



CAIRIBU URO-AGING RESEARCH INTEREST GROUP (UroAging RIG)

CAIRIBU URO-AGING KNOWLEDGE EXCHANGES

URO-AGING RESEARCH INTEREST GROUP

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3/23/26 [3-4PM ET]

(2PM CT | 12PM PT)



EXECUTIVE SUMMARY 3/23/2026

Goal: Facilitate knowledge exchange and spur collaborations in uro-aging research and adjacent fields

Summary

From Pilot Studies to Multicenter Trials: Designing and Conducting Patient-Centered Interventional Research on Lower Urinary Tract Syndromes in Older Women

Introduction to Pilot Studies

- **Dr. Huang** described pilot studies as small-scale tests for future larger scale primary data collection study with the goal of evaluating or confirming the feasibility of approaches to be used in a larger future scale study.
- Reasons for carrying out a pilot study include testing out logistics, creating partnerships/infrastructure for future work, gathering evidence you can pull off logistics of larger scale study for funders, and gathering evidence to support the hypothesis of a larger-scale study.
- A pilot study is not meant to (1) evaluate efficacy of an intervention that is just too small and underpowered to test the efficacy hypothesis, (2) determine effect size needed to power a larger scale trial - should be a clinically meaningful difference or change - not just what is seen in initial pilot study, (3) transition or blend into a larger efficacy, or (4) run an effectiveness trial without a prespecified plan.
- **The greatest value from pilot studies is that the process forces you to identify and contend with challenges you never even realized existed before** - "you don't know what you don't know" – pilot studies shed light on unanticipated challenges, considerations, and issues.

Goals of Pilot Studies

- Appropriate pilot study goals include:
 - Demonstrating feasibility of recruitment, intervention administration, participant retention, outcomes data collection
 - Testing and improving logistics by finding quicker, easier, more cost-effective ways to administer procedures
 - Estimating costs
- Key parameters to evaluate and help you decide whether to do the larger trial include:
 - Rates of outcome events in each comparison or intervention group
 - Variability of data derived from outcome measures
 - Range of effect size relative to clinically meaningful benchmark

What is Learned from Conducting Pilot Studies

- Dr. Huang referred to a pelvic yoga study she conducted as an example.
 - The research began as a single site pilot study with 19 participants randomized to a 6-week yoga program vs. waitlist control.
 - Then she conducted a multisite pilot trial of 56 participants randomized to a 3-month yoga program vs. a time-equivalent physical conditioning program.
 - Finally, she conducted a multisite efficacy trial of 240 participants randomized to 3 month yoga versus physical conditioning with 9 month follow-up.
- **Feasibility of recruitment and randomization:** She learned about which recruitment methods yielded the most participants, the proportion of initial screens who were found eligible, the number of subjects enrolled per site per month, demographic differences by recruitment methods, and the time it took to recruit. These metrics help determine what proportion of those recruited would really be willing to randomized to an intervention.

- **Feasibility of interventions and follow-up:**

- Intervention feasibility: Realistically, can your population of interest perform the intervention? How often do participants report potential injuries or side effects?
- Adherence to interventions: What proportion of participants follow the intervention 50%, 75%, 100% of the time?
- Feasibility of follow up: Assess drop-out rates and proportion who fully complete all outcomes assessment visits

- **Feasibility of assessing outcomes:** missing data rates, timeliness of data, data quality, range of data values

- **Other logistical and cost questions:** Staff/effort required, flow of screening/follow up visits, time needed for each study visit or assessment, method of measure completion (online/paper/other), transportation to study visits

What to Include in Pilot Studies

- Randomization?

- Cons: Larger, slower, and more \$ + less data on an intervention if not all participants receive it
- Pros: Helps figure out if randomization is even possible, opportunity to learn about the control group (adherence, placebo rate, etc), allows for collection of preliminary data on potential between-group effects

- Control group?

- Generally not needed to assess feasibility, logistics, and cost
- Helpful to have for evaluating effect and sample size estimates, especially if needing to control for placebo effect
- Phase I pilot trial (evaluating short-term safety) are generally uncontrolled
- Phase II pilot trial (evaluating range of doses) are randomized and controlled

Statistical Analysis

- It is useful to have descriptive statistics - recruitment, randomization (as applicable), retention, adherence
- In larger pilot studies, can estimate within-group change, variance of outcome measures, or other key variables
- In larger pilot trials, can estimate correlation of outcomes before/after interventions
- Also, developing confidence intervals (CI) can reflect the uncertainty of estimates of parameters
- Researchers should have a reason for sample size - don't use pilot trial status, funding amount, or "other people did it so we can do it too" as an excuse.
- **Use the Rule of 12 to achieve sufficiently precise estimates of mean and variance of continuous outcomes**
- **Use the Rule of 10 – 10 participants with the outcome for each predictor – for binary outcomes**
- Specify your criteria for assessing feasibility of proceeding with main trial: recruitment, retention, adherence, follow up completion
- For pilot studies meant to formally evaluate feasibility based on specific parameters, use a CI approach
- Sometimes may need almost as large of a sample (as for the larger study) to test hypotheses-based feasibility parameter
- Acknowledge there will be multiple kinds of issues
- Must have a justification for why the sample is valuable

Publication and Reporting

- Be aware of appropriate journals, and know your audience
- **Follow 2010 extended CONSORT guidelines for reporting**

Funding

- Sources of funding for pilot trials include:
 - Intramural funds: Discretionary PI funds or institution-based pilot funding programs
 - NIH funding K, admin supplement funding, R21, R34, small R01 grants for pilot trials
 - Other extramural funders: non-profit foundations or pharmaceutical company grants

Takeaways

- If you're preparing to embark on your first pilot study: **consider partnering with someone who has experience designing, implementing, and publishing trials**, and remember there are existing standards for protocol, consensus guidelines, and reporting trial examples to guide you
- Trials are the gold standard of research and have led to major practice-changing results; pilot trials should be designed and executed after sufficient training to ensure strong design, methods, and interpretation of results
- Pilot and feasibility studies are critical for developing and testing new study procedures (and securing future funding)
- Pilot trials will be smaller and under-powered to test hypotheses, especially about efficacy
- Pilot trials will have different goals compared to the future full-scale study