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Short Report

STRENGTHENING CAPACITY FOR A HARDLY REACHED PEDIATRICS POPULATION TO ACHIEVE EQUITY IN ACCESS TO CLINICAL TRIALS

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Abstract: **Introduction.** Diversity among clinical trial participants is important. Clinical trials that do not include a diverse range of pediatric participants may overlook relevant patient perspectives. The objective of the study was to evaluate knowledge among primary care physicians about available resources for pediatric patients' enrollment in clinical trials for rare medical conditions; identify barriers preventing children with rare diseases from suburban and rural areas from participating in clinical trials; create an optimization strategy to facilitate patients' enrollment into available clinical trials based on identified barriers.

Methods. To evaluate knowledge among primary care pediatricians serving a rural area, an interview was conducted about available resources for children with rare medical conditions to participate in the clinical trials. Parents of children with rare medical conditions were also recruited to answer relevant questions. After the interview, the parents were provided resources on clinical trials available for their children's medical conditions. An additional interview asked about the usefulness of those resources. Data from these surveys were transcribed into Excel and subsequently subjected to detailed analysis using SPSS.

Results. The primary obstacles encountered by physicians included their limited availability of time during clinical visits and a lack of knowledge about clinical trials. The parents of the children exhibited a significant interest in engaging in clinical trials. The barriers to participation included concerns about potential side effects and the financial burden of travel expenses. Post-survey patients indicated that the information they received proved to be invaluable. Ninety percent of participants believed the information during the intervention was useful, and 20% became interested in participating in clinical trials.

Conclusion. To achieve equity in clinical trial participation, pediatric patients need to be involved in the clinical trials. This could be accomplished by facilitating education in primary pediatrician clinics and eliminating logistics barriers.

Keywords: Clinical trials, equites, rare diseases, pediatrics.

INTRODUCTION Clinical trials are critical for testing new treatment approaches and drugs and establishing new standards of treatment. However, previous research pointed to a noticeable mismatch between trial populations and real-world patients [1]. Published studies have already summarized barriers affecting access to clinical trials at the patient level (distrust, unawareness, financial status, geography, social support, and logistics issues), provider level (lack of awareness, lack of research workforce, innate bias), study level (restrictive inclusion criteria, complex processes with multiple study visits), and institutional level (deficient screening and trial matching, lack of periodic institutional self-assessments) [2].

Access to clinical trials for rare diseases is a pervasive challenge, particularly for patients residing in suburban and rural areas. Health inequities among specific

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population subgroups have long been identified as a major deficiency of the U.S. healthcare system [3]. With acknowledgment of this problem, some pharmaceutical companies (ex., Bristol Myers Squibb, Pfizer) are now committed to locating at least 25 percent of U.S. research sites for new clinical trials in racially and ethnically diverse communities [4,5]. It has also become a priority for research funding organizations, including public and private agencies and foundations [3]. Inequities of ability to enter clinical trials from remote rural areas became obvious. This issue is acutely evident in the Panhandle Region of Texas, prompting the initiation of this project.

Primary care physicians' awareness of ongoing clinical trials and ability to direct patients toward clinical trials is limited. It is almost impossible to keep up with busy clinical practice and follow all ongoing clinical trials for rare diseases. Other challenges may relate to physicians' ability to recognize rare diseases at early stages and knowledge of available access to clinical trials. Challenges with patient-reported health state utility values in rare diseases exist due to small, heterogeneous populations, lack of disease knowledge, and early onset.

This study focuses on developing and optimizing a strategy that makes clinical trials more available for a suburban and rural pediatric patient population with rare diseases. The study also will assess primary care physicians' knowledge about resources available to support clinical trials for patients with rare medical conditions.

METHODS The study's methodology encompassed interviews with physicians and patients within the target region. To evaluate knowledge among primary care physicians about available resources for patients' enrollment in clinical trials for rare medical conditions, we recruited 30 primary care physicians' clinics in the Texas Panhandle.

Pre- and post-participation surveys were designed to extract valuable insights from the parents of sick kids. Because rare diseases are identifiable in non-densely populated dwellings, patients were asked to sign an informed consent before entering the study. For pediatric patients, informed consent was signed by the parents. Children seven years and older were also asked to sign an informed assent. Patients were given a choice to use either a paper version or an electronic-based Qualtrics survey software system to answer the questions.

Data from these surveys were meticulously collected, transcribed into Excel, and subjected to detailed analysis using SPSS.

RESULTS Fifteen primary care pediatricians agreed to participate in the study. Within the Texas Panhandle Region, physicians were discovered to be treating a substantial number of pediatric patients diagnosed with rare diseases. However, the participation of these patients in clinical trials was disappointingly low. Notably, the primary obstacles encountered by physicians included their limited availability of time during clinical visits and a lack of knowledge about clinical trials. An intriguing revelation was the prevalent reliance on specialists for trial referrals, even though patients expressed a strong preference for referrals from primary care physicians.

The average age of pediatric patients was nine years old. Most of the patients were neonates (18%), infants (18%), or toddlers (20%) when they were diagnosed with a rare disease. Most participants were males (53%) and Hispanic/Latino (50%). Only 5% participated in clinical trials, 83% were interested in participating. Fear of unknown results and side effects is also shown to be the major reason for not participating among pediatric patients (33%). Thus, the majority believe that learning more about clinical trials would make clinical trials more attractive (20%). Fifteen percent and 8% of the patients were unfamiliar with rare diseases, clinical research, and clinicaltrials.gov, respectively. However, most would like to be notified about clinical trials by their primary care physicians or specialists (80% and 35%, respectively). Several factors have been identified to facilitate access to clinical trials, including telehealth enrolment (48%), electronic signature for informed consent (58%), home medication delivery (53%), and home nurse visits (48%).

Overall, the patients participating in the study exhibited a significant interest in engaging in clinical trials. Nevertheless, they confronted substantial barriers, including concerns about potential side effects and the financial burden of travel expenses. There was a prevailing desire among patients for more comprehensive information about trial details, with electronic enrollment and signature processes garnering notable support.

Feedback from post-survey patients indicated that the information they received proved to be invaluable. Ninety

percent of participants believed the information during the intervention was useful, and 20% became interested in participating in clinical trials. Twenty-seven percent of patients identified travel as the major barrier against their participation, and 25% believed that a web-based user-friendly network would be useful in accessing clinical trials.

CONCLUSION The study's findings underscore the evident challenges of referral and participation in clinical trials for patients in suburban and rural areas, particularly in the Panhandle Region of Texas. These challenges stem from constraints faced by both physicians and patients. While physicians possessed adequate knowledge about accessing trial information, the inherent time constraints during clinical visits impeded their ability to inform patients effectively. Consequently, patients remained largely uninformed about relevant clinical trials for their medical conditions.

The study emphasized a distinct preference among patients for referrals from primary care physicians, contrasting with the prevalent reliance on specialists. Despite these barriers, patients demonstrated a resounding interest in participating in clinical trials. Their persistence in the face of these hurdles underscores the immense potential for equitable participation in rare disease clinical trials within underserved areas. Nonetheless, the project highlights the importance of bridging information gaps and improving accessibility to fulfill this potential.

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