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Rejoinder “Effectiveness in practice-based research: Looking for alternatives to the randomized controlled trial (RCT).” Why and when Randomized Controlled Trials are a necessity

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Tavecchio’s paper states that RCTs are impractical in the daily practice of social work and public health professionals. He argues that its external validity is limited and that the application of knowledge acquired by stringent application of RCTs takes too long. I believe his arguments are a little off and I shall argue why.

Recently, we experienced a very painful situation in the Dutch scientific community. On November 16, 2011, Don Poldermans, a well-respected Dutch cardiovascular medicine researcher was accused and convicted of fraudulent practices in his research (“Don Poldermans,” 2014): “Firstly, some of the randomized controlled trials did not obtain written informed consent from the participants before randomly allocating them to different strategies. These actions were a serious breach of medical research conduct. Secondly, the committee determined that the data were not collected according to the protocol described beforehand and reported in the publications. For example, events such as myocardial infarction were not diagnosed by a panel of independent researchers, but by a single person who made no documentation for the reason for the categorizations, which were later found to be contradictory to the patients' own medical records. Thirdly, the committee determined that, in several cases, these trials had fabricated data. Lastly, the committee found that untrustworthy data had been knowingly submitted for publication, another breach of proper scientific conduct.” Why is it important that these rules for scientific integrity are maintained? And why are RCTs a necessity in this line of research?

Firstly, RCTs should only be applied when there is a necessity to ascertain a causal conclusion. A well-executed RCT excludes alternative explanations for the results, so that these results can be attributed to the treatment and the treatment only. The necessity for this is most clear in situations when treatments are decisive for life or death. Not all of Poldermans publications have been scrutinized. An important and still influential study was his research of the application of beta blockers and statins in patients undergoing abdominal aortic aneurysm surgery (Kertai et al., 2004). The study claims that the use of statins and beta blockers reduces perioperative mortality and nonfatal myocardial infarction. Regretfully, this study was not an RCT. The patients were not assigned randomly to treatment or control groups. The study was not executed with double blind control measures. Therefore, alternative explanations can be valid. In their paper, they mention two important alternative explanations (Kertai et al., 2004, p. 350): 1. The positive results

can be valid only for the selection of patients that have been treated in this way. 2. It cannot be excluded that the patients in the treatment group received better medical attention. This should lead to most cautious interpretation of these results. Regretfully, the application of beta blockers and statins made it into the recommended guidelines of the European Society of Anaesthesiology (Poldermans et al., 2010). A recent meta-analysis which also included well-executed RCTs (Bouri, Shun-Shin, Cole, Mayet, & Francis, 2014, p. 1) concluded: “The well-conducted trials indicate a statistically significant 27% increase in mortality from the initiation of perioperative β-blockade that guidelines currently recommend.” Application of the guidelines may have cost the life of hundreds of patients. This is a most dramatic illustration of the necessity of well-executed RCTs.

The kind of causality research conducted with RCTs, concerns always a question of mono-causality. Simply put: Can a treatment be considered as the cause of the result achieved, with the exclusion of possible alternative causes? Surely, almost everything is multi-causal determined, but that is not the object or purpose of an RCT. As illustrated, often mono-causality is of utmost importance. But there are also many exceptions. In many cases, multi-causal research suffices: research that focuses on the results achieved and the identification of predictors that jointly provide an explanation of that result. But such a study is very dissimilar to an RCT and does not allow for mono-causal conclusions. For the investigation of multi-causality, there are various methodologies, including surveys, structural equation modeling, and linear equation models. This kind of research is also more suitable to answer the question of generalizability. Qualitative research is yet another form of research. This allows for examination of possible causes of an event or achieved result and the stipulation of possible conditions that have led to the event or achieved result. This kind of research can be most insightful and have a large practicality. But this is also very different from the goal of determination of mono-causality. It makes little sense to mix these various forms of research or to criticize RCT because it has a specific purpose that is different from the goal you want to achieve.

In most public health studies, there is no question of life or death. Tavecchio suggests that in the case of public health or social work we should not bother over all-too-stringent scientific methods. Nevertheless, treatments applied in public health and social work can be costly. Should we not ascertain that our and public money is spend in an effective manner? Is the question whether treatments in these fields are truly effective not important? When less stringent scientific methods are considered, the first question to be asked is whether the causal conclusion is relevant. If not, less stringent scientific methods can be applied. For instance, when the only question is whether patients are satisfied by a certain treatment, it does not matter whether this satisfaction is the result of the effect of the treatment itself or for instance extra attention or even the pleasant personality of the treating social worker. When costs are low or not an issue, it can be more important that the clients feel helped than the question whether the treatment can be interpreted as the only source of the desired

change. In general, when a desired change has been established with acceptable costs, the causal question may be of no or limited relevance. Regretfully, these points are insufficiently addressed in Tavecchio’s paper.

The alternative methods suggested by Tavecchio do not allow for stringent causal conclusions. Any conclusion concerning the results can be explained in various manners when the alternative methods are applied. Most often, alternative explanations remain possible, such as, the results can be solely obtained in a special group of clients or the results can be obtained by a specific group of (enthusiastic) social workers which is not easily replicated by other social workers. Or, desired results are partially established as the result of targeted attention, independent of a specific treatment method. Also, in a non-controlled study without any control group, positive results may have been caused by beneficial circumstances. The question whether the treatment can be seen as the causal agent of change or other circumstances cannot be answered when using alternative methods. When there is need to answer the causal question with some certainty, an RCT becomes a necessity.

An issue addressed by Tavecchio is the limited external validity of RCTs. He is correct that for trials the possibilities for generalization are limited. But this is completely beside the point: RCTs are designed for answering the causal question, not for answering the question of general applicability or general validity. The limited generalizability is inherent to the RCT when humans are concerned: an RCT is always a study of treatments and a treatment can only be applied to persons who volunteer to undergo that treatment. Without permission, it is unethical to apply any treatment, even when the treatment is relatively innocent. Most treatments are not innocent or cost time or efforts from the patients concerned. When volunteers are used, generalizability of RCT results to non-volunteers or not yet treated patients is limited. Next to volunteers, experimental control also limits the generalizability of RCTs. Control measures are a necessity to exclude alternative explanations for the results other than the treatment. But they also limit the generalizability to situations with no or less control. But again: generalizability is not the reason for applying an RCT. Only the causal question justifies the application of this method. Generalizability should be addressed by either replication of the RCT with a variety of volunteers and a variety of circumstances, or in a dedicated study. Some of the alternative methods suggested by Tavecchio may be helpful for answering generalization questions but none of these methods can answer the causal question in the same manner as an RCT does.

A third point that Tavecchio addresses is the long gap between a first RCT and the application of its results. Of course, the observation of the long gap is correct. Before we can apply positive results of an RCT, the next step is to ascertain that similar results can be found in a variety of patients and under a variety of circumstances. And yes, this is a time consuming procedure. The case of Poldermans, mentioned in the introduction of this rebuttal, illustrates that the lengthy

procedures that are applied in the medical field are sometimes too short: the recommendations based on Poldermans’ studies should never have been applied.

In conclusion: I do agree with Tavecchio in some respects, but I also regret his attempts to disqualify the RCT as a necessary method. My guess is that Tavecchio appreciates insufficiently the power of the RCT to exclude alternative explanations and the absolute necessity to do so whenever a specific causal question is relevant. I also regret that he does not address the question *when* practice-based evidence in public health and social work can be sufficiently valid. His observations are sound only for research questions concerning treatments or approaches that need not to address the causal question and need not to exclude possible alternative explanations. I also find it regretful that he mixes the two most important validity questions (causality and generalizability) as if they ever could be answered in a single study. In public health and social work, situations can arise when it is important to ascertain a desired result as such, while contemplations about the exact causal agent that has caused the result are of secondary importance. In that case, and only in that case we can abandon the RCT and use less restrictive research methods.

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