Why do doctors use treatments that do not work?

For many reasons—including their inability to stand idle and do nothing

One of the surprising things about James Lind's celebrated trial of citrus fruit for scurvy was not just that he ignored the evidence from his own trial but that in clinical practice he continued to advocate treatments that he himself had found ineffective, including those containing sulphuric acid.[w1] The history of medicine is replete with examples of treatments once common practice but now known not to work—or worse, cause harm. Only because the French surgeon Paré ran out of boiling oil did he discover that not cauterising gun shot wounds with it created much less pain and suffering.[w2] Leeches and blood letting were used for thousands of years for almost everything. Attempts to show that they were ineffective were resisted with great passion by the medical profession.[w3] More recently, we have had treatment with insulin for schizophrenia and vitamin K for myocardial infarction.[1 2] In case we are all feeling too smug about silliness in the bad old days, we have the recent crisis on finding that hormone replacement therapy does not prevent cardiovascular disease.[3] Why do we still use ineffective treatments?

One reason is that our expectations for the benefits of treatment are too high. As Voltaire said, "The art of medicine consists in amusing the patient while nature cures the disease." Or, in modern parlance: most drugs work in only 30% or 50% of people.[4] Because patients so often get better or worse on their own, no matter what we do, clinical experience is a poor judge of what does and does not work. Hence the need for adequately powered randomised controlled trials.

A second reason is we are taught that because medicine is based on the sciences, understanding the pathophysiology of disease is essential to effective treatment. And so it is for many treatments. Use of insulin for diabetic coma needs a full understanding of the pathophysiology. Similarly, our appreciation of how parachutes slow falls means we do not need a placebo controlled trial of parachutes.[5] But we have many examples where this approach, without empirical testing, is wrong. Until recently, medical students were taught the pathophysiological reasons why β blockers are contraindicated in heart failure (they are a good treatment for heart failure); why colloid is more effective than crystalloid for fluid replacement (it is worse); and that because the vascular supply of the scaphoid places it at risk of non-union, any suspected fracture requires a cast (active mobilisation results in better outcomes).[6 7] Lind's belief in the humoral basis of disease caused his resistance to his own trial evidence, and the medical profession to reject Louis's data on blood letting.[w4]

Even when empiricism is satisfied we can be misled by looking at the wrong outcome. Fluoride increases bone density. But it also increases the fracture rate.[8] Flecainide for the treatment of supraventricular tachycardia makes the electrocardiogram look normal, but only after clinical trials (that some thought unethical) did it emerge that it increases mortality.[9]

Some treatments have harms that outweigh their benefits and are not evident in trials. It was only after licensing in the United States and postmarketing surveillance that troglitazone was found to cause liver failure and had to be withdrawn.[w5]

Reasons for using ineffective or harmful treatments

- Clinical experience
- Over-reliance on a surrogate outcome
- Natural history of the illness
- Love of the pathophysiological model (that is wrong)
- Ritual and mystique
- A need to do something
- No one asks the question
- Patients' expectations (real or assumed)

Let us not stop at ineffective treatments. Much of the clinical examination and diagnostic testing is more of a ritual than diagnostically useful. We continue to order routine blood tests before surgery without controlled trials to show benefit, and several case series that show that these tests rarely change outcomes or even management.[10] Alternatively, what was once perhaps useful is now superseded by better investigation. When did whispering pectoriloquy last clinch a diagnosis of pneumonia?

Clinicians want to relieve suffering. We find it difficult to do nothing (the aphorism "Don't just do something, stand there!" seems ludicrous). So we send in the counselling teams after psychological trauma, probably making things worse.[11] Perhaps it is societal opinion (for which one ear of the medical profession is always pricked) that errors of omission are more reprehensible than errors of commission that is at fault. Is missing a rare diagnosis so much worse than harm from over-testing?[12]

What hope is there for not using treatments and tests that don't work? Medicine is not just a science—it is a human activity. It entails ritual, custom, and the expectations of doctors, patients, and society. To safeguard against ineffective or harmful health care we need doctors who want to do the best they can for their patients, who are willing to continually question their own managements, and who have readily available sources of information about what does work.

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Competing interests: None declared.

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Additional references w1-w5 are on bmj.com

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Well informed uncertainties about the effects of treatments

How should clinicians and patients respond?

Uncertainties about the effects of treatments are inevitable. Whatever the basis for judgments about the likely effects of treatments in individual patients, there is no escape from the reality that every such judgment initiates a clinical trial in which there can be no certainty that an individual patient will benefit. Sometimes the judgment will draw on the patient's past experience of the treatment, more usually on the clinician's experience of treating other patients. Increasingly, clinicians and patients are taking account of collective experience—the results of formal evaluations of treatments.[1] Maybe this is because they recognise that treatments can sometimes do more harm than good, sometimes on a devastating scale.

What should happen if, after weighing the best available evidence from collective experience and taking account of patients' preferences, residual uncertainty remains about which treatment options should be chosen? Should the clinician and patient simply press ahead with yet another poorly controlled clinical trial? It is surprising that such questions seem to have been addressed relatively rarely. One attempt to do so was published in this journal three years ago by a medical ethicist. "If we are uncertain about the relative intrinsic merits of [different] treatments," he wrote, "then we cannot be certain about those merits in any given use of one of them—as in treating an individual patient. So it seems irrational and unethical to insist one way or another before the completion of a suitable trial. Thus the answer to the question, "What is the best treatment for the patient?" is: "The trial." The trial is the treatment. Is this experimentation? Yes. But all we mean by that is choice under uncertainty, plus data collection. Does it matter that the choice is "random"? Logically, no. After all, what better mechanism is there for choice under uncertainty?"[2]

This approach to dealing with uncertainty is reflected in some of the guidance issued by the National Institute for Clinical Excellence, and it is implicit in the NHS Plan, which calls for a doubling in the numbers of cancer patients participating in clinical trials.[3] The dividends that result from adopting this response to uncertainty can be substantial: gradual and important improvements in the prognosis of children with leukaemia, for example, seem likely to reflect an expectation among paediatric oncologists that decisions about treatment should be taken within the context of controlled trials, so that uncertainties can be addressed and reduced.

Strategies for dealing with uncertainty need to be considered and debated more explicitly. For example, what does the "quality in health care" movement have to say? Has it given sufficient attention to the responsibilities of clinicians and health service managers to reduce uncertainties about the relative merits of different treatments, and thus improve the quality and cost effectiveness of services? What are the responsibilities of clinicians and managers implementing the clinical governance framework in the NHS? Should clinicians and institutions be held accountable for failing to address uncertainties systematically, as some have suggested they should be?[4] Are strategies for dealing with uncertainty being addressed in medical schools, and by professional organisations such as the medical royal colleges, encouraging clinicians to be more open with patients about the limitations of treatments and their potential for harm? And are organisations that endeavour to represent the interests of the public—the Consumers' Association, patients' groups, and the General Medical Council, for example—taking a sufficiently active role in promoting discussion about how people should respond to well informed uncertainties about treatment choices?

As another medical ethicist has noted, "Doctors must make many practical decisions, often on the basis of inadequate information. Too finely developed a critical faculty, endeavouring disinterestedly to learn the best that may be known and thought, may positively inhibit the ability to make such decisions."[5] But there is surely scope for dealing with inadequate information in ways that can help to identify really important uncertainties, uncertainties that are often reflected in dramatic variations in clinical practice and which cry out for coordinated efforts to improve

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