

Decision Aid to Support Shared Decision Making in Pediatric Refractory Immune Thrombocytopenia

Investigator: Cindy Neunert, MD, MSCS

Institution: Columbia University Medical Center

With multiple treatment failures among children with refractory ITP it is important to find a treatment that not only works, but that parents are knowledgeable about. Shared decision making is becoming increasingly more valued in the clinical setting, increasing communication between medical providers and patients. This is important for treatment decisions.

Prior to embarking on this study, there were no published studies that had been completed exploring the experience among parents with children with ITP in making treatment decisions alongside their child's medical provider. As a result, within this study the goal was to collect this information, and eventually use it in the development of a tool to help enhance information sharing between clinicians and families to ensure parents of children with ITP are fully knowledgeable about treatment options and are part of the decision-making process. Eventually, the tool would be available electronically to allow patients to provide pertinent information on their own time, and results would then be shared with the physician to facilitate communication. The shared-decision tool could also then be coupled with additional patient-related factors such as health-related quality of life, medication side effect assessment and bleeding severity to enhance overall patient care. The decision aid would also be intended to improve treatment effects with regards to patient-related outcomes and help to guide treatment decisions.

This project builds upon knowledge gained from the recent ITP Consortium of North America (ICON) ICON study, a comparative-effectiveness trial for pediatric patients with refractory ITP starting a second-line agent (n=117). Variation existed in selecting specific treatments, highlighting the fact that management of pediatric ITP is highly variable, and rarely based upon published guidelines in addition to being highly reliant upon parent/patient preferences and priorities. The study had two specific aims, however only the first has been completed as the project is on-going at this time.

Aim 1 (completed): Determine thematic areas surrounding therapy for refractory patients with ITP. This was accomplished by conducting focus groups with eligible parents recruited through Columbia University Irving Medical Center as well as through the Platelet Disorder Support Association and providing a structured interview with the group. Participants were asked about experiences with and preferences towards ITP therapy, what factors are important to them, what their goals for treatment are, how involved they were in the decision-making process, and accounted for variables such as proximity to treatment center, cost of treatments, ethnic/cultural beliefs, safety, and the age of the child with ITP. In addition, an assessment of educational and interventions provided to address these preferences was conducted. Focus groups were recorded, and transcribed, after being de-identified using specialized software. An analysis was then done to identify the nature, strength, and prevalence of key concepts identified.

Semi-structured interviews will be conducted with physicians caring for children with ITP across a variety of clinical practices to assess physician preference for integration of the clinical decision aid into clinical workflow.

So far, there have been four focus groups completed. One focus group occurred in person and additional focus groups occurred as virtual sessions. Analysis of the focus groups revealed the following codes and definitions as common themes when discussing second line therapy: 'Experiences with Treatment Decision Making', 'Factors Important in Decision Making', and 'Concept of Remission'. This data was accepted for a platform presentation at the American Society of Hematology Meeting December 2020.

What came out of the focus groups?

- Factors important to decision making appear consistent between participants
- Parents of children with refractory ITP did not always feel like critical decision-makers
- The concept of remission has different meanings to patients and clinicians
- Clinicians' views are not completely aligned with the patient experience

Many parents expressed that while their hematologist presented treatment options to them, the extent to which parents felt included in the decision was variable, and parents often felt they were not important in the decision-making process. Some parents reported not being fully educated about the options presented. Additionally, parents described that their child went through a 'trial and error' process without any notable or specific conversations that included the patient experience.

Most of the data collected suggest that parents have strong ideas about what communication around treatment options should be like, and what factors are important when making decisions about treatment. The information provided by these focus groups revealed that physician's views on choosing therapies 'based on patient preference' were not always aligned with the patient experience.

Aim 2 (to be completed): Develop and assess a prototype for the clinical decision aid.

Using information obtained from the focus groups, alongside members of the Columbia University Bioinformatics team, a prototype will be developed and then presented to the participants included in the focus groups. This will allow first stage feedback of the proposed decision aid. Overall, the function of the tool will be to increase patient understanding about available treatment options, improve targeted treatment effects with regards to patient-related outcomes, and help guide management decisions.

The decision aid will incorporate several decision-making steps, including engaging participants in the discussion about treatment options, explore treatment options, assess patient's values and preferences regarding treatment outcomes, reach a decision, and finally, evaluating the outcome of that decision.

Overall, the study confirmed there is a need for assisting physicians and patients to be engaged in shared decision making especially outside of specialized medical centers. Physicians and patients may differ in their interpretation of key concepts surrounding second line therapy, however working together is the goal. We plan to conduct semi-structured interviews with physicians across a variety of clinical practice settings.

Initial work from this funded project was published in *Blood* (2020) 136 (Supplement 1): 47.

PDSA has designed our research program to specifically focus on patient priorities and funds studies that will make a significant impact on ITP diagnosis, therapies, and quality of life. If you'd like to donate to our research fund, please visit <https://www.pdsa.org/pdsa-donation.html>.