Hein, S., & Weeland, J. (2019). Introduction to the special issue. Randomized controlled trials (RCTs) in clinical and community settings: Challenges, alternatives, and supplementary designs. In S. Hein & J. Weeland (Eds.), *Randomized Controlled Trials (RCTs) in Clinical and Community Settings: Challenges, Alternatives and Supplementary Designs. New Directions for Child and Adolescent Development*, *167*, 7–15.

1

Introduction to the Special Issue. Randomized Controlled Trials (RCTs) in Clinical and Community Settings: Challenges, Alternatives, and Supplementary Designs

Sascha Hein, *Joyce Weeland*

Abstract

This article provides an overview of the contributions to the special issue on Randomized Controlled Trials (RCTs) in Clinical and Community Settings: Challenges, Alternatives, and Supplementary Designs. The article introduces the challenges of conducting RCTs in dynamic real-world settings and outlines the need to consider alternative and supplementary designs. © 2019 The Authors. New Directions for Child and Adolescent Development published by Wiley Periodicals, Inc.

Globally, there is an urgent need for evidence-based prevention and intervention programs aiming to promote the development of all children and adolescents. The United Nations General Assembly's (2015) 2030 agenda for sustainable development includes seventeen global goals (i.e., the Sustainable Development Goals, SDGs) for the future of human development among which are good health and well-being (SDG 3), quality education (SDG 4), and peace, justice, and strong institutions (SDG 16). Currently, countless programs worldwide aiming to contribute to

New Directions for Child and Adolescent Development, no. 167, September 2019 © 2019 The Authors. New Directions for Child and Adolescent Development published by Wiley Periodicals, Inc.
Published online in Wiley Online Library (wileyonlinelibrary.com). • DOI: 10.1002/cad.20312

This is an open access article under the terms of the Creative Commons Attribution-NonCommercial License, which permits use, distribution and reproduction in any medium, provided the original work is properly cited and is not used for commercial purposes.

these goals are being implemented. Although some of these programs have been put to the test with scientific scrutiny, there are still many programs about which we do not know whether and how they are effective. Moreover, there is a large group of underserved children and adolescents about which we generally know very little. For example, in 2018, the United Nations Children's Fund (UNICEF) concluded that "*half a billion* of the world's children live in 64 countries that lack sufficient data to even assess if they are on or off track for at least *two-thirds* of all child-related SDG indicators." (UNICEF, 2018, p. 7; italics in the original text). Also, how do we scale up the implementation of evidence-based interventions so that they also reach children in these countries? More research is thus needed on current prevention and intervention efforts, specifically for underserved children and adolescents in vulnerable and hard-to-reach settings.

Randomized controlled trials (RCTs) are considered the gold standard design for evaluating the effectiveness of prevention and intervention programs. Studies employing this design—in which individuals are randomly assigned to different intervention conditions, and in which participants in these conditions are compared to each other—are thought to allow causal claims about the effects of an intervention. Randomization minimizes the effect of confounding factors such as selection (differences between the groups that are confounded with treatment or no-treatment condition) and maturation (naturally occurring changes over time that are confounded with treatment effects). Although the strengths of RCTs are widely recognized, the privileged position of this design has also been critiqued (e.g., Goodman, Epstein, & Sullivan, 2018; Kasenda et al., 2014; Miller & Brody, 2003; Shean, 2014; Thomas, 2016). Specifically, concerns have been raised that RCTs may not only be seen as the gold standard but as the "only standard" (Kazdin, 2019, p. 16). Like any method, the RCT has its strengths and limitations (Miller & Brody, 2003). Also, conducting an RCT in real-world-settings can be challenging. In some populations, the RCT design might be difficult, untenable, or sometimes even inappropriate (Miller & Brody, 2003). Particularly in cases of hard-to-reach and vulnerable populations, such as refugee and marginalized communities (see Ponguta et al., 2019, this issue) or children and adolescents in residential care institutions (see Tavecchio et al., 2019, this issue), researchers are confronted with many practical, methodological, political, legal, and/or ethical challenges. The goal of this issue is to catalog such challenges of conducting an RCT and explore alternatives and supplementary study designs that address these challenges.

Aims of This Issue

We are pleased to introduce this issue on *challenges, alternatives, and supplementary research designs to RCTs* in studying prevention and intervention programs aimed at promoting the development of children and

adolescents. In line with *New Directions for Child and Adolescent Development*'s aims and scope, this issue unites a broad range of scholars and perspectives focusing on (a) challenges that arise before, during, or after studying intervention effectiveness; and (b) possible alternative and supplementary designs to RCTs. This issue explores what may constitute best practice for future studies in which conducting an RCT is either unfeasible, undesirable, and/or not fitting. Across different contributions, this issue explores the utility of alternative and complementary designs from a multi-disciplinary perspective, incorporating viewpoints from research methodology as well as developmental, counseling/clinical, and community psychology.

Challenges

A unifying theme across contributions to this special issue is the attention to challenges of conducting effectiveness studies to determine whether a prevention or intervention approach may work under real-world conditions and, typically, difficult implementation circumstances.

In many prevention and intervention settings, there may be practical and methodological obstacles for conducting an RCT. For example, when assessing the effectiveness of educational interventions and practices (e.g., grade retention; Kim, 2019, this issue), universal prevention, or community-, workplace-, and school-wide implemented programs, randomization may not be feasible (Sanson-Fisher, Bonevski, Green, & D'Este, 2007). Further, if randomization is feasible in such settings there may be a risk that the active ingredients of an intervention are not confined and trial participants who were not intended to receive the intervention inadvertently do so (contamination). When assessing clinical interventions targeting complex conditions with low prevalence, such as children with autism spectrum disorder and comorbid depressive symptoms and suicidal ideation (Wijnhoven et al., 2019), target populations may be very small, which makes it difficult to conduct a meaningful RCT with sufficient statistical power. When assessing interventions in dynamic and challenging real-world settings, such as in low- and middle-income countries that are comprised of vulnerable population or affected by man-made or natural disasters, it may be difficult to implement the intervention with good integrity, to standardize outcome assessment, or to map and properly control for other ongoing interventions targeting the same populations (Grolnick et al., 2018; Strouse & Moore, 2019, this issue).

These obstacles are important to consider as they complicate the translation of the theoretical foundations of RCTs to research practice. In this issue, Wadhwa and Cook (2019) discuss the ("hidden") assumptions underlying RCTs. For example, when designing a study, different assumptions underlie our choice for a specific control condition (e.g., care-as-usual,

wait-list, or no-intervention) or outcome measures and when interpreting the results we make (implicit) assumptions about sample size and composition and the setting in which we studied an intervention. The authors illustrate these assumptions with examples of research on child and adolescent development. If strict control of the design is not possible, this leads to important threats to, for example, the external or internal validity and the statistical conclusion validity of RCTs (Wadhwa & Cook, 2019, this issue). In cases where circumstances may pose such threats, other designs may be a justified alternative to an RCT.

Besides the practical and methodological challenges that make an RCT unfeasible in some cases, an RCT may also be undesirable or unapt. In some cases, there may be legal or ethical concerns about randomly allocating participants to different groups or withholding an intervention. For example, in juvenile justice or residential care settings randomization may come with legal and ethical challenges (Butts & Roman, 2018; Tavecchio et al., 2019, this issue). There may also be cases in which an RCT alone may not be suitable to answer the question at hand. For example, when interventions target very heterogeneous populations, complex conditions, or are highly personalized, RCTs may be unsuitable as group-level comparisons lead to loss of information on individual differences (Goodman et al., 2018). Moreover, RCT designs are very costly and might limit the opportunity and available resources for frequent, extensive, and/or multi-method assessment of mechanisms of change. In cases where interventions are aimed to decrease the negative effects of crises and disasters, there may simply be no time to await the results of an RCT (Grolnick et al., 2018; Strouse & Moore, 2019, this issue). The translation of RCT results to practice takes a lot of time and the controlled setting under which an RCT is conducted sometimes complicate translation to less controlled real-world settings (Green & Glasgow, 2006). Supplementing RCTs with other study designs may also increase our understanding of for whom, why, and under what circumstances interventions are effective and may lead to valuable insights on intervention effectiveness that cannot be obtained from an RCT alone.

Also, an RCT may be premature to assess the impact of newly developed programs given the many challenges of implementing a protocolled program (i.e., with standardized program components) in dynamic real-world settings. The assessment of new prevention and intervention programs could, thus, for example, be done in a step-wise procedure by starting with a process evaluation (see Ponguta et al., 2019, this issue), then building toward quasi-experimental and, finally, an RCT design. This step-wise approach provides the opportunity to evaluate the implementation procedure and program integrity, and to adjust and sharpen the implementation process if necessary. As such, using different designs, besides RCTs, may be "indispensable in the early stages of the research spiral" (van IJzendoorn, 2019, this issue).

Examples of Alternative Designs

Two alternative designs are discussed in this issue. First, Geuke and colleagues (2019, this issue) discuss the use of Single-Case Experimental Designs (SCEDs), that is, experimental designs involving one or more participants (or a single classroom, school, or city) who participate in (a) repeated assessment of the target outcome(s) over time, (b) including a baseline assessment to document how the outcome variable develops prior to intervening, (c) active manipulation of the outcome variable using an intervention, and (d) analysis of intervention outcomes at the level of the individual participant. Geuke and colleagues (2019, this issue) specifically discuss and illustrate possibilities of statistical mediation analyses in SCEDs to test intervention effects and mechanisms of changes in youth populations as an alternative to between-individual comparisons. These methods are applied in a SCED in which the effects of cognitive therapy (over and above exposure therapy) on anxiety via coping are examined for a 9-year-old boy with an anxiety disorder. The validity and advantages of SCED to assess intervention effectiveness with scientific rigor are increasingly acknowledged (Kazdin, 2019).

Second, Kim (2019, this issue) discusses the use of propensity score methods to achieve a balanced state across different experimental conditions (e.g., intervention versus control) as an alternative to randomization. In propensity score analyses, participants in the control and the intervention group can be matched on their known characteristics. The author illustrates propensity score matching using the Project Achieve data, a 14-year longitudinal study of students from three school districts in Texas to examine the effects of grade promotion and retention on post-secondary enrollment. Kim also provides a tutorial for propensity score matching using the open-source statistical program R. The validity and applicability of this approach in research on preventions and interventions targeting children and adolescents has been increasingly acknowledged (e.g., Dong & Lipsey, 2018).

Examples of Supplementary Designs

Two complementary designs are being discussed in this issue. First, Ponguta and colleagues (2019, this issue) describe methods to explore the context in which an RCT will be done and to map possible enablers and barriers of program implementation and impact evaluation. Using this knowledge, they conducted a randomized control trial with a wait-list control group to assess a parenting intervention with refugee and marginalized communities in Beirut, Lebanon. Whereas RCTs focus on the outcomes of an intervention, process evaluations explore how and in which settings the intervention is implemented and received, which may help us with the interpretation of findings on program outcomes. The study by Ponguta and colleagues (2019, this issue) suggests that despite multiple challenges, implementation

of intervention programs and evaluation of these programs using randomized trials in fragile contexts is feasible.

Second, Tavecchio and colleagues (2019, this issue) describe an approach that engaged intended users of the research outcomes as participants in the research process, namely participatory peer research (PPR). They illustrate this approach in a small sample ($N = 10$) of young adults with mild intellectual disabilities (MID) and severe behavioral problems. Compared to an RCT, PPR may provide clinical practice with faster answers to urgent questions, maximize the participation of clients and other important stakeholders, and may help us bridge the gap between science and practice. Designs like PPR may create chances for (more intensive) collaboration between research and practice, through active participation of practitioners as well as of children, adolescents, and parents. This may lead to immediately implementable knowledge of practices and interventions.

Commentaries and Conclusions

The contributions in this issue address challenges and different methods for conducting RCTs in real-world clinical and community settings, as is synthesized and discussed in two insightful commentaries. Strouse and Moore (2019, this issue) comment from the perspective of an international nongovernmental sponsorship organization. According to the authors, inherent to working in dynamic real-world settings is the urgency to act quickly and flexibly, in some cases without sufficient time to plan and execute an RCT. They reflect on how the different designs described in the issue may be used in future projects in such settings. Strouse and Moore's commentary implies that even in fragile settings, such as in hard-to-reach and underserved populations and acute emergencies, rigorous impact evaluations using an RCT may be feasible but may have to be proceeded by real-time, alternative, evaluation methods that can provide immediate feedback about the program implementation. In contrast to the emphasis on agile science and rapid assessments, van IJzendoorn's (2019, this issue) plea for "slow science" reminds us that scientific evidence does not stem from a single study but an accumulation of replications, secondary and meta-analyses, and umbrella reviews. The author also emphasizes the importance of reliability, internal validity, and replicability of our study designs and argues that "what we cannot study in a replicable way should not be studied at all (until a valid method has become available)". Currently, our methodological toolbox may simply not yet be complete. We need to develop additional tools, but the author also gives examples of cases in which we may also be able to supplement our toolbox by making use of advances in other fields.

The contributors to this issue of *New Directions for Child and Adolescent Development* share a commitment to broadening our knowledge about which prevention and intervention strategies work, to translating this knowledge to practice, and, ultimately, to promoting child and adolescent

development across the world and creating equal opportunities for all children and adolescents. As with RCT designs, the proposed alternative and complementary designs have advantages and limitations. Moreover, the strengths of any study design depend on their scientific rigor (Shaffer, Kronish, Falzon, Cheung, & Davidson, 2018). This scientific rigor, however, might not only depend on methodological rigor, integrity, and quality of the data, but also on how well the acquired data can answer meaningful questions about child and adolescent developmental outcomes (e.g., as targeted in an intervention). In designing future studies, we also need to consider what meaningful questions related to the effectiveness of a specific programmatic approach might be and what methods can best be used to answer them. In some cases, RCTs, but in other cases other designs, might are the best fit to answer the questions at hand. The purpose of this issue is thus not to proclaim the abandonment of RCT designs. Rather, we hope that this issue contributes to the understanding of the challenges of conducting an RCT, and knowledge about the alternatives and supplementary designs worthy of consideration, as different designs may be vital and unique pieces of the puzzle. Ultimately, studying prevention and intervention efforts aiming to optimize child and adolescent development from different angles, using a combination of different study designs, will lead to an evidence base that is not only data-rich but also information-rich, and eventually, to a better understanding of program effectiveness.

References

Butts, J. A., & Roman, J. K. (2018). Good questions: Building evaluation evidence in a competitive policy environment. *Justice Evaluation Journal*, *1*(1), 15–31.

Dong, N., & Lipsey, M. W. (2018). Can propensity score analysis approximate randomized experiments using pretest and demographic information in pre-K intervention research? *Evaluation Review*, *42*(1), 34–70.

Geuke, G. G. M., Maric, M., Miočević, M., Wolters, L. H., & de Haan, E. (2019). Testing mediators of youth intervention outcomes using single-case experimental designs. In S. Hein & J. Weeland (Eds.), *Randomized Controlled Trials (RCTs) in Clinical and Community Settings: Challenges, Alternatives and Supplementary Designs*. *New Directions for Child and Adolescent Development*, 167, 39–64.

Goodman, L. A., Epstein, D., & Sullivan, C. M. (2018). Beyond the RCT: Integrating rigor and relevance to evaluate the outcomes of domestic violence programs. *American Journal of Evaluation*, *39*(1), 58–70.

Green, L. W., & Glasgow, R. E. (2006). Evaluating the relevance, generalization, and applicability of research: issues in external validation and translation methodology. *Evaluation & The Health Professions*, *29*(1), 126–153.

Grolnick, W. S. S., David, J.; Schreiber, M., Cohen, J., Cole, V., Jaycox, L., … Zatzick, D. (2018). Improving adjustment and resilience in children following a disaster: Addressing research challenges. *American Psychologist*, *73*(3), 215–229.

Kasenda, B., von Elm, E., You, J., Blumle, A., Tomonaga, Y., Saccilotto, R., … Briel, M. (2014). Prevalence, characteristics, and publication of discontinued randomized trials. *JAMA*, *311*(10), 1045–1051. Retrieved from 10.1001/jama.2014.1361

Kazdin, A. E. (2019). Single-case experimental designs. Evaluating interventions in research and clinical practice. *Behaviour Research and Therapy*, *117*, 3–17.

Kim, H. (2019). Propensity score analysis in non-randomized experimental designs: An overview and a tutorial using R software. In S. Hein & J. Weeland (Eds.), *Randomized Controlled Trials (RCTs) in Clinical and Community Settings: Challenges, Alternatives and Supplementary Designs*. *New Directions for Child and Adolescent Development*, 167, 65–89.

Miller, F. G., & Brody, H. (2003). A critique of clinical equipoise: Therapeutic misconception in the ethics of clinical trials. *Hastings Center Report*, *33*(3), 19–28.

Ponguta, L. A., Issa, G., Aoudeh, L., Maalouf, C., Nourallah, S., Khoshnood, K., … Leckman, J. F. (2019). Implementation evaluation of the Mother-Child Education Program among refugee and other vulnerable communities in Lebanon. In S. Hein & J. Weeland (Eds.), *Randomized Controlled Trials (RCTs) in Clinical and Community Settings: Challenges, Alternatives and Supplementary Designs*. *New Directions for Child and Adolescent Development*, 167, 91–116.

Sanson-Fisher, R. W., Bonevski, B., Green, L. W., & D'Este, C. (2007). Limitations of the randomized controlled trial in evaluating population-based health interventions. *American Journal of Preventive Medicine* *33*(2), 155–161. Retrieved from 10.1016/j.amepre.2007.04.007

Shaffer, J. A., Kronish, I. M., Falzon, L., Cheung, Y. K., & Davidson, K. W. (2018). N-of-1 randomized intervention trials in health psychology: A systematic review and methodology critique. *Annals of Behavioral Medicine*, *52*, 731–742.

Shean, G. (2014). Limitations of randomized control designs in psychotherapy research. *Advances in Psychiatry*, *2014*, 1–5. Retrieved from 10.1155/2014/561452

Strouse, D. L., & Moore, K. (2019). Commentary: Contextualizing alternatives to RCTs: Measuring the impact of a non-governmental sponsorship organization's projects to strengthen children's supportive environments. In S. Hein & J. Weeland (Eds.), *Randomized Controlled Trials (RCTs) in Clinical and Community Settings: Challenges, Alternatives and Supplementary Designs*. *New Directions for Child and Adolescent Development*, 167, 141–158.

Tavecchio, L., Van der Helm, P., Moonen, X., Assink, M., Stams, G. J., Wissink, I., & Asscher, J. (2019). Participatory peer research in the treatment of young adults with mild intellectual disabilities and severe behavioral problems. In S. Hein & J. Weeland (Eds.), *Randomized Controlled Trials (RCTs) in Clinical and Community Settings: Challenges, Alternatives and Supplementary Designs*. *New Directions for Child and Adolescent Development*, 167, 117–131.

Thomas, G. (2016). After the gold rush: Questioning the "gold standard" and reappraising the status of experiment and randomized controlled trials in education. *Harvard Educational Review*, *86*(3), 390–411.

UNICEF. (2018). *Progress for every child in the SDG era*. New York: UNICEF.

United Nations General Assembly. (2015). *Transforming our world: The 2030 agenda for sustainable development (Resultion A/RES/70/1)*. New York: United Nations General Assembly.

van IJzendoorn, M. H. (2019). Commentary: Addressing the replication and translation crises taking one step forward, two steps back? A plea for slow experimental research instead of fast "participatory" studies. In S. Hein & J. Weeland (Eds.), *Randomized Controlled Trials (RCTs) in Clinical and Community Settings: Challenges, Alternatives and Supplementary Designs*. *New Directions for Child and Adolescent Development*, 167, 133–140.

Wadhwa, M., & Cook, T. D. (2019). The set of assumptions randomized control trials make and their implications for the role of such experiments in evidence-based child and adolescent development research. In S. Hein & J. Weeland (Eds.), *Randomized Controlled Trials (RCTs) in Clinical and Community Settings: Challenges, Alternatives*

and Supplementary Designs. New Directions for Child and Adolescent Development, 167, 17–37.

Wijnhoven, L. A., Niels-Kessels, H., Creemers, D. H., Vermulst, A. A., Otten, R., & Engels, R. C. (2019). Prevalence of comorbid depressive symptoms and suicidal ideation in children with autism spectrum disorder and elevated anxiety symptoms. *Journal of Child & Adolescent Mental Health*, *31*(1), 77–84.

SASCHA HEIN, PhD, is a Professor in the Department of Education and Psychology at the Free University of Berlin. His research focuses on the impact of formal and non-formal learning as well as parenting on child and adolescent development in diverse cultures. In his recent projects, he studies the development of children and families growing up in crisis-affected, humanitarian, and marginalized contexts.

JOYCE WEELAND, PhD, is assistant professor at the Erasmus School of Social and Behavioural Sciences, Rotterdam, The Netherlands. Her research focuses on environmental risk factors for child and adolescent externalizing behavior problems and interventions aiming to prevent or decrease this behavior. Joyce is currently involved in different research projects that assess the effectiveness of prevention and intervention programs.