity
4. Detailed characterization of the patient by robust measures of patient severity, genetic information, and functional status
5. Patient differences controlled statistically rather than through randomization
6. Facility and clinical/patient buy-in through use of trans-disciplinary Clinical Practice Team
7. Strength of evidence built through the research process

PBE findings are more generalizable and transportable than RCT findings
### PBE Signature Feature: Interventions

2. **Consider all interventions** to determine relative contribution of each.

- Uses a detailed characterization of the care process through a well-designed point-of-care (POC) documentation system
  - User-defined and user friendly
  - Time sensitive characterization of all interventions

### PBE Signature Feature: Patients

4. **Detailed characterization of the patient** by robust measures of individual severity and functional status

- Includes Comprehensive Severity Index (CSI®)
  - Over 2,200 condition-specific signs, symptoms, and physical findings
  - Continuous score: 0 → ∞
  - Admission, discharge, maximum during stay, visit
- Genetic information
- Includes Functional Independence Measure (FIM) and/or other measures of functional status

### PBE Signature Feature: Clinical Practice Team

6. **Facility and clinical buy-in** through use of transdisciplinary Clinical Practice Team that:

- Develops and frames the questions
- Defines variables
- Gathers data
- Interprets data
- Implements findings
- Fosters clinical and individual buy-in (bottom-up)
- Facilitates knowledge translation

### PBE Signature Feature: Strength of Evidence

7. **Strength of evidence** built through the research process

- Added confounders preserve the significant association
- A change in outcomes follows a change in treatment as predicted by the PBE model
- Repeated studies on the same topic yield similar findings

### PBE Hallmarks

- Non-experimental: Follows outcomes of treatments actually prescribed
- Inclusive: Uses patient populations undergoing routine clinical care
- Pragmatic: Uses actual clinical outcomes
- Lower Cost than RCTs
- Faster than RCTs

### PBE and RCT Compared

“What is efficacious in randomized clinical trials is not always effective in real world of day-to-day practice...

Practice-based research provides the laboratory that will help generate new knowledge and bridge the chasm between recommended care and improved care.”

---

PBE and RCT

Progenitor of RCTs

RCT

Practice effects of RCT results

PBE

Comprehensive Severity Index (CSI®)

- Disease-specific: over 2,200 individual criteria subdivided into more than 5,500 disease-specific groups
- No treatments used as criteria
- Comprehensive (all diseases)
- Clinically credible: computes disease-specific and overall severity levels
- Can measure severity at multiple time points
- Statistically valid explanation of costs/outcomes

Coronary Artery Disease - IHD

Disease codes 411.11, 413-414.05, 414.8-414.9

<table>
<thead>
<tr>
<th>Category</th>
<th>Indicator</th>
<th>Value</th>
</tr>
</thead>
<tbody>
<tr>
<td>Coronary</td>
<td>Highest Creatinine</td>
<td>&lt;1.2 mg/dl</td>
</tr>
<tr>
<td>Artery</td>
<td>Lowest Cardiac Output</td>
<td>2.1 L/min</td>
</tr>
<tr>
<td>Disease</td>
<td>Highest AVO2 Difference</td>
<td>&lt;=6.0 ml/dl</td>
</tr>
<tr>
<td></td>
<td>Temperature</td>
<td>31-40.0°C</td>
</tr>
<tr>
<td></td>
<td>Systolic BP</td>
<td>&lt;=110 mm Hg</td>
</tr>
<tr>
<td></td>
<td>Diastolic BP</td>
<td>&lt;=219 mm Hg</td>
</tr>
<tr>
<td></td>
<td>Heart Rate</td>
<td>&lt;=180 beats/min</td>
</tr>
<tr>
<td></td>
<td>EKG Rhythm</td>
<td>None</td>
</tr>
<tr>
<td></td>
<td>Respiratory</td>
<td>None</td>
</tr>
<tr>
<td></td>
<td>Blood Pressure</td>
<td>None</td>
</tr>
</tbody>
</table>

PBE Study

- Connects outcomes with detailed process steps
- Adjusts for severity of illness to control for patient differences/selection bias

PBE Data Sources

- Medical record
- Genetic information
- Point-of-care documentation
  » Treatment process details not in the medical record
- Patient interview
**Post-Stroke Rehabilitation Study**

2001 – 2003; 1,161 patients

**Study Objectives**

PBE study designed to discover what combinations of medical devices, therapies, medications, feeding approaches, and their interactions work best for specific types of stroke patients treated in real-world practices.

---

**Post-Stroke Rehabilitation Study**

2001 – 2003; 1,161 patients

**Patient Heterogeneity**

- Age, gender, race, payer
- Stroke risk factors
- Type and side of stroke
- Functional Independence Measure (FIM) scores
- Severity of illness scores

---

**Significant Site Variation - Stroke Admission Motor FIM**

![Graph](image)

ANOVA, p<.001

---

**Significant Site Variation - Stroke Admission Severity of Illness score**

![Graph](image)

ANOVA, p<.001

---

**Post-Stroke Rehabilitation Study**

**Significant Site Variation - Process Variables**

- Rehab LOS
- Intensity of PT
- Intensity of OT
- Intensity of SLP

- Tube Feeding Use
- Medication use
  - Antidepressants
  - Antipsychotics
  - Opioid analgesics
  - Anti-seizure meds

**Post-Stroke Physical Therapy Form**

![Form](image)
Significant Site Variation
Physical Therapy (Severe Stroke)

Minutes of PT per patient per day

ANOVA, p<.001

Significant Site Variation
Occupational Therapy (Severe Stroke)

Minutes of OT per patient per day

ANOVA, p<.001

Significant Site Variation
Speech Therapy (Severe Stroke)

Minutes of SLP per patient per day

ANOVA, p<.001

Significant Site Variation - Stroke
Opioid Pain Medication Use

% of patients

Chi-Square, p<.001

Post-Stroke Rehabilitation Study

Examples of OUTCOME VARIABLES

• Change in FIM score
• Length of rehab stay
• Discharge disposition
• Contracture
• Death

• Deep vein thrombosis
• Major bleeding
• Pulmonary embolism
• Pressure ulcer
• Pneumonia

Outcome: Discharge Motor FIM
Severe Stroke (CMGs 108-114) – Full Stay

General Assessment
- Age
- Black race
- Mild motor impairment
- Admn Motor FIM
- Admission Cognitive FIM

PT Interventions
- Formal assessment
- Bed mobility
- Gait
- Advanced gait

OT Interventions
- Home management
- Swallowing
- Orientation
- Reading comprehension

SLP Interventions
- Anti-Parkinsons
- Modafinil
- Old SSRIs
- Atypical antipsychotics

General Interventions
- Days onset to rehab
- Enteral feeding

Medications

11/3/2009
Outcome: Discharge Motor FIM
Severe Stroke—1st 3 hour Therapy block only

<table>
<thead>
<tr>
<th>General Assessment</th>
<th>Interventions</th>
<th>OT Interventions</th>
<th>SLP Interventions</th>
</tr>
</thead>
<tbody>
<tr>
<td>Age</td>
<td>Bed mobility time in 1st 3 hrs</td>
<td>+ Home management</td>
<td></td>
</tr>
<tr>
<td>Severe motor impairment</td>
<td>Gait time in 1st 3 hrs</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Admission Motor FIM</td>
<td>Advanced gait time in 1st 3 hrs</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Admission Cog. FIM</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>No Dysphagia</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Neurotropic Impairments treated with meds</td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

Medications

- Other Antidepressant
- Old SSRIs
- Atypical antipsychotics

Discover Best Practices using PBE

- Practitioners: PBE data allow investigation of effects of combinations of treatments on outcomes, controlling for patient differences
- Payers: PBE data allow discovery of practices associated with better functional and clinical outcomes at lower cost
- Manufacturers: PBE studies show comparative effectiveness of medical devices and products and are less expensive and faster to conduct than RCTs
- Regulators: PBE studies permit pre & post-approval analyses for comparative effectiveness and safety

What Makes PBE Study Design Different?

- Helps to identify what works best in actual practice
- Is clinically valid, timely, and cost-effective
- Describes the patient in detail
- Captures details of interventions/treatments/process of care
- Captures vital information beyond administrative data and patient records
- Involves front-line clinicians throughout the study

PBE & RCT Compared

<table>
<thead>
<tr>
<th>Dimension</th>
<th>PBE</th>
<th>RCT</th>
</tr>
</thead>
<tbody>
<tr>
<td>Type of study</td>
<td>Observational Study</td>
<td>Randomized Controlled Trial</td>
</tr>
<tr>
<td>Intervention</td>
<td>All interventions deemed relevant</td>
<td>1 or 2 discrete interventions</td>
</tr>
<tr>
<td>Hypotheses</td>
<td>Focused or broad</td>
<td>Well-specified</td>
</tr>
<tr>
<td>Selection criteria</td>
<td>Minimal</td>
<td>Extensive</td>
</tr>
<tr>
<td>Sample size</td>
<td>As large as desired</td>
<td>Typically small</td>
</tr>
<tr>
<td>Control for participant differences</td>
<td>Detailed characterization and statistical control</td>
<td>Randomization</td>
</tr>
</tbody>
</table>

PBE & RCT Compared

<table>
<thead>
<tr>
<th>Dimension</th>
<th>PBE</th>
<th>RCT</th>
</tr>
</thead>
<tbody>
<tr>
<td>Blinding</td>
<td>No</td>
<td>Single, double, triple</td>
</tr>
<tr>
<td>Outcomes</td>
<td>Many</td>
<td>Few</td>
</tr>
<tr>
<td>Effect size</td>
<td>Often large</td>
<td>Often small</td>
</tr>
<tr>
<td>Confounders</td>
<td>Affect outcomes and are interesting</td>
<td>Not interesting; exclude them</td>
</tr>
<tr>
<td>Validity</td>
<td>High external</td>
<td>High internal</td>
</tr>
<tr>
<td>Causality</td>
<td>Assumed</td>
<td>Assigned</td>
</tr>
<tr>
<td>Ability to examine subgroups</td>
<td>Very-large and heterogeneous sample</td>
<td>Limited–small and homogeneous sample</td>
</tr>
</tbody>
</table>

PBE & RCT Compared

<table>
<thead>
<tr>
<th>Dimension</th>
<th>PBE</th>
<th>RCT</th>
</tr>
</thead>
<tbody>
<tr>
<td>Cost</td>
<td>Moderate</td>
<td>High</td>
</tr>
<tr>
<td>Culture (1)</td>
<td>High transparency</td>
<td>Top-down; blinded</td>
</tr>
<tr>
<td>Culture (2)</td>
<td>Local knowledge contributes, valued</td>
<td>Local knowledge excluded</td>
</tr>
<tr>
<td>Knowledge translation</td>
<td>High level of buy in; findings transportable</td>
<td>Far less buy in</td>
</tr>
<tr>
<td>Science of ....</td>
<td>Discovery &amp; innovation</td>
<td>Confirmation</td>
</tr>
<tr>
<td>Science of ....</td>
<td>Effectiveness</td>
<td>Efficacy</td>
</tr>
</tbody>
</table>