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CAPS Presidential Address

The value of patient registries in advancing pediatric surgical care

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ABSTRACT

Pediatric surgeons treat a variety of conditions that are distinguished by their low occurrence rate, complexity, and need for integrated multidisciplinary care. Although randomized controlled trials (RCTs) are considered the gold standard for generating evidence to inform best practice, they are poorly suited to rare diseases based on the variability of illness severity, unpredictability in clinical course, and the impact limitations of studying a single intervention at a time. An alternative to RCTs for comparative effectiveness research for rare diseases in pediatric surgery is the patient registry, which collects detailed and condition-specific patient level data related to illness severity, treatment, and outcome, and allows a large, disease-specific database to be created for the dual purposes of collaborative research and quality improvement across participating sites.

This review discusses the various functions of a patient registry in fulfilling its mandate of evidence-based practice and outcome improvement using examples from a variety of existing pediatric surgical registries. The value proposition of patient registries as sources of knowledge, facilitators of practice standardization, and enablers of continuous quality improvement is discussed.

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Members of CAPS, distinguished guests, ladies and gentlemen. It gives me great pleasure to welcome you to the 49th Annual meeting of the Canadian Association of Pediatric Surgeons, which falls in the 150th year following Canada's confederation. This year we are in Banff National Park in the heart of the Canadian Rockies, which ranks among the most beautiful spots in Canada, if not the world.

In surgery, advances in surgical care bring improved outcomes for patients. We speak frequently about surgical innovation, which is the act of introducing something new or the development of a new technology or technique. And it goes without saying that innovation driven by single minded surgeons, often as “aha” moments has been responsible

Abbreviations: CAPSNet, Canadian Association of Pediatric Surgery Network; RCT, randomized controlled trial; CDH, congenital diaphragmatic hernia; CDHSG, Congenital Diaphragmatic Hernia Study Group; ACS NSQIP, American College of Surgeons National Surgery Quality Improvement Program.

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for dramatic improvements in pediatric surgical care over the first 49 years of CAPS.

Think of these 3 seminal contributions to pediatric surgical care. Siggie Ein and Donald Marshall's realization that placement of a penrose drain into the abdomen could rescue a desperately ill premature infant with NEC [1], Jack Langer's concept of a transanal pullthrough for Hirschsprung's disease [2], and most recently, Tony Sandler's discovery that the healing powers of the newborn umbilicus made surgery for gastroschisis unnecessary [3].

Today, I am going to discuss the "innovation" of observational clinical data organized into patient registries, and how the systematic collection and analyses of these data can create an evidence platform for driving and sustaining care improvement in pediatric surgery. Fig. 1 summarizes the virtuous cycle of knowledge or evidence translation which begins with knowledge creation, its subsequent mobilization into "best practice", and the incremental improvement in treatment and outcomes which are driven by the field of implementation science. For the purpose of describing the role of patient registries in supporting this cycle, I'm going to focus on four phases: 1) knowledge synthesis; 2) knowledge standardization via best practice guidelines; 3) outcomes comparison through benchmarking; and 4) sustainability of change through continuous improvement.

Most of us are familiar with the Evidence Pyramid (Fig. 2A), which ranks evidence according to quality with randomized controlled trials (RCTs) and metaanalyses of RCTs at the top. The majority of the pediatric surgical literature available to inform best practice exists in the lower strata of the evidence pyramid in the form of single institution cohort studies. The rarity and complexity of many of the conditions we treat mean that even larger institutions may take years to acquire sufficient cases, and that care standards and outcomes may change significantly over that time period. Multi-institutional studies are a desirable alternative, and although RCTs offer the highest level of evidence, this approach is limited by the rarity and complexity of many of the conditions we treat, where isolating and evaluating a single intervention at a time, using RCT methods alone are difficult, if not impossible. And there are barriers to performing RCTs that are unique to surgery in general, and pediatric surgery specifically. How do we guarantee standardization, equivalency and equipoise amongst surgeons who must randomly perform one operation or another in the context of a trial? Patients (and especially parents) may be unwilling to participate due to loss of autonomy particularly when the risks of the two interventions being compared may be quite different. The lack of generalizability of findings beyond the inflexible trial conditions is another major concern. Given the time, effort and expense associated with completing RCTs, a strong case can be made in favor of a more pragmatic approach with fewer patient exclusions, so that the results are more likely to have "real world" application.

This brings us to another pyramid which is referred to as the "Data, Information, Knowledge, Wisdom Hierarchy, or simply the knowledge pyramid (Fig. 2B). The concept here is that a data collected during the delivery of patient care can be organized and analyzed to allow the transformation of clinical measurements into knowledge and wisdom, informing how best to treat a future, but similar patient cohort. This is the foundational concept behind patient registries as knowledge sources capable of improving patient care.

1. What are patient registries?

Patient registries are defined as a collection of standardized information about a group of patients to be used for a predetermined scientific, clinical or policy purpose [4]. There is an expectation of maximizing data through multi-institutional collaboration, and there is the potential to link biospecimens as a means of supporting basic science research. Increasingly and appropriately there are a demand and an expectation for patient engagement—giving patients a voice in what data should be collected and for what purposes it should be used [5]. It is important that data variables and particularly outcomes, be developed by the clinical end user, and it is the reason that administrative datasets cannot substitute for registries in answering most clinical questions. Data collection techniques vary between registries, ranging from active to passive data abstraction, voluntary submission of datasheets, and questionnaires, sent to physicians or patients (or both) at various stages of treatment and follow-up. Patient privacy is essential, and is protected, sometimes to the point of limiting the usefulness of the registry. This is especially true for rare disease registries where the risks of identity disclosure are higher, and are offset by avoiding any potential identifiers, including date of birth, in deidentified datasets.

Patient registries are frequently part of another structure called the "Clinical Research Network" (Fig. 3). While registry data are central to the network, its stakeholders, including clinicians, researchers, hospital administrators, health service policy makers and patients and their families set priorities for how the data are used. Clinical research networks have an essential infrastructure which supports activities of the network, including data management, project management, and access to the necessary biostatistics and health economics expertise to conduct methodologically rigorous research and then disseminate the results.

Fig. 4 demonstrates the international distribution of established disease-specific pediatric surgical registries. Congenital diaphragmatic hernia (CDH) and biliary atresia are the focus of several registries. Some of these registries including the Japanese and Canadian registries, seek to be population-based, which allows these registries to become data sources for studies of disease incidence and epidemiology. Sweden, which has a universal access healthcare system is unique in its registry capability. Virtually every Swedish citizen is part of an integrated national health database that collects standardized data on demographics, provider characteristics, detailed summaries of treatment including drug prescriptions, and long-term outcomes, both clinical and patient-reported. There are more than 90 national registries in Sweden which track an array of health issues, with an annual government funding commitment of $50 million [6]. The Congenital

Fig. 1. The virtuous cycle of Knowledge Translation.