Review of: "Getting It Wrong Most of The Time: Comparing Trialists' Choice of Primary Outcome With What Patients And Health Professionals Want"

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This is a very readable report of a simple but important study, which goes straight to the heart of a dilemma. What is the use of evidence if it doesn't correspond to what you want to know about?

Here I am offering a personal commentary rather than a structured review. I am old enough to remember the excitement and scepticism which greeted the first use of the phrase "evidence-based medicine" in the early 1990s. Despite some caveats, I was an early enthusiast. It seemed to me that the toppling of expert opinion in favour of careful quantitative analysis of what was actually known had to be a good thing. This process would surely identify big gaps which would form the entire new agenda of medical research. This article is one of many which shows how this did not happen. Even if the questions are the right ones, the answers are not, in 70% of cases. In effect this paper is like taking a water sample from a reservoir: if quality is so poor here, 30 years into the history of EBM, how have we managed to install such terrible purification processes and what are we to do about it?

This is by no means the only issue which makes at least 80% of research unfit for use in clinical decision making. Others are alluded to in passing throughout the course of this article. But primarily this study is about outcome measures in interventional trials of breast cancer and kidney disease, and how they relate to the outcomes which are actually most important to patients with these conditions.

It is perfectly possible to ask patients which outcomes matter most to them. Experience and common sense alike tell us that these outcomes will differ between different people with the same condition, and between the same person at one stage of their illness and another stage. But randomised controlled trials are an exercise in aggregation, with significance expressed statistically in terms of a group effect. And this has led to a mode of thinking which feeds back even to such ostensibly patient-centred endeavours as the establishment of sets of patient reported outcome measures (PROMs or PROs). These are ordered as priorities not for individual patients but for patient groups, and are intended as guides for researchers. Use of PROMs would certainly have helped to mitigate the gross mismatch seen in this study.

But what is to stop us considering an even more revolutionary idea? What is to stop researchers actually asking each person they recruit what is most important to them – what success would like for them as individuals? Why is this not a routine question and outcome metric for every trial?

With this in mind, let's go back to the article itself. The Background section is a little generalised, dwelling

on the complexities of data collection without making the point that the trials in question are very much about patient management. The conditions in question are potentially life-threatening and frequently life-changing. The interventions are almost certainly part of a package which may vary over time. The most important concern of the decision-maker should be to align with the priorities of the patient. Indeed, the role of the researcher seeking informed consent cannot be seen as separate from the role of the caregiver whose first concerns are the welfare and autonomy of the patient. I am making these points here because they are not made explicitly in the article, as I think they should be.

I think it would also be helpful in the introduction to acknowledge the decades of work which have already been done on various aspects of this question, particularly within the PROMs community, COMET and within the James Lind Alliance which is mentioned later.