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Initial development of a questionnaire evaluating perceived benefits and barriers to pediatric clinical trials participation

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ABSTRACT

Objective: To evaluate perceived benefits and barriers to pediatric clinical trials participation to improve decision-making and enhance recruitment and retention among minority youth with chronic health conditions (sickle cell disease, asthma) and their caregivers.
Methods: A questionnaire was developed based on the social ecological model using input from medical experts and community-based public health organizations. Parallel caregiver, adolescent/young adult (AYA; 16–39 years old), and child (8–15 years old) versions were field tested. Patients and caregivers completed the questionnaire, which included demographic items, perceived life stress and social desirability measures.
Results: Exploratory factor analysis rendered a four-factor solution for the caregiver version (direct treatment benefit, mistrust of research/researchers, trust in healthcare team to engage in safe research, and opportunity cost) and the AYA version (mistrust/no direct benefit, safety, direct treatment benefit/practical considerations, and social support for research). Factor structures differed for SCD and asthma caregivers; results were equivocal for the child version. Summated subscales were not significantly associated with patient demographics or social desirability, but significant correlations with perceived life stress and prior participation in research were identified.
Conclusions: While the factor structure should be confirmed with larger samples, findings indicate potential benefit, perceived harm due to mistrust of researchers, and logistics are primary factors in decision-making about participation in pediatric clinical trials. By addressing these benefits/barriers through adjustments to recruitment and informed consent procedures, researchers may address misperceptions of research, improve decision-making, and increase recruitment and retention particularly for ethnic minority children with chronic health conditions.

1. Introduction

Clinical trials are vital to the advancement of novel, evidence-based medical treatments and improvement of long-term health outcomes [1]. Yet, caregivers may be reluctant to enroll their children in clinical trials even if they are cognizant of the importance of pediatric research [2]. Moreover, studies suggest that disparities exist with regards to study enrollment rates [3] such that minority pediatric patients participate in medical research at lower rates than their White counterparts [4]. Recruitment and retention rates in cognitive behavioral intervention trials for children with chronic medical conditions are particularly low [5].

Recruitment of pediatric patients with sickle cell disease (SCD) and ethnic minority patients with asthma has been

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challenging, adding to the difficulties in advancing treatments for these populations [6,7]. SCD is the most common genetic disease among African Americans and affects more than 80,000 individuals in the US. Asthma, the most common chronic disease of childhood, disproportionately affects African American and Puerto Rican children [8]. Reasons for participation and refusal in research may vary based on ethnic minority status, societal/cultural factors [9,10], and personal barriers (such as stress/anxiety) [11]. Parents in prior studies have identified positive factors and perceptions contributing to research participation including altruistic motives, opportunities to learn more about their child's illness, access to free medication, willingness to contribute to medical knowledge, and desire for closer clinical care [12,13]. However, perceived importance of a particular research study's objectives and its relevance to one's child may also explain variability in parent willingness to allow participation in clinical trials [14]. Indeed, a number of barriers may hinder participation in research particularly for ethnic minority pediatric patients and families such as mistrust of medical research, misunderstanding of the research process, overestimation of risk, religious beliefs that counter medical science as well as concrete barriers such as time commitment, childcare, and transportation difficulties [13,15–19]. Also, a significant number of children with SCD do not receive care at comprehensive sickle cell centers funded by the National Heart, Lung, and Blood Institute, reducing opportunities for research participation [6].

Reasons for reduced engagement in pediatric SCD or asthma clinical trials have not been studied in a systematic manner; yet, improved understanding in this area can help improve accrual and retention [20]. For example, identifying key factors that influence the decision to participate in a pediatric clinical trial, whether structural (e.g. child care), concerns about risks, lack of support for participation, and/or perceived treatment benefit can guide recruitment and informed consent meetings. The purpose of this study was to develop and provide preliminary evidence of validity for a measure to aid in understanding decision-making about clinical trials enrollment among pediatric patients with chronic medical conditions. To ensure applicability of the measure to children with chronic health conditions who are less likely to enroll in trials, our study sample included children with health disparity conditions (i.e. conditions more prevalent among ethnic minorities [SCD or asthma] and their caregivers). For preliminary evaluation of convergent validity of the measure, we hypothesized that prior exposure to research would be associated with more favorable attitudes toward participation in clinical trials (endorsing benefits) and greater perceived stress would be associated with less favorable attitudes (endorsing barriers). Social desirability was also measured to evaluate the extent to which responses were biased to please the researchers (divergent validity).

2. Methods

2.1. Participants

Participants included patients with SCD (8–39 years) and/or their caregivers and patients with asthma (3–18 years) and/or their caregivers. Participants with SCD were recruited from two East coast pediatric sickle cell centers and an adult outpatient hematology practice associated with a local hospital. Participants with asthma were recruited from a pediatric pulmonary clinic at an East coast pediatric hospital. Participants with SCD and participants with asthma ages 8 through 15 years old completed the child questionnaire. Participants with SCD ages 16 through 39 years old and participants with asthma ages 16 through 18 years old completed the adolescent and young adult (AYA) questionnaire. Available caregivers of all participants completed the caregiver questionnaire. Eligibility criteria included the ability to speak and read English.

Of the 385 families approached for participation, 77.1% (n = 296) of families consented to participate in the study, 20.1% (n = 78) refused participation, and 2.8% (n = 11) did not meet inclusion criteria. Reasons for refusal included being approached at end of appointment (29.5%), “not interested” (28.2%), needing more time to decide (20.5%), distrust of research (11.5%), and feeling unwell (10.3%). Two participants were excluded from the analyses due to failure to complete the questionnaire prior to the end of the clinic visit. The final sample (n = 418) included 63 children with SCD, 88 AYA with SCD, and 154 of their caregivers as well as 40 children with asthma, 3 AYA with asthma, and 70 of their caregivers.

2.2. Development of the Pediatric Research Participation Questionnaire (PRPQ)

We developed the PRPQ for this study in four phases. In Phase 1, the project team, community partners, and a team of six SCD medical experts and researchers generated questionnaire items based on the Social Ecological Model. As applied to children with a chronic health condition, social ecology describes multiple levels of bidirectional influences on child and family adaptation to chronic health conditions from proximal influences (e.g. child's health condition, family, social support for research participation) to distal variables (e.g. healthcare team, views on research and researchers, societal values and culture) [21]. Because the social ecological model outlines personal, health care, and societal facilitators of and barriers to adaptation including interactions with health care systems, this model provides a comprehensive and appropriate framework for organizing perceived barriers and benefits to pediatric clinical trials enrollment (Fig. 1). Items were also selected and modified from the *Barriers to Research Participation Questionnaire* (BRPQ) a reliable and valid measure for African-American adults [18]. This process resulted in items reflecting barriers and benefits organized into each level of the patient's social ecology: Patient, Caregiver and Family, Community, and Societal/Cultural. Patient level items consist of Disease Related Correlates (e.g. knowledge of chronic illness, past participation in medical or psychosocial clinical trials), Patient Benefit (e.g. learn more about chronic illness, increased monitoring by visits to healthcare provider), and Patient Burden (e.g. may physically harm patient, require increased procedures). The Caregiver and Family level is comprised of Practicality/Feasibility (e.g. transportation costs covered must miss work to participate) and Family Benefit/Burden (e.g. family supports research participation, too much time spent at the hospital/clinic already). Community level items include Social Supports (e.g. health care team or friends support