Answers to your questions:
COVID-19, PEMPHIGUS, & PEMPHIGOID

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As I write my message for the 100th issue of the Quarterly, I am aware of the challenges we have all faced over the past few months. Although our lives have changed significantly due to the COVID-19 pandemic, there is one constant that I know remains: The IPPF is committed to improving the lives of all those affected by pemphigus and pemphigoid (P/P).

We know that many in the IPPF community are concerned about the spread of COVID-19, and we hear you. As we continue to learn more about the health implications of the pandemic, the IPPF will continue to provide valuable resources needed to manage your disease and life. Our staff is committed to providing you support through our peer health coach program, exploring the research of new treatment options, advocating on behalf of our community, and accelerating diagnostic times.

What you may not realize is that we accomplish all of this with just four full-time and six part-time employees. Though our commitment is international in scope, the IPPF operates as a small nonprofit organization that has helped thousands of people over the past 25 years.

In this 100th issue, you will see many facets of our work on your behalf. We count on volunteer support from advocates, patients, researchers, and students to share their stories and move our initiatives forward. Their commitment, along with your support, allows us to continue to spread awareness of P/P, advocate for improved therapies, and continue to help us search for a cure.

As we move forward during these uncertain times, the IPPF remains vigilant in achieving our mission. Remember, if you need us, we are here for you. Our community is strong, and together there is hope.

Marc Yale
IPPF Executive Director and MMP Patient
marc@pemphigus.org
**Principia Biopharma** is a late-stage biopharma-aceutical company that develops next-generation therapies with the potential to transform the treatment of patients who have immune-mediated diseases. I interviewed Dolca Thomas, MD, Chief Medical Officer at Principia, about patient participation in clinical trials and new drug development.

**What is Principia’s mission?**

*Dolca Thomas, MD (DT):* Our mission is to develop and bring novel therapies to patients with serious immune-mediated diseases by changing the way small molecules are developed. What I mean by that is bring therapies that are safe, improve quality of life, and take patients as close as possible to living a life free of disease.

**There are very large biopharma companies out there. What drew you to this company in particular?**

*DT:* I’ve been at many big companies and one thing that is hard to accomplish in a large biopharma is getting a high level of connectivity to patients and the science as you advance in your career. What drew me to Principia Biopharma was the emphasis that the company puts on people, both internally and externally, especially patients. Bonding matters at Principia. Bonding is important for how our drugs engage the target, for how we engage internally in the company, and how we connect with investigators, patients, scientists, and regulatory agencies. Developing and cultivating these bonds are important to achieve our mission.
What made Principia want to develop new treatments for rare diseases?

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DT: Focusing on rare disease can be challenging given the costs involved in developing a drug. Rare diseases may get neglected due to their small patient population and the challenges associated with conducting clinical trials in these patients. As a small company, we can be nimble in our decision-making, which has resulted in us focusing on indications where we believe we can really make a difference. We can work closely with physicians and investigators to leverage our strengths and to effectively make a big impact on the lives of patients with pemphigus who have been overlooked.

How important is patient input/feedback in Principia’s work on drug development?

 DT: Patient input and feedback is critical. Without patients we cannot conduct trials. It is our obligation to facilitate patient participation and share the value and impact they are making. The more biotech companies simplify and integrate patients in the clinical trials process, the more successful we are in understanding what the patients need and developing therapies that are meaningful to their health. Engagement of patient advocates is also a critical piece for the successful execution of clinical trials.

Why are patients important for clinical trials?

 DT: Patients are the link that brings a clinical trial together, and without them you can’t conduct studies that ultimately can change how doctors and clinicians treat patients, especially rare diseases. There are several benefits for patients who participate in clinical trials. Studies have shown that participants in clinical trials are more likely to receive more extensive care and follow-up from health care providers than patients who do not participate in clinical trials. And there is a “paying it forward” element to clinical trials that shouldn’t be overlooked. In addition, participation in clinical trials supports the development and access of new treatment options for patients in the future.

Why should patients enroll in the PEGASUS clinical trial?

 DT: Rilzabrutinib’s (formerly known as PRN-1008) mechanism of action is unique in that it has the potential to reversibly inhibit many different immune cells without broad immunosuppression. There is a potential advantage for patients when you can control the autoimmune destructive process associated with pemphigus without increasing the risk for infections, and with minimal or no corticosteroids use. The goal of the PEGASUS trial is to evaluate the ability of rilzabrutinib to achieve complete remission (clear skin) while minimizing side effects associated with steroids.

What makes rilzabrutinib different than other treatments currently on the market?

 DT: Pemphigus patients currently struggle with having to take high doses of corticosteroids to keep their disease at bay. There is considerable need for an oral, corticosteroid-sparing, fast acting therapy that reduces autoantibody and immune cells that trigger tissue inflammation and skin manifestations in patients.

Why did Principia Biopharma choose to work on pemphigus vulgaris (PV) and pemphigus foliaceus (PF)?

 DT: Rilzabrutinib’s unique mechanism of action is well poised to address the immune cells that are involved in disease manifestations of pemphigus. Patients with PV and PF typically are prescribed high doses of steroids, which often lead to a poor quality of life and many side effects. Rilzabrutinib has the potential to limit or eliminate the use of steroids for PV and PF long term. We believe that rilzabrutinib has the potential to be a true benefit to patients.

What is a BTK inhibitor and how does it work for pemphigus?

 DT: Bruton tyrosine kinase (BTK) is a protein that is inside many immune cell types, including B cells, macrophages and neutrophils. BTK signaling leads to activation of immune pathways associated with disease manifestation of pemphigus. Inhibition of BTK has the potential to reduce and eliminate the inflammation and disease manifestations associated with pemphigus.

What is your position and your role in working with the pemphigus clinical trial?

 DT: I serve as the Chief Medical Officer at Principia Biopharma, where my role is to advance multiple therapies in our product pipeline. With rilzabrutinib as our lead product candidate, my focus is to work closely
with our experienced clinical team, our clinical investigators, study coordinators, patient groups and key professional organizations to ensure we have the right resources and strategies that will ultimately benefit pemphigus patients.

**Is rilzabrutinib in the PEGASUS trial designed to be steroid-sparing, or will it eliminate the need for steroids altogether?**

*DT:* The PEGASUS trial has several parts. The first part of the trial (0-37 weeks) is designed to reduce steroids to minimal levels. The goal is to have patients achieve sustained complete remission (clear skin) for at least 8 weeks while on only 5 mg of prednisone daily. The second portion of the study (37 to 68 weeks), is designed to allow participants to be on no steroids and sustain complete remission. Additionally, after week 37, patients who had been receiving the placebo drug will be allowed to receive rilzabrutinib therapy.

**Was there a Phase 2 study done on the drug, and did the trial meet all of its objectives and endpoints?**

*DT:* We did conduct a Phase 2 trial where we evaluated control of disease activity and complete remission. The Phase 2 study met its primary objective and informed what is the optimal dose for controlling the disease and for achieving complete remission. While we are encouraged by our Phase 2 trial results, we hope our Phase 3 will provide the evidence that demonstrates efficacy and safety of the drug and paves the way for potential approval.

**How is rilzabrutinib administered to patients, and how often would patients need to take it (daily, weekly, etc.)?**

*DT:* Rilzabrutinib is taken orally twice a day, every day.

**What impact does Principia Biopharma feel this therapy will have on patients’ lives?**

*DT:* Our goal is that this will be a new therapy modality where patients can have greater control of their disease with the least amount of burden on their quality of life.

**How can the IPPF community help further in the drug development process?**

*DT:* The patient community is what makes the drug development process possible. We need the support and participation of patients for our clinical trial program to be successful, and much of that support comes from the IPPF community. We encourage all of you to learn more about our clinical trials by visiting our website: [www.principiabio.com/clinical-study/pegasus-study/](http://www.principiabio.com/clinical-study/pegasus-study/).

For more information about clinical trials, visit [www.pemphigus.org/research/clinical-trials](http://www.pemphigus.org/research/clinical-trials).

Marc Yale was diagnosed in 2007 with cicatricial pemphigoid. In 2008, he joined the IPPF as a peer health coach and was promoted to executive director in 2016. Marc currently resides in Ventura, California, with his wife Beth and daughter Hannah.

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**Find a Doctor: IPPF Physician Map**

The IPPF's **Find a Doctor** P/P physician map provides patients with contact information for medical and dental professionals familiar with P/P.

Access the map online at: [www.pemphigus.org/find-a-doctor](http://www.pemphigus.org/find-a-doctor)
In February, the IPPF participated in a workshop for patient organization leaders sponsored by the Alliance for Regenerative Medicine. The workshop focused on educational resources for cell and gene medicine. There are still many things we need to learn about pemphigus and pemphigoid (P/P), and the IPPF strives to be at the forefront of emerging therapies. Although the idea of this technology is very new to P/P, cell and gene medicine has the potential to dramatically improve our health and quality of life.

Prescription medications for P/P are typically used to manage disease, mitigate symptoms, and relieve pain. The idea behind cell and gene therapy is to target the exact cause of the disease so people with P/P no longer have recurring symptoms, ideally after a single treatment.

Many diseases or debilitating health conditions are caused by one or more genetic changes that occur inside the body. These diseases or conditions are caused by defective genetic code and have few treatment options. Today’s conventional therapies often treat the symptoms of the disease or slow down the disease. Physicians use cell and gene medicine to try to resolve the underlying genetic defect.

Potential Impact on P/P

Cell therapy is when doctors inject viable cells into a patient’s body to grow, replace, or repair damaged tissue to treat a disease. CAR T-cell therapy, which is a recent innovation, removes special immune system cells from blood and changes the cells so they recognize, attack, and kill diseased cells.

Gene therapy is when doctors put healthy genetic code into a patient’s body with the goal of preventing disease, reducing further damage and pain, and even potentially curing the patient. If a mutated gene causes an important cell-building protein to function poorly, gene therapy may be able to restore the function of the protein. Currently there are no gene therapies for P/P; however, the IPPF will continue to investigate and support studies that lead to a better understanding of these types of therapies to determine if a cure can be reached.

Within the field of cell and gene medicine, the study and application of regenerative medicine uses a combination of both tissue engineering and molecular biology. This new field brings together many experts from multiple fields to find solutions to some of the most challenging health issues in medicine. Regenerative medicine has the potential to restore, maintain, improve, or replace damaged tissue.

The IPPF plans to continue collaborating with the Alliance for Regenerative Medicine and scientists to advance our understanding of these new technologies and to educate all those affected by P/P. This is an exciting time in medicine and for the IPPF community. The more we can learn about the cause of our diseases and the mechanisms that activate them, the sooner that knowledge will help us find a cure.

Marc Yale was diagnosed in 2007 with cicatricial pemphigoid. In 2008, he joined the IPPF as a peer health coach and was promoted to executive director in 2016. Marc currently resides in Ventura, California, with his wife Beth and daughter Hannah.
Ensuring Lasting Smiles Act

The Importance of Advocacy

Becky M. Abbott, MPH

Far too often, patients experience issues in obtaining insurance coverage for medically necessary treatments and procedures. These coverage issues can often be ongoing and take months or years to determine whether or not a claim will be covered. The appeals and denials process can also be very frustrating and take a considerable amount of time to navigate. After experiencing issues with insurance coverage, many patients often reach out to their legislators to ask for help to resolve their issue. There are also patient advocates who travel to Capitol Hill to advocate for change and legislation that will help their communities.

These patient advocates play an important role and have the potential to influence decision making and help draft legislative policies on Capitol Hill. A little over four years ago—after experiencing years of frustration from going through the appeals and denials process several times and fighting for insurance coverage—my family joined other patient advocates on Capitol Hill. We traveled to Rare Disease Week in Washington, D.C., where my son Aidan, who has a rare disease called Ectodermal Dysplasias, shared his personal story. He explained our insurance struggles to obtain coverage to repair his congenital anomaly that requires complex dental and oral procedures. After Aidan shared his story, Senator Tammy Baldwin (D-WI) was inspired to write legislation, which Aidan had the privilege of naming: the Ensuring Lasting Smiles Act (ELSA).

ELSA (S.560/H.R.1379) would require all private insurance group and individual health care plans to provide coverage for any medically necessary services and/or treatments needed to repair any congenital anomaly. That coverage would include services and procedures for any missing or abnormal body part necessary to achieve normal body function, as well as dental and oral services related to the repair of the congenital anomaly.

Since its reintroduction on February 26, 2019, ELSA has garnered strong bipartisan support in both the U.S.
House of Representatives and the U.S. Senate. As of March 19, 2020, ELSA has 301 co-sponsors in the U.S. House of Representatives and 40 U.S. Senate co-sponsors. From the beginning, the National Foundation for Ectodermal Dysplasias (NFED) has been leading grass-roots efforts to raise awareness and educate members of Congress on the need for ELSA. This important, common-sense legislation has not only caught the attention of members of Congress but has also garnered the support of nearly 50 professional organizations and patient groups, including the IPPF. ELSA has the potential to help individuals born with all congenital anomalies.

The NFED, along with the other organizations supporting ELSA, continues to lead the efforts to see that this bill is passed by the end of the 116th Congress this year. ELSA has hit many important milestones along its advocacy journey. The Subcommittee on Health of the Committee on Energy and Commerce held a legislative hearing on Wednesday, January 8, 2020, and included ELSA on the hearing agenda. The Subcommittee on Health, Employment, Labor, and Pensions of the Education and Labor Committee held a hearing on January 28, 2020, and also included ELSA on the agenda. Just a few weeks ago on March 11, 2020, the Subcommittee on Health of the Committee on Energy and Commerce met in an open markup session and included ELSA in the markup session. It was voted through subcommittee and will now go to full committee markup.

This legislation is a true example of the power of patient advocacy and the reason why patients need to share their unique and powerful patient stories with their legislators. It only takes one advocate to raise awareness and start an initiative that has the ability to change lives across our country. While many of you may not be able to travel to Capitol Hill, you can advocate from home. Whether you are advocating for ELSA or another piece of important legislation that can help you and your community, it is important to share your story and speak from your heart. Your legislators need to be aware of your struggles and how they can help. In the upcoming weeks, take a few minutes to contact your legislators, share your story, and explain how they can help you and your family. Let’s raise our voices together and fight for change in our communities.

Becky M. Abbott, MPH, is the Manager of Treatment and Research for the National Foundation for Ectodermal Dysplasias & Co-Chair of the NFED Family-Driven Advocacy Committee. She lives in Wisconsin with her husband, Det. Thomas Abbott, and their two children, Aidan (who is affected by x-linked hypohidrotic ectodermal dysplasia) and Ryder.
Connecting at Rare Disease Week on Capitol Hill

Holley Wright

Though Rare Disease Week on Capitol Hill was only a few months ago, it seems like a world away when 12 IPPF rare disease advocates were together and able to hug, laugh, cry, meet wonderful new faces, and hear strong voices. Now that we are weeks into an ongoing isolation due to COVID-19, I am so grateful for that special time before so much closed.

IPPF patient-advocates traveled to Rare Disease Week from all corners of the US. When I arrived and entered my small hotel room, I felt alone. This was my first time attending Rare Disease Week, and I was in an unfamiliar city. Along with my excitement, I started to think about the “what ifs.” What if I couldn’t find the Reagan building where we were supposed to meet? What if I couldn’t find people from the IPPF? What if I didn’t have anything to say during the legislative meetings?

Of course, on the first day, bright and early, I found the building easily and located our familiar group. Marc Yale, the IPPF executive director, was ready with colorful IPPF t-shirts and folders full of the pertinent information we needed to be productive and well-informed. After a group photo, we joined advocates from other rare disease groups and divided into groups based on our home states or regions. The New Englanders were a mighty group, led by a gentleman whose young son suffered with a disease that would ultimately end his life as a young adult. As I sat with these fearless warriors, I listened to their stories. Each individual became dear to me. Their names, disappointments, and joys touched my heart, just as the IPPF community does. We were all in this dance of heartbreak and hope together. It’s a journey we never wanted to take, with pains and struggles we weren’t prepared for.

Each state group then traveled together and met with legislative representatives or staff members to advocate for various rare disease legislation. The congressional office staff members were extremely friendly
and amenable to our concerns. By the end of the first meeting, we fell into an easy rhythm and developed a cohesive message.

It was gratifying to know that we contributed to the democratic process with our solitary voice magnified by the creation of this rare disease group. I no longer felt alone since I was standing strong with over 900 members of the same family. I never ran out of judicious asks for my Connecticut legislators, and my words did not fail me. I felt prepared for the meetings. The “what ifs” I originally feared vanished.

In this new world of COVID-19, we know that virus may affect us rare disease patients in particularly harsh ways. We not only need to advocate for our community more than ever, but we also need to keep in touch, listen, and support each other.

Keeping Fit at Home
Carolyn Fota

Keeping fit at home is one of the greatest challenges that a pemphigus and pemphigoid patient may face; either we are immunocompromised from medication, have intense medical care needs, or the weather (such as a hot summer day) keeps us inside. Additionally, unwanted side effects such as weight gain, increased blood sugar, and high blood pressure from medication can add further stress and frustration to the situation. One month after being diagnosed with bullous pemphigoid in February 2016, I had gained over 20 pounds due to steroids, and my dermatologist directed me to stay at home because of significant medical needs. Over time, I was able to lose weight, regain strength, flexibility, and endurance by adding fitness into my daily routine.

Stretching first thing in the morning helps me to wake up, shake off the early morning aches and pains, and feel more alert. Some of stretches I do in the morning include the overhead stretch, hamstring stretch, knee rolls, chest stretch, and squats.

After breakfast, I slip on a pair of sneakers, walk to the end of my driveway to pick up my newspaper, check the mailbox, and walk around my home (outside) a couple of times. If it’s raining outside, I walk inside my home and try to go up and down the stairs a couple of times, too.

By midday, I like to work my muscles and do 30 minutes of yoga followed by a few minutes of meditation. I find that yoga helps me to relax, build strength, develop flexibility, and improve my balance. Also, I find that I’m more focused after a few minutes of sitting quietly and meditating. The popular app Headspace has good information on starting a meditation practice: www.headspace.com.

We may not think that chores or little tasks around the house burn very many calories, but they really do. Tasks like sweeping, cleaning the garage, mopping, and vacuuming burn over 100 calories per hour for a 150-pound person.

I try to end my day on a positive note. I love to unwind, have fun, and play music! Some of the activities I do in the evening include dancing to my favorite tunes, taking the stairs, walking inside or outside my home, marching in place, arm curls with soup cans, push-ups against the wall, and cooldown stretches.

While doing these activities, I try to make sure that I'm wearing appropriate clothes and shoes and carrying water. If I leave my house, I keep my cell phone handy, too. I also like to record my activity because it helps me build self-confidence as I regain my strength, positive body image, and independence.

Carolyn E. Fota, MHR, lives in Stafford, VA, with her husband, Frank and their three crazy cats. Carolyn enjoys walking, yoga, church, and writing.
Patient Education at the Yankee Dental Congress

Ellen Levine

If someone had told me prior to my pemphigus diagnosis that I’d be speaking at dental schools and a dental congress for the International Pemphigus and Pemphigoid Foundation, I would’ve looked at them like they had two heads. Pemphi-what? Public speaking? Oh no, not me. I’d rather have a root canal. Well, 13 years later, here I am, a person all too familiar with the words “pemphigus vulgaris” and conquering my fear of public speaking as a patient educator with the IPPF.

My journey with pemphigus vulgaris (PV) began 13 years ago in the magical Land of Oz. Not Dorothy’s Oz, but Australia. Believe me though, I could relate to Dorothy’s quest in search of the Wizard who might help her get home as I searched for a specialist who could diagnose and treat my disease.

By 2006, I had taught English in China, worked for a global engineering company in San Francisco, and had just moved to Australia to start a new adventure. I prided myself on my adaptability and readiness to take on new challenges. But these attributes did not prepare me for what was to come. I was 45 and looked and felt remarkably healthy when painful lesions started to appear on my gums and inside my mouth. The blisters peeled off like layers of tissue paper and I began to lose weight quickly. I panicked because as much as I didn’t want to trust my instincts, I knew something was terribly wrong. I returned to the United States in search of a diagnosis. I then saw 14 specialists, was misdiagnosed, and given inappropriate treatments before finally finding a physician who would treat me. When my biopsy confirmed PV, I had no idea how radically my life would change. This new word, “pemphigus,” like the disease, was going to become a part of my everyday life.

My PV progressed quickly from my mouth to almost every part of my body. The first few years of treatment were my “trial-and-error-pill-popping phase,” as I tried to determine the most effective medication and appropriate dosages, while minimizing adverse side effects. After a port-a-cath implant, I had close to 150 IVIG and dozens of rituximab infusions. Oftentimes, the side effects of the medications were worse than the blisters that covered my body. I learned that the power of these medications was not to be taken lightly. I adjusted my diet, since certain foods triggered oral lesions. I also altered the way I bathed because the blisters on my body and scalp—comparable to second degree burns—could not withstand water pressure. Having gone in and out of remission over the last 13 years, I now know my mouth and my body and how I respond to medication. I’ve learned to manage my disease. When I feel a flare emerging, my physician and I partner in determining the best course of action.

Armed with this firsthand experience and accumulated knowledge of my disease, I started volunteering for the IPPF. I co-lead IPPF Boston Support Group meetings; am an awareness ambassador; serve as a patient educator, where I speak at dental schools in the New England region; and volunteer at the IPPF exhibit booth at the annual Yankee Dental Congress (YDC).
Every year, I look forward to participating in the YDC, New England’s largest meeting for dental professionals seeking continuing education, dental products, services, and resources. With nearly 25,000 dental professionals in attendance, YDC is the fourth largest dental meeting in the country. Held in Boston each January, YDC is sponsored by the Massachusetts Dental Society, in cooperation with the dental associations of Connecticut, Maine, New Hampshire, Rhode Island, and Vermont.

The IPPF’s participation offers a strategic opportunity to educate thousands of dental professionals about P/P, which most often present initially in the oral mucosa. I remember when the IPPF first launched its Awareness Campaign by targeting the dental community. I couldn’t think of a more appropriate and thoughtfully designed program as I reflected on my own experience of seeing dentist after periodontist, oral pathologist after oral surgeon, physician after specialist, in my search for a diagnosis and treatment plan.

This past January, not only did I work the IPPF booth on the YDC exhibit floor, but I also had the special opportunity to participate in a YDC dental continuing education course alongside Dr. Donna Culton, Associate Professor, Department of Dermatology, University of North Carolina, Chapel Hill, and Dr. Joel Laudenbach, Associate Professor, Department of Oral Medicine, Atrium Health, titled “Know-It-When-You-See-It: Diagnosis and Initial Treatment of Oral Pemphigus Vulgaris.” We spoke to an audience of approximately 60 dental professionals and provided the dermatologist, oral medicine specialist, and patient perspectives. My presentation, which focused on the patient’s journey to a diagnosis, was between the two medical professionals’ presentations. While Drs. Culton and Laudenbach provided important scientific and clinical information about the disease, I gave my personal perspective of living with this rare disease.

Prior to this YDC speaking engagement, I had presented to hundreds of dental students during patient educator presentations. This was my first time speaking to an audience consisting solely of practicing dental professionals, yet my story was the same. The dental professionals in the audience will continue to spend most of their careers cleaning, examining, and assessing their patients’ oral cavities. These dental professionals may in fact be the first point of contact for a patient with a rare oral disease. They have the unique opportunity, as well as the obligation, to help identify any oral pathology they come across and steer the course of their patients’ lives. In doing so, they can save both their patients, as well as other specialists, precious time and resources.

Being an IPPF patient educator has pushed and challenged me in ways I never thought possible. Sharing a deeply personal medical experience to large groups of strangers in a public setting is nothing I would have imagined 13 years ago. But if the goal is to educate current and next-generation dental professionals so that future patients will be diagnosed faster and their suffering will be alleviated sooner, then I can step up and out of my comfort zone.

Ellen Levine is from Boston and works for the Armenise Harvard Foundation, a foundation that supports leading scientists at Harvard Medical School and at institutions in Italy, in the pursuit of scientific discovery in the field of medicine.
Finding my Authentic Voice at the Greater New York Dental Meeting

Staci White

When Becky Strong, IPPF Outreach Director, sent out a call for 2019 Greater New York Dental Meeting (GNYDM) volunteers, I said yes without knowing anything about the meeting other than the dates and location. I was seeking opportunities to become more actively involved with the IPPF. I also knew that Becky's energy and passion for raising awareness about the needs of our community would make it a good experience. Becky sent me an exhibitor badge and let me know I would be helping her at the IPPF exhibit booth. Other than strategizing about parking (if you live in the New York Metro area, you know this is a necessary part of any drive into NYC), I didn’t think much about the event until I arrived.

When I walked into the Jacob Javitz Center, I was not prepared for what I saw. It was as if dental professionals from all over the world created a 6th borough in the middle of Manhattan! It was fantastic and somewhat intimidating at the same time. It felt like there were thousands of dental professionals everywhere I looked. I have never seen so much information about any profession in one place. There were spaces that looked like luxurious living rooms for vendors to pitch their products, and even elliptical machines. The first few moments, I felt pure sensory overload.

As I quickly learned how to navigate this mini dental city and find my way to the IPPF booth, I felt trepidation rise in me. Although about two-thirds of pemphigus and pemphigoid (P/P) patients experience oral lesions first, that was not my truth. What was I doing here? How could I possibly advocate to dental professionals about performing biopsies when my journey began with vaginal blisters that were misdiagnosed by my former gynecologist and then appeared everywhere but my mouth? The only time I had mild oral lesions was during my first flare during the taper of my medication. They were so mild I didn’t notice them until after my doctor pointed them out.

When I saw Becky and another colleague at the exhibit table, my doubts quickly settled. I realized that my purpose for being there wasn’t just about statistics. I was there, we were there, to merge humanity with the data—to demonstrate what happens when patients are properly diagnosed and treated. We were there to illustrate what is often referred to in the nonprofit community as the, “So what?”

Upon my arrival at the table, Becky gave me a quick tutorial on the Biopsies Save Lives campaign, which focuses on dental professional outreach and accelerating the diagnosis times for patients. When I talked with my first dental professional of the day and shared information about the campaign, I gave it context by sharing a small part of my story, even though it did not include anything about oral lesions.

Raising awareness requires authenticity. It does not mean that all P/P patients have the same experience. We don’t. Raising awareness requires that we understand the complexities of our journeys and, most importantly, are willing to share our stories. My purpose volunteering at GNYDM was to increase awareness of P/P by providing dental professionals with quality information and connecting them to our collective story in a way that only a patient can. The Biopsies Save Lives campaign materials already focused on why P/P should matter to them as professionals and how they could help. In telling my story, I was illuminating the lives of others in our community. I was sharing our voice, not just my own.

Each time I spoke to someone about the campaign, I felt stronger. Each time I shared my story, I felt more empowered. Even if only a few professionals we engaged with consider biopsies for future patients, we
will have impacted lives in a significant way. It could be the difference between a prompt diagnosis and treatment or a long, painful, and frustrating journey.

Our collective voice can change the trajectory of other patients’ lives. Our collective story is powerful because it includes all of our individual uniqueness. Our journeys have purpose, and I believe part of that purpose is to pave the pathway for creating awareness of P/P. Awareness builds the foundation for much needed medical and legislative advancements for our community. As Michelle Obama states in her book, *Becoming*, “There is power in allowing yourself to be known and heard, in owning your unique story, in using your authentic voice.”

I encourage you to harness your power and use your authentic voice in support of the IPPF’s mission. I can tell you with certainty that it will not only change someone else’s life, it will transform your own.

Staci White is the Operations Manager for the Center for Genetic and Genomic Medicine and the Institute for Child Development at Hackensack Meridian Health. She currently resides in Hackensack, New Jersey. Staci was diagnosed with pemphigus vulgaris in 2011 and will celebrate four years in remission on July 20, 2020.
In response to some common questions we’ve received recently, the IPPF Medical Advisory Council has provided the following answers. A full list of questions and answers is available at www.pemphigus.org/covid19:

What precautions should patients with open P/P lesions do in light of COVID-19?
Direct spread through the skin is not a known source of transmission for COVID-19. Keep lesions clean and covered if around others.

If not on any treatment, does simply having an autoimmune disease like P/P make you more susceptible to this coronavirus?
Pemphigus and pemphigoid patients who are not receiving immunosuppressive therapies are not known to be at greater risk for COVID-19.

Does using topical treatments make you immunosuppressed?
Not usually. However, if the dosage is more than 20g of a class 1 steroid (clobetasol or betamethasone, etc.) some steroid systemic absorption occurs. It is possible this absorption can make a patient slightly immune-suppressed.

How long does it take for the following medicines to get out of a person’s system and their immune system to return to normal levels?
• **Rituximab**: The formal guidance is typically 1 year, although there is some variability in that response. We know from the published literature that many patients start to make new immune responses by 5-6 months after rituximab treatment.
• **Corticosteroids (systemic prednisone or prednisolone)**: Within days to weeks, but these cannot be arbitrarily stopped and will need to have the dose weaned properly due to adrenal suppression.
• **Class I Topical Steroids (Clobetasol/ Betamethasone)**: These don’t affect the systemic immune system unless ~20g or more are applied daily. Even if high doses are used, these would “wash out” in days to weeks as above.
• **Azathioprine/mycophenolate mofetil**: These take 3 months to “wash out” of a person’s system.

As always, our peer health coaches are available to share their experiences, treatments, living with their diseases, and more. We are also developing processes for virtual support groups. More information on these groups will be available soon.

As the situation continues to evolve and we develop further ways to support you, know that our community is strong and we are all in this together. If there’s anything we can do to help, contact us: info@pemphigus.org.
• **Cyclophosphamide**: Cyclophosphamide should presumably take 3 months based on myco-phenolate mofetil/azathioprine (MMF/AZA) data.

• **Cyclosporine**: Cyclosporine should presumably take 3 months based on MMF/AZA data.

• **Dapsone**: Dapsone doesn’t suppress the immune system in a way that would be expected to be problematic with COVID-19, and it “washes out” in a week or two.

• **IVIg**: IVIg doesn’t suppress the immune system.

**Does Rituxan put you more at risk of contracting a virus than being on high doses of prednisone?**

Rituximab does generally increase risk of viral infections. However, a randomized controlled trial published in Lancet (2017) showed that rituximab is better at controlling disease and resulted in a lower rate of infections compared to high-dose prednisone alone, so this issue would best be left to an individualized discussion with your doctor to determine the risk of disease versus the risk of treatment.

**Rituximab treatments have been postponed. What can be done in the meantime?**

IVIg could be considered if the disease is significant, or topical steroids and other non-immunosuppressive measures if that is sufficient to control symptoms. However, severe disease should most likely still be treated, as the risk of hospitalization from severe disease could be worse than treating now to get disease symptoms under control and then self-isolating at home to avoid the risk of infection. Speak with your employer and doctor about flex hours or work-from-home options if immunosuppressive therapies are used.

**I’ve been in remission, but now seem to be having a flare. Does taking medication put me at high risk for COVID-19?**

Many oral and IV medications may increase the chance that you will have a more severe course of COVID-19. IVIg is likely an exception.

**Are there extra precautions that patients should observe as states begin to reopen?**

Different states may be recommending different levels of precautions. Wear a mask or face covering in public; stay 6 feet away from others; wash your hands or use hand sanitizer if you are out in public spaces and avoid touching your face, nose, eyes, and mouth with unwashed hands; wash your hands immediately after returning to your home. Avoid mass transit if possible. Work places may offer flex hours or work-from-home options to help avoid “rush hour” when many commuters are using mass transit. If you cannot work from home or avoid mass transit – follow the guidelines above (face covering, physical distancing to the best extent possible, avoid touching your face with unwashed hands while commuting).

If you develop fever, chills, cough, shortness of breath, sudden loss of taste or smell, headache, sore throat, muscle pain, call your primary care doctor for guidance and remember to tell them if you are on immunosuppressive treatments.

**Should patients (whether on or off treatment) go out into society and businesses as they begin to reopen or should we stay home or request letters from doctors to continue to work from home until we know if there will be a big uptick in cases?**

Patients who are off treatment have the same risk from infection as a non-pemphigus or pemphigoid patient. Patients who are on immunosuppressive therapy have greater risk of infection, as well as a more serious course of infection. Flex hours or work from home would be prudent if there is a resurgence of COVID-19 in your community. Speak with your employer and doctor about options.

**What precautions should be exercised if a member of the household has to work outside of the home?**

Greater precautions are recommended if you are immunosuppressed and a member of the household works in a high-risk environment (for example, healthcare or a workplace that requires contact with multiple individuals.) Precautions the household member should consider include wearing a mask, frequent hand washing or use of hand sanitizer, avoid touching the face with unwashed hands while at work, washing their hands immediately after they return home, and surveillance for any of the symptoms of COVID-19 above.
The world has and continues to change due to the COVID-19 pandemic. Our altered lives are uncomfortable as we have gone from social beings to being sequestered in our homes. This situation is unpredictable, unpleasant, and uncontrollable. Information is ever-changing, and we are all feeling the stress.

As we monitor our physical health by taking our temperatures, watching for symptoms of COVID-19, and checking for lesions from pemphigus or pemphigoid, it is equally important to check in with ourselves about our mental health. We need to be kind to ourselves. No one has ever experienced this situation before.

It is also important to recognize that when we feel worried, we may not process information in the same way we did prior to the pandemic. We may develop biased thinking when we are anxious, and it’s possible to have selective attention and lack of concentration. In my own experience, my way of thinking becomes very black-and-white, I jump to conclusions easily, and I forget about the significance of gray areas.
Our mental health can physically impact our bodies as well. Recently, I have often felt tired or fatigued to the point that naps are essential for my survival and wellbeing. There may be days of inactivity or the inability to get out of bed. Stress can also affect our quality of sleep or digestive systems.

Behaviors may be changing, too. It’s easy to find yourself more disorganized and lacking structure throughout the day. You might be more impulsive than usual. Comfort eating, excessive drinking, or smoking can all be symptoms of stress response.

It’s important to be able to recognize these symptoms in ourselves so we can navigate ways to manage our anxiety. Here are some tips to manage your physical and mental health during the COVID-19 pandemic.

Create Structure
Decide on a daily routine and try to stick to it. It may be helpful to plan the night before and set alarms for waking up in the morning or completing tasks. When things feel uncontrollable, it’s important to focus on what we can control.

Take a Break
The news and social media can quickly become overwhelming. A constant stream of upsetting information can be harmful, and it’s okay to take a step back.

Get Dressed
Get up, get dressed, and get moving. The simple task of getting dressed can be an accomplishment. It can also help us get our bodies ready to exercise and move.

Set Daily Goals
Set a goal of something you want to accomplish each day, and do it! Something as simple as making your bed in the morning can set your day up for success and help you to accomplish other tasks as well.

Stay In Touch
Reaching out to friends and family can provide a sense of community while we are social distancing from one another. There are many ways to stay in touch virtually through things like Skype, FaceTime, texting, calling, or social media. It’s also important to stay in touch with healthcare providers and to let them know if your health changes due to flares or symptoms of COVID-19.

Use Gratitude
Try focusing on three things that you are thankful for each day. This can help support our wellbeing and set a positive tone for the day. Identifying what you are thankful for before going to bed at night may bring peace and positivity to the next day.

Write
This is a remarkable time in history. Writing can help us remember that we survived a pandemic while living with a rare disease. It also helps keep track of the positive things we’ve done to take care of ourselves.

Use Distractions
Distracting ourselves and staying busy with hobbies, work, or activities with family members can interrupt negative thoughts. It may help to focus on the positive things we can control each day.

Allow Space for Yourself
Try not to be too hard on yourself for how you’re feeling or coping during this time. It may be too difficult to be as productive or positive as usual. It’s ok to take things one day at a time.

Speak With a Professional
When we have a flare we see our doctors. When our mental health is suffering, it’s important to have an open mind about seeing a mental health specialist to help us regain our stability.

Whether you are experiencing active P/P, in remission, or still taking medications to control your disease, the IPPF recognizes that this is a time of uncertainty. What is certain is that the IPPF is here to support you. We are increasing the number of Patient Education Series webinars, peer health coaches are available to share their experiences, and we are looking into ways to connect more closely with you. There are definitely challenges right now, but we are in this together and we will get through it.

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.
As a practicing dermatologist that took my board certification exams last summer, I can tell you exactly what proteins are attacked by the body in pemphigus and pemphigoid (P/P). I can tell you the weight of those proteins, their function, and which other proteins they interact with in your skin. I can tell you what medications are recommended for treatment and counsel on reported side effects; however, I have never personally used a single one of them. I care deeply for my patients and their experience, but at the end of the day, I have never lived what they’re going through.

Each year, the annual American Academy of Dermatology (AAD) meeting typically draws thousands of physicians and medical personnel from across the globe. This year, I was scheduled to present on a panel session with Dr. Donna Culton (University of North Carolina), Dr. Annette Czernik (Mount Sinai), and Becky Strong (IPPF Outreach Director and pemphigus vulgaris patient). The content included evidence-based updates and practical clinical tips for management of P/P, challenging P/P cases, and the patient perspective. Unfortunately, this meeting was canceled due to the ongoing COVID-19 pandemic, but the important messages of the session can still be shared.

The patient perspective is one of the reasons I became interested in treating autoimmune blistering diseases and became involved with the IPPF. This is usually my favorite session at the AAD (I have not previously been a speaker for this session). As dermatologists, we have collected the history of many patients with conditions, including P/P. We ask questions like, When did symptoms start? Where do you have lesions? What medications have you tried? When we see patients in follow-up, we ask routine questions. How have you been? Are things better, worse, or the same? Any side effects on the medications? It is an entirely different experience to hear the full story of someone’s experience without worrying about documentation, billing, or treatment recommendations. It allows you to put yourself in someone else’s shoes and think about how devastating these conditions really can be. Hearing Becky’s story changed my career entirely in a positive and wonderful way.

At this year’s AAD session, Becky was also planning to provide patient advice for dermatologists based on her own experience—tips and tricks that we, as dermatologists, likely haven’t ever personally experienced and might not consider. Here is a list of Becky’s tips and tricks:

- For those with oral involvement, buy a children’s toothbrush because they tend to be smaller, rounder, and softer.
- For those with nasal involvement, consider using saline nasal spray or a humidifier in addition to the topical steroids.
- Instead of using tape to hold dressings in place, consider tighter clothing such as a camisole or leggings.
- Blot—don’t wipe—your skin after bathing.
- Counsel patients on the differences between ointments, creams, and gels and which formulations might work well in various locations on the body.
- Try Biotene® or xylitol products to help keep your mouth moist.

These tips and tricks won’t work for every patient, but they are great examples of real-world, day-to-day advice dermatologists can offer patients. Sessions that incorporate the patient perspective are invaluable opportunities for dermatologists to learn about helping patients treat and live with their conditions.

Additionally, I counsel my patients about the IPPF and their Peer Health Coach program. IPPF peer health coaches are extremely valuable resources for patients. Peer health coaches are P/P patients who are specially
trained to help reduce patient anxiety and uncertainty while providing unbiased disease and treatment knowledge. At any time, patients can submit questions to peer health coaches and receive either an email or telephone call in response. Coaches can provide a patient experience that dermatologists may not know.

For my fellow dermatologists, when you see your next P/P patient and consider the target protein being attacked by the immune system, how much that protein weighs, and how it functions in the skin, I hope you will also consider soliciting your patient’s experience with their condition. Learn their tips and tricks, and share those listed above. And for patients, please consider sharing your experiences with your dermatologist. You never know how many other patients you might be helping by doing so.

*Brittney Schultz is an Assistant Professor of Dermatology and Director of the Autoimmune Blistering Diseases Clinic at the University of Minnesota. She is a staff dermatologist at the Minneapolis VA Medical Center.*
The American Academy of Dermatology (AAD) Annual Meeting was scheduled for March 2020 in Denver, CO. However, due to the outbreak of COVID-19, the meeting was canceled. IPPF Outreach Director Becky Strong planned to participate in a pemphigus and pemphigoid panel session that included the patient perspective. Prior to the meeting cancellation IPPF Outreach Director Becky Strong interviewed dermatologists from the AAD Patient Advocate Task Force. Deputy Chair Dr. Moise Levy (Dell Children’s Medical Center) and Task Force member Dr. Emily Chu (Penn Medicine) explained the important role of the patient perspective and more information about the Patient Advocate Task Force, which encourages patient-doctor communication in order to achieve high quality patient care.

Please tell us about the Patient Advocate Task Force and about its purpose as part of the AAD.

Dr. Moise Levy (ML): The mission states that the Patient Advocate Task Force “is responsible for serving as a point of coordination on educational, legislative, and research issues of shared concern between patients, patient advocacy groups, the AAD, and the American Academy of Dermatology Association (AADA) that support our shared goal of promoting, advancing, and sustaining the highest medical care for all patients with dermatologic disorders.

How did the Task Force decide to include the patient voice in educational sessions?

ML: In 2015, Dr. Amy Paller, Patient Advocate Task Force Chair and Department of Dermatology Chair at Northwestern University, launched the Patient Integration Program at the AAD summer meeting. Dr. Paller and the Task Force found value in the patient voice and wanted its inclusion at AAD meetings. This initiative was initially met with lukewarm participation, so after doubling marketing efforts to encourage session directors to utilize the patient experience with
surveys, emails, and phone calls, the Patient Integration Program was re-launched at the 2017 Annual Meeting in Orlando with successful results.

One of the first sessions to offer a patient speaker in 2017 was Dr. Culton’s “Introduction to Pemphigus and Pemphigoid.” Why did the Task Force feel this was a good fit to have a patient speaker?

ML: The IPPF is part of the Coalition of Skin Diseases, which the Task Force has worked alongside for years. Two of the key members of that organization, you and Marc Yale, have always been passionate, compelling speakers. We thought this session with you and Dr. Culton would be perfect for launching the initiative.

What do you think the patient perspective offers that physicians or disease experts cannot provide?

ML: Patients and families are best suited to speak to the impact of all skin disorders on themselves. In addition, patients and families should be included in discussions about diagnostic and treatment options. For me, patients (and parents in particular), have been helpful in illustrating how poorly (in some cases) we work in interdisciplinary teams to provide needed care. Dr. Emily Chu (EC): Patients often offer important clues to the presentation of their diseases and response to therapies. I know I have learned a great deal about the evolution of diseases from my patients. The benefit of the Patient Integration Program is that we are able to bring the insights of some of our patients to a larger audience of health care providers.

When speaking specifically about pemphigus and pemphigoid, why is having a patient speaker important?

ML: I personally consider this group of disorders in the same light as many other conditions seen in dermatology. Sometimes patients experience extreme presentations, and treatments required present both personal and health concerns for many individuals. EC: Because patients affected with pemphigus and pemphigoid may have their quality of life so significantly impacted, the patient perspective is especially valuable as physicians consider treatment and other management options.

What has been the feedback from course participants that include the patient perspective?

ML: We have only received positive feedback from this program. Here are some quotes from our educational session directors:

The patient was great. The audience asked a ton of questions and stayed an extra 15 minutes for a very interactive Q&A. It was great, and I would highly recommend it again. -2017 Session Director

[The session] provided a humanistic side to patient care that is often lost in the old-fashioned lecture-style sessions. -2019 Session Director

I would like to thank the AAD Patient Advocate Task Force for this wonderful opportunity. It was the best experience I have had at a lecture, it was so moving, there were doctors in the audience with tears in their eyes after hearing her story. Amazingly, she took off her wig in front of everyone, unexpectedly, showing her courage. The audience gave her a standing ovation. I was speechless after her lecture. -2019 Session Director

How does the Task Force see patients becoming more involved in AAD activities? Are there any plans for growth in plenary sessions?

EC: We are thrilled that the Patient Integration Program has been so successful, and we plan to continue the program at the annual and summer meetings. Currently we do not have active plans to include patient speakers in the AAD meeting plenary sessions, although this is a topic we plan to discuss with our Task Force.

How can patients advocate with their own dermatologists?

ML: Patients and families of patients should feel the need to speak up if they are uncomfortable with any aspect of their care. In particular, care plans and behavioral aspects of diseases must be discussed. Certainly, offering to speak with other patients is always a plus. Ultimately, we function as a team. The patient is the boss and, as Francis W. Peabody stated in The Care of the Patient, “the secret of the care of the patient is in caring for the patient.” (Peabody FW, 1927)
The Diagnostic Odyssey of Pemphigus and Pemphigoid Patients

Wes Michael

In the rare disease community, you hear a lot about zebras. Why? In medical school, students are often taught that if you hear hoof beats, think horses, not zebras. In other words, what is the most likely diagnosis? It is more likely to be a common “horse” than a rare “zebra.” But, as you may hear repeatedly from rare disease patients, zebras do exist. Physicians need to be aware of how to spot a zebra. The longer a physician suspects a horse when it really is a zebra, the longer it will take to get to the correct diagnosis, which means a longer time without treatment.

I was recently at a rare disease conference where a patient said, “We talk a lot about finding out about the patient journey. Please don’t call it a journey. A journey sounds like something you’d find enjoyable. Please call it an odyssey. That is more accurate. It is not a fun experience.” How true! What is the patient odyssey and how does it vary by disease?

I am the president and founder of Rare Patient Voice, a market research company that helps patients and caregivers voice their opinions through confidential interviews and surveys to improve medical products and services. Rare Patient Voice recently collaborated with the IPPF to administer a survey to patients and caregivers to learn about their perspectives. Patients were asked about their diseases, including how long it took to get diagnosed, how many medical tests were required, the number of physicians they needed to see, if they were misdiagnosed along the way, if they received genetic testing, and if they experienced a delay in treatment. Participants of the survey were also given the opportunity to provide open-ended answers about their experiences.

As of writing, we have received 155 responses from P/P patients. These responses were compared with overall totals of similar survey results about diagnostic experiences from 3,000 patients across 436 rare diseases. The following are some initial observations based on these surveys.

Diagnosis

On average, P/P patients were diagnosed more quickly than other rare disease patients surveyed; however, there were a lot of misdiagnoses along the way. The average (mean) time to receive a diagnosis was 1.2 years overall—about the same for both pemphigus and pemphigoid, compared with 4.4 years for other rare disease patient responses. Fifty percent of P/P patients were diagnosed within six months, yet it took some patients a longer time to get properly diagnosed. It took one pemphigoid patient 14 years to receive a diagnosis, 15.5 years for a pemphigus patient, and five survey respondents indicated that it took between 5 and 10 years.
On average, patients received three to four medical tests and saw three to four physicians prior to being diagnosed. For pemphigus patients, 73 percent were misdiagnosed, and 61 percent believed their treatments were delayed. For pemphigoid patients, 59 percent were misdiagnosed, and 50 percent believed their treatments were delayed. These percentages were substantially higher than the average of other rare disease patients surveyed.

As one patient reported, “I had a lesion on my scalp. I went to the dermatologist who said I had an infected hair follicle, which was incorrect. When I had lesions in my throat, I went to urgent care thinking I had a throat infection. I was given a strep test, which was negative, and sent home. As soon as I went to my primary physician due to what I thought was frequent throat infections, I was sent to a dental specialist, who tested and correctly diagnosed me. If my dermatologist would have known about the symptoms of pemphigus, maybe I would have been diagnosed correctly from the beginning. If I would have had more knowledge on the subject, I could have gone right to my primary doctor instead of urgent care and been correctly diagnosed.” (female pemphigus patient, 62)

Some patients attributed the treatment delays to lack of physician awareness and knowledge of the condition. For example, one patient reported that, “[My] periodontist suspected that I had MMP, but neither he nor the pathologist did the correct procedures to diagnose this disease. The periodontist should have referred me to another facility that had knowledge of how to test for this disease.” (female mucous membrane pemphigoid patient, 74)

Treatment

Even after diagnosis, some respondents indicated that it was difficult to get to the correct treatment. One patient stated, “I had a hard time finding a treatment that worked for me. I had horrible side effects with several immune suppressant meds and finally found Rituxan®, that has been a life saver. I wish this drug could be accessible to everyone that needs it at a lower cost.” (female bullous pemphigoid patient, 42)

Another patient commented that, “Rather than diagnosis, my biggest problem was being matched with a dermatologist with experience/specialty in pemphigus.” (female pemphigus patient, 66)

Conclusion

The odyssey of P/P and other rare disease patients is complex. Upon examining these survey results, it is clear that delays in diagnosis, lack of physician knowledge, access to care, and adequate treatment options are common among P/P patients. It is important to examine these results and compare them with other rare disease patient responses in order to raise awareness about diagnostic issues within the rare disease community. Thank you to the patients who took the time to fill out the survey and provide Rare Patient Voice with their data.

Wes Michael is the President and Founder of Rare Patient Voice. He has been involved in market research for more than 30 years. He saw the need for rare patients to be included in market research and created Rare Patient Voice in 2013 to enable patients and caregivers to share their experiences with decision makers.
The Rare Disease Legislative Advocates (RDLA), a program of the EveryLife Foundation for Rare Diseases, is excited to announce that registration will open for Rare Across America on May 7, 2020. Rare Across America gives rare disease advocates an opportunity to build relationships with their legislators and educate them on legislation important to the rare disease community.

Under the Rare Across America program, RDLA staff schedules meetings for rare disease advocates with their members of Congress and/or the member’s staff. The meetings take place in the member’s district office during the month of August, when Congress is in recess. The RDLA team also helps to prepare advocates for their meetings, providing legislative resource materials and hosting pre-meeting training webinars. No prior advocacy experience is necessary.

“Changing public policy is a year-round commitment. We must keep the momentum going,” says Julia Jenkins, the EveryLife Foundation for Rare Diseases Executive Director. “Rare Across America is a great way to connect patients and their caregivers to members of Congress in their home districts, where they can build powerful relationships.”

Advocates interested in participating should register between May 4th and July 3rd at www.RareAcrossAmerica.org. If you have any questions about Rare Across America, please contact Shannon von Felden at svonfelden@everylifefoundation.org.

Shannon von Felden is the Program Director of the Rare Disease Legislative Advocates program at the EveryLife Foundation for Rare Diseases. Shannon is a policy and advocacy professional who works with rare disease advocates across the United States to further state and federal legislation.
We are humbled by your resilience, inspired by your strength, and driven by your trust.

During this unique time, argenx is proud to support the IPPF community. As we continue to develop antibody-based medicines for patients suffering from severe autoimmune diseases - including Pemphigus Vulgaris - we want you to know that the argenx Team is here for you during this global healthcare crisis.
Help Us Keep Hope Alive
Support current and future patients who need better treatments and a potential cure!

www.pemphigus.org/hope