

“Handbook for Clinical Research: Design, Statistics, and Implementation”

Flora Hammond, James Malec, Todd G. Nick, and Ralph M. Buschbacher, editors, 2015, 332 pages, Demos Medical, \$90.00

Review by Norman M. Goldfarb

“Handbook for Clinical Research: Design, Statistics, and Implementation” covers a broad range of topics for clinical researchers, especially those in academic environments. The bullet-point format offers clear, concise and practical steps for accomplishing a long list of things. Since the average chapter is less than four pages long, the level of detail within each step can be limited.

This book has been selected for
[The First Clinical Research Bookshelf](#)
Essential reading for clinical research professionals

The book consists of 83 chapters in three parts — Design, Statistics and Implementation — by 51 contributors. Representative chapters include the following:

- Single-Case Experimental Designs
- Observational Studies: Retrospective Versus Prospective
- Choice of Control Groups in Treatment
- Scoping Study
- Samples and Populations
- Data Cleaning
- Comparing Independent Samples With Continuous-Type Outcomes: Two Groups
- Sources of Error: Selection, Information Bias, and Confounding
- Successful Grant Applications
- Sources of Research Funding
- Developing the Idea With Stakeholder Input
- Preliminary Studies and Experience
- Letters of Support
- Treatment Manuals
- Participant Retention
- Data Dictionary
- Plan of Operation
- Protocol Deviations and Violations
- Data and Safety Monitoring

The following is one of the chapters that covers important topics not found in other books:

Developing the Idea with Stakeholder Input

John D. Corrigan, PhD

DEFINITIONS AND DESCRIPTIONS

- Participatory action research (PAR): Definitions of PAR tend to include (a) meaningful consumer involvement in all phases of the research process, (b) power sharing between researchers and consumers, (c) mutual respect for the

different provinces of knowledge among team members, (d) bidirectional education of researchers and consumers, and (e) conversion of results of research into new policy, programmatic or social initiatives (see White et al. 2004. Supplement page 3).

- Patient-centered outcomes research (PCOR): PCOR aims to help people make informed health care decisions and allows their voice to be heard in assessing the value of health care options. PCOR:
 - Assesses the benefits and harms of preventive, diagnostic, therapeutic or health delivery system interventions to inform decision-making — highlighting comparisons and outcomes that matter to people.
 - Is inclusive of an individual's preferences, autonomy and needs, focusing on outcomes that people notice and care about, such as survival, function, symptoms and health-related quality of life.
 - Incorporates a wide variety of settings and diversity of participants to address individual differences and barriers to implementation and dissemination.
 - May investigate optimizing outcomes while addressing burden to individuals, availability of services, technology, personnel and other stakeholder perspectives (see Suggested Readings and Resources).

INTRODUCTION

- The best research ideas are innovative, important and amenable to sound scientific investigation.
 - Not all ideas can be at the extremes of these criteria, and overemphasizing any one criterion can jeopardize the others.
 - For instance, an idea that stretches to be important may not be scientifically sound.
 - Conversely, defining a study by what data your methodology will provide may not allow a meaningful question to be asked (the proverbial "methodological tail wags the theoretical dog").
 - Good ideas are better than average on all three criteria and outstanding on at least one. The best research ideas get even better when vetted by knowledgeable audiences.
- Review by methodological experts can improve the science.
- Review by good scientific writers can improve the efficacy with which ideas are communicated.
- Review by stakeholders — the end users who are targeted for impact — will improve the relevance and affirm the importance of the study.
- The remainder of this chapter focuses specifically on the use of stakeholders to develop your research ideas.

IMPLICATIONS

- PAR and PCOR are two frameworks for engaging end users.
 - Both emphasize the research participant be treated as an essential end user who must be engaged in order to ensure that your research has an impact on those for whom it is intended.
 - While these approaches have both ethical and humanistic benefits, there is also the practical outcome that your research idea and subsequent research will be improved by the involvement of the stakeholders who will use the results.

BACKGROUND

- National Institutes of Health (NIH) grant funding is based on a proposal's overall impact as rated by peer reviewers.
 - Overall impact is "the project's likelihood to have a sustained, powerful influence on the research field(s) involved."
 - Impact is a function of importance (reflected in the significance and innovation criteria) and likelihood of achieving the ends proposed (reflected in the approach, investigators and environment criteria).
 - Significance — Does this study address an important problem? If the aims of the application are achieved, how will scientific knowledge or clinical practice be advanced? What will be the effect of these studies on the concepts, methods, technologies, treatments, services or preventive interventions that drive this field?
 - Innovation — Is the project original and innovative? For example: Does the project challenge existing paradigms or clinical practice; address an innovative hypothesis or critical barrier to progress in the field? Does the project develop or employ novel concepts, approaches, methodologies, tools or technologies for this area?
- The significance, if not innovation, of a research idea will be enhanced by engaging end users in both problem identification and idea refinement.

STRATEGIES

Standing Groups of End Users

- An advisory group can be a permanent fixture as part of a program's research enterprise.
- Members are recruited for their role relationship to the target of your research.
- Usually requires regular meetings and may need standard operating procedures to establish continuity and plan for departure of individual members over time.
- May be the most expensive option, depending on how often the group meets and from how far they travel.
 - New methods for Internet-based meetings may reduce costs but require a minimum of sophistication by all members.
 - Face-to-face meetings, at least initially or periodically, may be necessary for the group to bond to their purpose and each other.
- As an example, at Ohio State University, the Ohio Valley Center Advisory Council has met at least semiannually for 20 years to advise us on brain injury research and program development.
 - Members come from a five-state region and represent primary and secondary consumers, advocates, clinicians and researchers.
 - The Advisory Council is engaged, consistent with the principles of PAR.
 - One valuable contribution is that the Council engages in an every 4- to 5-year process of "envisioning" the future needs and directions of the field of brain injury rehabilitation. While the needs identified are more than what we can address in our research portfolio, the process provides a context for the areas of emphasis we maintain, add or jettison.
- PCORI promotes the development of Patient-Powered Research Networks (PPRNs). PPRNs can:
 - Take many forms
 - At a minimum, include a group of individuals with the condition and caregivers of individuals with the condition who seek others with the condition of interest to participate in improving care for similar individuals.

- Include other relevant stakeholders, such as clinicians, administrators, policy makers, or others involved in health care decision-making.
- Size can vary.
- Members should be:
 - Enthusiastic about participating in patient-generated health care research
 - Willing to take part in research studies, share data, create and adhere to protocols, consider participating in appropriate randomized trials
- PCORI's patient-centered approach also requires that all relevant stakeholders are engaged in multiple aspects of the investigational process, including:
 - Formulating research questions
 - Defining essential characteristics of study participants, comparators and outcomes.
 - Identifying and selecting outcomes that the population of interest notices and cares about and that inform decision-making relevant to the research topic.

Ad Hoc Input

- End users can be brought together on a project-specific basis, whether to provide feedback in proposal development or to be engaged from beginning to end as is consistent with PAR or PCOR principles.
 - Focus group methodologies work well for feedback during proposal development, but can also be used as part of knowledge translation.
 - Clinical trials that require consent from people not able to give it (e.g., use of a drug during the first hours after an injury causing unconsciousness) have developed innovative techniques for eliciting community input on an ad hoc basis (e.g., Exception From Informed Consent [EFIC] in the ProTECT™ trial).
 - It may be more difficult to accomplish the spirit of PAR or PCOR using ad hoc groups, especially the desirability of having end users involved in all aspects of research design, conduct, interpretation and dissemination.

PITFALLS

- Inadequately budgeted resources needed to engage end users in the way expected by PAR or PCOR
- Giving superficial "lip service" to stakeholder input
- Rushed sessions intended to elicit input that will waste everyone's time

HELPFUL HINTS

- Allow adequate meeting time to exchange information and communicate ideas so they are understandable.
- Allow time to build a culture of sharing and comfort with the process.
- Make an effort to truly learn from the stakeholders.
- In the words of Stephen Covey, "seek to understand before seeking to be understood."
- Scientific method is just one way of knowing. While your research grant will eventually be judged for its scientific merit, when vetting an idea with consumers and clinicians, suspend scientific judgment in order to hear what is important to them.
- Trust the process.
- Trust the stakeholders.

SUGGESTED READINGS

Participatory Action Research (Ed.). William Foote Whyte. p. 248. Newbury Park. CA: Sage Publications; 1991.

White GW, Suchowierska M. Campbell M. Developing and systematically implementing participatory action research. Arch Phys Med Rehabil. 2004;85(4 Suppl 2):S3—S12.

RESOURCES

EFIC: Exception from Informed Consent for Emergency Research:
www.nett.umich.edu/nett/efic

Participatory Action Research Toolkit:
www.dur.ac.uk/resources/beacon/PARtoolkit.pdf

Patient-Centered Outcomes Research:
www.pcori.org/research-we-support/pcor/

Patient-Centered Outcome Research Methodology:
www.pcori.org/research-we-support/methodology

The book is available in bookstores.

Reviewer

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