

PROTOCOL

Understanding Factors in Decision Making for Children With Medical Complexity

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Protocol Summary

Developing an Innovative Decision Support Tool for Pediatric Neuromuscular Scoliosis- Aim 1
Draft Protocol Summary

University of Utah IRB #: IRB_00154724

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Background and Introduction

Neuromuscular scoliosis (NMS) can result in severe disability for children. Non-operative management including bracing and physical therapy minimally slows scoliosis progression, but operative management with posterior spinal fusion (PSF) carries high risks of morbidity and mortality in part due to the multiple comorbid conditions seen in children with NMS. (1, 2) Decisions like PSF that have no clear best treatment option are best served by shared decision making (SDM). SDM is a collaborative process where patients, parents, and providers share their knowledge, preferences, and values to reach treatment plan agreement. Our prior qualitative study creating a framework of SDM for children with multiple comorbid conditions like children with NMS found that parents face immense decision-related uncertainty that often goes unacknowledged by providers. (3) However, parents felt more prepared to make decisions when providers acknowledged uncertainty. In a qualitative analysis of clinical encounters with children with NMS, parents, and orthopedic surgery providers, we found that providers mostly made qualitative statements about uncertainty and parents were mostly concerned about uncertainty related to their child's underlying comorbid conditions.

Purpose and Objectives

We will conduct feedback sessions with parents of children with chronic conditions on a clinical patient story. From this feedback, we will design an online factorial experiment to test different approaches to communicating uncertainty during surgical decision making for children with neuromuscular scoliosis.

Study Population

Age of Participants: all ages

Sample Size:
All Centers: 1043

Inclusion Criteria: Parents of children with medical complexity (N=1043, n=35 cognitive testing, n=1008 factorial experiment) who are at least 18 years of age and comfortable reading in English or Spanish. Children with medical complexity is defined as a child (age 0-18 years) with a complex chronic condition defined by ICD-9 or ICD-10 code developed by Feudtner, et al. 2014.

Exclusion Criteria: Exclusion criteria: Parents of children with neuromuscular scoliosis who have not undergone surgery for neuromuscular scoliosis (NMS) to avoid unduly influencing their decision-making process for NMS treatment. NMS will be defined as qualifying ICD-9 or ICD-10 codes for neuromuscular scoliosis (e.g. ICD-9 737.43, ICD-10: M41.40-7, M41.4). NMS surgery will be identified by documentation of spinal fusion in the medical history or orthopaedic clinic note in the medical chart.

Design

Non-Experimental and/or Descriptive Research Design

Interviews and Focus Groups

Experimental and/or Interventional Research Design

Randomized Trial

Study Procedures

Recruitment/ Participant Identification Process:

1,043 children (children of parent participants) will only be involved to identify their parents as eligible but will not complete any further study procedures. No PHI will be included in the research record.

Recruitment will occur at the University of Utah/Primary Children's Hospital (main campus and Lehi campus) as well as a secondary site, Children's Hospital of Los Angeles (CHLA). This section describes the recruitment processes at the main site (UofU/PCH). See below section for a brief description of recruitment processes at CHLA.

1,043 parents (participants) will be recruited using purposeful sampling to achieve a diversity of parent race/ethnicity representative of local demographics. We will also purposefully sample by patients' (children of parents) categories of complex chronic conditions as defined by the Feudtner 2014 classification. 35 parents will be recruited during vignette development for cognitive testing of the vignette (with 15 patients from our secondary site CHLA). The remaining 1,008 parents will be recruited during the factorial experiment (with 237 patients from our secondary site CHLA). See below section for more information on the recruitment process at our secondary site CHLA.

For recruitment via e-mail or physical mail, we will perform chart review of patients seen at the Complex Care Clinics or admitted to the inpatient pediatric teams at Primary Children's Hospital over the past 5 years and contact the parents/caregivers of the patients using the email listed in the electronic health record.

For in-person recruitment at Primary Children's Hospital Complex Care Clinics, potential participants will be

identified via chart review of the participant's child in the electronic health record 1-2 weeks before the clinic visit. On the day of the clinic visit, a member of the study team will confirm eligibility with the provider seeing the potential participant's child. Front office staff will introduce the study staff to potential parent participants at time of check-in for their visit. The study team will also use screening questions added to an existing clinic intake sheet to gauge potential participant's interest in learning more about the study. Potential participants will be asked to mark on the intake sheet whether or not they wish to be contacted to further learn about the study. If they are interested, potential participants will also provide limited PHI to help with contact/follow-up by the research team. This limited PHI will not be saved by the research team. The existing intake sheet is already used by the clinic staff as part of their standard practices. See Documents & Attachments for screening question and limited PHI that will be asked on the intake sheet.

For in-person recruitment from the inpatient pediatric teams, participants will be identified via chart review of the participant's child in the electronic health record on the day of recruitment. Study team members will confirm eligibility with the hospitalist attending for the eligible participant before the study team approaches the participant.

For patients admitted inpatient to the Lehi PCH Campus, potentially eligible participants will be identified via chart review of the participant's child (the patient) in the electronic health record on the day of recruitment. Eligible participants will then be contacted via hospital phone call or email recruitment to assess interest in learning about the study. A study team member will call in to the phoneline setup in the patient's hospital room and ask them if they are interested in participating in the study. If they are interested, a study member will perform informed consent and help them get started in the study. The study team will also send the participant a recruitment email. If eligible participants are interested via email, they will be given an option to fill out a brief REDCap form in the email to indicate their interest. Then, a study team member will contact them during their inpatient stay at Lehi and up to one week of their discharge to explain the study procedures to the participant and help them start the study. Recruitment at Lehi PCH Campus is for Part 2 of the study (the factorial experiment).

For the factorial experiment, in addition to the above procedures, we will also work with the Intermountain EDW data analyst team to build a recruitment dashboard to identify potentially eligible participants. We will conduct chart reviews of patients on this dashboard to confirm eligibility and use the recruitment strategies outlined above to approach them for recruitment. Once identified and approached, the team will use an Intermountain REDCap to track recruitment efforts and exclude already contacted patients in the dashboard. Patients will only be involved to identify their parents as eligible. No PHI of excluded patients will be included in the official research record, only in the Intermountain REDCap to maintain the recruitment dashboard. All Intermountain REDCap data will be deleted at the end of the study.

*A waiver of authorization for recruitment only has been requested to review the medical records of the participant's child/patient in the above scenarios for participant identification.

For the cognitive interviews during vignette development (n=35), to help with iterative development of the vignette, we will prioritize recruiting parents of children with medical complexity admitted to the inpatient pediatrics teams so that there will be naturally built-in longitudinal follow-up period (the hospital stay) in which we can obtain iterative feedback. We will identify potential participants via chart review and confirm eligibility with the attending hospitalist for that participant's patient. We will review the vignette in-person at the patient's bedside for feedback by presenting a paper version of the vignette to the participant for discussion with the study team member. Participants will be able to mark their edits and suggestions and the team member will take notes on their suggestions. Team members may approach a single participant up to four times for feedback during vignette development. If the participant's child is discharged, we will follow-up with them via their preferred mode of contact.

The study team will also contact participants from a prior IRB approved study (IRB_00128776) who have previously given permission to be contacted for future studies.

If response rate is low, we will perform a second wave of recruitment without purposeful sampling.

There will be no follow-up period for the factorial experiment.

The study team will provide eligible participants with written advertising (i.e., study invitation letter, flyer) when contacting participants remotely via email or physical mail. All recruitment materials will be uploaded to the IRB in an amendment prior to the recruitment period.

Recruitment Procedures at secondary site Children's Hospital of Los Angeles:

Children's Hospital of Los Angeles (CHLA) will be participating in study procedures. The CHLA study team has received their own independent IRB approval for all study procedures at their own institution (study number: CHLA-22-00281). The CHLA research coordinators will screen, recruit and consent participants at CHLA and help to input data into the University of Utah study's REDCap project. No PHI or data from the University of Utah or Primary Children's Hospital will be disclosed to CHLA. More information can be found in the CHLA IRB approved protocol document that is attached in the "Other" section of this application.

**Informed
Consent:**

Description of location(s) where consent will be obtained:

Consent will be obtained either in person at the Primary Children's Hospital Complex Care Clinics (CCC), pediatric orthopaedic surgery clinics, inpatient pediatric teams, and the Riverton Hospital pediatric orthopaedic surgery clinics or via telephone. We will use a consent cover letter for the factorial experiment or a full consent form for the vignette development.

For study procedures at our secondary site Children's Hospital of Los Angeles, consent will be obtained in person at CHLA inpatient pediatric teams and outpatient orthopaedic and comprehensive care clinics. See attached in the Other section CHLA's IRB approved "Research Information Sheets" they will be using to obtain consent.

Description of the consent process(es), including the timing of consent:

1) For vignette development, parents who are interested in learning about the study will be provided with a paper or electronic copy of the consent form for their reference and information about the study. Information provided to the patient will include the time commitment (30 minutes for consent, 30 minutes per feedback session, up to 4 feedback sessions), the intention of the study to understand parent preferences for information around decision making, data collection (notes and discussion), study withdraw process, risks, and benefits. We will also review the precautions we take to preserve participant privacy as outlined above and that the study personnel and related governing bodies of the institution and the government will be the only ones to access their study information. They will also be informed about remuneration for their participation. After reviewing the study protocol with the participant, the study team will ask the participant if they have any questions and give them adequate time to think about participating in the study before consent is obtained.

2) For the factorial experiment, potential participants will receive the consent cover letter. Information provided to the patient will include the time commitment, the intention of the study, data collection via REDCap, study withdraw process, and study risks and benefits. Participants will be given as much time as they need to think about whether or not they would like to participate in the study. Participation in reviewing the vignette and answering the surveys will represent consent to the study.

Requested Waivers/Alterations of Consent:

02. Type of Request

01. Purpose

Waiver of Informed Consent

Use of consent cover letter for factorial experiment.

Procedures:

Study design: In this study, we will design a randomized factorial experiment to test the effectiveness of various uncertainty communication methods and a parent narrative information sheet on parents of children with medical complexity. The goal of this study is to identify the most effective uncertainty communication methods and whether narrative information sheets are helpful. Helpful and effective results determined from this study will be included in a decision support tool that we will design in a separate study. We will conduct a randomized factorial experiment to test strategies to communicate uncertainty and to test narrative information sheets about neuromuscular scoliosis (NMS) treatment risks. We will create a clinical vignette about a parent of a child who has NMS and is facing the decision of surgical correction or non-surgical management. We will present all risks in a balanced probability frame (e.g. 95% chance of having uncomplicated surgery, which is equivalent to 5% chance of having a complication) and discuss the long term prognosis of NMS in the context of both natural progression and surgical correction. Risk estimates will be based on published literature. The narrative information sheet will be developed based on data from a prior study and will include deidentified parent narratives about their decision making for their child's NMS.

The study is divided into two parts: **1) vignette development and 2) factorial experiment.**

Study procedures will occur at the University of Utah/Primary Children's Hospital as well as a secondary site, Children's Hospital of Los Angeles (CHLA). This section describes the procedures at the main site (UofU/PCH). See below section for procedures at CHLA.

Setting: Participants will be recruited from Primary Children's Hospital (PCH) Complex Care Clinic, a

subspecialty medical home for children with medical complexity that provides outpatient primary care and care coordination, pediatric orthopaedic clinics, and Riverton Hospital pediatric orthopaedic clinics. We will also recruit from the inpatient general pediatrics (hospital medicine) teams.

Recruitment: Participants will be recruited in person and via e-mail or physical mail. **For in-person recruitment at the Complex Care Clinics and pediatric orthopedic clinics,** potential participants will be identified via chart review in the electronic health record 1-2 weeks before the clinic visit. On the day of the clinic visit, a member of the study team will confirm eligibility the provider seeing the potential participant's child. Front office staff will introduce the study staff to potential parent participants at time of check-in for their visit. The study team will also use screening questions added to an existing clinic intake sheet to gauge potential participant's interest in learning more about the study. Potential participants will be asked to mark on the intake sheet whether or not they wish to be contacted to further learn about the study. If they are interested, potential participants will also provide limited PHI to help with contact/follow-up by the research team. This limited PHI will not be saved by the research team. The existing intake sheet is already used by the clinic staff as part of their standard practices. See Documents & Attachments for screening question and limited PHI that will be asked on the intake sheet. **For in-person recruitment from the inpatient pediatric teams,** participants will be identified via chart review in the electronic health record on the day of recruitment. Study team members will confirm eligibility with the hospitalist attending for the eligible participant before the study team approaches the participant. **For recruitment via e-mail or physical mail,** we will perform chart review of patients seen at the Complex Care Clinics or admitted to the inpatient pediatric teams over the past 5 years and contact them using the email or address listed in the electronic health record. We chose a multi-pronged approach of in-person and via e-mail/physical mail to ensure that we capture populations both with and without home Internet.

- 1) For the development of the clinical vignette, we will recruit 10 parents in-person to provide iterative feedback on the vignette. We will recruit 5 English-speaking parents and 5 Spanish-speaking parents with parents from both sites represented in each language group.
- 2) Once participants are identified and approached in the factorial experiment, the team will use an Intermountain REDCap to track recruitment efforts and exclude already contacted patients in the recruitment dashboard. Patients will only be involved to identify their parents as eligible. No PHI of excluded patients will be included in the official research record, only in the Intermountain REDCap to maintain the recruitment dashboard. All Intermountain REDCap data will be deleted at the end of the study.
- 2) Once participants are identified at the Lehi PCH Campus, the team will contact participants via hospital phone call or email to introduce the study and ask for interest. A study team member will call in to the phonenumber setup in the patient's hospital room and ask the participant if they are interested in participating in the study. If they are interested, a study member will perform informed consent and help them get started in the study. The study team will also send the participant a recruitment email. If eligible participants are interested via email, they will be given an option to fill out a brief REDCap form in the email to indicate their interest. If interest is indicated, a study team member will call the participant to explain study procedures and help the participant get started in the study. The study team will follow-up with the participant as-needed to help them complete the study. The study team will contact the participant during their initial admission at Lehi PCH Campus up to 1 week post-discharge.

Study Procedures:

- 1) For the vignette development, we will perform iterative cognitive testing with 10 parents of children with medical complexity to develop the vignette for the factorial experiment. The clinical vignette will be available in English and in Spanish. Participants will receive the vignette in their preferred language. Participants will review the vignette for feedback and discussion with the study team. Participants will be able to mark their edits and suggestions and the team member will take notes on their suggestions. Team members may approach a single participant up to four times for feedback during vignette development. Contact information will only be collected from the participants for the iterative feedback period. Otherwise, no identifying information on themselves or their child will be collected.
- 2) During the factorial experiment, participants will watch a video of the clinical vignette. Participants will be randomized to receive one of various versions of the vignette that have different combinations of uncertainty communication and randomized to either receive or not receive the narrative information sheet. The experiment will be delivered through University of Utah Research Electronic Data Capture (REDCap). Each participant will receive one version of the vignette. After reading the vignette and the narrative information sheet (if they are randomized to receive it), participants will be asked to answer survey questions including outcome measures, sociodemographic information about them and their child, and information about their child's past medical history (see below primary and secondary outcomes). No identifying information on the participant or their child will be collected. If participants

enroll in the study and start study procedures in-person but do not complete the study, the study team will make follow-up phone calls with the participants to help them complete the study remotely.

Randomization: 2) For the factorial experiment, we will randomize each participant to receive one of eight possible versions of the clinical vignette or clinical vignette and narrative information sheet. We anticipate possibly having up to eight versions of the vignette and/or vignette + narrative information sheet combination. Thus, $n=63$ parents will be randomized to each version. Randomization will occur through the REDCap randomization function after enrollment in the study. The participant and study team members including the PI will be blinded to which condition the participant was assigned to. While study personnel may be present during the experiment for participants who undergo in person recruitment, they will be there for technical support, the participant will complete the experiment on their own.

Study Procedures at secondary site Children's Hospital of Los Angeles:

Children's Hospital of Los Angeles (CHLA) will be participating in study procedures. The CHLA study team has received their own independent IRB approval for all study procedures at their own institution (study number: CHLA-22-00281). For Part 1 of the study, the CHLA team will pass along the contact information of all consented CHLA participants via University of Utah REDCap to the University of Utah team for our team to complete virtual study interviews. For Part 2 of the study, the CHLA team will conduct all study procedures with CHLA participants and pass along the contact information of consented participants for the sole purpose of gift card remuneration. All data collected by CHLA will be done through UofU REDCap. The CHLA research assistant will be given limited data access to the University of Utah REDCap project: they will not be able to view University of Utah participant data and will only have access to a limited number of instruments within REDCap that are specific to the study procedures performed at CHLA. No PHI or data from the University of Utah or Primary Children's Hospital will be disclosed to CHLA. More information can be found in the CHLA IRB approved protocol document that is attached in the "Other" section of this application.

Primary outcome: Decision preparedness as measured by the PrepDM validated survey measure. The 10-item (1 to 5 scale) survey measure results in a composite mean score of all items with higher scores indicated greater patient preparedness to make a decision with their provider.

Secondary outcomes: We will collect measures of participant cognitive, affective, and behavioral responses. Cognitive outcomes include: 1) perceptions of risk (adapted from NCI HINTS), 2) perceptions of uncertainty (adapted from the NCI HINTS), 3) knowledge (newly developed). Affective (aka emotional) outcomes include: 1) worry about NMS-related risk (adapted from NCI HINTS), 2) trust in information (adapted from NCI HINTS), 3) hope (Herth Hope Index), and 4) decisional conflict (Decisional Conflict Scale). Behavioral outcomes include: 1) intention for NMS treatment (newly developed) and 2) intention for not delaying treatment (newly developed).

*Newly developed study measures and materials (vignettes and surveys) have been added in an amendment to the IRB protocol.

*Narrative information sheets used in the factorial experiment have been added in an amendment to the IRB protocol.

Statistical Methods, Data Analysis and Interpretation

Data analysis: We will use 3 factors with two options each and a narrative information sheet for the factorial experiment. We will have 16 different study conditions. Standard psychometric analyses (e.g., Cronbach's alpha) will be used to confirm the internal consistency reliability of self-report measures. For our primary outcome variable PrepDM, we will use a 4-way ANOVA model to describe differences in decision preparedness across each factor (e.g. ambiguity, complexity, normalizing and explanatory language, and narrative information sheet) (4). We will use post-fit marginal estimation to express effect sizes (adjusted means and adjusted mean differences), which incorporates mean centering to make the main effects interpretable in the presence of factor interactions. Regression models, with main effect and interaction terms, can be fitted that are identical to a 4-way ANOVA model. We will use these to model the secondary outcomes, using binary logistic, ordinal logistic, and linear regression, depending on the level of measurement of the outcome variable, and will add race and education level as covariates in all models. We will perform exploratory analyses for populations with and without NMS and by ethnicity and language.

Sample size: In a validation study of PrepDM in orthopedic decision making, the scale had mean SD of 3.7+/-1.0 after the decision aid intervention (4). We will randomize n=46 subjects into each of the 2x2x2x2=16 cells of the 3-way factorial design (each factor at 2 levels), for a total N=736 subjects. This is a sufficient sample size to include 4 main effects, 3 two-way interactions, 2 3-way interaction, and 1 4-way interaction, without overfitting the data. This provides 95% power, using a two-sided alpha 0.05 comparison, to detect an adjusted mean difference of 3.7+/-1.0 vs. 3.4+/-1.0 (0.3 standardized mean difference) between the two levels of the factor. That is smaller than a 0.5-point difference on the scale of each item and has been achieved in previous studies of decision aids in orthopedics.