ITP Natural History Study Patient Registry

The ITP Natural History Study Registry is an international patient-consented registry of individuals with ITP. The registry aims to collect, store, and retrieve data on the natural progression of ITP, enabling collection of data on diagnosis and treatment, management of care, quality of life, clinician reporting, and characterization of the ITP population as a whole. The registry is administered by PDSA and overseen by the National Organization for Rare Disorders (NORD) and a committee of leading hematologists, ITP patients, and caregivers. Registry questionnaires were built from common data element standards:

1. Participant Profile
   - Country, race, insurance, education, employment

2. Medical and Diagnostic Data
   - Diagnostic tests, doctors visits, family members, female reproduction

3. Treatment and Review of Systems
   - Types of medications and frequencies/dosage, diet, surgeries
   - Disease progression:
     - Q: “Rate function over the preceding 4 week period: Vision with usual glasses or contact lenses”
   - Research history:
     - Q: “Is the Participant willing to be contacted in the future about research related to his/her rare disease?”
     - Q: “Does the Participant currently participate in any clinical trials related to his/her rare disease?”

4. Quality of Life Adult or Pediatric
   - Enjoyment of life, fatigue, sleep, pain, social activities, mental health, physical health
   - Q: “In the past 7 days, the Participant felt anxious...
     - Never/Rarely/Sometimes/Often/Always”
   - Q: “In the past 7 days, the Participant could do sports and exercise that other kids his/her age could do ... With no trouble/With a lot of trouble/Not able to do”
   - Q: “In the past 7 days, the Participant worried about what could happen to him/her ... Never/Rarely/Sometimes/Often/Always”

ITP REGISTRY BY THE NUMBERS

Official Launch Date: Rare Disease Day, February 28, 2017
Patients Enrolled within 2 hours of launch: 100
Patients Enrolled within 24 hours of launch: 226
Patients Enrolled within first month: 337
Patients Enrolled as of 10/1/2017: 514
Participants with Surveys Complete: 347

Access to the ITP NHS Registry data is contingent upon project approval by PDSA’s ITP Registry Advisory Board. Contact PDSA Research Coordinator, Alexandra Kruse, at research@pdsa.org with any questions. Last updated October 1, 2017.