Quantitative Clinical Rheumatology

Shouldn’t Standard Rheumatology Clinical Care Be Evidence-Based Rather Than Eminence-Based, Eloquence-Based, or Elegance-Based?

Evidence-based medicine is increasingly accepted as the gold standard for medical care, advocated to replace traditional approaches, including eminence-based medicine, characterized by making the same mistakes with increasing confidence over an impressive number of years, eloquence-based medicine, in which brilliant oratory and a year-round suntan may overcome absence of any supporting data, and elegance-based medicine, in which the sartorial splendor of a silk-suited sycophant substitutes for substance (Table 1, adapted2).

Many health professionals, including students, trainees, and senior physicians, often use the term “evidence-based medicine” almost as a synonym for data from randomized controlled clinical trials3,4. A widely-used hierarchy concerning levels of evidence for patient care lists randomized clinical trials and metaanalyses as the highest forms of evidence, while observational studies and case reports are regarded as poorer forms of evidence5 (Table 2). However, much evidence to guide the clinician must be derived from sources beyond randomized controlled clinical trials1,6,7.

The clinical trial remains the gold standard to assess the efficacy of an active treatment versus a control treatment over defined periods. However, evidence-based medicine is not restricted to randomized clinical trials and metaanalyses as the highest forms of evidence, while observational studies and case reports are regarded as poorer forms of evidence5 (Table 2). However, much evidence to guide the clinician must be derived from sources beyond randomized controlled clinical trials1,6,7.

Table 1. Evidence-based medicine and its alternatives. Adapted with permission from Isaacs and Fitzgerald. BMJ 1999;319:16182.

| Evidence Based Medicine — The best approach to clinical knowledge. Requires both clinical trials and clinical observations outside of clinical trials. |
| Traditional approaches to clinical expertise: |
| Eminence Based Medicine — Making the same mistakes with increasing confidence over an impressive number of years. |
| Eloquence Based Medicine — Brilliant oratory and a year-round suntan may overcome absence of any supporting data. |
| Elegance Based Medicine — Where the sartorial splendor of a silk-suited sycophant substitutes for substance. |

Table 2. Grades of evidence for the purported quality of study design. Adapted with permission from Guide to Clinical Prevention Services. Williams and Wilkins; 19965.

| I | Evidence obtained from at least one properly randomized, controlled trial. |
| II-1 | Evidence obtained from well-designed controlled trials without randomization. |
| II-2 | Evidence obtained from well-designed cohort or case-control analytic studies, preferably from more than one center or research group. |
| II-3 | Evidence obtained from multiple time series with or without the intervention. Dramatic results in uncontrolled experiments (such as the results of the introduction of penicillin treatment in the 1940s) could also be regarded as this type of evidence. |
| III | Opinions of respected authorities, based on clinical experience; descriptive studies and case reports; or reports of expert committees. |

A need for breadth in the approach to evidence-based medicine is especially important in chronic diseases3,4,12-44. For example, the therapeutic pyramid was developed as a framework for rheumatologists to care for patients with rheumatoid arthritis (RA)45, based on short term clinical trial results indicating efficacy of nonsteroidal antiinflammatory drugs (NSAID) and disease modifying antirheumatic drugs (DMARD). However, the rheumatology community had to reassess this approach when longer term longitudinal observational analyses of patients, treated according to the recommended therapeutic pyramid, indicated that the longer term out-

Table 3. A comprehensive view of evidence-based medicine. Adapted with permission from Glasziou and Vandenbroucke. BMJ 2004;328:39-419.

| 1 | Different types of research are needed to answer different types of clinical questions. |
| 2 | Irrespective of the type of research, systematic reviews are necessary. |
| 3 | Adequate grading of quality of evidence goes beyond the categorization of research design. |
| 4 | Risk-benefit assessments should draw on a variety of types of research. |
| 5 | Clinicians need efficient search strategies for identifying reliable clinical research. |
comes were disappointingly poor. This approach was flawed in part due to absence of long-term clinical trials that were (and remain) unavailable for many logistical and ethical reasons.

Short term efficacy of NSAID and disease modifying antirheumatic drugs (DMARD) in clinical trials was not seen over long periods. Remission was generally seen only over 3–12 months. Although short term clinical trials and even a large metaanalysis [regarded as the highest form of medical evidence (Table 2)] indicated that the DMARD methotrexate (MTX), sulfasalazine, gold injections, and penicillamine were not distinguishable in efficacy for people with RA, substantive differences were seen over 5 years, as about 70% of courses of MTX were continued, compared to about 20% of the other DMARD.

Results from clinical care over one year were quite similar to those of clinical trials in indicating comparable efficacies of all DMARD over one year, although MTX was much more effective over 5 years. Such studies indicated that most patients with RA experienced radiographic progression and premature mortality over 5–10 years. Therefore, short term efficacy of many therapies cannot be assumed to persist, and long-term observational studies are required in addition to clinical trials to provide accurate evidence concerning results of therapies and outcomes of chronic diseases.

Three types of research studies contribute evidence concerning therapies and disease outcomes: randomized controlled clinical trials, prospective multi-center longitudinal observational studies, and reports from usual clinical care, generally by individual or a small group of practitioners. Each design has advantages and limitations: (1) Randomized controlled clinical trials, conducted according to the highest standards of quantitative measurement using established indices, provide the most rigorous data to compare efficacy of active versus control treatments. However, clinical trials include pragmatic and intrinsic limitations, particularly in chronic diseases, that are important not to ignore. (2) Large scale prospective longitudinal observational studies have provided many important observations concerning RA that were initially hypothesis generating, including high levels of work disability and premature mortality, and gastropathy associated with the use of NSAID. Given the challenges, including co-interventions, contamination of the control over time by new interventions, compliance, in addition to expense, and ethical issues involved in long-term clinical trials, few trials in rheumatology are conducted over much longer than 12–24 months. Nonetheless, the longitudinal observational study design is the only practical design for assessing the degree of benefit and toxicities of therapies over time. (3) Observations in usual clinical care by individual or a small group of practitioners in RA initiated reports of the degree of benefit and toxicities of therapies over time, frequent early radiographic damage, severe functional declines, work disability and premature mortality, absence of long-term remission, and better patient status at this time compared to previous decades. Such reports may lead to clinical trials and prospective longitudinal observational studies, as well as composite analytical reviews (as needed for individual clinical trials and longitudinal observational studies as well) to confirm and extend the findings.

Reports from standard clinical care are greatly enhanced by quantitative data, collected prospectively and for later analyses to provide evidence. However, most rheumatologists do not perform formal quantitative joint counts or collect patient questionnaires at most visits of most patients with RA. Therefore, most clinical rheumatology practice continues to be based largely on “gestalt” qualitative impressions — eminence, eloquence, and elegance — rather than evidence.

The most pragmatic approach to introduce quantitative assessment into standard rheumatology care is to ask each patient to complete a simple patient questionnaire at each visit. Patient questionnaires designed for standard care differ from research questionnaires in that they may provide medical history and review of systems data and be amenable to review and scoring in 15 seconds or less to guide clinical care, while saving time for the clinician and improving the quality and documentation of a patient visit. Patient questionnaire data provide the best evidence to predict severe long-term outcomes in patients with RA, including functional status, work disability, costs, joint replacement surgery, and premature death, as effectively as any clinical measure, including joint counts, radiographs, and laboratory tests.

Any rheumatologist can practice evidence-based clinical care by recording quantitative data at each patient visit. If no data are recorded at the time of the visit, the data can never be replaced. More evidence, and less eminence, eloquence, and elegance will enhance rheumatology care for patients and their rheumatologists.


70. Pincus T, Segurado OG. Most visits of most patients with rheumatoid arthritis to most rheumatologists do not include a formal quantitative joint count. Ann Rheum Dis 2006;65:820-2.


74. Wolfe F, Piilow F. Patient questionnaires for clinical research and improved standard patient care: is it better to have 80% of the information in 100% of patients or 100% of the information in 5% of patients? J Rheumatol 2005;32:575-7.


