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Implementing randomized effectiveness trials in large insurance systems

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Abstract

Background: The need to identify how best to structure health insurance and to deliver health care services is a central priority for comparative effectiveness research. Studies designed to evaluate these issues are frequently conducted in large insurance systems. We sought to describe the challenges faced when conducting trials in this context.

Methods: Using the Post-Myocardial Infarction Free Rx Event and Economic Evaluation (MI FREEE) trial as an example, we describe the methodological and practical challenges of conducting trials in large insurance systems.

Results: We encountered six key challenges while conducting MI FREEE trial, namely the need to obtain plan sponsor permission to experiment, the desire of plan sponsors to have all of their beneficiaries receive the same intervention, the inaccuracy of claims-based identification methods and the impact of claims lag on the timely enrollment of potentially eligible patients, the reluctance of patients to participate in insurance-based interventions and the potential need for informed consent, the frequent introduction of new cointerventions in real-world delivery systems, and the high rates of loss to follow-up because of insurance ''churn.'' We describe the approaches we used to overcome these challenges.

Conclusions: Studies in insurance settings are a powerful and necessary design for evaluating comparative effectiveness interventions. There are numerous strategies to address the potential logistical and methodological challenges that this research environment uniquely creates.

Keywords: Health insurance; Randomized trials; Cost sharing; Acute myocardial infarction; Adherence; Secondary prevention

1. Introduction

The need to identify how best to structure health insurance and to deliver health care services is a central priority for comparative effectiveness research. However, rigorous prospective studies of innovative policies and benefit designs or large quality improvement interventions occur infrequently, leaving a notable lack of data to support evidence-based policy making. New policies are often implemented in the context of health insurance systems, the primary purpose of which is to administer health care benefits rather than to conduct research. These experiments often are not designed in a manner that promotes rigor, limiting the opportunity to optimally learn generalizable lessons about better policy making.

More rigorous prospective randomized designs conducted in large insurance systems are a rarity [1,2]; and when they are attempted, pragmatic and methodological problems are encountered that are distinct from those seen in more traditional research settings. We are unaware of any systemic description of these problems, and, accordingly, the objective of this article is to describe the challenges that we faced when conducting the recently completed Post-Myocardial Infarction Free Rx Event and Economic Evaluation (MI FREEE) trial and to discuss the strategies we used to overcome them [3]. We focus on issues that are of particular relevance to cluster randomized policy studies with prospective participant recruitment conducted in partnership with large commercial insurers, although we have also recently faced many similar challenges while designing individually randomized comparative effectiveness trials of quality improvement interventions in other insurance settings. We hope that the lessons learned can serve to help develop best practices as researchers increasingly collaborate with commercial insurers to implement and test new payment and delivery system approaches.

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What is new?

Key findings

- We encountered six challenges while conducting the Post-Myocardial Infarction Free Rx Event and Economic Evaluation (MI FREEE) trial, namely the need to obtain plan sponsor permission to experiment, the desire of plan sponsors to have all of their beneficiaries receive the same intervention, the potential inaccuracy of claims-based identification methods and the impact of claims lag on the timely enrollment of subjects, the reluctance of patients to participate in insurance-based interventions and the potential need for informed consent, the frequent introduction of new cointerventions in real-world delivery systems, and the high rates of loss-to-follow-up because of insurance ''churn''.

What this adds to what is known?

- The need to identify how best to structure health insurance and to deliver health care services is a central priority for comparative effectiveness research. Studies designed to evaluate these issues are frequently conducted in large insurance systems.
- There are numerous strategies to address the potential logistical and methodological challenges that this research environment uniquely creates.

What is the implication and what should change now?

- Pragmatic studies in insurance settings are essential to identify strategies to improve health care delivery, and best practices to conduct such studies must be developed.

2. Case example: MI FREEE trial

The MI FREEE trial was a randomized policy experiment designed to evaluate the comparative effectiveness of two insurance benefit designs (full vs. usual prescription drug coverage) for secondary prevention medications prescribed to patients after acute myocardial infarction (MI) [4]. The study was motivated by the consistent observation that rates of long-term use or ''adherence'' to evidence-based cardiovascular medications are extremely low [5]. Of the many factors that contribute to this problem, the costs faced by patients when purchasing their drugs (generally in the form of copayments and coinsurance) are a well-recognized contributor [6]. Considering the proven efficacy of secondary prevention medications after MI, there was reason to believe that efforts to improve adherence by eliminating patient out-of-pocket spending could both improve health outcomes and reduce health care expenditures because of savings from averted hospitalizations and procedures [7,8]. The MI FREEE trial aimed to test this hypothesis.

The design of the MI FREEE trial has previously been published [3,4]. In brief, the trial included individuals recently discharged from hospital after acute MI who received health and pharmacy benefits from Aetna, a large health insurer in the United States. Potentially eligible patients, who were identified using administrative discharge claims submitted by hospitals to Aetna, either received full or usual coverage for secondary prevention therapies (i.e., any prescribed angiotensin-converting enzyme inhibitors, angiotensin receptor blocker, beta-blocker, or statin). Assignment occurred by cluster randomization at the level of the plan sponsor (i.e., the employer, union, government, or association that sponsors a particular benefits package), such that after the first eligible beneficiary of a given plan sponsor had been identified and assigned to a treatment arm, all subsequently eligible beneficiaries of that plan sponsor received the same coverage. The overall trial procedures are summarized in Fig. 1.

During the 34-month study period, 6,768 potentially eligible subjects were identified of whom 913 were not randomized because their plan sponsors opted to not participate. The remaining 5,855 patients (87% of those who were potentially eligible) were randomized an average of 49 days post-MI and followed for a median of 394 days (interquartile range: 201−663 days). Providing full coverage improved rates of adherence to each class of study medication by 4−6% points, reduced rates of first major vascular event (11.0 vs. 12.8 per 100 person-years, hazard ratio: 0.86; 95% confidence interval [CI]: 0.74−0.99) and total major vascular events or revascularization (21.5 vs. 23.3, hazard ratio: 0.89, 95% CI: 0.90−0.99) but did not significantly change not the prespecified primary endpoint, first major vascular event, or revascularization (hazard ratio: 0.93, 95% CI: 0.82−1.04) [3]. Providing more generous coverage led to a nonsignificant reduction in total per capita health care spending ($66,008 for the full-coverage group and $71,778 for the usual-coverage group; relative spending: 0.89; 95% CI: 0.50−1.56) and reduced the patient out-of-pocket costs for drugs and other medical services (relative spending: 0.74; 95% CI: 0.68−0.80).

3. Challenge 1: Conducting trials in insurance systems frequently requires plan sponsor permission

Large insurers provide benefits to millions of individuals, and thus, in principle, trials conducted in this environment should be more than adequately powered for even relatively rare conditions or outcomes. However, insurers provide and administer benefits on behalf of numerous plan sponsors, the largest of which are ''self-insured'' and for