The American Perspective: Participating in Research in ITP

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When I first meet with patients and families affected by ITP, I tell them all of the information that we know about ITP. As part of this conversation, I also always point out the many important questions about ITP that we cannot answer. Why do certain people develop ITP while others do not? How can we predict if there will be bleeding in the future? Will the ITP resolve on its own and, if so, when? If treatment is needed, which medicine will be most likely to improve their daily quality life? When I raise these questions, I acknowledge how frustrating it is that we do not have answers to these and other questions. These unanswered important questions are the reason that there is so much active research in ITP.

Because ITP is a rare condition, it takes many patients and researchers working together to answer these key questions. For this reason, your physician may offer you a chance to participate in research studies. Medical research can lead to a better understanding of how genetics and environmental factors lead to the development of ITP and how ITP and its treatment affect everyday life. Research studies can also help us to know which medications will work best for which types of patients. Participation in research is always voluntary and those who participate in research do so because they want to and have the choice to change their mind at any time. In order to make a decision about being part of a research study, it's important to understand the type of study being offered, what is needed to participate, and what is the potential for both harm and benefit. Sometimes, there may not be any benefit to you directly but the study will help us to learn more about ITP that may help others in the future. Before participating, the clinician leading the research must carefully explain the details of the individual study to you. An informed consent document is a written explanation of the study and the risks and benefits. When patients sign an informed consent form, it means that the clinician has carefully explained the study in detail and the patient has agreed to participate. Even though you sign this document, you can still choose not continue to be part of the research study at any time.

What are the different types of studies that a person with ITP might participate in?

Survey studies: Individuals answer questions about how they feel. These may be administered once or on a regular basis or at the time of new symptoms or treatments. Surveys are often included in drug studies to help determine if the drug helped patients with ITP to feel better.

Biology sample studies: Extra tubes of blood (or other types of body fluid, such as urine, saliva, or bone marrow) are collected, often when these are also being collected for your routine visit. Some samples are looked at right away while others are collected and stored in a biobank. A biobank stores information and samples and can be accessed by researchers who have

important questions that can be answered using these samples in the future. The researchers may analyze these samples in different ways including through genetic testing.

Observational studies: Information about an individual's symptoms, laboratory studies, and/or treatments are captured in a database. Observational studies sometimes also include surveys and sample studies. Sometimes the information is collected only once, and, other times, it may be collected at several different times to look for changes.

Clinical trials (drug testing/interventional studies): In clinical trials, individuals receive a research medication or treatment to determine whether it is safe and/or effective for ITP. There are several types of clinical trials. The goal of a phase I trial is to find the right (and safest) dose of a new treatment. A phase II trial evaluates whether a treatment is effective to treat ITP. A phase III trial compares the treatment to another treatment, standard of care, or to a placebo (a product that looks like the drug but has no drug effect).

These types of studies are all needed to answer important questions about ITP that we currently cannot answer. Since ITP is rare, investigators often work together in collaboration on research. Samples may be sent to a central repository, and a single clinical trial may be conducted at multiple centers across the globe. While participating in a study, an individual is assigned a study identification number so that his or her privacy is maintained and research teams at other hospitals cannot identify participants individually. The time and involvement needed to participate in research varies greatly between studies.

It can be frustrating for patients, families, and clinicians that important questions in ITP are currently inadequately answered. Offering participation in research is a vital part of addressing this lack of knowledge and providing hope that researchers can answer these questions in the future. Although many patients are offered participation in research, taking part in a research study does not make sense for everyone. Although research is offered, participation never impacts the clinical care that is provided.

If you are interested in learning more about participating in ITP research, please ask your health care provider which studies are open at your hospital or medical center.

Commented [RG1]: Does the UK ITP support group have a link to open trials that could be added here?