Quality Of Life And Demographics Of Patients With Immune Thrombocytopenia (ITP); Data From The Platelet Disorder Support Association (PDSA) Patient Registry

Alexandra Kruse[a], Caroline Kruse[a], Jared Whitman[a], Nancy Potthast[a], Vanessa Boulanger[b], Allison Seebald[b], Suzanne Rossov[b], Kelsey Milligan[c]

[a]Platelet Disorder Support Association, Cleveland OH, USA, [b]National Organization for Rare Disorders (NORD), Danbury CT, USA [c]Trio Health Analytics, La Jolla CA, USA

1. BACKGROUND AND AIMS

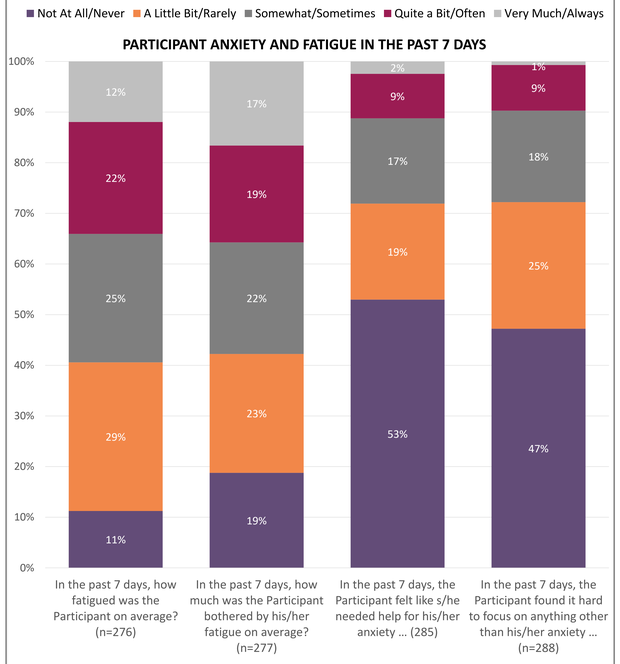
Patients with the autoimmune bleeding disorder Immune Thrombocytopenia (ITP) suffer from bleeding events as a result of low platelet counts. These events may manifest as bruises, petechiae, blood blisters, bloody stools, blood in urine, or even bleeding in the brain. The variability in pathophysiology and natural history of ITP makes it difficult to individualize care for these patients, and although patients have the option to receive treatment that includes medications (steroids, immunosuppressants, IVIG, TPO-receptor agonists) and splenectomy, many therapy options are accompanied by side effects, tolerability and toxicity issues. Quality of life for these patients is impacted by both the disease and the treatments themselves with patients commonly referring to concerns of anxiety and fatigue.

In 2017, PDSA in collaboration with the National Organization for Rare Disorders, launched the ITP Natural History Study Patient Registry to understand patient characteristics, their disease, disease management, and quality of life. The ultimate goal of the ITP registry is to provide data that help to establish patient-reported outcomes, leveraging ITP patients and caregivers as active participants in research. Here, we describe the demographics and quality of life for registrants to date.

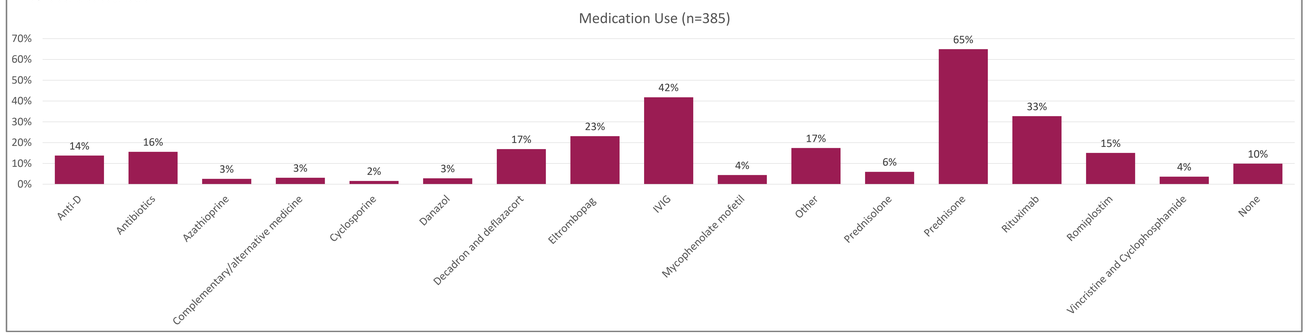
2. METHODS

The PDSA Registry utilizes six surveys covering patient demographics, medical and diagnostic information, treatment options, disease progression, and quality of life. As of February 2019, 538 patients have completed 1,975 surveys.

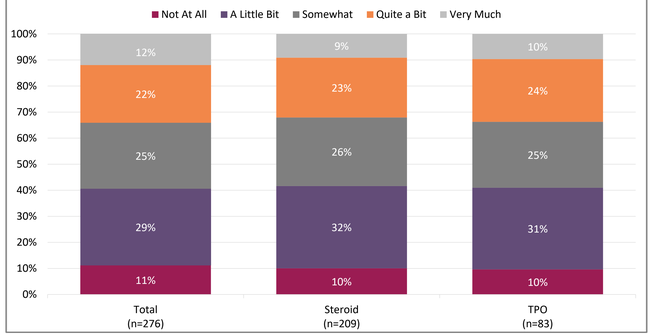
3. ANXIETY AND FATIGUE



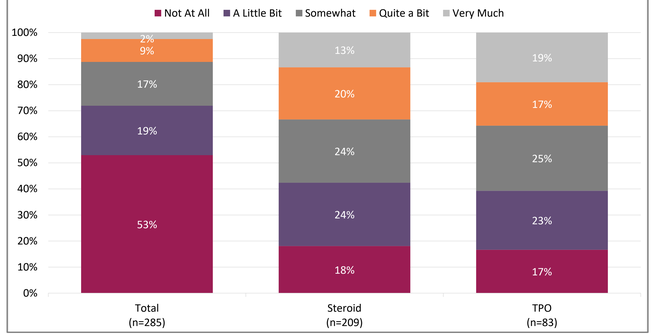
4. TREATMENT



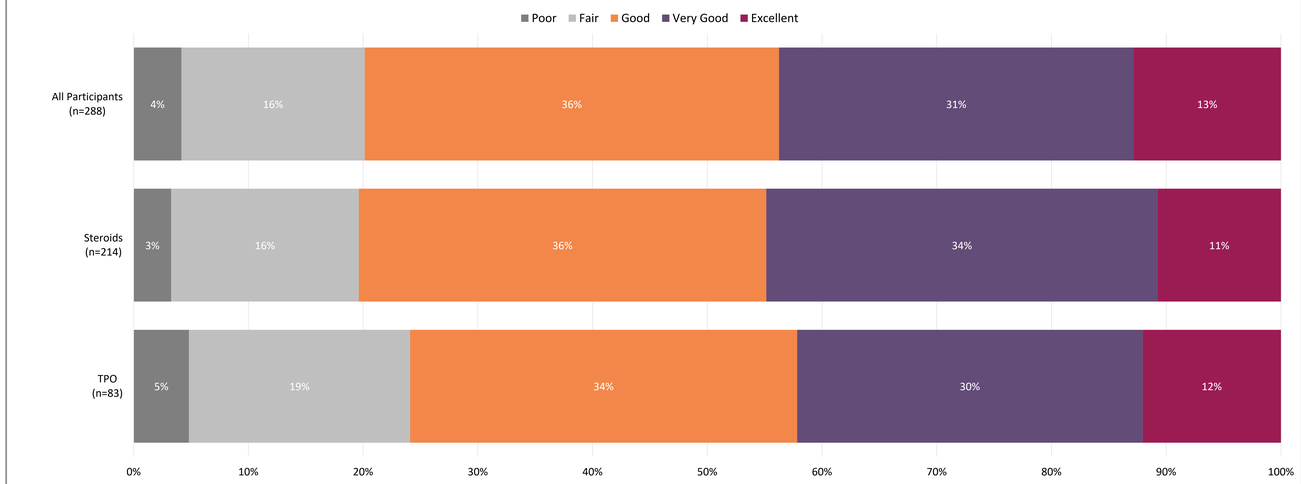
5. FATIGUE BY TREATMENT



6. ANXIETY BY TREATMENT



7. QUALITY OF LIFE BY TREATMENT



8. RESULTS

Patients in the registry (n=538) are mostly white (89%), female (76%), and reside in the United States. Among the registrants, 34% are enrolled in Medicare, Medicaid, or both, while 62% have commercial insurance. Average (median) age at diagnosis was 32 (31) years with 25% younger than 18 years. An official diagnosis took over one year after the onset of symptoms for 32% of patients.

When addressing questions corresponding to patient anxiety and fatigue, 11% of patients (n=285) stated they were often or always feeling like they needed help for their anxiety and 36% said that their fatigue bothered them quite a bit or very much *[section 3]*.

54% of registry participants provided data on treatment. These respondents (n=385) reported the use of 16 different types of treatments to manage their ITP. The most commonly used medications among registry patients are prednisone (65%), IVIG (42%), and rituximab (33%); 10% of participants reported that they did not use any medication. 27% of participants were splenectomized *[section 4]*.

89% of patients felt fatigued by their treatment; the same percentage of patients reporting fatigue was independent of treatment type *[section 5]*. On the other hand, although 47% of patients reported anxiety regardless of treatment type, 82% and 83% of patients reported anxiety with steroids and TPO agents, respectively *[section 6]*.

Quality of life was rated as poor to fair for 23% of adult patients who received a splenectomy (n=78), while 19% of patients who received another form of treatment (n=210), rated their quality of life as poor to fair. This demonstrates an improvement in quality of life for those who did not undergo the surgical option to treat ITP, but is not statistically different from the splenectomy group (p=0.447) *[Section 7]*.

9. CONCLUSION

The PDSA ITP Natural History Registry is comprised of registrants who are predominantly female, white, and diagnosed at a young age, with 1 in 4 participants diagnosed under the age of 18; these demographics are consistent with many other autoimmune disorders.

A third of the registrants indicate a prolonged time to diagnosis, >1 year, depicting the need to enhance diagnostic exams beyond diagnosing ITP based on exclusion or absence of other clinical variables. Patients receive an average of three different treatments to manage their ITP, demonstrating the difficulty in finding a proper treatment that works to elevate the platelet count, mitigate symptoms of disease, and limit potential side effects.

ITP affects quality of life across domains of emotional, functional, and reproductive health, and work and social life. These symptoms that accompany the disease interfere with daily activities and lead to anxiety, fear, depression, embarrassment of unexplained bruising, isolation, inadequacy, and frustration with a patient's inability to control their body and their health. We find that although levels of anxiety, while multifactorial, may correlate with type of treatment received, frequency of fatigue is persistent regardless of treatment type. Together, these multi-faceted effects of ITP often take a significant toll on patients' quality of life.

The registry continues to collect data with the intent of understanding the longitudinal impact of ITP.