

## Editorial



# Comparative effectiveness research in observational studies

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Comparative effectiveness research (CER), established as a branch of health services research to ascertain which medications or treatments are most effective for patients in specific circumstances, is currently emerging as a highly-promising research field. The most commonly-cited definition of CER was proposed by the Institute of Medicine in 2009, and states that CER is “the generation and synthesis of evidence that compares the benefits and harms of alternative methods to prevent, diagnose, treat, and monitor a clinical condition or to improve the delivery of care.” The scope of CER has been further broadened by encompassing not only clinical interventions but also community and other public health interventions which are implemented through a wide range of study designs (experimental, observational, prospective, retrospective, etc.). The overarching goal of CER is to better support the decision-making of patients, caregivers, clinicians, and regulatory officials based on credible information, ultimately leading to improved health outcomes. As can be observed in many biomedical and public health applications, biostatistics plays a core role in CER studies, providing fundamental tools for achieving the goal.

A growing body of quantitative methodological research for CER studies has been particularly devoted to observational studies of “real-world” settings where controlled experiments are not feasible for a variety of reasons, such as ethical, social, and practical considerations. The presence of confounding factors that affect treatment selection and are also correlated with treatment outcomes is a major concern in observational studies due to the problem that the decision of which patients to treat is non-random and thus potentially susceptible to bias, making it difficult to identify the causal effect of treatment on the outcome. Extensive efforts have been made to overcome this problem. For measured confounders, various methods, such as regression adjustment, stratification, propensity score techniques, and marginal structural models, allow the approximation of experimental control with statistical control. For unmeasured but potential confounders on which relatively little research has been done, instrumental variable (IV) procedures are widely employed under certain conditions. The idea of IVs was first derived in econometrics and later rapidly expanded to other research areas except healthcare science often featured with censoring because IV methods do not take censoring into account. This limitation has, however, fostered the development of novel statistical methodologies to deal with the joint problem of censoring and unmeasured confounding.

These cutting-edge CER approaches, including sophisticated statistical techniques, are being increasingly disseminated across diverse fields of health care. Advances in CER enhance our understanding of the scientific mechanisms underlying biomedical phenomena, helping us

to obtain valid and meaningful information regarding intricate cause-and-effect relationships in observational studies. Findings from appropriately-performed analyses in CER studies can bring substantial benefits to patients and their communities by enabling studies to have the largest impact on the topic in question, by utilizing ingenious adaptations of relevant protocols in other applied contexts, and by facilitating systems that can optimally reflect the needs of individuals engaged in subsequent decision-making.