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Barriers to participation in surgical randomized controlled trials in pediatric urology: A qualitative study of key stakeholder perspectives

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Summary

Introduction
Randomized controlled trials (RCTs) are considered the gold standard for assessing treatment efficacy. However, pediatric surgical RCTs have been limited in their ability to recruit patients. The purpose of this study was to identify barriers and motivators to pediatric participation in surgical RCTs.

Methods
We conducted a series of two focus groups with parents and one focus group with urology providers for children aged <2 years of age with a diagnosis of Society for Fetal Urology grade 3 or 4 hydronephrosis. We then administered a survey to referring pediatricians based on the initial analysis of focus group findings. Theme analysis was used for all qualitative transcribed text data obtained from focus groups and open-ended survey questions using team-based inductive approaches. Descriptive statistics were obtained for the remainder of the provider survey.

Results
Using qualitative text from stakeholders ($n = 38$) we identified four key themes across the data: *responsibility to my child*; *responsibility to my patient*; *responsibility to the field*; and *irreversibility of surgery*. Participants felt there was an obligation to be informed of relevant scientific research within a clinic research culture. However, there remains a disconnect for parents between randomized research studies that may ultimately benefit their child, depending on their age and concern their child is being treated as a 'guinea pig'. Some parents were willing to participate in RCTs but all were more open to participate in an observational study where the treatment decisions were felt to be under their control even when there was no "right answer" or multiple equivalent options for treatment. There was mixed opinion across the parents and providers whether research trial education and enrollment should be provided by the pediatrician or urologist. Active physician decisions were seen as critical within the context of a long term clinical relationship and provision of information of risks and benefits without pressure were considered essential for ethical research by both parents and providers.

Conclusion
While some parents are open to participation in surgical RCTs, providers and parents of children with hydronephrosis feel discomfort with the element of chance in surgical randomized trials. Parents and providers are more likely to participate in observational studies where treatment decisions may be made jointly by the physician and the parent. These findings suggest that pragmatic trial strategies with the option for participation in an observational cohort may improve recruitment of pediatric patients into surgical clinical trials.

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Table Perceived barriers to participation in surgical randomized controlled trials.

	Parents	Pediatric urologists	Pediatricians
Counseling and decision-making	Loss of control over treatment decision Child's inability to participate in decision	Discomfort with admission of uncertainty in treatment Time and effort needed for education regarding RCTs	Ethics of randomization in young children/lack of patient input into decision
Complications and risks	Stigma of child being labeled as "sick" Concerns about cost and long term complications	Adequacy of safeguards and justification of potential risks	Potential risk of sham surgery/anesthesia exposure Risk of side effects, unnecessary tests, and unknown long term consequences
Unique attributes of surgery	Irreversibility of surgery	No difference between medical and surgical RCTs	Lack of true equipoise between surgical and non-surgical treatment arms Irreversibility of surgery

Introduction

Randomized controlled trials (RCTs) are the gold standard for determining treatment efficacy. Surgical trials, however, are limited by the ability to recruit patients, with less than 1% of eligible patients accrued [1]. This limitation is even more significant in pediatric surgical trials [2]. Perceived barriers to participation in adult surgical RCTs include provider concerns, patient preferences, and availability of treatment outside the study setting [3]. To date, however, no study has assessed the acceptability of surgical randomization or the barriers to participation in the pediatric surgical setting.

The purpose of this study was to identify barriers to participation of children in surgical RCTs and to identify what barriers, if any, are unique to surgical RCTs compared with medical RCTs. The rationale for this study is that improved knowledge of parent and provider-identified concerns regarding randomization of surgical treatment would provide valuable information about how to improve study design, recruitment, and participation in pediatric surgical clinical trials. We hypothesized that parents, surgeons, and referring providers are more willing to participate in randomization of medical therapy compared to surgical therapy and that primary barriers to surgical RCTs would differ across these groups.

Materials and methods

Study design

To evaluate parent and physician attitudes to participation in surgical RCTs, we utilized a constructivist framework [4]. Constructivism assumes that individual experience holds knowledge that is a product of the knower (person) and the known (topic of interest); individuals draw from patterns of their experiences through reflection to come to their own interpretations of situations and are considered by the researcher to be constructed and valued in the context in which they are shared. A two-part, emergent qualitative exploratory design was chosen to allow for both in-depth qualitative data description and iterative characterization of factors influencing surgical randomization as perceived by the primary stakeholders in this decision: parents, pediatric urologists, and referring pediatricians. This approach was chosen to develop a comprehensive picture of pediatric RCTs that taps into the unexpected intangibles that matter to stakeholders [5].

Participant sample

A volunteer sample of parents and providers was sought at the Children's Hospital Colorado (CHCO) for participation in focus group discussions. Parents were eligible to participate if they were a primary or secondary caregiver of a child aged <2 years with Society for Fetal Urology grades 3–4 congenital hydronephrosis initially seen by the pediatric urology department between 2010 and 2012 with >1 year of follow-up. Recruitment was conducted via mailed flyers and postings in the pediatric urology clinic and on the CHCO website. Regional pediatric urologists were invited to participate by phone and mailed invitation. Surveys were administered to regional referring pediatricians at an annual pediatric conference at CHCO and were informed by themes elicited via focus group discussion to tap into individual-level physician experiences rooted in practice.

Data collection

Focus group interviews of four to eight participants were conducted to allow for consideration and modification of individual perspectives of issues based on group interaction. To avoid undue influence or power differential between parent and provider, parent and provider focus groups were held separately [4,6]. Focus groups were facilitated by an expert in qualitative methods (J.J.) with no personal association with participants. Goals of facilitation were to enhance participation by all members, moderate group dynamics and enable in depth probing of emerging themes. Semi-structured interview guides (Appendices I and II) were developed to elicit perceived risks and benefits of participation in a randomized study based on prior literature [1,3]. Questions were designed to