FEATURES

Advocacy Day with the FNIDCR
Bryon Scott

In Memoriam:
DR. STEPHEN KATZ
Janet Segall

Rare Disease Day 2019
Kathleena D’Anna

Self-care
MY MYTH VS. MY TRUTH
Becky Strong

Rituxan®
HOW WILL YOU REACT?
Toby Speed

Anecdotes vs. Evidence
VALIDATING THE DIAGNOSIS PROCESS
Jessica Spilman and Michaela Gascon

Nutrition and Oral Health
Kelly Calabrese

SECTIONS

Foundation
Message from the Executive Director
Marc Yale

Awareness Update
Biopsies Save Lives
A NEW IPPF CAMPAIGN
Kate Frantz, MPH

Research & Treatments
Spotlight on 2019 American Academy of Dermatology Annual Meeting
PRINCIPA BIOPHARMA UNVEILS POSITIVE DATA FROM PHASE 2 PEMPHIGUS VULGARIS TRIAL
Dolca Thomas, MD

Psychologically Speaking
Pemphigus vs. Godzilla
Terry Wolinsky McDonald, PhD
Message from the Executive Director

As the IPPF celebrates its 25th anniversary this year, I can't help but remember what it meant to me 12 years ago when I learned about an organization that helped people like me. When I first reached out to the IPPF, I connected with Janet Segall, our founder. Janet was so calm and reassuring. She made me realize that there was hope. I suddenly didn’t feel so alone and was comforted knowing the IPPF was there to support me along my journey with this disease.

For 25 years, the IPPF has been the voice of patients in the pemphigus and pemphigoid (P/P) community. Whether we're advocating in Washington, DC, with the FNIDCR, raising awareness by educating medical professionals about our new “Biopsies Saves Lives” campaign, supporting the development of potential new treatments like the Phase 2 clinical trial Believe-PV study, or being the voice on the other end of the phone when you speak with a Peer Health Coach, the P/P community relies on the IPPF to be there.

Although the IPPF has come a long way over the last 25 years, there is still more that we don't understand about P/P. Let's celebrate what we have accomplished, but be steadfast in our commitment to the future. The IPPF must increase awareness of these diseases to accelerate diagnosis times, encourage new targeted therapies with fewer side effects to help people manage their diseases and symptoms, and learn what triggers our bodies to malfunction in the first place.

The IPPF is working hard to support you, and we need your help to face the challenges ahead!

I know first-hand how tough these diseases can be. However, it will take all of us to advocate to our federal, state, and local officials; continue our wave of awareness directed at doctors and researchers across the country; and share our stories with all who are willing to listen. Together we've helped thousands of people over the last 25 years. With your continued involvement, we'll help thousands more over the next 25 years!

Together, we're improving the lives of all people affected by P/P!
A NEW IPPF CAMPAIGN

Kate Frantz, MPH

The IPPF Awareness Program recently launched a strategic media and marketing campaign that stresses the importance of a biopsy when diagnosing pemphigus and pemphigoid (P/P). As many patients can attest, the pathway to a correct P/P diagnosis is often long and winding with multiple referrals, misdiagnoses, and ineffective treatments. To accelerate diagnosis times, the new campaign centers on a single take-away for dental professionals: “Biopsies Save Lives—4 questions to ask your patient to determine if a pemphigus and pemphigoid biopsy should be considered.” Made available on a new handout, this information not only helps dental professionals recognize the symptoms of P/P, but also goes a step further to emphasize the importance of a biopsy. This last step, while crucial, is often missed. For patients like Becky Strong, IPPF Outreach Manager, this had serious consequences: “If my dentist would have asked me these questions and had done a biopsy, I wouldn’t have had to suffer an additional 16 months.”

Time is of the essence to get patients diagnosed sooner and on a pathway to improved health outcomes so they don’t have to experience a diagnosis journey like Becky’s. While the IPPF will continue with school presentations, continuing education courses, exhibits, and Awareness Ambassador outreach, we are changing our strategy so we can get our message out faster to an even larger number of dentists. To begin, we will target five regions in the United States: Texas, California, Florida, New York, and New England. Together, these areas include 41 percent of dentists practicing in the US. We will be exhibiting at dental conferences in these regions, placing ads in dental association journals and at conferences, reaching out to dental societies, and engaging in strategies that will make it easier for dental professionals to find us online. Research shows that messages must be seen repeatedly before action is taken. Therefore, we are employing a multi-touch campaign to increase the likelihood that biopsy information is seen multiple times and remembered by dental professionals. We hope to take what we learn
from these five regions and expand our outreach to even more states in 2020.

Why focus on dentists?
Based on 2011 data, it takes the average P/P patient five healthcare providers and ten months to obtain a correct diagnosis. Approximately two-thirds of P/P patients develop oral symptoms first, and nearly 25 percent of patients see a dentist first. Of P/P patients who see a dentist when trying to obtain a diagnosis, almost half (46 percent) report that their dentists are not knowledgeable about their symptoms, and that their dentists perform no action relevant to their P/P.

More recently, the IPPF surveyed 292 dentists at 13 dental conferences in the US between 2015-2018. Preliminary data shows varying levels of confidence among dentists with 46.5 percent of dentists reporting low levels of confidence (ranking of 1 or 2 on scale of 5) in recognizing the clinical presentation of P/P. Cumulative confidence levels remain consistently low year after year. It is clear that more aggressive outreach and education is needed to help dentists feel confident when it comes to recognizing P/P.

How can you help?
Cut out the “Biopsies Save Lives” card below and take this to your dentist. You can also download the card here: https://pemphig.us/biopsy
Consider signing up to become an Awareness Ambassador. Ambassadors are trained to hand out educational materials to dentists in their local communities and share important messages on social media. If you’d like to learn more, please visit: www.pemphigus.org/awareness/ambassadors

1) IPPF. KJT Study (2011). Pemphigus & Pemphigoid Awareness and Diagnosis Pathways Survey.
2) IPPF. 2018 Preliminary Data: Dental Professionals’ Self-Reported Confidence Levels in Recognizing the Clinical Presentation of Pemphigus and Pemphigoid.

Kate Frantz, MPH, is the IPPF Awareness Program Director. She lives in Michigan with her husband and daughter.

If your patient answers YES to 3 or MORE of these questions, a biopsy should be considered.

1. Do you have more than one blister or lesion in your mouth?
2. Have your blisters or lesions lasted for more than a week?
3. Have you continually had blisters or lesions that don’t heal?
4. Do you have blisters or lesions in any locations outside the mouth?

Pemphigus and pemphigoid are rare, autoimmune, skin and mucosal blistering diseases. Patients often experience delayed diagnosis and they commonly present with oral symptoms first. These include blisters, lesions, pain when brushing or eating, and the peeling of oral tissue with simple pressure.

You can accelerate diagnosis times!
The IPPF Awareness Program’s new Biopsies Save Lives campaign is to accelerate diagnosis times.

We are counting on you to make a difference in the lives of P/P patients and their families. Donate today to educate dental professionals so they can recognize P/P symptoms, order a biopsy, and more quickly diagnosis patients.

www.pemphigus.org/donate

Both conventional H&E histology (in formalin) and DIF (in Michel’s/Zeus) are needed for a diagnosis. Specimens must contain intact epithelium over the underlying connective tissue.

More info and photos at https://pemphig.us/biopsy
There are different types of advocacy, and this is especially true for the IPPF. While several patient advocates were visiting congressional offices during Rare Disease Week, I was asked again to represent the IPPF as part of the Friends of National Institute of Dental and Craniofacial Research (FNIDCR), Patient Advocacy Council (PAC). The FNIDCR, along with the American Association for Dental Research (AADR), hosts an Advocacy Day on Capitol Hill each year.

The day started at the National Institute of Health’s (NIH) National Institute of Dental and Craniofacial Research (NIDCR). Here we had the opportunity to meet with NIDCR leadership and staff. NIDCR is the federal government’s lead agency for scientific research on dental, oral, and craniofacial health and disease. Conversation was driven by NIH staff with input from the represented organizations. At one point, they discussed efforts to modify Clobetasol (a prescription topical steroid) into an oral rinse. I voiced my interest in this project and its potential benefit to pemphigus and pemphigoid (P/P) patients. I’m glad I did because we were not on their radar as potential users of the drug. Thankfully, we are now! After our meeting, we went to the NIDCR research lab where we were shown a project related to dry mouth that is nearing completion. It served as a wonderful opportunity to see first-hand the type of research being conducted by NIDCR.

The day’s advocacy training session included important messages, such as:

- Oral health is integral to overall health. Continued investments in NIH and NIDCR are critical to improving the health of our nation.
- The work of NIDCR has led to vast improvements in the oral health of Americans. It is important to encourage Congress to ensure this research is sufficiently funded in FY20.
- The oral cavity holds tremendous promise for indicating disease and health issues. We can ask Congress to protect the important work of NIH and NIDCR and raise the caps.

The afternoon was spent meeting with congressional staff. It was a “hurry up to the next office, then wait in the hallways” kind of day. I am always prepared to discuss P/P and the IPPF with other constituents as we wait, and this year definitely afforded me that opportunity. There were several UNC dental research students in my group, and a few of them had even attended the Dental Day at last year’s IPPF Patient Education Conference. I spent most of my downtime answering questions from the students about how pemphigus affects my life and how a dentist missed the diagnosis. While I cannot guarantee the outcome of how members of Congress will vote, I am sure there are several future dentists or dental researchers that will have P/P on their radars.

Bryon Scott, ADAC, is the IPPF Awareness Ambassador Coordinator and a pemphigus vulgaris patient. He lives in North Carolina, but travels across the country helping government entities and businesses comply with the Americans with Disabilities Act.
Spotlight on 2019 American Academy of Dermatology Annual Meeting

PRINCIPIA BIOPHARMA UNVEILS POSITIVE DATA FROM PHASE 2 PEMPHIGUS VULGARIS TRIAL

Dolca Thomas, MD, Chief Medical Officer at Principia

We are encouraged by the positive results coming from Principia Biopharma’s Phase 2 clinical trial in patients with pemphigus (vulgaris and foliaceus). In a late-breaking oral presentation at this year’s AAD Annual Meeting, Principia announced Phase 2 clinical data from the Believe-PV study for PRN1008 (an oral pill) which is being developed for the potential treatment of pemphigus. The Phase 2 study reached the primary efficacy measurement of control of disease activity (CDA) on low-dose corticosteroids.

“The primary goal of treating patients with pemphigus is to control the disease and heal the skin, however a significant challenge is to avoid adverse events associated with the prolonged use of corticosteroids that are typically required to achieve clinical improvement,” stated Dr. Dedee Murrell, Professor and Head of the Department of Dermatology at The St. George Hospital Clinical School, University of New South Wales in Sydney, Australia and the lead principal investigator. “PRN1008 has the potential to rapidly and effectively treat patients’ disease, while significantly reducing the exposure to moderate to high corticosteroid doses.”

In addition to reaching the primary endpoint of CDA in 54 percent of patients on low-dose corticosteroids by Week 4, a complete response rate of 25 percent was also achieved with 12 weeks of treatment. The treatment was also generally well-tolerated, which suggests a favorable risk-benefit profile for patients with pemphigus.
The most frequently reported treatment-related adverse events were nausea (15 percent), upper abdominal pain (11 percent), and headache (11 percent). There was one treatment-related serious adverse event in a patient with a localized patch of leg cellulitis, whose treatment with PRN1008 resumed after three days for a further eight weeks without event recurrence. If you would like to learn more about PRN1008 (an oral pill), the Phase 2 trial, or the ongoing Phase 3 clinical trial (the PEGASUS study) of PRN1008, visit: www.principiabio.com/patients

The study is being conducted to evaluate whether PRN1008 plus corticosteroids is safe and effective in helping pemphigus patients achieve complete remission, and to determine the ability of PRN1008 to reduce corticosteroid use.

PRN1008, an orally-administered Bruton’s tyrosine kinase (BTK) inhibitor, is investigational and has not been approved for commercialization.

In Memoriam:
Dr. Stephen Katz

Janet Segall

On December 20, 2018, Dr. Stephen Katz passed away. Dr. Katz was the director of the National Institute of Health’s (NIH) National Institute of Arthritis and Musculoskeletal and Skin Diseases (NIAMS). He became director in August of 1995 and remained in the position until his death.

Dr. Katz was an outstanding doctor and researcher. His focus was immunology and the skin, and he was very interested in autoimmune skin diseases. He trained many dermatologists interested in immunological skin disease from around the world and held leadership positions in various professional societies. Dr. Katz received many awards for his work.

Dr. Katz was an important friend to the IPPF. I met Dr. Katz on my first trip to NIAMS in the spring of 1998. I asked him some very pointed questions, and I think he saw how important my mission was to me. In all the times I met with him, he treated me with dignity and respect.

I received the following message from Dr. Katz in June 2009:

“I happily remember our first meetings where you were trying to figure out where you needed to go and how you needed to get there. One thing was clear at that time—you knew what you ultimately wanted—recognition of the importance of pemphigus and other blistering skin diseases as a public health issue, as well as a coordinated and concerted effort to learn more about the diagnosis, treatment and potential prevention of these diseases. Your perseverance has certainly paid off in that the IPPF is now a reality..... I know you have worked at this and I admire not only your efforts but your accomplishments.”

We honored Dr. Katz at our first Annual Patient Education Conference in New York in 2006. He told me that he came to the meeting because I personally asked him to attend. I was very honored to have been acknowledged by Dr. Katz and was proud that he took a huge interest in our Foundation. I am sorry that he has passed on, but his legacy and work continue. Rest in peace, Dr. Katz.

Janet Segall is the Founder of the IPPF and a PV patient since 1983. She is a Peer Health Coach and works in Sacramento, CA, in the mental health field.
I began my own pemphigus journey in 2001, and like most of you, I was up against a serious chronic illness that had never been on my radar. *Pemph-what?* It is now 2019, and other than my immediate circle of friends and family and all of you, it feels like most of the world remains clueless.

After I was diagnosed, I used to carry note cards in my wallet with general explanations of autoimmune illnesses—pemphigus vulgaris (PV) in particular. I didn’t want to explain it to someone who asked but may not have had a real desire to know or understand. It was easier to hand them an explanation of the basics. Now I just carry small pieces of paper with the names of my illnesses and some associated websites. (Like many of you, I have more than one serious autoimmune illness.) Most people may never read the paper, but those who are truly interested can “look it up” and find more information about these diseases.

I feel like no matter how much educating I do, the lack of knowledge about these orphan diseases remains simply appalling to me. I may not know about or understand many other rare illnesses, but when I come across a disease new to me—whether from a patient or someone I meet or read about—I make a point of Googling it for at least a basic understanding. I wish I could say the same for other professionals with whom I’ve come into contact. In my experience it feels like there are simply too many rare and complicated illnesses out there. So, if an illness is both rare and not infectious, it may not be a priority to most people.

A few months ago, I was sent a beautiful article about the protocols and support systems (community and hospital-based) in place for various cancer diagnoses. It was written by a friend’s daughter who was diagnosed with cancer and successfully treated. The article was very well-written, optimistic, educational, and
informative for patients. In addition to support groups and wigs, they provided yoga and other recreational activities—even massages! Now, I do realize that all these services are not available everywhere, but it was still a wow moment for me. Having lost friends and family members to cancer, I was happy to know that these options even existed; they are certainly needed.

Recently, another family member was diagnosed with cancer. She went to a facility in a different state. Almost immediately after her diagnosis, an oncologist was called in for a consultation and discussion of treatment options. Similar services that my friend’s daughter received were discussed as part of the treatment plan. They even offered a mental health component with a psychiatric nurse, certified social worker, and psychologist. Additionally, she had access to tests, treatments, and groups—as well as art, creative writing, and mindfulness classes—that were not considered out-of-pocket expenses. I know that cancer is a word everyone recognizes, but not all cancers are the same. It makes me question why there are not more medically approved services available for pemphigus and pemphigoid (P/P) patients.

The IPPF continues to educate professionals and provides free services for people diagnosed with P/P, including access to educational and supportive services for their families. Education and awareness have also come a long way. Still, people know more about Godzilla than they do about P/P. What is wrong with this? What else can we do? We can continue to get out there and educate, educate, educate; we can contribute financially; we can donate our time and skills. We can also continue to work with other rare disease groups to increase awareness. We must continue work to raise congressional awareness on Capitol Hill and find ways to help fund more services. Wouldn’t it be amazing to see P/P and related services covered by Medicare?

My husband is a science-fiction buff. He often asks me questions like, “Who do you think would win, Wonder Woman vs. Godzilla vs. the 50-Foot Woman vs. Mothra?” Many of us have heard of these fictional characters, so as ridiculous as it sounds, we can hypothesize answers to these questions. But Godzilla vs. pemphigus or pemphigoid? P/P are certainly aggressive attackers of their host bodies. How would you answer that question?

Terry Wolinsky McDonald, PhD, is a PV patient, clinical psychologist, and former IPPF Board member living in Pittsburgh, PA and Sarasota, FL. She is a regular contributor to the Quarterly in her “Psychologically Speaking” column.

Find other stories and the latest info on the IPPF news site: pempress.com
This year marked my first, but certainly not last, time attending Rare Disease Week on Capitol Hill. It was my desire to educate that motivated me to leave my comfortable Southern California home and fly across the country to brave the icy Washington, DC, winter.

In summer 2014, after nearly two years of suffering with painful mouth ulcers and mysterious scalp and skin lesions, I finally received my diagnosis of pemphigus vulgaris (PV). I was 22 years old, had just graduated from college, and was in the process of completing my medical school applications. I’m now in my final year of medical school, and I understand why it took numerous dentists, four primary care doctors, and two dermatologists before someone decided to do a biopsy.

There is a saying we learn within the first few weeks of medical school: When you hear hoof beats think of horses, not zebras. But I’m a zebra, and I’m positive there were other zebras sitting next to me in class on a daily basis. From my perspective, it’s such a strange mantra to be drilled into our eager minds. While it’s true that most physicians will see common diseases—the horses—no single person is a textbook. We must always remember the zebras.

I went to DC not only to raise awareness of my own rare condition, but also to be a part of something bigger. Something important. To be honest, I had no idea what to expect when I arrived, but I left Rare Disease Week with more than I ever thought possible. I learned a lot from keynote speakers distinguished in their fields and master communicators who taught us how to effectively tell our stories. Advocating and educating at the United States Capitol exposed a previously unexplored world to me in which I can use my unique position as both a patient and a physician to promote positive change in the rare disease community.

Going to the conference also gave me the opportunity to explore the DC area for the first time. While I was out and about, I saw dozens of familiar orange bags around the city held by other conference attendees, and I felt a warm sense of belonging. Throughout the week, I was able to connect with other individuals who have been through similar experiences. Together, we shared stories, advice, and hardships. Overall, I had an amazing experience at Rare Disease Week, and I can’t wait to attend again!

Kathleena D’Anna

Kathleena D’Anna is a 2019 DO candidate. She is a Fourth Year Osteopath Medical Student and lives in Southern California with her siblings.
Thank you to our corporate sponsors, Argenx and Kroger, for helping us send advocates to Capitol Hill for Rare Disease Week 2019.
When I open any magazine or social media platform, I start to believe that caring for myself involves a lot of exercise, a beach body, a bubble bath, and a glass of wine. Everywhere I look, I see people taking care of themselves by exercising to the limits: lifting weights, running marathons, and losing tons of weight. Or they spend hours with organic bath bombs, lounge around without a care in the world, and have wild nights partying with friends.

For some, this may be what self-care looks like. If this is you, please keep doing whatever you need to keep up your mental and physical well-being. For me, it took a while to realize that there is no right or wrong way to take care of my mind, body, and emotions. Self-care can mean many different things depending on where I’m at in my life.

Sometimes my version of self-care looks like an extra cookie, crying in the shower, or pausing to listen to my children’s laughter. Other times it looks like pushing hard at the gym, stepping out of my comfort zone, or having a night on the town with friends.

For me, it took a while to realize that there is no right or wrong way to take care of my mind, body, and emotions. I’ll admit, there was a time when I just didn’t feel right unless I started the day with a 5K, and Friday night meant time to party and play hard. After being diagnosed with pemphigus, self-care changed for me.
With everything on my plate (family, diagnosis, work, doctor appointments, finances, dog, volunteering, homework, housework, etc.) life was anything but relaxing. Trying to convince myself how good my gym time or girls’ night felt actually added more stress to my life instead of taking it away.

I do try to eat healthily and exercise regularly, but I no longer allow myself to feel the pressure of not being perfect. There have been days when I’ve considered it a glowing success to actually get out of my bed and go downstairs. Caring for my well-being started to include giving myself permission to just breathe, allowing myself to acknowledge how hard I tried, and recognizing I made it through the day, even if 99 percent of my goals didn’t get accomplished. I had to give myself permission to cry and feel the weakness in my body so I could move past it and be better.

Strength now means that I am the best me I can be today, and I am strong enough to try my best tomorrow. This is true for me as a patient and as a caregiver. I was once told that if I didn’t take care of myself, I wouldn’t be able to take care of anybody else. This was probably the best piece of advice I received, and I’ve carried it with me in my personal and professional life.

It’s not selfish to take care of my needs. Nobody else can determine what is the best form of self-care for me. I need to know myself and recognize my needs. Doing so makes me strong, healthy, and fierce. Sometimes I can go much further just by taking a step back. Life becomes clearer and easier so that I can accomplish so much more.

We all have different ways to take care of ourselves. Whether you cook or clean to relax (if this is you, come on over!), prefer to read, snuggle with your favorite childhood stuffed toy, or count by random patterns, it’s important to take the time to take care of yourself. We are the experts of our bodies and minds, and society cannot tell us how to best take care of our own needs. There is not a right or wrong way to make ourselves better. What’s important is that we are prioritizing ourselves and our health.

Becky Strong is the IPPF Outreach Manager. She was diagnosed with PV in 2010 and is currently in remission. She lives in Michigan with her family.

Looking for a Support Group?

It doesn’t have to be formal to be a group. All you need is another person, a place to sit, and time to talk. The important thing is to share your experiences and get the support you need.

To find others in your area, contact Becky Strong: becky@pemphigus.org.
Has your dermatologist been talking to you about Rituxan®? Chances are he or she has mentioned possible side effects—including a few that are nasty, but rare—along with the assertion that most people tolerate the drug well. If you’re a pemphigus vulgaris (PV) patient who’s been treated with prednisone, CellCept®, IVIG, or other medications, you know all about side effects. You feel ready for this next step in your treatment and are optimistic that your doctor has weighed benefits against risks in determining this is the best path for you.

But, as you head into your first Rituxan® infusion, how do you know for sure you’ll be okay? What if you’re one of those folks who has a reaction?

I was diagnosed with PV in December 2010, and to date I’ve had four rounds of Rituxan®. All four have been problematic, as I experience an allergic-type reaction each and every time I undergo infusion. My experience is not typical, but it can, and does, happen. While I’ve experienced relief from the drug in terms of lesions healing and titer counts going down, the hard time I have taking it has impacted me in three ways:

- I feel a lot of apprehension heading into treatment.
- The reaction experience itself is unpleasant.
- Infusions take much longer for me than for most people, adding expense and exhaustion to the process.

When my doctor recommended Rituxan® four months after my diagnosis, I was taking prednisone and CellCept®, and I hadn’t completely accepted that PV was a new part of my life that would need to be continually managed. I was still hoping that if I followed instructions and took my medications, it would fade away. I don’t like to take medication unless it’s absolutely necessary, not even cold medicine.

Once my symptoms were under control, my dermatologist referred me to a hematologist, who agreed that I was a good candidate for Rituxan®. I told him I’d think about it. We made an appointment to meet again a few weeks later. During that time, I did some research on the internet, and Rituxan® sounded awfully powerful.
Indeed, I scared myself out of taking it. I don’t think I was in touch with the IPPF yet, as I was still in denial about having the disease. That was unfortunate—the IPPF would have helped me.

In October 2011, I had a bad flare that involved a large part of my body. For a while, my disease had been active in my mouth and throat, which I considered par for the course. Then it was everywhere. I was visiting my daughter in California—3,000 miles from home—when it happened. Over a couple of days, I developed so much pain in my mouth that I could hardly swallow. My clothing hurt me; I couldn’t walk. We went out to dinner one night with my daughter, son-in-law, and his parents, and I ordered something I thought would be bland. To my surprise, the food ravaged my mouth and gums, and I began to cry at the table. Quite embarrassing, to say the least. The rest of my stay was torturous, as my symptoms took over my life.

I called my dermatologist from the airport and left him an urgent message. When I got home, he put me on 60 mg of prednisone, and again he advised Rituxan®. This time I agreed.

I reached out to one of the IPPF Peer Health Coaches around then and read up on what to expect at the infusion center. I was to receive 1,000 ml of the drug over a four- or five-hour period, along with preparatory medications, and a second infusion two weeks later. I settled into the chair with my book, snack, and blanket. After the IV was in and the Benadryl, steroid, and other medications administered, the Rituxan® was started at a low rate. Immediately I felt a tingling sensation throughout my body, not unpleasant. The infusion rate was bumped up a few minutes later, and I hardly noticed a difference as I began talking to another patient nearby.

About 20 minutes after the infusion began, a few things happened in quick succession. My throat and neck started to itch, and the itchiness rose to my face. I tried to pay attention to the conversation I was having. Was this itching normal? Why wasn’t the Benadryl helping? Within seconds my ears began to itch fiercely, and then I realized I couldn’t swallow. Next my tongue began to feel thick, and I couldn’t speak. And what was this? I was short of breath. I couldn’t catch my breath! All of this happened in about a minute.

I tapped the bell at my side, and a nurse ran in and disconnected the tube. Immediately there was a sea of concerned faces in front of me—all of the nurses and a doctor—asking me what was happening. “Itchy,” I managed to whisper. One of the nurses pushed Benadryl into the IV line while a second hooked me up to oxygen.

It took maybe an hour for the reaction to subside and my system to calm down. Eventually, the Rituxan® was started again at a low rate, and it never went above 75 ml/hr. After about 10 hours, they had only gotten about 600 ml of the drug solution into me, and I went home. I had to come back the next day for the remaining 400 ml, which they’d prepared fresh, since the prior day’s leftover medication had to be discarded.

I dreaded coming back in the second day, because I was so afraid it would happen again. The speed of onset combined with the sensation of not being able to breathe or swallow frightened me, even though I knew the medical team would be there to set things right. The infusion rate was again kept at 75 ml/hr, and I was able to get through with no reaction. Two weeks later I again had infusions two days in a row, receiving 500 ml each day. Each time I was there for at least eight hours.

This has been the pattern ever since. I’ve had Rituxan® once a year for the past three years, and on the first day of infusion I always react within the first half hour. I itch, my throat closes up, and the medical staff rushes in and brings me back to safety. They know to watch me carefully, and we even joke about how I’m there from breakfast to dinner. Sometimes I have to stay after closing hours to finish my dose with a nurse and a doctor. Other patients come and go, but I stay. But I’d rather avoid my marathon infusions, if I could. I go through the same cycle of dread and fear each time I set up another round of Rituxan®, before I even set foot in the infusion center.

Most people don’t have problems with Rituxan®, and you may happily be one of them. I hope so! But in case things don’t go exactly as planned, may I suggest packing extra drinks and snacks, a cozy blanket, slippers, and headphones in your tote bag? There’s nothing like some cool music or your favorite podcast for whiling away the hours while the Rituxan® drips slowly (and safely!) into your veins.

Toby Speed is a PV patient and the author of seven children’s books, lots of poetry, and a murder mystery. She has three daughters and two granddaughters and is on the brink of relocating from New Hampshire to North Carolina.
Anecdotes vs. Evidence
VALIDATING THE DIAGNOSIS PROCESS
Jessica Spilman and Michaela Gascon

How long does it take to ascertain the correct diagnosis? Which doctors will patients see, and who will they be referred to next? These seemingly direct questions are often all too complicated in rare diseases. The pathway is circuitous, and all constituents are desperately interested in finding the answers sooner. Patients are often shuffled around without much guidance or reassurance that a meaningful diagnosis can be reached. Providers lack exposure and familiarity with rare diseases, leading to trial and error or passing the patients along to another who might know how to help. Everyone is frustrated: patients, providers, and advocacy groups.

If we could better educate providers about disease symptoms and triggers, perhaps patients could be diagnosed sooner and begin appropriate treatment sooner as well. That’s exactly what the International Pemphigoid and Pemphigus Foundation (IPPF) had in mind. Pemphigus and pemphigoid are rare autoimmune diseases that present in various ways and do not improve without active treatment. Patients typically see many different providers over a substantial period before finally receiving the correct diagnosis, which the IPPF saw as a major problem. With limited funds available for healthcare provider education and outreach, though, they were interested to know where to prioritize healthcare professional education efforts to shorten this time-to-diagnosis and create a better experience for patients.

KJT Group was commissioned to design a quantitative survey for patients diagnosed with pemphigus and pemphigoid to answer three main questions:

1. How long did it take to be correctly diagnosed with pemphigus or pemphigoid?
2. Which medical specialists did you see during the diagnosis journey?
3. Who accurately diagnosed you?

KJT Group recruited a sizable group of 87 patients who were already connected with the IPPF to participate. Their responses were rich and descriptive, yielding robust data for a diagnosis pathway analysis. We found that while most patients seek medical attention within three months of their initial symptoms, they had to see an average of five physicians over nearly a year before receiving the correct diagnosis. With our data, we were able to generate a succinct analysis of the most common referral patterns and the types of physicians who most often made correct diagnoses. Harnessed with this information, the IPPF was able to optimize educational efforts geared at accelerating the referral process, with the goal of achieving a faster diagnosis for patients.

Decisions about how to allocate limited funds to tackle a sizable and important problem must be made smartly. Although conducting a large-scale quantitative study within any rare disease space is a challenge, it is worthwhile! Rather than relying on qualitative anecdotes, having robust evidence to drive these strategic investment choices gives confidence the decisions will be impactful, ultimately improving the quality of life for rare disease patients.

Jessica Spilman, MPH, is VP, Research at the KJT Group, and Michaela Gascon is COO at the KJT Group.
I vividly remember the days when blistering sores were so prevalent in my throat, tongue, gums, cheeks, and lips that anything I consumed hurt. I couldn’t eat much, but I was able to drink an ice-cold smoothie consisting of fresh watermelon, pear, and basil blended with ice. This drink felt very soothing, calming, and reduced my pain for a moment. Also, pea soup (not very hot) was another smooth and calming food. It can be difficult to get proper nutrients into the body when everything hurts. Each of us experiences a different pain threshold and so the process is very individualized. Trial-and-error is the way for most of us.

Brushing my teeth was another big undertaking. I found toothpaste made of baking soda with natural botanical oils very soothing. Trying different toothbrushes was another project as well. The very soft bristle toothbrushes were too soft; they did not clean my teeth well. And of course, the stiffer tooth brushes hurt. Luckily, I was able to find a happy medium. For each of us, the choice is going to be different depending on how much pain one can take.

Since it was difficult to get the nutrients my body needed to heal, supplementing was important for me. Vitamin C powder mixed in water was imperative since citrus burned and caused me even more pain. Another product was a protein powder consisting of pea or collagen protein that offered great nutrient support with vitamins, minerals, and amino acids. Mixing the protein powder in coconut or almond milk was soothing. Lastly, I took some liquid homeopathic remedies to help support my endocrine system for stress. I encourage you to research, ask questions, and don’t stop being your own best detective. I wish you all the best.

Kelly J. Calabrese MS, CCN is a Board Certified Clinical Nutritionist in Colorado Springs, CO. She has been a patient since June 2017 with PV and has been in remission since December 2017. She has been a contributing writer for the Quarterly and writes on health, wellness, fitness, and nutrition.
Celebrate the IPPF's 25th Anniversary
at the
2019 IPPF Patient Education Conference

Save The Date:
October 11-13

Hosted by
Dr. Aimee Payne, MD, PhD &
Dr. Victoria Werth, MD

Wyndham Philadelphia Historic District
Philadelphia, PA

More details coming soon.