Introduction

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ABSTRACT

BACKGROUND: The Patient Protection and Affordable Care Act brought considerable attention to comparative effectiveness research (CER).

OBJECTIVES: To (a) suggest best practices for conducting and reporting CER using “real-world data” (RWD), (b) describe some of the data and infrastructure requirements for conducting CER using RWD, (c) identify statistical challenges with the analysis of nonrandomized studies and suggest appropriate techniques to address those challenges, (d) recognize the value of patient-reported outcomes in CER, (e) encourage the incorporation of observational data into randomized controlled studies, and (f) highlight the importance of incorporating payers in industry-sponsored research.

SUMMARY: The first article in this supplement, “Something old, something new…” provides a policy perspective on the recent evolution of CER. It reviews the historical context, discusses the “promise and fear” of CER, and then describes the new role of the Patient-Centered Outcomes Research Institute (PCORI) in defining and sponsoring CER. The second paper, “Ten Commandments,” proposes a series of tenets for planning, conducting, and reporting CER done with RWD. Oriented for basic-to-intermediate researchers, it combines standard scientific research principles with considerations specific to nonrandomized, RWD studies. The third article, “Infrastructure Requirements,” points out that effective use of secondary data requires addressing major methodological and infrastructural issues, including development of analytical tools to readily access and analyze data, formulation of guidelines to enhance quality and transparency, establishment of data standards, and creation of data warehouses that respect the privacy and confidentiality of patients. It identifies gaps that must be filled to address the underlying issues, with emphasis on data standards, data quality assurance, data warehouses, computing environment, and protection of privacy and confidentiality. The fourth paper, “Statistical Issues,” discusses how the validity of analytic results from observational studies is adversely impacted by biases that may be introduced due to lack of randomization. It reviews some of the methodological challenges that arise in the analysis of data from nonrandomized studies, with particular emphasis on the limitations of traditional approaches and potential solutions from recent methodological developments. The fifth paper, “Considerations on the Use of Patient Reported Outcomes (PROs),” describes how PRO data can play a critical role in guiding patients, health care providers, payers, and policy makers in making informed decisions regarding patient-centered treatment from among alternative options and technologies and have been noted as such by PCORI. However, collection and interpretation of such data within the context of CER have not yet been fully established. It discusses some challenges with including PROs in CER initiatives, provides a framework for their effective use, and proposes several areas for future research. Lastly, “Developing a Collaborative Study Protocol…” indicates that there is the potential, the desire, and the capability for payers to be involved in CER studies, combining elements of their own observational data with prospective studies. It describes a case example of a payer, a pharmaceutical company, and a research organization collaborating on a prospective study to examine the effect of prior authorization for pregabalin on health care costs to the payer.

CONCLUSION: Researchers at Pfizer routinely conduct CER-type studies. In this supplement, we have proposed some approaches that we believe are useful in developing certain kinds of evidence and have described some of our experiences. Our experiences also make us acutely aware of the limitations of approaches and data sources that have been used for CER studies and suggest that there is a need to further develop methods that are most useful for answering CER questions.

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reviews the challenges associated with the use of PROs in CER and provides a framework for their effective use in such trials. Finally, the last article in this supplement discusses the importance of CER studies and offers a collaborative approach for conducting such studies that will better equip patients, physicians, and payers to make more informed decisions about which health care resources are most appropriate for specific clinical conditions and patients.

The supplement describes up-to-date research techniques and policies related to CER and represents a guide by which Pfizer conducts similar research. This supplement therefore provides the reader with an understanding on one company’s approach to ensuring that their scientific investigations within the umbrella of CER studies follow strict guidelines to ensure credible application of CER for evidence generation and use of our medicines.

**DISCLOSURES**
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**REFERENCES**