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Review

Towards a learning system for pediatric outcomes: Harvesting meaning from evidence

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ABSTRACT

Quality measurement in pediatrics is challenged by a system that lacks a fundamental data infrastructure for children's healthcare in general and in particular in cardiomyopathy. We suggest that the thoughtful application of mixed health services research methods can serve as powerful tools for applied research that supports a priori thinking, which in turn can drive both prospective studies and the analyses of retrospectively collected data within the schemas of strong quasi-experimental designs. This can provide the means for transforming practice into evidence, and practice within uncertainty into deep knowledge. The process of learning is iterative and typically incremental, constantly being infused by every day work experience and hard-earned lessons by clinicians providing clinical care. Developing sustainable learning towards improved outcomes in pediatric cardiomyopathy can be done using the applied science of health services research to translate clinical practice into research.

1. Introduction: Through a Quality Lens

The history of quality measurement in pediatrics has been one of fits and starts. In the 1930's, the American Public Health Association raised concerns about the overuse of tonsillectomy in New York City [1,2]. In 1975 the American Academy of Pediatrics' (AAP) published their groundbreaking efforts to develop a systematic approach to measuring the quality of pediatric conditions [3]. Landmark studies on the appropriateness of pediatric hospitalization and surgical procedures appeared in the late '80's and early '90's [4–6]. Articles published at that time suggested that one quarter to one third of procedures were inappropriate and stimulated a public outcry, leading to conceptual and management challenges [4–10]. In 1997 the "Four D" model articulated the need for distinctions between children and adult quality measurement metrics, and for funding to support child health services research, including quality of care research. The model described four fundamental challenges for assessing children's health care as critical: Changing Developmental status of children; Differences in the epidemiology of disease between children and adults; Dependence of children upon their caregivers; and, the Demographic patterns typical for families with children [11].

In recent years, the autonomy of the medical profession has been challenged as system transformation moves us along a path in theory focused on co-production of optimal outcomes, provider accountability and system value, all while maintaining or increasing productivity and profitability [12]. The Child Health Insurance Program Reauthorization Act (CHIPRA, 2009), created the Pediatric Quality Measures Program (PQMP), which built on two decades of quality measurement efforts [12–23]. This represented the first large scale, focused investment in assessing the quality of child health care issues. At the outset, the CHIPRA mandated the development of a core set of validated measures for Medicaid required undesirable compromises. Large swaths of clinical pediatrics lacked scientifically sound process or outcomes measures. The science of measurement of pediatric health remains in need of thoughtful development that will frame the purpose, the philosophy, and the methods to be developed. Beneficiaries of such efforts will include physicians, patients, payers, and society

Quality measurement is also challenged by a system that lacks a fundamental data infrastructure for children's healthcare. The system doesn't have the financial drivers for dedicated investment in pediatric research as is commonplace for expensive care for adults, and by a paradigm that emphasizes short term benefits even though small changes now may yield larger benefits over time. Further structural issues include a lack of comparative effectiveness research to clarify the relative benefits of various clinical approaches, and a lack of innovation in accommodating appropriate variations in care. Good variation may include those driven by a responsiveness to patient preferences. Clinicans hesitate to address quality topics related to overuse of clinical

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interventions in pediatrics [4].

We find ourselves at an existential time in healthcare, with business models and public trust eroding. There is a contrast between the increasing demand for accountability, limited opportunities to assess the comparative effectiveness of many pediatric interventions and the insufficiency of resources for the research that is needed in many areas of care, including care of children with cardiomyopathies [25]. While leading authorities call for the development of a learning health care system, the types of measures that can support broad learning in pediatrics are lacking [26,27].

2. Epistemological Framework

All clinicians understand that there are things we do not know and things we cannot predict. Uncertainty is pervasive in medicine. Philosophers describe epistemic uncertainty, in which the scientific basis is insufficient to fill all the gaps with knowledge, and aleatory uncertainty, which often is described as stochastic uncertainty and presented as probabilities [28,29]. We define clinically meaningful uncertainty as implying that well informed and well intentioned clinicians may disagree regarding the preferred approach. Outcomes and processes are sometimes insufficiently aligned for our preference as in the adage: “the operation was a success, but the patient died.”

What we do as clinicians depends upon what we know and how we know it. It must make clinical sense or clinicians will resist change or work around the new processes [30]. Epistemologists have articulated three prerequisites for knowledge–truth, belief, and justification [31]. When we know something we experience a true justified belief. In the context of clinical medicine, this is complicated by the changing nature of what we classify as truth. We develop what we believe by what we experience as justified by some sort of evidence, be it anecdotal, referential or scientific. We live in a clinical environment that is informed by a science that is ever changing. In Karl Popper's language, we generate corroborating evidence by making rigorous and unsuccessful attempts to disprove theories [32]. The evidence accumulates for the alternative hypotheses over time. But the accumulation of knowledge is typically not symmetrical. Randomized trials generate strong evidence about very narrow groups of individuals who are enrolled in tightly and detailed controlled trials. Study subjects typically are enrolled under very restrictive conditions that are mostly at odds with real world conditions and constraints. New alternative approaches, such as observational and quasi-experimental designs and adaptive clinical trials are more relevant for studying real world questions, such as describing patient safety risks, faulty diagnoses by physicians, lack of knowledge transfer during patient handoffs, and issues with teamwork in high-stress, fluid, and dynamic clinical environments. These newer study designs can generate evidence that are more sensitive to nuance and context, and thus may have broader applicability across clinical populations but they are subject to a variety of validity and reliability threats. The insufficiency of the pediatric evidence base is not surprising as there are fewer pediatric studies than adult focused research that produce germaine evidence (Fig. 1). For example, the unique evidentiary challenges in the study of off-patent drugs in children remains an overlooked public health priority [33]. One of the major challenges to conducting drug, policy and service intervention trials in children is the relatively small number of eligible patients and of validated surrogate endpoints. Meaningful outcomes related to pediatric care may emerge decades after the event [34]. This has several key implications for research: prospective studies are very expensive and pose major logistical challenges, and, the passage of time allows for exposure to competing causes of the outcomes of interest, which reduces the capacity to identify true signal and requiring larger sample sizes. These reasons may explain why so few RCT trials of children with heart failure, for example, have been completed, and none have shown improved efficacy [35]. Clinical outcomes may not be suitable primary end points for policy and health-service interventions because the effects can be too diffuse to detect in pediatric trials [36].

2.1. Clinician's Dilemma: “How do I treat the patient in front of me when there exists meaningful uncertainty about what to do in the real world context for this actual patient?”

The Institute of Medicine's definition of quality acknowledges several types of uncertainty [37][1]. It is probabilistic, reflecting aleatory uncertainty, and grounded in current understanding, reflecting the epistemic uncertainty. The definition suggests a gradient (“degree”) of quality rather than a dichotomy of good and bad, which is consistent with how the field accrues knowledge and the emerging nature of complexity. (Fig. 2) The Evidence-Based Medicine (EBM) movement represents a paradigm shift away from what has been termed the scientific Medical Model to a more statistical and epidemiological one. A critical shortcoming of the EBM approach is that it frequently does not help the physician treat the patient in front of her when meaningful uncertainty exists about what to do for your patient. The patient has comorbidities and many circumstances that would have excluded them from the definitive clinical trial and in contexts that differ from those of the clinical trials. Furthermore, investment in clinical research often follows cost, disadvantaging populations with rare diseases (such as pediatric cardiomyopathies), those who relatively are not expensive to treat (such as children), and those who will die quickly or prior to treatment. The field of comparative effectiveness research has ascended largely because of such compelling ethical, societal and scientific challenges to our understanding of what constitutes optimal management for whom. As acknowledged in a report commissioned by the Assistant Secretary for Planning and Evaluation, “Many small populations have distinctive health and health care needs but have been difficult to study” [38].

For all of its universality, knowledge and uncertainty are not well understood [28]. Recent discussions of knowledge as explicit (experienced as knowing) and tacit (useful and practical, but not perceived as explicit understanding) are useful when considering the practice of medicine for individuals versus that for populations. Understanding heuristics or the ‘clinical gut’ is a manifestation of successfully integrating a scientific base with personal and second-hand experiences, as well as the personal experience of the patient (“she just looked sick to me”). The art of medicine ought to lie in the skillful application of tacit knowledge by well-prepared clinicians grounded in deep familiarity with patterns of sickness and informed by the existing evidence regarding diagnosis and treatment. Such tacit knowledge has been captured and described (although not without debate) at the level of the clinical practice [39,40]. Still, as a professional tennis player struggles to convey what “feel” or “touch” means to them, so it is not possible to simply transmit clinical judgement in declarative statements, guidelines and statistics. Approaches that build upon stakeholder engagement or expert consensus, like appeals to the wisdom of crowds, are better suited to capture tacit knowledge at higher levels of aggregation [41]. At one level, “reflection-in-action” by the physician is the ability to think while acting and act while thinking. All of us have this ability and use it every day; few of us become aware of it, fewer still use it to improve their practice, and fewer still do it as part of a workplace group, such as in a clinical microsystem team. Similarly, situational awareness is our capacity to identify the presence of (or deviations from) normal patterns while sensitive to the stimuli around us [42].

Impactful research begins with a worthwhile and ethical question and can help build a framework for a learning system built upon clinically meaningful research. We have identified the context of uncertainty as creating something we call the Clinician's Dilemma, defined

[1] The degree to which health services for individuals and populations increase the likelihood of desired health outcomes and are consistent with current professional knowledge [34].