

Use of the PRECIS-II instrument to categorize reports along the efficacy-effectiveness spectrum in an hepatitis C virus care continuum systematic review and meta-analysis

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Abstract

There is increasing recognition of the importance of the distinction between efficacy and effectiveness research in the design, conduct, and evaluation of interventions and program outcomes. There is a concurrent increase in the application of systematic reviews and meta-analyses. These two lines of inquiry are only beginning to meet. There is an emerging need for systematic reviews and meta-analyses to account for differences in degrees to which included studies reflect either efficacy or effectiveness design. Based on ongoing work on a formal systematic review of the hepatitis C virus care continuum, this paper describes and discusses the rationale for, and how the PRECIS-II instrument can be used on, and modestly adapted to, studies included in the systematic review examining the extent to which studies include elements of efficacy or effectiveness or a combination of the two. We also highlight that use of such an instrument may have general applicability to and value in the conduct of systematic reviews and meta-analysis. © 2017 Elsevier Inc. All rights reserved.

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1. Introduction

Efficacy and effectiveness studies provide two distinct ways to examine the impact of an intervention (e.g., a pharmacologic treatment or a behavioral intervention). They address two distinct research questions [1,2]. All aspects of study design, including methods for sampling, recruiting, and data analysis, inform whether the study is asking (and answering) an efficacy or effectiveness research question [1–3]. It is critical to recognize what differentiates questions of efficacy and effectiveness, to situate published studies along a spectrum of efficacy and effectiveness, to specify the research question being addressed, and to understand which type of study provides answers to which research questions [4–7].

Efficacy studies are designed to focus attention on the impact of a specified intervention on a specified outcome under precisely defined, and often idealized, conditions [2,3,8]. These study design elements help ensure high internal validity, which improves causal inference with respect to the direct impact of that specified intervention, but only under those precisely defined circumstances (e.g., on a precisely defined population and under precisely defined conditions of intervention implementation) [6,9]. As such, some have referred to efficacy studies as “explanatory studies”; in some settings, these studies may best “explain” biologic mechanisms or the idealized potential of an intervention, such as a pharmacologic agent, to yield a specific outcome [2,3,10]. In general, efficacy studies can be seen as addressing the question “can an intervention work” when the investigators do everything possible to help the intervention work.

In contrast, effectiveness studies are designed to focus attention on the impact of an intervention as applied in practice on specified outcomes, under conditions experienced in general practice, facilitating generalizability by focusing on the question of “will an intervention work” in general practice. In other words, while an effectiveness

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What is new?**Key findings**

- The PRECIS-II instrument can be modestly adapted to assess studies of the HCV care continuum and to categorize them with respect to the degree they address questions of efficacy and effectiveness.

What this adds to what was known?

- For studies examining more than one step in the HCV care continuum (e.g., testing, linkage to care, and treatment), the instrument may need to be applied more than once.
- Systematic reviews, which increasingly examine both observational and RCT data, may need to consider the degree to which included studies rely on efficacy and effectiveness design elements.
- The PRECIS-II instrument, modestly adapted, may be a useful tool allowing systematic reviews to categorize included papers with respect to variability in efficacy or effectiveness design.

What is the implication and what should change now?

- Systematic reviews and meta-analyses would be more informative if included reports were categorized along the efficacy-effectiveness spectrum and this categorization was taken into account in analyses.

study can examine the impact of an intervention and in fact may do so with an randomized controlled trials design, this type of study asks whether an intervention “will work” in general practice [3,11]. These studies more robustly demonstrate the impact of interventions in general practice but not idealized settings; as such, some have referred to effectiveness studies as “pragmatic” studies as they aim to examine the impact of an intervention in practice [2].

Of note, a study examining the use of the terms efficacy and effectiveness in the published literature found that they were used inappropriately and inconsistently, and consequently, often lead to the misclassification of studies [12]. Furthermore, while some scholars use the terms synonymously, for some readers the terms “explanatory” and “pragmatic” may be taken to imply superior scientific rigor through the term “explanatory” and inferior scientific rigor through the term “pragmatic”; we will use the terms efficacy and effectiveness in attempt to highlight their equal importance in addressing different but simultaneously important research questions [3].

Studies can be thought of as existing along a continuum of efficacy and effectiveness, in which the ends of the spectrum are characterized by either a pure efficacy study design or a pure effectiveness study design, with a range of studies in between having some characteristics of both [2,13]. Furthermore, it should be noted that both efficacy and effectiveness questions can be addressed using either randomized controlled trial or observational study designs and that conversely randomized controlled trials can be designed to more fully address either questions of efficacy or effectiveness. The design features of randomization in randomized controlled trials contribute potently to achieving higher internal validity and minimization of potential confounding, but randomized controlled trials can nonetheless focus on questions of efficacy or effectiveness to varying degrees.

Systematic reviews and meta-analyses are increasingly used to synthesize the enormous amounts of published material, including published data from observational and experimental studies related to hepatitis C virus and drug use epidemiology [14]. Concomitantly, the results generated from systematic reviews and meta-analyses are increasingly being used to inform policy [14]. The reliance on the results of systematic reviews and meta-analyses for policy decisions requires that they generate sound and unbiased syntheses and pooled estimates. As has been noted by Stroup et al. and others, because of existing pressures for timely data and because some data are only available or are more quickly available from observational studies, systematic reviews and meta-analyses have increasingly included and examined observational studies as well as randomized controlled trials [15–17]. Guidelines for the optimal reporting of systematic reviews of observational data have been proposed [15,18].

We are currently conducting a systematic review of the hepatitis C virus care continuum among people who use drugs [19]. This paper describes and discusses the rationale for, and how the PRECIS-II instrument can be used on, and modestly adapted to, studies of the hepatitis C virus care continuum, examining the extent to which studies include elements of efficacy or effectiveness or a combination of the two [19]. We discuss how included studies may reflect efficacy or effectiveness designs to varying degrees overall, and for each of the care continuum steps considered, and how such assessments may inform the design, conduct, and results of systematic reviews and meta-analyses. We also highlight that use of such an instrument may have general applicability to and value in the conduct of systematic reviews and meta-analysis.

2. Definitions**2.1. Efficacy**

In efficacy studies, patient eligibility and exclusion criteria are generally restrictive in order to create an ideal environment that eliminates or holds constant as many

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