Preface

What Should the Reader Expect from This Book?

The background for this book is a series of almost-parallel conferences on clinical research methodology organized by the senior and junior Yazici, in either Bodrum or Istanbul by the senior and in New York by Yusuf Yazici. However, it was Emmanuel Lesaffre, our third coeditor and several times a speaker in these conferences, who came up with the idea of this book. These annual conferences have been held since 2006 as a single-day or weekend courses. The audiences have been, we like to think, purposefully small with around 40–50 people, to encourage interactive discussion. The full texts of the majority of the presentations for the New York courses are available on PubMed as manuscripts derived from the lectures, free to download at the web page of Bulletin of the Hospital for Joint Diseases (www.nyuhjdbulletin.org).

It should therefore not come as a surprise to the reader that most of the contributors to this book have been speakers at these conferences.

We acknowledge that an excellent text and source book on similar topics as in this book has been available, namely, Evidence-Based Rheumatology by Peter Tugwell, also a speaker in our methodology conferences, since 2004. However, we reasoned that there still was an unmet need for our book for several reasons. A decade has passed since the publication of the volume by Tugwell et al. Ten years is a long period, and we believed that many things in rheumatology have significantly changed in quantity, direction, and quality. Furthermore, Evidence-Based Rheumatology has been almost an official guidebook of the Cochrane Foundation and its extension, the OMERACT (Outcome Measures in Rheumatology) group. Finally, a continuous and critical appraisal of the evidence for what we do or should do is to us the very essence of evidence-based medicine. With our book, we attempt to stand up to this challenge, both recommended and perceived.

We begin with an account of the birth of evidence-based medicine or, perhaps more correctly, “new evidence-based medicine,” as we like to call it. Being particular about evidence in medical practice has surely been around ever since Enlightenment. On the other hand, it surely needed a boost toward the end of the last century with the
tremendous increase in our understanding of, and the remedies to, many diseases. We argue that there were several important considerations for the emergence of evidence-based medicine. First, the biomedical model, most successful in explaining and managing acute illness, was surely not that successful in chronic diseases. Rather, a biopsychosocial model was more promising. The new evidence-based medicine was needed both to guide the practicing physician away from explaining the entirety of the multisystem disease of unknown etiology by the biomedical model and to check the science in the biopsychosocial model. The unnecessary medical services, especially in the light of ever-rising costs of medical care, were another main reason behind the new evidence-based medicine.

The chapter titled “Evidence-based medicine in rheumatology: how does it differ from other diseases?” also reminds us that the birth of rheumatology, as a separate subspecialty of internal medicine, is contemporary with the new evidence-based medicine. The idea and the scientific methodology of evidence-based medicine were surely most welcome in that “the multisystem disease of unknown etiology” has been and is the main preoccupation of our still relatively young discipline.

After all this rationalizing and theorizing, the chapter titled “A review of statistical approaches for the analysis of data in rheumatology” abruptly and intentionally brings us to the reality of numbers. With specific instructions to stay away from the integral sign, something that still makes most clinicians uncomfortable, the author makes a serious attempt to explain to the reader the arithmetic, both what is and what ought to be, behind evidence-based rheumatology. We like to think that the data-driven approach the author utilizes will help to explain the common, descriptive statistical approaches currently in use. His main focus is on the intuitive ideas behind the methods rather than on their technical aspects. Practical guidelines are also present throughout this chapter. Finally, the reader is also introduced to Bayesian methods that are becoming more and more popular.

The third chapter begins by emphasizing the importance of disease criteria in rheumatology since many of the rheumatologic diseases are, yet, constructs. In addition, the authors propose that our current separation of criteria as diagnostic or classification, although having an aura of practical or scientific sophistication, is ill founded. They underline that the cerebral exercise behind the two is the same. The thought barriers to this unhelpful bi-labeling are discussed while proposals for preparing more useful diagnostic/classification criteria are provided.

The chapter titled “Biomarkers, genetic association, and genomic studies” on biomarkers, including those of the genetic kind, is an assiduous account of our tribulations, accomplishments, hopes, and not uncommon wishful thinking around how to improve our laboratory capabilities to diagnose and monitor rheumatologic diseases. We like to think that this chapter, by using rheumatoid arthritis (RA) as a case example, shows how demanding, be it biochemical or genetic, it is to come up with a sensitive, specific, or more importantly a clinically useful disease marker.

The ensuing chapter “Outcome measures in rheumatoid arthritis” is on outcome measures in RA. While what happens to the patient at the end of what the physicians do or fail to do is the primary concern of medicine, it is rather surprising that we have not made a sincere effort to this end until recently. We used to think that we
physicians, with our laboratory tests and imaging gadgetry, always knew better than the patients themselves how they fared. Well, we now know this is not the case, and this chapter is all about how this recent realization relates particularly to RA.

The chapter titled “Issues in setting up a study and data collection” is a handy manual on both how to go about a research project and, while doing or interpreting that, avoid a biomedical bestiary of biases.

The following two chapters are detailed accounts of the randomized clinical trial (RCT), most certainly the flagship of evidence-based medicine, especially the new evidence-based medicine ever since the groundbreaking MRC streptomycin tuberculosis trial of 1948. Over 65 years have passed since the first-ever scientific application of this very important research tool. As expected, there are many finer points and caveats concerning the many forms of RCT now available dealing with the study design, its analysis, and most importantly the fit of the one with the other. The Bayesian RCT, which is more popular in our age of haste, is also introduced.

The following chapter by Ted Pincus is a healthy and critical discussion of perhaps the undue importance we give to the RCT as our golden measure of evidence. He gives in-depth examples to document his concern. One cannot help thinking that perhaps it is not only the undue importance the researcher, the practitioner, and the health authorities gave to this tool but rather its more recent abuse that justifies many of Ted Pincus’ concerns.

Whatever their causes, issues with the RCTs have recently led the medical community to put increasing faith in observational studies. One reason for this turn is our current ability to electronically collect and interpret vast amounts of data in a very short time. An additional reason for the recent popularity of observational data might be the contemporary interest in the inductive scientific approach.

Keeping up with our general and hopefully useful critical approach, Marie Hudson and Samy Suissa give us an expert overview of the many pitfalls in observational studies, especially in those studies that stem from data based on large patient repositories, such as administrative databases. A clear account of how to best recognize and avoid them is provided with special emphasis on data collection time–related biases in which the authors are leading experts.

With RCT struggling to maintain its flagship status in the face of many whips and arrows, recent years quietly put another mode of research into the pinnacle of evidence in evidence-based medicine. Meta-analysis is the science of reanalyzing the outcomes of different studies on a same subject by amalgamating individual research reports. Its most popular approach is to combine the experience from the RCTs. The Achilles heel of this approach is to put apples and oranges together in such analyses. We believe the authors of this relatively short chapter “Systematic reviews and meta-analyses in rheumatology” give a very useful account of how not to do this.

Our penultimate chapter is “Ethical issues in study design and reporting.” Any talk about ethics has the danger of being either too dry or too juicy. In this chapter, we have tried to avoid a discourse on the Helsinki declaration or provide particular examples of physician or industry misconduct which everybody knows and recognizes either from boring texts or daily tabloids. We have attempted to better describe what is not readily recognized not only by the public but many a time by the medical
profession itself. This we like to call the cerebral form of ethics, as distinct from the pudendal form of the tabloids. The cerebral form, we propose, is perhaps more pertinent to many things we do not like about the science and practice of medicine today.

Our final chapter is “Future directions.” It is our sincere hope that the readers of this book will have formulated their own priorities once they have gone this far in our book.

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