

Evolving Evidence-based Medicine: Thinking Beyond Randomized Controlled Trials

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ABSTRACT

Evidence-based Medicine (EBM) is focused on the conscientious, explicit and judicious use of current best evidence in making decisions about the care of the individual patient. Traditionally, it involves the integration of individual clinical expertise, by a practitioner, with the best available external clinical evidence from systematic research, where clinical evidence is garnered from randomized controlled trials (RCTs). Unfortunately, the time horizon from RCT design to results publication is typically measured in years, rather than months, days, minutes or seconds. This often means that the production and application of evidence cannot be done in a timely fashion. This paper purports that these factors, plus the advent of advanced analytics that uncover insight from healthcare data sources, warrant the augmentation of EBM to include current data-driven medical techniques and insight.

Keywords: Evidence-based Medicine, Randomized Clinical Trials,

1. INTRODUCTION

Evidence-Based Medicine (EBM) is an essential component of scientific clinical decision-making (Sackett et al., 1996). Its intention is to provide the best available evidence obtained from clinical research to justify physicians' clinical practice. Typically, the evidence for beneficial and adverse effects of studied interventions are collected and classified by different ranks of evidence quality.

Eddy (2005) provided a detailed account of evidence-based medicine, as it has evolved since the 1970's. He identified two ways in which evidence can be applied to improve health care: Evidence-Based Guidelines (EBG) and Evidence-Based Individual Decision making (EBID), and argued that they are both necessary and complementary aspects of Evidence-Based Medicine. EBG refers to the approach where a set of population or sub-population level guidelines and policies are produced based on the best known evidence, i.e. published clinical study results. As pointed out by Eddy (2005), some key characteristics of the approach are: 1) they are typically conducted by small groups of specially trained people; 2) they follow an explicit, rigorous process, e.g., systematic review, critical analysis of quality of evidence, etc.; and 3) their products aim to be generic, i.e., the guidelines are intended to apply to a class or group of patients defined by some clinical criteria, rather than to an individual patient. EBID, in contrast, focuses on helping physicians identify and bring relevant evidence into decision making for individual patients. While it was originally designed with physicians as the chief decision makers, it can be equally applied to other actors in the health system – pharmacists, case managers, nurses, and even patients. It seeks to provide support to individuals so as to enable them to properly filter and interpret evidence relevant to a specific case.

Traditionally, “evidence” in both EBG and EBID refers to published results from clinical studies, particularly Randomized Controlled Trials (RCTs). This paper proposes that as technology has evolved, patient needs have heightened and innovation in RCTs remained unchanged. Also, the initial definition of evidence now seems

narrow and is becoming increasingly inadequate as clinical questions are growing at a much faster rate than can be answered by traditional methods of evidence generation and aggregation.

Over the years, the research community has identified a set of concerns with RCTs. The first being that RCTs cannot provide direct answers for everyday practice, and cannot easily answer basic questions such as “Does the treatment work in the real world of everyday practice?” The second issue raised is a direct consequence of the use of ideal experiment conditions, RCTs are known to be ill-suited for the detection of the effects of intervention combination or interventions for different patient groups with different medical conditions as usually seen in real clinical settings. The third issue is that the conclusions from RCTs have limited generalizability, because of the aforementioned limitations, and cannot be safely extrapolated to other patient groups, which are different from the homogeneous groups in RCT studies. Fourth, the choice of outcome, or endpoint measures, in RCTs tends to be focused and often does not provide a complete picture of the benefits and harms that are important to patients.

The existence of repositories of electronic health records (EHRs) as possibly sources of observational data for the generation of new evidence or the refinement of RCT-generated evidence and the new available tooling enables a new world of evidence.

2. THE NEW WORLD

In this new world of evidence, where the definition of evidence is expanded to include both results of RCTs and insight from data-driven medicine, there is opportunity to make practitioners more efficient and increase positive outcomes. Every patient encounter in this world is a source of data, which can be leveraged for evidence generation. Such evidence could then be delivered back to the point of care (Figure 1). The core engine, in Figure 1, is the analytics for conducting comparative effectiveness and outcomes research, and tools for similar patient cohort identification based on clinical and health characteristics. In addition, traditional sources of evidence can be accessed to provide other relevant evidence. For the generation of such evidence there will always be the need for RCTs, particularly to assess the efficacy of new drugs, treatments and medical devices.

In addition, the need for granular evidence, i.e. evidence on what combination of interventions or practices work best for specific types of patients under specific conditions, leads to an exponential explosion of potential hypotheses to be investigated. Thus, it will be critical to develop methodologies that can be used to efficiently search for patterns in large amount of practice data, and identify the most promising hypotheses to be further tested using statistical models, observational studies, and in some cases RCTs.

Even the best-designed clinical practice guidelines (CPGs) - the result of RCTs - do not always apply to an individual patient in the real-world clinical setting, particularly a patient with complex medical needs. CPGs, even when delivered appropriately in the context of care of a specific patient, will always have inherent limitations, because by design they are given at a high level and aimed at the “average” patient of a population or subpopulation. As a result of the rapid advances in medical treatments and diagnostic devices in recent decades, combined with an aging population leading to more patients with complex co-morbidities, physicians and other care providers are now increasingly faced with intricate questions in medical decision making that cannot be answered by predefined CPGs. One way to realize this opportunity is to develop patient similarity metrics that can be used to assess clinical similarity between patients based on their longitudinal medical records. Such metrics will allow “patient like this” type of decision support in real time, where a group of patients with similar clinical condition and history can be retrieved, and their longitudinal records can be analyzed to help determine the best course of action for the patient being seen. It is envisioned that such “individual level” evidence will become an increasingly important part of EBM. Figure 2 illustrates the emerging vision of this new evidence landscape.

To provide such “individual level” evidence at the time of service delivery, dynamicity is another key concern. It is envisioned that the use of additional "delivery context" information for clinical pathways in the longitudinal clinical records is necessary. Such information can be customized for dynamic service delivery, where it requires understanding of a target user’s current wellness status and context at the time of care.

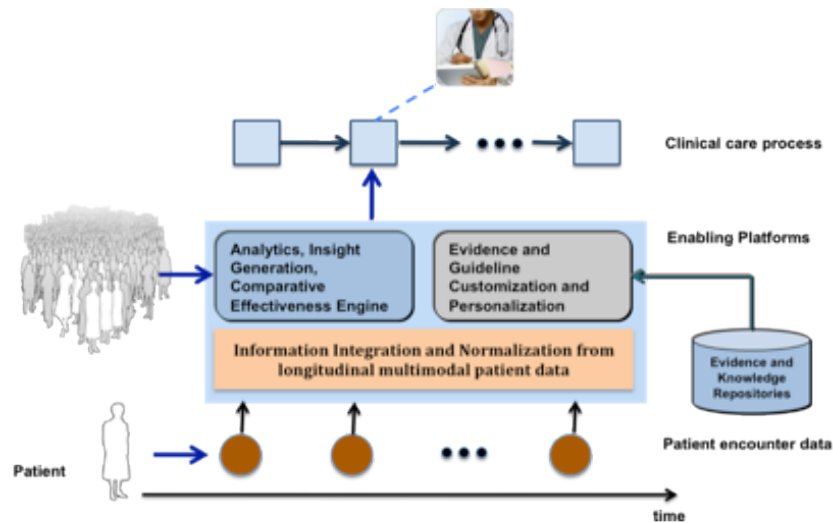


Figure 1: New Vision of Evidence Cycle

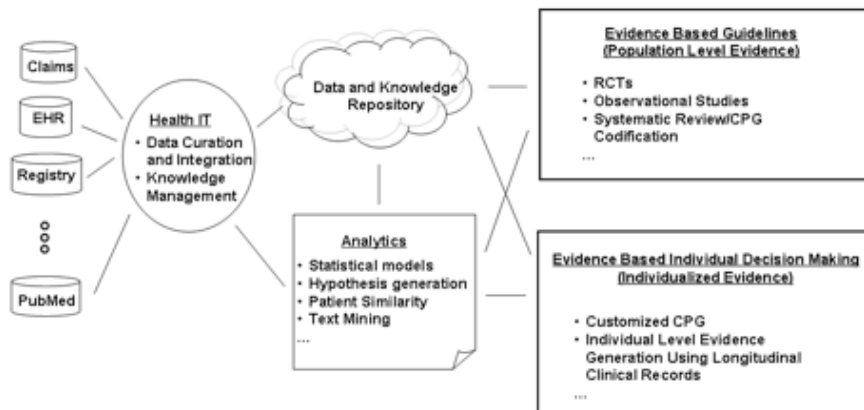


Figure 2: New Evidence Landscape

3. CONCLUSION

This paper proposes that augmenting the traditional view of evidence-based medicine to include the notion of leveraging insight gained from healthcare data silos (i.e. data-driven medicine) is the next evolutionary step for the medical informatics field. This paper examined randomized controlled trials, and the factors that are necessitating the use of other techniques to increase the diagnostic ability of the practitioner at the point of care.

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