Chapter 21 Evidence-based practice - the ethical dimension

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What has come to be called evidence-based practice is an approach to the evaluation and application of research evidence to clinical practice that, while ultimately traceable back to Enlightenment understandings of the generation and testing of empirical knowledge and, more recently, to writings of the logical positivists and Karl Popper has a relatively recent origin. While, as with most intellectual developments, it is difficult to identify a particular person who was the first to articulate the idea, most authors trace its inception to the work of the British epidemiologist Archie Cochrane, who suggested (Cochrane, 1972) that because healthcare resources will always be limited they should be used to deliver interventions and services that well-designed evaluations have shown to be effective. A group of epidemiologists, biostatisticians and experts in medical informatics at McMaster University restated these principles (EBM working group, 1992) in a way that formed the basis for subsequent developments. They named their approach Evidence-Based Medicine (EBM), though it has since come to be applied to a range of healthcare professions resulting in a slight change in terminology to Evidence-based Practice (EBP). Indeed, the influence of the concept has continued to expand beyond healthcare to the extent that the term Evidence-Based Policy Making (EBPM) is now being employed (e.g. Solesbury, 2001; Cartwright and Hardie, 2012). A recent example of this is the UK government's decision to establish four centres that will "produce and disseminate research to local decision-makers, supporting them in investing in services that deliver the best outcomes for citizens and value for money for taxpayers" (Cabinet Office, 2013). This document opens with the statement "Our world leading 'What Works' approach will ensure evidence is at the heart of decision making". Although a number of acronyms have been used for this approach to evaluating evidence I will use EBP here as it is more relevant in the context of this book.

The very pervasiveness and the extent of the influence enjoyed by EBM/EBP/EBPM indicates that it is important to understand just what is entailed by the approach and the ethical issues that some authors and commentators have raised in relation to it. The aim of this chapter is to examine some of the complexities, challenges and indeed contradictions inherent in the

approach and to explore some of the implications for the ethical practice of healthcare in its many forms.

What exactly is Evidence Based Practice?

A useful place to start is with the definition of EBM put forward by Sackett et al (1996), whose book on EBM (Sackett et al, 1997) is arguably one of the most influential texts in the area.

"*Evidence-based medicine is the conscientious, explicit, and judicious use of current best evidence in making decisions about the care of individual patients. The practice of evidence-based medicine means integrating individual clinical expertise with the best available clinical evidence from systematic research*". (Sackett et al., 1996, p.71).

Greenhalgh (2010, p.1) has defined the approach more precisely as

"*the use of mathematical estimates of the risk of benefit and harm, derived from high-quality research on population samples, to inform clinical decision-making in the diagnosis, investigation or management of individual patients*."

There are a number of elements in these definitions that warrant attention but it has probably been the identification of "current best evidence" or, more specifically, "mathematical estimates of the risk of benefit and harm" as the starting point of the process that has been especially influential on the development of thinking and policy in this area. Attempts to operationalise precisely what is meant by the phrase have led to the development of various hierarchies of evidence. Although these hierarchies are somewhat different from each other in their details they have largely converged upon the same approach to ranking evidence. The logic underpinning all of them is that the best evidence is garnered from approaches to data generation and evaluation that conform most closely to the standard hypothesis-testing approach of the natural sciences. This entails establishing a situation analogous to a controlled experiment in which the researcher manipulates one variable and determines its effect on another variable while holding constant or controlling for all other variables that might also affect the outcome. This has resulted in the Randomised Controlled Trial (RCT) being placed at or near the top of the hierarchy and only being supplanted by meta-analyses, in which the data from several such trials is combined and analysed or by systematic reviews in which evidence on a particular issue is searched for across a range of sources and evaluated

according to strict criteria. An example of such a hierarchy is that proposed by Guyatt et al (1995)

1. Systematic reviews and meta-analyses
2. Randomised controlled trials (RCT) with definitive results (confidence intervals that do not overlap the threshold clinically significant effect)
3. Randomised controlled trials with non-definitive results (a point estimate that suggests a clinically significant effect but with confidence intervals overlapping the threshold for this effect)
4. Cohort studies
5. Case-control studies
6. Cross sectional surveys
7. Case reports

Here we can see the standard model of ranking based on the premises of EBP in which RCTs and material based on them are at the top and data derived from other approaches is ranked lower. For many authors the RCT is the gold standard for acquiring reliable information and for offering us the best guidance of how to make decisions about treatment allocation (what treatment for which condition) and funding (which treatment(s) from a range of options should be paid for by states or insurance companies). For example, a recent report for the UK Government (Cabinet Office Behavioural Insights Team, 2012) states "*Randomised controlled trials (RCTs) are the best way of determining whether a policy is working. They have been used for over 60 years to compare the effectiveness of new medicines."* (p. 6).

However, the whole enterprise of EBM, and consequently of the central role in it granted to the RCT has been subject to vehement criticism. For example Miles (2009) has written:

" Practising holistic medicine will necessarily involve the abandonment of the core tenets of EBM. That medicine should be informed, and not dictated to, by its accumulated and accumulating science base demonstrates *the fundamental irreconcilability of EBM with*

good medicine, despite EBM's absurd and hubristic attempts to equate itself with good clinical practice as if the two were synonymous and coterminus" (p. 947).

It might seem strange that an approach that is explicitly committed to identifying and using the best available evidence of what works best for treating particular health problems has been subjected to criticism of any sort, let alone criticism that strikes at the very heart of the whole enterprise. It may seem odd that people can hold such diametrically opposed views about the approach and it may seem axiomatic that the only ethical course for practitioners is to offer their patients, clients and service users treatment for which there is good evidence that it will both help cure them and expose them to no or minimal risk or at least provide a favourable benefit: harm ratio. After all, who would want to receive a treatment for which the only available evidence that it worked was based on precedent, authority or anecdote and which was as likely to hurt as help them? In other words, why even ask the question of whether EBP, particularly the central place accorded to RCTs within the approach, raises ethical issues? Surely it would be unethical to practice in any other way? Indeed one of Cochrane's (1972) primary concerns was that too often medical care was based on interventions for which the evidence was poor and that there was therefore considerable risk of harm at both the individual and population level. He wanted to ensure that there should be equitable access to treatment for which there was good evidence that it worked.

In order to understand why it is even possible to speak of ethical concerns in relation to EBP it is necessary to understand the underlying assumptions of EBP, the procedures involved in generating the inputs to EBP and their limitations, the implicit values underpinning EBP and the ways in which particular sorts of evidence are given priority.

Epistemological assumptions of EBP

Epistemology is the branch of philosophy that is concerned with the nature of knowledge and the basis upon which we are entitled to make claims about truth. EBP is grounded in a realist epistemology within which we can come to have knowledge of entities and objects that exist independently of us through a process of observation and empirical manipulation of variables to isolate cause-effect relationships. This in itself is not especially contentious and simply places EBP within the tradition of empirical scientific research established in the 18th Century. There are alternative epistemologies to realism, such as critical realism and constructionism

(e.g. Bhaskar, 1975; Gergen and Gergen, 2003), but this is not the place to engage in a detailed analysis and comparison of the various conceptualisations. The important point to note from the perspective of critics of EBP is that the approach entails unarticulated value judgements about the nature of the phenomena being studied. This is not a problem for physical scientists, who have enjoyed great success by adopting the realist approach. Gravity, for example, does not have a moral status; it simply acts in a particular way on bodies. It is therefore possible to study the ways in which material entities are influenced by gravitational forces without committing to an ethical perspective on the matter.

Things are different when it comes to healthcare, however. Probably no one would seriously argue that a person is better with than without a cancerous tumour or an agonising and disabling headache, so studies on ways of eliminating tumours and reducing pain can more or less unproblematically adopt a realist position in which the issue is simply what treatment works best to achieve a desired end. The end itself can be defined in terms of a reduction in scores on certain parameters such as the size of the tumour or a reduction in pain scores. The issue is not always so straightforward though. This is apparent, for example, in the manner in which "disorders" appear and disappear from the Diagnostic and Statistical Manual (DSM) of the American Psychiatric Association. Homosexuality was removed from the second edition of the DSM in 1972, but up to then it would have been perfectly legitimate to develop evidence based guidelines for the treatment of homosexuality. The situation of people who hear voices is also instructive in this regard. Hearing voices is generally considered a symptom of psychosis and is treated, often with medication or cognitive therapy, with the aim of reducing or eliminating the symptom. However, many people who hear voices simply want to be able to live with and manage them. This has led to the establishment of groups such as the Hearing Voices Network in the UK. The point here is that for many healthcare professionals hearing voices is a problem that needs to be solved while for voice-hearers it is simply an aspect of themselves and their identity that they wish to understand and manage. Hence, we must be mindful of the terms in which the "problem" is constructed. Healthcare professionals construct it in one way, leading to the selection of a particular outcome for measurement, while people who hear voices have a different construction and, consequently, a different desired outcome. Of course, healthcare professionals are the ones who are primarily involved in determining what constitutes the hierarchy of evidence, so in this

instance the views and perspectives of one group (healthcare professionals) are given priority over the views of others (the "patients") when it comes to developing treatment guidelines, resulting in the search for particular types of evidence. At the core of this argument is the status of the concept of evidence. Essentially, evidence is for or against some proposition, so in asking about the evidence base for something we are implicitly choosing to value one thing above another. As Kerridge (2010) has written "Evidence-based medicine ... has and confers both epistemic and moral authority (p.365). This consideration leads directly to discussion of another ethical issue related to EBP - whose interests and values does it reflect?

Whose values and interests are served by EBP?

Marks (2009) has argued that 'In medicine and health care there is a large and increasing gap between what gets measured and what matters most to clients and patients' (p. 476) and Cornish and Gillespie (2009) have written:

"*RCTs are particularly suitable for determining which of a limited number of interventions is most effective at producing a pre-determined health outcome within a specific stable context. They answer to scientific interests in comparing the effects of different pharmacological treatments, or other clearly defined interventions, and to health professionals' interests in choosing between treatments. RCTs are excellent means of achieving these particular ends, but these are not the only ends that may be served by health research. Indeed, to place RCTs at the top of the hierarchy may be to prioritize certain interests, and thus, an exercise of power, rather than a reflection of an objective hierarchy among methods*" (p. 803).

The interests served by placing RCTs and their products at the top of the hierarchy are arguably those of the people whose job it is to prioritise and fund health treatments. Even a well-designed and definitive RCT will only show that on average a particular treatment does better than no treatment, an alternative or a placebo but it will not allow determination of whether a particular treatment will work for a particular patient in a particular setting. Indeed, this is why some authors prefer to speak of "epidemiology-based practice".

As Kerridge (2010), among others, has pointed out, a hierarchy that prioritises the needs and values of providers and funders can be used to justify restriction of expenditure and patient

choice and to restrict the options open to clinicians, thus limiting the autonomy of both groups. This is a potential concern both in systems in which healthcare is funded from the public purse, as in the UK, and in which healthcare is largely paid for through insurance schemes, as in the USA. In the UK the National Institute for Health and Clinical Excellence (NICE) was established in 1999 to *"reduce variation in the availability and quality of NHS treatments and care*" (NICE (a), 2013). Their approach to doing this is described by NICE in these terms:

"*NICE guidance supports healthcare professionals and others to make sure that the care they provide is of the best possible quality and offers the best value for money.* " (NICE (b), 2013).

In relation to developing clinical guidelines the process is initiated by means of a referral from the Department of Health. A guideline development group is then established, which assesses the available evidence and makes recommendations. It is worth noting here that the initiative comes from a branch of government and part of the agenda is to identify interventions that offer the best value for money. Now, there is nothing inherently wrong with wanting value for money, indeed taxpayers (and those contributing to insurance schemes) would probably demand it. Nevertheless, the requirement that value for money be an important consideration in making recommendations regarding treatment creates a tension from the outset between what might be best for the individual (and would be preferred by them and by those treating them) and what the state or insurance company is willing to fund. Indeed, there have been a number of cases in the UK in which individuals and groups have challenged NICE guidance that denied them treatment that they or their clinicians preferred. For example there have been challenges to restrictions on cholinesterase inhibitor treatment for Alzheimer's dementia (Sellers and Easey, 2008) and an (unsuccessful) challenge to the guidelines for Chronic Fatigue Syndrome (Dyer, 2009).

Another problem with prioritising particular interests above others is that an exclusive focus on the scientific understanding and treatment of a disease can lead " directly to the assumption that what is right for the disease is automatically right for the patient, representing a fundamental misunderstanding of the relationship between the partial nature of the disease and the totality of the person" (Miles 2009, p.944).

What is the "evidence" in EBP?

A central assumption underpinning EBP is that evidence of what has proven to be effective in well-conducted studies conducted elsewhere can be sensibly and meaningfully applied to similar problems encountered by the evidence-base practitioner wherever they happen to be working. This transferability of findings is normally assumed to be guaranteed by the methodological architecture of RCTs, that is random selection and allocation of participants, control of confounding variables, use of control groups, double- or triple-blinding etc. These procedures enable the researcher to claim that the observed effect (the reduction in tumour size, the reduction in pain scores, the reduction in depression scores etc.) is due to the "cause" embodied in the particular intervention being evaluated (perhaps a new cancer drug, a physiotherapy intervention or cognitive behavioural therapy).

Cartwright and Hardie (2012) provide an extensive and detailed analysis and critique of the assumptions underpinning and the processes involved in the move from (in their terminology) "It worked there" to "It will work here". Essentially they are concerned with what warrants the claim for such transferability and this is at least partly based in the distinction between efficacy and effectiveness. "Efficacy" is what is demonstrated in a standard RCT in which there is a positive outcome, i.e. the intervention was successful in some sense. "Effectiveness" is whether the intervention will work in the world outside the constraints of the RCT. As Cartwright and Hardie (2012) put it "No matter how much gold standard evidence you have that "it worked there", you cannot pave the road from there to here with gold bricks" (p.8). Now this may not appear to be an ethical issue (beyond the usual ethical concerns regarding RCTs such as informed consent etc.) but given the implicit ethical injunction entailed in EBP it is certainly important to examine the basis upon which the claims underpinning it are made. Cartwright and Hardie (2012) argue that in order to have confidence that an intervention or policy will work in a particular context we must find evidence that gives us reason to believe that the intervention played a causal role in the situation in which it was tested (the RCT), we must be able to identify the support factors that enabled its success and we need evidence that these support factors actually apply in our particular context. Essentially, support factors are factors that must be present in the environment in which the trial was conducted and without which the intervention will not work. They give the example of a study designed to test the impact of homework on reading test scores. In order for this intervention to work it needs the support of a host of other

factors, including student motivation, student ability, supportive family, study space, consistent lessons and work feedback. Without these it would not work and if we wish to use it as an intervention in our setting we must have good reason to believe that these factors will be present here too. This may seem obvious but it is easy to ignore it and Cartwright and Hardie give examples of a policy that worked well in one context failing in other because such factors were not attended to. It takes a lot of work to move from having evidence that something worked somewhere to being confident that it will work in another context.

How complete is the evidence base?

There is a growing concern with the way in which the involvement of commercial interests in researching interventions can systematically distort the evidence base. A particular concern is the involvement of multi-national pharmaceutical companies in the development, testing and marketing of new drugs. Such companies usually do the initial development of new drugs themselves but they fund academic and clinical researchers to conduct the all-important clinical trials. A number of recent authors (e.g. Goldacre, 2013; Moncrief, 2013) have expressed concern about the way in which this close involvement of commercial interests in clinical evaluations can distort the findings of trials in a manner favourable to the companies and their products. Particular concerns arise in relation to industry-sponsored trials, where the sponsors (drug companies) often own the data and the academics who ostensibly conducted the trial, analysed the data and whose names appear on the paper have limited access to the raw data from the trial (Lundh, Krogsbøll and Gøtzsche, 2011).

A more fundamental concern is the role of drug companies in identifying new types of disorder in order to create a new market for their products. A fairly recent example of this is the case of female sexual dysfunction, which has been described as “a textbook case of disease mongering by the pharmaceutical industry and by other agents of medicalization, such as health and science journalists, healthcare professionals, public relations and advertising firms, contract research organizations, and others in the “medicalization industry". (Tiefer 2006, p.0436). Essentially the argument is that following the development of Viagra and its success in treating erectile dysfunction in men the drug companies sought to

extend the market to women. In order to achieve this it became necessary to have a disorder in women that could be treated with Viagra. This depended on collaboration between the industry and academic researchers that aimed to normalize certain patterns of female sexual arousal and performance and, in the process, pathologise others. Hence, female sexuality was regulated in order to provide a market for the medicine.

Conclusions

The intention here has not been to demonize EBP, RCTs, hierarchies of evidence and the people involved in advocating and researching these topics. Nor has it been to construct a "straw person" in respect to the enterprise of EBP. Indeed, practitioners of EBP have been quick to respond to criticism of the approach and modify and adapt it accordingly. So much so, in fact, that they may be at risk of endangering its scientific status by the introduction of ad hoc auxiliary assumptions or re-interpreting it (cf Popper, 1989). Rather the aim has been to examine some of the complexities, challenges and indeed contradictions inherent in the approach and to explore some of the implications for the ethical practice of healthcare in its many forms.

Reflective questions

1. Is it possible to take patient/service user perspectives into account when making decisions regarding the provision of healthcare services? How might this be done?

2. Is it ethical to ration the provision of services based on cost?

3. If we accept that cost-based rationing is inevitable how can we take account of the values and priorities of those using the services?

4. In what circumstances, if ever, would it be ethically justifiable for which the evidence base was weak or absent?

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