Pediatric Robotic versus Open Pyeloplasty: A Pilot Randomized Control Study

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Specific Aims

Technological innovations are an ongoing reality in clinical medicine, most dramatically in the operating room. Each advance is often heralded as an improvement in patient care, promising to correct deficiencies and advance standard operative procedures. Unfortunately, these new technologies often lack sufficient evidence to support such claims. Robotic assisted laparoscopic (RAL) surgery continues to gain popularity among pediatric surgical specialists, particularly for reconstructive procedures such as correction of ureteropelvic junction (UPJ) obstruction or vesicoureteral reflux (VUR).[1,2] In these operations, where traditional open surgery yields a nearly 95% clinical success rate,[3-6] it is difficult to demonstrate improvement with a new surgical technique. Despite these impressive outcomes with the open approach many pediatric urologists are shifting towards newer RAL approaches, claiming faster recovery[7-9] and improved cosmesis[10] with a nominal increase in surgical cost.[11] To date, no randomized controlled trials exist in the pediatric population comparing robotic to open surgery. However, this is not unusual. There is a striking dearth of comparative effectiveness research in pediatric surgical specialties, especially given the wide range of available therapies for a variety of surgical problems. Conducting randomized controlled trails of surgical interventions can be very difficult, especially in the pediatric population. One barrier frequently encountered is difficulty recruiting patients and families to participate in a randomized controlled trial (RCT) that involves surgical procedures.[12-16] Patients and their families often reject RCTs, because they do not wish treatment to be decided by chance, the family is concerned that the doctor-patient relationship is affected by participation in a RCT, or the parent may have feelings of personal responsibility if the treatments are unequal.[12-14] In order to use RCTs to determine treatment superiority, appropriate and meaningful outcomes must be identified and measured.

Currently, all studies of pediatric robotic surgery have focused on traditional outcomes, e.g., length of stay, pain scores, pain medication usage, scar perception, cost analysis, and capital gains benefits.[7,8,10,17-19] While these measures are important to study, we believe that researchers must also focus on patients' treatment experiences or patients' evaluations of their own outcomes. By focusing on these 'patient-centered outcomes' we will be able to improve outcomes from surgical procedures in ways that are most important to patients and their families.[20] The field of urology has a tradition of patient engagement in surgical outcomes,[18,21,22] but in general, patient and family input into outcomes measured in surgical clinical trials has been sparse.

We propose a pilot randomized controlled trial comparing robotic and open techniques for surgical correction of congenital UPJ obstruction (pyeloplasty) in pediatric patients. This study will serve as a proof of concept trial to demonstrate feasibility of recruiting pediatric patients to participate in a randomized study for surgical procedures and delineate patient-centered outcomes. Should this study prove randomization is feasible, we will seek funding to conduct a large, randomized comparative effectiveness trial with sufficient power to determine whether open or robotic-assisted pyeloplasty has superior patient-centered outcomes.

We propose to accomplish these goals with the following Specific Aims:

Aim 1: Utilize novel patient-centered research methods to:

- Identify outcomes of importance to patients and families related to pediatric pyeloplasty.
- Determine patient and family preferences regarding acceptability of randomization and blinding of pediatric surgical patients and construct a recruitment approach to achieve enrollment.

Aim 2: Conduct a randomized pilot study with goal of enrolling ten to twenty pediatric patients (age 2 – 8 years) to either open or robotic pyeloplasty for treatment of primary UPJ obstruction.

Sub Aim 2a: Collect preliminary data regarding the patient-centered outcomes identified in Aim 1.
Sub Aim 2b: Evaluate the effectiveness of the recruitment approach for randomized surgical studies developed in Aim 1.

We believe that our use of innovative and novel patient-centered research methods will lead to a significant breakthrough in developing a recruitment approach that is both acceptable to pediatric patients/families and optimizes their willingness to participate in RCTs of surgical interventions. Additionally these same innovative research methods will allows us to explore what outcomes are meaningful to patients and their families, which

will hopefully allow us to counsel patients and families in a way that maximizes their chances of achieving patient- and family-centered goals.

A. Significance

A.1. Congenital Ureteropelvic Junction (UPJ) Obstruction

Ureteropelvic Junction (UPJ) obstruction is a common diagnosis in pediatric urology, occurring in 1 in 500 births, and requires surgical correction in one third of cases.[23] UPJ obstruction is characterized by impaired urine flow from the renal pelvis into the proximal ureter, resulting in dilation of the collection system and potential renal damage. *Intrinsic UPJ* obstruction stems from any combination of narrowing or kinking of the ureter, and congenital abnormalities of the ureteral wall or lumen, which then results in poor urine flow across the UPJ. Intrinsic UPJ obstruction is flow-dependent – once a threshold flow is reached, an obstruction is induced. *Extrinsic UPJ* usually results from anomalous vasculature of the lower kidney, which causes proximal ureteral compression, resulting in intermittent obstruction.

A.2. Treatment Options for UPJ Obstruction

The three treatment options for UPJ Obstruction are described below:

A.2.1 Open Pyeloplasty (OP) – Traditionally considered the gold standard for treatment of UPJ obstruction, OP has a success rate ranging from 90 – 100%.[24,25] This approach requires a flank incision ranging from 2 to 6 cm in length in children. The renal pelvis and ureter are dismembered; the obstructive segment is usually excised and reconstructed to create a funneling into the ureter. Patients are hospitalized for 1 – 2 days and are discharged with an external catheter or an internal ureteral stent.

A.2.2 Laparoscopic Pyeloplasty (LP) – This minimally invasive technique utilizes the same principles as OP, but is performed intracorporally using trocars and laparoscopic instruments. Usually three or four separate, small incisions (~1 cm) are used. Long-term success rates have been shown to be identical to OP with similar costs; however, patients usually experience shorter hospitalization, but longer operative times.[17,20,26-29] Patients are hospitalized typically for 1 day and discharged home with either an external catheter or internal ureteral stent as in OP. LP has failed to be widely adopted because of the high technical demand and steep learning curve, especially in pediatric patients who have decreased operative working space.

A.2.3 Robotic-Assisted Laparoscopic Pyeloplasty (RALP) – RALP is an extrapolation and advancement of LP, providing a solution to the technical demands and steep learning curve of LP.[30-32] RALP provides a 3D, magnified image, with increased instrument freedom and articulation. The surgeon sits at a console and his movements are transmitted to the intracorporal robotic arms. Three or four small incisions are utilized to place the robotic arms, all less than 1.0 cm. Rather than placing the in incision on the flank, as is done with OP, RALP incisions can be concealed in the umbilicus and on the lower abdomen. These patients are typically discharged the day after surgery with an internal ureteral stent. Robotic surgery has revolutionized the surgical landscape, offering the ability to perform increasingly complex operations through a minimally invasive approach without the significant learning curve associated with a pure laparoscopic surgical approach.[30-33] In fact, recent data show decreased utilization of LP, as RALP becomes more common.[1,34]

A.3. Standard of Care: Robotic vs. Open Pyeloplasty

In adults, a robotic approach has become the standard of care; however, in children a dichotomy exists. At some institutions, children as young as 6 months[35,36] undergo a robotic procedure, while at other institutions, children as old as 18 years old still have open procedures.[1,2,34] Few studies comparing pediatric RALP and OP clinical outcomes exist; none are randomized. However, the following has been shown:

- Current literature suggests long-term outcomes between RALP and OP are equivalent.[8]
- RALP patients require less pain medication compared with OP.[8,37]
- Nearly two-thirds of families would prefer robotic surgery scars.[10]
- Shorter mean hospitalization for RALP, typically by an entire day (2.3 vs. 3.5 days, respectively).[8]

No clear consensus exists as to whether robotic or open pyeloplasty should be the standard of care. The only way to resolve this issue is to conduct a randomized controlled trial in which outcomes that matter to both patients and surgeons are considered. However, two clear issues must be overcome in order to succeed. First,

we need to determine what measures and outcomes truly matter to patients, as well as physicians. Second, we need to insure that the many factors that have impeded prior RCTs of surgical procedures are overcome.

A.4 Patient-Centered Outcome Measures
Studies of surgical procedures have tended to focus on clinical outcomes (e.g., length of stay, pain control, complications). While these types of 'traditional measures' are clinically important to ensure safety and efficacy of surgical procedures,[38] they do not always take into account the perspective of the patient or family. Research has demonstrated that patient experience and clinical quality/outcomes are distinct entities.[39] We believe that researchers must pay attention to patients' treatment experiences or patients' evaluations of their own outcomes. By focusing on these 'patient-centered outcomes,' we will be able to improve outcomes from surgical procedures in ways that are most important to patients and their families.[20]

In the field of urology there has been sparse attention to patient- and family-centered outcomes in clinical trials.For example, studies of RALP have mostly used generic research instruments to assess preoperative and postoperative quality of life and scar perception after open and robotic pyeloplasty.[10,40] While these studies provide useful data, they lack patient and family input into the creation of these research instruments and identification of outcomes that are meaningful to the patients and their families. In order to conduct an accurate, useful comparative effectiveness study aimed at determining which surgical modality is superior for treatment of pediatric UPJ obstruction, we must identify and collect data on patient/family-- outcomes.

A.5. Difficulties of Conducting Randomized Controlled Trials in Surgery
There are few RCTs comparing surgical techniques in pediatric surgery [41] as these studies can be difficult to conduct in the pediatric population.[42] One barrier frequently encountered is the challenge of recruiting patients.[12-16] Surgical RCTs are often discontinued early due to slow or poor recruitment.[43] Patients and families often reject RCTs because they do not wish treatment to be decided by chance, they have a preference regarding which procedure they want performed, they may be concerned that the doctor-patient relationship could be affected by participation in the study, or the parent may have feelings of personal responsibility if the treatments were found to be unequal.[12-14]

B. Innovation
This study will help us to overcome the barriers identified in Section A, to conduct a proof of concept trial to demonstrate feasibility of randomization, delineate patient-centered outcome measures, and provide the framework for a large randomized controlled trial comparing robotic and open techniques for surgical correction of congenital UPJ obstruction (pyeloplasty) in non-obese pediatric patients.

The first area of innovation in this proposal is our use of innovative and novel patient-centered research methods will lead to significant breakthroughs in developing a recruitment approach that is both acceptable to pediatric patients and their families and also optimizes their willingness to participate in randomized controlled trials of surgical interventions (Aim 1 and Aim 2b). Additionally, these same innovative research methods will allow us to explore which outcomes are meaningful to patients and their families, which will aid us in advising them in ways that maximize their chances of achieving patient- and family-centered goals (Aim 1 and Aim 2a). To accomplish these aims, our study will make use of the Pediatric Patient Engagement Research Core (PEC), which is a resource ***unique*** to our institution.

PEC is supported by the IU Center for Pediatric and Adolescent Comparative Effectiveness Research (PACER) and Indiana CTSI Community Health Engagement Program. The PEC team offers services to academic researchers related to patient-centered outcome measurement, recruitment, study acceptability, and protocol adherence. Once the target study population is identified, researchers are often limited in their ability to approach and collaborate with the patient and family. These factors are due, in part, to lack of patient and family involvement during the development and implementation phases of research protocols. The PEC team's approach uses designers as research 'translators' to bridge communication gaps between investigators and patients. This approach is truly innovative, and results in research protocols and interventions that are fundamentally patient-centered, because they are developed in partnership with the patients. By utilizing visual communication design specialists as facilitators, we are able to foster a co-designing relationship between the study investigators and patients and their families. Section C.2 will expand further on the innovative design research and visual sociology methodologies that will be utilized for our study.

The PEC team has been highly successful at using innovative patient-centered approaches with other researchers at our institution. For example, the PEC team has previously worked on the Pearl Grlz, CLIC* (Communicating Life in Our Community/Communicando la vida en nuestra comunidad) and the Avondale Health Study [44] [45]. In these projects the PEC team was able to utilize design research and visual sociology methodologies successfully to better engage patients in the research process. They were able to achieve remarkable outcomes for these projects by overcoming barriers to patient recruitment, fostering enthusiasm for study participation, and increasing engagement in the studies themselves.

The second area of innovation is in our urology department's commitment and willingness to allow for the blinding of patients, families, and postoperative care team (floor nurses, medical assistants) to the type of operation that was performed (robotic or open pyeloplasty). See the attached letter of support for more details. The important of this cannot be minimized, as surgeons' unwillingness to allow for randomization of procedures is one of the key reasons for the lack of such studies in the medical literature.[46] However, given the seriousness of this issue, we still feel it is important to conduct a small feasibility study such as this before asking for funding for a larger, fully powered randomized controlled trial.

All patients, regardless of approach, will have the same number of dressings placed strategically after the procedure to simulate both possible types of surgical incisions.[47] Thus, we hope to create a 24-hour window, during which the patient, family, and nurses are blinded to the procedure. If successful, this will significantly reduce bias created by knowledge of the operative approach. This will not alter standard of care and all surgical incisions will be assessed prior to discharge by the surgeon, who will be aware of the actual procedure performed. These techniques will also be novel, and have not been performed to this extent in other studies.

C. Approach

Our study will serve to demonstrate feasibility of recruiting pediatric patients to participate in a randomized control trial for a surgical procedure, delineate patient-centered outcomes, and provide the framework for a large randomized controlled trial comparing robotic and open techniques for surgical correction of congenital UPJ obstruction (pyeloplasty) in non-obese pediatric patients. Our study aims are as follows:

Aim 1: Utilize novel patient-centered research methods to:

- Identify outcomes of importance to patients and families related to pediatric pyeloplasty.
- Determine patient and family preferences regarding acceptability of randomization and blinding of pediatric surgical patients and construct a recruitment approach to achieve enrollment

Aim 2: Conduct a randomized pilot study with goal of enrolling ten to twenty pediatric patients (age 2 – 8 years) to either open or robotic pyeloplasty for treatment of primary UPJ obstruction.

Sub Aim 2a: Collect preliminary data regarding the patient-centered outcomes identified in Aim 1
Sub Aim 2b: Evaluate the effectiveness of the recruitment approach for randomized surgical studies developed in Aim 1

C.1 Aim 1 - Recruitment Approach and Identification of Patient/Family-Centered Outcomes

In collaboration with the PEC, we propose to employ innovative patient-centered research approaches in order to identify that would be meaningful to parents/patients who are undergoing treatment of primary UPJ obstruction. Secondly, we want to explore the acceptability of participation in a randomized clinical trial by this population and what factors might optimize their willingness to be participants. We will explore which information parents wish to receive without being coercive or untruthful. We will also address appropriate compensation and other factors that might contribute to participation.

Figure 1 details a process map for the work that will be performed for Aim 1 including the composition of the group, the focus and content of each group session, and the deliverables that each session informs. Specifically, we propose to conduct a total of 5 patient/parent advisory group sessions. Sessions 1,3,4, and 5 each of which will be up to 4 hours in duration in order to address these two objectives (See Figure 1). Session 2 will be the only session that includes adolescents (aged 13 – 17). It will be a virtual session that involves video chats and messaging through an online password protected communication platform. The aim of this session will be to address measurement. Session 1 will target parents/patients (ideally patients ages 6-10 year old) who have undergone either OP (N=5 patients/parent pairs) or RALP (N=5 patients/parent pairs)

procedure within the last 12 months. Session 1 will address both measurement and randomization acceptability. The second session will address measurement only and include adolescents (ideally ages 13 - 17) who had either the OP (N=5 patients) or RALP (N=5 patients) procedure performed. The third and fourth sessions will address randomization acceptability only and include parents of children 2-8 years old with no history of surgery. All session participants will be recruited from the pediatric urology clinic at Riley Hospital for Children and through community outreach in and around Indianapolis, IN. Healthy subjects for sessions 3 and 4 will be recruited in the community via a recruitment flyer as well as through the INResearch database. Participants for session 4 will also be recruited through the Riley Hospital Pediatric Urology Clinic. These meetings will take place during Y1 of the grant. Participants in the in-person sessions 1,3,4, and 5 will be compensated for their time at a rate of $20/hour. Participants in the session 4 clinic and non-clinic community interviews will receive $10 as the interview is expected to only take 30 minutes. In addition, compensation will be provided for food, parking, and childcare if necessary for participants. Participants in session 2 will be compensated $10 for completion of video chat number 1, $5 for completion of each activity, $20 for video chat number 2, and $20 if all activities have been completed. Participants will also have an opportunity to submit an additional audio recording activity for an additional $20.

The PEC team consists of Sarah Wiehe (director and co-investigator), Helen Sanematsu (faculty at Herron School of Art + Design and visual communications design specialist), and two visual communications experts/designers.[48] The PEC team will utilize innovative visual sociology and design research methods that are often used in public health communication and have been shown to be effective at delivering messages to their intended audiences.[49] The visual communication specialist will employ hands-on creative workshops that provide insight into patient/parent experiences and facilitate 'co-designing' of interventions with input coming from both patient/parents and study investigators.[48,50,51]

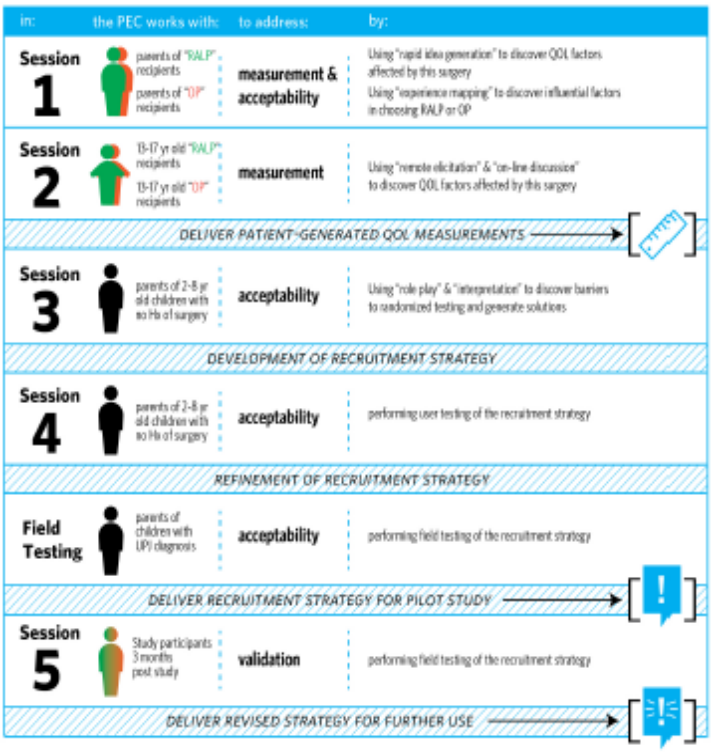

Figure 1. Patient/Parent

Sessions 1 and 2 will address measurement. For session 1, we propose a "rapid idea generation" activity in order to uncover insights into potential measures of importance to patients/parents such as: a) **100mph Thinking** where session participants are prompted to generate as many answers as possible in a short period of time and then are asked to look for similarities within the results or, b) **Mind-mapping** where participants generate branching concepts from a provocative word (See Figure 1). For example, with "100mph Thinking" we might give parents ten minutes to write simple phrases that answers this question "How could this surgery affect your child's day to day experience in five years?" on a sticky note. The parent would be asked to fill out as many sticky notes as they can. These notes are then posted and arranged into groups of like qualities. Parents are then asked to write a simple phrase that answers a follow-up question such as "How could *this specific quality* affect your child's day to day experience in five years?" Once again they are asked to fill out as many sticky notes as they can. This is repeated for all evocative groups, or until everyone's "creative juices" have dried up. These results will help uncover concerns parents have that they might not have even known they had about these surgeries. For session 2, we propose using "remote elicitation" & "on-line discussion" to discover QOL factors affected by this surgery. To accomplish this, we will host an online session that involves uploading videos, answering questions, and two video chats through a password protected online communication platform (RedCap). Video chats will be facilitated through Skype. Participants will be asked to respond to activities online and there will be a minimum of six activities presented. Examples of some activities include draw their scar, identify who sees their scar,

explain how they would support a friend nervous about surgery, describe moments they have thought about their scar, list five goals they would like to accomplish in the future, and describe what they remember about their surgical experience. These results will help uncover concerns patients have that they might not have even known they had about these surgeries.

For the sessions that address randomization acceptability (Sessions 1, 3 and 4) we propose to utilize "interpretation" activities like **Telephone Taboo** where participants have to restate 'research' language in their own words while being forbidden from using a certain word or phrase, or **"role-play"** activities like **Freaky Friday** where participants assume the roles of research assistants and recruit each-other in different situations.

Following these four sessions, the PEC team and study investigators will analyze the findings from each of the session in order to develop the following deliverables:

1. Recommendations for measurement domains from parents' perspective and from patients' experiences into adolescence and how they map to those measures proposed for the pilot study in Aim 2.
2. Recommendations and design for recruitment strategy to be piloted in Aim 2 including a) consent form, b) study information sheet, c) script to be used by the study facilitator to introduce the consent form and information sheet, and d) story boards for expanded communication/informational piece (website, video, app, etc.)

The data gleaned from Aim 1 can also be used to increase the chance of success for a wide variety of subsequent studies in surgical specialties where comparative effectiveness research is necessary.

C.2 Aim 2 - Randomized Pilot Study
We will conduct a randomized pilot study of the OP versus RALP surgical technique with the goal of enrolling ten to twenty pediatric patients between the ages of 2 – 8 years who require treatment for primary UPJ obstruction. We will collect preliminary data regarding the patient-centered outcomes identified in Aim 1 and we will also evaluate the effectiveness of the recruitment strategy that was developed in Aim 1.

C.2.1 Study Inclusion/Exclusion Criteria

Inclusion Criteria	Exclusion Criteria
• Primary UPJ obstruction diagnosed by scan and presenting to the pediatric urology clinic for evaluation • Patient between age 2 – 8 years old	• Serious comorbidities (cardiovascular or respiratory disease, or other congenital anomalies requiring surgical intervention). • BMI greater than the 95th %tile for age.

C.2.2 Patient Identification and Recruitment. All participants will be recruited from the pediatric urology service at Riley Hospital for Children in Indianapolis, IN. We will identify pediatric patients with a UPJ obstruction requiring surgical intervention, using a double check system to avoid missing potential study subjects. Our clinical schedulers or surgeons in clinic will identify all potential study patients and flag them for the study PIs or RAs to review patient history. In addition, each week one of the study PIs (Whittam) will assess all new patient referrals to identify any additional potential study candidates.

Once eligible patients are identified, they will be introduced to a member of the research team. Following the recruitment strategy developed in Aim 1, the study team member will introduce the study to the patient/family including presenting the expanded communication/ information pieces, the recruitment video and brochure, that were developed for the pilot study. If they agree, they will provide verbal consent and be shown the recruitment video. This strategy will include a general description of the different surgical modalities, as well as risks and benefits of each procedure. Emphasis will be placed on the fact that both options are equivalent. If they decide to be on the study, they will be consented in clinic, randomized to the study, and asked to complete a brief questionnaire as to why they decided to participate. During the enrollment process, the ICS and Assent (for the participant who is 7 years or older) will be reviewed verbally. Participants will be provided with a hard copy to read and time to ask questions. After signing the consent and assent, participants will be given a copy for their record.

If they decline they will be asked to complete a brief questionnaire as to why they decided not to participate.

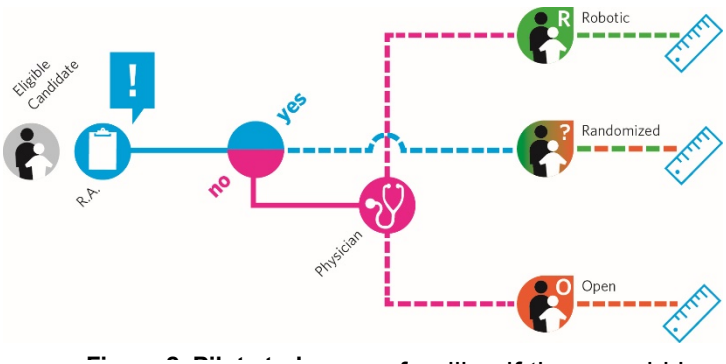

Figure 2 shows the potential recruitment paths when an eligible patient is approached to participate in the study. The blue dotted line reflects those patients/families that agree to participate in the randomized pilot study. The study RA will obtain informed consent from these families utilizing the documents designed in Study Aim 1. The pink dotted line reflects those patients/parents who are NOT willing to participate in a randomized study. Following their decision NOT to participate in the randomized study, the study RA will ask these families if they would be willing to 1) participate in a group session with the PEC team regarding their decision not to participate in the randomized study (See Figure 1, Session #5 and Figure 2) if they would be willing to allow our study team to collect data on the outcome measures being utilized for our study (See Section C.2.7). These families would not be randomized, but instead would be allowed to decide for themselves which procedure they would like to have performed. By utilizing this approach we are guaranteed to obtain useful data, even if we are unable to successfully implement a pilot randomized trial.

C.2.3 Compensation. Study participants will be compensated for their participation per findings from Aim 1. Aim 2, families who agree to participate in the randomized pilot study will receive a parking voucher during their child's hospitalization and subsequent follow-up appointments. They will receive $25 dollars for the initial follow up visit at 2 weeks and $10 for each follow-up visits (6 weeks, 3 months, 9 months) for a total of $55 if they complete all the follow up visits.

C.2.4 Patient Randomization Procedure Once parent/guardians have signed informed consent, patients will be immediately randomized to either RALP or OP by using a pool of 20 cards of which 10 say "robotic surgery" and 10 say "open surgery." Each patient will then have a card randomly picked for them, thus ensuring an equivalent number of patients in each arm of the study. The attending surgeon for the patient will assist or perform the operation to maintain continuity of patient care.. A single robotic surgeon will be involved in all robotic procedures and one of four senior surgeons will be involved in any open procedure.

C.2.5 Operative Intervention Each subject will have anesthetic induction and intubation. All patients will undergo a cystoscopy (endoscopy of the bladder) and retrograde pyelogram (instillation of contrast into ureter and across the UPJ) to confirm diagnosis of UPJ obstruction. An internal double j stent will be placed in the affected ureter (small, completely internal tube resting in the ureter held in place with curl in the kidney and bladder) and a bladder catheter placed. A standardized regional anesthetic block will be performed regardless of surgical approach. The pain service will place the block and follow the patient for pain management post operatively. Either RALP or OP will then be performed. Each patient will have standard anesthesia reversal and extubation.

Upon completion of the procedure, dressings will be placed on all surgical incisions, as well as anticipated locations of where incisions would be for the other procedure. Thus, all patients will have the same numbers of bandages (4) on all possible surgical sites, regardless of surgical approach.

C.2.6 Postoperative Care The patient will be transferred to the surgical floor once all standard PACU criteria have been met. All medication administration will be documented for postoperative pain. A clear liquid diet will be initiated, and diet will be advanced as tolerated. On postoperative day one (POD #1) the foley catheter will be removed and intravenous fluids will be stopped. All pain medication will be converted to an oral route with a plan for an early afternoon discharge if patient meets discharge criteria. Discharge criteria includes tolerating a regular diet, pain controlled on oral medication, ambulating without assistance and voiding spontaneously. Patients are followed at 1 week, 2 weeks, and 6 weeks post op. Their subsequent follow up appointments (3 months, 6 months,, and 12months) are from the time of their prior visit. .

C.2.7 Measures. The patient-centered outcome measures that will be utilized in this study have been informed by the work of Aim 1. The list of outcomes below is not an exhaustive one, but does serve to outline that has been affirmed by the work in Aim 1.

Outcome Measure	Interval	How Obtained
Length of Hospital Stay (days)	Once, after discharge	Chart review by RA
Pain Medication Use (dose/freq)	Once, after discharge	Chart review by RA
Surgical complications	Once, after discharge	Chart review by RA, Clavian-Dindo classification[52]
Total Hospital Charges and Estimated Costs	Once, after discharge and data deposited to PHIS database	PHIS database will be queried for charge and cost data submitted by the hospital
Pain Scores	Every 4 hours while hospitalized 1 and 2 week post op follow up	Chart review by RA (hospitalization and post op follow up
Patient Experience: Side effects of medication and impact; Experience of having a temporary ureteral stent 4; Ease of removing stent; Return to activity; Financial burden reported by family	2 week post op follow up	REDCAp survey or at time of 2 week post op follow up
Time until parental return to work	2 week post op follow up	REDCAp survey or at time of 2 week post op follow up
Scar perception	At hospital discharge, and at 1,2, and 6 post op follow ups, and 3mo follow up scheduled at 6week follow up	REDCap survey or at time of post op follow ups, using the Patient Scar Assessment Questionnaire[53] (PSAQ)
Quality of Life (post intervention)	At hospital discharge, at 1 2, and 6 week post op follow ups ,and 3 mo return visit scheduled at 6week follow up	REDCap survey or at time of post op follow ups, using the Glasgow Children's Benefit Inventory (GCBI)

Post op follow up refers to a follow up encounter in clinic or by phone. Type of encounter is determined by time interval and medical need

After completion of the pilot study, these outcomes will allow for an accurate power calculation, which will allow us to determine the number of patients needed for a large-scale study to determine superiority of either RALP or OP in the treatment of pediatric UPJ obstruction.

C.2.8 Validation of the Recruitment Strategy Patients/families that agree to either participate in the randomized pilot study or alternatively those who agree solely to the collection of outcomes data will be brought together by the PEC team in order to further refine the recruitment strategy that was developed in Aim 1 (See Figure 1). These two groups will attend separate sessions in order to better understand what components of the recruitment strategy were compelling and how the strategy can be improved.

C.3 Study Timeline

	YEAR 1				YEAR 2			
	Qtr 1	Qtr 2	Qtr 3	Qtr 4	Qtr 1	Qtr 2	Qtr 3	Qtr 4
Identification of Patient-Centered Outcomes								
Feasibility Study								
Data Analysis and Publication of Findings								
Writing of R01 Level Grant								

C.4 Potential Pitfalls

As this is a feasibility study, there are potential hurdles that we may face in regards to executing this study. First, it is possible that patients/families will not chose to enroll in this study. This is unlikely, since Aim 1 will seek to optimize patient participation. If we determine with Aim 1 that a pilot study as designed would not result in any recruitment, we will revise our approach, including altering the methodology to an observational study (without randomization). This would allow us to still gather patient-centered outcomes so that a subsequent effort can determine a tolerable method of randomization. Second, it is also possible, despite assurances, that surgeons will not allow their patients to be randomized. In order to prevent this occurrence, we will perform reviews at 3-month intervals of all pyeloplasties performed at the institution. If randomization is

not occurring we will meet with all pediatric urology faculty, and the section chief (Dr. Mark Cain) will encourage surgeon participation. Overall, we believe both pitfalls are very unlikely. Moreover, determining points of failure such as these is the rationale for a feasibility study in the first place. Even if a pilot randomized trial is not feasible, we will still have a great deal of useful patient-centered outcomes upon which to base an observational study and that can be used to design a more effective randomized trial in the future.

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