

# Efficacy and Effectiveness: Issues for Physical Therapy Practice and Research: Examples from *PTClinResNet*

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# To open, we would like to stipulate to the following:

- ◆ Both efficacy and effectiveness studies are and will be needed in Physical Therapy.
- ◆ All types of research designs, from case reports, to RCTs, to observational studies, to qualitative studies, are and will be needed in PT.
- ◆ Each type of research design is better suited for some questions than others.

# Stipulations (continued) ...

- ◆ The value of a given research design is dependent upon:
  - The question type (efficacy v. effectiveness and associated internal and external validity needs)
  - Ethical considerations
  - Feasibility and pragmatic concerns
  - Perspectives of research funders, third-party payors, reviewers, investigators, clinicians, and patients (stakeholders)

# Evidence-based practice....

- ◆ Is predicated on being informed by the "best available evidence."
- ◆ What kind of "research" should generate the evidence that affects clinical practice?
- ◆ What kind of evidence is *most useful*?

# EVIDENCE-BASED PRACTICE IS ...

"the integration of best research evidence with clinical expertise and patient values."

Sackett, Straus, Richardson, Rosenberg, & Haynes, 2000  
[Haynes, Devereaux, & Guyatt, 2002]

# Clinical Research is designed to find solutions to real-world clinical problems...

## ◆ Efficacy

Efficacy is concerned with the questions, *Does the investigational treatment cause an effect? And Can this treatment work under ideal circumstances?*

## ◆ Effectiveness

Effectiveness addresses the question, *Does it work in the real world?*

# Important considerations..

## ◆ *Efficacy...*

- RCT: if a specific treatment cannot be shown to work under the best of conditions/careful administration, there "isn't a ghost of a chance that it will be effective in actual practice" [Streiner (2002), Can J. Psychiatry, 47:552-556.]
  - ◆ must rule out possible confounds to establish cause and effect
- Observational study: although correlation does not prove causation, if there is no correlational relationship between two variables, there can be no cause-and-effect relationship between them.

## ◆ *Effectiveness...*

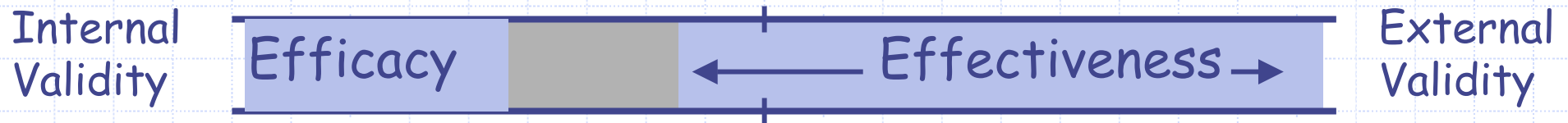
- studies emphasize the applicability of the treatment and therefore try to mirror in an RCT or measure (Observational study) some/many of the situations that clinicians and patients encounter in everyday life.

# Three key 'facets' to clinical research..

- ◆ Validity Concern: Internal and external validity
- ◆ Question Type: Efficacy or effectiveness?  
(based on state of prior knowledge)
- ◆ Research Design Type: RCT, Observational, Qualitative
  - Research question and validity concerns
  - Ethical considerations (clinical equipoise)
  - Cost and acceptability, other pragmatics

Efficacy and effectiveness studies utilize different aspects of validity...

clinical research [intervention] studies continuum:



Most studies fall somewhere in between these anchors

# Relationship between Research Design and Efficacy Questions

## *Efficacy* Questions (Cause and Effect):

Experimental  
(RCTs)



Quasi-  
Experimental/  
Observational  
designs

Clinical Trials Phase II (pre-  
effectiveness phase)

- Utilize "natural" variations
- Correlations identify possible causal factors (pre-RCT efficacy)
- When RCTs impractical/unethical

[Internal  
Validity]

[External  
Validity]

# Relationship between Research Design and Effectiveness Questions

## *Effectiveness* Questions (Generalizability/Real-world Applicability):

Experimental  
(RCTs)



Clinical Trials Phases III and IV  
(post-efficacy phases):

- looser inclusion criteria
- examine several independent variables, including:
  - effects of clinician's judgment vs. protocol
  - clinician training
  - required patient follow-up

[Internal  
Validity]

Utilize "natural"  
variations in  
multiple "independent  
variables"

Quasi-  
Experimental/  
Observational  
designs

[External  
Validity]

# Randomized Controlled Trials for ...

## ◆ Efficacy

- More explanatory (basic cause and effect)
- More controls for possible confounds and biases
  - ◆ More homogeneous sample
  - ◆ Standardized intervention, clinician training
  - ◆ Conservative analysis (e.g., drop-outs and people who receive the "wrong" treatment)

## ◆ Effectiveness

- More pragmatic (more implementation and generalizability questions)
- Variations allowed/desirable
  - ◆ More heterogeneous patient sample
  - ◆ Interventions less tightly controlled; clinicians' time demands, multiple concerns may dictate less standardization and training

# Designs can be chosen for the right or wrong reasons...

- ◆ Relationship/value to the research question
- ◆ Convenience, cost, pragmatics
- ◆ Perceptions of design value, rightly or wrongly, by given audiences or reviewers (i.e., "politics")
- ◆ Skills/experience, training (or lack thereof) of the primary research group (with experimental/observational designs, measures, analyses)

# PTClinResNet



## Common Theme:

- ◆ With a diverse set of four diagnoses, we focus on identifying and testing strategies for improving function and reducing disability through interventions that are designed to enhance muscle performance and are based on a dynamic task-oriented or muscle-oriented approach.

# Multi-site Multi-institution



Visit us at:  
<http://pt.usc.edu/clinresnet>

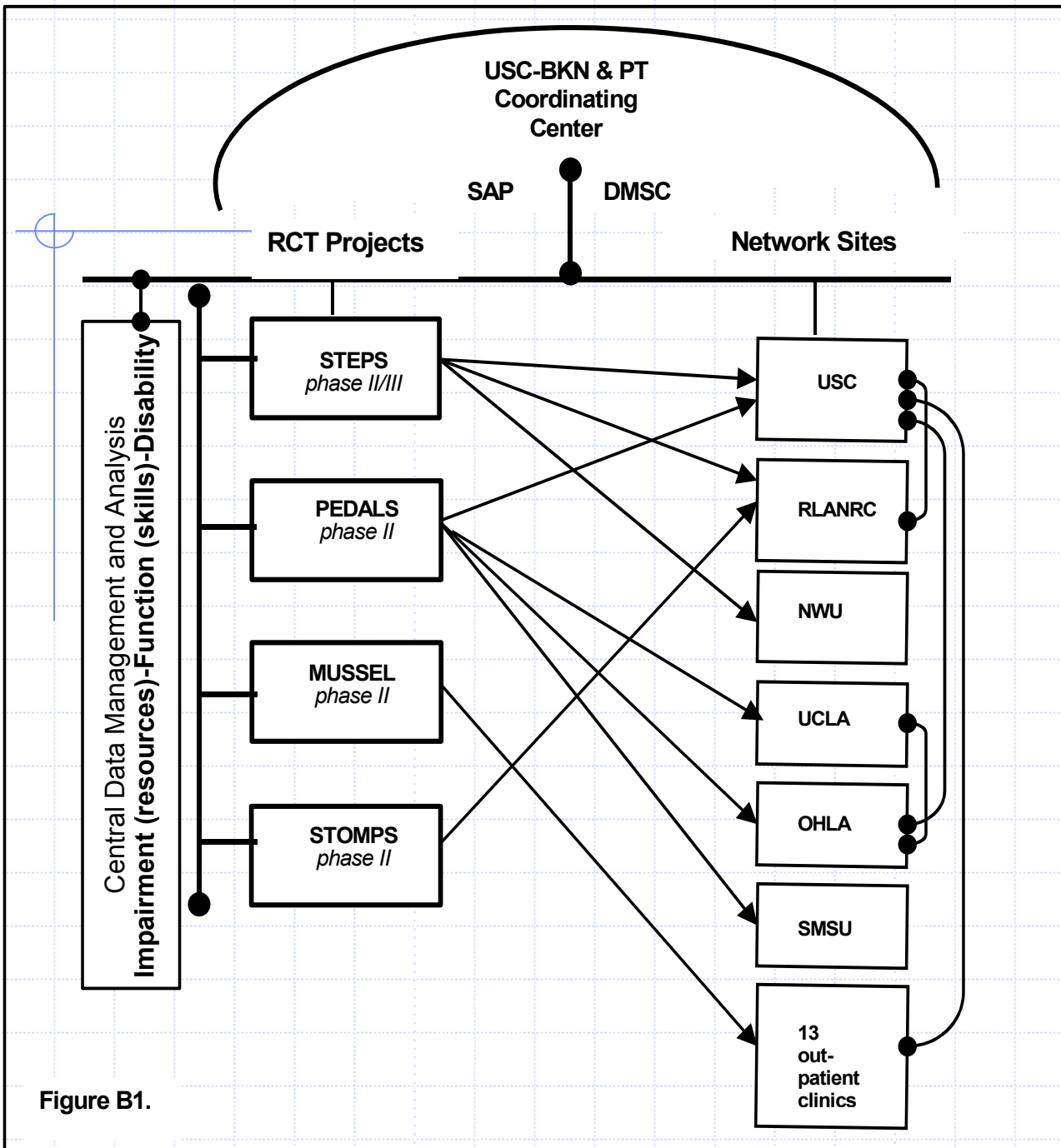


Figure B1.

# Given this focus..."enhance muscle performance"...

- ◆ The chosen *intervention* is naturally quantifiable (e.g., frequency x intensity x duration) and can be operationalized within an experimental paradigm.
- ◆ Practice variations are based upon aspects of *task specificity* and *muscle specificity* and so it is not clear how best to provide the intervention:
  - for a given patient group (with specific motor control problem)
  - a given outcome and temporal goal (long term benefit).
- ◆ Based on the state of prior knowledge (methods to enhance muscle performance), we designed *four efficacy trials* with attempts to accommodate effectiveness concerns.

# In each case, we asked....

- ◆ What is the state of prior knowledge? What should be the starting point?
  - Has efficacy (value, effects on impairment, function, disability, quality of life) for “strengthening” interventions been established in our four diagnostic groups (stroke, CP, SCI, low back)?
  - Has the optimal “dosing” and specificity (regimen) for enhancing muscle force production capabilities been identified?
    - ◆ Particularly, are there optimal protocols for enhancing muscle function that are *muscle-specific* and/or *task-specific*?

# What we know...and don't know...

The response of muscle to overload conditions is arguably one of the most robust research and practice expectations, used to great effect---at least at *impairment levels*—in informed physical therapy and human performance interventions.

(e. g., Bearne et al., 2002; Krebs et al., 1998; Landers et al., 2001; Ploutz-Snyder et al., 2002)

But, ...

- Chandler et al. Is lower extremity strength gain associated with improvement in physical performance and disability in frail, community-dwelling elders? *Arch Phys Med Rehabil.* 1998;79:24-30.
- Dolan et al. Can exercise therapy improve the outcome of microdiscectomy? *Spine.* 2000;25:1523-1532.

# What we know...and don't know...

The impact of muscle overloading interventions is not as clear for other levels of disablement...

functional-level impact      ?

disability impact              ?

For different patient groups with variable etiology

central vs peripheral

And each with a different movement problem and associated clinical problems

motor control, abnormal synergies, muscle imbalance

# Implicit Guiding Principles:



## ◆ Disablement-Rehabilitation Framework

- What is the relationship between impairment-level change, function/skill, and disability?

[In the real world, interventions must affect what is meaningful to patients and payors.]

## ◆ Maintain scientific rigor

- Design the intervention based on scientifically sound principles to enhance muscle performance
- Design to prioritize internal validity needs

[In the real world, intervention effects must be demonstrated in ways meaningful to scientists and payors.]

# Implicit Guiding Principles:



- ◆ Support effectiveness initiatives where possible to heighten the clinical meaningfulness and potential applicability of the work
  - Incorporate a balance between 'efficacy' and 'effectiveness' issues, given
    - ◆ state of the field (i.e., what has or has not been adequately demonstrated to the scientific, professional, and reimbursement communities)
    - ◆ known variations in clinical practice/exercise regimen

[In the real world, interventions must involve what is meaningful to clinicians and payors.]

# Clinical trials are staged, with movement toward establishing clinical effectiveness...

- ◆ Phase I: safety, feasibility, determine implementation parameters (e.g., dosing), side effects
- ◆ Phase II: efficacy, more safety
- ◆ Phase III: effectiveness relative to usual care/placebo/comparison interventions
- ◆ Phase IV: broader effectiveness, generalizability to other populations

## PTClinResNet clinical trials are staged...

- ◆ The first aim is to generate evidence to evaluate the 'efficacy' of physical therapist interventions focused on resistance exercise for muscle strengthening through four different randomized controlled trials.

# Four Randomized Controlled Trials:



- ◆ STEPS: David Brown, Ph.D., P.T., Lead I
  - Strength-traineffectiveness post stroke
- ◆ MUSSEL: Kornelia Kulig, Ph.D., P.T., Lead I
  - Muscle specific strengthening effectiveness post lumbar microdiscectomy
- ◆ PEDALS: Eileen Fowler, Ph.D., P.T., Lead I
  - Pediatric endurance development and limb strengthening
- ◆ STOMPS: Bryan Kemp, Ph.D., Lead I
  - Strengthening and optimal movements for painful shoulders in chronic spinal cord injury

# STEPS...to investigate the effects of strength training, as an adjunct to gait training, on locomotor outcomes in persons with chronic stroke hemiplegia

- ◆ Who: Patients with stroke, 4 mo-5 yr post)
- ◆ What: Body-weight supported treadmill training (BWSTT) and strengthening exercise
  - BWSTT and Arm ergometry (sham)
  - Locomotor-based Strength Training (LBST) and Arm ergometry (sham)
  - BWSTT and LBST
  - BWSTT and Muscle-specific Strength Training
- ◆ How: 4x/week for 6 weeks (24 sessions); standardization of interventions and assessments
- ◆ Outcomes: overground walking velocity (P), muscle function, disability
- ◆ Duration of follow-up: 6 months

# STEPS...scientific rationale

What is known?

muscle strength  
strength training



walking velocity  
↑ LE strength and  
walking velocity

task-specific training (BWSTT)

→ ↑ walking velocity

What is unknown?

What is the combined effect of strength training and task-specific training on locomotor outcomes in persons with chronic post-stroke hemiplegia?

# STEPS: Effectiveness aspects

- ◆ Many outpatient clinics have access to equipment that could be used for this exercise intervention.
- ◆ In spite of significant functional limitations including balance and walking difficulties, 46% did not receive therapy in the post-acute phase!
- ◆ For those who did, the exercise interventions provided were highly variable, not consistent with current principles of exercise training, and the intensity of the exercises was rarely progressed.

# PEDALS...to investigate the effects of a cycling exercise intervention on muscle torque, function and disability in children with Cerebral Palsy.

- ◆ Who: Children and adolescents with CP, age variability (7-11, 12-16 yr), and motor control (fair/good)
- ◆ What: stationary bicycle cycling (endurance & strength)
- ◆ How: 3x/week for 12 weeks (30 sessions)  
Standardization of intervention and assessments
- ◆ Outcomes: muscle torque, function, and disability
- ◆ Duration of follow-up: 1 month

# PEDALS...scientific rationale

## What is known?

- ◆ weakness associated with CP can be reduced with strengthening exercises
- ◆ Strength training exercises in CP does not ↑ spasticity
- ◆ Physical fitness is compromised in adults with CP
- ◆ Cardiorespiratory endurance can be improved with a cycling exercise program

## What is unknown?

- ◆ What cycling parameters are effective for children with CP
- ◆ Does the training effect outcomes beyond the impairment level?

# PEDALS...Effectiveness aspects

- ◆ Standard PT for Pediatrics varies with age of child, federal and state funding; change in medical status
- ◆ Strengthening with weights or loads are not a common tool used for children with PT in clinical practice
- ◆ If cycling is used with CP, it is to gain ROM, but not for PRE or Cardiorespiratory fitness.
- ◆ Self-efficacy with a cycling program has the potential to extend outside the clinic for these children.

# MUSSEL...

to investigate the effects of strength and endurance training on short term (6-month) and long term (5 years) improvement in function, quality of life, and re-injury prevention in people status-post microdiscectomy

- ◆ Who: Patients post-surgery who have no referral for PT [with exceptions]
- ◆ What: Endurance and Strength Training
- ◆ How: 3x/week for 12 weeks; protocol based on literature and clinicians' consensus, standardization of intervention
- ◆ Primary outcome: pain, function, disability
- ◆ Duration of follow-up: 5 years

# MUSSEL...scientific rationale

## What is known?

- ◆ Removal of degenerated disc decreases symptoms but may compromise the mobility of the segment
- ◆ Early post-surgical strengthening seems safe

## What is unknown?

- ◆ What exercise parameters are effective, and how can they be individualized?
- ◆ Does the training have a relationship to muscle morphology
- ◆ What is the long term outcome (i.e., exceeding the return of symptoms 1-3 years, "window")

# MUSSEL...Effectiveness aspects

- ◆ Many outpatient clinics have access to equipment that could be used for this exercise intervention
- ◆ Post-surgical care depends on patient's symptoms and available insurance
- ◆ Interventions are highly variable

# STOMPS

...to investigate the effects of a combined shoulder exercise and movement optimization technique program on pain reduction in chronic paraplegic-type SCI.

- ◆ Who: SCI with shoulder pain, variable SCI and pain onset, unknown/radiologically unconfirmed etiology
- ◆ What: Strengthening exercise and optimal movement technique program
- ◆ How: 12 week exercise program (home program)  
Standardization of intervention and assessments
- ◆ Outcomes: Pain (P), function, and disability
- ◆ Duration of follow-up: 1 month

# STOMPS...scientific rationale

## What is known?

- ◆ High prevalence of shoulder pain in SCI population
- ◆ Prevalence of shoulder pain increases with age, duration of injury, and level of injury.
- ◆ High prevalence of shoulder pain has been attributed to the excessive weight bearing demands and "overuse" or altered use of the upper extremities.
- ◆ Shoulder muscle strength imbalance found in symptomatic shoulders but not in controls.

## What is unknown?

- ◆ What is the combined effect of muscle-specific exercise and optimal movement training on shoulder pain reduction in paraplegic-type SCI

# STOMPS...Effectiveness aspects

- ◆ Physical therapy interventions for shoulder pain in SCI is likely sporadic at best.
- ◆ Tailored programs for SCI are rare and most likely found only in specialized areas (e.g., Model SCI Centers).
- ◆ Consumers are frustrated with the state of medical treatment for shoulder pain

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