

Review Article

Comparative Effectiveness Research and Cancer Epidemiology: New and Exciting Research Avenues under the Patient Protection and Affordable Care Act

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Abstract

The Patient Protection and Affordable Care Act (PPACA) put into federal law numerous opportunities for cancer research and cancer epidemiology. The legislation created the Patient-Centered Outcomes Research Institute (PCORI) and established the types of research needed to compare treatments in patient populations and generate relevant outcome data. Comparative Effectiveness Research (CER) uses randomized trials and observational cohort designs to establish the magnitude of comparable treatment effects in real-world populations. The field of cancer research and epidemiology is uniquely positioned to lead the evolution of clinical evidence as it relates to CER and the goals and objectives of the PPACA and PCORI. The amount of data and outcomes in tumor registries and secondary databases gives researchers ample opportunities to conduct research on cancer treatments, epidemiology, diagnosis, and survival.

ABBREVIATIONS

CER: Comparative Effectiveness Research and Control Event Rate; PPACA: Patient Protection and Affordable Care Act; SEER: Surveillance, Epidemiology, and End Results; NCDB: National Cancer Data Base; PCORI: Patient-Centered Outcomes Research Institute; PCOR: Patient-Centered Outcomes Research;

INTRODUCTION

The Patient Protection and Affordable Care Act (PPACA) put forth a call for increased accountability in the quality and cost of healthcare in the United States [1]. The legislation reinforced the need for comparative effectiveness research (CER) where multiple treatments are compared to establish their efficacy in relation to patient outcomes. The PPACA also proffered a renewed focus on “patient-centeredness” where 1) medical decisions are made to match patient values and preferences, 2) underserved or vulnerable subgroups in the population are specifically targeted, and 3) clinical evidence is heavily integrated into clinical practice.

Cancer research is uniquely positioned in regards to the types of treatments and outcomes associated with the PPACA and its

empirical and clinical objectives. There is also outcome data on millions of individual cancer patients via tumor registries and secondary data sources such as the Surveillance, Epidemiology, and End Results (SEER) and National Cancer Database (NCDB) databases. With the sheer volume of outcome data available to cancer researchers, it behooves our scientific community to lead the charge in emerging CER objectives under the PPACA.

METHODS

The methods of research design and statistical analysis within CER are not novel in terms of their everyday use by researchers and clinicians. However, their application in terms of meeting the goals and objectives of the PPACA constitutes new and exciting avenues for cancer research [2]. A pragmatic and concise review of the literature for these methods under the guise of the PPACA was warranted to fully take advantage of these new research opportunities.

Comparative effectiveness research defined

The PPACA defined CER as “research evaluation and comparing health outcomes and clinical effectiveness, risks,

and benefits of two or more medical treatments, services, and items." PPACA further described treatments, services, and items as "healthcare interventions, protocols for treatment, care management, delivery, procedures, medical devices, diagnostic tools, pharmaceuticals, integrative health practices, and any other strategies or items being used in the treatment, management, diagnosis, or prevention of illness or injury in individuals" [1]. In order to bolster the research efforts towards these ends, the PPACA created the Patient-Centered Outcomes Research Institute (PCORI) which set forth standards for research evidence that could lead to integrating clinical evidence into practice [1].

Patient-Centered Outcomes Research (PCOR) and CER can be considered isomorphic as they have the same objectives for comparing interventions. With such an aligned and interdependent relationship between the definition of PCOR and the methods and analyses associated with CER, a diverse and rewarding set of empirical and clinical research agendas can be sought out by cancer researchers in the context of healthcare outcomes under the PPACA. With the sheer amount of outcome data that exists in cancer research, scientists in this area can produce clinical evidence based on vast and diverse sets of patients from real-world populations.

Comparative effective research designs

PCORI described what types of research designs can be employed as CER and established the empirical benchmarks expected of CER studies [1]. The two primary CER designs are randomized trials and observational cohort studies (retrospective and prospective) [2]. In comparison to randomized controlled trials, cluster randomized trials are better suited to meet PCOR and CER objectives because randomization occurs at the intervention level rather than at the patient level [2]. For observational designs, certain criteria must be met for retrospective and prospective cohort designs to yield valid and generalizable differences in treatment effects [1]. Most importantly, cohort designs must assess treatment effect differences between comparable treatments in explicitly defined patient populations while accounting for pertinent confounding phenomena [1]. Cohort designs can further generate evidence related to the etiology, progression, and prognosis of disease states [3].

One primary objective of the PCORI is to reinforce the study of disease states in underserved or key subgroups so that they can be represented in the clinical knowledge base [1]. Sensitivity analysis is often performed on subgroups of a population related to ethnicity, gender, age, and/or comorbidities [4]. Cancer research focused on comparing treatments in these subgroups

will always be welcomed additions to the literature as clinical pathways associated with the advent of the PPACA, because this objective is written directly into the law itself [1].

From the applied research perspective, PPACA, PCORI, and CER create avenues for long-term research success for cancer researchers. Retrospective cohort designs are very feasible research designs because the data and outcomes already exist [2]. Observational designs require a priori research questions, power analyses, meticulously crafted and transparent methodological protocols, and meet all of the aforementioned design criteria [1]. Yet, from an applied research standpoint, the PPACA and its subsequent goals and objectives for CER evidence provides researchers of all levels of expertise and clinical demands the opportunity to make important contributions to a new and growing body of clinical evidence [4].

CONCLUSION

From an epidemiological and methodological perspective, cancer research is uniquely positioned to produce vast amounts of CER evidence for the diagnosis and treatment of numerous kinds of cancer. The PPACA legislation created a plethora of new and exciting avenues for conducting clinical, diagnostic, epidemiological, and survival research [1]. The field of cancer research, with its vast repositories of short-term and long-term secondary outcome data, is a perfect match for the now legally accepted, user-friendly, and more feasible randomized trials and observational cohort studies called for by the PPACA and PCORI [2].

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