

Decision Making in a World of Comparative Effectiveness Research: A Practical Guide

Howard G. Birnbaum and Paul E. Greenberg (Eds., Adis, 2017)



This timely collection of 21 articles, organized and edited by Howard Birnbaum and Paul Greenberg on the current and future relationship between comparative effectiveness research (CER) and decision making, is a much-needed addition to the field. Our ISPOR task forces on CER have done a great deal to advance the methods and processes. This “Practical Guide” provides valuable insights on how CER is or can be used by different stakeholders for different types of decisions. With 47 highly qualified contributors, it is not possible to mention all of their names in the space of this short review; many of them will be well known to VOS readers, but it’s also encouraging to see a number of relatively new contributors. Hence, the aim here will be to provide a brief overview and share a few insights from the varying perspectives and wide range of important CER-related topics. The focus of this volume is on users and decision-makers rather than researchers. The crosscutting theme of the book is how we can—globally—better use CER to create a “learning health care system.”

Interestingly, despite CER being all about real-world data and evidence, the editors wisely resisted the temptation to present any new CER data and analyses. The articles are mix of background on how different health care systems (within and outside the US) use CER information, well done and fascinating interviews with insightful users and practitioners, and reviews of key developments and issues by experts both inside and outside of the pharmaceutical industry.

One might wonder about the shelf life of the information in this book. For readers who would like to learn about the state of the art in CER use as of 2017, the material here is surprisingly up-to-date, including numerous discussions and mentions of, for example, the latest value assessment frameworks and developments with regard to FDAMA Section 114 health economic information. Nonetheless, many of these developments represent the slow evolution of the complicated scientific and policy interaction that has been playing out over the last 25 years of ISPOR’s history. This collection will provide a useful milestone for years to come. As underscored in this collection, however, the development and use of CER information has been “surprisingly” and “frustratingly” slow.

Although the CER in Unites States (US) receives the bulk of the attention, there is also a great deal of discussion about systems outside the US as well as global issues such as the financing of R&D and its relation to value-based pricing and reimbursement. These issues are inherently global given that the knowledge embedded in new medicines, devices, and other innovative technologies and any supporting CER are essentially global public goods. Of course, rather than attempting to describe the structure and operations of these complex HTA institutions, the papers emphasize the single aspect of how these systems generate and use CER. For the US, the limited use of CER—and cost effectiveness—by the private sector is addressed by several authors, and there is a thorough review of how US public payers—Medicare, Medicaid, and the VA—use CER, emphasizing the future uncertainties. Outside the US, the role of CER in the following systems, among others, is discussed: United Kingdom, Japan, France, Germany, Japan, Canada, and the Global Fund.

From my perspective, the emergence of evidence synthesis techniques has been one the major advances in HEOR research in the last 25 years. There is an excellent introduction and history to the tool of indirect treatment comparisons, and there is an overview of other innovative issues and techniques such heterogeneity of treatment effects, machine learning, stakeholder engagement, clinical practice guidelines and pathways, and pre-registration of CER studies.

In sum, I can highly recommend this thought-provoking collection; it would be of interest to anyone with an interest in how we have gotten to where we are with CER, and how we can best proceed. HEOR researchers and health system stakeholders—including policy makers, health system reformers, and CER entrepreneurs—would benefit from this wealth of information, insights, and prescriptions. All of this provides some reason for optimism despite the severe undersupply of CER information for patients worldwide—a sad reality that calls for greater resources and effort.

As a teaser, I have pulled out a few quotes to stimulate the interest and curiosity of some readers:

“The marriage of big data and CER represents the potential to revolutionize decision-making and realize the goal of . . . a ‘learning healthcare system’”.

“Currently, higher US brand prices appear to subsidize in large part drug innovation for the rest of the world. This is unlikely to be sustainable, and Europe in particular needs to assume a larger role and share.”

“There are challenges in creating patient centeredness. For instance, it can be difficult to find the ‘right’ patients and stakeholders to inform trial design and implementation.”

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