OUR NEW EXECUTIVE DIRECTOR | PEMPHIGOID GESTATIONIS | NATURAL HISTORY STUDY

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As many in our community know, 2021 was a very successful year for the IPPF. From record attendance at our Virtual Patient Education Conference to creating connections for researchers at our Scientific Symposium, we found continued opportunities to grow the impact of our organization. Years of awareness efforts culminated in offering a continuing education course with the American Dental Association. Our peer health coaches adapted to the ongoing pandemic and provided additional support to patients and caregivers. These are just a few highlights from a year with many to choose from.

What you may not know is that Kevin Mead, the IPPF’s Executive Director, stepped down at the end of 2021 to pursue other opportunities. Though Kevin’s time with the IPPF lasted only 15 months, his impact was immediate. Kevin successfully applied for and received several high-profile grants, streamlined operations, and managed a virtual staff and team of volunteers with a deft hand.

Upon Kevin’s departure, the IPPF Board of Directors evaluated the needs of the Foundation to determine how to best move forward in the coming years. The Board conducted an exhaustive search and selected a staff member who shows a deep understanding of the IPPF’s vision, mission, and a commitment to the needs of our community.

The IPPF Board of Directors is pleased to announce Patrick Dunn as our newest Executive Director. Patrick has been working for the IPPF since 2014 when he joined the staff as a health communications specialist. His role grew over the years, and he has most recently worked as the IPPF’s Communications and Marketing Director. “It’s a tremendous honor to serve pemphigus and pemphigoid patients and their caregivers, as well as the medical professionals who support them every day,” Patrick said. “I’m excited to work in this new capacity, especially knowing the talent that the rest of our staff brings to their jobs. The IPPF has never been stronger, and there are many initiatives in the works to make a brighter future for patients.”

Patrick Dunn officially assumed his new role as the IPPF Executive Director on February 28, 2022. We are all looking forward to this next chapter. Patrick can be reached directly by email or phone (916) 435-6751.
IPPF 2021

PATIENT SUPPORT

16 Patient Education Webinars
29 Support Group Meetings
783 Peer Health Coach Cases
5,005 Find a Doctor Map Visits

VIRTUAL PATIENT EDUCATION CONFERENCE

762 registered attendees from 46 countries

AWARENESS PROGRAM

12 Patient Educator Presentations
316 Optometry & Medical Students Reached
1,110 Dental Students and Faculty Reached
1,128 ADA Dental Professionals Reached for CE Course
YEAR IN REVIEW

NATURAL HISTORY STUDY

- 40 new participants
- 277 submitted surveys
- 689 total registrants

INTERNATIONAL SCIENTIFIC SYMPOSIUM

- 10 Countries
- 30 Sessions
- 46 Abstract & Poster Submissions
- 250 Participants

ADVOCACY

- 38 sign on letters to legislators and regulators
- Advocated to over 100 congressional members
In October 2015, Carolyn Fota was a newly retired Lieutenant Colonel, United States Army, Medical Service, who was finally going to enjoy time with family after a simple surgical procedure at Walter Reed National Military Medical Center in Maryland. However, within 24 hours of the procedure, hives and then blisters broke out across her body. It was the beginning of a nightmare that no one ever expected.

By December, a small army of medical staff at Stafford Hospital, Virginia; Fort Belvoir Community Hospital, Virginia; Walter Reed National Medical Center, Maryland; and the Mayo Clinic, New York, were collaborating on Carolyn’s intense medical support. Finally, in February 2016, she was diagnosed with bullous pemphigoid (BP). Treatment consisted of oral and topical steroids, tetracycline, nicotinamide, probiotics, Benadryl, magic mouthwash, and vitamin D3. Topical steroids were applied to her legs and arms, which were then wrapped in sterile dressings. She underwent intensive wound care for the next six months. It was during this time that Carolyn first connected with the International Pemphigus & Pemphigoid Foundation (IPPF).

“The IPPF was a blessing to me and my family and provided critical support when we needed it,” Carolyn said. “Through patient services like peer health coaching, support groups, educational webinars, website information, research updates, clinical trials, and the annual Patient Education Conference, I learned a lot about BP, and I made a lot of friends.”

Carolyn is active in the IPPF community through peer health coaching, Mid-Atlantic Support Group leadership, advocating on Capitol Hill to our nation’s leaders, and participating in various IPPF projects whenever asked. She became a member of the IPPF’s Board of Directors in the summer of 2021 and was recently selected to lead the Board as its Chair.

In a statement about her new position, Carolyn said, “I am deeply honored to be trusted to serve as the Chair of the Board of Directors. The IPPF has a rich tradition of having some of the most outstanding leaders serving this organization, and I stand on the foundation they have built. I promise to work hard in navigating the organization in terms of strategy, financial stewardship, and caring for our community.”
The 2021 RareVoice Awards were held on December 15, 2021, by the EveryLife Foundation for Rare Diseases. The evening celebrated rare disease advocates who make their voices heard year-round to advance policies that benefit the rare disease community.

We’re very proud of Marc Yale for his nomination in the Federal Advocacy Patient/Organization category, which honors advocates or organizations that have worked to create and pass federal legislation. And we’re thrilled to share that Hannah Yale won the 2021 RareVoice award for State Advocacy by a Teenager, which honors teens who have advocated for state or federal legislation. Congratulations, Hannah!

Hannah Yale has been an advocate for the EveryLife Foundation and the IPPF since 2017. She has attended Rare Disease Legislative Advocate’s (RDLA) Rare Disease Week on Capitol Hill annually since 2017, and she is also a member of the Young Adult Representatives of RDLA. In 2020, Hannah served on the Funding Committee for Living in the Light’s “I Stay Home for Rare” Emergency COVID-19 Relief Fund.

Hannah is living with Ehlers-Danlos Syndrome, although she began her rare disease advocacy to support her father, Marc, and her mother (who also has a rare disorder). Hannah is currently a student at St. Mary’s College of Maryland, where she is majoring in public policy and minoring in English and philosophy.

About the EveryLife Foundation for Rare Diseases

The EveryLife Foundation for Rare Diseases is a 501(c)(3) nonprofit, nonpartisan organization dedicated to empowering the rare disease patient community to advocate for impactful, science-driven legislation and policy that advances the equitable development of and access to lifesaving diagnoses, treatments, and cures.

Anna Lane is the IPPF Communications and Marketing Manager. She lives in Denver, CO, with her family.
Launched in March 2017, the IPPF Natural History Study is a patient registry sponsored by the National Organization for Rare Disorders (NORD) and the US Food and Drug Administration (FDA). This online data system collects, stores, and retrieves patient data for analysis in research studies. With the increase in attention from pharmaceutical and insurance companies on rare diseases like pemphigus and pemphigoid (P/P), there is bound to be a need for accurately defining the disease profile, characteristics, and outcomes. Therefore, a natural history study is an important asset for the entire IPPF community, including patients, caregivers, clinicians, researchers, and industry partners.

Natural history studies have traditionally been used by clinical researchers to understand disease progression in poorly characterized diseases. Now, studies like these are being used in a variety of ways to facilitate clinical drug development and can help accelerate therapies for P/P patients. These real-world, patient-focused studies can be incorporated into the early clinical development planning process, accelerate drug development, improve regulatory approval and reimbursement policies, and bring new life-changing therapies to patients in need of an improved quality of life. For example, a natural history study may be designed to help researchers and pharmaceutical companies understand P/P better through patient-reported data in their natural environment. A natural history study can monitor disease progression over time through stages such as resolution, maintenance, or remission.
Natural history studies can inform stakeholders involved in the clinical product development process by:

- providing better insights for researchers about disease characteristics, their small patient populations, and disease subtype etiology
- identifying the most important and relevant endpoints for patients
- helping to determine when follow-up may be needed
- improving patient selection and eligibility for clinical trials
- creating a historical comparator in case of single arm clinical trials.

Natural history studies also help patients, clinicians, and payers manage proper care for patients who are most likely to benefit from therapies by:

- assessing disease and the burden of the disease in a real-world setting, helping to inform clinical practice and standard of care
- determining which subtypes of a disease have increased burden
- detecting the subpopulations of patients who are more or less likely to respond to current therapies
- finding patient subpopulations that are likely to have the greatest benefit versus risk with new therapies.

According to the FDA, “Although knowledge of a disease's natural history can benefit drug development for many disorders and conditions, natural history information is usually not available or is incomplete for most rare diseases; therefore, natural history information is particularly needed for these diseases.”

Ultimately, the number of patients who enroll in a natural history study can decide the course of progress in the disease. Often, we may ask ourselves, “Why don’t more doctors know about this disease?” or “Why does it take so long to get a diagnosis?” To create more awareness around specific diseases and reduce diagnostic delays, we must first understand those diseases. A natural history study can do this and improve the lives of all those affected by pemphigus and pemphigoid. For more information about how to join the IPPF Natural History Study, visit www.pemphigus.org.

Marc Yale was diagnosed in 2007 with cicatricial pemphigoid. In 2008 he joined the IPPF as a peer health coach. He became 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.
Our Spotlight section features a medical professional whose work regularly impacts the lives of pemphigus and pemphigoid patients. Get to know a new physician, researcher, or other medical professional who knows these diseases best. This issue, we’re featuring Dr. Annette Czernik.

Annette Czernik, MD, FAAD, is the medical director of DermMedical in Wilton, CT. She is also an associate clinical professor of dermatology at Mount Sinai Dermatology Faculty Practice and assistant clinical professor of dermatology at Yale New Haven Hospital. The focus of her practice and research over the last 10 years has been investigating optimal treatments for patients with recalcitrant autoimmune blistering disease.

Dr. Czernik is the recipient of several notable research awards and has authored numerous peer-reviewed publications and book chapters relating to her work in pemphigus, pemphigoid, and other autoimmune conditions. She has spoken at several national and international meetings and is a supporter of the International Pemphigus & Pemphigoid Foundation. Currently she is the principal investigator on several pemphigus and pemphigoid clinical trials and routinely mentors physician assistant students and residents.

How did you become interested in pemphigus and pemphigoid (P/P)?
When I was a medical student, I approached Dr. Jean-Claude Bystryn at New York University (NYU)’s Tisch Hospital to hire me as a research associate for one year. During this year, I had planned to gain clinical and research experience to solidify my desire to pursue a dermatology residency. The position to work with Dr. Bystryn was competitive and required both a telephone and an in-person interview.

The in-person interview took place during a late Friday afternoon in July. Dr. Bystryn sat across from me in his office. He leaned back in his chair and put his feet up on his desk, revealing his seersucker pants (I assumed he was about to set off for the Hamptons on Eastern Long Island). He said, “I have no money in melanoma. I only have money in pemphigus.” I had no real idea what pemphigus was, but I assured him that I was excited to work in this area and had read all of his journal articles on the topic.

That was the beginning of a long journey of learning about immunobullous diseases from Jean-Claude Bystryn and Sergei Grando. Initially, I studied how the experts treated the disease. Today, 15 years later, I’ve become an expert and specialist in my own right. Now I train medical students and dermatology and rheumatology residents on how to best approach these diseases.

What is one thing you’d want all patients to know early on in their journey with P/P?
That they will be okay. We are able to get over 90 percent of patients into complete remission with pemphigus, and we are working on better treatments for pemphigoid. There are a lot of resources online that
can be beneficial in understanding these diseases, but also some that require caution. I support reaching out to the IPPF and accessing their countless educational resources. Some patients will read online that there is a high mortality rate, making them understandably anxious about these diseases and their treatments. A lot of patients are young and healthy and have never dealt with a medical problem before. Having an immunobullous disease can be overwhelming.

I believe that telling patients that they will be okay tends to reduce their anxiety and helps to build trust. Once they begin to trust us, they can let go. This helps us guide their treatment. We find it helpful to remind patients that the disease did not develop overnight, and we cannot clear patients overnight. The average length of time to remission is two years. It is a marathon, not a sprint. With proper treatment and follow up, the vast majority of patients are comfortably in remission, in time.

**What can patients do to better advocate for themselves?**
Communication. The IPPF has great resources for patients on how to advocate. I rarely have a pemphigus or pemphigoid patient these days that aren’t masters of self-advocacy! Here are some tips:

- Make lists when you visit your doctor so you don’t forget questions. And don’t be afraid to ask for clarification if you don’t fully understand an explanation.
- Ask your doctor the best way to contact their office (patient portal, email, phone).
- Come to the visit with an open mind and not fixed on any one treatment option.

**What is one fun fact about yourself?**
My husband and I recently found ourselves as the owners of a neglected periwinkle flower “garden.” It had been abandoned for many years. It was not recognizable. At some point, someone had sprayed weed killer everywhere, and there were shriveled dead roots, lots of poison ivy, and weeds over three feet high. There were five or six small, visible roots of periwinkle left on the land.

Prior to this summer, I hadn’t done very much yardwork in my life, except for reluctantly mowing the lawn as a kid. For some reason, this garden has become my personal project. As if resuscitating these abandoned periwinkles has some metaphorical meaning. When I removed as many weeds as possible, we began to see a few flowers bloom. Seeing the little purple flowers come up felt like a direct “thank you” for our work. The status of this garden is still questionable, but in the last two months the periwinkles have made remarkable progress. I am hopeful that by next summer, we will be to able to see full periwinkle groundcover.
My Experience with Pemphigoid Gestationis

Ashton Brown

When I got pregnant with my first and only child in 2010, I didn’t know what I was about to go through. Of course, I had all of the normal new-mom worries, but everything changed around the 26th week. I was diagnosed with pemphigoid gestationis (PG), a rare autoimmune bullous dermatosis of pregnancy. I’ve been told that this form of pemphigoid is rare, even among a family of rare diseases.

My symptoms started with terrible itching on my palms and feet. I got tiny little bumps after scratching my palms, but still didn’t think anything of it. I have had sensitive skin ever since I was a child, so I assumed it was something minor. However, when I got in bed at night and my legs itched so badly that I couldn’t sleep, I knew something was wrong. It felt like my skin was on fire. I would scratch and scratch and not get any relief. One night, I noticed I had little, red bumps all over my legs, and I decided to go to the emergency room since it was so late. I was told that I had scabies. They gave me a printout that explained scabies and, after reading it, I knew that wasn’t what I had. I still followed the treatment for scabies because I didn’t know how to tell a doctor they were wrong.

As expected, my skin got worse. My whole body itched now, and blisters appeared on my wrist. I was panicked and in so much pain. I went to my family doctor, and she told me that I needed to see a dermatologist as soon as possible. She tried to get me an appointment immediately, but I couldn’t be seen for two weeks. She didn’t know what I had, but she knew I couldn’t wait that long. She told me to go to the emergency room in the next county over because they had
an on-site dermatology office that could hopefully get me in sooner. I didn’t know what to expect.

When I arrived at the emergency room, I was seen very quickly. The first doctor immediately told me that he also had no idea what was happening to me. I started to feel discouraged. He told me that he was going to make some phone calls and he would return. It was two hours before I saw him again, but this amazing man spoke with the dermatologist, explained my symptoms, and got me a dermatology appointment for the next morning. He had been on the phone the entire time I was waiting. He stressed the importance of going to that appointment. I could never thank that doctor enough because he led me on the path to getting answers.

I showed up early to my appointment the next morning. The dermatologist immediately told me that he thought I had PG. He performed a biopsy on a blister from my arm and sent it for testing. He told me that PG is very rare and that he’d only ever seen one other case in his 12-year career. But that was how he knew exactly what I had. My OB/GYN had been in the field for 30 years, and I was his first PG case. The biopsy came back and confirmed that the dermatologist was right: I had PG.

Over the next couple of weeks, the blisters continued to spread until they covered 90 percent of my body. I couldn’t sleep because I would scratch in my sleep and the blisters would open and become infected. I couldn’t wear clothes because my blisters would weep, and my clothes would stick to them. When I’d take my clothes off, my skin would come off with them. I was miserable. I think anyone with similar skin issues likely knows the struggle of taking high doses of prednisone—at one point I was taking 100 mg of prednisone a day.

I was hospitalized on and off over the next two months of my pregnancy. When I was in the hospital, I was asked if it was okay for medical students to observe my skin. I always agreed because I knew that it was unlikely that they would see a PG patient again. But I wanted them to know it was a possibility. If they ever had another patient in the future with similar symptoms, I wanted them to remember me.

I had my son early at 34 weeks. I was put to sleep for the delivery—after two epidural attempts, the doctors determined that my pain receptors were blocked by my PG. Since I could feel everything and they needed to do a cesarean, that was my best option. They told me that after they delivered my son, my skin instantly changed colors. It went from red/pink to purple/tan. My son had a few blisters on his skin when he was born, but they cleared up quickly and he wasn’t bothered by them. He’s now 11 years old and as healthy as can be!

A few months ago, I lost my dream job. I was more stressed than ever. I started getting a small rash on my neck, and it spread slowly every day. It was itchy and painful, and it brought back a lot of feelings I had during my pregnancy. I thought I might be pregnant again. When I had PG in 2010, I hadn’t met my husband yet, so he didn’t know how bad my symptoms had been. I’ve tried to tell him about my experience.
with PG, but I think it’s hard to understand the severity of it unless you can physically see it. I really didn’t take any photos during my pregnancy since I couldn’t focus on anything other than the pain. So finally, after a decade of not seeing any photos of myself with active PG, I requested my medical records from my dermatologist. It took a few weeks for them to arrive, but when they did, I almost couldn’t believe it was me in the photos. My skin looked like something from the set of *The Walking Dead*. When I saw the photos, I knew I had to do something to raise awareness of PG because someone out there might be feeling the same way I felt.

I started using TikTok in February 2021. (I was late to the bandwagon because I thought it was just for dancing teenagers.) I started posting funny videos and after just a couple of months, I had thousands of followers. When I received my medical records and decided to raise awareness of PG, I used TikTok as my platform. I made a video that showed a photo of my legs with PG, and it went viral. It received nearly 3 million views, though that wasn’t even the best part about it. Since the video went viral, I was able to help at least three or four women get a potential PG diagnosis. I felt like I’d made a difference for those women, and that’s all I wanted.

A couple of days after the video went viral, a media company reached out to me and asked if I’d be willing to let them publish my story. They published several online articles, as well as some magazine articles, mostly in the UK. It was because of these articles that I found out about the IPPF. I received a message request from a man I didn’t know on Facebook. He told me he’d read my story and that his wife had PG with their first child. He said that women in the future might not have to struggle as much because they could find me and my videos. He let me know that he wished they had been able to access a resource like my video during her pregnancy. He also told me about the IPPF’s annual Patient Education Conference, and he said they rarely have PG speakers. Knowing that I wanted to continue to help people, I reached out directly to the IPPF and asked if they needed any help. Because of that, I was able to share my story with PG at the 2021 Virtual Patient Education Conference!

Even with all of my struggles with PG, I’m one of the lucky ones, though I didn’t realize this for many years. I still get flare-ups on my skin when I get my period, or if I experience elevated stress levels. I started looking for more answers, and I found a group of women on Facebook from all over the world who were experiencing the same thing. When I found this group, I felt overwhelmed with emotion because I had felt alone and like no one understood. But there’s a group of hundreds of beautiful women who know exactly how I feel! I learned that so many women were never even properly diagnosed. So many of them didn’t get the option for treatment, had multiple pregnancies, and experienced PG symptoms multiple times. Feeling alone with a disease of any kind is something that no one should experience. This is why raising awareness about PG is so important. Even if there are only a couple of women each year who reach out, we can at least give them comfort in knowing that they are not alone. That is so powerful!

Pemphigoid gestationis needs to be talked about more. It’s important to know that this condition is triggered by pregnancy, but it never really goes away. Women that experience PG must know: you are not alone.

For more information about PG, visit the National Institutes of Health Genetic and Rare Diseases Information Center: https://rarediseases.info.nih.gov/diseases/6497/gestational-pemphigoid.

Ashton Brown lives in Morganfield, Kentucky, with her husband and 11-year-old son. She uses her social media platform to raise awareness about PG.

Find other stories and the latest info on the IPPF news site: pempress.com
As part of the reauthorization of the Prescription Drug User Fee Act (PDUFA V) in 2012, the US Food and Drug Administration (FDA) launched a program to help ensure that patients’ experiences, needs, perspectives, and priorities were considered and meaningfully integrated into the development and review processes of drugs. This approach is now known as Patient-Focused Drug Development (PFDD), an innovative way to collect input from patients who are willing to share their personal experiences of living with a disease or condition.

The initiative also provides the opportunity for patient advocacy organizations like the IPPF to hold PFDD meetings. These meetings can be externally led by the organization with the option of inviting key stakeholders to attend as well. The Externally-led Patient-Focused Drug Development (EL-PFDD) meetings allow the FDA and pharmaceutical companies to hear directly from patients, caregivers, and health care professionals about their key symptoms, the impact the disease has on patients’ daily lives, and patients’ experiences with currently available treatments.

At the conclusion of these meetings, organizations provide a detailed summary of the patient input collected during the EL-PFDD session and summarize it into a “Voice of the Patient” report. These reports are then made public, which helps to create more disease awareness. These reports also show the FDA and the pharmaceutical industry the specific ways in which new therapies could improve patients’ daily lives.

The FDA now encourages pharmaceutical companies to look for opportunities to incorporate the patient perspective into the research and development processes of new therapies. Many of these companies are increasing their efforts to engage with patients and patient advocacy groups. Ideally, this results in patient opinions regarding potential new ideas, processes, and services being incorporated into drug development methods.

Some of the IPPF’s pharmaceutical partners have sought out the patient perspectives through patient advisory board meetings, meetings with researchers, and surveys. Understanding the IPPF community’s needs can help inform potential protocols and locations for clinical trials.

In November 2021, the IPPF sent a Letter of Request to the FDA asking to hold an EL-PFDD meeting in 2022. We believe that members of our community are willing to share their experiences to advance medical research and foster a greater understanding of pemphigus and pemphigoid. As a result of this request, we’re excited to announce that the FDA’s Center for Drug Evaluation and Research, Division of Dermatology and Dentistry, and the Center for Biologics Evaluation and Research has invited the IPPF to hold an Externally-led Patient-Focused Drug Development meeting on P/P. The FDA believes that it will be of great value and interest to the patient community, patient advocates, researchers, drug developers, and the FDA. By prioritizing this meeting, the FDA and drug developers will discover that P/P patients are individuals with unique disease burdens. Ultimately, we hope this meeting will continue our effort to improve patient outcomes, accelerate diagnosis times, and encourage drug development and treatments that better the lives of all those affected by P/P.

Marc Yale was diagnosed in 2007 with cicatricial pemphigoid. In 2008 he joined the IPPF as a peer health coach. He became 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.
In close cooperation with Drs. Ralf Ludwig and Katja Bieber of the Lübeck Institute for Experimental Dermatology at the University of Lübeck, Germany, the IPPF Scientific Seminar Series is a scientific educational program with experts from various international institutions who present their scientific findings on pemphigus and pemphigoid. The content highlights recently published and/or unpublished data in both basic and translational research. IPPF Research and Advocacy Coordinator Marc Yale and Dr. Ludwig recently discussed the new series.

Marc Yale (MY): Dr. Ludwig, can you please share the impetus that motivated you and Dr. Bieber to reach out to the IPPF about starting the Scientific Seminar Series?

Dr. Ralf Ludwig (RL): Many years ago, we became aware that the IPPF had organized a scientific meeting on pemphigus and pemphigoid (P/P) in 2010 in Bethesda, Maryland. Since then, we met with the IPPF, and you personally, at scientific conferences organized by the IPPF. In 2017, we were very delighted to host the Scientific Conference of the IPPF in Lübeck.

With the travel limitations imposed by the COVID-19 pandemic in 2020, we missed the interactions and the exchange of ideas and concepts with clinicians, researchers, and patients that we so much enjoyed at these meetings. We thus reached out to the IPPF to help bring that community back together—at least virtually—to share and discuss the latest on P/P.

Marc Yale (MY): Why do you feel that the IPPF Scientific Seminar Series is unique? What type of insights should attendees hope to gain by attending?

Dr. Ralf Ludwig (RL): So far, the IPPF has hosted five seminars, all of which have attracted clinicians, researchers, and patients. The presentations have focused on recently published, or even unpublished data, often with a direct impact on clinical practice. For example, the presentations of Dr. Kridin and Dr. Kasperkiewicz on vaccination and the risk of COVID-infections.

With this format, we have the unique opportunity to address timely topics at an early stage because the program is only scheduled a few months out. Hence, the attendees can expect to hear the latest developments in basic, translational, and clinical research in P/P. This stimulates a vivid discussion among all participants of these webinars. By the way, your moderation of the discussion, as well as the event overall, is superb!

Marc Yale (MY): Where do you feel like the biggest gaps are currently in the research of autoimmune bullous diseases, and how can the IPPF community help address them?

Dr. Ralf Ludwig (RL): Overall, I think that research is going great in P/P, as exemplified by the emergence of novel treatment options, such as FcRn- or BTK-inhibitors, as well as the use of the CAAR T cell technology to deplete autoreactive B cells in pemphigus. The major pitfall, I think, is the lack of patient data, including applied treatments and responses, as well as access to well-characterized patient materials. I think the IPPF could be of great help to coordinate both.

Marc Yale (MY): Why are diseases like P/P ideal models for the study of other more common diseases?

Dr. Ralf Ludwig (RL): Pemphigus and pemphigoid are prototypical autoantibody mediated diseases, where disease manifestation is clearly linked to the presence of the known specific autoantibodies. Hence, new therapeutics, such as FcRn inhibitors and CAAR T cells, have mainly emerged in pemphigus. These offer the potential to be translated into use in other diseases with a similar pathogenesis.
**MY:** In your opinion, how can research be accelerated to reduce the diagnostic delay and treatment burdens for people living with these diseases?

**RL:** I think research is mainly doing fine with regard to diagnostics. The diagnostic delay is, in my opinion, due to a lack of awareness of P/P among medical practitioners. In order to promote disease awareness, the P/P community needs to reach out to medical professionals outside dermatology.

Regarding the burden of disease, I think we need better treatment options. Again, in my personal opinion, the best way to accelerate research in this field is through collaborations among academics, as well as jointly with industry partners. One major pitfall is funding for research, especially for larger consortia focusing on P/P. If significant funding could be attracted to P/P research, I expect that improved treatment options will emerge much quicker. One such possibility would be proof-of-concept clinical trials using repurposed drugs—those that are licensed for other indications, but where data indicates a potential benefit for P/P patients.

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Ralf Ludwig obtained his medical degree at the Johann Wolfgang Goethe University (Frankfurt/Main, Germany). He received his training as a dermatologist at the Department of Dermatology at the University of Lübeck, where he became a member of the board of directors at the Lübeck Institute for Experimental Dermatology in 2014.

Dr. Ludwig’s main research interest is the modulation of chronic cutaneous inflammation. In order to unravel the pathogenies of these complex diseases, he mainly employs in vitro model systems and pre-clinical animal models. Translation into patient care is achieved by close cooperation with the Department of Dermatology and the Comprehensive Center of Inflammation Medicine at Lübeck.

Dr. Ludwig currently investigates pathogenesis of autoimmune skin blistering diseases in order to improve the so-far unsatisfactory treatment of patients.
I was diagnosed with pemphigus vulgaris (PV) after only one visit to my primary care office and a subsequent referral to a dermatologist. Starting in the fall of 2017, I experienced lesions (scabs) on my scalp that would not heal. I finally went to my primary care doctor early in April 2018 and was referred to a dermatologist soon after.

My first biopsy was for fungus and psoriasis, but after those results were negative, I mentioned to my dermatologist that I had some sores in my mouth. She quickly performed an immunofluorescence biopsy, and it was positive for PV. By the end of April, I was seeing a specialist in my area and had started treatment. This was before rituximab had been approved by the FDA as a first-line treatment, so I started with prednisone, mycophenolate, and doxycycline. As of the fall of 2020, I have only been taking a maintenance dose of mycophenolate and doxycycline.

I found the IPPF through my own web search in 2018. I became a participant in the Natural History Study and connected with other patients on social media. I also attended my first South Florida Support Group meeting in the summer of 2018. It was a very welcoming group. I still have a magnet I received at that first meeting that I look at every day. It says, “DIFFICULT IS NOT IMPOSSIBLE.” I have continued to participate in this support group and have made friends who are living with this disease. The South Florida Support Group has also provided me with information about how to cope with PV, participating in clinical trials, and other materials and resources available through the IPPF.

Another way I’ve gotten involved with the IPPF is by becoming an Awareness Ambassador. Through this program, I distributed information to my dentist’s office, as well as several other dentists’ offices in South Florida. Recently, when I was able to go back into my dentist’s office for a cleaning, I found out that the

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hygienist I was used to seeing was not coming into the office due to her existing health conditions and risk factors for COVID-19. I turned to the IPPF again to make sure I could educate my new hygienist about how to clean my teeth differently because of my disease.

In October 2019, I attended the IPPF Patient Education Conference in Philadelphia. It was a fabulous experience! I was in the presence of the most experienced researchers, doctors, patients, and many others who shared their knowledge through various presentations. Attending the conference gave me the opportunity to be a part of something, and I am thrilled to be involved. I am now an IPPF Healing Hero, continue to be an Awareness Ambassador, and have taken an active role within the South Florida Support Group. I was also able to attend the last two Virtual Patient Education Conferences.

Another IPPF resource I use is the Quarterly, which keeps me aware of treatment options and their efficacy. It helps me connect with other patients’ stories and allows me to continue to grow as an advocate for PV and other rare diseases.

Because of the IPPF, I learned about the National Organization for Rare Disorders (NORD) and attended their virtual conference in July 2020. Additionally, I have become an active patient advocate on Capitol Hill. By attending virtual meetings, I have been able to help representatives in Congress, including my state senators, hear a rare patient’s perspective on important bills. This advocacy work has not only been very meaningful to me as I get to know my representatives, but also for me to understand the legislative process. Most recently, I participated on a panel through the IPPF Patient Education Series in January that focused on Rare Disease Week on Capitol Hill.

I am grateful that the IPPF has been there for me as I needed a safe place to gather information to better understand this rare disease. Though I am not in remission, my disease has always been mild. If I have a flare in the future, I will know what treatments are available. My life today is enhanced because I have been able to use the IPPF resources that are available to patients like me.

Mindy Zimmerman was diagnosed with pemphigus vulgaris in 2018. She lives in South Florida.
Support Groups

Many P/P support groups are meeting virtually during the COVID-19 pandemic. Learn more about local groups at pemphigus.org