NIH Collaboratory

Health Care Systems Research Collaboratory

Rethinking Clinical Trials®

Pilot and Feasibility Testing

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Essentials of Embedded Pragmatic Clinical Trials Seminar

Learning goal

Identify approaches to evaluate the capabilities and challenges of the partner healthcare system and test key elements of the intervention during pilot or feasibility studies

Important things to know

- Pilot testing the ePCT methods increases likelihood of completing the trial and can prevent silly mistakes
- You need a biostatistician in the pilot/feasibility stage
- “Process issues” can derail the ePCT
- Use the pilot study to maximize acceptability, maintain affordability, and consider scalability of your intervention

ePCTs are not efficacy trials

- ePCTs bridge research into clinical care
- Intervention is integrated into real-world healthcare settings

During the pilot phase

- Establish close partnerships with healthcare system (HCS) personnel
- Test and validate EHR data collection and extraction
- Assess how well the intervention can be integrated into the clinical workflow
- Identify multiple local champions at each study site

Build partnerships

- Is the intervention aligned with the priorities of the partner HCS?
- How ready is the partner?
  - Are extra resources needed to support the intervention, identify participants, and extract necessary data?
  - How many sites are available to fully participate?
  - How much provider training will be needed, and can training use existing HCS infrastructure?
- If the intervention proves successful, what adaptations would be needed to implement it in other healthcare settings?

Resource: Health system partnerships

Establishing Close Partnerships with Healthcare System Leaders and Staff

From the *Living Textbook of Pragmatic Clinical Trials*
www.rethinkingclinicaltrials.org

Aspects of feasibility that can be piloted

Verify that target population can be identified via the EHR	Test phenotypes needed for sample identification	Validate data collection & extraction methods
Test data sample for quality & accuracy	Coordinate processes with local champions	Test the training materials for frontline providers & staff
Test appropriateness & usability of study toolkits or other materials	Evaluate informed consent materials	Evaluate whether fidelity/adherence measures can be achieved to justify the full-scale ePCT

Use what you learn to design the ePCT

Evaluate power calculations

If cluster randomization is involved, collect data to confirm estimate of intraclass correlation (ICC) for power calculations

Quantify feasibility for pilot study aims

- Eligibility
- Recruitment
- Randomization
- Adverse events
- Retention
- Missing data
- Intervention fidelity

Keep in mind realistic targets for the study's patient population

Quantifying example 1

Demonstrate effective recruitment and retention, which we define as the ability to recruit an average of 10 patients per month per site and retain 80% of participants for final data collection at 6 months

Quantifying example 2

Determine whether the intervention can be delivered with reasonable feasibility, which we define as 70% of the enrolled participants engage in the intervention

Determine whether the smoking cessation intervention can be delivered with reasonable feasibility, which we define as 20% of the approached participants engage in the intervention

Quantifying example 3

Demonstrate ability to collect primary outcomes and minimize missing data to less than 5% of primary outcome measures

Demonstrate ability to collect primary outcome of depression symptoms (patient reported) and minimize missing data to less than 10% of primary outcome measures

Resource: Pilot and feasibility testing

Assessing Feasibility: Pilot Testing

and

Feasibility Assessment Scenarios from the Collaboratory's Demonstration Projects

From the *Living Textbook of Pragmatic Clinical Trials*
www.rethinkingclinicaltrials.org

Case study from NIH Collaboratory: Suicide Prevention Outreach Trial (SPOT)

SUICIDE PREVENTION OUTREACH TRIAL

- Collaborative care model to test treatments intended to reach large groups of adult patients who have serious thoughts of suicide
- 4 clinical sites
- 16,000 expected patients
- Gregory Simon, MD, MPH, Principal Investigator, Kaiser Permanente Washington Health Research Institute

Suicide Prevention Outreach Trial

- Pragmatic trial of outreach programs to prevent suicide attempt
- Automatically enroll outpatients reporting frequent thoughts of death or self-harm on routine depression questionnaires
- Randomly assigned to continued usual care or one of two outreach programs
  - Risk Assessment and Care Management
  - Dialectical Behavior Skills Training
    - Both provided outreach for up to one year
    - Both intended as supplements to existing treatment
- Analysis by intent to treat, regardless of intervention uptake or adherence

A priori limits on interventions

- Total cost no more than $100 per person
- Centralized delivery by online messaging (via EHR portal)
- Delivered by masters-prepared mental health providers
- Scalable to full health system population

Pilot study process

- Three waves of pilot testing – approx. 40 in each wave
- Full implementation of invitation process
- Care management / coaching limited to 3 months
- No ascertainment of outcomes

Pilot study questions

- Expected rate of initial engagement
- Incremental gain with additional waves of invitation
- Optimal wording of invitation messages
- Proportion requiring telephone follow-up

What they learned / changed:

- Gain from 3rd wave of invitation is worth the effort
- Initial language describing the program was confusing
- Approximately 30% of invites require telephone follow-up
- Uptake rate tops out at 40%-45%

What they didn't do:

- Attempt to assess intervention impact or effectiveness
- Select participants for higher likelihood of participation
- Offer telephone services as alternative to outpatient care
- Extend beyond 3 cycles of invitation
- Personalize program to preferences or concerns of providers or clinics

In the end, it's about

- Avoiding silly mistakes
- Maximizing acceptability
- Maintaining affordability
- Remembering scalability

Resource: More feasibility examples

Spotlight on Four Demonstration Projects

From the *Living Textbook of Pragmatic Clinical Trials*
www.rethinkingclinicaltrials.org

Ensuring trial readiness

- Troubleshooting and iterative testing
- Flexibility to accommodate local conditions and changes over time
- Continuous engagement with healthcare system
- Readiness tasks
  - Recruitment plans are finalized
  - Ethical/regulatory aspects are addressed
  - Intervention is fully developed and finalized
  - Data collection methods are adequately tested
  - Budget and timeline are realistic and feasible

Readiness checklist

Milestone	Completed
Recruitment plans are finalized	
All sites identified (documentation of site commitment)	
Methods for accurately identifying participants validated	
All agreements for necessary subcontracts in place	
Ethical/regulatory aspects are addressed	
Coordinated IRB oversight in place	
Finalized plans for informed consent or waiver of informed consent	
Finalized data and safety monitoring plan	
Intervention is fully developed and finalized	
Finalized intervention (including materials and training at sites) ready for site implementation	
Finalized protocol is IRB approved (informed consent and data collection forms, if applicable)	
Data collection methods are adequately tested	
Validated methods for the electronic health record information	
Validated study surveys, interviews, or other data collection modes	
Demonstrated quality assurance and harmonization of data elements across healthcare systems/sites	
Statistical and data analysis methods have been adequately developed	
Budget is realistic, feasible, and accounts for potential changes	

Resource: Trial readiness criteria

Implementation Readiness Checklist

From the *Living Textbook of Pragmatic Clinical Trials*
www.rethinkingclinicaltrials.org

Important things to do

- Conduct a pilot or feasibility study of the intervention to inform the final design of the ePCT
- Work with a great biostatistician and an informatician (if needed)
- Develop a partnership approach to working with your healthcare system
- Identify multiple local champions for all your sites
- Anticipate, identify, and make a plan to address changes in the healthcare system