Gathering and Learning From Relevant Clinical Data: A New Framework
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Abstract

Given the rising costs of health care in today's economic environment, the need for effective, value-driven care has never been more pressing. While the U.S. health care system strives continually to improve patient outcomes, it struggles with the inadequacies due to variation in care and the inefficiencies of unnecessary resource utilization. The tools traditionally used to study care, from retrospective studies to randomized controlled trials, may be inadequate to address the complicated, interdependent questions related to defining effective care. To overcome the deficiencies of these traditional tools and better optimize our health care system, a new kind of methodology is required—one that integrates the functionality of previously existing tools in a novel way.

Standardized Clinical Assessment and Management Plans (SCAMPs) were designed to accomplish this goal. A SCAMP is a care pathway, designed by clinicians, to guide medical decision making around a particular disorder. SCAMPs are unique in that they invite knowledge-based diversions from their recommendations and are accompanied by data collection and continuous improvement processes. Through these mechanisms, SCAMPs successfully reduce practice variation, optimize resource use, and create an integrated medical learning system which overcomes many of the inadequacies of traditional research tools. As such, the SCAMP paradigm may represent an important breakthrough in the effort to define and implement effective health care.

Editor's Note: A Commentary by H.C. Sox and W.F. Stewart appears on pages XXX–XXX.

In this era of economic uncertainty and ongoing health care reform, medicine is faced with the imperative of controlling rising costs while maintaining high-quality, safe, and equitable standards of care. Comparative effectiveness research, with the goals of defining "effective care" and better guiding medical decision making, aims to help achieve this balance while avoiding alternative methods of cost containment like care rationing or reimbursement reduction. A well-functioning system of comparative effectiveness research must operate in a program of continuous improvement to keep pace with medical knowledge and provide practical recommendations. Despite the pressing need for such a system, achievement of this ideal has proven largely illusory.

Attempts to carry out effectiveness studies have been largely drawn from the toolkit of clinical research, from case–control studies to clinical trials. However, these methodologies may be ill equipped to properly inform comparative effectiveness research and achieve the ideal of continuous improvement. Through the lens of a theory-building framework, we here examine the tools traditionally employed by clinical research and argue that, although they may be well suited to answer highly specific medical questions, these tools are inadequate to properly address the more complicated and interdependent questions related to defining effective care. We then describe a novel approach that bridges many of the gaps left by traditional clinical research tools and that is integrally linked with a continuous improvement process, an approach we call the Standardized Clinical Assessment and Management Plan (SCAMP).

A Theory-Building Framework

Every theory goes through several phases of development as knowledge about that theory accrues. Theory building can be divided into two distinct stages—descriptive and prescriptive theory building (Figure 1). After a phenomenon is observed and described, descriptive theory building attempts to define relationships between the phenomenon and attributes associated with that phenomenon through correlation studies. Causality cannot be proven through a descriptive study, but an understanding of correlation allows one to hypothesize about a causal link between an attribute and an outcome. This requires a “cognitive leap”—a jump from a proven correlation to a theory that an attribute or set of attributes actually causes the outcome of interest. Causality can subsequently be assessed through prescriptive theory building, whereby an experiment to test the theory is designed and carried out. If proven, the theory earns credibility as both an explanatory and predictive model, allowing the theory to be used to inform decision making with regard to that outcome.

Frequently, an anomaly—an outlier, diversion, or unexpected finding—can be observed in both descriptive studies (i.e., an unexpected attribute or an unexpected relationship between an attribute and an outcome) and prescriptive studies (i.e., an unexpected outcome of an experiment). Anomalies are often excluded from analyses but can be rich sources of information. They can be used to revise the underlying explanatory model, such that formerly unexpected anomalies become predictable outcomes.
perspective

Figure 1 The theory-building process. Descriptive theory building involves characterizing the relationship between a phenomenon and attributes associated with that phenomenon. A “cognitive leap” from this established relationship can then be made to formulate a theory on a causality between attributes and outcomes, which can be tested and refined through the process of prescriptive theory building. Anomalies (outliers, diversions, or unexpected outcomes) detected throughout this process can be used to revise the underlying theory. Ultimately, a proven theory can be used to create an explanatory model for the phenomenon. Adapted from Christensen CM, Carlile PR. Course research: Using the case method to build and teach management theory. Acad Manage Learn Educ. 2009;8:240–251.

The traditional clinical research toolkit

Research in clinical medicine goes through descriptive and prescriptive processes in developing an understanding of disease management. For clinical decision making to be well informed, the theories underlying that decision making should be established by strong descriptive studies and subsequently supported by prescriptive studies (Figure 2). Traditional clinical research tools used to carry out descriptive studies include case reports, cross-sectional surveys, cohort studies, and case–control studies. The only real methodology for conducting a prescriptive study and demonstrating causality in clinical medicine remains the clinical trial, which includes the “gold standard” approach of the randomized controlled trial (RCT).

In an ideal world, the results of clinical studies provide enough data to define “best practice” recommendations for disease management. Such recommendations can be disseminated through consensus statements like Clinical Practice Guidelines (CPGs), which are based on systematic review and synthesis of the medical literature as well as expert opinion. CPGs can significantly improve patient care by helping to standardize clinical care and establish quality standards. Interestingly, the recommendations of a CPG actually represent a prescriptive theory on how a patient with a particular disorder should be managed, but without outcome measurements and revision standards, CPGs are static and are, in-and-of themselves, not knowledge building.

Limitations of Traditional Research Tools

Each traditional clinical research tool (Figure 2) was developed to answer specific medical questions on selective subject populations. Although these tools have been and will continue to be useful in answering many specific disease-related questions, they have significant limitations, some of which have been poorly acknowledged by the academic medical community.

Limitations of clinical trials

Clinical trials remain the only well-developed method for conducting prescriptive studies in medicine, but they are subject to biases and confounding that can cloud their results. Trials are also extremely costly in terms of both time and money, taking years and sometimes hundreds of millions of dollars to complete. Whether such trials are cost-effective in the setting of limited funding for clinical research is thus a topic of concern. In addition, clinicians are often resistant to the results of clinical trials because of concerns over the influence of private funding and the generalizability of study results. Because of these constraints, there are some fields for which trials are nearly impossible to run and guidelines do not exist.

The challenge of continuous improvement

The dotted lines in Figure 1, which depicts the theory-building process, represent jumps and revision cycles that, ideally, would be simple transitions in carrying out and improving a study. In reality, it is nearly impossible to revise an ongoing study if new evidence or ideas surface. Instead, an entirely new study must be designed and implemented to address a change in baseline understanding, requiring a “start from scratch” mentality. Obstacles to studies must then be overcome again, making continuous improvement using traditional clinical research tools a challenge.

Longevity of results

Given the rapid pace of advancement of medical knowledge, concern arises over the longevity of clinical research results. Results from the “gold standard” of evidence generation, the RCT, can have a surprisingly short period of relevance. In one study, 23% of systematic reviews and guidelines published by the U.S. Agency for Healthcare Research and Quality had a useful average half-life of 5.8 years. Another study found that CPGs published by the U.S. Agency for Healthcare Research and Quality had a median survival of 5.5 years. In one study, 23% of systematic reviews and guidelines needed update within 2 years of publication, and the median survival time of the reviews examined was 5.5 years. Another study found that CPGs published by the U.S. Agency for Healthcare Research and Quality had a useful average half-life of 5.8 years. This short survival period compounds concerns over the difficulty of trial design and guideline formulation, as repeating
Lack of tools to identify anomalies

As previously mentioned, anomalies in clinical practice can play an important role in developing our understanding of disease management. Anomalies in patient management and outcomes can also be a valuable source of innovation in some instances. However, many studies regard anomalies as outliers and neither include them in analyses nor share them with the medical community, leading to failed recognition of potentially important insights.

Lack of operational learning

Tools like CPGs create the opportunity for a different kind of learning in medicine, operational learning, which involves implementing guidelines and protocols and optimizing organizations around providing those services efficiently.

Operational learning is also knowledge building and composed of both descriptive and prescriptive processes; however, none of the traditional clinical research tools are well equipped to examine this. It is for this reason that quality improvement and other forms of operational learning have evolved their own methodologies, separating themselves from clinical research.

Lack of facilitating the “cognitive leap”

As Figure 1 demonstrates, a clear gap exists around making the “cognitive leap” required to jump from a descriptive understanding of disease to a prescriptive experiment. Currently, making this leap requires knowledgeable individuals or organizations to identify gaps in knowledge, develop novel theories, and initiate the rigorous process of evaluating those theories. These hurdles may lead to the loss of valuable ideas. Although some have proposed the development of high-level boards to address this issue, no good system has been developed to facilitate this process.

Difficulty addressing issues relevant to today’s care

The current clinical research tool set leaves two additional gaps that are relevant to modern health care. The first is the need for economic analyses, especially given the increasing interest in controlling health care costs and optimizing resource utilization. Such analyses are difficult to conduct with traditional clinical research tools, as none of the tools were designed to address economics directly. Even when economic data exist, incorporation of these findings into guidelines is limited.

The second gap revolves around the need to address complex, interdependent medical questions. Progress in medical care has increased the population of patients with multiple complex medical problems, making the management of each individual disorder more difficult. In addition, the number of available therapeutic and diagnostic modalities has increased tremendously, amplifying the number of management options that fall within “standard of care.” These factors make studying clinical medicine today a complicated task, one that is not well addressed by tools designed to answer highly specific medical questions in highly specific patient populations.

The Need for Integration

The clinical research enterprise has struggled to come up with an effective and definitive solution to the problems it faces. Groups have suggested increasing the funding and number of trials, encouraging more physician and subject participation in clinical research, and enhancing physician compliance with guidelines as potential solutions. Although such measures are important steps, a significant oversight remains—working to improve the tools of clinical research themselves.

As Figure 2 shows, the tools available for clinical learning are modular and designed to be used independently, despite the necessary interdependencies of medical knowledge building. Although such modular tools provide researchers with flexibility in how to approach a given medical question, they are not designed to optimize the performance of knowledge generation. A tool designed to integrate descriptive and prescriptive theory building could incorporate many of the functions carried out by separate traditional research tools and might offer a powerful solution to the issues facing the health care system.

To properly address the aforementioned limitations, such an integrated tool would need to capture data to inform descriptive studies, facilitate the formulation of theory and creation of prescriptive studies, measure and analyze outcomes, and identify and learn from anomalies. Furthermore, the tool would need to function across a large and diverse patient population with complex medical problems, permit analyses of cost-effectiveness and resource utilization, and be amenable to rapid revision and continuous improvement. Not only could such a tool efficiently generate clinical knowledge but it could also work to reduce practice variation and standardize patient care, which the quality improvement literature has shown to improve patient outcomes, decrease care costs, and enhance efficiency.

The advancement of health care information technology along with the introduction of decision analysis has made the development of this kind of model feasible. We offer the paradigm of SCAMPs as an example of such an integrated medical learning tool.

What Is a SCAMP?

In pediatric cardiology, limited clinical data exist because of the small sample population.
sizes of relatively rare diseases. This lack of evidence precludes the definition of best practices, and CPGs designed for longitudinal care of children with congenital heart disease are sparse and inadequately informed. Not surprisingly, pediatric cardiology is characterized by wide institutional and individual variation in clinical practice.

Given the high degree of uncertainty behind clinical decision making and the various constraints faced in our field, we hypothesized that a new kind of clinical research tool was required to build clinical knowledge and improve patient care. We designed SCAMPS to achieve this goal. A SCAMP is a care pathway, designed around existing practice methods, that standardizes the care of a relatively diverse patient population with a particular diagnosis. It is accompanied by a systematic and robust, but also selective, data collection process. One distinguishing feature of a SCAMP is its active invitation and capture of knowledge-based clinician diversions from its protocol, which are perceived to be a rich source of information and innovation. On the basis of frequent periodic review of collected data and diversions, a SCAMP undergoes progressive modification of its care delivery algorithm.

The development of a SCAMP is carried out by a multidisciplinary team of physician and nursing experts assisted by data coordinators and biostatisticians. The SCAMP process is characterized by the following eight steps:

1. Establish a foundation for sound clinical practice through literature review to compose a background position paper on a particular disorder and, if necessary, conduct a focused retrospective study to analyze current practice.
2. Formulate plausible findings, or aim statements that address known gaps in knowledge regarding the management of the disorder; these become the focus of targeted data collection.
3. Build expert consensus on the entry criteria, assessment recommendations, and management algorithms (decision trees) for the SCAMP.
4. Develop data forms and IT tools that facilitate implementation of the SCAMP by providing management recommendations, in addition to collecting targeted clinical information and reasons for clinician diversions.
5. Enroll patients in the SCAMP, which requires both proper identification of eligible patients and supplying the necessary data forms to providers at the level of the patient encounter.
6. Enter targeted clinical data (collected from data forms and extracted from the electronic medical record) and reasons for clinician diversions into a database.
7. Analyze SCAMP data and diversions, using statistical approaches along with best clinical judgment, to assess the clinical and cost-effectiveness of recommendations.
8. Periodically and iteratively revise the SCAMP based on this analysis and relevant updates from the medical literature.

In practice, a SCAMP exists as an electronic or paper form (Supplemental Digital Appendix 1, http://links.lww.com/ACADMED/A235) provided to a clinician at the point-of-care. The clinician uses the form during the patient encounter to collect relevant patient information and guide his or her medical decision making. If the clinician decides to divert from SCAMP recommendations, this diversion and the reasoning behind doing so are captured. Data collected from both the form and the medical record are analyzed by statisticians, and the data are subsequently used by the committee in charge of the SCAMP to modify and iteratively improve the SCAMP algorithm. In this way, every SCAMP patient encounter becomes an opportunity for learning, and each SCAMP provides care recommendations which are kept up-to-date through a continuous improvement process.

Inference forms and IT tools to divert from SCAMP recommendations, which are not only permitted but also encouraged in order to engage clinicians and access innovation from everyday practice. SCAMPS also have the ability to address multiple clinical questions simultaneously, and interplay between different SCAMPS can allow for the examination of complex medical problems. Additionally, by collecting data on compliance and resource use, SCAMPS create a system for operational learning and the examination of cost-effectiveness.

Last, and perhaps most important, the SCAMP review and modification process prioritizes continuous improvement, allowing for rapid revision of SCAMP algorithms and up-to-date recommendations. Through the efficiencies generated by an integrated approach, we estimate that the marginal yearly cost of SCAMP implementation is approximately $50,000 (depending on scope), a number which compares favorably to the costs of conducting a clinical study or updating an existing guideline.

**Progress with SCAMPS**

To date, over 15,000 patients have been enrolled in 50 SCAMPS across a network of over a dozen pediatric and adult centers. SCAMPS have been developed for all aspects of clinical care including outpatient, inpatient, operational, and procedural settings. They span the management of both rare and common diagnoses, and both specialty and primary care practices. Twenty-two SCAMPS have undergone periodic review and improvement, demonstrating the efficacy of the SCAMP modification process.

**Enhancing Research and Shaping Practice With SCAMPS**

**A successfully integrated approach**

As Figure 3 demonstrates, a SCAMP, in contrast to traditional clinical research tools, can be considered an integrated model of clinical learning. Each SCAMP encompasses both descriptive and prescriptive theory building through its design and implementation. After recognizing key areas of uncertainty in practice (identified as plausible findings), a SCAMP captures targeted data on a diverse set of patients (descriptive theory building) and proposes management recommendations that are studied by collection of information on compliance, outcomes, and anomalies (prescriptive theory building). The so-called “cognitive leap” is intrinsically built into this process.

The integrated nature of SCAMPS also addresses many of the aforementioned limitations imposed by traditional research tools and CPGs. Anomalies are acknowledged through the capture of diversions from SCAMP recommendations, which are not only permitted but also encouraged in order to engage clinicians and access innovation from everyday practice. SCAMPS also have the ability to address multiple clinical questions simultaneously, and interplay between different SCAMPS can allow for the examination of complex medical problems. Additionally, by collecting data on compliance and resource use, SCAMPS create a system for operational learning and the examination of cost-effectiveness.

The explicit goals of SCAMPS are threefold: to reduce clinical practice variation, to optimize resource utilization, and to improve patient care. The standardization
Figure 3 Fitting the Standardized Clinical Assessment and Management Plan (SCAMP) process into the theory-building framework. The process of designing and implementing a SCAMP is intended to span across both descriptive and prescriptive theory building.

The network of institutions involved with SCAMPs continues to expand. This spread not only works to increase vastly the breadth of data collection and evidence generation but also demonstrates the broad applicability, scalability, and collaborative nature of SCAMPs. It is also an important validation of the buy-in that SCAMPs have achieved among clinicians. In an anonymous survey at our institution, providers preferred SCAMPs as a means to incorporate evidence-based medicine into their practice over CPGs, care pathways, or other protocols. Furthermore, the implementation of some SCAMPs has demonstrated substantial improvements in patient care.

SCAMPs have also found support from payers, with a consortium of insurance providers (Blue Cross Blue Shield of Massachusetts, Tufts Health Plan, Harvard Pilgrim Health Care, and MassHealth) providing early and important funding to the initiative. The popularity of SCAMPs with multiple stakeholders in the health care system is an important validation that the principles behind SCAMPs resonate well within the culture of medicine.

A disruptive innovation?
As a novel integrated clinical research tool, a SCAMP might be viewed as a disruptive innovation within the clinical research enterprise. SCAMPs address unique research questions not addressable by traditional tools and can efficiently generate high-level evidence meeting or exceeding the standards of clinical research. In addition, while SCAMPs function similarly to CPGs in their ability to standardize care, they offer much more flexibility than CPGs and can undergo continuous improvement. Finally, because SCAMP recommendations fall within the standard of care, a SCAMP is considered a quality improvement effort and is exempt from the institutional review required for traditional research activity.

As experience with SCAMPs continues to grow, SCAMPs have the potential to become part of a medical learning system that will augment or perhaps at times supplant the functionality of traditional clinical research. We anticipate that for some questions, variability in disease course and in biologic measures will prevent SCAMPs from drawing inference on the basis of uncontrolled clinical observation. Nevertheless, SCAMPs will help highlight these areas of uncertainty and identify questions better addressed by clinical trials.

Concluding Remarks
Integrated medical learning tools like SCAMPs have the power to address two major issues simultaneously. First, they represent an improvement over traditional clinical research tools through their ability to efficiently generate the clinical and operational knowledge needed to define standards of effective care. Second, they offer a practical and well-accepted methodology to standardize clinical practice, thus optimizing resource use while improving patient care. That these issues can be addressed by a single integrated tool may be an important advancement in the effort to build a safer, higher-quality, and more cost-effective health care system.

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