

#### Essay 4. What kinds of efficacies evade Randomised Controlled Trials (RCTs)? Mason Alford

*Essay prompt: same as in essay title*

In his 1976 paper, Allan Young defines efficacy as ‘the ability to purposively affect the real world in some observable way, to bring about the kinds of results the actors anticipate will be brought about’ (Young 1976: 7). The first step in any understanding of efficacy, therefore, is to specify those relevant actors involved in its construction. Efficacy, within Young’s framework, lies in the eye of the beholder, and it follows that the measurement of efficacy pivots on the crucial definition of ‘beholder’. Within the sphere of the randomized controlled trial (RCT), efficacy is confined to a largely biomedical and quantitative understanding. This leaves ‘little room for the role of the patient in assessing efficacy’ (Waldram 2000: 606). Efficacy in these terms is reduced to a decontextualized, biochemical and pharmacological phenomenon, divorced from the hopes and fears of the social actors pertinent to the sickness episode. Mark Nichter distinguishes this reductionist ‘curative efficacy’ from ‘healing efficacy,’ which ‘involves the perception of positive qualitative change in the condition of the afflicted and/or concerned other’ (Nichter 1992: 226). While the curative approach frames efficacy as a discrete and static entity, healing efficacy is fluid and shifting, intimately linked to the perceptions and expectations of social actors. RCTs fail to account for efficacy as a subjective outcome embedded within a relational and therapeutic context. They remain confined to a curative framework that celebrates efficacy as a discrete, isolated episode; it is unable to reframe ‘healing as process’ (Etkin 1992: 102), or allow for an understanding of ‘incremental efficacy’ (Csordas 1996: 106). Indeed, as James Waldram notes, ‘assessments of efficacy are shifting, often building on one another over time’ (Waldram 2000: 611). This paper contends that randomized controlled trials fail to account for therapeutic efficacy as a dynamic and negotiated process among social actors, thereby neglecting overarching efficacies – social, material and symbolic.

The effect of medicine on the relationship between those enacting illness and treatment lies outside the analysis of double-blind experimentation. Social efficacy evades the scope of RCTs because the latter methodology recklessly privileges pharmacologists alone with the accolade of objectivity; solely biomedical researchers are granted access to legitimate knowledge claims. The expectations and hopes of the broader social network

participating in the sickness episode are dismissed within RCTs, as ‘concealed history augments the appearance of an obvious transcendent truth’ (Kaptchuk 1998: 432). In order to fabricate a decontextualized and universal reality, RCTs must dismantle that which is locally alive and culturally specific. Susan Whyte, Sjaak van der Geest and Anita Hardon flag the limitations of the RCT approach by countering that ‘in real life efficacies are assessed not by pharmacologists but by social actors, who have their own criteria and expectations’ (Whyte et al. 2003: 23). Efficacy is necessarily situated in the perceptions of the patient, who is ‘not Rational Man looking for medical efficacy; but more often is looking for efficacy through meaning in a socio-political and economic context’ (Crandon-Malamud 1991: 33). Efficacy is thus embedded in a web of dynamic social actors, whose judgments on the efficacy of treatment influence perceptions across the social network. Social interactions are overlooked by the randomized controlled trial; the ‘beholders’ of efficacy are too narrowly defined by the RCT.

The social efficacy of medicine overlooked by randomized controlled trials becomes apparent in Anita Hardon’s ethnographic portrayal of children’s coughs and colds in Manila. The significance of a child’s cough extends beyond the pharmacological deviance purported by RCTs and extends into social politics and a geography of blame. A young Filipino mother’s description underscores this social significance of illness: ‘If the cough is continuous, the child’s father is disturbed. Because I am the mother, if the child is sick, I am blamed’ (Whyte et al. 2003: 25). From the perspective of the young Filipino mother in this context, efficacy is not limited to the potential cure of a biological malady, but also the restoration of social harmony within the family. In this account, medicine for the child carries with it the social efficacy of positively confirming the young Filipino mother in her maternal role, both in her own eyes and in those of her husband. In this way, ‘social and pharmacological efficacies are co-produced in the therapeutic process’ (ibid.: 30). This ethnographic account highlights the limitations of RCTs in not operating under any positivist notion of health, opting instead for a concentration on the remediation of divergence and irregularity. Such negativist orientation renders the RCT unable to incorporate the social efficacy of medicine into its analytical framework, given that ‘for patients, the most important thing is not objective measures of anything, but whether or not they can climb stairs, lift their grandchildren, or mow the lawn’” (Moerman 2002: 63). Social efficacies defined by harmony in social relationships and the fulfilment of self-potential fundamentally evade the RCT’s measuring capacity.

The scope of the RCT also neglects the ‘meaning response’ derived from the material and symbolic efficacy of medicine. The materiality of medicine—its colour, shape, form and medium of administration—is embedded within a semantic network that carries influence in the assessment of efficacy. Among American medical students, warm-coloured capsules (pink) serve as more effective stimulants, while cold-coloured ones (blue) function as superior depressants (Moerman 2002: 48). Notably, this ‘meaning response’ is fluid, shifting with corresponding changes in locally embedded semantic networks. For example, among men in Italy, for whom blue is associated with the excitement and thrill of the Italian national soccer team, the blue capsules serve as superior *stimulants*. In this context, blue means ‘success, powerful movement, strength and grace on the field’ (ibid.: 49). Such locally embedded symbolic efficacies evade the enterprise of RCTs, which measure efficacy in the narrow terms of pharmacological potency and biochemical predictability.

In discussing the symbolic efficacy of medicine, it is important to distinguish ‘meaning response’ from the notion of ‘placebo.’ Far from being limited to inert treatments which lack an active pharmacological ingredient, the ‘meaning response’ ‘effects showed up not only when people took placebos, but also when they took real drugs’ (Moerman 2002: 50). Independent of its biochemical reactivity, the effect of medicine could be heightened or lowered, expanded or constricted, by the associated expectations and meanings linked to the treatment. This ‘meaning response’ can help explain the differential assessments of efficacy associated with various mediums of drug administration—‘it is widely recognized,’ explains Moerman (ibid.: 51), ‘that tablets are weak, capsules are preferable, and injections possess the greatest potency.’ These associations remain intact regardless of the underlying pharmacological activity of the medication, and it is ‘this symbolic value of medicines that evokes the meaning response’ (Whyte et al. 2003: 28) with consequences for health outcomes and incremental assessments of efficacy.

This symbolic efficacy of medical treatment is made clear in the 1960 Kansas City experiment testing a surgical procedure designed to relieve symptoms of heart angina. Based on a functionalist theory envisioning heart arteries as rusty pipes, the complex surgery involved ligating arteries to redirect blood flow and ease heart pain. Half of the participants in the Kansas City study received the surgery, while the other half received a ‘sham surgery,’ in which the entire procedure was done except for the critical step of ligating the arteries. Six months after the surgery, patients were assessed by cardiologists who were unaware of which patients had received ligations and which had not. One patient in the Kansas City study (whom I will refer to as Richard), when asked if he felt better, said ‘Yes. Practically

immediately I felt better. I felt I could take a deep breath [...] I figure I'm about 95 percent better' (Moerman 2002: 59). Richard's arteries had not been ligated; he had received the 'sham surgery.' It is important to note that, although Richard had not received the technical intervention, he had still been given 'all the elements of meaning he needed' (ibid.) to move forward in the healing process. The very act of surgical treatment—including the associated acts of signing into the clinic, lingering in the waiting room and receiving anaesthesia—served to validate his pathology, engage his expectations and kindle his hope. The functionalist explanations of the surgeons, portraying the heart as a fixable machine, had provided Richard with a symbolic framework within which he could situate his healing process, given that 'the notion that we can, by shutting off the flow of blood down one pipe, enhance the flow into another pipe—sort of like what happens in the bathroom sink when you turn off the shower—makes very good sense' (ibid.). Medical technologies have meaning, which create expectations, which lead to physiological effects—'we all know that surgery is *really* powerful' (Moerman 2002: 53). Such symbolic efficacy of surgical treatment is evaded by the randomized controlled trial, which posits that therapeutic efficacy lies not in the perceptions of the patients, those social 'beholders' of efficacy, but in the decontextualized biomedical repercussions of the intervention.

Richard's case also bespeaks the overlooked social efficacy of surgical treatment. In the assessment of 'incremental efficacy' (Csordas 1996: 106) by the cardiologists in the months following the surgery, there is a social exchange between patients and providers who together negotiate the observed efficacy of the operation, which always occurs within a field charged with symbolic and social efficacies. Efficacy is borne from this mutual interaction, this bi-directional influence between social actors that together converge upon common (or discordant) judgments of efficacy. The effectiveness of intervention is mediated between mutually concerned parties: 'patient and practitioner exchange views in a treatment encounter so that their assessments influence each other; the patient's expressions about the efficacy of a treatment affect the practitioner's judgment of it too' (Waldram 2000: 607). It is not only the retrospective reaction of patients and practitioners that mutually reinforce each other in negotiating efficacy, but also prospective expectations before the treatment that also influence its assessment. The novelty and excitement surrounding Richard's treatment—the bilateral internal mammary artery ligation—serve to frame the expectations and enthusiasm of both his cardiologists and himself, and 'it is known that the providers of drugs are affected by their meanings too: physician enthusiasm enhances the 'meaning response' of the patient' (ibid.).

This is the crux of the RCT's shortcomings: that efficacy is framed as a dehumanized biochemical event, and not a fundamentally social one.

The de-contextualization of efficacy in the randomized controlled trial also gives rise to problematic notions of the 'primary' and 'side' effects of medication. Underlying the biomedical designation of a 'primary' effect of medicine lies an epistemological claim of intended purpose, meaning and effect, all of which may or may not be shared by the social actors engaging with that therapy. In this way, randomized controlled trials neglect the efficacy of 'side' effects in constructing meaning for their users, instead dismissing them as extraneous nuisances that detract from the drug's supposedly prevailing benefit. Such reasoning neglects the social and cultural context within which the healing process develops, given that 'the appropriation and expropriation of pharmaceuticals outside of biomedical contexts are fraught with meanings beyond what is simply and empirically observed, including complex constructions of primary and side effects' (Etkin 1992: 108). Pharmacological repercussions of therapy are interwoven with locally based illness etiologies, and the 'issues of primacy of action are further obscured when the less superficial contextualized aspects of therapeutic encounter are brought to bear' (ibid.: 102). Much more than an auxiliary annoyance, 'side effects' often carry functions and meanings that evade the randomized controlled trial. Nina Etkin's ethnographic work in Nigeria documents how the 'Hausa consider bitter medicines to be dangerous for pregnant women because of gastro- and uterotropic effects, and they expropriate and initialize this "secondary" effect when abortion is the desired outcome' (ibid.: 104). Efficacy in this case is constructed by both culture and condition—underlying semantic networks link the taste of medication (bitterness) with some secondary effect (gastrointestinal disruption and/or uterine contractions), and this effect is leveraged when the therapeutic context demands uterine disturbance. Other practices of the Hausa underscore the semantically charged nature of 'side effects: 'Among signs of disease regress, dermal manifestations such as rash and urticarial assure Hausa that, in one direction, disease agents do not breach the corporeal and metaphorical barriers to the more vulnerable interior (*ciki*)' (ibid.). The side effect of skin rash, disregarded in RCTs as an unfortunate component of some beneficial drugs, is locally transformed into an important indicator of the regression of disease; skin rash becomes a measure of efficacy and a sign of 'healing [which] may or may not entail curing' but is defined in terms of the 'symbolic aspects of treatment' (Nichter 1992: 226). The limited scope of the randomized controlled trial is once again manifested in its non-distinction between curative and healing efficacies. 'Clearly, then, the primacy or subordination of effects depends on why a medicine is administered, the

intentions of the user and prescriber, and the anticipated outcome—in short, its cultural context’ (Etkin 1992: 102). These efficacies of so-called ‘side effects’ evade the analysis of RCTs.

The RCT is thus fundamentally a reductionist enterprise, aiming to isolate a whole into observable parts. This is the first and fatal step, for ‘life is lived as a synthesis, a putting together of parts into wholes’ (Whyte et al. 2003: 36). The individual does not experience any one of the efficacies separately (Hsu 2012), but integrates social efficacy, symbolic efficacy and indeed pharmacological efficacy. There is a dialogue and interaction between these various forms of efficacy among social actors, who continually blend and co-evaluate their expectations and responses to therapy to produce shifting assessments of ‘incremental efficacy’ (Csordas 1996: 106). This integration of the social, symbolic, and biochemical is evaded by randomized controlled trials, which strive to parse and sequester efficacies that are experienced as an interrelated whole. By privileging biomedical pharmacology with the accolade of objectivity and neglecting to acknowledge efficacy as a multi-directional social negotiation, RCTs fail to situate the act of giving and taking medicine ‘into larger processes of dealing with problems and living life’ (Whyte et al. 2003: 36).

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