

Building Strong Evidence in Challenging Contexts: Alternatives to Traditional Randomized Controlled Trials



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NOVEMBER 2017

OPRE REPORT #2017-112

INTRODUCTION

Randomized controlled trials (RCTs) have long been considered the “gold standard” for evaluating program impacts. Randomization minimizes selection-related differences between the treatment and control groups so that any differences in outcome can be attributed to the program itself. However, RCTs are not always appropriate or feasible. In some cases, an RCT design may not align with the program model, research question, or population of interest. In other cases, the necessary resources may not be available to effectively and efficiently carry out an RCT.

On September 22 and 23, 2016, OPRE brought together a diverse group of participants from federal agencies, research firms, foundations, and academia to discuss these issues. Participants explored alternatives to RCTs and their assumptions, trade-offs, benefits, and challenges. Specifically, the meeting addressed the following questions:

1. When is it appropriate to use alternatives to traditional RCTs?
2. What are some rigorous alternatives to traditional RCTs for assessing program impacts?
3. How have these alternatives been employed to strengthen the evidence base for social programs?
4. What are the next steps for the field?

In this brief, we summarize key themes that emerged from the meeting.

WHEN IS IT APPROPRIATE TO USE ALTERNATIVE APPROACHES?

Presenters highlighted several scenarios in which an RCT may not be an appropriate or feasible option. These included situations where a sample size is too small, resources are inadequate, or it may not be ethical to have a control group that does not receive the intervention. Presenters also emphasized the importance of aligning a study design with the research questions, noting that using an RCT in a

situation where it is not appropriate may undermine rigor.

Discussion centered on the importance of building a strong evidence base, particularly for populations or settings that are challenging to study. To this end, presenters highlighted the need to conduct rigorous implementation, process, and impact studies with both quantitative and qualitative methods. They also emphasized the need to build a strong evidence base in challenging settings and populations, underscoring that evidence is context-specific and a lack of evidence in a specific population or setting can exacerbate existing inequities.

Using an RCT in a situation where it is not appropriate may undermine rigor.

During a roundtable discussion, presenters emphasized the importance of a strong and reciprocal relationship between researchers and community partners. The presenters highlighted how they have leveraged their partnerships to develop best practices for conducting community-based research and to choose an appropriate study design for the questions that the community is interested in addressing. The discussion focused on the importance of these partnerships throughout the research cycle from IRB approval to the dissemination of findings.

WHAT ARE SOME RIGOROUS ALTERNATIVES FOR ASSESSING PROGRAM IMPACTS?

Presenters highlighted numerous alternatives to randomized control trials when these are not feasible or appropriate. The appropriate approach will depend on several factors, including the context and timing of service delivery, the population of study, and the counterfactual or possible comparison group for program participants. These considerations are critical to understanding the underlying assumptions and trade-offs that influence the validity of alternative approaches. Below we highlight some of

the design and statistical approaches that were discussed by the meeting presenters.

Alternative approaches:

- ❖ [Single Case Research Designs](#) (including sequential introduction and withdrawal, rapid iterative alternation, and time-lagged introduction)
- ❖ [Randomized Roll Out Designs](#) (including stepped wedge and dynamic wait-lists)
- ❖ [Interrupted Time Series Designs](#)
- ❖ [Double Randomized Preference Trials](#)
- ❖ [Kernel Matching/Optimization](#)
- ❖ [Regression Discontinuity](#)
- ❖ [Instrumental Variables](#)
- ❖ [Hierarchical Bayesian Analysis](#)
- ❖ [Value-added Modeling](#)

HOW HAVE THESE ALTERNATIVES BEEN EMPLOYED TO STRENGTHEN THE EVIDENCE BASE?

Presenters described three federally funded efforts to encourage the production of stronger research evidence. These efforts cast evidence as a body of work rather than the result of a single study. Established standards for several study designs ensure rigor and elevate the importance of relevance and feasibility.

- ❖ The U.S. Department of Education *What Works Clearinghouse* uses panels of national experts to set quality standards for a variety of study designs, including designs that do not involve random assignment. Certified reviewers then evaluate research studies for inclusion in the Clearinghouse's database of internally valid research on education interventions. The quality standards ensure that studies included in the Clearinghouse meet a high bar for rigor so that it can serve as a trusted source of research evidence to inform education policy and practice.
- ❖ The National Institutes of Health *Healthcare Systems Research Collaboratory* engages in pragmatic clinical trials, often using electronic health records and clustered randomization at the provider or clinic level. The goal is to encourage cost-effective, large-scale research studies using healthcare delivery organizations as research partners. Many studies do not use a traditional RCT design, and the Collaboratory seeks to work closely with demonstration projects to ensure that their

research designs and analyses are both well-suited to the context and methodologically rigorous.

- ❖ The Corporation for National and Community Service *Social Innovation Fund* (SIF) has supported the evaluation of promising community-based programming. SIF grantees work with local nonprofit organizations and federal staff to design appropriate and feasible program evaluations. The goals are to channel resources to programs that work; produce a body of research evidence to guide program design; build local evaluation capacity in the nonprofit sector; and broadly encourage evidence-based grant making.

WHAT ARE THE NEXT STEPS?

Participants presented on a growing list of study designs that allow researchers to answer policy-relevant questions and build rigorous causal evidence, even in challenging research contexts. These designs can take advantage of the roll-out and scale-up

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of interventions to identify what works, for whom, and under what circumstances. Many of the examples included in the meeting involve strong collaboration between researchers and local service providers, which also builds the capacity of practitioners as producers and consumers of research, increasing the relevance and applicability of research evidence.

While these methods offer promising opportunities to build a body of research evidence, presenters identified trade-offs between increasing technical complexity and the transparency of research findings. As alternatives to the traditional RCT grow and multiply, standards for identifying and ways of communicating about "rigorous" evidence also must evolve if research evidence is to inform policy and practice.

WANT TO LEARN MORE?

To access the online meeting archive, including a detailed schedule, meeting materials, and presentation slides, please visit the OPRE Innovative Methods Meeting website at www.opremethodsmeeting.org. The site also includes materials from other innovative methods meetings that OPRE has organized and will be updated to include future meetings.

MEETING AGENDA

Building Strong Evidence in Challenging Contexts: Alternatives to Traditional Randomized Controlled Trials

September 22 and 23, 2016 – Washington, DC

Day 1: Thursday, September 22

Welcome and Opening Remarks – Naomi Goldstein, Deputy Assistant Secretary for Planning, Research and Evaluation, Administration for Children and Families

Setting the Stage

[*Building strong evidence in challenging contexts*](#) – Nancy Whitesell, University of Colorado, Denver

Roundtable Discussion – Working with Communities to Design Research and Evaluations

Moderator – Aleta Meyer, Office of Planning, Research and Evaluation

Panelists:

Domestic violence example: Lisa Goodman (Boston College) and Deborah Heibel (REACH Beyond Domestic Violence, Inc.)

Tribal example: Nancy Whitesell (University of Colorado, Denver) and Alicia Mousseau (University of Colorado, Denver)

Diverse urban populations example: Bowen Chung (University of California, Los Angeles) and Aziza Lucas Wright (Charles R. Drew University of Medicine and Science)

Working with Small Samples

Moderator – Nicole Deterding, Office of Planning, Research and Evaluation

[*Single case research design in early childhood contexts*](#) – Jennifer Ledford, Vanderbilt University

[*Designing controlled trials with the power of optimization*](#) – Nathan Kallus, Cornell University

[*Bayesian analysis for small subgroups: Intuitive inferences with heightened precision*](#) – Mariel Finucane, Mathematica Policy Research

[*The challenges of developing a counterfactual for place-based initiatives: Promise Zones*](#) – Calvin Johnson, Department of Housing and Urban Development

Alternative Forms of Randomization

Moderator – Anna Solmeyer, Office of Planning, Research and Evaluation

[*Stepped wedge designs and the Washington state EPT trial*](#) – James Hughes, University of Washington

[*The extended family of randomized roll-out designs, including stepped wedge and dynamic wait listed designs*](#) – Hendricks Brown, Northwestern University

[*A hybrid double randomized client preference trial: Benefits and challenges*](#) – Gerald August, University of Minnesota

[*Leveraging lotteries for school value-added*](#) – Joshua Angrist, Massachusetts Institute of Technology

Day 2: Friday, September 23

When Randomization is Not Possible

Moderator – Anupa Bir, RTI International

[*Regression discontinuity and extensions*](#) – Coody Wing, Indiana University

[*Using simulated instruments for addressing descriptive and causal policy questions*](#) – Vivian Wong, University of Virginia

[*A short comparative interrupted time-series analysis of the impacts of Jobs-Plus*](#) – Howard Bloom, MDRC

[*Recent advances in non-experimental comparison group designs*](#) – Elizabeth Stuart, Johns Hopkins University

Federal Efforts and Future Directions

Moderator – Nicole Constance, Office of Planning, Research and Evaluation

[*The Social Innovation Fund: Utilizing a range of research designs*](#) – Lily Zandniapour, Corporation for National and Community Services

[*The NIH Collaboratory: Working with grantees and stakeholders to strengthen research*](#) – Elizabeth DeLong, Duke University

[*What Works: Clearinghouse standards for alternative designs*](#) – Jonathan Jacobson, Department of Education

[*Considerations for including non-experimental evidence in systematic reviews*](#) – John Deke, Mathematica Policy Research

This brief was prepared by the Office of Planning, Research and Evaluation, Administration for Children and Families, U.S. Department of Health and Human Services. It was developed under Contract Number HHSP23320095651WC. RTI International assisted with preparing and formatting the brief. The ACF project officer is Anna Solmeyer. The RTI project director is Anupa Bir.

This brief is in the public domain. Permission to reproduce is not necessary. Suggested citation: Malin, J. and Deterding, N. (2017). *Building Strong Evidence in Challenging Contexts: Alternatives to Traditional Randomized Control Trials*, OPRE Report #2017-112, Washington, DC: Office of Planning, Research and Evaluation, Administration for Children and Families, U.S. Department of Health and Human Services.

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