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Message from the Executive Director

The past four months have involved a lot of changes for both staff and me as I’ve settled into my new role of IPPF Executive Director. I’m happy to report that this time of transition has gone smoothly, and I’m even more optimistic about the future of the IPPF and our community than I ever have been before.

Though I’ve served in various positions at the IPPF for the past 7.5 years, being the Executive Director means I now have the unique privilege of witnessing how every aspect of our community comes together to make us all stronger. As we look forward to the rest of the year, I thought you might be interested in what I’ve seen these past few months. Here are just a few highlights:

Research

In the rare disease world, new research initiatives can seem few and far between. However, in the past few months, the IPPF has seen a dramatic increase in research interests from industry partners. This is an encouraging sign, as we hope for more clinical trials and potential new therapies for both pemphigus and pemphigoid in the future. Most importantly, industry and research partners are recognizing the importance of incorporating the patient perspective in the early stages of investigating potential new therapies.

Events

You may have noticed an increase in the frequency of our virtual events. Throughout the COVID-19 pandemic, we’ve tripled the amount of patient webinars on our calendar. This has been in direct response to your requests for more information, and our outreach team has certainly delivered.

Looking forward to the second half of 2022, we recently made the decision to again hold our Patient Education Conference as a virtual event. Though we will miss seeing you in person, the continued uncertainty around the pandemic makes a large gathering of potentially immunocompromised people unwise. This year’s conference will be October 21-23. Like our previous two virtual conferences, I expect we will reach many more members of our community than our in-person events. Connecting a greater number of people from around the globe who are living with pemphigus and pemphigoid is a silver lining of these virtual conferences—one that we plan to incorporate well into the future.

Staff and Volunteers

I have to take a minute to tell you about the IPPF staff and volunteers. As Executive Director, I get to be the one to talk about our accomplishments, but it’s the staff and volunteers who have achieved them. I’m honored to work with people who care so deeply about helping others. Every day, I witness an unwavering commitment to serving our community. Every challenge brings a renewed focus on the same question: How can we improve the quality of life for people affected by pemphigus and pemphigoid? Our staff may be small, but each one of them cares about you. And so do I.

Patrick Dunn, IPPF Executive Director
patrick@pemphigus.org
A Recap of the 2022 International Blistering Disease Consensus Group Meeting

Marc Yale

The IPPF recently hosted a meeting with the International Blistering Disease Consensus Group at the American Academy of Dermatology (AAD) Annual Meeting on Saturday, March 26th, in Boston, MA.

At the meeting, the Foundation was pleased to announce that Becky Strong, IPPF Outreach Director, will be representing the IPPF on the newly formed Global Dermatology Coalition. The mission of the Coalition is to improve patient outcomes globally by raising awareness of the impact of dermatological diseases and increasing the healthcare prioritization of these conditions. The Coalition will be providing a global platform for interested stakeholders to take collective action to raise global awareness of the burden of dermatological conditions, put dermatology on the global health policy agenda, and build dermatology advocacy capacity globally. The Coalition will focus on:

- Collating data and generating new data (as needed) on the burden of dermatological conditions
- An evidence-based awareness campaign to prioritize dermatological conditions
- Collective advocacy to global and regional governmental institutions (e.g., WHO)

The IPPF also shared its plans to host an Externally-Led Patient Focused Drug Development (EL-PFDD) Meeting with the Federal Drug Administration (FDA), as well as its continued commitment to crucial research projects like the IPPF Natural History Study and work with academic researchers on scientific studies.
Dr. Victoria Werth, Professor of Dermatology at the Hospital of the University of Pennsylvania, and the Veteran’s Administration Medical Center, discussed the FDA’s interest in instituting a new patient assessment tool that will have a simple score for pemphigus and pemphigoid (P/P), called an Investigator Global Assessment score (IGA). Dr. Werth stressed that IGA score of zero or one, which does not account for partial improvement. Dr. Joly explained that this situation is quite difficult for patients with mucous membrane pemphigoid (MMP) since many patients are in partial remission and may never achieve complete remission. He suggested that if an IGA scoring system was to be instituted, it must be very simple, definitions must be easily understood and include references to usual typical clinical features, and it must be applicable to blistering and nonblistering subtypes. Additionally, he discussed how the proposed IGA scoring system or the current BPDAI measures must be validated properly before using them as measurement tools for drug development.

All attendees agreed that it would be beneficial for the FDA to hear the perspective of patients from the IPPF community. A suggestion was made that the IPPF create an awareness campaign to educate both the FDA and legislators on the issue. The IPPF will consider different ways to reach congressional representatives and include patients, caregivers, dermatologists, researchers, and other rare disease groups. The IPPF could also collect data through a community survey to evaluate opinions on what measurements constitute a meaningful disease improvement (i.e., number of lesions, quality of life improvement, etc.). This could then be shared with the FDA at the future Externally-Led Patient Focused Drug Development Meeting.

Ultimately, patients affected by P/P need more treatment options for this potentially life-threatening set of diseases. The IPPF community plays an important role in advocating for better disease measurement scores, which can then be used to design better clinical trials in the future.

Marc Yale was diagnosed in 2007 with cicatricial pemphigoid. In 2008 he joined the IPPF as a peer coach. He became the executive director in 2016 and recently started his role as IPPF research and advocacy coordinator. Marc currently resides in Ventura, CA, with his wife Beth and his daughter, Hannah.
I recently heard that **Drs. Luis Diaz and Russell Hall** had both officially retired. I’ve known both of them for a very long time, Dr. Diaz almost since the beginning of the IPPF’s existence. He was one of the first doctors to join our Medical Advisory Board back in the ’90s and was a speaker at our very first Annual Patient Meeting in Chicago in 1998. Both Dr. Diaz and Dr. Hall worked tirelessly on behalf of the IPPF and pemphigus and pemphigoid (P/P) patients.

Dr. Diaz has been a long-time physician and treated many people with pemphigus. He often traveled to Brazil and Colombia where he found pockets of patients with pemphigus foliaceus (PF) and pemphigus vulgaris (PV). He investigated possible triggers of these diseases, including the disclosing mechanisms operating in PF and PV. He also researched the etiology of Brazilian PF (Fogo Selvagem) as a joint project with Brazilian investigators. The research projects of this laboratory have been supported by the National Institutes of Health (NIH). His laboratories are credited with landmark research studies during the last 30 years related to developing the animal model of PF and PV, the description of the mucosal and mucocutaneous phenotypes of PV, epidemiological and immunological studies on endemic PF in Brazil, IgG4 anti-desmoglein 1 restriction of the humoral IgG response in endemic PF, and the unique IgM anti-desmoglein 1 response in endemic PF. ([https://www.med.unc.edu/derm/research/luis-a-diaz-md-professor-emeritus/](https://www.med.unc.edu/derm/research/luis-a-diaz-md-professor-emeritus/)).

Dr. Diaz received his Doctor of Medicine from the Universidad Nacional de Trujillo in Peru. He did his postdoctoral fellowship at the Mayo Graduate School of Medicine in Rochester, NY. He did his dermatology residency at the State University of New York, Buffalo, NY. He received Honorary Recognition as Best Doctors in America: 1979, 1992-1997, 1999-2000, and 2004-2020.
At the 2018 IPPF Patient Education Conference in North Carolina, we honored Dr. Diaz with a lifetime achievement award for his dedication to his patients and to the P/P community as a whole. He is currently a Professor Emeritus at the University of North Carolina, Chapel Hill.

Dr. Russell Hall has been involved with the IPPF for many years and played an important role working in consultation with other medical professionals, IPPF staff, and patients. His special interests have been investigating the pathogenesis of autoimmune blistering diseases with a special interest in dermatitis herpetiforms (DH), BP, and PV. Within his laboratory, they are looking at the role of the B cells in the development and maintenance of auto-antibodies. His laboratory has been involved in clinical trials for both PV and BP. His team has also investigated how gluten sensitivity might play a role in the development of DH.

Both Dr. Diaz and Dr. Hall worked tirelessly on behalf of the IPPF and pemphigus and pemphigoid (P/P) patients.

Dr. Hall graduated from Westminster College in Fulton, Missouri, and the University of Missouri—Columbia School of Medicine. He was trained in internal medicine and dermatology at St. Louis University, the University of Missouri—Columbia, and Johns Hopkins School of Medicine. He completed a research fellowship in the Dermatology Branch of the National Cancer Institute, National Institutes of Health. After five years at the NIH he joined Duke’s faculty as an assistant professor of medicine in 1984. In 1998 Dr. Hall was named Chief of the Division of Dermatology and served in that position for more than a decade. Dr. Hall was named J. Lamar Callaway Professor of Dermatology in 2002 and played an integral role in the transition of dermatology from a division to a department serving as Chair of the Department from 2009 to 2021.

Dr. Hall authored over 150 scientific articles, reviews, and book chapters focused on the clinical and investigative aspects of immune mediated skin diseases. He served on the Board of Directors of the Society for Investigative Dermatology and as President from 2017-2018.

It has been a pleasure working with Drs. Diaz and Hall for many years. We will always be appreciative of their dedication to P/P patients and the IPPF.

Janet Segall is the Founder of the IPPF and a PV patient since 1983. She is a Peer Coach and works in Sacramento, CA, in the mental health field.
Advocacy

The Institute for Clinical and Economic Review (ICER) recently released a white paper on rare diseases that offers several potentially misguided proposals that would not benefit our community or help bring new treatments to market for pemphigus and pemphigoid (P/P). A far more thoughtful proposal from Reps. Doris Matsui (D-CA), Mike Thompson (D-CA), Mike Kelly (R-PA) and Markwayne Mullin (R-OK) is H.R. 6160, the Access to Rare Indications Act, which recognizes the fact that the vast majority of rare disease patients still don’t have an FDA-approved treatment. This illustrates that clinicians shouldn’t have to struggle to convince payers that medically accepted off-label treatments are “medically necessary.” Most importantly, it gives rare disease patients the same opportunity to get an off-label treatment that non-rare patients enjoy.

Medical necessity is the yardstick by which insurance companies, including Medicare and Medicaid, decide if they will pay for a particular treatment. When drugs are prescribed, payers look first to its label to see if it treats or manages the particular condition in question. If the prescribed use is off-label, they look to evidence-based compilations of drugs and their uses (compendia). This seems to work well for common conditions and in most cancers, which have robust compendia to address heavy reliance on off-label uses. Congress also ensured Medicare and Medicaid cancer patients have access to promising off-label uses that are not yet incorporated into compendia but are supported in published peer-reviewed literature.

Like cancer, off-label use is often the only treatment option for P/P patients. Treatment decisions are based on clinical guidelines, published research, and

Marc Yale

The Importance of the Access to Rare Indications Act
disease-specific expert consultation. Unlike cancer, there are no rare disease compendia to track off-label treatment protocols. So, there is a disconnect between the resources used to make treatment decisions and those used by payers to determine coverage. Just as insurers look for evidence of medically necessary cancer treatments in sources that contain up-to-date and reliable information, they should look for evidence on rare disease treatments in sources where that information exists: peer reviewed journal articles, clinical guidelines, and the handful of clinicians who dedicate their careers to specific rare conditions.

They recognize that we can't take for granted what those with more common conditions have: coverage for the treatments they need.

Many P/P patients are prime examples of why Congress should extend to rare diseases similar options to those it put in place for cancer many years ago. To illustrate that point, I have shared my story and talked publicly about my experience. I live with mucous membrane pemphigoid, a rare, life-threatening autoimmune disease that causes blistering lesions throughout the body. I fought with my insurance for two years to get the right treatment, even though it is the standard of care for my condition. My insurance put me on other medications and told me the prescribed treatment was off-label, experimental, and not on any compendia. While I fought with insurance, I lost my eyesight, my career, and was hospitalized. The financial and emotional burden of not only the disease, but getting the “wrong” treatment, was enormous.

Now I’m on what is considered the standard of care treatment for pemphigoid, and my condition is stable. Patients like me can eventually get the treatment we need if we can find highly specialized clinicians practicing in institutions with the resources to push through prior authorizations, reconsiderations, and appeals. It takes time to fight off insurance companies’ efforts to insist that patients try cheaper alternatives first, which can be onerous and even harmful when they are ineffective or contraindicated. Educating payers, convincing their medical review staff of the evidence supporting off-label use, and the ineffectiveness of cheaper alternatives does not save any time for other patients, either. Worst of all, the cycle begins all over again for each new patient, even when their doctors are dealing with the same insurance company.

Patients living in rural and underserved areas who are unable to travel to specialists and subspecialists may have an even more difficult time getting treatment and insurance coverage. Tragically, if the patient is a Medicare beneficiary and the treatment is a Part D product, there is no coverage at all beyond labeled indications and uses listed in compendia. This can be very discouraging and make patients want to simply give up.

Patients should not have to live through experiences like mine just because the diverse set of rare disease mechanisms and specialties will never support the kind of compendia oncologists rely on. The IPPF, along with more than 60 patient organizations, support H.R. 6160, not because they want “special” status or additional “protected classes,” but because they recognize that we can’t take for granted what those with more common conditions have: coverage for the treatments they need. People could then spend more time on their jobs, with their families, and save their caregivers and doctors endless hours of uncompensated time.

It will take Federal legislation to apply the medical necessity standard equitably so that rare disease patients have the same level of coverage available to most everyone else. I encourage you to reach out to your representatives in Congress and call on them to begin working on this crucial issue by supporting H.R. 6160, the Access to Rare Indications Act. It takes the conviction that comes from hearing from patients struggling every day to get the treatments they need to recognize that more must be done.

Marc Yale was diagnosed in 2007 with cicatricial pemphigoid. In 2008 he joined the IPPF as a peer coach. He became the executive director in 2016 and recently started his role as research and advocacy coordinator. Marc currently resides in Ventura, CA, with his wife Beth and his daughter, Hannah.
The Coalition of Skin Diseases (CSD) is a voluntary coalition of patient advocacy groups addressing the needs and concerns of millions of people whose lives are affected by skin diseases. For the IPPF, working with the CSD provides an opportunity to advocate on behalf of dermatology patients. We have the ability to amplify the voice of our community to help improve access to healthcare and provide better funding for researchers to find or repurpose medications to treat pemphigus and pemphigoid (P/P).

Each year, the CSD holds an annual meeting where we have the chance to network with leaders from organizations that support people with other skin diseases. We’re able to learn about what they’re doing to meet the needs of their communities so that we can better serve our own. Due to the COVID-19 pandemic, it’s been over two years since the coalition was able to meet in person, and this spring we were grateful to meet in person and work together.

On March 26, 2022, the IPPF staff attended the CSD Development Day at the Annual American Academy of Dermatology (AAD) Meeting in Boston, MA. Executive Director Patrick Dunn, Outreach Director Becky Strong, and Research and Advocacy Coordinator Marc Yale, participated in this meeting that explored the challenges that each patient community experiences. It was humbling to learn that many of the issues that the IPPF community face (misdiagnosis, time to receive a diagnosis, lack of Federal Drug Administration (FDA) approved treatments, and difficulty finding knowledgeable doctors) are also felt by many others. It was exciting to work together and explore possible solutions that would benefit the many organizations of the CSD. As a result of various data collected at the meeting, the CSD hopes to publish a paper with the results and present it to the AAD leadership team in hopes of working together to improve outcomes and clinical guidelines for patients.

During the CSD meeting, we also heard from Arash Mostaghimi, MD, MPA, MPH, from Brigham and Women’s Hospital, on the state of physicians, particularly dermatologists, throughout the pandemic. He described the many challenges that dermatologists experience in helping patients get the care that they need. Dr. Mostaghimi felt that most physicians experience similar struggles as their patients. He highlighted the importance of patients and physicians working together and discussed the range of emotions that physicians feel as they fight insurance companies, pharmacies, and hospital organizations to get their patients the care they need.

While this was only one meeting, the IPPF meets with member organizations of the CSD throughout the year. We look forward to learning more about how the IPPF can work with dermatologists and other organizations to lessen the burden of skin diseases.

Becky Strong is the IPPF Outreach Director. She was diagnosed with PV in 2010 and is currently in remission. She lives in Michigan with her family.
Bullous pemphigoid (BP) is the most common autoimmune blistering disease, and patients often suffer from ongoing blistering, hives, and persistent itch. Currently there are no FDA-approved medications for BP, and treatments tend to consist of nonselective immune suppressing medicines. Novel treatments are desperately needed and will require appropriate clinical trials to rigorously test these medications. Part of the challenge with BP clinical trials has been the lack of a standardized disease scoring system to quantify disease activity that can be measured over time in a clinical trial. The assumption is that a certain change in disease activity would demonstrate treatment benefit and could be used to justify FDA approval.

Fortunately, the International Bullous Disease Group has developed the Bullous Pemphigoid Disease Area Index (BPDAI), which allows clinicians to determine a specific disease activity score based on the number and size of lesions (blisters/redness). With the BPDAI, the blistering community now has a tool to potentially pilot for use in clinical trials. One of our goals is to see how the BPDAI activity changes over time in BP patients who are on standard therapies. This specific type of clinical trial is called an observational trial, which essentially means that we will be monitoring patients at defined time points while patients are on any standard therapy that is recommended by a treating dermatologist. Results from this observational-type study could provide information on how changes in the BPDAI might be used to predict success in a clinical trial of a new medication.

One of the other challenges of BP clinical trials is the difficulty in recruiting and keeping patients in the trial. This difficulty can occur for various reasons, including—but not limited to—the fact that many patients with BP are elderly and have varying social support networks, multiple clinical trial visits can require substantial time commitments, or too great a distance is required to travel to academic centers. In an attempt to improve patient participation, a unique feature of this observational trial is that we have included remote visits that can be done from the comfort of a patient’s home. The trial is essentially a hybrid trial in which participants meet our research team for screening and potential enrollment if specific criteria are met. Then, follow-up visits are performed remotely. This gives patients the opportunity to take on a substantial role in the research study by capturing photos of the various sites of disease involvement with their smart phone and rapidly uploading the photos for us to remotely view and calculate a BPDAI activity score. Specific surveys related to how the disease affects a patient’s quality of life can also be captured by a simple email.

This trial is sponsored by the National Institutes of Health (NIH), and we are excited to share it with the IPPF and blistering disease communities. Our research team at Emory University School of Medicine is looking forward to working with BP patients to design more effective clinical trials to allow for eventual approval of novel therapies against this devastating disease. For more information, visit clinicaltrials.gov (https://clinicaltrials.gov/ct2/show/NCT04728854) (Identifier NCT04728854), or contact our research team in our Dermatology Center for Outcomes Research and Safety at 404-778-2772.

Taylor Adkins is a Clinical Research Coordinator at Emory University School of Medicine. Emily Cole is an Assistant Professor in the Dermatology Department at Emory University School of Medicine. Ron Feldman is an Associate Professor and Director of the Autoimmune Blistering Disease Clinic and Dermatology Center for Outcomes Research and Safety at Emory University School of Medicine.
To say the journey toward the diagnosis of my blistering disease has been smooth and reassuring would be disingenuous. It began with an odd symptom I mentioned to my dentist when the mucous membrane in my mouth began sloughing off in thin strips. It was not painful, but disconcerting to pull long strips of flesh out of my mouth with my tongue. I was told sodium lauryl sulfate was the culprit and received the recommendation to use a toothpaste without this ingredient. After changing toothpastes, the symptom improved and I didn’t think anything else about it.

About a year later, I noticed my gums were swollen and would bleed when I brushed my teeth. It freaked me out since I grew up going to the dentist every six months. My mother worked for a dentist, which resulted in my continuously being told how beautiful my teeth were. My gums looked like I had gingivitis, and I was so embarrassed. At my next appointment, I still had the indentions from the face mask and head gear on my face from my CPAP machine. I was told it was common for people’s gums to look like this if they use a CPAP. Then came the inquiries into my cleaning habits of the machine. I left feeling my ugly gums were all my fault and something I could have prevented.

The next evening, I noticed my first large blood blister on my upper gum. I took a picture and sent it via email to the dentist, who wrote a prescription for Mary’s Magic Mouthwash and referred me to see a periodontist. It was a few weeks before I could get an appointment.

Being told I had a rare, autoimmune blistering disease resulted in many internet searches.
In the meantime, I faithfully used the mouthwash, but continued to have new blisters form on both the lower and upper gums. At my periodontal appointment, I had been in the dental chair about five minutes before I was told it was one of three things: lichen planus, pemphigus, or pemphigoid. The conversation also covered whether a biopsy should be performed. The agreement was that there wasn’t a need for a biopsy since the treatment would be the same regardless of the blistering disease. A new treatment with a steroid mouthwash (dexamethasone .5 mg/5ml X4/day) was prescribed.

Being told I had a rare, autoimmune blistering disease resulted in many internet searches. I stumbled upon the IPPF and read as much information as I could find on the website. I was given the names of a couple of medical professionals near me who were familiar with pemphigus and pemphigoid. I scheduled a date and time for an appointment within a few weeks. However, my family all came down with COVID-19 after Christmas. My brother was hospitalized, and I had to reschedule the appointment.

Around this time, my scalp started itching, and there were blisters forming there as well. I soon noticed five tiny, itchy blisters on my arm. I had not yet been to my appointment at the faculty practice of the Indiana University School of Dentistry and felt I needed to find a dermatologist in my city who was familiar with pemphigus and pemphigoid patients. The dermatologist performed a punch biopsy of a blister on my scalp and ordered blood work for BP180 and BP230 IgG antibodies, along with Desmoglein 1 and 3 antibodies. Oddly, the punch biopsy showed the blisters on my scalp were lichen planus, and the blood work showed high antibody titer for BP180, which I was told indicates bullous pemphigoid (BP). I was started on a tapering high dose of prednisone, a cream for my arm, and a dental paste for my gums. I am now finished with the prednisone taper. The blisters on my scalp have stopped forming, and the blisters and erosions on my gums have slowed down and are not bleeding as badly! A couple of days ago, I did additional blood work to see if I have a normal amount of Glucose-6-Phosphate Dehydrogenase to get approval to start taking Dapsone, which I have been told is supposed to be the treatment of choice for BP.

I have met with Angela Ritchie, DDS, in Indianapolis, who is involved with the IPPF community. She performed a test to determine how dry my mouth is and found it was very dry. She added some new medications and a new diagnosis of mucous membrane pemphigoid (MMP), but said I needed to have another biopsy done to confirm the type of pemphigoid because MMP can cause blindness. She also recommended that I see an ophthalmologist as soon as I could get scheduled.

I am sharing my story not because I have all the answers, but simply because I do not. Despite this, I have found a place of hope and people who care through the IPPF. The more we can share our personal journeys, the more hope we can offer to those who are beginning their own.

Kelly is a 62-year-old wife and mother of two children. In April 2021, she completed her Master of Arts degree in Clinical Mental Health Counseling and obtained her associate license. She currently works as an instructional assistant in Muncie, IN. She loves playing and watching most sports and games with family.
In 2012, I was diagnosed with pemphigus foliaceus (PF). I was 21 years old and finishing an internship to earn my bachelor’s degree in social work. At that age, it felt like a curse to experience the ugliness of this condition. How was it possible that my own body was attacking itself? Why now? Lots of my questions were answered over time, but some are still, and could forever be, unknown to me.

I was started on prednisone at the moment the biopsy came back positive. Since the very beginning, I sought information and guidance for possible treatments and palliative care options to ease the pain the medication produced. I experienced symptoms of early depression and generalized anxiety, mostly induced by the prednisone and my new diagnosis. We are likely all familiar with these feelings.

In 2013, my doctors talked to me about Rituxan®. They told me it benefited patients with pemphigus vulgaris (PV). I did my research and opted to take the medication. As I navigated the approval process, I didn’t want to get my hopes up in case it was denied by my health insurance coverage. After a long process of justifying the treatment with academic papers and other evidence, my insurance company approved my treatment, and I only had to pay for hospital visits. The approval process concerned me. What if I needed the medication again and my insurance wouldn’t cover it because it was not approved for my PF? How could other patients work this out?

I wanted to do more. In 2015, I saw the application for Rare Disease Week on Capitol Hill, an event conducted by Rare Disease Legislative Advocates. I applied and received notice that I was one of the selected patients to receive their stipend. It was both amazing and saddening that I was the only person from Puerto Rico that year. Our political status as a Commonwealth of the United States of America grants us some rights, but it also demands more responsibilities than the benefits we receive. Our
political system establishes that a congressional representa-tive is elected by the citizens to represent Puerto Rico. This is the Resident Commissioner in Washington, DC. They don’t have a right to vote, but can be involved in lobbying activities and express Puerto Rico’s concerns. Also, they can identify federal and state funding to promote wellness in our archipelago.

After the amazing experience of attending Rare Disease Week, I had to take a break because of a new job. Four years passed, and the COVID-19 pandemic became our new reality. I had the opportunity to advocate virtually during Rare Disease Week on Capitol Hill. I was very fortunate to share this moment with another Puerto Rican who advocates for his son’s condition of Laminopathy-related Congenital Muscular Dystrophy (L-CMD) and disability. He directs a nonprofit organization called the Eli Foundation. Over the past few years, the organization has worked very hard to meet with our Representatives in Puerto Rico and conduct press tours to share the stories of people with rare diseases.

On July 29, 2021, the Puerto Rican Senate approved legislation to promote education and sensibility toward people with rare diseases, and to acknowledge and recognize Rare Disease Day in Puerto Rico as the last day of February every year. On February 28, 2022, the Commonwealth of Puerto Rico recognized Rare Disease Day for the first time.

Right now, we are keeping up the momentum. We have scheduled meetings with Puerto Rico’s Department of Health; legislators; and the Resident Commissioner, Congresswoman Jenniffer González. If the national bills we are supporting get approved, there will be more work to do in Puerto Rico to receive the benefits. But, we are building a community to stand as a united front and to continue advocating for rare disease patients.

Angélica N. García Romero is a pemphigus foliaceus patient and advocate. She is 31 years old. Her academic preparation is in social work, and her practice is in nonprofit management. She was born and raised in Puerto Rico, where she lives today with her partner.
I’ve always been blessed with good health and a sharp mind. I was an exercise junkie and prided myself in working through injuries, illnesses, and a host of internal signals that I was overdoing it. I was a mom, an intensive care doctor, and a fitness fanatic. I did not tolerate moderation from myself or those around me. I took for granted that the gifts from Mother Nature and my ancestors would usher me painlessly into old age.

Just before the COVID-19 pandemic transformed the world as we knew it, I pivoted away from clinical medicine. After 20 years of my identity being defined by two letters attached to my name—MD—I walked away without a firm plan for the future. I needed to return to my creative roots. I packed up my apartment and moved across town, where I would live incognito and make a fresh start. I was planning to be an independent consultant to families with relatives in the ICU. I thought it would all happen overnight, and I’d live happily ever after in my new luxury (translation: extravagant) apartment with large corner windows and granite countertops in an elevator building with a 24-hour doorman, a pool, and a gym. I imagined my son and I would have French toast and mimosas every 4th Sunday at our favorite café and take in a movie on nights when he found himself without a better option. I’d use my oven for cooking, rather than storage, and have cocktail parties and opera subscriptions.

Halloween has always been my favorite holiday. It’s the one week each year that I allow myself to finish off the stash of candy I hoard for an unexpected deluge of costumed little people that I know will never come. But this year was different. I’d barely managed to complete my usual 10-mile walk-run in the park the day before, and I was winded and light-headed from walking one block to buy my favorite candies: Tootsie Roll Pops and Milk Duds. I blamed it on being awakened several times during the night by an unrelenting itch in a spot on my back just out of reach. It was unlike any itch I’d experienced before; a burning sensation as much as an itch, and the hydrocortisone that had always worked on my
eczema did nothing to soothe it. Then I remembered the disturbing experience I’d had in the shower the day before. As the hot water hit my back, I spasmed like a caught fish reeled onto the deck before it realizes that fighting for the solace of the sea is futile. And there were three new bumps on my neck.

After two weeks of sleepless nights, and no relief after changing my detergent, soaps, body lotion, and makeup, I reluctantly called a dermatologist. I didn’t have an internist. I’d relied on my colleagues to sign the annual medical forms required by my employee health department. I had not been on the other side of an exam table since I’d seen a neurologist in a panic after experiencing my first ocular migraine more than 10 years earlier. I thought I knew as much about my body as any doctor who would be seeing me for the first time. After all, I was an experienced pediatric intensive care doctor. I’d overlooked the fact that while I knew quite a bit about childhood ailments, 65-year-old adults were not just big children.

I was fortunate to find a compassionate physician with an available opening the next day. He listened patiently to my story, examined the rash (which had blossomed overnight to cover my whole body), and told me he was certain I had scabies (although my rash was in all the wrong places). I was to apply an ointment from head-to-toe and leave it on for 12 hours, wash everything that could go into a washing machine, and seal in air-tight plastic bags anything that couldn’t. I was also to contact my landlord for a bedbug inspection. I asked for a biopsy of one of the bumps, and he obliged me. Armed with a prescription for a stronger anti-itch cream, I left the office, exhausted and skeptical. How does an obsessive germaphobe who always keeps a bottle of Purell within arm’s length contract scabies?

Four days later, my phone rang at 10:00 p.m. The call was from a cell phone number I didn’t recognize. It was my dermatologist, who had saved my call for last so he wouldn’t be rushed. “Dr. Bishop, I received your biopsy results, and you don’t have scabies. You have bullous pemphigoid.”

In one brief phone call, this gentle and compassionate stranger rocked the world as I knew it. I was no longer the strong, youthful, indefatigable, superwoman who would never fall prey to her limitations.

During my 20 years of practice at a major medical center, three years of residency at a top-ranked children’s hospital, and four years of medical school, I couldn’t recall ever hearing of bullous pemphigoid (BP). I had been the student who read and memorized the captions beneath the illustrations in the textbook. How had I missed the one that described the condition that would later afflict me?

In one brief phone call, this gentle and compassionate stranger rocked the world as I knew it. I was no longer the strong, youthful, indefatigable, superwoman who would never fall prey to her limitations. I imagined myself in freefall— somersaulting towards a scary and dark unknown netherworld— catapulting from confident and respected physician to quivering, chronically-ill patient, facing an unknown future. I felt alone and isolated, left to find my way without a compass.

Dr. A. explained what he knew about this rare disease, the pity in his voice only partially shielded behind “med speak.” I grasped only an occasional word here and there; the rest sounded garbled, as though he was speaking under water. I was focused on my life sentence.

As a physician, I always researched illnesses that I knew little about. I owed it to my patients to be as knowledgeable as I could be so that my decisions about their care were based on evidence. Now that I was the patient,
I was even more determined to read every reference I could find.

In the days that followed, I sat hunched over my laptop, scouring PubMed. I was searching for answers to my questions: *Why me? Will this be forever? Will my 96-year-old mother outlive me?*

I then remembered how my ex-husband had found out he had diabetes just before our 15-year wedding anniversary. I’d watched him navigate his diagnosis, normalizing it as much as he could. He was determined not to let the condition define him. He’d hesitated at first, but later joined a support group.

I closed the PubMed site and opened a new Google search bar. As if they were on autopilot, my fingers tapped the keys with a new sense of urgency: B-U-L-L-O-U-S P-E-M-P-H-I-G-O-I-D S-U-P-P-O-R-T G-R-O-U-P S-N-Y-C. One entry appeared: pemphigus.org. I spent the next several hours reading every article, and marveling at how normal the people with this condition looked and acted. They had jobs, families, and hobbies. They had lives. Many were in remission and, most importantly, they had futures. A ray of hope returned to my world.

It’s been five months since my diagnosis. With the help of the IPPF, a brilliant medical team, and my family, I am healing. My BP is under control. The itching returns periodically, but it’s tolerable. The simple act of getting out of bed, once a chore, is again routine. I am enjoying my favorite foods, freed from the medications that nauseated me. I’ve even been able to enjoy dinner with my son at our favorite eatery. My memories of those tough early days are fading. The after-effects of the steroids are slowly going away. Spring is coming, and I hope to return to my beloved running trail in Central Park. I’ll be satisfied with two miles and comforted knowing that the body I no longer recognize may one day feel familiar.

My journey with this disease has taught me so much: how it feels to be the one with a chronic condition instead of the sympathetic outsider looking in; how challenging and unsympathetic our medical system can be, even to “medically-privileged” patients; how willing my new BP friends are to share their practical tips and tricks; and how helpful support groups can be. It will be a while before I no longer see myself as a patient. But with the help of the IPPF, the love of my family, and support from my fellow BP warriors, I know I can soldier on.

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*Naomi Bishop is a semi-retired physician-writer living in New York City. She was diagnosed with bullous pemphigoid in November, 2021. She currently shares an empty nest on the Upper West Side of Manhattan with five sun-loving plants. When she’s not working, she reads voraciously, and enjoys podcasts and conversations with her two adult children. The four things she could never live without are The Atlantic, The New Yorker, New England Journal of Medicine, and her iPhone.*
The morning of December 30, 2021, began in our new home like almost every other work day. Coffee was consumed, emails checked, and the day was planned out in our minds. The window treatment installers came that day, and with COVID numbers rising around the holidays, we left the windows open after they’d gone. A little extra caution can go a long way. Besides, the air outside was beautifully crisp.

Then, in an instant, our world changed. The first thing my husband and I noticed was the smell. We wondered why someone was barbecuing in Colorado in December. Then again, we knew that Coloradans are hardy. As we closed the windows, we saw it: churning, swirling, brown air encircling our home. This was no barbecue. It was a fire. We packed a few key items into a go-bag, and we were out the door and driving out of town. Seven minutes later, we received a reverse 911 call about the fire. Those seven minutes made all the difference.

Leaving the traffic of evacuees in our wake, we were free to follow the bright sunny sky and clean air. We secured a hotel room a few hours away before it was sold out, shopped for supplies, and tried to offer support to evacuees arriving after us.

“Hey, where is your house located?”
“Any word about the condition of your house?”
“Our home may be gone.”
“We may lose everything.”

And there it was. The common thread among our little group standing in the hotel lobby. We may lose everything. The camaraderie of this group of complete strangers helped get us through those super scary hours.

The time immediately after the fire reminded me of another stressful period of my life: when I was first diagnosed with mucous membrane pemphigoid (MMP) and began a series of sleepless nights alone with my iPad and Google.

“Where is your MMP located?”
“What is your treatment plan?”
“Can I ever be happy again?”

“Will I survive this disease?”
Will I survive? Survival is one of the common threads among our support group members. We meet, we share, we survive. We offer encouragement, humor, advice, and strategies. We seek to thrive.

One of our members summed it up best:
“Our IPPF Support Group not only has been a fun way to meet others on Zoom, but also to learn more ways of living and coping with this disease. With the long, necessary isolation to protect ourselves from COVID-19 that most of us practice, our group provides much needed socialization, especially with others who understand our situation so well. Additionally, it has linked us with others who live nearby, adding additional, personal Zoom and telephone friendships.”

The Rocky Mountain Area Support Group makes a real difference. Our collective knowledge and experience contribute to each and every one of us. Two of our members living in the same area help each other by sharing local care team options. Watching other members find their way has been inspirational.

Two months after the Marshall Fire, we moved back into our home, grateful that all the houses on our little street survived when over a thousand homes were lost. We will never be the same. Even as I write this article, we are on fire alert with go-bags positioned at the front door.

The support of our informal group of evacuees from the fire was valuable during that short window of time in December. The Rocky Mountain Area Support Group is even more indispensable as we continue to share this adventure together. Join us at our next virtual meeting on Sunday, July 17th. Register at https://www.pemphigus.org/events/.

Lisa Casden is a public speaking coach to executives and emerging leaders within financial services, both domestically and abroad. Diagnosed with MMP in 2019, she is inspired by those who have shared their journeys with others.
My wife’s disease story began during a visit to her ophthalmologist. The doctor was ending the visit when he suddenly returned to check her eyes again, proceeded to say that she seemed to have a serious eye condition, and then suggested that she have a biopsy by another eye specialist. At the time, my wife Sandy didn’t have any eyesight issues. Since we were unsure whether it was needed, we made an appointment with my ophthalmologist. During the visit, he asked if she had any pain in her eyes. She explained that she didn’t, but that she had some blurriness that went away after a short time. Without excruciating pain, the ophthalmologist disregarded his suspicions.

Over time, Sandy began experiencing more blurriness as well as pain, but most of her pain was in her mouth. The doctor was then convinced the pain was something profoundly serious and caused by blisters in her mouth and throat, which were a side effect of her disease. Sandy continued using eye drops, and it turned out that her pain in her eyes was from the preservatives in the variety of eye drops being prescribed. During one of her eye appointments, the doctor told her he had exhausted what he could do and referred her to a cornea specialist. When visiting the cornea specialist, he immediately said that she had mucous membrane pemphigoid (MMP) and that she should go to a research hospital. He also suggested that she see a rheumatologist.

When she saw the rheumatologist, they told her she needed to have an official diagnosis of her condition. The rheumatologist also suggested that she should begin taking medication in order to control her disease. Additionally, it would make it easier to satisfy medical insurance companies of the seriousness of her condition. We needed a diagnosis for her. At some point, I read that it can take over a half dozen doctors’ diagnoses to convince insurance companies to approve medication coverage.

After seeing an ophthalmologist at the University of California, San Francisco (UCSF), Sandy was officially diagnosed with MMP and was advised to get infusions of “Medication A” to slow the progression of the disease. Now that Sandy had been officially diagnosed, I searched...
online to determine how much the medication would cost. I learned that it was expensive, and we would need help paying for it.

We received a lot of advice from our son-in-law, Alan, who is a medical insurance agent. In addition, we got advice from our medical insurance’s member advocate (MA). The help from these two resources enabled us to get financial help for the medication.

I learned that there are several organizations that provide financial help for a wide variety of diseases. We got help from the company who produces “Medicine A,” which has its own foundation. Of course, we had to provide proof of financial need. After being accepted, we learned that Sandy also needed more frequent infusions of a different medication. Once again, we provided proof of financial need and were accepted. In this case, the same company that provided “Medicine B” also provided a nurse to administer it at home. Since the cost of medications to treat pemphigus and pemphigoid are often high, I encourage patients and caregivers to explore all their options for financial assistance.

When we realized that we would need financial help to pay for the costs of Sandy’s treatment, we started learning more about the therapies for her condition. This is when Alan began helping us find our way through the bureaucracy. He had me check with our medical insurance for help, and they provided us with the MA. Our advocate made us aware of how to deal with the required insurance approvals and next steps. She was an indispensable resource. Alan further suggested that we get itemized statement costs of the infusions. Since some of the infusions had to be performed at a setting that could provide emergency resuscitation, we reached out to her doctors to make sure the hospital had the correct permissions. At the same time, I completed the financial forms for “Medicine A.”

While trying to satisfy the requirements to receive financial help, we learned that there are several organizations that offer financial assistance for medications. The internet is an invaluable resource. In addition to utilizing an MA and reaching out to friends and relatives for advice, we also got ideas from Sandy’s infusion doctor, the infusion manufacturer’s foundation, infusion nurse, and the home health agency that did the infusions. Some other helpful resources in the US may include: Medicare (800.633.4227), Social Security (800.772.1213), your state’s Medicaid office, the State Pharmaceutical Assistance Program (SPAP), and state health insurance programs.

Editor’s note: Although the IPPF does not provide financial or prescription assistance, there is additional information on this topic available on the IPPF website. This information is provided for informational purposes only. The IPPF is not affiliated with, nor endorses, the companies and organizations listed. www.pemphigus.org/prescription-assistance

Kenneth Levine’s wife, Sandy, was a patient with MMP that lived in El Paso, TX, with their daughter and son-in-law.
My First Rare Disease Week on Capitol Hill

Twelve years ago, my life changed forever when I was diagnosed with pemphigus foliaceous (PF). I was covered in lesions and blisters from top to bottom, including my legs, arms, torso (front and back), scalp, and face. For the first year or so, I felt so alone until my wife found the IPPF. We attended an IPPF Patient Education Conference to learn more about the disease and to meet others who were going through the same situation. When I left the meeting, I had gained so much information and met many others who offered peer support. For once I did not feel alone.

After a couple of months, I contacted the IPPF to connect with a Peer Coach and was in touch with one immediately. He was very understanding, offered peer support, told me that I was not alone, and that he would help me through this journey.

A few years later, prior to reaching remission in 2016, I mentioned to my wife that I wanted to give back to the pemphigus and pemphigoid (P/P) community. I wanted to help others in the same way that the IPPF had helped me. I gathered information to start a support group in the Austin, TX, area. I held a meeting and helped others who were going through what I went through. I told my wife that I felt like I could do even more. I was then approached and asked to assist the IPPF with Spanish-speaking patients and became an IPPF Peer Coach. I still held support group meetings while also helping others as a coach. I felt that I could do even more, so when I was asked to participate in Rare Disease Week on Capitol Hill and tell my story, there was no question that I would be more than happy, and honored, to join.

This year’s Rare Disease Week on Capitol Hill was my first time attending the virtual session, and it was an experience I will never forget. I would gladly do it again. I was a bit nervous going into the session, as I would likely be speaking with members of Congress. I attended a training session and was able to write down what I wanted to say about my journey to hopefully make a difference in the community and for other rare disease patients. It was very impactful to share my story, ask legislators for their support, and make them aware of the daily battles rare disease patients face. I not only shared about the daily struggles we face, but also the long-term side effects from medications.

The importance of advocacy is not only to share my own journey, but to be a voice for all patients. It allows us to be heard so that we can encourage new treatments that are needed to improve our quality of life. In my meetings, I spoke about how going through step therapy treatments affected me and how it affects others who are also going through similar treatments. My ask to legislators was for insurance companies to stop making us go through step therapy treatments, having us suffer, and allowing our disease to continue to spread. Instead, we should be able to receive the treatments that are needed right away not only to gain control of our diseases, but also to reach remission. This gives us the opportunity to live a somewhat normal life.

Receiving treatments deemed necessary by our doctors as soon as possible helps to potentially reduce the amount of medications that patients have to take. This also helps limit side effects and can give patients and our families a better quality of life.

I wear many hats with the IPPF and give back to the P/P community in every possible way. I am a PF patient, coach, and support group leader. I have spoken at the FDA listening session and am now a rare disease advocate. I hope to continue to advocate for rare diseases by attending Rare Disease Week on Capitol Hill for years to come.

Rudy Soto was diagnosed with pemphigus foliaceous in 2009 and has been in remission since 2016. He lives by the motto, “can’t grind me down.”

Rudy Soto

The importance of advocacy is not only to share my own journey, but to be a voice for all patients.
Spotlight

Four Questions with Dr. Donna Culton

Our Spotlight section features a medical professional whose work regularly impacts the lives of pemphigus and pemphigoid (P/P) patients. Get to know a new physician, researcher, or other medical professional who knows these diseases best. This issue, we’re featuring Dr. Donna Culton.

Dr. Culton completed her medical degree at the University of North Carolina at Chapel Hill. While there she also received her PhD in the Department of Microbiology and Immunology where she studied autoreactive B cell development and regulation. She continued her training at UNC, and following her Dermatology residency, she began applying her knowledge of autoreactive B cell pathophysiology to pemphigus by studying B cells and autoantibodies from patients. Her laboratory also generated a novel murine model of pemphigus allowing for a better understanding of mucosal pemphigus vulgaris. In her current position as Associate Professor of Dermatology at the University of North Carolina at Chapel Hill, she serves as the Director of the Clinical Immunofluorescence Laboratory and the Associate Director of the Clinical Trials Unit at UNC and sees pemphigus and pemphigoid patients from North Carolina and neighboring states in her specialty Autoimmune Blistering Disorder clinic. She has served as an investigator in clinical trials in mucocutaneous autoimmune diseases; has contributed to consensus statement publications as part of the International Bullous Diseases Group; and supports outreach, education, and advocacy through her involvement with the IPPF.

How did you become interested in pemphigus and pemphigoid (P/P)?

I became interested in the immune system and autoimmune disease early in medical school and eventually chose to pursue a PhD studying B cell development and immune system mechanisms for inactivating autoreactive B cells (and why these mechanisms sometimes fail). B cells are supposed to respond to foreign proteins and make protective antibodies. As we all know, sometimes B cells accidentally respond to important self-proteins and these autoantibodies create disease. While my PhD research was focused on lupus, I had the good fortune of meeting two mentors during that time (Luis Diaz, MD, and David Rubenstein, MD, PhD) who introduced me to P/P—and I was hooked! I continue to be fascinated by these diseases and the different parts of the immune system that go awry during their development. Understanding these mechanisms of disease at a very detailed level is what will allow us to develop new treatments that are uniquely targeted to P/P. As a medical community we know a lot about P/P, but still have so many questions to answer about how and why these diseases develop!

What is one thing you’d want all patients to know early on in their journey with P/P?

Early on in a patient’s journey of diagnosis and treatment, it is easy to feel overwhelmed, alone, and even depressed. However, each patient becomes part of this larger P/P community. The IPPF provides resources that treating physicians can’t always provide, such as peer support, a connection to other patients, resources on wound care and self-care, and a community. I encourage all patients, particularly those early in their journey, to take full advantage of these IPPF resources.

What can patients do to better advocate for themselves?

Three things are critically important:

1. Patients should listen to their body and report any new symptoms or unusual changes to their P/P physician. Not every new symptom will be related to P/P or the treatments (in fact, many times they are not), but that is up to your treating physician to help figure out with the help of your general doctor.

2. Every patient should have a great primary care physician (general doctor)! While a general doctor may not be an expert on P/P, patients need a team of physicians that are willing to communicate and collaborate with each other to provide the best care possible.

3. I encourage patients to play an active role in their treatment plan and goals. While most patients have the goal of complete absence of disease, achieving that goal may come with more aggressive treatment and an increased risk. It is important to understand your treatments and the risks and make an informed decision with the help of your treating physician.

What is one fun fact about yourself?

I have been making candles and body products like soap, lip balm, and body creams for about 10 years now! This hobby allows me to use both my creative and scientific sides when choosing the ingredients and fragrances for each product. There is an assumption that these products are 100 percent skin-safe, but when gifting them to others I never promise recipients they won’t get a rash—only that I know a good dermatologist if they do!