NIH PRAGMATIC TRIALS COLLABORATORY
Rethinking Clinical Trials®

HCSRN 2022 Annual Conference
Essentials of Embedded Pragmatic Clinical Trials Workshop
April 11, 2022

Title: Essentials of Embedded Pragmatic Clinical Trials

Program Description

This workshop introduces concepts in the design, conduct, and implementation of embedded pragmatic clinical trials (ePCTs), with a particular focus on methods relevant to health services researchers. ePCTs are randomized trials conducted within health care systems and use streamlined procedures and existing infrastructure to answer important medical questions for patients, providers, and health system leaders. Such trials have the potential to inform policy and practice with broadly generalizable, high-quality evidence at lower cost and greater efficiency compared with traditional explanatory clinical trials. The workshop will provide an introduction to the investigative opportunities for embedded health systems research, along with strategies for conducting clinical trials that provide real-world evidence necessary to inform both practice and policy. Firsthand ePCT experiences and case studies from the NIH Health Care Systems Research Collaboratory Demonstration Projects will support and illustrate the topics presented.

Learning Objectives

1. To clarify the definition of ePCTs and explain their utility.
2. To introduce attendees to the unique characteristics and challenges of designing, conducting, and implementing ePCTs within diverse health care systems.
3. To increase the capacity of health services researchers to address important clinical questions with ePCTs.

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HCSRN Annual Conference – Pasadena CA

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Agenda

April 11, 2022			
DURATION	**TOPIC**	**SPEAKERS**	**GOALS**
1:00 – 1:05 p.m.	**Welcome**	Kevin Weinfurt	• Meeting goals and expectations • Introductions
1:05 – 1:30 p.m. 25 mins	**What Are Embedded PCTs (ePCTs)?**	Kevin Weinfurt	• Identify key considerations in the design and conduct of ePCTs and how they differ from explanatory trials • Learn why a critical element in the success of an ePCT is engaging health system partners at all levels and through all phases of the study • Understand the real-world priorities and perspectives of health system leaders and how to obtain their support • Identify challenges of partnering across diverse health systems
1:30 – 1:45 p.m. 15 mins	**Objectives and Trial Design:** ***An Overview of Hybrid Designs***	Devon Check	• Overview of the 3 types of effectiveness-implementation hybrid trial designs and when they may be appropriate for ePCTs
1:45 – 2:15 p.m. 30 mins	**Measuring Outcomes**	Devon Check	• Describe methods for measuring outcomes using data sources such as electronic health records (EHRs) and patient-reported outcomes (PROs)
2:15 – 2:45 p.m. 30 mins	**ePCT Experimental Design & Analysis**	Patrick Heagerty	• Learn about cluster-randomized and stepped-wedge study designs • Recognize the analytical challenges and trade-offs of pragmatic study designs, focusing on what PIs need to know **Includes Q&A with attendees
2:45 – 3:00 p.m.	**Break**		

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DURATION	**TOPIC**	**SPEAKERS**	**GOALS**
3:00 – 3:30 p.m. 30 mins	**Pilot & Feasibility Testing**	Miguel Vazquez	• Identify why it's important to do a pilot study to maximize acceptability, maintain affordability, and consider scalability of the ePCT intervention • Learn key approaches to evaluating the capabilities of the partner health system and testing key elements of the intervention
3:30 – 3:45 p.m. 15 mins	**Ethical & Regulatory Oversight Considerations**	Kevin Weinfurt	• Learn about the regulatory and ethical challenges of conducting ePCTs
3:45 – 4:00 p.m. 15 mins	**Writing a Compelling Grant Application**	Michael Ho	• Identify elements of a compelling ePCT application • Tips on NIH matchmaking
4:00 -4:45 p.m. 45 mins	**ePCTs in Context: Panel Discussion**	**Moderator** Kevin Weinfurt **Panel** Michael Ho Miguel Vazquez Stacy Sterling	• Presentation of case studies from three Collaboratory Demonstration Projects: Nudge, ICD-Pieces and GGC4H **Includes moderated Q&A with attendees
4:45 – 5:00 p.m. 15 mins	**Next Steps**	Kevin Weinfurt	• Final Q&A • Wrap up, including identifying sources for further learning.

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Speaker Biographies

Devon K. Check, PhD
Duke University School of Medicine
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Devon Check, PhD is a health services and implementation researcher. She is an Assistant Professor in the Department of Population Health Sciences at Duke and a member of the Duke Cancer Institute. Her primary research interests are quality of care and implementation of evidence-based practices in oncology. Dr. Check's work combines quantitative and qualitative methods to understand and address barriers to the delivery of high-quality, equitable care during and after cancer treatment. She is a Co-Investigator for the NIH Health Care Systems Research Collaboratory Coordinating Center and leads the implementation science resource efforts for Collaboratory demonstration projects.

Patrick Heagerty, PhD
University of Washington
heagerty@uw.edu

Dr. Patrick Heagerty is Professor and former Chair of the Department of Biostatistics at the University of Washington. He received a PhD from the Johns Hopkins University, and a BS from Cornell University. He has extensive experience as an educator, independent and collaborative scientist, and administrator. He has developed fundamental methods for longitudinal studies with a focus on prognostic model evaluation and structural longitudinal models, and he has detailed rigorous methods for the design, analysis, and interpretation of cluster-randomized trials conducted within health care delivery systems. Dr. Heagerty has co-authored two leading texts (Analysis of Longitudinal Data, Oxford 2002; Biostatistics: A Methodology for the Health Sciences, Wiley 2004). He is an elected Fellow of the American Statistical Association and has twice been honored by professional societies for specific research contributions (in 2000 as the Snedecor Award winner; and in 2005 by the International Biometrics Society for the best paper published in the society's flagship journal, Biometrics). Dr. Heagerty directs the Center for Biomedical Statistics (CBS), a core partially funded by the NIH Clinical and Translational Science Award (CTSA) with responsibility for coordination of biostatistical collaboration in Seattle and the greater Northwest region (Wyoming, Alaska, Idaho, Montana). The CBS

houses the data coordinating centers for several U01 and R01 funded projects including GARNET (Genomics and Randomized Trials), BOLD (Backpain Outcomes using Longitudinal Data), UH3 funded pragmatic trials including LIRE (Lumbar Imaging Reporting with Epidemiology), and PCORI funded trials evaluating surgical interventions and psychiatric treatment strategies. The CBS has previously conducted high-impact multi-site randomized trials including INVEST (Investigational Vertebroplasty Safety and Efficacy Trial, NEJM 2009), the Carpal Tunnel Surgical Trial (Lancet 2009), and LESS (Lumbar Epidural Steroid Injections for Spinal Stenosis, NEJM 2014). Dr. Heagerty is the Director of the Biostatistics and Research Design Core for the NIH Health Care Systems Research Collaboratory, for the NIH Mental Health Research Network, and a member of the Executive Committee for the FDA Sentinel Innovation Center. Dr. Heagerty is also a licensed teacher (NY State: Mathematics, Biology, and Chemistry) and has taught from middle school to graduate school (UW SPH Outstanding Teacher Award, 2009).

Michael Ho, MD
University of Colorado School of Medicine
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Dr. Ho is a Staff Cardiologist at the VA Eastern Colorado Health Care System and Professor at University of Colorado School of Medicine**.** He is also the Co-Director of the Data Science to Patient Value Program and Vice Chair of Quality for the Department of Medicine. His research over the past 15 years has focused on understanding the quality and outcomes of cardiovascular care, including the prevalence of medication non-adherence in cardiovascular diseases, the adverse consequences of medication non-adherence, and testing different interventions to improve medication adherence.

Stacy Sterling, DrPH, MSW, MPH,
Kaiser Permanente Division of Research
Stacy.A.Sterling@kp.org

Stacy Sterling, DrPH, MSW, MPH, is with the Drug and Alcohol Research Team (DART) and the Behavioral Health Research Initiative. She received her doctoral training at the University of North Carolina Gillings School of Global Public Health, and her Master's degrees in Public Health and Social Welfare at the University of California, Berkeley. Her research interests include developing systems for implementing evidence-based, integrated, behavioral health services into primary care, adolescent behavioral health prevention and early intervention, and alcohol and drug and mental health treatment outcomes and access. She is the Principal Investigator of a study funded by the Conrad N. Hilton Foundation to develop predictive models for adolescent substance use problem development; the Kaiser Permanente Principal Investigator on a trial funded by the Hilton Foundation of single vs. multisession screening, brief intervention and referral to treatment (SBIRT) for adolescents and parents in pediatric primary care; the Kaiser Permanente Principal Investigator of an National Institutes of Health National Institute of Alcohol Abuse and Addiction adolescent SBIRT trial in pediatric primary care and of an NIH/NIAAA survey of pediatrician attitudes toward and practices of adolescent behavioral-health risk screening and intervention; and of studies funded by the Robert Wood Johnson Foundation and Center for Substance Abuse Treatment of adolescents in drug and alcohol treatment in Kaiser Permanente. She has overseen the implementation of region-wide alcohol SBIRT in Kaiser Permanente Northern California adult primary care.

Miguel Vazquez, MD
UT Southwestern Medical Center
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Miguel A. Vazquez, M.D., is Professor of Internal Medicine at UT Southwestern Medical Center in Dallas and the Clinical Chief of the Nephrology Division at UT Southwestern and Nephrology Chief of Service at Parkland Hospital in Dallas. His patient care specialties include chronic kidney disease, end stage kidney disease and kidney transplantation. He attended medical school at the University of Puerto Rico in San Juan, and moved to UT Southwestern for his internship and residency in internal medicine. He also completed his fellowship in nephrology and research in immunology and transplantation at UT Southwestern.

Dr. Vazquez is active in patient-oriented research. His current research efforts are focused on improving care for patients with chronic kidney disease and coexistent diabetes and hypertension as part of the pragmatic clinical trial ICD-Pieces. His research efforts also include the Kidney Precision Medicine Project and studies related to dialysis vascular access. Dr. Vazquez is board certified in internal medicine and nephrology by the American Board of Internal Medicine. He is a Fellow of the American College of Physicians and was named a Fellow by the American Society of Nephrology in 2011.

Kevin Weinfurt, PhD
Duke University School of Medicine
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Dr. Weinfurt is Professor and Vice Chair for Research in the Department of Population Health Sciences in the Duke University School of Medicine. Dr. Weinfurt is also a Professor in the Duke departments of Psychiatry and Behavioral Science, Biostatistics and Bioinformatics, and Psychology and Neuroscience. He is a faculty member of the Duke Clinical Research Institute and Faculty Associate of the Trent Center for the Study of Medical Humanities and Bioethics. Dr. Weinfurt conducts research on measuring patient-reported outcomes, medical decision making, and bioethics.

Dr. Weinfurt was a principal investigator in the NIH PROMIS Network, where he led the development of the SexFS to measure male and female sexual function and satisfaction. Currently, he is co-chair of the coordinating center for the NIH Health Systems Research Collaboratory and served as the former President of the PROMIS Health Organization. As an educator, Dr. Weinfurt co-directs Duke's masters-level Clinical Research Training Program and has taught graduate courses in patient-reported outcomes research and multivariate statistics along with undergraduate courses in introductory psychology, judgment and decision making, and the psychology of medical decision making.

Dr. Weinfurt received his PhD in psychology at Georgetown University and did graduate work in the history of science and philosophy of mind at Linacre College, Oxford.