Improving randomized controlled trial evidence in pediatric critical care: The perspectives of trialists

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Background

The care of all critically ill children should be informed by evidence from high-quality randomized controlled trials (RCTs). Unfortunately, such evidence is not always available. Only 320 RCTs have been published in this field, and they are typically small (median sample size of 50 children). They are also difficult to complete: 30% were stopped early, most commonly for futility or recruitment problems.¹

Objectives

To identify strategies that pediatric critical care trialists consider to be acceptable, feasible and effective ways to improve the evidence available from RCTs in the field.

Methods

Design: Qualitative study using semi-structured interviews.

Study population: Clinical researchers in pediatric critical care who have successfully completed and published at least one RCT (as first or last author).

Sampling: First we identified the authors of RCTs published 2015-2015 using PICUTrials.net.¹ Then we used purposive sampling to achieve diversity with respect to the characteristics of the researchers (country of origin, experience, and profession) and of their RCTs.

Data collection: Single investigator conducted semi-structured interviews with all participants. The interviews lasted 30-45 minutes and used a pre-tested interview guide. The audio recordings were transcribed verbatim.

Analysis: We used an inductive approach to data analysis, collecting and analyzing the data concurrently in an iterative process. We used a conventional content analysis approach to analyze the interview transcripts.² Two investigators independently coded 4 transcripts using open coding. From this, we developed a preliminary list of codes through consensus and used this to code the remaining transcripts. After coding we organized the codes into meaningful categories.

Participants

26 trialists from 7 countries participated. 20 (77%) were physicians and they reported a median (IQR) of 23 (20-28) years of clinical experience. 8 (31%) had completed more than 1 RCT and 17 (65%) had completed a multicentre RCT.

Participants’ country of origin

Major themes that emerged from the interviews

Building communities

“it’s the realization that it’s a social world. People have many things that they could be doing. For people to put energy into your trial, if it’s multi-centred, they need to feel it is of value. You need to reach people through your values.”

“The personal contact will always give you a bit more confidence, make you enthusiastic, feel if you’re on the same level, if you have the same goal.”

Getting started as an investigator

“We don’t do a great job with our medical trainees of integrating things – study design and statistics into medical training. We just tell them, ‘You have to come out with a project, but we don’t [train them].’ If it’s crazy if they said, you have to come out of a cardiology Fellowship knowing how to do an echocardiogram but they never taught you how to do it... so why is this any different?”

Getting started as an investigator

“I think getting the opportunity as being relatively junior, to be part of a trial... I learned a lot from that part, just being part of these studies even if you’re not the PI and you’re not the complete junior investigator but the median person... Because then it’s worth it to do all that training for the study. I think that really helped me get a lot of independence. Doing it, but on the same time, you’re not on your own.”

Working within the system

“If you talk to the people who participated [in the study], they can definitely say that the patient care improved along with nursing excitement about conducting a study that had direct applications for their practice. And so, that was definitely a secondary gain that you can talk to people about.”

“And that made them excited because they weren’t going to be PIs and they weren’t going to be able to do that but they cared enough to contribute to what was being developed in the field.”

Building on success

“And there’s nothing that sustains a process like a few wins. You know? And I think we’ve had a few good wins. And so it’s kind of made it fun.”

“That’s a superb study that will give an answer... if these guys come up with the next study... I think they’ll get funded. Because of what they’ve done previously.”

Conclusions

Experienced trialists identified strategies to improve the research enterprise, including building a sense of community, training and experience for new investigators, and to ensure fair recognition for research efforts. To be adopted, these strategies will require action by a variety of actors: individuals, institutions, groups of researchers, formal research networks, and national and international professional organizations.

Future studies should seek the views of researchers from a wider range of countries (including more middle-income countries) and individuals from granting agencies, industry, and hospital and academic administration who may have different perspectives.

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