

TESTING TREATMENTS

Chapter 9, 9.1 TESTS OF TREATMENTS: HELP OR HINDRANCE?

public that researchers cannot simply do as they like.

Research is subject to many other forms of regulation. Laws specific to research exist in most countries. All countries in the European Union, for example, must comply with the Clinical Trials Directive, which lays out the requirements in relation to so-called 'clinical trials of medicinal products' – essentially this means drug trials. Several countries also operate regulatory systems that affect all or most types of research in healthcare. Many other laws can potentially affect research, even though they were not designed with research as their primary purpose. For example, data protection laws, intended to protect the confidentiality of people's personal data, apply, in many countries, to medical research. A range of different agencies is also usually involved in regulating research in most countries.

The conduct of research is also governed by professional codes of practice and by international statements. Doctors and nurses, for example, are bound by the codes of practice of their professional bodies, and can risk losing their registration or having other sanctions applied if they violate these codes. And international statements, such as the World Medical Association Declaration of Helsinki, are often highly influential in setting standards even when they have no legal force.

DO REGULATORY SYSTEMS FOR TESTING TREATMENTS GET IT RIGHT?

Although the level of regulation can be reassuring, current regulatory systems impose very onerous burdens on anyone wishing to study a poorly evaluated treatment rather than offer it to patients in normal clinical practice. In many countries, the sheer complexity of the system – involving laws, agencies, codes of practice, and so on – is overwhelming and time-consuming. Researchers may need to get multiple approvals from different places, and sometimes have to face resultant contradictory requirements.

Moreover, taken as a whole, the system can seriously discourage and delay the collection of information that would

IN AN IDEAL WORLD

'In an ideal world, wherever possible, we could be gathering anonymised outcome data and comparing this against medication history, making exceptions only for those who put their anxieties about privacy above the lives of others . . . In an ideal world, wherever a patient is given any treatment, and there is genuine uncertainty about which treatment is best, they would be simply and efficiently randomised to one treatment, and their progress monitored. In an ideal world, these notions would be so routinely embedded in our notion of what healthcare looks like that no patient would be bothered by it.'

Goldacre B. Pharmaco-epidemiology would be fascinating enough even if society didn't manage it really really badly. *The Guardian*, 17 July 2010. Available online: www.badsience.net/2010/07/pharmaco-epidemiology-would-be-fascinating-enough-even-if-society-didnt-manage-it-really-really-badly

make healthcare safer for everyone. For example, data protection laws and codes of practice on confidentiality, although introduced with the best of intentions, have made it extremely difficult for researchers to collect routine data from medical records that may help to pinpoint treatment side-effects. And for researchers planning clinical trials, it can take several years to get from a trial idea to recruiting the first patient, and even then recruitment to trials can be slowed by regulatory requirements. But while researchers try to get studies through the system, people suffer unnecessarily and lives are being lost.

In practice, what this means is that clinicians can give unproven treatments to patients, as long as patients consent, if therapies are given within the context of 'routine' clinical practice. By contrast, conducting any study of the same treatments to evaluate them properly would involve going through the protracted regulatory process. So clinicians are discouraged from assessing treatments fairly, and instead can continue to prescribe treatments without committing to

BIASED ETHICS

'If a clinician tries a new therapy with the idea of studying it carefully, evaluating outcomes, and publishing the results, he or she is doing research. The subjects [sic] of such research are thought to be in need of special protection. The protocol must be reviewed by an Institutional Review Board (IRB) [equivalent to a research ethics committee in Europe]. The informed consent form will be carefully scrutinised and the research may be forbidden. On the other hand, a clinician may try this new therapy without any intention of studying it, merely because he believes it will benefit his patients. In that situation, trying the new therapy is not research, the trial does not need IRB approval, and consent may be obtained in a manner governed only by the risk of malpractice litigation.

It would seem that the patients in the second situation (non research) are at much higher risk than are the patients in the first situation (being part of formal clinical research). Furthermore, the physician in the first situation seems more ethically admirable. The physician in the first situation is evaluating the therapy, whereas the physician in the second situation is using the therapy based on his or her imperfect hunches. Nevertheless, because ethical codes that seek to protect patients focus on the goal of creating generalizable knowledge, they regulate the responsible investigator but not the irresponsible adventurer.'

Lantos J. Ethical issues – how can we distinguish clinical research from innovative therapy? *American Journal of Pediatric Hematology/Oncology* 1994;16:72-75.

addressing any uncertainty about them (see Chapter 5).

The regulatory system for research, in its preoccupation with risk and protecting potential research participants, has become over-protective and overlooks the fact that patients and the public are increasingly involved as partners in the research process (see Chapter 11). However, there is one encouraging note. Research regulators are beginning to acknowledge that

the ‘one-size-fits-all’ approach to research ethics review may be unnecessarily burdensome.¹ In the UK, for example, procedures for ‘proportionate review’ are now being evaluated to see whether a simplified and swifter review process can be safely used for research studies that do not raise any material ethical issues.

INFORMATION AND CONSENT

Requirements relating to provision of information and consent for studies are one of the ways in which the regulatory system acts to discourage rather than encourage research to address uncertainties about treatments. It is important – and ethical – to consider the interests of *everyone* currently receiving treatment, not just the few who participate in controlled trials.² The standard for informed consent to treatment should therefore be the same whether people are being offered treatment within or outside the context of formal treatment assessments. To come to a decision that accords with their values and preferences, patients should have as much information as they want, and at a time that they want it.

When treatment is being offered or prescribed in day-to-day practice, it is accepted that people may have different individual preferences and requirements, which may change over time. It is also recognized that people may vary not only in the amount or type of information they want, but also in their ability to understand all the information in the time available, and in their degree of anxiety

RETHINKING INFORMED CONSENT

[Some] have come to suspect that informed consent is not fundamental to good biomedical practice, and . . . attempts to make it so are neither necessary nor achievable. We hope that the juggernaut of informed consent requirements that has been constructed across the last fifty years will be reformed and reduced within a far shorter period.’

Manson NC, O’Neill O. *Rethinking informed consent in bioethics*. Cambridge: Cambridge University Press, 2007, p200.