Embedded Pragmatic Clinical Trials: Accelerating Evidence Generation in Nephrology

American Society of Nephrology Kidney Week, November 8, 2019
Moderators: Catherine M. Meyers, M.D., FAHA; NCCIH
Susan R. Mendley, M.D.; NIDDK
Contemporary Challenges for Clinical Research

- New approaches & strategies for the clinical trial enterprise
  - Stakeholder interactions & leveraging multidisciplinary expertise
  - Methods for preserving randomized trial design & optimizing use of available data
  - Dissemination and Implementation

Lauer MS, Collins FS. JAMA 2010;303:2182-3
Clinical trials get practical
Many clinical trials don’t help doctors make decisions.
A new breed of studies aims to change that

By Jennifer Couzin-Frankel, in Philadelphia, Pennsylvania

• Need for trials that inform practice & policies
• Decision makers include patients, clinicians, payers, & policy makers
NIH Health Care Systems Research Collaboratory

**Goal:** To strengthen the national capacity to implement cost-effective, large-scale research studies that engage health care delivery organizations as research partners.

**Aim:** To provide a framework of implementation methods and best practices that will enable the participation of many health care systems in clinical research. Research conducted in partnership with health care systems is essential to strengthen the relevance of research results to health practice.
NIH Collaboratory PCT

- 19 Pragmatic clinical trials
- Diverse clinical settings, across 12 NIH Institutes
- >850 clinical sites, >85% of the US, >20 Health Care Systems, >800,000 participants
Pragmatic Clinical Trial Programs
NIH Collaboratory—ePCTs in Nephrology

- Time to Reduce Mortality in ESRD (TiME) trial
  - Multicenter, cluster-randomized, parallel group trial of HD session ≥4.25 hr in incident patients compared to usual care
  - Develop approaches to embed a large, randomized trial into routine HD care
  - Endpoints—mortality, hospitalization, lab measures of safety
NIH Collaboratory—
ePCTs in Nephrology

- Improving Chronic Disease Management with Pieces (ICD-Pieces)
  - Stratified, cluster randomized study of patients with CKD, DM, HTN to test if a collaborative model of primary care-subspecialty care using novel information technology and practice facilitators can implement best practices and reduce hospitalizations

- Management of Phosphorus in ESRD (HiLo)
  - Cluster randomized trial of HD patients to test a target serum phosphate >6.5 mg/dl vs standard 5.5 mg/dl using a hierarchical composite outcome of mortality and hospitalization
Panel Members

Laura M. Dember, MD
Professor of Medicine, Perelman School of Medicine, University of Pennsylvania

Qilu Yu, PhD
Lead Biostatistician, NIH, National Center for Complementary and Integrative Health, Office of Clinical and Regulatory Affairs

Miquel Vazquez, MD
Professor of Medicine, University of Texas Southwestern Medical Center

Wynne E. Norton, PhD
NIH/NCI, Office of the Director, Division of Cancer Control and Population Sciences
Rethinkingclinicaltrials.org