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Health research throughout the lifespan

Course Objectives and Importance
Richard Stevenson, M.D.
Learning Objective 1: To understand the advantages and limits of alternative designs for conducting comparative effectiveness research. Included will be prospective randomized controlled trials and strategic analyses of clinical databases.

Learning Objective 2: To learn how to apply a recently developed reliable and valid tool to measure treatment fidelity (protocol adherence) for CIMT and other interventions.

Learning Objective 3: To identify and be able to monitor factors likely to influence decisionmaking and implementation of CIMT and other treatment protocols, based on comparative effectiveness findings.

Introduction

• Cerebral Palsy is a complex and heterogeneous set of conditions of motor impairment, often complicated by co-morbid or secondary conditions
• 28% of CP is unilateral (large sub-group)
• Treatments address the motor impairment(s) and/or secondary complications that interfere with motor function, positioning, comfort
• Goals of treatment vary widely
• Cerebral palsy research crossroads

Research and Practice: Key points

• We have accomplished a great deal
• We need to build on our successes
• Collaboration is key
• Research and practice must be closely intertwined
• Maintain our focus on implementing evidence-based practices with high fidelity to all who will benefit
The challenges in effective delivery of evidence-based practices (EBP)

- “30 – 40% of interventions have no reported evidence-base”
- “Another 20% of interventions provided are ineffectual, unnecessary, or harmful”
- Gap in research to practice “exists despite numerous systematic reviews providing guidance about what does and does not work for children with CP”


Recent happenings...

- Collaborative meeting at NIH on CP Research
  - Common Data Elements Project
  - Registry Project
  - Basic/Translational
  - Comparative Effectiveness Project

Identification of major gaps in scientific evidence

- Evidence is building but many evidence gaps exist, especially concerning which CIMT protocols produce the best results.
- To date, few studies have sufficiently large samples with diverse characteristics (e.g., age, severity of impairment, comorbidities) to determine if some CIMT protocols work better for certain subgroups of children.
- Many CIMT and other efficacious treatments for CP are insufficiently described and documented to permit full replication (even among “green” bubbles).
- Different approaches may be complementary, but we need to conduct longitudinal research to study sequential treatments or combined interventions.
Other challenges …

- Care often dictated by geography, health care financing, local leaders’ knowledge/beliefs
- Despite growing evidence for efficacious interventions, little comparative effectiveness research to directly assess alternative CIMT and bimanual protocols in terms of effects
- Implementation of EBP into clinical treatment often involves wide deviations from the tested (proven) protocols
- How best to assist and train clinicians (both referring and treating), administrators, families?

What are your thoughts?

1. What do you see as the major challenges to integrating CIMT into your practice?
2. Which CIMT approaches do you think are the most promising and best supported by robust evidence?
3. Have you been able to implement any of these into general practice? If yes: Which ones? How? Did you make adaptations? If no: why not?
4. What do you see as important next steps? Can AACPDM be a facilitator?

Collaborative Research, Collaborative Practice: Keys to Evidence-Building and Knowledge Translation

- Patients and families, family organizations
- Colleagues of all disciplines
- Funding agencies, public and private
- Within organizations
- Between organizations
- Researchers and clinicians
- Opportunities to build and sustain coalitions
Comparative Effectiveness Trials: Overview of CHAMP & Baby CHAMP

Sharon Landesman Ramey, Ph.D.

Strong Research Designs are Key to Building Evidence and Knowledge Translation

Comparative effectiveness research is the conduct and synthesis of systematic research comparing different interventions and strategies to prevent, diagnose, treat, and monitor health conditions. The purpose...is to inform patients, providers, and decision-makers about which interventions are most effective for which patients under specific circumstances. To provide this information, comparative effectiveness research must assess a comprehensive array of health-related outcomes for diverse patient populations.

Federal Coordinating Council for Comparative Effectiveness Research, definition; [28 July 2010].

Children with Hemiparesis Arm & Hand Movement Project (CHAMP)

Multisite RCT funded by NIH comparing the relative efficacy of alternative CIMT therapies for young children (ages 2 - 8) with hemiparetic cerebral palsy

Participating Sites:
University of Virginia
Virginia Tech Carilion Research Institute
Ohio State University

This study directly compares different amounts (doses) of CIMT and different types of constraint to assess what "works best" for young children.
Clinically Urgent Questions and
Specific Aims

What are the effects of variation in 2 key features of pediatric CIMT: namely, dosage and type and duration of constraint?

Specific Aims

1: To evaluate the effects of two dosage levels of pediatric CIMT: 3 hr sessions/5 days/week for 4 wks (60 hrs) vs 2.5 hr sessions/3 days/week for 4 wks (30 hrs)

2: To evaluate the effects of two different restraint conditions for CIMT: full-time, full arm cast vs part-time, part-arm splint

3: To explore the role of children’s individual differences and family variables in predicting differential responses to treatment

Study Design:
Comparative Effectiveness Trial via
2 X 2 Factorial Design + U&C control

5 treatment groups (N=135)

- Usual and customary (U&C) control for 6 mos., then family can choose preferred crossover therapy (N=27)
- Higher Intensity (60 hr) + Full-time constraint (N=27)
- Higher Intensity (60 hr) + Part-time constraint (N=27)
- Lower Intensity (30 hr) + Full-time constraint (N=27)
- Lower Intensity (30 hr) + Part-time constraint (N=27)

CHAMP Assessments: Timing and Content

- All assessments blinded to group assignment
- Conducted just prior to treatment, then repeated at 1, 6, & 12 mos post-treatment (crossover children followed with same outcomes)
- All sessions fully videotaped (for admin. fidelity and double-scoring)
- CHILD DIRECT ASSESSMENT BATTERY includes:
  - Assessing Hand Assessment (AHA) – double-scored by certified AHA assessors
  - Peabody Developmental Motor Scales (PDMS-2) Subscales for Visual Motor and Object Manipulation – scored by assessor
  - QUEST – Subscales for Dissociated Movement and Grasp
- Parents complete PEDI-CAT, Pediatric Motor Activity Log (PMAL) and perceptions of stress related to therapy (for child and parent)
- Supplemental data from Therapists’ Systematic Daily Logs; weekly recording of treatment session
- Emerging Behaviors Scale (derived from compilation across above independent sources)
Discussion

- Thoughts about the study? Questions?
- In what way does this study address some of the gaps in evidence and practice?
- Are there gaps this trial does not address?
- What findings from this comparative effectiveness trial would be useful for you in your practice and/or research?

Overview of Baby CHAMP
Total N = 72 (24 per treatment group)

- Comparative Effectiveness Multisite RCT funded by NIH
- Three protocols chosen - all highly promising
  - Full-time constraint + 3 hrs/day X 5 days/week X 4 weeks in home PLUS parent companion support
  - Part-time constraint + 3 hrs/day X 5 days/week X 4 weeks in home PLUS parent companion support
  - No constraint (Bimanual) + 3 hrs/day X 5 days/week X 4 weeks in home PLUS parent companion support

First RCT/Comparative Effectiveness Infant Trial (multisite, adequately powered)
Ages 6 to 18 months when enrolled, treatment as soon as feasible
No U&C control (rationale based on multiple prior reviews)

Some novel aspects of assessment in Baby CHAMP

- Mini-AHA (first study to use with certified assessors)
- Adapted form of Bayley Scales of Motor Development (both arms assessed) (based on Case-Smith)
- Inclusion of kinematics and behavioral scoring (Heathcock)
- Cortisol Testing to assess stress levels in both
  - Parent/Caregiver
  - Infant
- Functional Near Infrared Spectroscopy (fNIRS) (LaConte & King-Casas) to measure Laterality Index (resting state and movement)
- Biological & perceived stress in parents; biological and behavioral stress in infants (Ramey, adapted from CCHN protocol)
- Inclusion of cognitive and social-emotional outcomes (multi-domain development)
- Active assessment of the parent-therapist relationship
Discussion

• How does this trial address evidence gaps?
• How does it differ from the CHAMP trial?
• What findings from Baby CHAMP will be high interest to you? Why? How might you use these to influence practice and to design future research to expand knowledge base?

How to delineate and measure key features of treatment. Use of a new reliable, valid Fidelity of Therapy Tool

Stephanie DeLuca, Ph.D.

Treatment Fidelity is…

the degree to which the treatment actually delivered to a patient matches the treatment specifications.

This necessitates a well-specified (ideally written or manualized) treatment protocol. Our field of pediatric medical rehabilitation has lagged in this area.

Treatment fidelity helps in “unpacking the black box” of treatments - because it provides systematic information about whether the content and approach are “true to the model” (intended treatment). It facilitates replicability and maintenance of high standards.
Traditional Uses of Treatment Fidelity

• To monitor the delivery of a new or combined treatment during clinical trials.
• To assess whether the treatment can be delivered by others when conducting replication trials and
• To be sure that when an efficacious treatment is implemented in real-world clinical care settings that the treatment is faithful to the specified, tested model.

Innovative Uses of Treatment Fidelity (page 1 of 2)

• As a guide in the process of training the individual clinicians or practitioners in the delivery of a new or combined treatment
• As a way to monitor clinical performance and service delivery to drive up consistency and quality in an era of evidence-based practice
• As a way to plan and measure “planned deviations” in treatment model to potentially realize extra benefits, efficiencies, and/or ease without compromising expected benefits

Innovative Uses of Treatment Fidelity (page 2 of 2)

• To determine whether the highest level of fidelity produces greater patient benefits; if not, to explore
• what the minimal or threshold levels of treatment fidelity are,
• whether some features of the original treatment model are not “active ingredients” critical to realizing measurable benefits, and
• whether some patient or setting characteristics make it particularly difficult to deliver the treatment with high fidelity – and thus there is a need to develop more tailored models for these patients
Despite consensus that Treatment Fidelity is important...

- It rarely is specifically reported in clinical outcomes studies, other than in general terms (e.g., % who completed planned treatment, missed # of treatments) – except for very large-scale, well-funded Phase III trials
- Measuring Treatment Fidelity is challenging when it moves beyond “expert” or “supervisor” judgments trusted to be in place
- Many treatments for children with disabilities are not sufficiently specified or available in manualized form, preventing rigorous fidelity measurement

Construction of the CHAMP Fidelity Tool for pediatric Constraint-Induced Movement Therapy (P-CIMT)

- Define the critical or core features of pCIMT (literature review and expert content analysis)
- Identify other therapy features essential for high quality treatment, even if not unique to pCIMT (professional standards, ethics, face validity)
- Distinguish structural features (providers, dosage, delivery site) from behavioral skill level in delivering some core elements of pCIMT

Common Core Elements of P-CIMT (cf. Ramey, Coker-Bolt, & DeLuca)

- Structural Core Elements
  - Constraint of less impaired Upper Extremity (type, amount)
  - High Intensity (requires specification)
  - Treatment Plan with specified goals
  - Transition Plan building on progress
  - Trained provider/parent
  - Natural environment

- Functional Core Elements
  - Active Shaping & Successive Approximations (Higher goals)
  - Repetitive practice with feedback

- Good Pediatric Practice principles
Sources of data we use for CHAMP Fidelity of Implementation Tool

- Weekly videotapes of one entire treatment session for each of 4 consecutive weeks

Score the video-recording on each of the 6 areas labeled below as demonstrating (Listed below each section are examples of things to consider, and a comment space is provided):

- 3 = Meets High Standards & Expectations for P-CIMT (MR3 Cycle)
- 2 = Acceptable Standards & Expectations for P-CIMT (MR3 Cycle)
- 1 = Does Not Meet Standards & Expectations for P-CIMT (MR3 Cycle)

Inter-Rater Agreement Across Fidelity Measure

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Fidelity Discussion

- Have you used fidelity measures in your research or practice?
- Do you see value in using them? If so, what would it take for your clinic or research to incorporate these?
- Is there a role that AACPDM can play to improve development and use of fidelity of treatment measures?
Parents and clinicians as collaborators and contributors to comparative effectiveness research

Amy Darragh, Ph.D., OT

Baby CHAMP Parents as Partners: Orientation

Parents as Partners approach means you will work closely with your therapist(s) to learn to use some of the therapy methods with your child during everyday activities and in everyday environments.

We are engaging and supporting parents as a formal component in the intervention, with the objective of maximizing the impact and sustainability of the intervention.

Your child will receive ACQUIRE therapy.

ACQUIRE stands for:

A - Acquisition of new motor skills through
C - Continuous practice and shaping to produce
Q - Quality movement of the
U - Upper extremity through
I - Intensive therapy and
R - Reinforcement in
E - Everyday patterns and
places.
ACQUIRE: All 3 Forms

Therapist-Led Shaping Cycle

Parent-Led Practice Cycle

Movement-Reinforcement-Repetition-Refinement (MR3)

At the heart of this intensive therapy is the MR3 cycle. The four elements of this cycle are based on well-established principles of learning and development. The four elements are:

- **Movement**: You will encourage, prompt, stimulate, and guide movement using the therapy techniques you will learn today.
  - *Examples can include any movement intended or accidental*

- **Reinforcement**: You will provide immediate, positive, and varied responses to your child’s movement. This serves as a natural reward for your child’s effort and progress. It can include verbal praise, positive physical expression, concrete rewards, or rewards that are a part of completing the activity.
  - *Examples of reinforcers and rewards:*

- **Repetition**: You will encourage practice, rehearsal and extended play involving a particular movement pattern or skill. It should feel integrated into the play and functional activities in which you engage with your child, not forced, mechanical, or invasive.
  - *Examples of repetition are:*
Refinement: Refinement occurs during repetition. Refinement occurs when the therapist or parent recognizes that the child is ready for a challenge. When your child is able to perform the movement pattern, skill, or functional task that he/she has been practicing appropriately (defined with your therapist) 70 – 80% of the time, you will encourage him/her to move to a more advanced level, with greater performance expectations. You will provide guidance, examples, and instruction regarding the new expectations to assist your child with this shift.

Examples of Refining a Movement Include:

- Shaping. Shaping is a systematic process you will use to help your child learn a new behavior. It involves what we call "successive approximation", that is, you will work with your child on a sequence of behaviors that increasingly come closer to a final, targeted behavior. The final behavior, movement, skill or task will be broken down into parts. You will assist your child with each of these parts or steps, starting with the most basic and moving to the more complex or challenging. You can use reinforcement strategies to reward his or her achievement of each step.

Examples of Refining a Shaping Might be … and Remember we are going to show you a video to help you understand these terms

- Massed Practice. Massed practice is a form of shaping in which your child will perform multiple, closely spaced trials of the behavior he or she is learning. In essence, your child will be practicing a motor pattern, skill or task over and over again in a short period of time to facilitate acquisition of the motor behavior.

- Hand over Hand Facilitation. When your child begins to learn a new movement, skill or task, you may have to help him or her by supporting his/her body or moving or manipulating his or her arm and hand to accomplish it.

- Fading. As your child begins to learn or perform the skill or movement, you will reduce the amount of physical assistance you are giving to your child’s body, arm or hand, and allow him or her to try the movement on his or her own.
Modeling. When your child begins to learn a new movement, skill or task, you may need to demonstrate exactly what it looks like and how you would like him or her to perform the movement or task. You can do this slowly and use verbal descriptions you believe your child will understand.

MR3 Can be Completed with:
- Routine activities. Routine activities like bath time, mealtime, and dressing are wonderful opportunities for your child to practice his or her new skills with you.
- Non-routine activities. You may also choose to set aside some playtime with your child to introduce new toys or a new way to play with favorite toys.

Goal

Why we are not making parents into therapists, nor are we excluding them from active, meaningful engagement

- Other Pros and Cons?
- Agree?
- Disagree?
- How do we promote Fidelity of Treatment Implementation?
- How do we complete and maximize results in comparative effectiveness trials in the future?