

TESTING TREATMENTS

Chapter 5, 5.3.1

PROFESSIONALISM AND UNCERTAINTY ABOUT THE EFFECTS OF TREATMENTS

ADDRESSING UNCERTAINTY IS PROFESSIONAL

'One of the key attributes of professionalism . . . should be the ability to identify and address uncertainty in medicine. Every day professionals confront and cope with uncertainties about disease pathogenesis, about diagnosis, and about treatment. Yet the intrinsic uncertainties in all these spheres of medical activity are seldom acknowledged explicitly and some professionals remain uncomfortable about admissions of uncertainty – in their dealings with patients especially. Uncertainty is also a prime stimulus for medical research to improve human health, which is central to the MRC's mission. In the future it will be increasingly important for medical professionals to take on board the results of accumulated research findings relevant to their area of practice so that they are aware where continuing uncertainties exist and what research is ongoing or needed to address these. Overall, a mark of professionalism for the future will be research awareness for the benefit of patients. Some medical professionals will actively participate in research but all should seek to encourage it and, where appropriate, to involve their patients actively in the medical research agenda, and implement the results of this research in their professional practice.'

From: Medical Research Council response to Royal College of Physicians consultation on medical professionalism. 2005

Caffeine for breathing problems in premature babies

Large variations in the treatments used for a particular condition provide clear evidence of professional uncertainty about the relative merits of different treatments. And entrenched practices may mean that it takes a very long time for such uncertainties to be addressed by fair tests. The use of caffeine in premature babies provides a telling example. Such babies often have trouble breathing properly and sometimes stop breathing very briefly – this condition is known as apnoea of prematurity and affects most

babies born at less than 34 weeks' gestation. In the late 1970s, caffeine treatment was shown to reduce these episodes and then became used by some paediatricians.

However the effects of caffeine remained disputed. Although fair tests had shown that caffeine reduced the episodes of apnoea, many paediatricians did not think that the episodes were sufficiently serious to justify use of the drug, and some were concerned that it might not be safe in these tiny babies. This meant that some babies were given the treatment and others weren't. When these widespread uncertainties were finally assessed by a large international study more than 30 years after the treatment had been introduced, it turned out that this simple therapy not only reduces the breathing difficulties but also, and very importantly, significantly improves the likelihood of long-term survival without cerebral palsy and delay in infant development. Had this uncertainty been addressed when the treatment was introduced, fewer babies would have gone on to develop disabilities.^{15, 16}

Antibiotics in pre-term labour
 Fair tests of treatments with hoped-for beneficial effects, and which are assumed to be harmless, can show that neither is true. Doctors prescribe treatments with the best of intentions, particularly when they may offer hope in a desperate situation. For example, a theory suggested that 'silent' (sub-clinical) infection might trigger early labour and preterm delivery. The theory led doctors to prescribe antibiotics for some pregnant women in the hope that this might help to prolong pregnancy. No one seriously thought that using antibiotics in this way would cause any serious problems. Indeed, there is some evidence that women themselves were keen to have antibiotics – in a spirit of 'let's try this; it can't do any harm'.

When a fair test of this treatment was eventually done, the results had clear clinical implications. For a start, no benefits were identified. On top of that, long-term follow-up of the babies in the study showed that those who had been exposed to antibiotics were more likely than those in the comparison groups to have cerebral palsy and problems with speech, vision, and walking. These risks of antibiotics had remained unrecognized over the decades that