Implementing Evidence into Practice: The Role of Pragmatic Trials

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Overview of Presentation

- Background information
- Review of terminology/concepts
  - Pragmatic trials
  - Implementation research
- COMPASS & TARGET trials
  - Implementation challenges
  - Lessons learned
- Key considerations for conducing pragmatic trials

Background

QUALITY OF HEALTH CARE DELIVERED IN THE U.S

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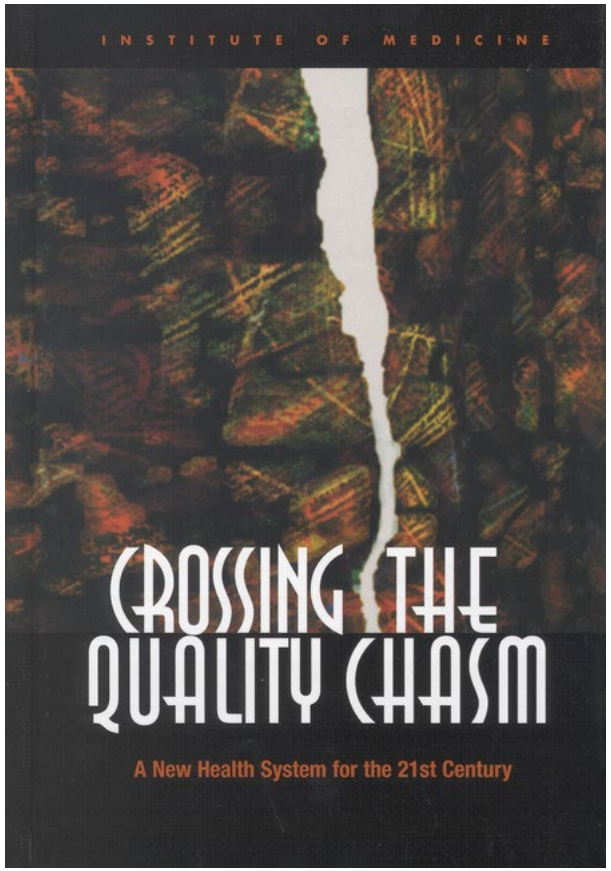
QUALITY OF HEALTH CARE THAT *SHOULD BE* DELIVERED

2001 IOM REPORT



Background

- Most of the billions of dollars the US spends each year on biomedical research is wasted.
  - Poorly designed studies
  - Biased studies
  - Asking the wrong question
  - Inadequate reporting on methods
  - Inadequate dissemination

Moher D, Glasziou P, Chalmers I, et al. Increasing value and reducing waste in biomedical research: who's listening? *Lancet*. Apr 9 2016;387(10027):1573-1586. doi:10.1016/S0140-6736(15)00307-4

Quality of Physical Therapy Care

- Variation in care not explained by “need”
- Underuse/overuse of treatments

Exercise

- Underuse: underdosing in older patients
- Overuse:
  - Outpatient versus home exercise
  - Individual versus group exercise
  - Therapist-led versus non-therapist-led exercise

Research Waste in Physical Therapy

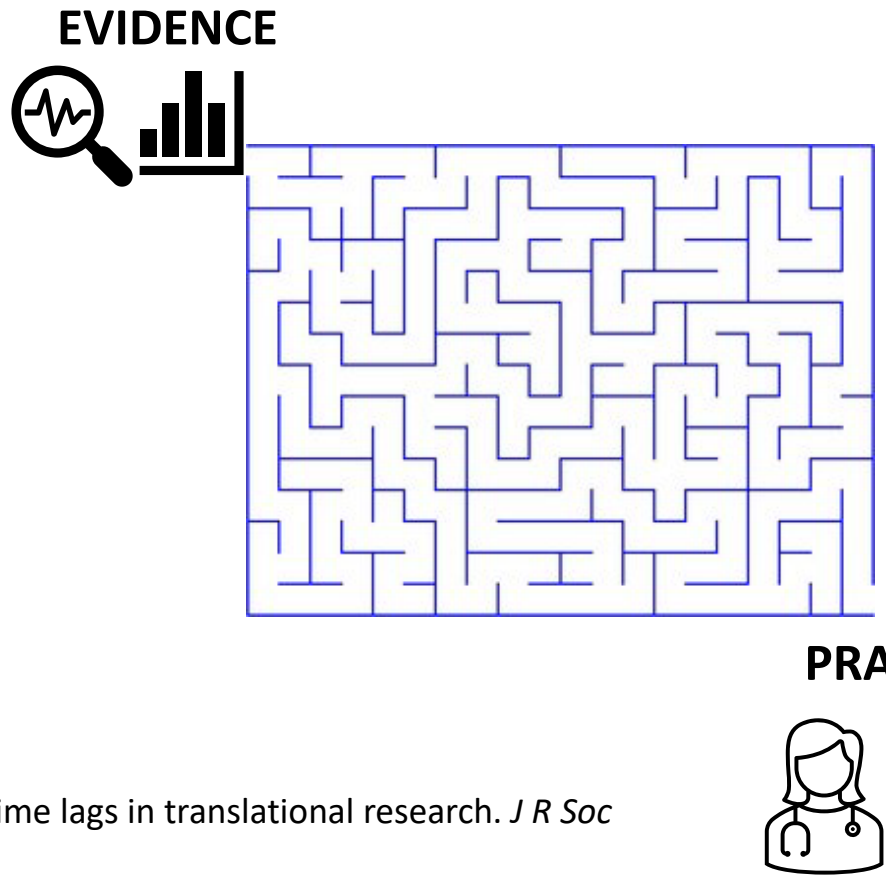
- 10-fold increase in trials for shoulder pain treatment: 31 to >300 from 1998-2014
  - Little change in the low quality of the trials
  - Little advancement in definitive conclusions on effective treatments

- Problems with musculoskeletal trials
  - Low priority questions
  - Designing trials without assessing relevant literature
  - Bias: design, reporting, publication
  - Lack of methodological detail
  - Small samples
  - Not reflective of clinical practice

Buchbinder R, Maher C, Harris IA. Setting the research agenda for improving health care in musculoskeletal disorders. *Nat Rev Rheumatol*. Oct 2015;11(10):597-605. doi:10.1038/nrrheum.2015.81

Even when we have good evidence….

- Translation is slow or non-existent
- Only 14% of research evidence makes it to practice
- 17-year lag



Morris ZS, Wooding S, Grant J. The answer is 17 years, what is the question: understanding time lags in translational research. *J R Soc Med*. Dec 2011;104(12):510-20. doi:10.1258/jrsm.2011.110180

Potential Solutions

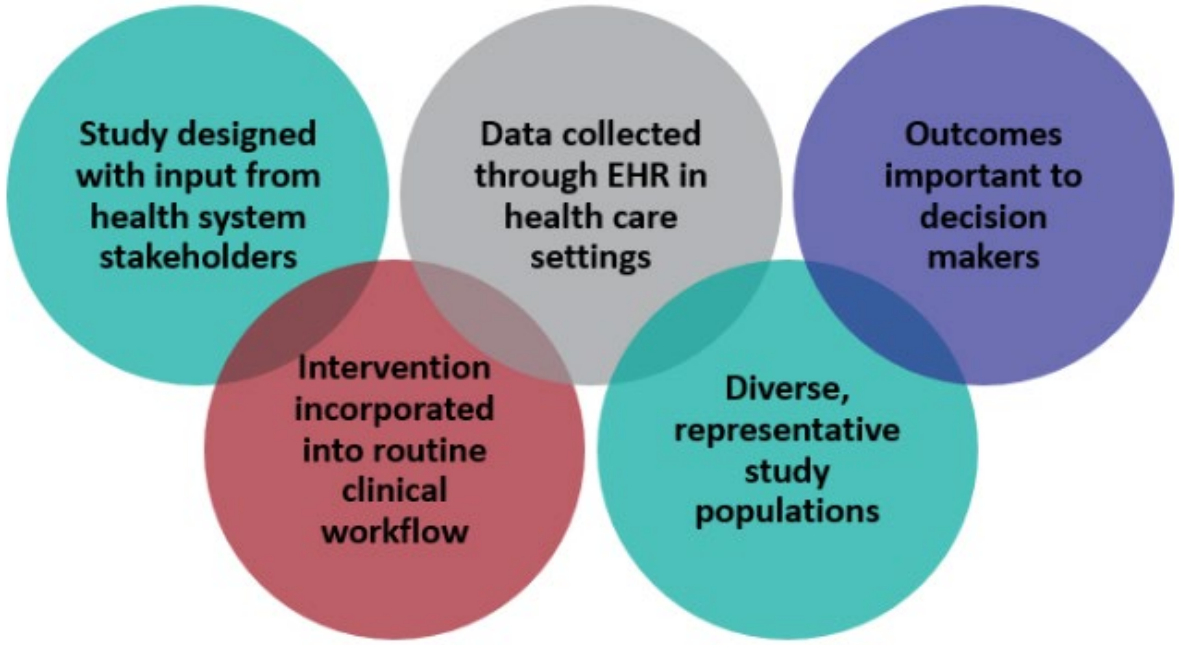
- Embedding research within real world settings
- Giving greater attention to external vs internal validity
- Practicality vs causal certainty
- Context versus methodological rigor
- Stakeholder engagement
  - Talk to end users early and often
- Design with implementation in mind

Pragmatic Trial Design

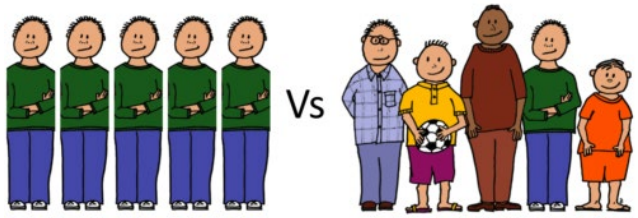
Implementation Science Methods

Pragmatic Clinical Trial

Designed for the primary purpose of informing decision-makers regarding the comparative balance of benefits, burdens and risks of a biomedical or behavioral health intervention at the individual or population level. [Califf and Sugarman 2015]



NIH Collaboratory Rethinking Clinical Trials - The Living Textbook - Rethinking Clinical Trials

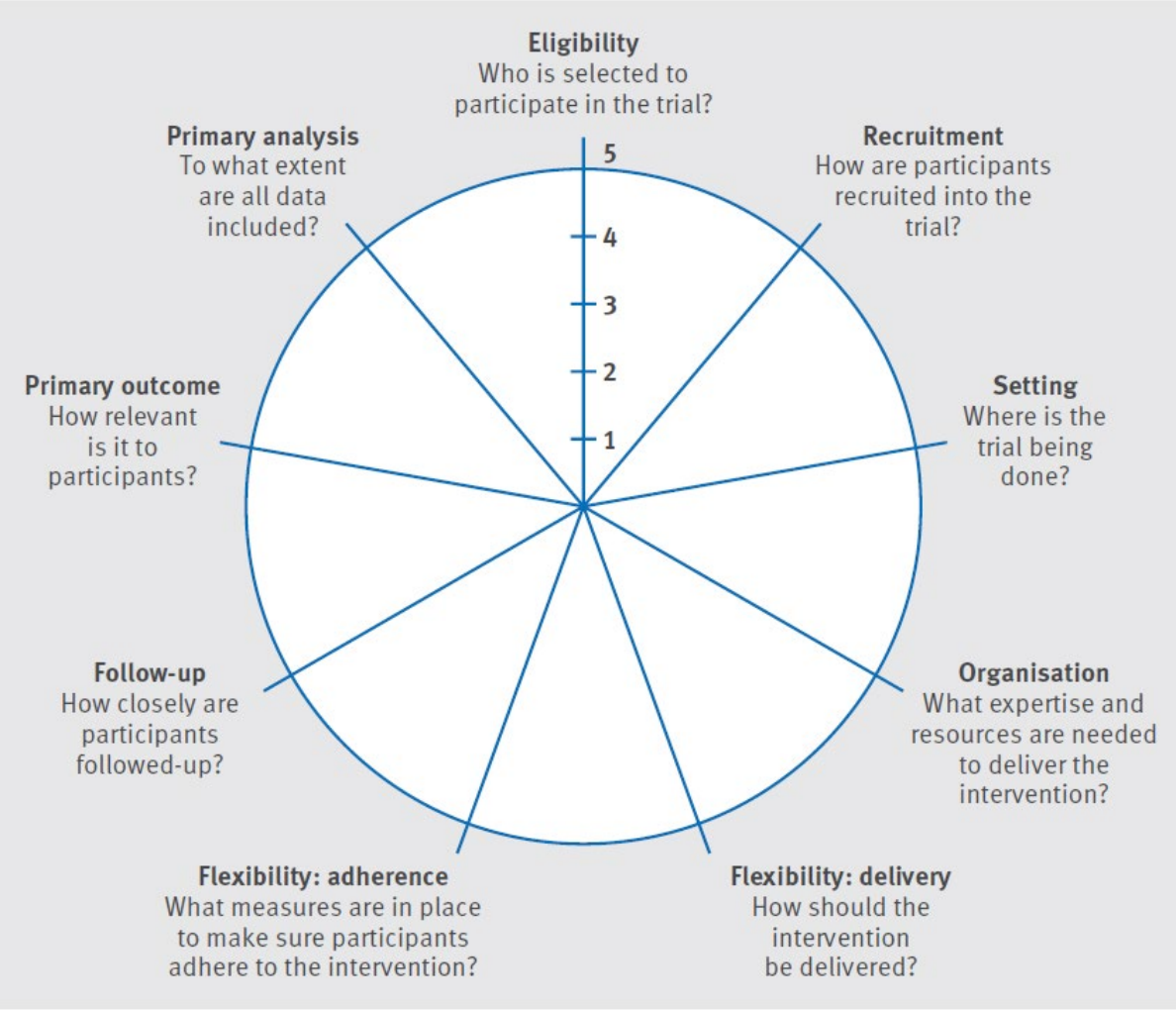


Explanatory/Pragmatic Continuum

(Efficacy vs Effectiveness)

Attribute	Explanatory RCT	Pragmatic RCT/Quasi-experimental Design
Question	Does the intervention work under ideal circumstances?	Does the intervention work in real-world practice?
Setting	Resource-intensive, ideal setting (e.g., academic medical center)	Real world, everyday clinical setting where care typically delivered
Study Population	Highly selected, homogenous, strict inclusion/exclusion criteria, subject incentives	Heterogenous population, few incl./excl. criteria
Recruitment	Resource intensive	Recruited during usual care
Randomization	Randomized, blinded	May or may not be randomized
Providers	Highly experienced, trained	Representative, usual providers
Intervention	Strictly enforced & standardized, no concurrent interventions, adherence monitored	Applied with flexibility, concurrent interventions, no special efforts to enforce intervention management
Comparator	Placebo, sham	Active comparator; usual care
Outcomes	Objective, higher subject burden, surrogate measures	Patient-centered, patient-reported, lower subject burden
Primary analysis	Excludes non-compliant participants	Intention to treat analysis
Validity	High internal validity	High external validity

PRECIS-2 Tool



The PRagmatic-Explanatory Continuum Indicator Summary 2 (PRECIS-2) wheel.

1. Very explanatory
2. Rather explanatory
3. Equally pragmatic and explanatory
4. Rather pragmatic
5. Very pragmatic

PRECIS-2 Tool*

DOMAIN	Definition
Eligibility	To what extent are the participants in the trial like those who would receive this intervention if it was part of usual care?
Recruitment	How much extra effort is made to recruit participants over and above what would be used in the usual care setting to engage with patients?
Setting	How different are the settings of the trial from the usual care setting?
Organization	How different are the resources, provider expertise, and the organization of care delivery in the intervention arm of the trial from those available in usual care?
Flexibility (delivery)	How different is the flexibility in how the intervention is delivered and the flexibility anticipated in usual care?
Flexibility (adherence)	How different is the flexibility in how participants are monitored and encouraged to adhere to the intervention from the flexibility anticipated in usual care?
Follow-up	How different is the intensity of measurement and follow-up of participants in the trial from the typical follow-up in usual care?
Primary outcome	To what extent is the trial's primary outcome directly relevant to participants?
Primary analysis	To what extent are all data included in the analysis of the primary outcome?

*Loudon et al. BMJ 2015: 350: h2147

Implementation Research

"the scientific study of the development and use of strategies to adopt and integrate evidence-based health interventions into clinical and community settings to improve individual outcomes and benefit population health."

Seeks to understand the within context behavior of individuals, communities, & organizations that influence the adoption, implementation, and sustainability of evidence-based interventions.

- Practitioners & support staff
- Consumers & family members
- Organizations
- Policymakers

NIH definition: PAR-22-105

Implementation Science Terminology

- **Innovation:** the object of the implementation process (includes evidence-based interventions, guidelines, practices, devices, policies); "the thing" we are trying to implement.
- **Adoption:** active or intentional decision to incorporate an innovation (can occur at the individual, organizational, community level)
- **Implementation:** the act of putting an innovation into actual use
- **Sustainability:** the extent to which a newly implemented treatment, practice, etc. is maintained or institutionalized within a service setting's ongoing, stable operations
- **Implementation Strategy:** actions taken to enhance adoption, implementation, and sustainability of evidence-based interventions
- **Fidelity:** the degree to which the intervention was implemented as intended
- **Penetration/Reach (patient level):** the extent to which individuals eligible to receive the evidence-based intervention actually receive it

Proctor et al. 2011, PMID: 20957426

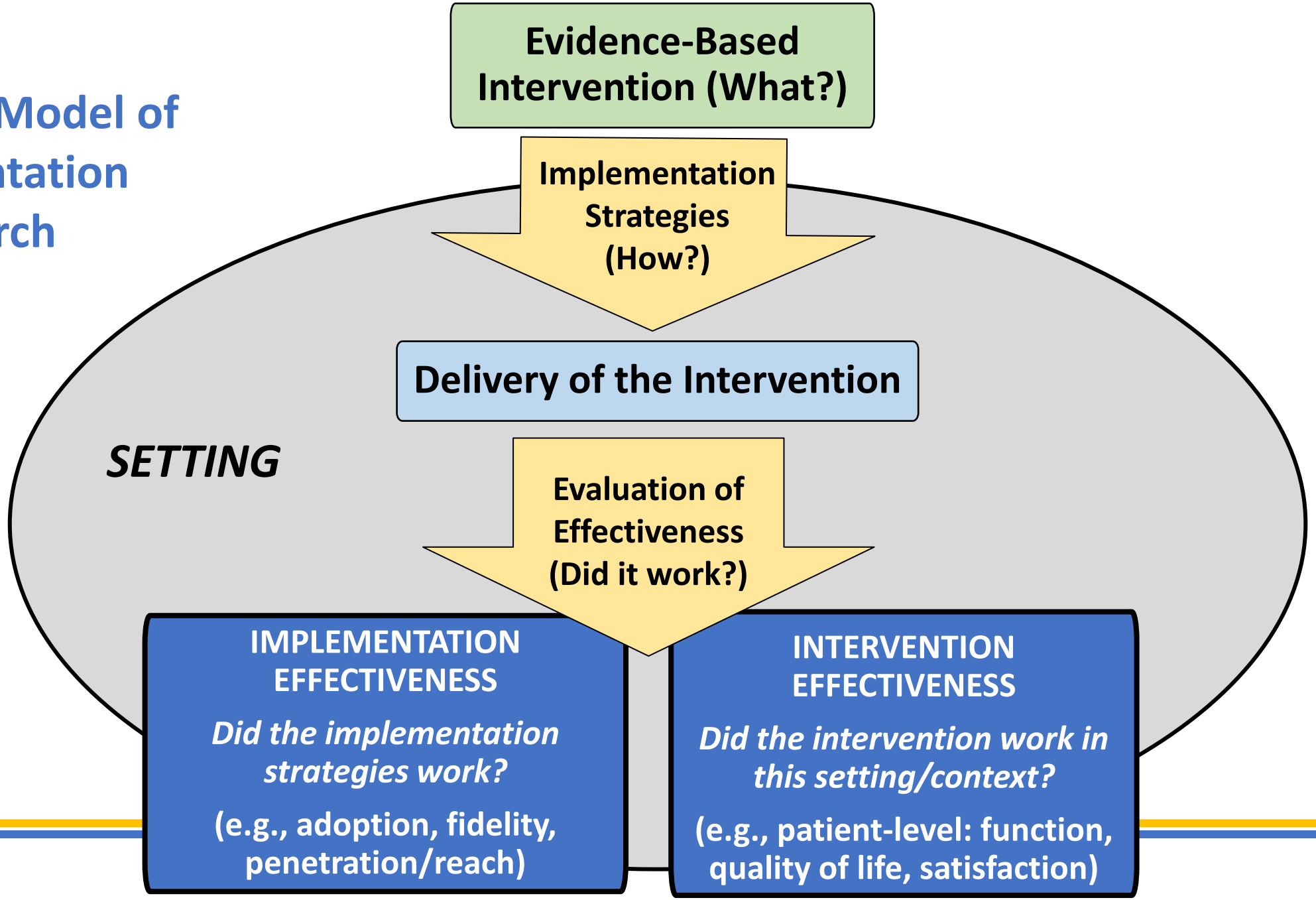
Intervention versus Implementation Effectiveness

- **Intervention Effectiveness:** does the intervention improve patient outcomes?

- **Implementation Effectiveness:** how much and how well are providers/organizations using the evidence-based intervention?

Curran: Implementation Science Communications (2020) 1:27 https://doi.org/10.1186/s43058-020-00001-Implementation science made too simple: a teaching tool

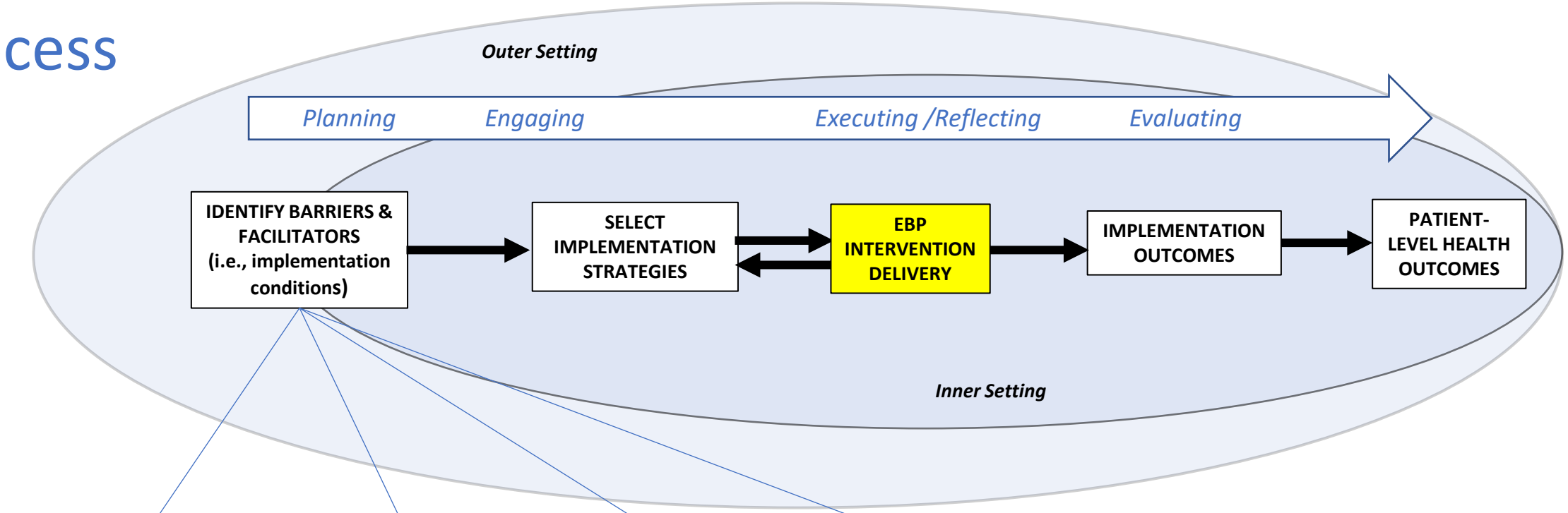
Conceptual Model of Implementation Research



Factors Influencing Implementation of an Innovation

- Outer Setting/External Environment
- Inner Setting (Organization)
- Individuals Involved
  - Providers
  - Patients/Caregiver
- Innovation

Implementation Framework & Process



Outer Setting

- Patient needs & resources
- External policies & incentives

Inner Setting (Practice-level)

- Culture
- Implementation climate
- Readiness for implementation
- Tension for change
- Relative priority
- Leadership engagement
- Available resources
- Learning climate
- Goals and feedback
- Incentives & Rewards

Individuals Involved

- Knowledge & beliefs
- Self-efficacy
- Identification with organization
- Other personal attributes

Intervention Characteristics

- Evidence strength
- Relative advantage
- Adaptability
- Complexity
- Costs

Consolidated Framework for Implementation Research https://cfirguide.org/

Implementation Strategies

Use evaluative & iterative strategies

- Identify barriers & facilitators
- Assess for readiness
- Examination of implementation

Adapt and tailor to context

- Tailor strategies
- Feedback from providers
- Promote adaptability

Train & educate stakeholders

- Ongoing training
- Educational materials
- Train the trainer

Change infrastructure

- Data systems
- Physical environment
- Equipment

Provide interactive assistance

- Facilitation
- Local technical assistance
- Clinical supervision

Develop stakeholder relationships

- Identify & prepare champions
- Implementation meetings
- Identify early adopters

Financial Strategies

- Incentive/allowance structure
- Fund innovation
- Access new funding

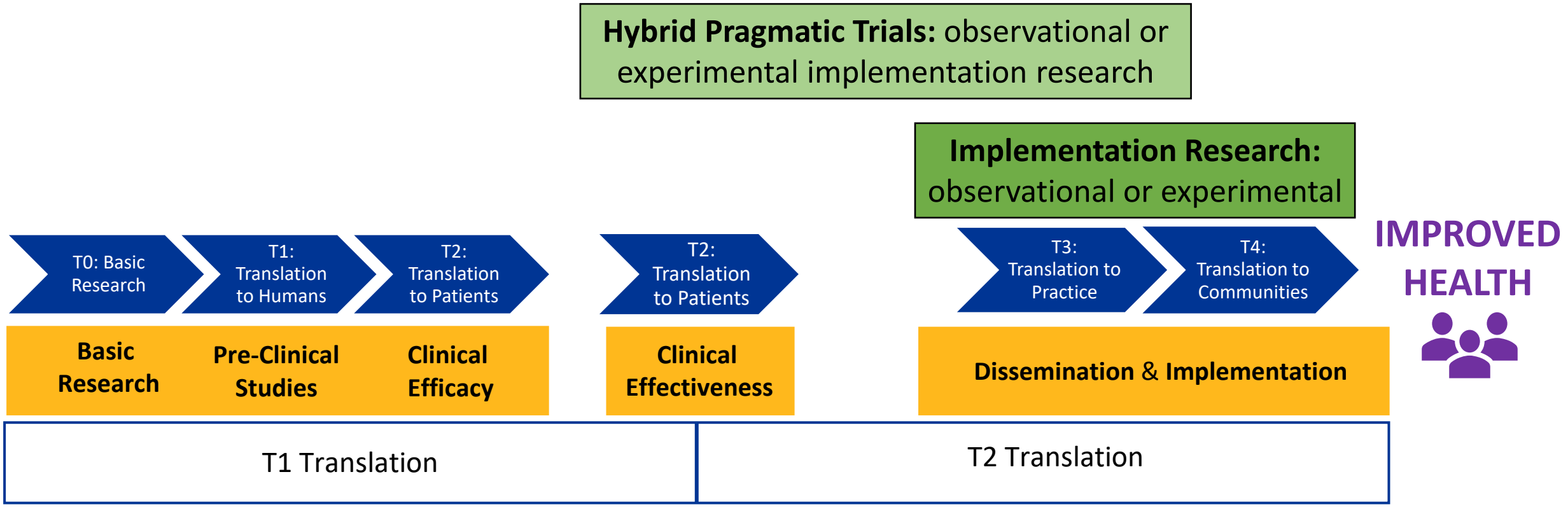
Engage consumers

- Patient/family input
- Increase demand
- Use mass media

Clinician support

- Remind clinicians
- Audit & feedback
- Revise professional roles

Translational Continuum & Implementation Reserach



Effectiveness-Implementation Hybrid Designs

DESIGN TYPE	PRIMARY AIM	SECONDARY AIM
Type 1	Determine Effectiveness of an Intervention	Better understand context for implementation/barriers & facilitators
Type 2	Determine Effectiveness of an Intervention	Determine feasibility and/or (potential) utility of an implementation strategy
Type 3	Determine Effectiveness/Impact of an Implementation Strategy	Assess clinical outcomes associated with implementation

Curran GM, Bauer M, Mittman B, Pyne JM, Stetler C. Effectiveness-implementation hybrid designs: combining elements of clinical effectiveness and implementation research to enhance public health impact. *Med Care*. Mar 2012;50(3):217-26. doi:10.1097/MLR.0b013e3182408812

COMPASS

COMPREHENSIVE POST-ACUTE STROKE SERVICES

Circulation: Cardiovascular Quality and Outcomes
Volume 13, Issue 6, June 2020;: e006285.
https://doi.org/10.1161/CIRCOUTCOMES.119.006285

American Heart Association.

ORIGINAL ARTICLE

Randomized Pragmatic Trial of Stroke Transitional Care

The COMPASS Study

See Editorial by Reeves

Pamela W. Duncan, PhD, PT, Cheryl D. Bushnell, MD, MHS, Sara B. Jones, PhD, MPH, Matthew A. Psioda, PhD, Sabina B. Gesell, PhD, Ralph B. D'Agostino Jr, PhD, Mysha E. Sissine, MSPH, Sylvia W. Coleman, MPH, RN, BNS, CLNC, Anna M. Johnson, PhD, MSPH, Blair F. Barton-Percival, MSW, Janet Prvu-Bettger, ScD, MS, Adrienne G. Calhoun, MA, Doyle M. Cummings, PharmD, Janet K. Freburger, PhD, PT, MS, Jacqueline R. Halladay, MD, MPH, Anna M. Kucharska-Newton, PhD, MPH, Gladys Lundy-Lamm, MA, Barbara J. Lutz, PhD, RN, Laurie H. Mettam, MEd, Amy M. Pastva, PhD, PT, MA, James G. Xenakis, PhD, Walter T. Ambrosius, PhD, Meghan D. Radman, MPH, Betsy Vetter, Wayne D. Rosamond, PhD, MS, and on behalf of the COMPASS Site Investigators and Teams.

ELSEVIER

Contents lists available at ScienceDire

EClinicalMedicine

journal homepage: https://www.journals.elsevier.com/eclinicalmedicine

Research Paper

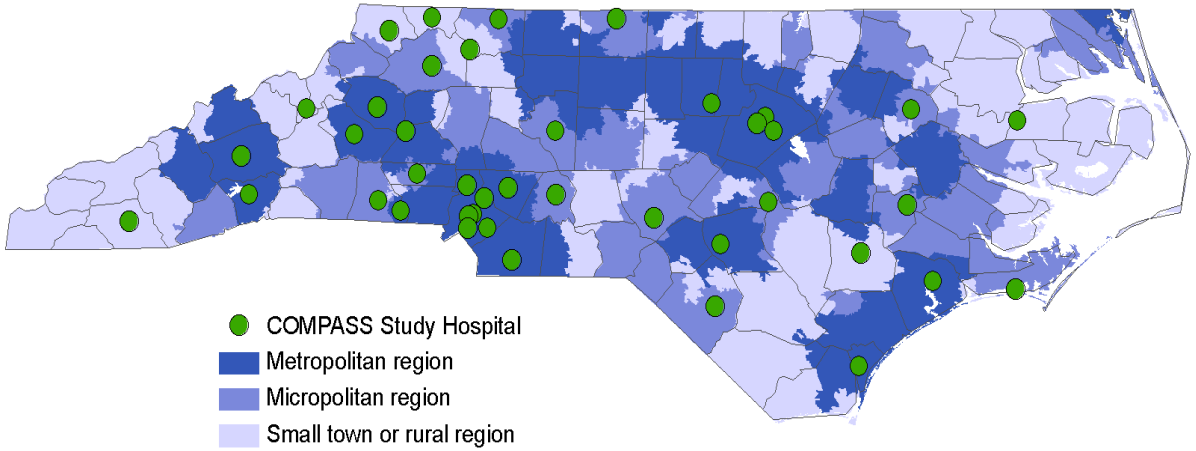
Stratified care to prevent chronic low back pain in high-risk patients: The TARGET trial. A multi-site pragmatic cluster randomized trial

Anthony Delitto[a,*], Charity G. Patterson[b], Joel M. Stevans[b], Janet K. Freburger[b], Samannaaz S. Khoja[b], Michael J. Schneider[b], Carol M. Greco[c], Jennifer A. Freel[d], Gwendolyn A. Sowa[e], Ajay D. Wasan[f], Gerard P. Brennan[g], Stephen J. Hunter[g], Kate I. Minick[g], Stephen T. Wegener[h], Patti L. Ephraim[i], Jason M. Beneciuk[j], Steven Z. George[k], Robert B. Saper[l]

TARGETTRIAL

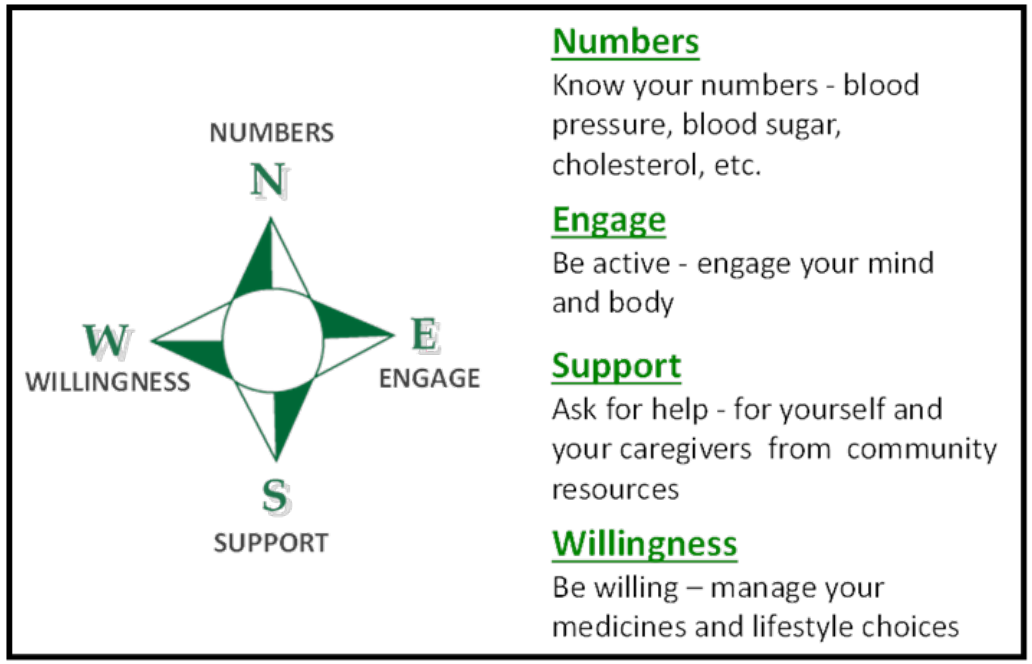
The Comprehensive Post-Acute Stroke Services (COMPASS) Trial

- PCORI-funded, cluster-randomized trial of 40 hospitals in North Carolina (enrolled 6,024 patients)
- Examined the comparative effectiveness of the COMPASS transitional care model (COMPASS TC) vs usual care
  - Primary outcome: self-reported function at 90 days
  - Several secondary outcomes
- Secondary aim to examine implementation of COMPASS TC (Hybrid Type 1)



The COMPASS Intervention (Evidence-based Practice)

- **Post-acute care coordinator and advanced practice provider (PA, NP) take ownership of the transition to home.**
- **2-day call & 2-week visit**
- **E-care plan generated at 2-week visit**



The TARGET Trial

- PCORI-funded, cluster-randomized trial of 72 primary care practices from 4 health systems (enrolled 2,300 patients)
- Examined the comparative effectiveness of a risk-stratified approach versus usual care for treatment of acute low back pain
    - Primary outcomes: transition to chronic pain; self-reported disability at 90 days
- Secondary aim to examine implementation of stratified approach (Hybrid Type 1)

TARGETTRIAL

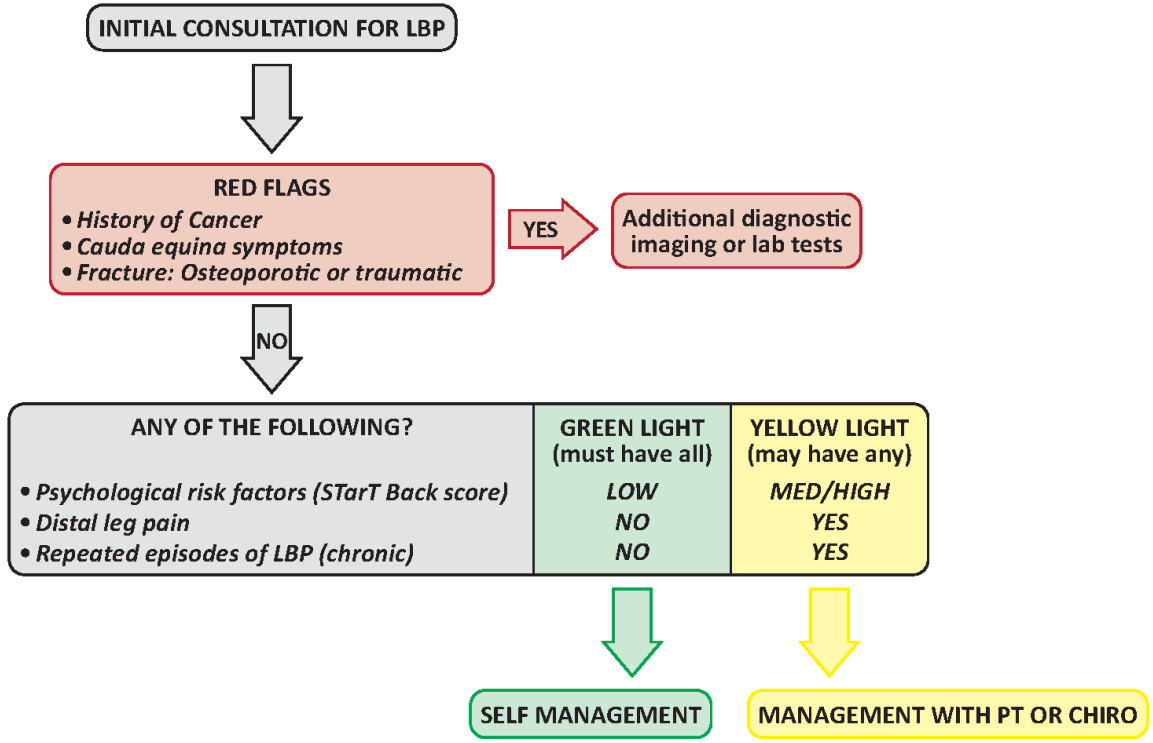
The TARGET Intervention (Evidence-Based Approach)

Figure 7 - STarT Back Risk Stratification Tool Questions

Thinking about the last 2 weeks, do you agree or disagree with the following:

1. My back pain has **spread down my leg(s)** at some point in the last 2 weeks.
2. I have had pain in the **shoulder** or **neck** at some time in the last 2 weeks.
3. I have **only walked short distances** because of my back pain.
4. I have **dressed more slowly** than usual because of back pain.
5. It's **not safe** for a person with a condition like mine to be physically active.
6. **Worrying thoughts** have been going through my mind a lot of the time.
7. I feel that **my back pain** is terrible and **it's never going to get any better**.
8. In general I have **not enjoyed** all the things that I used to enjoy.
9. Overall, how **bothersome** has your back pain been in the last 2 weeks?

SCORING: **LOW Risk** - 3 or less items **MED/HIGH risk** - 4 or more items

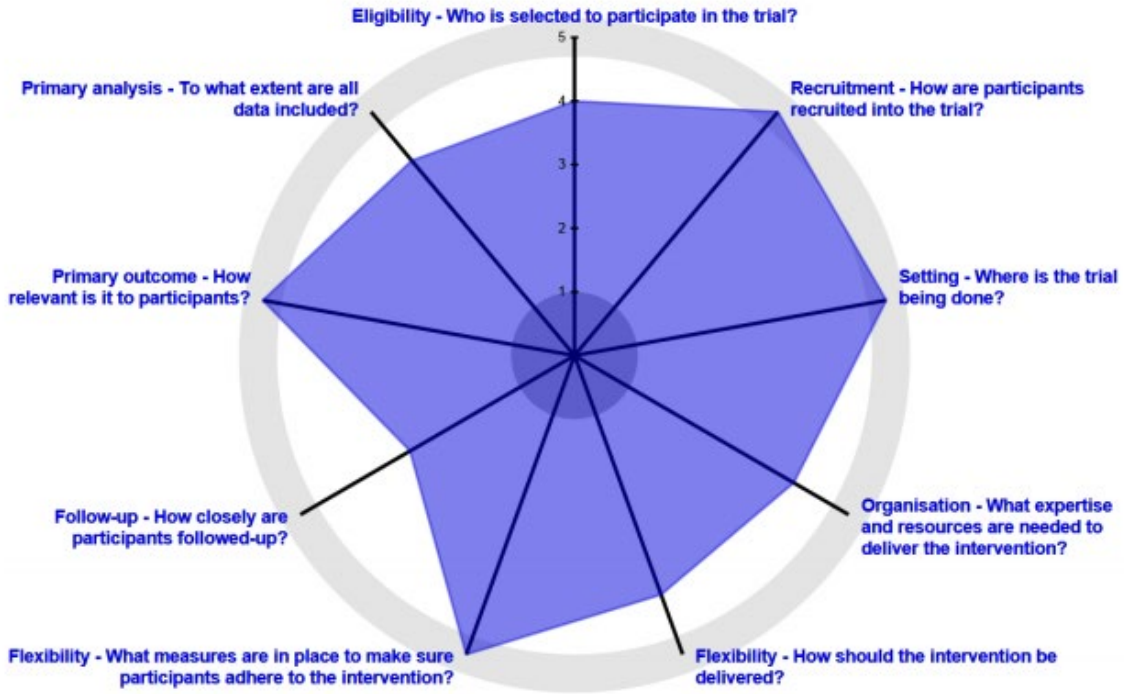


Risk stratify LBP patients and refer high risk patients to psychologically informed PT.

PRagmatic Explanatory Continuum Indicator Summary (PRECIS Tool)

COMPASS TRIAL

eFigure 1: PRECIS-2 Scoring of the COMPASS Study



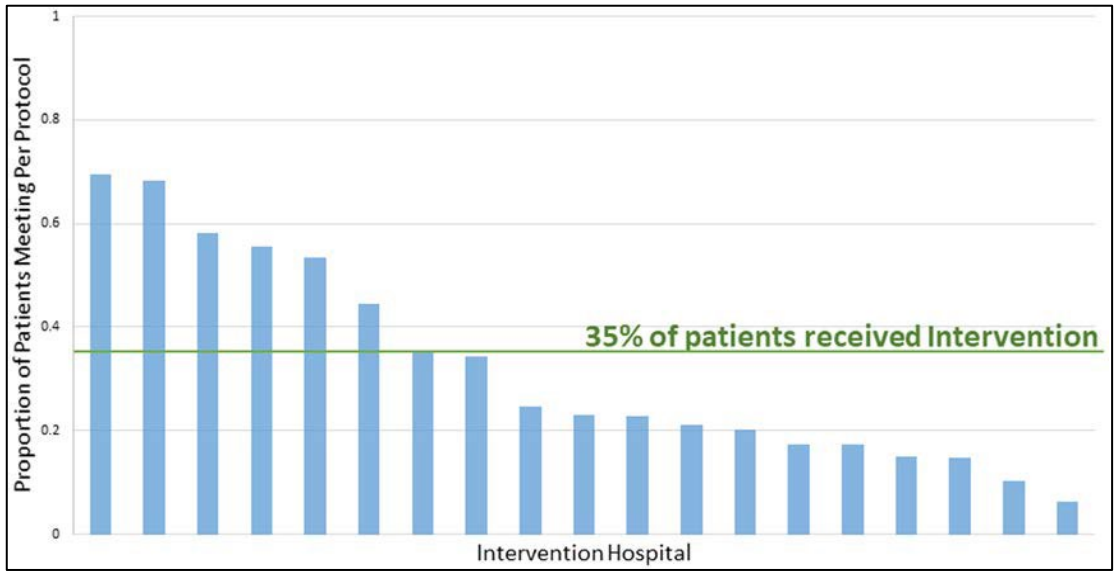
TARGET TRIAL

Fig. 2. TARGET Trial scored on Pragmatic Explanatory Continuum Indicator Summary 2 (PRECIS-2) wheel.

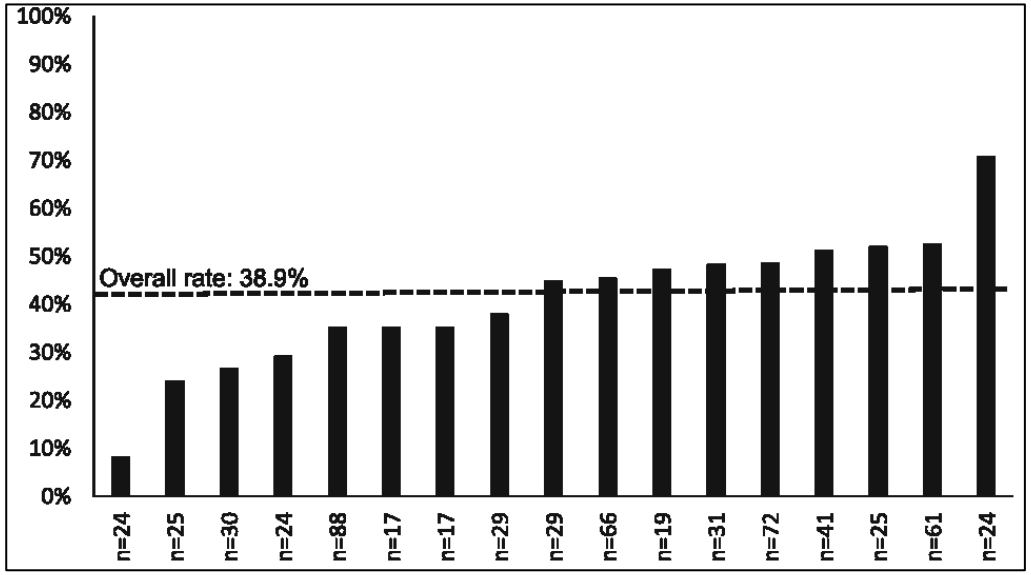
COMPASS & TARGET Trials

- Both were negative trials
- Powered to detect a difference
- Both had challenges with implementation of the intervention

COMPASS Intervention Hospitals (n=19)



TARGET Intervention Primary Care Clinics (n=17)



Implementation in COMPASS & TARGET Trials

Gesell *et al. BMC Health Services Research* (2019) 19:978
https://doi.org/10.1186/s12913-019-4771-0

BMC Health Services Research

RESEARCH ARTICLE Open Access

Implementation of a billable transitional care model for stroke patients: the COMPASS study

Sabina B. Gesell[1*], Cheryl D. Bushnell[2], Sara B. Jones[3], Sylvia W. Coleman[2], Samantha M. Levy[4], James G. Xenakis[5], Barbara J. Lutz[6], Janet Prvu Bettger[7], Janet Freburger[8], Jacqueline R. Halladay[5], Anna M. Johnson[3], Anna M. Kucharska-Newton[3,9], Laurie H. Mettam[3], Amy M. Pastva[7], Matthew A. Psioda[4], Meghan D. Radman[2], Wayne D. Rosamond[3], Mysha E. Sissine[2], Joanne Halls[10] and Pamela W. Duncan[2]

Middleton *et al. BMC Musculoskeletal Disorders* (2020) 21:776
https://doi.org/10.1186/s12891-020-03800-6

BMC Musculoskeletal Disorders

RESEARCH ARTICLE Open Access

Implementing stratified care for acute low back pain in primary care using the STarT Back instrument: a process evaluation within the context of a large pragmatic cluster randomized trial

Addie Middleton[1*], G. Kelley Fitzgerald[2], Anthony Delitto[3], Robert B. Saper[4], Katherine Gergen Barnett[4] and Joel Stevans[2,5]

ACRM AMERICAN CONGRESS OF REHABILITATION MEDICINE

Archives of Physical Medicine and Rehabilitation

journal homepage: www.archives-pmr.org

Archives of Physical Medicine and Rehabilitation 2020; ■:■■■-■■■

SPECIAL COMMUNICATION

Movement Matters, and So Does Context: Lessons Learned From Multisite Implementation of the Movement Matters Activity Program for Stroke in the Comprehensive Postacute Stroke Services Study

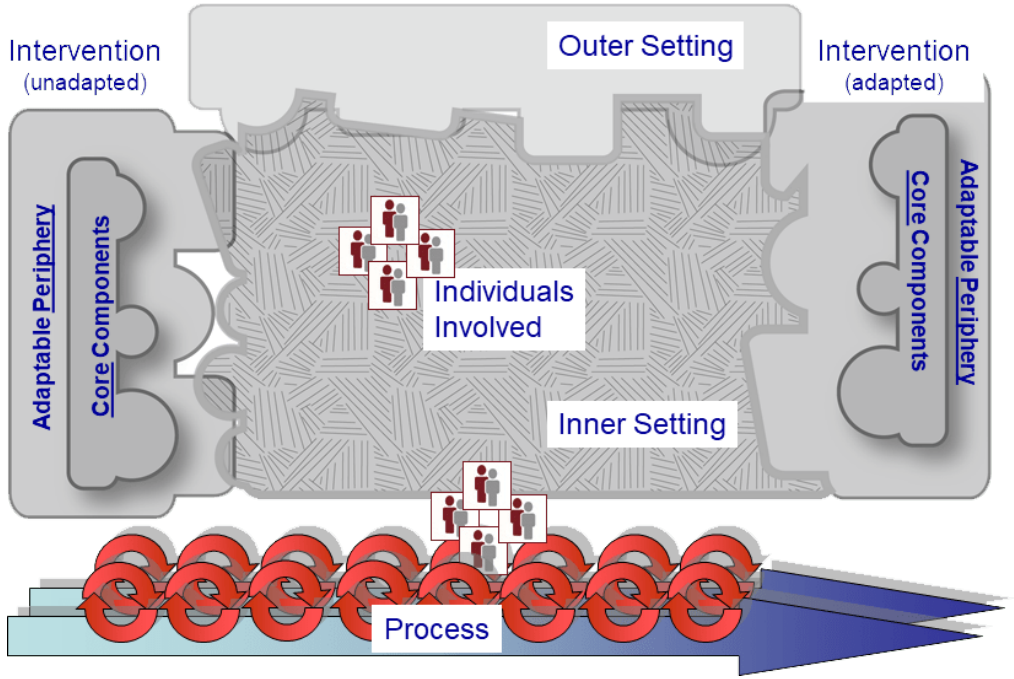
Amy M. Pastva, PhD, PT, MA,[a] Peter C. Coyle, PT, DPT, PhD,[b] Sylvia W. Coleman, MPH, RN, BNS, CLNC,[c] Meghan D. Radman, MPH,[d] Karen M. Taylor, MPT,[c] Sara B. Jones, PhD, MPH,[d] Cheryl D. Bushnell, MD, MHS, FAHA,[c] Wayne D. Rosamond, PhD, MS, FAHA,[d] Anna M. Johnson, PhD, MSPH,[d] Pamela W. Duncan, PhD, PT, FAPTA, FAHA,[c] Janet K. Freburger, PhD, PT, MS,[b] for the COMPASS Investigative Team

Implementation Frameworks

REAIM: Evaluation Framework

R	REACH
E	EFFECTIVENESS
A	ADOPTION
I	IMPLEMENTATION
M	MAINTAINANCE

CFIR Framework



COMPASS Trial: Barriers (also Facilitators!) to Implementation

Inner Setting

- Hospital culture
- Hospital administration
- Readiness for change/resources

Outer Setting

- Community-Based Healthcare Providers
- Availability of Services
- Patient transportation
- Patient Insurance

Individuals involved (hospital staff, patients)

- Self-efficacy
- Identification with organization
- Knowledge & beliefs

Intervention Characteristics

- Relative advantage
- Complexity/Adaptability
- Cost
- Patient Insurance

COMPASS - Implementation Strategies

- 2-day boot camp for intervention hospital staff
- Bi-monthly peer problem solving calls
- One-on-one same day consulting
- Financial support for the post-acute care coordinator
- Leadership buy-in, front-line champions
- Changed EHR structure/processes for identifying stroke patients likely to be discharged home
- Monthly quality metrics
- Feedback/input from stakeholders

Tailored Strategies – Adapt Intervention

Tailor Strategies

- 6-hour site visit to tailor implementations strategies.
- Monthly data feedback on performance led to new implementation strategies.

Adapt Intervention

- Relaxed requirements on time to 2-day call and 14-day clinic visit.
- Stream-lined assessments at the 14-day clinic visit.

Successful Implementation of COMPASS Intervention

- Hospitals: in rural settings, higher organizational readiness, vertically integrated systems
- Providers: empowered middle management leaders, self-identified champions
- Patients: insured, rural residence, living closer to clinic

- Within hospital analysis – physical function improved in patients who actually received the COMPASS intervention

Implementation of TARGET Intervention

- Screening with START Back (37.8% of LBP patients screened 9,030/23,913) of those identified as high-risk: 39% referred

Barriers	Facilitators	Solution (future)
• Physician engagement • Workflow • Time • Patient's needs • Staff knowledge/beliefs • Technology	• Adaptability of process • Individual collecting the data	• Front desk screen or screen prior to visit • Automate referral to PT (MD has to opt out)

Key Considerations for Conducting Pragmatic Trials

- Build partnerships/develop team – engage end users *early and often*
- Select topics of mutual interest – leadership buy-in
- Keep the intervention as simple as possible
  - Feedback from providers/staff on intervention & implementation strategies
- Plan for sustainability from the beginning
  - Design for implementation (both the intervention and the strategies)
  - Tools that can remain after you leave
- Choose the right outcomes
  - Intervention & Implementation Effectiveness (meaningful, low burden, actionable)

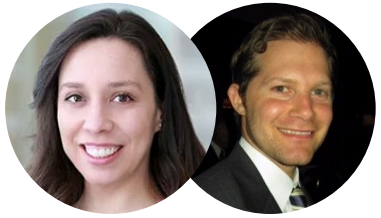
Key Considerations for Conducting Pragmatic Trials

- Consider data infrastructure issues early
- Consider study oversight
  - Quality improvement versus research?
  - Informed Consent
- Pilot test!! Vanguard site (Wake Forest)
- Plan for/expect changes to happen – embrace this
- Consider dissemination of findings/communication with stakeholders
  - Meaningful ways to communicate with stakeholders during and after the trial

Fall Webinar Series: Using Health System Research to Revolutionize Rehabilitation Care (https://sites.brown.edu/learrn/)

Cara Lewis, PhD, HSPP
Pragmatic Measurement of Implementation Outcomes: How to Operationalize Outcomes Important to Health Systems
September 16, 2022 – 1:00-2:30 PM ET

Catherine Quatman-Yates, PT, DPT, PhD
Selecting Rehabilitation Metrics that Matter to Guide Transformative Improvements for Patients, Clinicians, Health Systems, and Society
September 21, 2022 – 1:00-2:30 PM ET



Elena Mendez Escobar, PhD, MBA
John Goldie, MBA
Health Equity Accelerator – BMC's Approach to Health Justice
October 7, 2022 – 12:00-1:30 PM ET

Ericka Merriweather, PT, DPT, PhD
Confronting Racism in Pain and Rehabilitation Research: Reframing and Reimagining Study Designs
October 28, 2022 – 11:00 AM - 12:30 PM ET

Julie Fritz, PT, PhD, FAPTA
Pragmatic and Embedded Trials: Strategies for Generating Real World Evidence
November 14, 2022 – 1:00-2:30 PM ET

Elizabeth Skidmore, PhD, OTR/L, FAOTA, FACRM
Engaging Stakeholders and Health Systems to Optimize Pragmatic Trials
November 30, 2022 – 1:00-2:30 PM ET

LeaRRn

BROWN

BOSTON UNIVERSITY

University of Pittsburgh

MR3 Medical Rehabilitation Research Resource NETWORK

NIH Eunice Kennedy Shriver National Institute of Child Health and Human Development

Rehabilitation Clinical Trials: Innovations, Designs, and Tribulations

2nd Annual Scientific Retreat

September 29-30 2022 | 11am EDT via Zoom

Keynote Speakers

Catherine Lang, PT, Ph.D., FAPTA
Professor of Physical Therapy, Neurology, Occupational Therapy
Washington University School of Medicine

Wendy Weber, ND, Ph.D., MPH
Chief, Clinical Research in Complementary and Integrative Health Branch
NIH National Center for Complementary and Integrative Health

Closing Remarks

Theresa Cruz, PhD
Director, NIH-NICHD National Center for Medical Rehabilitation Research (NCMRR)

THANK YOU
&
QUESTIONS