ITP

Immune Thrombocytopenia

Platelet Disorder Support Association

Empowering ITP Patients

REGISTRY POWERED BY NORD

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Meet ITP Warrior LOGAN

Logan

My name is Logan and I am 13 years old. I love to play basketball, swim, and play video games. When I was 8 years old, on New Year's Eve, we got a call from my doctor telling us something was terribly wrong. My mom had taken me to see my doctor the day before because I was covered in terrible bruises, and we didn't know why. They told us to go to the Blood and Cancer Center, and didn't give us a reason, just that they were waiting for me. After many tests they decided I have immune thrombocytopenia (ITP).

I was TERRIFIED of needles. I've had many treatments and probably more than 100 blood draws, each of which included being poked with a needle. The hardest thing I've had to deal with is being separated from my friends. When I was first diagnosed, some friends avoided me for fear of catching my disease and even told me that I wasn't invited to birthday parties because their parents were worried I would get hurt. I miss school a lot because when my platelets are really low, the fatigue is so bad and just getting out of bed is tough and all I can do is sleep. It's hard to explain to people how I feel and that I'm not making things up in my head because I "look fine." Kids my age have no idea what a platelet is and friends and even teachers think I'm pretending to be sick to get out of doing things. It's no fun making up missed assignments or missing out on daily school activities and fun times with classmates and friends. It's also hard to watch other kids taking part in "normal" activities and sports when I can't because of ITP. After an accident at school where I hit my head, I had to be admitted to the hospital. Luckily my brain was okay, and I wasn't bleeding there, but my platelets had dropped to about 3,000. After being in the hospital for a few days, a treatment helped raise my platelets to safe levels and I could finally go home.

I feel like a big help could be the ability to get an at-home CBC, so I don't have to go to the hospital all the time, or even something that constantly monitors platelet counts, like a Dexcom® continuous glucose monitor, but for platelets. This would help reduce pokes and help patients safely take part in sports and other activities with confidence, so people with ITP could live life.

What is ITP?

IMMUNE THROMBOCYTOPENIA

Summary

ITP, immune thrombocytopenia (also known as immune or idiopathic thrombocytopenic purpura) is an autoimmune bleeding disorder characterized by abnormally low levels of blood cells called platelets, a situation which is referred to as thrombocytopenia. In ITP, platelets are marked as foreign by the immune system and eliminated via the spleen, the liver, and by other means. In addition to increased platelet destruction, some people with ITP also have impaired platelet production.

Platelets are specialized blood cells that help maintain the integrity of our blood vessel walls. Platelets also help prevent and stop bleeding by accelerating clotting. A normal platelet count ranges from approximately 150,000 to 400,000 per microliter of blood, depending on the laboratory. If someone has a platelet count lower than 100,000 per microliter of blood with no other reason for low platelets, that person might have ITP. ITP is generally called newly diagnosed when it has been present for less than 3 months, persistent when present for 3 to 12 months, and chronic when present longer.

With few platelets, people with ITP often have bleeding symptoms such as spontaneous bruising, petechiae, tiny red dots on the skin, or for women, heavy menses. More severe bleeding symptoms include blood blisters on the inside of the mouth, blood in the urine or stool, and bleeding in the brain.

While it may seem like ITP is a simple disease, there are many nuances to the diagnosis, mechanism of disease, and management, in addition to the variability of outcomes between and among children and adults. This includes variation in the severity of bleeding at any given platelet count, as well as how individual patients respond to various forms of treatment.

In addition to serious physical bleeding-related manifestations of the disease, ITP is also associated with debilitating fatigue; impaired quality of life (QoL) across domains of emotional, functional, and reproductive health; work life, and social life. These symptoms that accompany the disease may interfere with daily activities and lead to anxiety, fear, depression, embarrassment of unexplained bruising, bleeding, epistaxis, isolation, inadequacy, and frustration with a patient's inability to control his or her body and health.

Management depends on severity of symptoms, platelet count, age, lifestyle, response to therapy and its side effects, the presence of other medical issues that affect the risk of bleeding, QoL as discussed above, and, of course, personal preferences of both the patient and the physician.

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Meet ITP Warrior JOEY

Joey *(as told by his mom, Leilani)*

We first noticed that something might be wrong with Joey when he was just 2 years old. His body was covered in bruises – way too many to count. We expressed our concerns during Joey's 2-year checkup, and his pediatrician ordered lab work. Late that night, the phone rang. The doctor said Joey's platelet count was only 18,000 and told us to come straight to the emergency room. "Your son could be bleeding internally," he said. After several more tests, Joey was diagnosed with ITP. He spent the rest of the year in and out of the hospital trying to bring his platelet count to safe levels.

We were handed the diagnosis of a rare disease, had few answers, and began to worry. We had so many questions about Joey's future. Will Joey have a future, or can he bleed to death? Will he be able to live and play freely like other children? Will he be happy? Overwhelmed and helpless, we couldn't help but feel that we were somehow letting Joey down. As parents, we try to protect our children from the dangers in the world, but how were we to protect Joey from something we had never even heard of?

Eight years after the shocking diagnosis, Joey continues to struggle with ITP and so do we. He has endured myriad tests and doctor's appointments. His biggest challenge has been being able to play like a normal kid. When Joey was diagnosed, he was restricted to "light play." These restrictions have guided his way of life since then. Joey has been advised by his doctor that he will need to re-evaluate the sports that he participates in because it gets more dangerous for him since he never knows when his platelet count is low. We often find ourselves obsessing over Joey's platelet count. It is always on our minds. Always. Every year we must educate his teachers and coaches about ITP and the importance of contacting us immediately if he is seriously injured. Joey feels embarrassed and has not shared with his friends his medical condition. He refuses to wear his medical ID bracelet. We get it. It's hard being different from everyone else. So, can you imagine how hard it must be for him to navigate fourth grade living with a chronic condition without the support of his friends? His platelet count remains low and he constantly questions why he has ITP. Maybe one day he will share his condition with his closest friends.

When people see Joey, they don't know his daily struggles, because he appears to be a "healthy" 10-year-old boy who tries to live a "normal" life. He does well in school and is part of many activities such as acting, dance, and his elementary school's Leadership Academy. Joey also participates in baseball, basketball, and soccer when his platelet count is at safe levels.

Joey believes the only solution to ITP is to find a cure, and his dad and I share in that hope.

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How Is ITP Diagnosed?

There is no accurate, definitive test to diagnose ITP. Rather, it is a diagnosis of exclusion. When all other causes of low platelets are considered and ruled out, the diagnosis is ITP. There are, however, many possible causes of low platelets and more are being discovered each year. Therefore, it is possible that the diagnosis of ITP is given when there is an underlying illness, genetic anomaly, environmental trigger, or some other reason for low platelets. A misdiagnosis may prompt an incorrect or potentially harmful treatment.

What Causes ITP?

While no one knows the precise cause of ITP at this time, researchers continue to make progress in understanding it and the similarities it has to other autoimmune diseases. Since ITP may vary greatly between individuals, causes of ITP may also vary.

Current theories suggest that bacteria and virus byproducts, free radical damage (oxidative stress), immune system defects, and intestinal changes all may play a role in the development of ITP. Some cases of ITP may be drug-induced, the result of eating particular foods, or may be caused by other diseases.

Treatment

OVERVIEW

While there is no well-established cure for ITP, fortunately almost all patients find their platelet count improves following treatment. What proves difficult for many patients with ITP is finding the treatment that works for them without unwanted side effects. In some individuals, the disease may go into remission for an extended period of time, perhaps for the remainder of a person's life. ITP may also recur at any time, and there is currently no way to predict the course of the disease.

Criteria for treatment: In many children and some adults, therapy may not be necessary at the time they first see the physician, and the disorder may resolve spontaneously. The decision to initiate treatment depends on the severity of bleeding, the severity of the thrombocytopenia, the age of the patient (increased risk of bleeding in adults and especially in the elderly), coincidental disorders that might predispose to bleeding (tendency to fall, concurrent antiplatelet or anticoagulants), lifestyle (eg, youth and athletic), and risks and side effects of each intervention. These same factors may also contribute to deciding which treatment to use.

FIRST-LINE/EMERGENCY THERAPY

Treatment with corticosteroid drugs (eg, prednisone, dexamethasone, methylprednisolone) is usually the mainstay of initial therapy. These drugs function by suppressing the clearance of antibody-coated platelets and perhaps by increasing platelet production. They may also decrease the risk of bleeding by improving blood-vessel lining cell function. Very high doses

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Meet ITP Warrior KRISTIE

Kristie

During the fall of 2013, I was enjoying life as a typical 28-year-old: newly married, we just had gotten a puppy, my husband and I were starting to plan some trips, and I was excelling in my career as an educator. I had been randomly bruising on and off for months and it wasn't until September of that year that the bruises became very large and troublesome. After seeing my doctor and running some blood work, it was determined I had a condition known as ITP. It seemed my entire world crashed all around me and I didn't know who to turn to or which direction was up.

After a course of high-dose steroids that worked for several months, a few rounds of IVIg that worked for even longer, and a miscarriage, I learned I was pregnant again in 2016. My platelet count had been teetering around 75,000 without any medications or treatments and now I was terrified of having to deal with this disease while carrying our new baby. From the moment I found out I was pregnant, my platelet count stabilized. I remained in remission my entire pregnancy with platelet counts above 150,000; I continued my remission for 14 months following the birth of our son. He was not only my rainbow baby, but my miracle baby who gave me my life back. Our son did what no doctor or treatment had been able to do...put me into an extended remission.

I suffered 3 major relapses in 2018 after having contracted the flu. Over the course of 7 weeks I went through 3 rounds of IVIg and have remained in remission since June 2018. Over the past 5 1/2 years, I've learned to take one day at a time and enjoy every moment. Having ITP has really tested me to no end, but it's also changed me as a person. It's made me appreciate life and all the little moments. The hardest part about this disease has been handling its uncertainty and spontaneity. But I refuse to let it control my life, because I've learned I'm in control of my own happiness. I don't know if I will see a cure for ITP in my lifetime, but I'm hopeful I will. In the meantime, I'm taking each day as it comes and living life to the fullest because this is my story and it isn't over yet.

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(especially of dexamethasone) may impair the production of antiplatelet antibodies with the hope that the platelet count will remain elevated after the patient stops taking the medication. Additional studies are, however, needed to affirm the long-term benefit of such a "high-dose" approach. In general, the duration and dose of corticosteroids should be minimized because of their immediate and long-term side effects. Corticosteroids are, therefore, used to control the disease until a transition to other forms of treatment may be initiated in those patients who do not achieve a spontaneous remission.

If platelet counts do not improve after corticosteroid treatment, or when individuals present with severe bleeding, treatment may include adding IVIg. IVIg is usually administered every 2 to 4 weeks by infusions on an as-needed basis, depending on the count and bleeding. This rarely leads to a cure. Platelet transfusions are reserved for emergent situations because they are likely to be destroyed relatively quickly by the autoantibodies.

The orphan drug anti-D (WinRho SDF®, Rhophylac®), a specific form of gamma globulin, was approved by the FDA to treat ITP in individuals who are red blood cell RhD antigen positive, do not already have antibodies on their red cells, and have not undergone a splenectomy. The drug may be used repeatedly, including in children who have the acute or chronic form of ITP. Concerns have been raised, however, because of a small number of individuals who have had severe side effects from brisk red cell destruction and its consequences soon after infusion.

SECOND-LINE THERAPY

One option in the second-line setting involves the use of thrombopoietin receptor agonists (TPO-RAs). TPO-RAs function by stimulating the body's production of platelets by megakaryocytes in the bone marrow, which release proplatelets that mature into platelets. By increasing the rate at which platelets are produced in the body, TPO-RAs may overcome the heightened rate of platelet destruction caused by antiplatelet antibodies and their ability to impair megakaryocyte function. Two TPO-RAs approved by the FDA for use in ITP are eltrombopag (Promacta®) and romiplostim (Nplate®), while others are in development or are approved for other related indications.

In 2015, eltrombopag was approved for the treatment of ITP in children 1 year and older who have had an insufficient response to corticosteroids, immunoglobulin, or splenectomy; romiplostim was similarly approved in late 2018. Response rate (depending upon the definition of response) to both agents ranges from 40% to 80% and, once obtained consistently, was durable with ongoing treatment. The drugs are generally well tolerated and long-term safety studies have mitigated initial concerns about thrombosis and bone marrow scarring. Some patients (an unknown percentage) will experience sufficient improvement in their ITP over time to discontinue treatment.

Another option is the anti-CD20 antibody, rituximab (Rituxan®), which reduces IgG antibody production; there are now several biosimilars. About half the patients respond initially, but only 20% to 30% are cured in long-term outcome studies. Women of childbearing age with ITP for <1 to 2 years have a >50% cure rate; all others have a very low rate of cure. Rituximab is

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Meet ITP Warrior JOHN

John

I was diagnosed in 1982 with ITP after being discharged from the Marine Corps due to excessive bruising. They felt I joined the Marines to get medical care. A month later I got a nose bleed that lasted 9 hours. I had to take a 3-day boat ride from my small town in Alaska to Seattle, because no one in Alaska could treat me. I got an apartment, a hematologist, 6 weeks of steroids, 10 pints of my own blood, found a surgeon, and had my spleen removed. I did this all by myself at the age of 17.

After that I went into remission until I was 45 years old. I woke up one morning with petechiae all over my arms and legs. I saw my hematologist weekly. My wife of 13 years divorced me because of my ITP. I tried prednisone, IVIg, Nplate, dexamethasone, Promacta, and Rituxin, and when I tried vincristine in 2015 I was so sick from treatments that I stopped working. I got very depressed from being on the medications and being alone, and I tried to commit suicide.

Today my biggest challenge is to stay positive and live my life to the fullest without living beyond my health means. I wish the medical establishment would understand that ITP is not just about the blood. It is about the mind and the energy level, too. I hope one day they will treat all of me. Maybe one day they will look into gene therapy.

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generally well tolerated, but infusion reactions may occur. Administration may be repeated when a durable response has been seen, but concern over repetitive administration of this immunosuppressant is warranted.

A third option is a splenectomy (typically laparoscopic), because the spleen plays a major role in destroying antibody-covered platelets and in making antiplatelet antibodies. Splenectomy improves platelet counts in approximately 70% of patients initially and may induce a long-term remission in 60%. The high long-term success rate must be weighed against the lower, but there is a genuine increased risk of thrombosis and serious infection that necessitates appropriate vaccinations and urgent evaluation for serious febrile illnesses. Most guidelines recommend deferral of a splenectomy for a year from diagnosis in order to determine if the patient will go into remission. Splenectomy does, however, remain an option in patients who fail other forms of treatment or in resource-challenged areas where more expensive alternatives are not available.

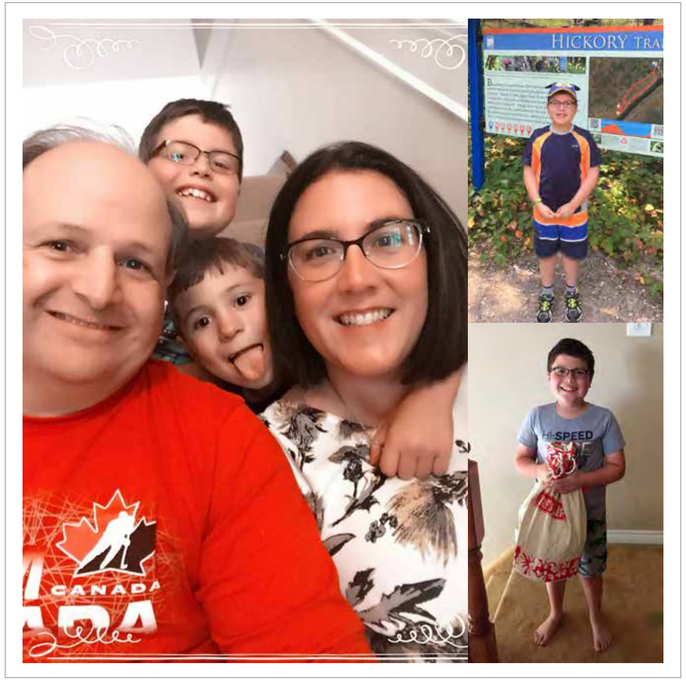
Tavalisse™ (fostamatinib disodium hexahydrate) was also approved by the FDA in 2018. It is indicated for the treatment of thrombocytopenia in adults with ITP who have had insufficient response to a previous treatment. Approximately 20% of patients refractory to other forms of management responded based on prespecified criteria, but almost 40% did so using less stringent but still clinically meaningful endpoints. This medication has a number of side effects (hypertension, diarrhea, headache, and abnormal liver tests) but one advantage is that it has the least risk of thrombosis of any licensed treatment of ITP.

THIRD-LINE THERAPY

A small percentage of patients fail to respond to or tolerate first-line or second-line treatments. For those individuals, options include dapsone, Imuran® (azathioprine), Cytoxan® (cyclophosphamide), Sandimmune® (cyclosporine), Danocrine® (danazol), CellCept® (mycophenolate mofetil), and vincristine (vinca alkaloids) or combinations. Several other novel forms of treatment are in clinical trials.

If the patient has antibodies or evidence of Helicobacter pylori infection, treatment with antibiotics and proton pump inhibitors may ameliorate the condition. Antibiotic-associated remission of ITP is much more common in Asia and in some parts of Europe than it is in patients who have lived their entire life in North America.

Meet ITP Superhero LUCA



Luca *(written by his parents, Jennifer and Luigi)*

Luca was a happy, energetic, considerate, and loving boy. He was diagnosed with ITP at age 7 following an outbreak of petechiae primarily on his back. We were questioned about potential child abuse, but following a blood draw we learned his platelet count was very low. Initially, Luca was considered a "nonbleeder" and we followed a "watch and see" approach. During year 2 of his having ITP, his average platelet count decreased, and he had minor nosebleeds. Halfway into his third year with ITP, Luca became a bleeder. During this time, Luca started experiencing frequent long-lasting nosebleeds, some of which required an emergency room (ER) visit to stop. He developed mouth blisters, gum bleeds/wet purpura, widespread petechiae, lots of bruises, a stomach bleed including throwing up clots, and blood in his urine. He developed a broken blood vessel once in his eye that we suspect was due to his low platelet levels. He started to develop petechiae on his head that would bleed and scab over. He had mild headaches. We were constantly reminded that brain bleeds are rare. Luca grew resistant to steroids and most first-line treatments. Rituxan isn't covered in Ontario for ITP, so he was treated during active bleeds. With platelets under 5,000, Luca developed a mild fluctuating headache and excessive fatigue. His doctors felt he was just sick. The following day, he slurred his words and was rushed to the ER with an intracranial hemorrhage (ICH). He had two craniectomies and died just days later, at the age of 10. We are unsure if Luca's ICH was spontaneous, or due to the pressure from a bike fall he had less than a week before (he was wearing a helmet), or other casual effects of active play.

The biggest burden was fear. Once Luca became a bleeder, he felt like "a ticking time bomb." Bleeds occurred with little warning when we were about to leave for school and work, during school in front of Luca's peers, or just before bedtime. We managed Luca's anxiety through cognitive behavioral therapy (CBT), art therapy, and reiki.

The solution is to observe the platelet threshold below which a child has serious bleeds and to manage not just rescue. Barriers to "off-label" treatments for rare disorders should be removed. Maintain vigilance for all possible signs of ICH and risk factors for ICH (change in bleeding pattern, severe chronic thrombocytopenia, gastrointestinal bleeds, hematuria, and steroid resistance).

Who we are: PLATELET DISORDER SUPPORT ASSOCIATION (PDSA)

Vision

To be recognized as the premier resource for patients, their families, health care providers, and government agencies who want to know about the symptoms and treatment of ITP and other platelet disorders.

Mission

The Platelet Disorder Support Association is dedicated to enhancing the lives of people with ITP and other platelet disorders through education, advocacy, research, and support.

PDSA Overview

Patient-founded in 1998 to educate and empower those with ITP and other platelet disorders, the PDSA is now a powerful force serving and unifying the global community of patients, practitioners, caregivers, advocates, and key disease stakeholders dealing with ITP. We build awareness, educate the global community, and provide critical connections and resources that empower patients to take charge of their disease and encourage practitioners to exercise patient-centered medical care. The website gets 90,000 visitors per month from more than 130 countries

PATIENT EDUCATION: PDSA is home to the most extensive patient-focused library of current disease, treatment, and research information available online and in print, including

- **Twenty-nine educational booklets available in multiple languages**
- **Numerous scientific journal articles**
- **Two quarterly and one monthly newsletter to report the latest research, treatments, patient journeys, and ITP community-related news**
- **Video Insights – an extensive collection of patient stories and educational insights from world-renowned experts**
- **The Annual ITP Conference connects the global community of patients, caregivers, practitioners, and key disease stakeholders to transform the future for people with ITP and other platelet disorders**

AWARENESS AND ADVOCACY: PDSA is committed to ongoing awareness and advocacy, and collaborates with other patient advocacy groups, researchers, and government agencies to drive public policy, develop new treatment options, and fund research to find a cure.

- **ITP Awareness Month: Since it's designation in 2010, PDSA celebrates each September with education and awareness-building activities, including Sport Purple for Platelets Day℠ and Global ITP Awareness Week℠**

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Meet ITP Warrior HAYLEY

Hayley

It started out as fatigue, unexplained weight gain, and the belief that "everything is downhill after turning 30," progressed to excessive bruising, blood in my stool, and a menstrual cycle worse than post childbirth, and ended with a diagnosis of ITP in December 2011. Before my official diagnosis, I had an abnormal but inconclusive colonoscopy, so I called my OB/GYN and requested a blood test. I was shocked when a nurse called: my platelet count was 5,000, I needed to pack a bag to be admitted to the hospital for what would likely be an extended period of time, I was to go directly to the cancer center at our local clinic, and under no circumstances was I to drive myself. The first-line treatment was high-dose steroids. When my platelets began to drop as I tapered off prednisone, it was decided I would have my spleen removed. As a result of my splenectomy, I developed a life-threatening blood clot in my portal vein. During my 2-week hospital stay I was constantly monitored. My platelets had to be high enough so that my blood would clot, all while I was being treated with IV blood thinners to dissolve the clot. All of this occurred during the first 5 months of being diagnosed.

At the time I was a stay-at-home mom to my 7- and 4-year-old sons. The ITP and related health issues cost me and my family lost time together while I spent what added up to months of time in hospitals and clinics during my first 3 years with the disorder. The blood clot in my portal vein caused increased pressure in my esophageal veins. This caused veins to rupture and bleed to the point of massive blood loss. One such event in 2015 left me needing 17 transfusions of blood products and being placed on life support for 3 days during which an emergency procedure was performed that saved my life. I then spent another 2 weeks in the hospital. After two separate treatments of rituximab, my ITP has been in remission since April 2013. The portal vein clot will always be there, which means I'll be on blood thinners for the rest of my life. My esophageal veins are currently monitored via a yearly endoscopy, but it has sometimes been as frequent as monthly. I also see my hematologist every 4 to 8 weeks.

One of the hardest aspects of being diagnosed with a rare disease is the feeling of loneliness that comes with it. It doesn't matter how good your support is at home, when diagnosed with a rare disease you've never heard of or met anyone else with, you feel completely alone. I'd love for doctors to share information about PDSA and its annual conference and local support groups with those patients newly diagnosed. That way they would have someone to talk with who has been through the roller coaster of this illness.

Who we are: PLATELET DISORDER SUPPORT ASSOCIATION (PDSA)

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- **Advocacy Partners: AARDA – American Autoimmune Related Diseases Association; A-Plus – American Plasma Users Coalition; ASH – American Society of Hematology; CPAG – Coalition of Patient Advocacy Groups; FDA Alliance, Genetic Alliance, IAPO – International Alliance of Patients' Organizations; ICON – Pediatric ITP Consortium of North America; International ITP Alliance; NORD – National Organization for Rare Disorders; PBSA – Patients for Biologics Safety and Access; PPTA – Plasma Protein Therapeutics Association; THSNA – Thrombosis and Hemostasis Society of North America**

RESEARCH PROGRAM: PDSA supports investigations that examine the pathogenesis and management of primary ITP and present the most promising outlook to significantly improve diagnosis, therapies, and patient QoL.

- **The Barbara and Peter T. Pruitt Jr. ITP Research Award: Two $20,000 grants awarded annually to investigators conducting innovative ITP patient-centered research**
- **ITP Natural History Studies Registry: International patient-consented registry of individuals with ITP collecting data on the natural progression of ITP**
- **ASH Friday Morning ITP Breakfast and ICON Dinner Meeting: Annual premier scientific meeting and non-CME research conference held at the ASH Annual Meeting and Exposition**
- **Clinical Trials Guide: Description of and international listing of controlled patient studies**

PATIENT SUPPORT: Patient-centered programs and services connect patients with ITP and caregivers, build awareness, promote community, and empower patients with ITP to take control of their disease.

- **ITP Helpline℠: Free personalized patient support and referral service answering more than 5,000 calls and emails each year**
- **ITP Patient Connect℠: 47 local support groups including those in the United States, Canada, and New Zealand, 2 groups via teleconference, the PDSA.org Discussion Forum, Facebook page and closed group, Twitter, YouTube, Vimeo, and Instagram channels**
- **POKE-R℠ CLUB for Kids With ITP: Award-winning clinical support program developed to empower kids with ITP**
- **Financial assistance to senior high school students, college students or adults interested in continuing education who are living with ITP or a similar inherited or non-inherited platelet disorder**
- **Pump It Up For Platelets℠! National Walk/Run: Volunteer-driven events connect patients with ITP and caregivers, offer an opportunity for individual engagement, and raise awareness**

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Meet ITP Warrior BARBARA

Barbara

I am a 61-year-old woman with ITP. I've never known myself without ITP, because I was diagnosed at the age of four. Yes, it has been a lifelong "monkey on my back," but it hasn't kept me from living my life. I have had a full life. I had a wonderful childhood, I went to nursing school, fell in love and got married, had a daughter, adopted a son, raised my kids, watched them get married, and am now enjoying my first grandchild! My husband and I have been married for 41 years now, and I have been fortunate enough to have him by my side.

My journey with ITP has been long and arduous. I have only had 4 months of remission since I was diagnosed. I have had many unsuccessful treatments, and I have fallen victim to many of their side effects. I've lost my hair, had numbness in my fingers, had acne all over my body, had the moon face and back hump of steroids, muscle and body aches, joint pains, insomnia, depression, migraine headaches, extreme fatigue, kidney damage, liver damage, high blood pressure, diarrhea, a compromised immune system, and premature ovarian failure, just to name a few. ITP has left me without a spleen and the knowledge that I have had a micro-bleed in my brain. I can't recall a time when I didn't have any bruises. I remember as a child, our neighbors thought my parents were beating me, until they found out I had ITP. I always have petechiae, too, usually on my feet and lower legs. These are signs of bleeding to which I need to constantly pay attention.

I realize that I walk through life quite differently than a "normal" person. I look for the wide-open spaces, to keep myself from getting bumped into. I avoid sharp knives and sharp corners. I am always acutely aware of my surroundings. I realize my limitations with activities that have the potential of harm. This all comes with the territory of having ITP. But that isn't the worst of it. The hardest thing about having ITP is knowing that I could easily bleed to death in a simple accident.

Fortunately, though, I am not one to dwell on the negative. I believe in living life and loving life and finding the joy and beauty that surrounds us. I feel I am here for a reason, and I try my best to advocate for those with ITP. Walking through life with only 3,000 to 7,000 platelets is daunting, but there is always hope. Hope that a new treatment would work for me. And hope that there will be funding for more research, because without research, there is no cure in sight.

ITP
IN THE REAL WORLD

Clinical Story

Patients with the autoimmune disease 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. Treatments vary by severity of disease, but include medications (immunosuppressives, IVIg, platelet boosters) and splenectomy. Both the disease and the treatments affect QoL for these patients, who commonly state concerns of anxiety and fatigue. In 2017, the PDSA, in collaboration with NORD, launched the ITP Natural History Registry to understand patient characteristics, their disease, disease management, and QoL. Here, we describe the demographics and QoL for registrants to date.

The PDSA Registry contains 6 surveys covering patient demographics, medical information, diagnostic information, treatment utilization, disease progression, and QoL. As of February 2019, 538 patients had completed 1,975 surveys. Patients in the registry are mostly white (89%, 470/528), female (76%, 399/528), and reside in the United States. Approximately a third of patients (34%; 170/499) have Medicare, Medicaid, or both, while 62% (311/499) have commercial insurance. The average age at diagnosis was 32 years, with a median age of 31 years. A quarter of patients in the registry (25%; 125/502) were diagnosed at an age younger than 18 years. Time from onset of symptoms to diagnosis was more than one year for 32% (169/528) of patients. In response to specific questions regarding anxiety and fatigue, 11% (32/285) of patients stated they were often or always feeling like they needed help for their anxiety and 36% (99/277) said that their fatigue bothered them quite a bit or very much. Of the 538 registrants, 288 provided data on treatment and 27% (78/288) indicated having had a splenectomy. For patients who received a splenectomy, QoL was rated as poor to fair for 23% (18/78). For patients who had not had a splenectomy, 19% (40/210) rated their QoL as poor to fair, which is improved, but not statistically different from the splenectomy group (z=.758, *P*=.447). Quality of life did not differ significantly for patients using steroid treatments or thyroid peroxidase (TPO) from the total patients in the registry (significance level of .05, for the response "Poor" or "Fair," patients using steroids compared with the total registry: z=-.142, *P*=.889; patients using TPO compared with the total registry: z=.780, *P*=.435), though this could be due to significant overlap in the samples. Fatigue in patients using steroids or TPO also did not significantly differ from the total registry fatigue (significance level of .05, for the response "Not At All," patients using steroids compared with the total registry: z=. 417, *P*=.674; patients using TPO compared with the total registry: z=.409, *P*=.682), though anecdotal evidence would suggest there may be a correlation between fatigue and steroids or TPO. Data collection continues through the registry with the intent of raising disease awareness and understanding the impact of the disease.

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Meet ITP Warrior AYLA

Ayla

I am a "normal" 17-year-old senior in high school. I get good grades, hang out with friends, love watching and making movies, and I am passionate about playing the piano and violin. I play in multiple orchestras and have won many awards for my films. From the outside looking in, everything looks good!

What people do not know is that at 3 1/2 years old, I was diagnosed with immune ITP. For the last 13 1/2 years I have been fighting this life-threatening and chronic disorder like a boxer in a heavyweight fight. I have been called a "bad bleeder," and a "modern medical mystery," and I have spent hundreds of days in the hospital, coming near death multiple times. I have tried at least a dozen different types of treatments and have been on them for hundreds and hundreds of days. I spend more days feeling bad than good. Even after all these years, I am only managing my condition and staying alive by being on multiple treatments.

The struggles with a condition like this are many. They include fatigue, a battered body, blood blisters in my mouth (which make it impossible to eat) and severe bleeding where even the tiniest bloody nose could land me in the hospital, including one time when I lost 50% of the blood in my body. I've endured more than 300 IVs, blood draws, and finger pokes, 900 days of prednisone, and had my spleen removed at age 7.

The hardest thing I have dealt with from the beginning is how all of this continuously affects my quality of life. I have never been like other kids. I could not go to birthday parties that revolved around physical activities. I have not been able to experience sports or skiing like most kids. I have not known freedom to do what I wanted without the possibility of serious repercussions. I missed so much school that I was not able to take the classes I wanted, which has affected my opportunities for college. I would have loved to have gone to an out-of-state film school, yet my fear of leaving my doctors hindered that option. I have been tethered to a hospital and have lost a lot of freedoms that most people take for granted. I have lived in fear for too many days wondering if ITP would take my life. Yet through it all, the biggest burden is the unknown. Every day I live not knowing if this condition will one day end my life. And while I tell people "ITP is something I have and not who I am," I continue to pray for that cure and a reprieve from a life of anxiety, fear, and pain.

The solution for me is time, money, and effort to research not only how ITP may be better managed, but also to one day cure it. I would love to enter my adult years knowing that there may be an end in sight to help me live pain-free without worrying about bleeding to death.

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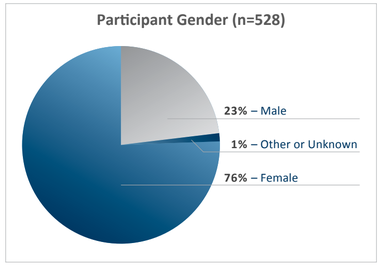
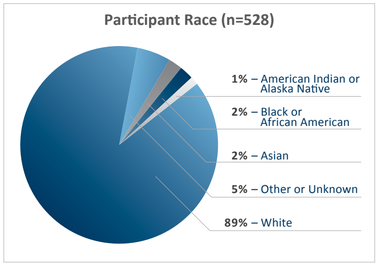
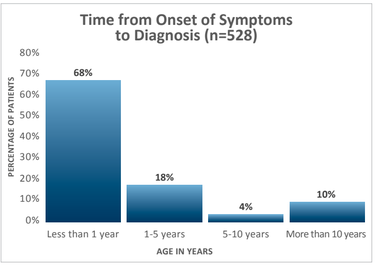
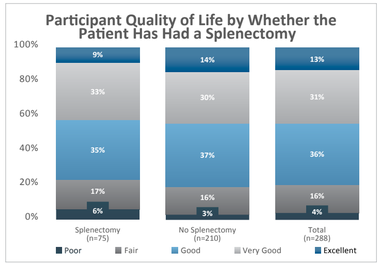


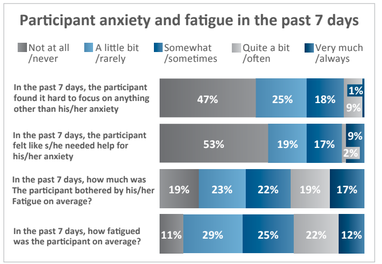


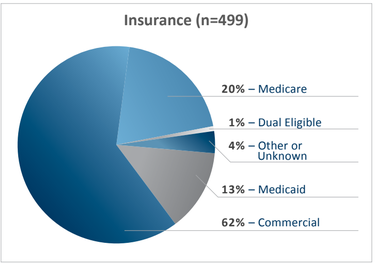


Registry participants are mostly white (89%, 470/528) and female (76%, 399/528).

The majority of participants were diagnosed within 1 year, though 10% (53/528) of participants were not diagnosed for more than ten years after the onset of symptoms.



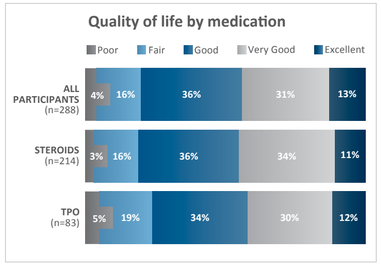


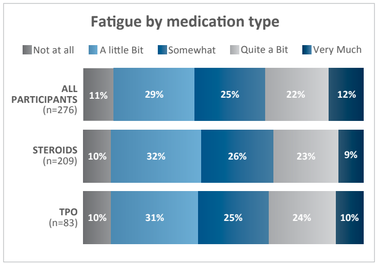


Participant quality of life did not vary significantly by whether or not the participant had had a splenectomy, though the splenectomy procedure is known to cause side effects.

Registry participants reported that fatigue was an issue more often than anxiety.

62% (311/499) of participants in the registry have commercial insurance.





Participants in the registry did not significantly differ on quality of life by medication, though both TPO and steroids are known to have difficult side effects.

Though participants in the registry did not report a significant difference in fatigue due to medication, there has been anecdotal evidence to suggest a link may exist.