Why it’s time to stop considering Evidence-Based Policy and Evidence-Based Medicine as analogous when it comes to Randomized Controlled Trials: An argument from Clinical Equipoise

Charlotte Zemmel
Newnham College
University of Cambridge

ABSTRACT

Randomized Controlled Trials (RCTs) play a large role in both Evidence-Based Medicine (EBM) and Evidence-Based Policy (EBP). However, in this paper, I question whether the role of RCTs is directly analogous in the two movements. I centre my argument around the concept of ‘Clinical Equipoise’, a principle which states that an RCT in clinical research can only continue if there is ‘genuine uncertainty within the expert medical community about the preferred treatment’. By illustrating how there cannot be an equivalent ‘Policy Equipoise’ principle, I suggest that policymakers should proceed with caution when appropriating methods from EBM. I show how clinical practice and social policy rely on such different community structures that drawing analogies between EBM and EBP is misguided and can disadvantage Evidence-Based Policy-making.

SCIENCE ⇒ POLICY

Evidence-Based Policy is strongly influenced by Evidence-Based Medicine, a movement dedicated to ensuring that clinical treatment is supported by systematic evidence. However, assuming that these two movements are two sides of the same coin runs the risk of missing the important differences that separate clinical treatment from policy-making.

Keywords Evidence-Based Policy · Evidence-Based Medicine · Randomized Control Trials · Clinical Equipoise
Evidence-Based Policy and Evidence-Based Medicine in Randomized Controlled Trials

Introduction

The Evidence-Based Policy movement (EBP), which represents the claim that social interventions should be based on the outcome of rigorous experimentation, often manifests as the conduct of Randomized Controlled Trials (RCTs).[1] In this method, novel social interventions are tested in an experimental group of citizens, and if the intervention is more successful than the status quo, the intervention is recommended for public policy. Such a method is taken directly from Evidence-Based Medicine (EBM), a movement dedicated to grounding clinical decisions in stronger, scientific evidence. Whilst in EBM RCTs, the interventions are novel treatments for disease, in EBP, the interventions range from programmes to prevent teen pregnancy,[2] nutrition education plans for mothers,[3] or even legislation that makes wearing a helmet whilst riding a bicycle mandatory.[4]

Since RCTs sit at the top of a rigid evidence hierarchy in both EBM and EBP, they constitute the strongest sense in which the two movements are considered analogous. However, I question whether the analogy between the two movements, concerning how RCTs are used in policy decisions, is warranted. While attention has been paid to the issues regarding the causal evidentiary strength of RCTs in the two movements,[5] I focus here on the justification for conducting RCTs to begin with. In EBM, the Principle of Clinical Equipoise serves as the gatekeeper of clinical trials, hence, it plays a central role in enabling RCTs to provide the evidence that clinicians value. This principle states that patients can only be enrolled in a clinical trial if there is “genuine uncertainty within the expert medical community – not necessarily on the part of the individual investigator – about the preferred treatment”. The evidentiary role of RCTs in clinical research is thus to convince the DMC that there is sufficient evidence in favour of the novel intervention to cause agreement within the committee’s members, making it positively unethical for new patients, either inside or outside of the trial, to not receive this new treatment.

Therefore, RCTs play a determinate role in clinical policy decisions; the purpose of trials is to dismiss the epistemic uncertainty amongst a specific community of experts, whose decision has a direct effect on whether a new intervention is licensed. In this regard, the emphasis placed on RCTs in EBM can be easily explained as a necessary condition for consensus amongst the relevant community. If EBP supporters are to maintain that RCTs should sit at the top of an evidence hierarchy in social policy research by continuing to draw analogies to EBM, they must illustrate that RCTs play this same determinate role in clinical policy decisions.

Clinical Equipoise in RCTs

It is not any uncertainty that is required for the equipoise condition in clinical trials to be met, but rather ethnically relevant uncertainty within the epistemically relevant community. In clinical trials today, it is Data Monitoring Committees (DMCs) that “assess the continuing validity and scientific merit of the trial, [ensure] that clinical equipoise is maintained during the trial, and [monitor] participant recruitment, protocol compliance and data quality”. DMCs are individuals with expertise in assessing clinical trial data - they are often clinicians or biostatisticians. Hence they conform well to equipoise’s criterion of “expert medical community” whose uncertainty is ethically and epistemically pertinent to whether the novel intervention should be licenced by clinical policy boards. The evidentiary role of RCTs in clinical research is thus to convince the DMC that there is sufficient evidence in favour of the novel intervention to cause agreement within the committee’s members, making it positively unethical for new patients, either inside or outside of the trial, to not receive this new treatment. Therefore, RCTs play a determinate role in clinical policy decisions; the purpose of trials is to dismiss the epistemic uncertainty amongst a specific community of experts, whose decision has a direct effect on whether a new intervention is licensed. In this regard, the emphasis placed on RCTs in EBM can be easily explained as a necessary condition for consensus amongst the relevant community.

1 It should be noted that this does not always happen in practice - the outcomes of RCTs often generate controversy rather than form a consensus. Ubel & Silbergleit have responded to this issue by postulating a principle of “behavioral equipoise” which defines a new criteria for overcoming uncertainty within the relevant community by thinking about behavior rather than just belief.
Evidence-Based Policy and Evidence-Based Medicine in Randomized Controlled Trials

nent role in social policy decisions. As I illustrate below, I do not think this is possible.

At its heart, the EBP movement constitutes an effort to increase the use of evidence in policy making, and decrease governments’ dependency on expert opinion. However, this by no means implies that evidence plays a determinative role in policy making. Evidence rarely directly results in policy, but rather, “contributes to informed discourse” surrounding candidate policies. This mirrors the fact that social policy is formulated by “policy communities”, composed of a range of actors such as social scientists, civil servants and politicians, each with varying amounts of both experimental and political authority and responsibility. Ray Pawson illustrates this point by arguing that EBP is constructed out of a series of “intricate and potentially fragile relationships” between various consumers and producers of evidence. For example, industry can be thought to be both a recipient and a provider of policy-relevant information. Far from evidence determining policy outcomes, as in EBM, the use of evidence in policy making is “premised on a partnership” between information and action.

The reason why evidence cannot play a determinative role in social policy is because there is no single community whose uncertainty is ethically relevant. Rather, there are many, each with different evidentiary requirements. In his attempt to construct a Principle of “Policy Equipoise”, Douglas MacKay proposes that we should think of the government as constituting this relevant community, thus, citizens can only enter trials if there is sufficient disagreement amongst ‘the government’. The issue with this view is that it is wholly unclear who within the government must disagree in order for equipoise to be declared. As the discussion above on the social structure of EBP suggests, ‘the government’ as an epistemic community should not be considered homogenous.

The non-determinate relationship between RCT evidence and policy is best illustrated with an example. Khosrowi and Reiss argue that the emphasis on RCTs in developmental economics research in recent years has led to an impoverishment of interest in macroeconomics such as work on democratization, fighting corruption, and assessing governance. On the other hand, there has been a growth of interest in microeconomics, such as the enforcement of specific laws, or the building of specific infrastructure. The reason for this switch in focus is that macroeconomic questions are not the kind of questions that can be answered through RCTs, but rather, they require broader forms of reasoning. Thus, RCT evidence would not convince a macroeconomist. Since the emphasis on RCTs in evidence-based medicine is justified by their necessary role in forming a consensus in the relevant community, this example reveals that RCTs cannot perform the same role in evidence-based policy, unless one is willing to accept that the viewpoint of the macroeconomist is not ethically or epistemically relevant in policy making.

Conclusion

To conclude, I return to the example of clinical equipoise. This concept appears viable in medical contexts because of the direct relationship between the justificatory practices of RCTs and clinical policy. Thus, RCTs are necessary to dispel ethically relevant disagreement within the epistemically relevant community. In EBP, there is no singular relevant community, hence, the concept of equipoise does not track well. This failure of policy equipoise highlights the deeper issue at stake here, which is that the contrasting community structures in clinical research and social policy mirror different standards for whose uncertainty matters. The range of scientific and political agents that constitute policy communities should indicate to the reader that deeper considerations should go into deciding between social interventions than can be provided by RCTs alone. The burden now falls on EBP defenders to justify the elevated importance placed on RCTs in their discipline.

Acknowledgments

The author wishes to thank the anonymous reviewers for their excellent suggestions.
References


About the Author

Charlotte Zemmel is an MSc Natural Sciences student at Newnham College, studying History and Philosophy of Science. Her main research focus is on social epistemology, philosophy of medicine, and the philosophy of science policy. Her current work is focused on investigating the relationship between uncertainty, policy decision making, and Randomised Controlled Trials. She can be contacted at ccz23@cam.ac.uk.

Conflict of interest The Author declares no conflict of interest.