

Essay 3. RCTs and Relational Efficacy. Leah Schwartz

Essay prompt: 'What kind of efficacies evade Randomised Controlled Trials (RCTs)?'

Drawing upon her three decades of ethnographic research in Tibet, medical anthropologist Vincanne Adams writes, 'From the perspective of practitioners of Tibetan medicine, the standard of the randomized controlled trial is both seductive and problematic. Seductive, because desires for approval by Western medical scientists sometimes belie the ability for these practitioners to see how uneven the epistemological playing field really is. Problematic, because placing a bet that their treatments will win in this game – and submitting to the logic that unbiased science will offer definitive truths about treatment efficacy – also renders them vulnerable' (Adams 2002: 669). In her research on a burgeoning Tibetan pharmaceutical industry, Adams presents a particularly illuminating narrative, that of Fei Fei Li, a Chinese-born engineering student who ran a clinical study of Tibetan therapeutics for *Helicobacter pylori* (HP) infection:

After one year of research on a total of 60 patients, Li found that in all cases, the Tibetan medicines eradicated the symptoms that biomedicine has typically associated with HP. That is, by the Tibetan definitions of the diseases and the symptoms used to determine these diseases, two kinds of Tibetan medicine worked well to cure all of the patients. In the cases that were pursued in a five-month follow-up, all symptoms were still eliminated. However, using biomedical measures showed that in no case was Tibetan medicine able to eradicate HP. Rather than interpreting these results as an opportunity to question the associations being made by biomedical researchers between the defined symptoms and HP bacteria, the Tibetan doctors involved in Li's study interpreted the results as evidence of the failure of Tibetan medicine, because it could not eradicate the HP infection... A year later, she returned to do a long-term follow-up study of her patients, and learned that a majority of the patients were still symptom-free. However, the hospital refused to support a full project to document the extent of these outcomes on grounds that, as the head of the digestive unit told her, 'The research had already proven to be unsuccessful in demonstrating the efficacy of Tibetan medicine'. (Adams 2002: 663)

Adams' detailed account of Li's brief foray into Tibetan medicine underscores what is at stake for various parties in the evaluation of efficacy. For biomedical researchers, such as Li, the utilization of imported research methodologies – in this case the randomized controlled trial (RCT) and associated notions of efficacy – perpetuates and instantiates the hegemonic role of biomedicine in non-Western contexts; in Li's case, this resulted in her expressing remorse for having introduced notions of efficacy that she believes would not

have been employed had she not introduced them herself. For practitioners of indigenous medicine, in this case Tibetan doctors, the use of an RCT to evaluate their work resulted in an 'extraordinary loss of confidence' in their professional abilities, which, in turn, resulted in a devaluation of their therapeutic arsenal (ibid.). Perhaps those who have the most to lose, though, are the patients; indeed, despite the fact that all the patients enrolled in the clinical trial remained symptom-free after a year, future patients who might similarly benefit from the therapy under investigation are prevented from doing so because biomedical measures deemed the therapy to be ineffective. Regardless of the nature of their individual contributions, each stakeholder plays a role in constructing and legitimizing a certain understanding of efficacy that has real, and potentially harmful, implications.

In this paper, I will argue that RCTs employ a narrow conception of pharmacological efficacy, one that is deeply rooted in biomedicine and that is often taken for granted by researchers who conduct and analyse RCTs. As a result, other, equally important notions of efficacy are left unattended. First, I will suggest that RCTs fail to measure any notion of efficacy that is conceived within a body of medical knowledge that understands healing as processual (e.g. Etkin 1988; Waldram 2000; Barry 2006). Finally, I will argue that 'relational efficacy', which encompasses Susan Whyte et al.'s (2003) concept of 'social efficacy', similarly evades the measures of RCTs.

The Randomized Controlled Trial: An Imperfect Tool

The RCT has been considered the 'gold standard' for clinical experimentation for at least the last century. Briefly, the RCT involves the random assignment of subjects to experimental and control groups; the ideal RCT also involves a process of 'double-blinding' in which neither the patients nor the researchers possess knowledge of whether the experimental therapeutic or a placebo has been given/taken. Christine Barry (2006: 2648) astutely points out a common criticism of the tool, namely that the real-world clinical context is very different from the trial laboratory. Furthermore, she contends that even the most elegant RCTs only measure a small subset of symptoms and therapeutic effects, typically those that are short term and easiest to measure. In this way, Barry, along with many other critics, asserts that the RCT is an 'imperfect tool' and that therefore to imbue it with the legitimacy to establish objective medical truth is fundamentally misguided (ibid.). Indeed, she also writes that 'the production of scientific evidence is a social as well as a scientific process. There is no such thing as The Evidence, just competing bodies of evidence' (ibid.).

In highlighting the shortcomings of the RCT as a tool for establishing medical truth, it becomes possible to understand just how problematic the utilization of such a tool in non-Western contexts might be. Indeed, if we are to accept that RCTs are an imperfect tool even for evaluating biomedical interventions, which share the same epistemological underpinnings as the tool itself, what are we to make of cases in which RCTs are employed to assess the efficacy of therapeutic interventions borne from a divergent epistemology? On this, Barry writes that ‘RCTs usually omit the measurement of important elements of “what works” in alternative medicine, which often acts in a different way to biomedical drugs,’ arguing that ‘evidence, when seen from the perspectives of the users and practitioners of alternative medicine, hinges on a very different notion of therapeutic efficacy’ (ibid.: 2647). Glyn Adams takes Barry’s critique one step further, suggesting that, even when the same diagnostic instruments are used to make claims about the efficacy of biomedical and other medicines, the empirical evidence may be understood differently (Adams 2002: 672).

Accounting for the Processual Nature of Healing

In ‘Cultural Constructions of Efficacy,’ Nina Etkin observes,

One of the most formidable obstacles to full comprehension of efficacy and other characteristics of indigenous medical systems is the failure to understand healing as *process*. In its totality, medical treatment should be understood as a complex and processual ordering of biological and behavioral expectations which can to varying degrees be differentiated from one another. Thus, efficacy might mean a number of things, ranging from full symptom remission to some physical sign (e.g., fever, salivation, emesis, etc.) which is interpreted as a requisite *proximate* effect that indicates that the curing/healing *process* is under way and can be expected to proceed to the *ultimate* outcome—i.e., restoration of health with, perhaps, other proximate effects anticipated along the way. (Etkin 1988: 302, original emphasis)

James Waldram draws an important contrast between Etkin’s description of indigenous medical systems and conventional practice in biomedicine, noting that, whereas the former may view linear time as irrelevant, biomedicine sets out temporal benchmarks, at which point the absence of disease pathology becomes equated with cure (Waldram 2000: 611). This distinction raises two practical issues with regard to the pharmacological efficacy that RCTs take as their object of inquiry, each of which I will describe using an illustrative ethnography.

In her work with the Hausa of northern Nigeria, Etkin describes the Hausa treatment for eye and skin inflammation, as well as for cough. In the case of eye and skin inflammation,

the Hausa first treat wounds with plants intended to cause irritation and induce bleeding, followed by plants intended to reduce swelling and maintain hemostasis. In the case of treatment for cough, Hausa therapies include the burning of plants and the inhalation of smoke (Etkin, 1988:308). In both of these cases, the various phases of the treatment process have different expected outcomes. For example, the Hausa believe that the inhalation of smoke, which may initially cause further irritation, will later produce decongestant and antitussive effects. Similarly, in the treatment of eye and skin inflammation, the initial use of caustic plants, which are intended to expel dirt from the wound, are but one step in the process of healing the wound, though outsiders might judge their use as only exacerbating the problem. Etkin's ethnography thus brings to light a key aspect of the efficacy of many indigenous treatments that evade RCTs, namely that, logistically and theoretically, the notion of efficacy must be borne of the same epistemological foundations as those of the therapy under investigation. If we are to understand Hausa treatment as processual, it follows that the evaluation of its efficacy will include the evaluation of a series of outcomes over a long period of time rather than a single outcome investigated at an arbitrary point in time, as is the case with an RCT. On this last point, Waldram notes that biomedicine seems to propose 'a logical point in time after treatment at which efficacy can be determined [...] Just how that point is established never seems to be addressed, but this sometimes appears rather opportunistic on the part of researchers' (ibid.: 612).

Barry similarly takes issue with this aspect of RCTs in her work centred on claims of efficacy in homeopathy. Perhaps too simplistically, Barry argues that, because homeopathy views treatment for chronic illnesses as extending over long timescales (in some cases, as long as a lifetime), the utilization of RCTs to assess the efficacy of RCTs makes no sense on either a practical or theoretical level. Using this same logic, she notes that a similar argument can be made for the treatment of individuals through Alcoholics Anonymous, which is founded upon the notion that individuals are never fully cured of alcoholism, but that healing is instead a life-long process (Barry 2006: 2651). From this perspective, it would seem that the use of an RCT to assess the efficacy of Alcoholics Anonymous would necessarily ignore a fundamental aspect of the treatment under investigation. Ultimately, Barry's and Etkin's ethnographies clearly highlight how a conception of efficacy produced within a framework that understands healing as processual evades RCTs. At the same time, these works also draw attention to the problematic use of RCTs to evaluate the efficacy of treatment regimes with which they do not share a common epistemology.

'Relational Efficacy' and the RCT

Another important efficacy that evades RCTs is 'relational efficacy', a term I propose to use to describe a type of efficacy that is produced through the relational nature of actors within a particular social context. This is intimately linked to Whyte et al.'s (2002) notion of 'social efficacy', which 'draws attention to the way medicine works through suggesting something about the people involved' (ibid.: 23). However, I use the term 'relational efficacy' to refer to a broader set of social relations that include not only those between patients, providers, families and communities, but also the relations that each of these actors has with the medicine itself. Furthermore, 'relational efficacy' is meant to attend to the ways in which the pharmacological effects of medicines mediate the social relations in question. Still, 'relational efficacy' is very much related to notions of 'social efficacy' and the 'meaning response', as well as to Helman's concept (2000) of a 'total drug effect', first introduced by Claridge (1970). To further explore 'relational efficacy', I will first describe the related terms mentioned above before referencing three ethnographic accounts, each detailing distinct sets of social relations: between patients and doctors, between patients and their families, and between patients and medicines.

Importantly, Whyte et al. embrace the notion that multiple efficacies contribute to the 'total drug effect' (2003: 30):

the total drug effect depends on a number of elements *in addition* to its pharmacological properties. These are: The attributes of the drug itself (such as taste, shape, colour, name). The attributes of the patient receiving the drug (such as experience, education, personality, sociocultural background). The attributes of the person prescribing or dispensing the drug (such as personality, professional status or sense of authority). The setting in which the drug is administered—the 'drug situation' (such as a doctor's office, laboratory or social occasion) [...] All of these aspects can play a role in generating the 'meaning response' because they can determine the confidence the patient has in the treatment and the expected outcome. (Helman 2000: 170, quoted in Whyte et al. 2003: 172, original emphasis)

Indeed, Helman proposes that the variation in individuals' responses to the same medication can be understood as the result of a difference in the mixing of the various influences he describes. Both Helman's notion of a 'total drug effect' and Whyte et al.'s notion of 'social efficacy' underscore the recognition that, despite attempts to do so by designers of RCTs, it is impossible to fully untangle the pharmacological effects of a drug from the social context in which it is given/taken. For this reason, we must embrace a notion of efficacy that is co-produced by these additional influences.

Several authors have attended to the importance of the relationship between patients and their providers in studies of efficacy. Waldram observes how

the view of the patient is not necessarily distinct or neatly separable from the view of the practitioner in any treatment encounter. These views often interact and affect each other. The physician/healer may ask how the patient is doing, and the response may help form the practitioner's determination of the success of the treatment. Similarly, the physician/healer may inform the patient about the success any particular procedure or ceremony or the results of a test, which will factor in the patient's assessment of his or her condition. (Waldram 2000: 607)

In light of this dynamic, Waldram argues that efficacy is negotiated in each clinical encounter, lending further evidence to support his claim that it evolves over the course of a sickness episode. Another way in which the relationship between doctor and patient contributes to a drug's 'relational' efficacy is through patient perceptions of medical authority, which lend varying degrees of legitimacy to the drug in question. Anita Hardon's ethnography of cough treatment in the Philippines provides a complementary account of the way in which social relations between patients and their families contribute to the 'total drug effect':

Medicines provide women the reassurance that something can be done about the illness (a sense of agency if you like) and children with the recognition that they are ill and entitled to good care. They also show others in the community that the child is being looked after—obviating judgments of parental negligence. Social and pharmacological efficacies are co-produced in the therapeutic process [...] The 'calming' down of the cough is desirable socially—the sound of the cough signals poor care. It not only irritates the child's lungs, it also irritates others, like fathers and mothers-in-law, potentially leading to social distresses that go beyond the illness condition of the child. (in Whyte, van der Geest and Hardon 2003: 30)

Hardon's ethnography is particularly illuminating because it describes how multiple efficacies are co-produced during the course of an illness. Moreover, Hardon carefully locates the effects of the medicine in each of the social relations in which they are manifested. By doing so, she suggests that the taking of medicine is not only a medical act, but also a social one (ibid.: 171).

Finally, Hardon and Etkin both write about another relational aspect that is important in defining efficacy: that between the patient and the medicine. In Hardon's work, she describes the lay notion of *hiyang*, which literally means 'compatibility' and which

underscores the individual ways in which medicines work upon illness (ibid.: 28). More specifically, Hardon observes, ‘Efficacy depends on the suitability of a drug for a particular person. *Hiyang* explains why the drug works for one patient, and not for another’ (ibid.). Etkin describes a similar concept employed by the Hausa to understand failures in therapeutic efforts; rather than locating the failure in *either* the individual *or* the medicine, the Hausa take it ‘as a sign that the medicine and the individual were not “right” (suited) in that particular instance’ (Etkin 1988: 301).

Whereas biomedical protocols assume that a given drug exerts the same effects on every patient, this is clearly not so the case in other medical systems. As Hardon writes, ‘*hiyang*-like concepts emphasize individual differences in efficacy: bodies are not the same and pharmacological efficacy is relational—it depends on the compatibility between the pharmaceutical and the individual taking the drug’ (in Whyte, van der Geest and Hardon 2003: 32–3). This final example contributes to the concept of ‘relational efficacy’ in perhaps the most direct way by highlighting how efficacy is influenced by variations in individual biologies, as well as by the interaction of these biologies with individuals’ social contexts. This efficacy is certainly evaded by RCTs, which take drugs as the sole site of efficacy-production.

Conclusion

In this paper, I have focused on what I believe to be two critical types of efficacy that evade RCTs: an efficacy that understands healing as processual, and what I have called ‘relational efficacy’. In both of these cases, however, I hoped to draw attention to the fact that, while we may discuss various efficacies individually, they are in fact mutually constitutive, which Claridge explained best through his concept of the ‘total drug effect’. As the authors of *The Social Lives of Medicines* write, a double-blinded randomized controlled trial ‘is designed to isolate for purposes of analysis (to dissolve a whole into parts). But life is lived as a synthesis (a putting together of parts into wholes). Not only do efficacies tend to combine, but the acts of giving/taking medicine and looking to effects are integrated into larger processes of dealing with problems and living life’ (Whyte et al. 2003: 36). One obvious implication of this analysis is that we should situate studies of efficacy within their proper contexts, rather than in the laboratory.

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