

# THE PLATELET NEWS

*The Exclusive Publication for PDSA Members*

**At Every Turn, Faith,  
Hope, and Family Keep  
ITP Patient Brenda Shy  
Moving Forward**



**Education. Advocacy. Research. Support.**

## PLATELET DISORDER SUPPORT ASSOCIATION

**Caroline Kruse**, President and CEO

**Jeff Cooper**, Website and  
Social Media Manager

**Jennifer DiRaimo**, Research Program  
Manager

**Brenda Foster**, Administrative Director

**Teri Howe**, Director of Development and  
Communications

**Emily Innes**, Programs and Marketing  
Associate

**Loni Kirsch**, Administrative Assistant

**Alexandra Kruse**, Research Coordinator

**Jennifer Jurcisek**, Chief Financial Officer

**Jody Shy**, Director of Programs and Events

**Joan Young**, Founder

## PDSA BOARD MEMBERS

**Peter Pruitt**, Chairman, Coral Gables, FL

**Dale Paynter**, Vice Chair, Cambridge, ON,  
Canada

**Kim Everett**, Treasurer, Manitowoc, WI

**Beth Siegelbaum**, Secretary, Norwalk, CT

**Karen Avrick**, Deer Park, NY

**Robert Feiner**, Austin, TX

**Melissa Hilsabeck**, Huntington Beach, CA

**Linda McGuirl**, Point Pleasant, NJ

**Derek Zimmerman**, Royal Palm Beach, FL

The Platelet News is published quarterly by



A nonprofit organization dedicated to enhancing the lives of people with ITP and other platelet disorders through education, advocacy, research, and support.

**For more information contact:**

### PDSA

8751 Brecksville Road, Suite 150  
Cleveland, OH 44141

Phone: 440-746-9003

www.pdsa.org • pdsa@pdsa.org

PDSA is a 501(c)(3) organization.  
All contributions are tax deductible.

## HIGHLIGHTS



**At Every Turn, Faith, Hope, and Family Keep Brenda Shy Moving Forward**



**The ITP Community Makes a Splash for ITP Awareness Month 2022**



**PDSA advocates for the ITP patient community**



**PDSA Fundraisers Get Creative**

## IN THIS ISSUE

**3** From the President and CEO

**4** ICIS Meeting 2022

**6** ITP in Seniors

**8** At Every Turn, Faith, Hope, and Family Keep ITP Patient Brenda Shy Moving Forward

**12** Ask The Experts

**13** In Appreciation

**13** In Honor & In Memory

**14** Pruitt Award Recipients 2022

**16** ITP Awareness Month 2022

**18** Going #GLOBAL4ITP

**20** Advocacy In Action

**23** Resources Spotlight

**24** Fundraiser Spotlight

Thank you to sponsor



for supporting the PDSA  
website **www.PDSA.org**

*IMPORTANT: The Platelet Disorder Support Association does not provide medical advice or endorse any medication, vitamins or herbs. The information contained herein is not intended nor implied to be a substitute for professional medical advice and is provided for educational purposes only. Always seek the advice of your physician or other qualified healthcare provider before starting any new treatment, discontinuing an existing treatment and to discuss any questions you may have regarding your unique medical condition.*



## FROM THE PRESIDENT AND CEO



Now that the world is slowly but surely recovering from the isolation of the pandemic, most organizations have returned to in-person meetings and events, which has made for a very busy summer and fall for the PDSA staff. Since July, I have been to Seattle, Chicago, and Washington, DC, and traveled to six Canadian cities. As I have an immune deficiency and have received treatments that could

lower my body's resistance to infection, I remain diligent in taking precautions to reduce my exposure to contracting COVID-19, and I'm happy to say, so far, so good!

I have been very proactive in educating myself on available prophylactic treatments and antivirals should I get COVID-19. I also have actively participated in several medical and scientific studies to both better my understanding of my own health condition and contribute to the understanding of science that might help others.

In April 2021, I learned of a new study at the NIH (National Institutes of Health) Clinical Center that would examine the immune response of people with immune deficiencies or dysregulations to the COVID-19 vaccines. I jumped at the chance to be involved in the study. As a bonus, the study coordinators were looking for healthy controls, so I volunteered my husband, Ken, to also participate!

Within a month of getting each COVID-19 vaccine, we had to submit blood sections and answer a questionnaire. One of the benefits of the study was that Ken and I were able to send saliva samples to test for COVID-19 infection every other week throughout the entire 12-month study. This really gave us peace of mind, especially after traveling or being around people who later informed us they had been exposed or tested positive for the virus.

Through my participation in the study, I was pleased to find out that I was able to mount a response to the vaccine (some immune deficient patients were unable to produce antibodies), and in January 2022, I had antibodies to nucleocapsid, which identifies individuals who have had a recent or prior COVID-19 infection. Since I don't recall being ill nor had I knowingly been exposed to anyone who had COVID-19, I will surmise that the antibodies are from my monthly IVIG treatments – which now have recorded high levels of SARS-CoV-2 antibodies from donors who have had the virus.

Ken and I also enrolled in a study led by researcher Joud Hajjar, MD, MS, Chief of Adult Allergy and Immunology at Baylor College of Medicine. Dr. Hajjar's current research is focused on the interplay of the microbiome and immune dysregulation, which could possibly drive inflammation which can cause immune-compromised individuals to feel fatigued.

We have enjoyed our participation in these studies and hope that our involvement will make a difference for others. Uncovering more information surrounding these unanswered questions can help researchers and health care providers ensure that patients with rare diseases like ITP and primary immune conditions get the safest and most effective care. There are numerous ITP clinical trials, research studies, and even opportunities for ITP patients to share their stories with pharmaceutical companies and researchers to establish a better understanding of the true burden of disease and unmet needs important to patients. PDSA lets our members know about these opportunities on a regular basis. If you would like to join our patient speaker's bureau and share your story, please contact us at [pdsa@pdsa.org](mailto:pdsa@pdsa.org). I hope you will also consider participating in and contributing to our ITP Natural History Study Patient Registry for which we are still enrolling patients to complete the COVID-19 survey. By sharing your personal experience, you will contribute greatly to this important study and make a difference for the ITP patient community.

And while on the topic of stepping forward to make a difference in the lives of others, I want to personally congratulate and acknowledge the selfless efforts of PDSA's Board Chair Peter Pruitt. In addition to his tireless dedication, time, and energy to PDSA, Peter was honored this summer with the **Alvah H. Chapman, Jr. and Betty B. Chapman Humanitarian Award** in recognition of his outstanding leadership in helping homeless men, women, and children in Miami-Dade County, Peter's hometown. Since 2011, Peter has been a board member and immediate past board chair of the Chapman Partnership, an innovative and leading homeless assistance organization. We join the Chapman Partnership in honoring Peter's selfless support. We are fortunate to have his involvement and leadership.

Caroline Kruse  
President and CEO



*Back to in-person meetings! Caroline with an ITP nurse from New Orleans at THSNA Summit 2022.*

# Intercontinental Cooperative ITP Study Group (ICIS)

## 7th Expert Meeting on ITP By James Bussel, MD



**The Intercontinental Cooperative ITP Study Group (ICIS) meeting, held September 22-24, 2022 in Lenzerheide, Switzerland, was very interesting. It was focused on refractory ITP (rITP) from a number of points of view. Given that the attendees were among the world leaders in work on ITP, it was a thought-provoking meeting. It ranged from definitions of rITP in children and adults, to biomarkers and laboratory investigation, to treatment studies, quality of life, and finally the future with novel approaches. There were many intriguing presentations, but for space considerations, here are a few highlights:**

Two of the topics of presentation involved the definition of rITP in children and adults. Previously, the 2010 *Blood* consensus article by Rodeghiero et al. had defined rITP as a lack of response to a sustainable treatment, very low platelet count, ongoing bleeding and/or high risk of serious bleeding, and failure of splenectomy. This was increasingly felt, both before and at the ICIS meeting, not to be a satisfactory definition primarily because of the inclusion of failed splenectomy but also the feeling that it should have a more biologic basis. Splenectomy is rarely performed in children and very infrequently performed in many centers in adults. The latter is a result of the expanding number of available highly effective treatments such as rituximab, the three TPO agents (avatrombopag, eltrombopag, romiplostim), and fostamatinib. However, in addition to the relatively well-defined concern of overwhelming post-splenectomy sepsis, thrombosis post-splenectomy has more recently come into focus as an important risk over time including stroke. A very important part of the discussion upon which consensus was rapidly reached was the realization that: a) the previous definition was not a useful and appropriate one, and b) that applying newer creative approaches could lead to a more up-to-date, functional definition. A newer definition was not agreed upon although case examples clarified the goal of “identifying rITP” earlier and also how confusing it could be.

PDSA Medical Advisor Howard Liebman presented a series of three cases in which ITP had been misdiagnosed for years and the patient labeled refractory when actually they had another diagnosis, e.g., myelodysplastic syndromes (MDS) and inherited thrombocytopenia. This presentation emphasized the role of misdiagnosis in rITP and reminded the participants that knowledge of classical hematology

may be lacking in many practitioners, e.g., ones who specialized in malignant hematology or solid tumors. Other presentations looked, for example, at anti-nuclear antibodies (ANA) and other “clinical laboratory” tests and their perhaps limited importance in rITP although potentially very useful purpose in pointing out secondary causes of thrombocytopenia.

The scientific highlights of the meeting were presentations by Drs. James Zehnder, Matthieu Mahévas, and Nichola Cooper. Jim Zehnder started by briefly reviewing his past work on the initiation of chronic ITP by oxidative stress creating platelet membrane damage and inducing immune reactions to it. He then discussed the role of clonality in ITP and explained that T cell clonality could not be identified by flow cytometry (unless there was a specific antigen that could be identified on the clonal T cells that was distinct from other T cells). He explained how genomics and the use of screening panels had allowed identification of clones similar to those called “ChIP.” He explained that these were now identified in an increasing number of patients with a variety of diseases but definitely appeared to play a role in refractory ITP.

PDSA Medical Advisor Nichola Cooper talked about her work exploring cytotoxic T cells in the pathogenesis of refractory ITP. She and her team identified CD8 cytotoxic clonal T cells in patients with refractory ITP. These cytotoxic T cells appear to cause the thrombocytopenia. Her work will be published soon in the medical journal *Blood* and suggests that these patients may need therapy particularly targeted to this T cell population. This is important because most treatment used in ITP patients is directed against

the antiplatelet antibodies that drive the ITP in most cases. As with Matthieu Mahévas' work described briefly in the next paragraph, this is a major advancement in understanding and potentially treating rITP.

Matthieu Mahévas presented his work on B cells and plasma cells and BAFF (B cell activating factor) looking both in the peripheral blood and the spleen. He hypothesized that the use of rituximab alone would eliminate B cells but lead to an increase in the levels of BAFF, thus stimulating B cell progenitors and possibly plasma blasts. He presented studies of the spleen from rituximab refractory patients to illustrate the germinal center overgrowth (B cell area) in ITP patients. He then presented the concept that these effects of rituximab counterbalanced the therapeutic effects and in turn provided preliminary data on the use of anti-BAFF in combination with rituximab as a way to maintain the positive effects of rituximab and neutralize the "negative" ones. In a pilot study of 15 patients, remarkably good results were demonstrated at six months with 13 of 15 patients in an unmaintained response at that time. Dr. Mahévas and the French Hematology group is launching a randomized controlled trial comparing the combination to rituximab alone. This could be a major advancement on the use of rituximab alone and explain not only a percent of "refractory" patients but also how to manage them.

I presented briefly new work on fostamatinib in ITP using the Rigel patient assistance program. In a good number of patients, the data suggested that in real-world usage the results with fostamatinib may be better than those reported in the FIT1 and FIT2 studies (the fostamatinib in itp (fit) program was designed to evaluate short- and long-term treatment effects.) The approach in terms of patient support was very encouraging in making it easier for patients to receive and monitor their ITP while on fostamatinib.

In summary, this was an excellent meeting in which a great variety of interesting material on refractory ITP was presented and accompanied by considerable discussion. The great majority of the content presented at the meeting will appear in 14 chapters on different areas that were discussed. The group agreed that a special edition of the *British Journal of Haematology* would work well, and the journal committed to publishing the meeting output. The hope is to have this come out by the end of the second quarter of 2023.

*Dr. James Bussel is Professor Emeritus of Pediatrics, Medicine, and Obstetrics at the Weill Medical College of Cornell University in New York City and a PDSA Medical Advisor. ■*



**Platelet Disorder Support Association**  
Empowering ITP patients

**25 YEARS**

**SAVE THE DATE!**

**ITP CONFERENCE 2023**

**The premier patient event**

**July 21 - July 23 • Chicago, IL**

**Join us as we celebrate 25 years of raising awareness, advancing patient-centered research, and creating better outcomes for people with ITP.**



# ITP in Seniors

By Lib Elder

**A popular presentation featured at ITP Conference 2022 focused on immune thrombocytopenia (ITP) in seniors. The talk was given by PDSA Medical Advisor Craig Kessler, MD, a hematologist at the Lombardi Comprehensive Cancer Center at the Georgetown University Medical Center, in Washington, DC. ITP patient Lib Elder, serving as a volunteer conference session correspondent, offers the following summary of Dr. Kessler's presentation.**

Dr. Kessler's talk was of particular interest to me because not only was I born an Elder, but I am also 65 now. And I wish I could say his presentation was "all good news" for those of us ITPers in the senior age bracket, but that wouldn't be very accurate. In truth, as we age our risk of complications from ITP grows. That's not to say we are without hope, it just means we need to exercise greater vigilance and make certain our doctors are aware of the different concerns we may have versus a more youthful ITP patient. For this talk, Dr. Kessler defined "elderly" as over age 50 but admitted the literature is more precise for those over 60.

It comes as no surprise that aging brings an increased risk of stroke, falls, and a variety of challenges to our health. In the ITP patient, these risks are greater. Clearly, someone with plenty of platelets can still be injured in a fall but for someone with ITP that risk is exponentially greater when experiencing low platelet counts. Injuries to the head are more likely to result in bleeding, and bleeds are more likely to be more serious. If an ITP patient has been treated with steroids in the past, they may have an even greater risk of broken bones than the general elderly population overall, since there are unique risk factors among those who have used corticosteroids.

Another unique risk among seniors noted by Dr. Kessler is the increased likelihood of secondary ITP compared to primary ITP found more frequently in younger patients. Secondary ITP is when your low platelet count is due to a separate 'non-ITP' issue such as cancer, connective tissue disease, or other autoimmune conditions. Bleeding risks in folks our age are also higher compared to younger patients with ITP, especially when platelet counts fall below 20,000. And, as if that wasn't enough, infection risks are greater too, in addition to the risk of developing hematologic malignancies, gastrointestinal cancers, and liver cancers.

Dr. Kessler suggested that seniors with ITP should, in general, aim to maintain a platelet count of 30,000-60,000 if they don't have other health conditions that increase their risk of bleeding, and 30,000 to 80,000 if they do. Increased risk of bleeding could be based on previous bleeding related to ITP, high blood pressure, kidney issues, and use of aspirin. He reminds us of the importance of communicating with our doctor to make sure we understand which treatments to use or avoid as we manage our ITP.

Steroids, long the treatment of choice for many hematologists in managing patients with ITP, seemingly shift from "tried and true" to "inappropriate" over the course of just a few birthdays. Indeed, this

entire class of drugs, which may serve the younger ITP population well, now comes with risk and is no longer considered a good option for senior ITP patients. It's difficult to quantify what this increased risk looks like, but a good analogy is to think about your risk of being struck by lightning while walking on a golf course in a thunderstorm; it's roughly one in a million – unless you're carrying a set of golf clubs, at which point it increases to 50 in a million. It doesn't change from unlikely to likely, but the odds are significantly greater. So, if you can drop the clubs, or avoid the treatments that increase those risks altogether, it just makes sense to do so. And of course, the most effective way to avoid those risky treatments is to proactively manage your ITP effectively and hopefully avoid the crashes that can necessitate their use.

Dr. Kessler points out that the newer TPO agonists are a great way to manage ITP in the elderly. Rituximab also has shown good results in lowering bleeding risks in seniors with ITP. If one doesn't work, you may have to try another because, just as with other forms of treatment, there is no way yet to reliably predict which therapy might work best for you. Keep in mind that splenectomy, the surgical removal of the spleen, should be the last choice for the elderly or used only to get the platelet count up quickly in an emergency.

Dr. Kessler finished up his talk by encouraging seniors with ITP to consider COVID-19 vaccinations and boosters as the risks of contracting the SARS-CoV-2 virus are more serious in the elderly. He also reviewed vaccine safety and efficacy in seniors in reducing the viral impact and risk of death associated with COVID-19.

ITP can be scary for anyone, at any age. Knowing there are additional risk factors for seniors with ITP means that we elders need to ensure we have a knowledgeable physician and remain aware of the additional risks we have so we can do our best to avoid them if possible.



*Lib Elder, an ITP warrior, longstanding PDSA member, and volunteer extraordinaire, joined us in Seattle to offer her patient perspective and summary of several general sessions held during ITP Conference 2022. ■*



Designed to help alleviate the stress, pain, and anxiety of frequent needle pokes, the POKE-R Club for Kids program is an exclusive benefit for PDSA Members in the U.S. and Canada. When children join the POKE-R Club, they receive a kit that includes: a Buzzy® distraction device to help relieve pain at the injection site, distraction cards, and poker chips and scorecard to keep track of their "pokes" and win fun PDSA prizes.

**Meet a few of our POKE-R Club members and learn how the ITP POKE-R Club helps empower kids with ITP:**

## MEET SEPPI

*Age: 7 | POKE-R Club Member since 2019*



We found a great community in the PDSA! Seppi joined the POKE-R Club, and Buzzy (and his constant sidekick, Wilbur) has been his faithful companion to all lab visits. It has been a true godsend! **Before Buzzy, we'd have to hold him down for labs but now he sits proudly by himself.** We're happy to say that exactly one year after the start of one of the scariest years we've had with his ITP, and six months following the completion of his Rituxan infusions, Seppi's ITP is officially back in remission! This journey has robbed him of so much of his childhood and he's oftentimes had to grow up too fast. We're excited to watch him be able to be a kid again!

## MEET ANNABELLE

*Age: 11 | POKE-R Club Member since 2021*



**Buzzy bee does make it easier for me,** even if it is just waiting for me in the car for when I am done with my shots. I do think that kids should continue to use Buzzy because I am sure they will all love it. Once I even let my little brother use it when he was getting a needle poke, and it helped him! **Although ITP is not easy, Buzzy makes it seem like it is.** And yes, I do use Buzzy for other shots sometimes. I don't always use Buzzy because I have gotten used to the poke, but when I do, it sure helps!

## MEET LOGAN

*Age: 17 | POKE-R Club Member since 2014\**



**Buzzy changed my life.** I got one when I was about 10 and I joined the POKE-R Club. I'm 17 now and I still use it and recommend it to peers in my medical classes. **The POKE-R Club helped me stay positive and look forward to rewards instead of dwelling on pokes when I was having to get frequent blood draws.** I'm super grateful that PDSA offered the POKE-R Club for kids like me.

*\*Logan was one of the ITP POKE-R Club's first members!*



# At Every Turn, Faith, Hope, and Family

## Keep ITP Patient Brenda Shy Moving Forward

By Teri Howe

On the heels of an annual wellness exam in Spring 1999, Brenda Shy vaguely remembers her primary care doctor reporting that her platelets were low and suggesting a follow-up appointment to have her bloodwork rechecked. Not understanding what that might have meant, she filed it in the back of her mind and then unintentionally forgot about it. It was a busy time in the Shy household. With ten-year-old twin boys and a three-year-old daughter, Brenda and her husband, Keith, had a lot on their plate. In addition to the typical hustle and bustle of young family life, Brenda had a demanding federal civil service job and was working on attaining her Master of Business Administration (MBA) degree, Keith was getting a new business off the ground, and their daughter was suddenly diagnosed with type 1 diabetes. It's fair to say that the last thing Brenda was thinking about was her health.



*ITP Conference 2022 was a family event for Brenda. (L-R) Sister Barbara, daughter Candace, Brenda, and sister Gwen were all smiles spending the weekend in Seattle together.*



## ITP WARRIOR: COVER STORY

But that all changed shortly after Thanksgiving that same year. Just two days after hosting her family for the big holiday dinner, Brenda woke on a Saturday morning to find strange red marks on her face, arms, and body. Perhaps it was an allergic reaction or a sign of stress? Either way, she was concerned enough to head to a nearby urgent care center. There, the attending physician conducted an exam and ordered bloodwork. It wasn't long before the doctor would return to the room with a surprising report, "You have no platelets and you need to be transferred to the hospital immediately. I've called for an ambulance to take you." Shocked and confused by the doctor's orders, Brenda froze as she tried to understand his concern. Surely, she didn't need an ambulance. "Not really knowing what I was dealing with, I told him I could drive or call my husband to get me. I wasn't feeling bad. He then said he couldn't let me, or my husband, drive me to the hospital because if I got in an accident on the way and got cut or injured, I could bleed to death. And then I realized I was dealing with something very serious."

At the hospital, Brenda was formally diagnosed with immune thrombocytopenia (ITP). She had little time to process the unexpected turn of events and was admitted for treatment immediately. First came the intravenous immune therapy WinRho®, followed by several infusions of platelets – neither of which did much to sustain an increased platelet count. Next up was prednisone, which did the trick. Brenda responded positively to a course of the corticosteroid and, after a week of being hospitalized, was finally released to go home. She remained on prednisone for the following six months.



*Brenda getting a Rituxan treatment, smiling through it all.*

As many ITP warriors know, the positive side effects of prednisone use come with a cost, and Brenda struggled to navigate the ups and downs of her ITP along with the ongoing effects of the steroid. Prednisone remained her hematologist's 'go to' medication to elevate her platelet count as needed for close to 15 years. The steroids wreaked havoc with her sleep and mood, and the telltale 'moon face' and not-so-common, yet possible, side effect of hair loss added insult to injury. "The effects of the prednisone, to me, were worse than the ITP itself. I didn't look like myself and I certainly didn't feel like myself. But the prednisone did sustain me for quite a while." Brenda continued to work, pursue her MBA, and be the best mother she could while coping with the ongoing concern about her platelet counts and overwhelming fatigue. Looking back, she remembers how she got through it all, "I would just take one day at a time. Do what I could when I could, and rest when I needed to rest."

Brenda also found great comfort in her connection to PDSA. She took advantage of our many resources to stay informed and up to date on all things ITP, regularly browsing our website,



*Surrounded by her family, Brenda has an amazing support system.*



*Brenda graduating with her MBA.*



*Brenda jumped right into ITP Conference 2017!*



*Brenda is committed to living life and trying to have fun. Here, she and husband, Keith, do it in style as they celebrate their 35th wedding anniversary.*

downloading our educational booklets, and reading *The Platelet News* cover to cover. "I learned a lot through the PDSA. It was helpful to know that I could turn to them to find answers to my questions."

In 2014, just after Brenda was admitted into the hospital once again for a very low platelet count, her physician attended a medical conference where he had the opportunity to consult with an ITP specialist about her case. The specialist recommended Brenda consider Rituxan® (or rituximab), a monoclonal antibody administered intravenously as immunotherapy. She was pleased to hear of an alternate treatment and was excited at the prospect of trying something different. "But then I saw the black box warning regarding possible fatal infusion reactions – and I thought 'wait a minute. I need to be better informed on this.' I looked further

into it and was educated on how the procedure would take place and all the precautions that would be taken to prevent an adverse event. At that point, I felt comfortable with it and gave my consent to start the weekly infusions." Her first course of Rituxan proved a successful treatment, providing Brenda with a welcomed two-year remission before her platelets plummeted again in 2016. Thankfully, the delivery of another round of Rituxan would effectively bring her platelets back to a safe level for several more years.

When ITP Conference 2017 was hosted in Chandler, Arizona, Brenda was determined to make it there. She had hoped to attend this special patient event for years and, as she is quick to share, it was a life-changing experience. "I wasn't really sure what to expect, but when I got there, I immediately felt like I was in the right place, with others who knew exactly what I was going through. We spoke the same language. It was so comforting." Brenda returned home inspired by the connections she made and the information she gained over the 3-day conference. Things were looking up!

But another startling turn was on the horizon for Brenda. In May 2018, she was diagnosed with breast cancer. Suddenly, her hematologist who had treated her ITP for so many years was now her oncologist as well. Brenda recalls the surreal nature of her cancer diagnosis: "It's almost as if the ITP prepared me for what was coming. I knew the ropes of ongoing testing and treatment. I was already familiar with the building and the labs and the people who worked there." With her deep faith in God and her husband and family always by her side, Brenda faced her breast cancer head-on and weathered the storm of treatment, including surgery, chemotherapy, and radiation. Fortunately, while receiving treatment for cancer, her platelets remained at a good level, until November 2019. Then, another round of Rituxan increased her counts for almost two more years. But with a waning response to this treatment, her doctor added a pulse dose of Dexamethasone, a long-acting steroid, to boost her results. While this seemed to work for a few months, the efficacy of the Dexamethasone was short-lived, and a second dosage had a quickly diminishing response.

Fortunately, Brenda had the opportunity to attend ITP Conference 2022 this past July in Seattle, Washington – this time, with her daughter and two sisters. She appreciated the chance to participate in sessions led by ITP specialists and to learn about new research and available treatments, and to get answers to her questions, stating, "This conference has helped me figure some things out, things to talk with my doctor about." Brenda also was honored to have the opportunity to share her personal ITP journey with the audience during a special Patient Panel presentation, understanding how her story might give others hope and something to hold on to. There, she eloquently detailed her 22-year roller coaster ride with ITP and revealed how she has been able to remain strong in the face of her chronic illness: "What has helped me is my faith in God. It's kept me grounded in dealing with my chronic illnesses. Also, my husband and my family; they've been there to support me in every possible way, and I am grateful to have them by my side. My hematologist, who became my oncologist, has also been there for me and has tried to stay on top of the latest treatment strategies and I know he has the best interest in mind – even when I go in for an appointment and he ends up having to roll me right up to the hospital to stay at a



moment's notice because my platelets are so low! And the PDSA has provided me with the information and support I need to continue to fight and advocate for the best possible outcome when dealing with ITP."

With more than two decades of doctor appointments, blood draws, hospitalizations, treatments, side effects, fatigue, and continuing unknowns, Brenda is steadfast in the face of ITP, having learned to keep its impact in perspective and doing her best to remain positive. "I continue to put my trust in God and thank Him every day for the grace and sufficiency to make it one day at a time. I live my life joyfully and enjoy each day that is given to me. I don't let it stop me from doing the things that I want to do." She lives by the motto, 'I have ITP, but it doesn't have me,' and proves this point beautifully by sharing, "Since being diagnosed with ITP, I've graduated with my MBA and watched my children graduate from college. I've traveled throughout the United States. I retired after 35 years of federal civil service as a manager. I celebrated 35 years of marriage. I stepped into year 60 *like a boss*. And I am determined to keep fighting – because *I am an ITP warrior*."

Today, Brenda finds herself at a crossroads for the next potential treatment of her ITP and is working closely with her doctor to identify what she should try next. Not surprisingly, this crossroads doesn't frighten Brenda. She sees it as an opportunity and remains confident that they will find something that will work. And, as she has done at every turn, she moves forward with hope and in faith, with her family by her side. ■



*An ITP warrior through and through.*



*On stage at ITP Conference 2022, Brenda participated in the Patient Panel to share her ITP journey.*

## MEDICAL ADVISORS

**Donald Arnold, MDCM, MSc, FRCPC**

McMaster University  
Hamilton, Ontario, Canada

**James Bussel, MD**

Weill Medical College, Cornell  
University  
New York, NY

**Douglas Cines, MD**

University of Pennsylvania  
Hospital  
Philadelphia, PA

**Nichola Cooper, MD**

Hammersmith Hospital, Imperial  
College  
London, United Kingdom

**Terry Gernsheimer, MD**

University of Washington  
Seattle, WA

**Rachael Grace, MD, MMSc**

Boston Children's Hospital  
Boston, MA

**Craig Kessler, MD**

Georgetown University Hospital  
Washington, DC

**David Kuter, MD, DPhil**

Massachusetts General Hospital  
Boston, MA

**Michele Lambert, MD, MSTR**

Children's Hospital of  
Philadelphia  
Philadelphia, PA

**Howard Liebman, MD**

Keck School of Medicine,  
University of Southern  
California  
Los Angeles, CA

**Diane Nugent, MD**

Children's Hospital of North  
Orange County  
Orange, CA

**John Semple, PhD**

Lund University  
Lund, Sweden

**Michael Tarantino, MD**

The Bleeding and Clotting  
Disorders Institute  
Peoria, IL

## EMERITUS

**Robert McMillan, MD**

The Scripps Research Institute  
La Jolla, CA

## ASK THE EXPERTS

**This regular *Platelet News* feature includes patient questions about various topics important to the ITP and other platelet disorders community. If you have a question that you would like answered by one of PDSA's medical advisors or other experts, please email your question to: [pdsa@pdsa.org](mailto:pdsa@pdsa.org) and enter "Ask the Experts" in the subject line.**

On the second day of ITP Conference 2022, the PDSA medical advisors gathered to answer questions about immune thrombocytopenia (ITP) in adults. The panel comprised of Howard Liebman, MD; Diane Nugent, MD; John Semple, PhD; Craig Kessler, MD; David Kuter, MD, DPhil; Terry Gernsheimer, MD; Ilene Weitz, MD, and James Bussel, MD.

**Q: What is considered remission? If it's having normal levels for a certain amount of time (like 6 months or a year), does it have to be without any medication in order for it to count as remission?**

**A:** Dr. Kuter began by stating he doesn't frequently use the term "remission" in ITP. In diseases like cancer, remission is defined by the absence of disease activity. However, if ITP patients are in "remission," this does not mean the condition is cured. Therefore, he believes it is more appropriate to say that an individual has an adequate (otherwise known as a hemostatic) platelet count either on or off therapy. Dr. Kessler agreed that he would rather discuss the term "hematologic remission" with his patients, which he considers to be a drug-free platelet count greater than 100,000/ $\mu$ l. Dr. Weitz also discussed that perhaps ITP should not be classified using remission status, but rather how well a patient responds to treatment. To conclude this question, Dr. Kuter suggested that rather than focusing on the numbers, patients and physicians should focus on whether an individual has a safe platelet count and can maintain their lifestyle off therapy.

**Q: I'm at the beginning stages of ITP and diagnosis. Are a larger number of immature platelets observed? And when people's platelet counts fall, is it generally a slow or a fast decline?**

**A:** Dr. Nugent opened with an example using IVIG. When patients first present with bleeding symptoms – petechiae, bruises, blisters in the mouth – and are given IVIG, doctors can observe their platelet counts improving in a matter of days. After 3 or 4 weeks, the effects of the IVIG may wear off and the platelet counts may drop again. However, this time the bleeding symptoms are not present. This occurs because the bone marrow, where platelets are produced, has had a chance to catch up and create younger, larger, and more effective platelets. Likewise, when patients are given steroids, one immediate effect is that platelets are mobilized from the spleen quickly back into the blood. Dr. Kuter discussed an alternative reason why bleeding symptoms disappear without the rise in platelet count. Our blood vessels are lined with closely sealed endothelial cells, but the space between the endothelial cells loosen when an individual is thrombocytopenic. Steroids work to reseal these endothelial cells to stop bleeding events before platelet counts have a chance to rise.



## IN APPRECIATION



At PSDA, we rely on the philanthropic support of our members and donors to help us fulfill our important mission. Together, we continue to shape the future for those living with ITP.

Those who donate or raise \$1,000 or more within a calendar year for PSDA are recognized as **Circle of Hope** donors – and their leadership support advances our efforts exponentially.

**We are pleased to recognize the following Circle of Hope donors whose contributions were received July 1 – September 30, 2022.**

Anonymous (2)  
Cathy and Raul Aldama  
Rob Ammerman  
Chris Angelo  
Alexis Ashby  
Cindy and J. Philip Ayliff  
Gerald Barnes  
Gigi Barry  
Mary and James Benvenuto  
Amita and Ranjive Bhalla  
Esther and Paul Brandt  
Barbara and David Bugen  
The Caine Family Foundation  
Brendan Cameron  
Christie Catalano  
Frances Collins  
Charlotte Cunningham-Rundles,  
MD and James Bussel, MD  
Meghan and Annabelle Cureton  
Druanne Davies  
William Deaver  
Jennifer and Luigi DiRaimo  
Cindy and Richard Dummermuth  
Derek Dummermuth  
Rachel and Patrick English  
Kim and Jim Everett

Robert Feiner  
Marcia and Jim Freed  
Mary-Helen Galleno  
Jan and Steve Gardner  
Terry Gernsheimer, MD  
Goldman Giving Fund  
Jen and Benjamin Grimes  
Kim and Rodney Hall  
Stephanie Halperin  
Deborah and H. William Harlan  
Kristin Henrikson and Jon Brandt  
Noelle and Mat Heyman  
Arlene and David Horowitz  
Madeline and John Hromyak  
Sherrill Hudson  
Bart Hynes  
Stacey Iliopoulos  
Karen and David Imig  
Joanne Janssen  
Jerry Jones  
Joan and Richard Jordan  
Michael Jurs  
The Rachel and Drew Katz  
Foundation, Inc.  
Emily and Kris Kile  
Louise Kittel Mason

Caroline and Ken Kruse  
Irene Kuter, MD and  
David Kuter, MD, DPhil  
Diane and Arthur Levin  
Angie Levitt  
Jenn and Andy Lindal/For the  
Love of Gracie  
Josette and Donald Loeblein  
Edward Lopez  
Marcy Luffman  
Mary Lou Lyons  
Katharine McCleary  
The McGuirl Family  
Irma and Daniel Miles  
Stephanie and Mike Moran  
Deborah and William Mulligan  
Tracey Parker  
Joseph Ponte  
Jacqueline and Bruce Prescott  
Patricia and John Proescholdt  
Barbara and Peter Pruitt, Jr.  
Shirley and Peter Pruitt, Sr.  
Patricia Pulley  
Ashley and Greg Randolph  
Virginia Rennie  
Toni Roberge

Karen Rosenbaum and  
Ben McClinton  
Mark Ruelle  
Hady Saheb Medical Services  
Jordyn Sak  
Kellie Sak  
David Seidl  
Stanley Shashoua  
Jody and Jon Shy  
Julie and Michael Spieker  
Dana and Thomas Stotz  
Kathy and Ross Tanner  
Caroline and Roger Todd  
Kristen and Steve Tomczak  
Jessica and Cody VandeVelde  
Bert Vongpaisal  
Theresa and Tony Waxlax  
Ilene Weitz, MD and  
Howard Liebman, MD  
Donna and Kent Winter  
Susan and Lewis Wirshba  
Cheri and Derek Zimmerman  
Joyce and Dale Zimmerman

### Want to join in the Circle of Hope?

Contact President and CEO Caroline Kruse to learn how your generosity can help sustain our lifesaving work.  
[ckruse@psda.org](mailto:ckruse@psda.org) 440-746-9003

## IN HONOR

**We received contributions from July 1 to September 30, 2022 in Honor of:**

Alexia Engels  
Marcia Freed  
Mary-Helen Galleno  
Kai Henrikson-Brandt  
Almabella Hoff  
Dave Segebart  
Ryan Shoemaker  
Peyton VandeVelde

## IN MEMORY

**We received contributions from July 1 to September 30, 2022 in Memory of:**

Barry J. Bresbris  
Luca DiRaimo  
Michelle Eady  
Ruth Gubrud  
Hazel  
Halle Lentini  
Grace Lindal  
Melvin Ray Ogden  
Margherita Rullo  
Devin Winter  
Sheryl Lynn Womack

**If you are considering a tribute donation, please contact PSDA, 8751 Brecksville Road, Ste. 150, Cleveland, OH 44141 or [psda@psda.org](mailto:psda@psda.org) or call 440-746-9003.**



# Meet PDSA's 2022

## Barbara and Peter T. Pruitt Jr. Research Award Recipients

**PDSA encourages and funds patient-centered ITP-related research on an annual basis to support projects that have the potential to improve an ITP patient's quality of life by further understanding the pathogenesis and management of primary ITP to support the development of new therapies and a cure.**



**Megan C. Brown, MD, MSc**

*"Heavy Menstrual Bleeding and its Effect on Quality of Life in Adolescent Girls with ITP"*

Dr. Brown is the Director of Young Women's Bleeding Clinic and Assistant Professor of Pediatrics in the Division of Hematology, Oncology and Bone Marrow Therapy at Emory University and the Children's Healthcare of Atlanta. Her project is titled "Heavy Menstrual Bleeding and its Effect

on Quality of Life in Adolescent Girls with ITP." The purpose of the study is to determine the frequency of heavy menstrual bleeding in girls with ITP and to determine what effect menstrual bleeding has on quality of life.

The symptom of heavy menstrual bleeding has not gained a lot of attention within the medical community. For providers caring for individuals with ITP, heavy menstrual bleeding often leads to treatment and even at times, hospitalization. Dr. Brown and her team strive for their study to be the first to describe the menstrual cycles of adolescents with ITP, focusing on bleeding quantity, effect on quality of life, treatment strategies, and the correlation between lab values and these measures. Given that there is no data available to even describe this population with regard to menstrual

symptoms, this may be the best first step to characterize menstrual bleeding in ITP.

Through the use of a pictorial blood assessment chart, and two bleeding (menstrual) validated surveys, information on quantity of blood loss with each cycle, perceived blood loss, quality of life, and fatigue data will be collected. The study will also record data on medications used that affect menstruation (such as hormone therapies) as well as ITP-directed therapy (such as first- and second-line treatments). Laboratory workups (including platelet counts, complete blood counts, iron studies, etc.) will also be recorded.

Dr. Brown and her team propose that heavy menstrual bleeding is an underreported symptom for adolescent girls with ITP that likely leads to a reduced quality of life. Specific goals of the study include:

- 1) Quantifying the frequency of heavy menstrual bleeding in adolescents with ITP,
- 2) Evaluating if there is a relationship between menstrual blood loss and platelet count over time in adolescents with ITP,
- 3) Describing the effects of menstrual bleeding on quality of life and fatigue in adolescents with ITP.

This project has the potential to create awareness in the medical community of heavy menstrual bleeding in girls with ITP and may pave the way for preliminary data to inform a larger study that could determine optimal treatment strategies for this population.





### **Maria De Jesus, PhD**

*“Centering Youth Voices and Experiences: Improving the Quality of Life Among Adolescent Patients Living with Chronic Immune Thrombocytopenia (ITP)”*

Dr. De Jesus is an Associate Professor at American University in Washington, DC. Her project is titled “Centering Youth Voices and Experiences: Improving the Quality of Life Among Adolescent Patients Living with Chronic Immune Thrombocytopenia

(ITP).” The purpose of her project is to look at the health-related quality of life (QoL) among adolescents living with chronic ITP; specifically, how QoL is impacted by treatment decision-making and patient-centered outcomes, as well as the effects of treatments themselves. QoL data will also be stratified to look at potential differences in QoL reported based on age, gender, and treatment exposure.

Incorporating the patient perspective by using patient-centered outcomes (PCO) when studying adolescents with ITP is essential

since treatment decisions are complex and platelet count only partly explains the disease burden. A PCO is any report of a patient’s health condition that comes directly from the patient, without interpretation by a clinician or anyone else. Health-related quality of life includes assessing all life domains affected by ITP and treatment, including physical health (e.g., fatigue and pain), psychological health (e.g., self-esteem and symptom concerns), independence (work and daily living activities), and social relationships.

Centering the voices and experiences of adolescent patients living with chronic ITP in a rigorous study is key to garnering evidence that will fill an important gap in the pediatric hematology literature on how chronic ITP and the effects of treatment impact quality of life, and guide treatment decision-making. Dr. De Jesus and her team plan to create digital stories (a way to share patient stories online) in addition to highlighting the results from individual interviews to help put together a clearer picture of the adolescent experience with ITP. Results from reported experiences will also be combined with data from the validated Kids ITP Tool (KIT) quantitative survey, which measures how adolescents with ITP are impacted in a quantitative way, thereby generating a richer patient-centered understanding than simply using just a survey. ■

*Are you looking for*  
**GREAT  
HOLIDAY  
GIFTS?**



**Visit PDSA’s Platelet Store**  
for these terrific ITP awareness items  
and many more!

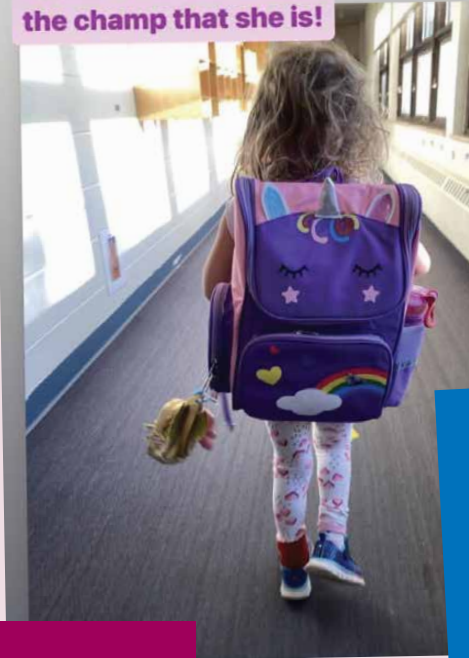
**Shop <https://bit.ly/2JgD37U>**



# The ITP Community Makes a Splash for ITP Awareness Month 2022

ITP Awareness Month 2022 celebrated advancements in the understanding of ITP and the strength of our community. And none of it would have been possible without YOU. All month long, ITP patients, caregivers, friends and family, and professional partners and advocates used their voices to raise awareness and empower ITP patients. We appreciate you joining PDSA in honoring the global network of people working together to improve outcomes for those living with ITP. Thank you for sharing your photos and thoughts on what you want people to know about ITP! #ITPKnowledgeIsPower

It is #purpleforplatelets day!!! My cutie is rocking her purple like the champ that she is!



"Advocate for yourself! After all, you know yourself best."

"There is more than meets the eye."



"ITP awareness goes beyond September!"



We "Sport Purple for Platelets". In honor of our precious son Devin Winter ❤️



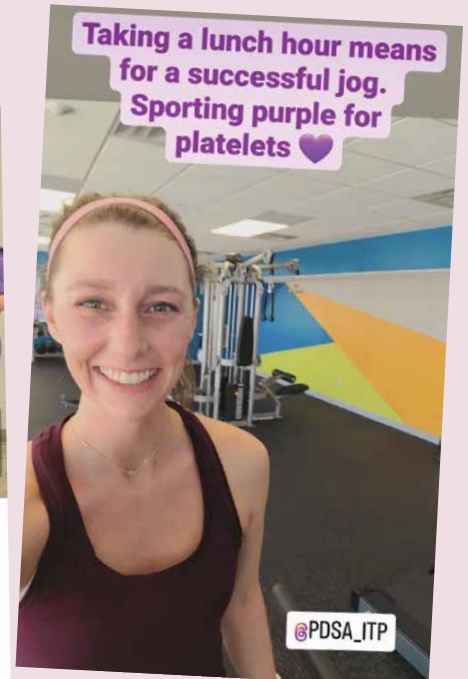
## ITP AWARENESS MONTH 2022



"[I] can work through the highs and lows."



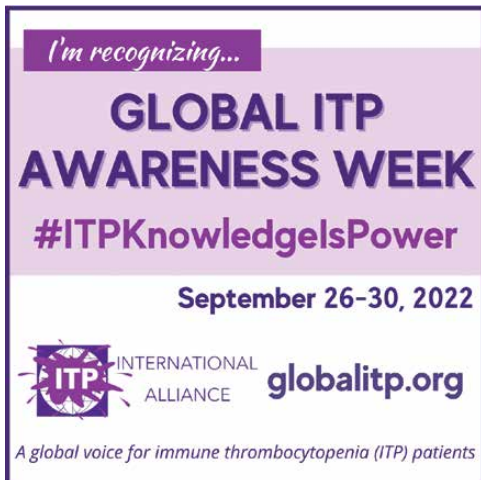
"The 'experts' on the disease are those of us who are living with it."



"Research and education [are] key."







Television journalist Erica Fontana reports on PTI Argentina and the #PTIElConocimientoEsPoder campaign.

Around the world, the ITP community celebrated Global ITP Awareness Week September 26-30. Members of the International ITP Alliance celebrated the strength of the ITP community and mobilized their organizations to raise awareness for ITP.



**Argentina** - Argentina conducted various radio and television interviews to spotlight the patient experience and hear from hematologists and collaborated with a television journalist to spread awareness across Argentina. They also joined PDSA in launching a #PTIElConocimientoEsPoder (#ITPKnowledgelsPower) campaign.

Melbourne was illuminated in purple during Global ITP Awareness Week.



**Australia** - Global ITP Awareness Week, over 90 buildings and structures across Australia were illuminated in purple to raise awareness for ITP, as part of ITP Australia's "Light Up for ITP" campaign.



**Brazil** - PTI Brasil launched the "I am more than purple" campaign and took to social media to spread information about ITP. To mark the end of ITP Awareness Month and Global ITP Awareness Week, PTI Brasil hosted a national patient meeting on a virtual platform.

*PTI Brasil founder and president, Marilia Lia, discusses ITP and female health with an obstetrician specializing in high-risk pregnancies.*



**AIPIT Onlus**

**Italy** - Associazione Italiana Porpora Immune Trombocitopenica (AIPIT) celebrated Global ITP Awareness Week and ITP Awareness Month by participating in "Mille Vele" regatta, sailing with Mauro Pelaschier, an Olympic gold medalist!

*Barbara Lovrencic, president of AIPIT, sailed in the "Mille Vele" regatta.*

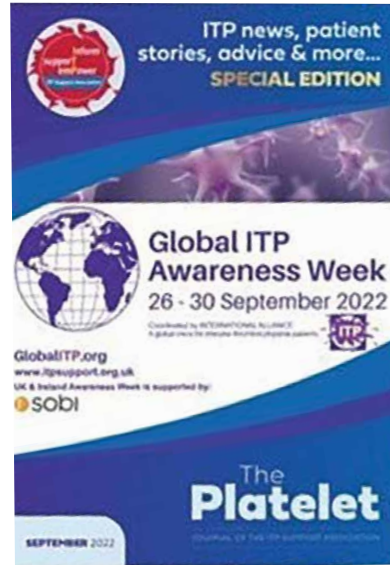


**Netherlands** - Recognizing the unique challenges living with ITP poses to teens and young adults, ITP Patiëntenvereniging Nederland hosted events to bring young ITP patients together, connect, and share their experiences. They also introduced a youth ambassador program to help raise awareness and support adolescents with ITP.



Patiëntenvereniging  
Nederland

ITP patients and hematologists met for an Advisory Council meeting to share their stories and discuss pressing issues to the ITP community, including fatigue and quality of life management.



**United Kingdom** - The ITP Support Association released a special edition of the *Platelet Journal*, featuring patient stories, Global ITP Awareness Week events, and reports on the work of the association.

The ITP Support Association released a special edition of the *Platelet Journal* to celebrate Global ITP Awareness Week.



## Pump It Up For Platelets!

Going the distance for the ITP community

### Start planning now for your 2023 event!

Walk/run events can take place **in-person or virtually**.

**That means you can Pump It Up For Platelets! YOUR way!**  
Gather with your friends and family at a local park, head to your favorite trail, or walk in your neighborhood and Facetime with teammates along the way.

Start your own event or join and support an existing walk/run to help support the ITP community!

**PDSA is here to help every step of the way!**

## Learn more at [ITPwalk.org](https://ITPwalk.org)

Questions? Contact PDSA Director of Programs and Events Jody Shy at [jshy@pdsa.org](mailto:jshy@pdsa.org) or (440) 746-9003.

Special thanks to  
our sponsors:



## Thrombosis and Hemostasis Societies of North America (THSNA)

THSNA held its 5th comprehensive scientific meeting dedicated to thrombosis and hemostasis issues in Chicago, IL, August 16-18. THSNA is a collaboration of the fourteen leading non-profit organizations in both fields. PDSA is one of those organizations and PDSA President and CEO Caroline Kruse serves as a member of the THSNA Board of Directors.

The Thrombosis and Hemostasis Summit of North America highlights basic science, clinical, and laboratory information that reflects the latest understanding of bleeding and clotting disorders, with the goal of improving patient care. THSNA 2022 hosted more than 800 healthcare professionals and coagulation scientists who are committed to the diagnosis, management, and improved understanding of thrombotic and bleeding disorders. A number of PDSA's medical advisors either attended or presented at THSNA, including Drs. Craig Kessler, David Kuter, Diane Nugent, and Michael Tarantino.



*PDSA volunteer Ken Kruse speaking with a pharmacist who has an interest in ITP at the PDSA education table.*



*Caroline Kruse with PDSA Medical Advisor Michael Tarantino, MD, who presented a PDSA sponsored Pre-Summit Workshop on "Immune Thrombocytopenia in Adults and Children."*



*Catherine Broome, MD, Cindy Neunert, MD, and PDSA Medical Advisor David Kuter, MD, DPhil, present the program "Immune Thrombocytopenia: Expert Guidance to Integrate Emerging Therapies into Clinical Practice."*

## Network for Rare Blood Disorders Organization (NRBDO)

PDSA's Research Program Manager, Jennifer DiRaimo, facilitated an educational webinar through NRBDO on September 22, 2022. NRBDO is a coalition of national patient groups in Canada, formed to share the best practices in health care delivery for people with rare blood disorders. The webinar focused on research programs for patient groups and featured the ITP Natural History Study Registry and PDSA's two grant opportunities in hopes to inspire other patient organizations in Canada to set up their own research programs. The webinar attracted leaders from various patient groups, some who are in the beginning stages of establishing their own patient-centered registry. Various organizations shared their experiences with forming and maintaining a patient-centered registry. It was a collaborative meeting where everyone learned from each other, all while increasing further awareness about ITP and other platelet disorders. Jennifer DiRaimo is a board member at NRBDO representing PDSA and helping to further advocate for the needs of ITP patients in Canada.



*PDSA Research Program Manager Jennifer DiRaimo (top row, middle) listens to colleagues discuss their research programs.*





*PDSA exhibited at NORD, providing educational resources to the rare disease community.*

including a keynote address from the US Food and Drug Administration (FDA) Commissioner who discussed the role of patient involvement and input in scientific discovery and the importance of collaboration between key stakeholders in rare disease care. The Summit's general sessions addressed the most pressing topics to the rare disease community, including global research initiatives, innovative treatments, and strategies for enhancing diversity, equity, and inclusion in research and advocacy efforts; breakout sessions allowed for more intimate discussions between rare community members, clinical experts, industry partners, and patient advocates.

PDSA was proud to present a poster abstract on our ITP Patient Connect Program. The poster, "Patient Community Building in Rare Diseases: The Invaluable Role of Technology as a Lifeline of Information, Support, and Community through the COVID-19 Pandemic," examined how PDSA pivoted to a virtual setting for our local support group meetings in response to the COVID-19 pandemic and consequently expanded our reach to serve more ITP patients and caregivers than ever before. PDSA President and CEO Caroline Kruse also presented during a breakout session titled "Why Data Matters and What You Can Do with It." Using PDSA's own ITP Natural History Study Registry as an example, she discussed the importance

## National Organization for Rare Disorders (NORD)

In late October, PDSA staff attended and exhibited at the Rare Diseases and Orphan Products Breakthrough Summit, hosted by NORD in Washington, DC. With nearly 800 individuals in attendance, the Summit facilitated critical discussions and networking opportunities for the rare disease community,

of the patient voice and patient-driven data to advancing rare disease research and the real-world impact it has on the lives of patients.

PDSA is proud to serve the ITP community as a member organization of NORD, and for the opportunity to meet and engage with other patient advocates across the rare disease spectrum at the 2022 Summit, working together to support and create better outcomes for our patient communities.



*Caroline Kruse and Emily Innes presenting their poster abstract on PDSA's ITP Patient Connect program at the Rare Diseases and Orphan Products Breakthrough Summit in Washington, DC.*



*PDSA President and CEO Caroline Kruse was a panelist during the breakout session, "Why Data Matters and What You Can Do with It."*



*Caroline spoke about the importance of patient-driven data to rare disease research alongside Alex Betourne, PhD, PharmD, RDCA-DAP Scientific Director, Critical Path Institute, and moderator Michelle Campbell, PhD, Senior Clinical Analyst for Stakeholder Engagements, CDER, US Food and Drug Administration (FDA).*

## Immunoglobulin National Society (IgNS)

PDSA was proud to share its mission and resources with attendees of the IgNS National Conference, held in National Harbor, MD, September 29 - October 1. The conference brought together nurses, physicians, pharmacists, and other health care partners in the immunoglobulin (Ig) and specialty biologics industry to learn more about advances in Ig research, treatment, and clinical practice. Ig therapies, such as IVIG, are often used as a first-line treatment option for ITP patients, and PDSA's exhibit booth provided access to patient-friendly resources for clinicians to take back to their patients and share with other health care professionals who treat ITP.

*Emily Innes, PDSA Programs & Marketing Associate, manned the PDSA exhibit booth and distributed educational materials to nurses, physicians, and pharmacists.*

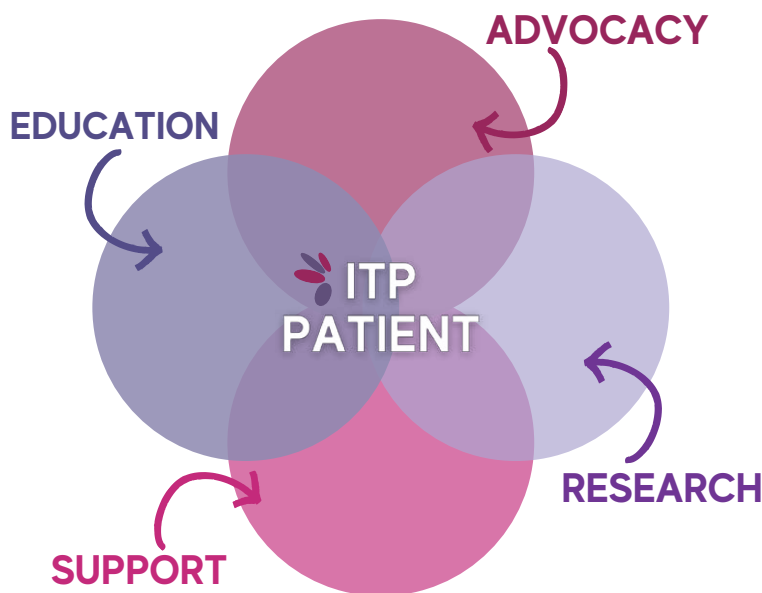
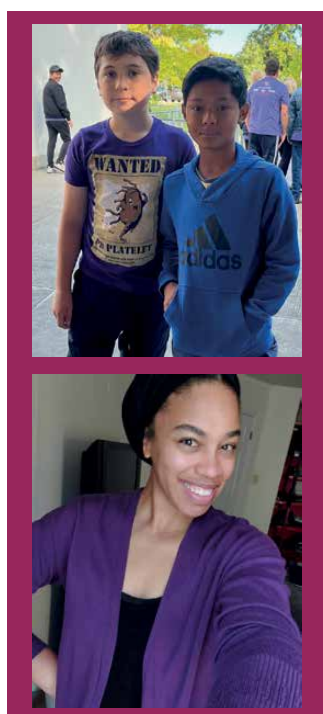




# Keeping ITP patients at the center of all we do.

PDSA exists for the ITP patient community. We hope we have been a supportive presence for you or for someone you love.

Our impact has grown exponentially thanks to the meaningful generosity of individuals like you. Together, we will continue to build PDSA's intentional and responsive programming and services to inform, support, and improve the lives of ITP patients.



By partnering with PDSA as a donor – YOU help fuel our patient-centered efforts and enable us to do **more** for the ITP patient community.

**Can we count on your continued generosity?**

Your renewed membership or outright gift is 100% tax deductible and 100% impactful.

**SUPPORT PDSA TODAY!**  
Visit [pdsa.org/give](https://pdsa.org/give)



# New Patient Resources

## Treatment Tables Now Available at PDSA.org

We are excited to introduce our new easy-to-use one-page treatment table handouts for patients and health care providers! This resource includes essential treatment-related details for current ITP therapies available to treat both children and adults in a single spot that can be downloaded easily! These hand-outs can also be used to compare various therapies side by side! The treatment tables were co-developed with PDSA Medical Advisors and the Pediatric ITP Consortium (ICON). For more information, visit [pdsa.org/treating-ityp](http://pdsa.org/treating-ityp). Treatment tables can be found under each listed therapy.

FOR CHILDREN	
MEDICATION NAME:	CORTICOSTEROID: PREDNISONE/PREDNISOLONE BRAND: DELTASONE, RAYOL, PREDNICOT, PREDNISONE INTENSIF, AND STERAPID DS.
HOW IS IT GIVEN:	Oral tablet or liquid. Administer after meals or with food or milk to decrease GI irritation.
HOW DOES IT WORK:	Reduces destruction of antibody-coated platelets, effective platelet production. May also reduce ITP effect on the blood vessel.
COMMON DOSING REGIMENS:	1 - 2 mg/kg/d divided daily for a maximum of 21 days twice daily for 4 days (maximum 60 mg twice daily for 1 - 3 days have been used in emergent situations).
COMMON SIDE EFFECTS:	Mood swings, anger, anxiety, insomnia and other gain, Cushingoid faces (puffy and rounder facial features), ulcers, high blood pressure, high blood including thinning and "senile purpura", hair loss.
RARE BUT SERIOUS SIDE EFFECTS:	With long-term use: osteoporosis (weakening of hip, pelvis, vertebrae, infections, adrenal insufficiency).
TYPICAL TIME TO RESPONSE:	Initial response 5-14 days. Peak response 7-21 days.
LIKELIHOOD OF INITIAL RESPONSE:	Approximately 75%. Higher response rate with other agents.
LIKELIHOOD OF LONG-TERM RESPONSE (3-5 YEARS):	Platelet counts generally fall as the dose is discontinued. No evidence that long-term response is affected.
OTHER CONSIDERATIONS:	Mood alterations can affect social interactions.
FOR ADULTS	
MEDICATION NAME:	SPLENECTOMY
HOW IS IT GIVEN:	Surgical procedure requiring hospitalization.
HOW DOES IT WORK:	The spleen plays a major role in platelet clearance.
COMMON DOSING REGIMENS:	Generally performed laparoscopically (camera) but laparotomy (open procedure) is sometimes required.
COMMON SIDE EFFECTS:	Immediate surgical discomfort. Hospitalization for 2-3 days if there are no complications. Most patients can return to their normal activity level by 6 weeks.
RARE BUT SERIOUS SIDE EFFECTS:	Life-long increased risk of blood infection (sepsis), and thrombosis (blood clots).
TYPICAL TIME TO RESPONSE:	1-3 days.
LIKELIHOOD OF INITIAL RESPONSE:	70-80% have an initial response while 10-15% have no meaningful response.
LIKELIHOOD OF LONG-TERM RESPONSE (3-5 YEARS):	~50-70%, because approximately 30% will relapse.
OTHER CONSIDERATIONS:	Generally deferred for at least 12 months after diagnosis to allow time for spontaneous remission to occur. Vaccines are required prior to surgery with boosters post-surgery. Antibiotic prophylaxis and emergent management of fevers is recommended. Splenectomy may not eliminate ITP because the spleen is not the only organ that regulates platelet counts. The possibility of an accessory spleen may be considered if a patient does not respond or relapse occurs.

## The Holiday Season is Almost Upon Us!

And Amazon has made it easy for YOU to support PDSA while completing your holiday shopping

**It's true!** All you have to do is register first on AmazonSmile. AmazonSmile is a program created by Amazon to honor consumers' charitable interests by making donations to their charity of choice.

When you shop through AmazonSmile, you'll find the exact same Amazon.com low prices, vast selection, and convenient shopping experience you're used to, **with the added benefit of generating a donation to PDSA (at no cost to you) valued at 0.5% of every qualified purchase you make!**

To get started:

1. Register with [smile.amazon.com](http://smile.amazon.com) – or download AmazonSmile through the Amazon Shopping app for both iPhone and Android devices.
2. Before you shop, choose the **Platelet Disorder Support Association** as your "charity of choice."
3. Start shopping! Qualified purchases will generate a donation to PDSA.

It's that easy!

**Every dollar counts and PDSA thanks you in advance for thinking of us during the holiday season!**

amazon smile



# PDSA Fundraisers Get Creative!

The PDSA ITP Community is an incredibly generous one! Born of the desire to give back and to help others in their ITP journey, many of our ITP warriors have held creative and successful fundraisers to support PDSA's patient-centered programming and services.

These recent fundraisers are "out of the box" spinoffs from our popular Pump It Up For Platelets! walk/run awareness and fundraising events.



*Jordyn takes a break to enjoy and share a mountaintop view.*

## Appalachian Trail Hike – Going the Distance for ITP Research

Jordyn Sak understands all too well the challenges that an ITP diagnosis brings – but this ITP warrior isn't willing to let it stop her from living a full and active life. In fact, she has set off on the adventure of a lifetime 'thru-hiking' the Appalachian Trail, which means she will complete the entire trail from start to finish. Based on her recent health journey, she has turned this challenging adventure into a fundraiser for ITP research and awareness through PDSA! Jordyn began her 2,194.3-mile hike along the famed Appalachian Trail in Spring 2022 and asked followers to support her efforts by sponsoring her for every mile she completes. To date, she has raised over \$4,200 for PDSA.

## 2nd Annual Peyt's Petals and Pies

Peyton, a young ITP warrior, decided in 2021 to raise awareness and funding for ITP research through a special homespun event for which she grew sunflowers (the state flower of Kansas, where her family resides) and baked homemade goods to sell. A family event, this creative – and delicious – fundraiser has grown quite a following in just two years, and recently raised \$9,315 for PDSA.



*Cody, Peyton, and Jessica VandeVelde set up for Peyt's Petals and Pies fundraiser.*



*Peyton personally plants and tends to the sunflowers that are eventually sold in her fundraiser.*

## 2nd Annual Devin Bowl in memory of Devin Winter

The annual Devin Bowl is held in honor of Devin Winter, who passed away at the young age of 24 from complications of two rare bleeding disorders – autoimmune lymphoproliferative syndrome (ALPS) and immune thrombocytopenia (ITP). Devin's parents, Kent and Donna Winter, are committed to actively raising awareness and advocating for the ITP patient community so that no one else will have to experience the loss of a loved one from ITP like they did. This year's event took place on September 24, 2022, with team competitions, 50/50 drawing, raffles, and a silent auction. More than 70 people participated in this special event to help raise \$14,000+ for PDSA in Devin's memory.



*Now in its second year, the Devin Bowl has established an engaged and supportive following!*



### Eileen's Special Cheesecake in honor of Joseph Ponte

In recognition of ITP Awareness Month, Eileen's Special Cheesecake, in Manhattan, NY, teamed up with the Platelet Disorder Support Association (PDSA) and AUTOImmunity Racing to bring attention to ITP by encouraging its customers to learn more and to support their efforts to raise both awareness and funding for ITP research. Throughout the month of September, Eileen's featured a "round up" fundraiser at the bakery with their loyal customers. "Ask me about ITP" t-shirts were worn by staff and posters with facts and information about ITP lined the perimeter of the store. And on ITP Awareness Month's culminating Sport Purple for Platelets Day!, Eileen's sold a limited promotional "purple sprinkle" cheesecake, with 100% of proceeds going to PDSA to underwrite ITP research. A total of \$1,300 was raised.

For Eileen's owners, this is personal. Their son, Joseph Ponte, was diagnosed with ITP in 2020 and the entire family is stepping forward to shine a light on the condition and to help fund much-needed ITP research.



*Joseph Ponte and family on Sport Purple for Platelets Day at Eileen's Special Cheesecake.*



*Joseph Ponte poses with friends from AUTOImmunity Racing who helped draw attention to this special event by parking the No. 43 ProSport Competition Aston Martin GT4 race car with Eileen's Cheesecake and PDSA livery in front of the bakery.*

### Poses for Platelets!

In response to their son Cade's ITP diagnosis, the Randolph Family proactively engaged with PDSA to learn more and to support our patient-centered efforts. In 2020, they organized a creative Poses for Platelets ITP awareness and fundraising event. In October 2022, the 3rd annual Poses for Platelets awareness and fundraising event was held, featuring donation-based yoga for adults, plus a fun gathering with raffle prizes afterward for families. With 50 people in attendance, this year's event raised \$25,000 for PDSA to support ITP research and patient programs and services.



*A special yoga session set the stage for a successful Poses for Platelets fundraiser.*

### Putting 4 Platelets Golf Fundraiser



*Putting 4 Platelets crew had fun competing with one another while supporting their friend Marcia and PDSA!*

While wintering in The Villages, Florida, ITP warrior Marcia Freed and her husband, Jim, enjoy an active lifestyle. Golf is a favorite pastime for the Freeds – as is spreading awareness of ITP and hosting a popular ITP Patient Connect support group! Blending her love of PDSA and the ITP patient community, Marcia has created the popular "Putting 4 Platelets" fundraiser, held on the links in The Villages. The second annual "Putting 4 Platelets" was held in February 2022 with 44 attendees participating in a variety of fun putting contests. A total of \$1,600 was raised and thoughtful gift bags including information on ITP and PDSA plus fun prizes were awarded to every participant.

*If you're interested in hosting a special fundraising event to raise awareness of ITP and to help PDSA do more for the ITP community, let us know! We are here to help you in any way we can. Contact Director of Programs and Events Jody Shy at [jshy@pdsa.org](mailto:jshy@pdsa.org) or (440) 746-9003.*

*Find out more about and support any of PDSA's Pump It Up For Platelets! awareness and fundraising events at [ITPwalk.org](http://ITPwalk.org).*



*The Randolph Family (with Cade sporting his cool purple hair) were thrilled to have Cade's hematologist, Dr. Melissa Rose of Nationwide Children's Hospital in Columbus, join them for their special event.*

If you have ITP  
(immune thrombocytopenia)...



Stop counting.

Keep living.

Move beyond focusing only on your platelet count  
and talk to your doctor about what really counts.

Four questions can help determine how ITP affects  
your life and how to talk to your doctor about it.



Scan the code to learn more and take  
the ITP Quality of Life Quiz

Keep  
living  
with ITP



Novartis Pharmaceuticals Corporation  
East Hanover, New Jersey 07936-1080

© 2021 Novartis

10/21

163559



# THERE ARE OVER 70+ ACTIVE CLINICAL TRIALS AVAILABLE ACROSS THE GLOBE FOR PATIENTS WITH ITP.

## IS THERE A CLINICAL TRIAL NEAR YOU?



### What Is A Clinical Trial?

Clinical trials are controlled patient studies that must be performed before a new drug or treatment is approved by the Food and Drug Administration (FDA) in the United States and similar organizations in other countries. Clinical trials are also completed after a drug or treatment is approved to document the effectiveness of the treatment, or to gain approval to use the drug or therapy for a different disease or age group. There are three

main phases to clinical trials, completed in sequence. A Phase I study tests the safety and side effects of the drug. A Phase II study is initiated to determine the efficacy of the drug, to see if it works as anticipated. And, a Phase III study tests the efficacy of drug or treatment and documents the side effects in a large population of volunteers.

### Can A Clinical Trial Benefit Me?

*Benefits vary among individuals and may include closer and more frequent health-related monitoring by researchers, the chance to sample a new drug at no cost before it is available to the general ITP community, and the chance for participants and researchers to learn more about ITP.*

### Where Can I Go For More Information On Clinical Trials?

You can visit PDSA's webpage at <https://pdsa.org/about-itp/clinical-trials> for more information about clinical trials and what you should be mindful about when participating in one. Currently, the following types of clinical trials are listed:

- **Clinical Trials Actively Recruiting in the USA**
- **Clinical Trials Actively Recruiting in Canada**
- **Clinical Trials Actively Recruiting Internationally**
- **Clinical Trials Active No Longer Recruiting**
- **Completed Clinical Trials**
- **Clinical Trials for Other Platelet Disorders**

**If you have any questions, or would like to promote your clinical trial with us, or you wish to obtain further information please contact [research@pdsa.org](mailto:research@pdsa.org)**

## CORPORATE MEMBERS

Corporate Members share in our commitment to the ITP patient community and support our mission of enhancing the lives of ITP patients through education, awareness, research, and support.

**GOLD LEVEL MEMBER**



**SILVER LEVEL MEMBER**





NONPROFIT ORG  
U.S. POSTAGE  
PAID  
CLEVELAND, OH  
PERMIT NO. 2280

8751 BRECKSVILLE ROAD, SUITE 150  
CLEVELAND, OH 44141

Postmaster: Send address changes to  
PDSA  
8751 Brecksville Road, Suite 150  
Cleveland, OH 44141



PDSA remains committed to operating accountability and transparency. We are proud to be accredited by the **Better Business Bureau**; recognized as a Gold Level participant by **GuideStar**; and designated as an official "Give with Confidence" charity by **Charity Navigator**.



# KNOWLEDGE IS POWER

**When it comes to ITP patients, the Platelet Disorder Support Association has 20/20 vision**, a clear commitment to improving health outcomes and quality of life through patient-centered research.

**Your health is our priority – and we are dedicated to investigating what matters most to you.**

We are particularly proud of our ongoing efforts to meet the immediate needs of the ITP community throughout the pandemic by accelerating research and educating patients, caregivers, and clinicians on the impact of the COVID-19 virus and its vaccines on the ITP patient population.



PDSA 20/20 ITP Research Campaign:  
*Changing the Lives*  
of ITP Patients

**The 20/20 ITP Research Campaign exists to support PDSA's Research Program and puts the collective generosity of our donors to work for you.**

Your investment will directly reinforce our influence on patient-centered research and will help yield life-changing knowledge to improve the lives of ITP patients around the world.

**Learn more and make your gift today at [pdsa.org/research](https://pdsa.org/research)**