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I’m excited to share that the IPPF had a strong presence during Rare Disease Week 2021. Ordinarily, this would have been in-person on Capitol Hill in Washington DC, but with in-person appointments curtailed, it was held virtually.

Over a dozen IPPF advocates held meetings with their congressional delegations. Some were seasoned advocates, while newbies (like me) were stewarded through the process by Marc Yale, our advocacy and research coordinator. Gamification (I did not know what it was either) meant that our advocacy efforts earned us $5,000 from the Every Life Foundation for Rare Diseases (https://everylifefoundation.org/). Among those whose activities earned this donation were Mei Ling Moore, IPPF Peer Health Coach. She said, “This is my first time participating in Rare Disease Week, and I feel really great about attending. I got to meet many of the 900 attending advocates and learn about other rare diseases. I listened to the struggles and disappointments that other patients and caregivers experience—and that widened my scope of compassion. We have a long way to go as movement is slow in DC, but we will persevere.”

Marc’s current and prior work on advocacy is creating real momentum in regard to legislation and regulation that affects all of our patients, as well as those who care for them. I urge you to contact Marc (marc@pemphigus.org) to become a part of this.

I also want to recognize my fellow Vermonter, Sharon Radtke. In addition to Rare Disease Week, Sharon has been working hard on getting continued support and funding for tele-health. Even outside of COVID-19, this has real impact on the quality and frequency of care for rural patients that are distant from specialists and the institutions within which they practice. Sharon is now recognized as a patient expert on the needs of these patients with rare conditions.

Thank you for all your support of the Foundation.

Kevin Mead, IPPF Executive Director
kevin@pemphigus.org
Challenges in Being Diagnosed and Living with Pemphigus

This past spring, we announced the publication of a white paper, “Elevating Awareness of the Pressing Needs of the Pemphigus Community.” The paper is in response to the results of a Patient Forum held as part of our Virtual Patient Education Conference in October 2020. The Patient Forum was sponsored by Principia Biopharma, a Sanofi Company, and brought together pemphigus patients, caregivers, patient influencers, healthcare professionals, thought leaders, and advocates to discuss the most important disease-related topics affecting the pemphigus community. The white paper discusses the consensus reached by the speakers and panelists related to the most urgent needs of pemphigus patients. It also includes decisive calls to action.

During the Patient Forum, panelists discussed their experiences with delayed diagnoses and the physical, mental, and financial impact of pemphigus. The consequences of a delayed diagnosis to a patient’s health and well-being can be significant, therefore the speed and accuracy of diagnosis must be improved.

The following is an excerpt from the white paper directly related to the challenges in being diagnosed and living with pemphigus:

Misdiagnosis and delays in diagnosis are common

Because pemphigus is a rare disease, medical and dental professionals are unaccustomed to seeing it in practice. As a result, people with pemphigus are often misdiagnosed as having a more common illness that is seen more frequently by healthcare providers. Clinically, pemphigus can mimic other oral inflammatory conditions, such as lichen planus, candidiasis, and periodontal disease.

People with pemphigus are most commonly misdiagnosed as having erythema multiforme (a skin condition considered to be a hypersensitivity reaction to infections or medications), herpes simplex virus (a viral infection that causes oral and genital herpes), or candida (a fungal infection caused by yeast). Laboratory studies, including a biopsy and immunofluorescent studies, are needed to distinguish pemphigus from other blistering and erosive diseases and achieve a correct diagnosis.

Besides misdiagnosis, significant delays in diagnosis are also common. According to the IPPF, the average patient sees 5 providers over 10 months in search of a correct diagnosis. A study cited by Dr. Werth found that a diagnosis takes more than 6 months for 40% of patients. The consequences of a delayed diagnosis to a patient’s health and well-being can be significant. During the long process of trying to obtain a diagnosis, patients can experience the following:

- Uncertainty, fear, and anxiety
- Worsening symptoms
- Increased pain and suffering
- Severe weight loss
- Risk of disease progression to other sites
- A delay in treatment
- Difficulty achieving remission
- Increased risk of a poor response to treatment
- Lost productivity and a reduced quality of life
- An increase in healthcare expenses

During the Patient Forum, the panelists discussed their own experiences with delayed diagnoses. Staci White mentioned that she did not receive an accurate
diagnosis until her disease had become so severe that she required a trip to the emergency room. She and other panelists also shared recommendations for how the pemphigus community can elevate awareness about the need for faster and more accurate diagnoses:

**Keep in mind that some healthcare professionals have only heard of pemphigus in medical school. Raise awareness of the disease by sharing your experience. Be an open book and talk about it. Share IPPF posts on your social media platforms.** – David Baron

**Advocate for yourself. Share your story with others. Don’t deny that you have the disease. Rather, embrace it.** – Janet Segall

**Tell your personal story on all platforms available to you. Encourage your family and your network to tell their story as well. Make it personal.** – Staci White

**Get in front of lawmakers, including members of Congress, and tell your story. Talk about the patient perspective and the science. Partner with industry.** – Jennifer Mangone

**Pemphigus impacts quality of life**

Once a patient has been diagnosed with pemphigus, he or she faces many challenges living with the disease over their lifetime. During the Patient Forum panel discussion, participants discussed their own experiences facing the physical, mental, and financial impact of pemphigus:

“I had wounds on 75% of my body. I had to move back in with my parents in my 30s. After trying every drug, I went into remission. The physical wounds healed, but the mental ones stayed.” - David Baron, living with pemphigus vulgaris; Chairperson, IPPF Board of Directors

“The disease took a big toll on me. I was covered with lesions, including on my face, and I had a customer-facing job. The disease led to anxiety and depression. I didn’t want to go out except for work. I missed family functions and my kids’ soccer games.” - Rudy Soto, living with pemphigus foliaceus; IPPF Peer Health Coach

“I had a severe case of pemphigus with wounds on 80% of my body. From the steroid treatment, I gained a tremendous amount of weight and experienced moon face and joint pain. I’ve been in remission for four years, and I’m trying to reclaim my body. It’s important not to dismiss that, even with remission, the disease still has a physical and mental impact.” - Staci White, living with pemphigus vulgaris; Member, IPPF Board of Directors

Based on the urgent needs discussed during the Patient Forum, the panelists and speakers agreed on the following calls to action:

1. Elevate awareness of pemphigus and the patient journey and perspective among key audiences, including clinicians, thought leaders, government agencies, and lawmakers, among others.
2. Increase awareness of ongoing clinical trials of promising investigational candidates to improve participation in trials, with the goal of developing more effective and well-tolerated treatments for pemphigus.
3. Improve affordability of and access to therapies.

The white paper provides an in-depth analysis of the challenges facing pemphigus patients during both diagnosis and treatment.

Access the white paper for an in-depth analysis of the challenges facing pemphigus patients during both diagnosis and treatment: https://go.pemphigus.org/elevating-awareness-pemphigus-white-paper
ADDRESS Study for Adults Living with Pemphigus

A CALL FOR THE PATIENT COMMUNITY TO LEARN MORE ABOUT PARTICIPATING IN THE DRUG DEVELOPMENT PROCESS

John Holtz and Kathy Perez

Patient-focused awareness

At argenx we believe that working with patients in the early stages of drug development is critical. Patients who are living with rare autoimmune conditions are best able to share the needs of their community. All aspects of life can be affected by a rare disease diagnosis, and researchers must keep these needs at the forefront of clinical studies. One trial that is working directly with the pemphigus community is the ADDRESS study. The team at argenx is working toward including pemphigus patient feedback into trial designs in an effort to ensure that the community’s needs are being heard.

In 2019, the economic burden of 379 rare diseases in the US economy was found to be nearly $1 trillion by the EveryLife Foundation for Rare Diseases. The report surveyed 1,399 members from the rare disease community, including the pemphigus community, and identified direct medical costs and indirect costs experienced by patients and families living with a rare disease. The first of its kind, this survey not only identified the economic burden of rare diseases, but also highlighted the need for additional patient-centered research and therapy development.1,2

The Food and Drug Administration (FDA) has realized the importance of listening to the patient community as well. This was highlighted in the February 2021 FDA Patient Listening Session on pemphigus and pemphigoid (P/P). During the listening session, five people living with P/P shared their journeys and
the emotional impacts that the diseases have had on each of their lives. These patients identified inadequate treatment options, diagnostic delays, and undertreated areas of the disease (mental and social burdens) as urgent issues that have negatively affected their daily lives. The pemphigus community also discussed the negative impact of an incorrect diagnosis, a decrease in quality of life, and the financial toll of treatments as further issues of living with a rare disease.³

Thank you to all patients who participate in clinical trial research. Your participation is a step toward making progress.

Patient-focused clinical trial

Pemphigus and pemphigoid are rare autoimmune diseases that cause the body’s immune system to create antibodies that attack and damage healthy cells in the skin and mucous membranes. Pemphigus vulgaris (PV) is the most common type of pemphigus and causes blisters on the skin and mucus membranes, such as the mouth and/or eyes.

Early in the COVID-19 pandemic, our team at argenx worked with the International Pemphigus & Pemphigoid Foundation (IPPF) to convene a panel of pemphigus patients to gain their feedback and input for consideration in developing our Phase 3 clinical trial design, ADDRESS. Based on feedback, the argenx team was able to make adjustments to the protocol to allow for the potential for home health visits, the provision of personal protective equipment, and alternate sites of care. Understanding the perspective of the pemphigus patient and their experience during COVID was critical to designing a patient-focused trial.

Additionally, our team discussed the patient’s treatment goals in an effort to understand what was important. Patients indicated that quality of life, access to treatments, safety, and low side effects were all areas of potential improvement. This information continues to guide our work to support the IPPF community. Elevating awareness and participation in clinical studies, such as the ADDRESS study, aims to help researchers understand more about pemphigus and the patients being treated.

Thank you to all patients who participate in clinical trial research. Your participation is a step toward making progress.

About the ADDRESS study

Find out more about the ADDRESS study at addresspemphigusstudy.com and clinicaltrials.gov NCT04598451.

The ADDRESS study is designed to assess how effective and safe the investigational study drug efgartigimod* SC (SC or subcutaneous, under the skin) is compared to a placebo (a drug with no active ingredient) as a possible treatment for adults living with PV or PF (pemphigus foliaceus). Both study drug and placebo are given with prednisone. The study aims to reach patients in 100 countries.⁴

Participants will be enrolled in the ADDRESS study for up to 41 weeks. If all criteria are met at the end of the study, then participants may be able to join an open-label extension study (ADDRESS+ study) where everyone will receive efgartigimod* SC.⁴

Eligibility requirements for the ADDRESS clinical study participants:

- Are at least 18 years of age or older
- Have a clinical diagnosis of pemphigus vulgaris (mucosal, cutaneous, mucocutaneous) or pemphigus foliaceus
- Have no known negative reactions to oral prednisone
- Have not been diagnosed with another significant serious disease
- Began to show symptoms for the first time within the past four years
There are additional study requirements that participants must meet to take part in the study. Read more about additional requirements at addresspemphigusstudy.com.

*Efgartigimod SC is an investigational study drug that is currently being studied in multiple disease states. Efgartigimod SC is an investigational study drug that is not currently approved for use by any regulatory agency as efficacy and safety have not been established.

Interested in participating in a clinical trial?

Advancing rare disease research and potential therapy development includes factors such as advocacy and participation in clinical trials studying the safety and efficacy of treatments. Patients may have the opportunity to directly influence research through the study design, treatment goals, and outcomes of these trials.

We owe the participants of clinical trials a debt of gratitude. They have contributed to the understanding of pemphigus and informed the medical community how best to advance research. Future generations may benefit from the knowledge gained with each clinical trial.

References


John Holtz is the Associate Medical Director for Dermatology at argenx. He received his PhD in physical chemistry in 1997 and MBA in 1998 from the University of Pittsburgh. He has worked in pharmaceuticals and biotechnology, focusing on immunology, including autoimmune diseases and allergy, for over 20 years. Currently, he is contributing to the scientific platform and medical affairs functions for pemphigus and other autoimmune skin diseases at argenx.

Kathy Perez is the Head of Global Patient Advocacy at argenx. She joined argenx in 2019 to lead the Patient Advocacy and Policy initiatives in support of rare autoimmune diseases including myasthenia gravis, ITP, pemphigus/pemphigoid and CIDP. Kathy comes to argenx with over 30 years in the Biotech industry.

argenx is a global immunology company committed to improving the lives of people suffering from severe autoimmune diseases and cancer. Partnering with leading academic researchers through its Immunology Innovation Program, argenx aims to translate immunology breakthroughs into a world-class portfolio of novel antibody-based medicines. argenx is evaluating efgartigimod* in multiple serious autoimmune diseases. argenx is also advancing several earlier stage experimental medicines within its therapeutic franchises. argenx has offices in Belgium, the United States, Japan, and Switzerland. For more information, visit www.argenx.com and follow us on LinkedIn.

*Efgartigimod SC is an investigational study drug that is currently being studied in multiple disease states. Efgartigimod SC is an investigational study drug that is not currently approved for use by any regulatory agency as efficacy and safety have not been established.
Clinical studies are research studies that evaluate the effect of an intervention on specified outcomes. In a clinical trial, volunteer participants receive specific interventions according to the research plan or protocol. These interventions may be medical products, such as drugs or devices; procedures; or changes to participants’ behavior, such as diet.¹

Clinical trials used in drug development are sometimes categorized by phase. These phases are defined by the FDA. Each phase has a different purpose and helps researchers answer different questions.²

Phase 1: Researchers test an investigational study drug or treatment in a small group of people for the first time. The purpose is to study the drug or treatment to learn about safety and identify side effects.

Phase 2: The investigational drug or treatment is given to a larger group of people to determine its effectiveness and to further study its safety.

Phase 3: The investigational drug or treatment is given to large groups of people to confirm its effectiveness, monitor side effects, compare it with standard or similar treatments, and collect information that will allow the investigational drug or treatment to be used safely.

Phase 4: After a drug is approved by the FDA and made available to the public, researchers track its safety in the general population, seeking more information about a drug or treatment’s benefits, and optimal use.


The International Pemphigus & Pemphigoid Foundation (IPPF) is proud to partner with the American Dental Association (ADA) on a continuing education (CE) course: Know-It-When-You-See-It: Diagnosis and Treatment of Oral Pemphigus Vulgaris. The CE course was initially presented as a live webinar on April 29, 2021. Demand for the course was so high that an encore live presentation was offered on June 3. In total, there were over 4,000 registrants for these webinars. In addition to the live events, the 1.5-hour course will be available for the next year as part of the ADA’s enduring content. To help dental professionals become familiar with PV and the procedures required to diagnose it, the CE course focuses on case-based examples to illustrate signs and symptoms of PV and evidence-based methods to confirm diagnosis.

The speakers for the CE course include Donna Culton, MD, PhD (The University of North Carolina at Chapel Hill); Joel Laudenbach, DMD (Atrium Health); Rebecca Strong, RN (International Pemphigus & Pemphigoid Foundation)

For the past seven years, the IPPF has worked diligently to raise awareness of pemphigus and pemphigoid in the dental community with the goal of accelerating the path to diagnosis. Many patients have shared their diagnosis stories and made an indelible mark on dental students and professionals. IPPF volunteers and staff have dedicated their time and talents to make our exhibits and educational events successful. All of this hard work has contributed to the launch of this course with the ADA.

"Know-It-When-You-See-It: Diagnosis and Treatment of Oral Pemphigus Vulgaris" is currently available for CE credit from the American Dental Association.

View the course at https://go.pemphigus.org/ada-ce
THANK YOU TO THE Sy Syms Foundation AND THE Unger Family Foundation FOR THEIR CONTINUED SUPPORT OF THE IPPF AWARENESS PROGRAM
Four Questions with Dr. David Fivenson

Our new 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.

David Fivenson, MD, FAAD, is a nationally recognized specialist in autoimmune skin disease, wound care, clinical research, and cutaneous T cell lymphoma. Dr. Fivenson has published over 140 peer-reviewed articles, has lectured extensively at national and international medical conferences, and has been repeatedly listed with Who’s Who in America, Best Doctors in America, and Castle Connelly’s Top Docs. He is on the editorial board of the Journal of the American Academy of Dermatology as well as a peer reviewer for several other dermatology journals. He has been an investigator on more than 150 clinical trials for both common and rare skin diseases. Dr. Fivenson is part of the St. Joseph Mercy Health System Dermatology Residency Program and is Program Director for the Complex Medical Dermatology Fellowship, which is supported by St. Joseph Mercy Health System Dermatology Graduate Medical Education.

How did you become interested in P/P?
I got interested in P/P early in my residency when I started treating patients with bullous pemphigoid using tetracycline and niacinamide. This eventually led to my applying for an FDA Orphan Products Grant to do a blinded clinical trial that I started shortly after finishing residency as a young attending physician at Henry Ford Health System. At Ford, I ran the immunofluorescence lab for autoimmune skin diseases as I finished residency with double certification in dermatology and immunodermatology. I have remained very interested in management and diagnosis of these patients and have tried hard to find steroid-sparing methods to better manage my patients.

What is one thing you’d want all patients to know early on in their journey with P/P?
This is a chronic disease that needs long-term, systematic therapy and not shotgun, short courses of steroids. I see this done by other physicians and feel that it makes for more steroid toxicity and overall more resistant disease compared to continuous therapy on a longer trajectory.

What can patients do to better advocate for themselves?
Some of the best ways for patients to advocate for themselves are to find credible information about their disease and prepare questions in advance of doctor appointments. I think the IPPF is a great source for this kind of information and not Dr. Google.

What is one fun fact about yourself?
I have 28-year-old twins and am a leader of the environmental advocacy movement within dermatology, having helped start the AAD Expert Resource Group on Climate Change and Environmental Affairs.
I am encouraging everyone to get vaccinated against COVID-19. I happily advise my patients concerning individual diseases and medication guidelines with regard to this important vaccination. There are many myths and misinformation out there, and one of the most common that I see with my patients is that if you have an autoimmune disease, you are more at risk for complications and should not get the vaccine. This is completely false. People with autoimmune disease are at greater risk for COVID-19 infection and need the vaccine more. I tell my patients who have an autoimmune disease that if they are on immune suppressors, then they are more at risk for COVID-19 infection. And if my patients are not on an immune suppressing medication, their overactive immune system makes them more at risk for the severe COVID-19 infections called cytokine storms due to their immune system overreacting to the virus.

These are some of the common myths about COVID-19 vaccines and my responses:

**MYTH: It's too new and experimental.**
The method used to make the mRNA vaccines has been around for years. The COVID-19 mRNA vaccines currently in use were made quickly due to a worldwide, concentrated effort. Additionally, there are hundreds of millions of people that are now vaccinated, making it one of the largest clinical trials of any medication or vaccine on record!

**MYTH: It has aborted fetal parts.**
This is simply false. There is a small strand of mRNA made in a lab by a machine, not extracted from any living cells.

**MYTH: It changes your DNA.**
This is also false. mRNA is what is made from our DNA to allow protein to be transcribed (produced) in the cytoplasm of the cell. mRNA from the vaccines cannot reach the nucleus of the cell where our DNA lives. The mRNA disappears from the body completely within a few days after getting the vaccine. The mRNA contains instructions for creating only the spike protein of the coronavirus that causes COVID-19. This allows the body to develop an immune response without being exposed to the actual virus.

We all need to get vaccinated for COVID-19—and the sooner the better. The longer people wait and are hesitant, the more the virus circulates and can mutate. If the pandemic continues to go on for too long, mutations can develop that may not be covered by current vaccines. Then the whole pandemic could take off again, possibly worse than ever. So, tell your friends, neighbors, and relatives it is their duty and for their health, as well as yours and the health of everyone else on this planet, to get the COVID-19 vaccine!

Dr. David Fivenson is part of the St. Joseph Mercy Health System Dermatology Residency Program and is the Program Director for the Complex Medical Dermatology Fellowship, which is supported by St. Joseph Mercy Health System Dermatology Graduate Medical Education.

The IPPF COVID-19 information page is updated regularly with new information from our Medical Advisory Council:
https://www.pemphigus.org/covid19
A Look at the Upcoming 2021 IPPF Scientific Symposium

Kevin Mead

In cooperation with the Pegasus Project and Phillips University in Marburg, Germany, the IPPF will deliver the 2021 IPPF Scientific Symposium this September. Unlike prior IPPF Scientific Symposia, this one will be fully virtual. However, that will not detract from the ground-breaking presentations that will be made. The event will bring together clinical and scientific experts in the field of autoimmune bullous disorders. The symposium is an opportunity for physicians and scientists from around the world to come together under the common goal of studying pemphigus and pemphigoid. It will be a virtual event featuring three days of live sessions and abstract discussions from September 19-21.

I had the chance to catch up with a few members of the Organizing Committee, who were able to give their thoughts on the upcoming event. Dr. Dario Didona, resident physician at Istituto Dermopatico dell’Immacolata in Italy, is particularly looking forward to the abstract sessions presented by younger researchers. “It is important to exchange experience and data with the most important experts in the field, especially as there are many different approaches. The young researchers and clinicians are building up a network within pemphigus and pemphigoid, and it is critical to support that.”

Dr. Jens Waschke, professor at Ludwig-Maximilians-Universitat Munchen, agrees. “The strength of the research field is that clinicians are also the best scientists performing experimental studies at an extremely high level. Together with experts in cell biology, the expertise on a single group of diseases rarely is so high at any other specific meeting. This makes this meeting especially efficient for all, and primarily for the young people for whom it is particularly important and motivating to meet experts they only know from the literature.”

Dr. Michael Hertl, professor in the Department of Dermatology and Allergology at Philipps-Universitat in Marburg, Germany, believes that the directed focus of the meeting is immensely helpful. “In contrast to a general scientific meeting, we have here the chance to exclusively focus on bullous diseases. We have only one track of presentations, so everyone can be involved and learn from the whole event.”

Clinicians and researchers are encouraged to register for this event, and of course patients and others with an interest in the science behind bullous disease research are also welcome: https://go.pemphigus.org/sci2021

Kevin Mead joined the IPPF as the Executive Director in October 2020. He has over 25 years of experience in association management, fundraising, and project management.
2021

IPPF VIRTUAL INTERNATIONAL
SCIENTIFIC SYMPOSIUM

September 19-21

REGISTRATION AND INFO:
https://go.pemphigus.org/sci2021
My dad, Marc Yale, has always been one of the biggest heroes and role models in my life. He is hardworking, loving, smart, selfless, and strong. I have witnessed his strength for my entire life, and I hope to be like him one day.

During my sophomore year of high school, my dad became chronically ill. Several months passed as he and my mom visited multiple doctors’ offices without any answers about what was happening to him. During that time, his illness worsened. Eventually, he became blind in his left eye.

Finally, after six months of suffering, my dad was given a proper diagnosis of mucous membrane pemphigoid (MMP). After receiving his diagnosis, he continued to do his own research and eventually connected with the IPPF. They provided him with a list of doctors who knew how to treat MMP, and he was able to receive the proper treatments. Through the IPPF, he was also able to connect with other patients who were going through similar experiences.

When the IPPF created the Peer Health Coach Program in 2008 to assist patients and caregivers around the world, they asked my dad to become one of their first peer health coaches. In 2016, my dad became the IPPF’s executive director, and in 2020 he transitioned to the advocacy & research coordinator.

My involvement with the IPPF, their programs, and resources began when I was still living in California. My dad and I would have to fly to Boston for him to receive the necessary treatment and to see his specialists. While in Boston, the IPPF helped us arrange for a place to stay with local members of the community who were generous to let us stay in their homes.

In October 2019, my dad and I visited China for the 7th Shanghai International Bullous Skin Disease Symposium. This was a unique and life-changing experience. During the symposium, I was introduced to a diverse group of my dad’s colleagues from around the world. I also met many students at Shanghai’s Jiao Tong University while visiting the Ruijin Hospital School of Medicine, including Professor Meng Pan.

During our visit to the hospital my dad was asked to speak to local patients and their caregivers about his experience and journey with MMP. After his
presentation, he was able to speak directly with audience members. There were many people that approached him simply to express gratitude that they were able to meet another patient who had gone through a similar experience. He was able to talk to them about being in remission and provide a little extra hope.

One of my favorite ways that I have been involved with the IPPF is by attending Rare Disease Week on Capitol Hill in Washington, DC. The first time I attended, I was able to meet other pemphigus and pemphigoid patients, caregivers, and doctors. We were all there to advocate for the rare disease community.

Since then, I have attended Rare Disease Week as a representative from California, Montana, and Wyoming. By advocating during Rare Disease Week, I have the power to speak directly to members of Congress about legislation that will benefit people with all types of rare diseases. I am proud to be able to represent the IPPF and my dad when I attend. The last year that I attended, my advocacy partner was also there representing her father who had a different rare disease. We proudly stormed Capitol Hill while calling ourselves “the Dadvocates”! Participating in Rare Disease Week has shown me that by joining together and using the strength of our voices, we can make a positive impact for others who may feel powerless in the face of so much uncertainty.

The IPPF has been so helpful to my dad throughout the past fifteen years, as well as to our entire family. We have found a community of people who understand what we are going through, and who provide support and hope to one another. The IPPF is filled with compassionate staff members and volunteers and provides inspiring programs and resources. The Foundation has helped my family and me remember that in times of fear, anger, and anxiety, we are never alone.

Mariah is the daughter of a patient with pemphigoid. She is currently a preschool teacher living in Missoula, Montana, and has been an Early Childhood educator for the past ten years. She received her bachelor’s degree in Liberal Arts with an emphasis in Psychology at Antioch University in Santa Barbara.
Many people were raised with the mantra that failure is not an option. So what happens if failure becomes a requirement—by a patient’s health insurer, no less?

This painful irony is familiar for those living with pemphigus, pemphigoid, and other skin and autoimmune conditions. Somewhere along their treatment path, they’ve likely been required to fail first through an insurance barrier known as step therapy. Step therapy often requires a patient to first try a medication preferred by their insurance company before receiving the one prescribed by their health care provider. Sometimes it’s two—or even three—medications that must be tried first. With each drug, the trial period may be weeks or months. Collectively, these steps may leave patients in want of effective treatment for the better part of a year.

Further complicating the process, if a patient changes insurers or switches plans because of a new job, they may be forced to jump through those same hoops again—even if it means failing on drugs they’ve already tried.

The concept of step therapy is rooted in medicine, as health care providers will attest. Of course, it makes sense to start patients on a more moderate drug before progressing to a more intensive one. It may make sense to start on a drug with fewer side effects before risking one with greater side effects. Sometimes, physicians will begin with a drug that has a lower out-of-pocket cost before progressing to one that costs the patient more. But that decision rightfully belongs to a health care provider and their patient. It should stem from a relationship of trust and reflect the patient’s lifestyle, symptoms, comorbidities, and tolerance for certain drugs.

Insurers distort the concept. Health plans mandate a progression of drugs that isn’t based on what’s best for the patient, but what’s most lucrative for the insurance
company. In the process, they usurp decision-making power that should remain with health care providers and patients.

Inappropriate and profit-driven step therapy has plagued the dermatological community for far too long. That’s why advocates are fighting back by supporting the Safe Step Act in the US Congress. Following more than two dozen state bills that curb step therapy, the Safe Step Act would tackle the issue on a federal level. Because some health plans are regulated by state laws and others by federal laws, action by Congress is vital to getting step therapy in check.

The Safe Step Act would protect patients by putting commonsense time limits on step therapy. It would also offer health care providers a straightforward way to appeal step therapy when it’s medically inappropriate for their patient. This bipartisan legislation has captured the support of more than 85 patient and health care organizations.

How can advocates help get the Safe Step Act over the finish line? By contacting their members of Congress and voicing support for the Safe Step Act (S 464 in the Senate and HR 2163 in the House of Representatives). By using their social media presence to build support using #SafeStepAct and #StepTherapy. And by rallying family, friends, and fellow advocates to take action. When it comes to reining in step therapy, failure is not an option.

Mike Walsh is the executive director of the DermCare Access Network, a national nonprofit coalition advocating for patient access to optimal dermatological care.
Step Therapy:
To get the medicine prescribed by their health care provider, patients must first prove that older, less expensive or insurer-preferred alternatives don’t work. Also known as “fail first.”

OVERVIEW

In some cases, step therapy makes sense. A logical progression of treatment options may represent best practice for certain diseases or reflect the wisdom of clinical guidelines.

In other cases, step therapy can be excessive, arbitrary and even damaging to patients’ health. Insurers may use step therapy as a deliberate access hurdle meant to protect their own profits.

Doing so hurts patients, whose skin condition may worsen or who may suffer unnecessarily in the process of failing insurer-preferred treatments. It also undermines the relationship between the physician and patient, to whom treatment decisions rightfully belong.

POSITION

Policymakers can help patients by implementing the following four principles:

1. **Step therapy must be rooted in clinical evidence.**
   If insurers dictate a progression of treatment options, they must be able to trace their requirement to clinical guidelines from relevant medical associations.

2. **Repeated failures are inappropriate.**
   Patients who have tried a treatment with a previous insurer should not be asked to fail on the same drug again.

3. **Patients deserve a straightforward exemption process.**
   Some patients will have allergies, side effects, comorbidities or other health factors that render a step therapy protocol inappropriate. They and their health care providers need a straightforward process for bypassing the requirement.

4. **Insurers’ communication must be timely and clear.**
   Delays associated with step therapy can impact a patient’s life and health. That means that responses to a patient’s request for a step therapy exemption should occur within a reasonable timeframe. Similarly, details about the exemptions process should be readily available and in plain language for patients and health care providers to access.

With these safeguards in place, legislators can ensure that insurers do not overuse or misuse step therapy to the detriment of patient health.

DermaCareAccess.org
Nothing about 2020 was normal. Anticipation and anxiety related to COVID-19 and our diseases created a lot of emotions. The pandemic lockdown made everything feel suspended. In what may be one of the most overused terms, the unprecedented situation required everyone to pivot.

Despite this, a major highlight of my year was the IPPF Virtual Patient Education Conference. Prior to attending, I was skeptical that the IPPF could manage to pull off its annual conference as a virtual event. My former experience in the travel and tourism industry has given me a unique perspective on the impressive task taken on by the handful of IPPF staff and volunteers year after year. Having attended IPPF conferences in San Francisco (2013), Chicago (2014), New York (2015), Austin (2017), North Carolina (2018), and Philadelphia (2019), I am also aware of all the fringe benefits. These include the extras besides the scheduled events, such as meals out, awards, recognitions, and the casual conversations in the lobby or elevators. One year, I had the most insightful conversation with a professor of dermatology while waiting for my Uber to the airport.

However, there proved to be advantages to hosting a virtual meeting, including the ability of participants to attend who might not have been able to otherwise. In past years, I was one of the only members of my local support group to travel to these conferences. Fortunately, I was able to spend the time and money to fly to the various locations so that I could hear directly from the experts on the latest research. Each year, I brought home a suitcase full of samples generously provided by conference sponsors. Sharing the swag and information with the South Florida Support Group was part of my role.

Between airfare, hotel, conference registration, and meals, the three-day meeting—while worth it—hasn’t always been an option for everyone. Eliminating these
costs was likely helpful for many of last year’s attendees. The virtual setting also allowed patients to attend who wouldn’t have been able to attend an in-person conference due to their health or personal situation. The opportunity to have more individuals connect was very exciting. For example, the fact that medical experts like Dr. Dedee Murrell, from Sydney, Australia, would present was something I had not predicted. Having heard of her reputation, I never imagined I would get to listen to her lecture.

Additionally, the interactive tools like live polls and chat were an unexpected highlight. The breakout rooms with exhibitors and sponsors also went well, and I enjoyed the opportunity to discuss a range of topics directly with presenters. I’ve heard from other patients that they learned at least one nugget of life-changing information.

Personally, I found last year’s Virtual Patient Education Conference very informative and exciting. I loved the option to attend in my pajamas. I even did some yoga stretching during one of the speakers, and it was delightful.

The upcoming 2021 Patient Education Conference (October 22-24) will again be virtual. Hopefully, even more individuals are now comfortable using the technology to attend such events. This year’s conference will again include opportunities for personal connection with other patients and speakers, including casual conversations similar to an in-person conference. There will also be designated times for support groups to meet. This will give each region an opportunity to meet with their local participants and discuss the annual plan for future group meetings.

Humans seek connection. Finding commonalities with others brings comfort. For our community, the IPPF’s support of patients, physicians, and researchers as we all pave a path forward is crucial. Luckily, I found the IPPF quickly after diagnosis and was able to attend my first conference within two months of receiving biopsy results. As someone who has been actively involved ever since, I realize it’s in my self-interest to learn about the latest scientific knowledge. Sharing that with other patients only feels natural.

Despite the challenges of 2020, the IPPF team created a successful Virtual Patient Education Conference experience. I am looking forward to how we can build on that foundation and improve the experience for 2021!

Daphne Smolka is a Patient Educator and Awareness Ambassador for the IPPF. She was diagnosed with pemphigus vulgaris in 2014 after being misdiagnosed in 2013. Currently on the road to remission, Daphne lives in South Florida with her two children and dog, Milo.
SAVE THE DATE

IPPF VIRTUAL PATIENT EDUCATION CONFERENCE

// OCTOBER 22-24 //

Sign up to be the first to know when registration opens: https://go.pemphigus.org/2021-conference-info
The Quarterly is funded in part by an educational grant from argenx.

2021

IPPF VIRTUAL INTERNATIONAL
SCIENTIFIC SYMPOSIUM

September 19-21

REGISTRATION & INFO:
https://go.pemphigus.org/sci2021