Practice guidelines and practical judgement: the role of mega-trials, meta-analysis and consensus

LARGE randomized controlled trials (mega-trials), meta-analysis and explicit techniques of consensus development are all relatively new to medicine, and many doctors seem uncertain quite what to make of them. The techniques are aspects of an intellectual movement which has the avowed aims of reducing harmful or expensive variations in clinical practice, and encouraging the rapid dissemination of useful innovations.[1]

One area in which mega-trials, meta-analysis and consensus techniques are brought together is in devising clinical guidelines — also known as standards, protocols, algorithms and statements. At present guidelines are something of a growth industry; whether or not they have value depends upon how they are constructed and implemented.

The most objective method for establishing best medical practice is the randomized controlled trial. The randomized controlled trial is regarded as a 'gold standard' for effectiveness studies owing to its lack of systematic bias.[2,3] As medicine has progressed, the need for discriminating ever smaller differences between treatments requires the statistical power obtained by studying ever larger numbers of patients. This has led to the mega-trial: a big, multi-centred, usually multinational randomized controlled trial, which is as simple as possible, and designed to compare the effectiveness of rival therapeutic protocols.[4,5] The protocol which performs 'best' in the trial (usually the most effective, safest, cheapest or most cost effective) may be proposed as a clinical guideline.

Large, simple randomized controlled trials provide information unobtainable in any other way.[4,6] However, mega-trials are expensive, time consuming and logistically difficult, so that they are often left behind by innovations. The research situation is not always representative of clinical practice, and impersonally applying the 'best protocol' to patients may be less effective than personally tailored management, because the former approach fails to take account of individual expertise and the potential of the placebo effect.[6] Mega-trials should be interpreted in the context of available scientific knowledge, including smaller, more tightly controlled 'explanatory' trials designed to test hypotheses rather than compare protocols. Individual patients may then be treated in accordance with the best validated hypothesis.

If therapeutic trials are regarded as providing the empirical data for clinical practice, there still remains the difficulty of bringing together this information and making sense of it. This is where meta-analysis comes in.[7] Meta-analysis is a development of the expert review article or editorial. It is a systematic overview, using explicit criteria for assembling a comprehensive survey of the relevant literature in order to answer a predetermined question. There is a process of critical evaluation, for instance ranking the studies in order of scientific quality and according each a weighting on this basis. Finally meta-analysis may employ a variety of statistical techniques to combine or summarize the results. By such pooling of information the meta-analysis may yield more information than any one of the individual studies from which it has been synthesized. The ability to create statistical significance by combining several small randomized controlled trials into one large hypothetical 'meta'-study must, however, be treated with caution for scientific purposes, as the procedure is subject to various forms of statistical bias.[8]

Meta-analysis is best seen as a response to scientific uncertainty and an aid to decision making. It is salutary to remember that meta-analysis is specific to medicine and the social sciences, and is not a part of the 'hard' biological sciences such as physiology and molecular biology. Meta-analysis has developed because of the limitations of medical research, and because medicine is a practical profession where decisions must be made even where there is scientific uncertainty. Meta-analysis is not an empirical science, instead it is a method of interpreting the results of empirical science, akin to the opinion of an expert. Compared with an expert and authoritative opinion the meta-analysis offers gains in objectivity and explicitness, but a loss in practical judgement (meta-analysis is usually done by statisticians, health economists, epidemiologists and the like; while practical judgement is developed only by experienced clinicians).

In some situations where there is insufficient evidence for meta-analysis to draw clear conclusions, guidelines may nevertheless be considered necessary or desirable, and they can be developed by techniques of professional consensus.[1,9,10] In the past, medical consensus was largely implicit and spontaneous, nowadays it is increasingly explicit and formally derived. Furthermore, although professional consensus should take into account the results of available randomized controlled trials, and incorporate previous attempts at review and meta-analysis, it can move beyond science to address issues such as implementation.[9,10]

Russell and colleagues have defined three principal methods of consensus development: peer review, Delphi techniques, and consensus conferences.[9] Peer review comprises small group discussions. Groups can be top-down (external), composed of experts and opinion leaders; bottom-up (internal), composed of those who will implement the conclusions; and intermediate forms of the two. Delphi techniques involve repeated cycles of postal questionnaires analysed and fed back to expert panels until stable agreement is reached. Postal (or electronic mail) questionnaires have the advantage of being cheap, and are intended to give equal weight to all participants. Consensus conferences are usually large gatherings of experts and opinion leaders who meet with the intention of producing a statement after a period of evaluation and debate. A distinctive feature of modern consensus is that both its methods and its conclusions are explicit, and therefore available for internal audit and/or external accountability.

However, consensus methods can be perverted into a mask for professional self-interest, a disguise for intra-professional power struggles (where rival factions attempt to impose protocols on each other), or may be hijacked by outside interest groups.[11,12] A great deal depends upon who initiates the consensus process and why, the methods they use, and how the guidelines are interpreted. No matter how structured and explicit the technique, at some point an 'arbitrary' decision must be made as to appropriate methods for consensus generation — including the vital question of who should be asked to participate.

Mega-trials, meta-analysis and consensus have different purposes and are useful in different ways. They form a spectrum from the narrowly specialized but objective randomized controlled trial to the broadly applicable but subjectively-generated consensus, with meta-analysis somewhere in between and feeding both science and the consensus process. The three techniques move from science, through decision to practice, and are thus complementary not competitive.[13] These modern approaches, used appropriately,

should inform clinical judgment; they cannot dictate practice. The individual clinician should be practising in a way which takes into account properly developed guidelines, just as he or she would take into account the opinions of respected colleagues. However, guidelines do not remove the need for judgement.

Guidelines are inevitably abbreviations and abstractions secondarily derived from actual practice: explicit but crude summaries of implicit and subtle skills.[14] Where complex human activities are concerned, explicit is equivalent to incomplete. What is left out is that tacit knowledge which makes a difference to practice, but which cannot be written down or captured in prescriptive form because it is inaccessible to reflection.[15] Tacit knowledge is what makes the difference between inexperience and experience, between learning by apprenticeship and learning from a book.[16] At best guidelines can merely allow space for the exercise of judgement. As James McCormick has said, judgement should be based upon knowledge, tempered by scepticism, and enriched by experience of medicine and familiarity with the patient.[17]

As the costs of health care rise, so will the demand to regulate medical decisions, and a proliferation of guidelines seems inevitable (NHS Management Executive. Improving clinical effectiveness, 1993).[18,19] A battle between clinicians and various non-clinicians (such as public health doctors, managers, health economists, medical sociologists, insurance companies, pressure groups and politicians) for control of clinical guidelines may be anticipated, as each group has rather different objectives. In such circumstances, the limitations of guidelines may be forgotten. Guidelines can be generated and will function, only against a background of internalized (tacit) professional values and standards developed through the wise exercise of clinical autonomy.[20] Good guidelines depend upon pre-existing good practice; guidelines are not the cause of good practice.

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Nurse practitioners in primary care: here to stay?

OVER the past decade there has been a steady expansion in the number of nurses practising as nurse practitioners. Inconsistencies and ambiguities in the literature relating to the nurse practitioner have led to a debate regarding the definition of the term.[1,2] In essence, it is a form of advanced nursing practice legitimized by a specific training programme. This formal training for those who aspire to the nurse practitioner role has been pioneered in the United Kingdom by the Royal College of Nursing (Institute of Advanced Nurse Education). This is a course at diploma (and soon degree) level validated by the English National Board for Nursing, Midwifery and Health Visiting and accredited by the University of Manchester.

The evolution of nursing practice has led to the inclusion of skills not previously encompassed within a nursing role. This role expansion was in the past intended to complement and supplement the physician's care.[3] The nurse practitioner is a provider of health care whose responsibilities cross the traditional boundaries between nursing and medicine.[4]

Nurse practitioners have developed in the UK in two key areas; as members of primary health care teams, and as specialists in the care of specific groups such as homeless people, mentally ill people and children.[1] It is perhaps no coincidence that nurse practitioners have emerged in areas where there has been a paucity of medical services, and in many cases with the cooperation of a member of the medical profession.

The introduction of target payments and performance-related pay into the primary health care arena has led to general practitioners reviewing their methods of working in order to accommodate new clinical sessions as well as their traditional surgery and home visiting commitments. The changes envisaged under both the new contract for general practitioners and the 1990 National Health Service act have necessitated general practitioners delegating a greater proportion of their workload to other workers. The primary health care team, Stott suggests, will of necessity be expanded to cope with these increasing demands.[5] This is one of the main contexts within which the nurse practitioner is likely to come of age. The rise in the number of practice nurses has facilitated this process and indirectly unlocked the potential for an advanced nursing role.

The emergence of the nurse practitioner has not been accom-