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Message from the Executive Director

Dear reader,

Welcome to the first Quarterly of 2023! Here at the IPPF, our year is off to a great (and busy) start. In the following pages, you’ll find reports and articles about many of our recent achievements, as well as some reflection on the past year’s programmatic efforts. Some of this information, such as our 2022 Year-in-Review, reflects the ways in which the IPPF staff shows up every day to support our community. In other articles, you can read about how years of work can culminate in major milestones for pemphigus and pemphigoid (P/P) patients. This is especially the case for our Externally-Led Patient Focused Drug Development (EL-PFDD) meeting, held in January. This event resulted in dozens of P/P patients sharing their stories in front of the US FDA and other key interest leaders. It was a truly successful initiative, and a unique global collaboration between the IPPF (based in the US); PEM Friends (based in the UK); Association Pemphigus & Pemphigoïde France; and the Pemphigus/Pemphigoid Friends Association (based in Japan). Perhaps most importantly, our EL-PFDD meeting was a major step in our community’s ongoing elevation of the patient voice.

In this issue of the Quarterly, you will also find articles on how the IPPF collaborates with other organizations and professionals to broaden the reach of our patient advocacy and support efforts. It is certainly true that in the rare disease world, there is strength in numbers. These collaborations serve to amplify the patient voice among the medical professionals who can make a correct diagnosis and the decision-makers working on new treatments.

As we look forward to the rest of 2023, I’m excited to see how these initiatives will come together in support of people affected by P/P. Of course, none of this would be possible without the support of people like you. Whether you’re a patient yourself, a caregiver or friend of a patient, a medical professional, or an industry partner, the IPPF continues to thrive because of your engagement.

Thank you,

Patrick Dunn, IPPF Executive Director
patrick@pemphigus.org
2022 IPPF YEAR IN REVIEW

Patient Education Series Webinars

3,502 total registrants over

13 webinars

Support Groups

703 registered participants

34 meetings

Patient Education Conference

458 registrants from

24 different countries

679 Peer Coach Cases
Over 100 congressional members reached via IPPF advocacy efforts

- Students reached at Patient Educator Presentations

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This past fall, as we approached another year of the COVID-19 pandemic, there were hints of a closer return to normalcy. However, returning to normal often looks different for everyone, and since the start of the pandemic in March 2020, it’s clear that certain things have become permanent in our everyday lives.

The IPPF has functioned as a remote team for many years (even prior to the pandemic), but we look forward to gathering with each other and spending time with the pemphigus and pemphigoid (P/P) community each year at our annual Patient Education Conference. We were hopeful that we’d be able to meet in person for the 2022 conference, but the decision to host a virtual event felt like the safest option for both our team and those who are potentially immunocompromised. Luckily, we felt more prepared than ever to host the three-day virtual event.

Last February, the IPPF welcomed Patrick Dunn as our new Executive Director. For many years prior, he worked as the IPPF Communications and Marketing Director to make sure things were running smoothly, often behind the scenes. It was a nice chance for us to introduce him in his new role, and for the P/P community to see him front and center. It’s been a very successful and busy year under Patrick’s leadership.

The theme of this year’s three-day virtual conference was “Uniting Our Global Community.” The event started on a Friday afternoon and featured sessions on COVID-19 boosters and vaccinations, steroids and immunosuppressants, and infusion therapies. The second day of the conference focused on topics related to patient and caregiver stories, mental health, topical treatments and wound care, oral care, eye health, insurance issues, and more.

The final day’s agenda included an international patient story, the role of caregivers, the argenx BALLAD study, inter-organizational partnerships, insurance, advocacy, research, clinical trial participation, and a preview of the IPPF’s Externally-Led Patient-Focused Drug Development meeting with the FDA in January 2023.

As in previous years during the COVID-19 pandemic, we were able to connect with a greater number of people from around the world who are living with P/P. Despite not being able to connect in person, we are thankful for the opportunity to reach a larger number of people through the virtual platform. We are looking forward to connecting at the 2023 Patient Education Conference. Please look out for more information this spring about the event.

Anna Lane is the IPPF Communications and Marketing Manager. She lives in Denver, CO with her family.
To ensure that the IPPF can provide the most current information about pemphigus and pemphigoid (P/P) treatments, we have developed and continue to maintain close relationships with doctors and leaders in the medical community.

In 2022, the IPPF actively collaborated with the European Academy of Dermatology and Venereology (EADV) and expanded our relationship building by participating at the 2022 EADV Congress, held in Milan, Italy on September 7-10, 2022. This was the IPPF’s first time attending the Congress, and the objective was to connect with stakeholders, including healthcare professionals and industry representatives, to bring attention to the work that our organization is undertaking to improve the lives of patients living with P/P. The IPPF participated in the fifth annual co-organized session with the EADV Patient Working Group, an Autoimmune Bullous Disease Working Group Meeting, and multiple GlobalSkin events including the Patient Advocacy Village, and the “Skin Matters” Reception.

The IPPF also attended the International League of Dermatological Societies’ (ILDS) World Skin Summit which took place in Lima, Peru, October 13-15. This was a unique global event that brought together ILDS members, leaders of national dermatology societies, and partner organizations like the IPPF. Jennifer Austin, GlobalSkin CEO, was part of the event organizing committee and assembled a delegation of patient organization leaders to participate in the Summit, including myself; Tran Hong Truong from the Vietnam Psoriasis Network (PSORVIET); Antoine Gliksohn from the International Albinism Network; Martin Petroco from the Asociación Civil para el Enfermo de Psoriasis and Asociación Civil de Dermatitis Atopica Argentina; and both Carolina Huaman and Maria del Pilar Arinabar from the Asociación de psoriasis y artritis psoriásica Perú (APAPSO PERÚ). Jennifer Austin had the privilege of presenting to participants about the Global Research on the Impact of Dermatological Diseases (GRIDD) Project that the IPPF community has participated in. She also emphasized the importance of patient organizations and how they can be partners to healthcare professionals in supporting dermatology patients.

As the IPPF continues to lead the way in voicing the needs of the P/P community, our advocacy on behalf of people affected by P/P will surely affect positive change on the issues that are most important to patients and their families. In 2023 and beyond, we plan to work closely with like-minded advocacy partners to inspire policies that are supportive of patient needs, increase disease awareness and education, and improve access to high quality healthcare by working with medical professionals and societies.

Marc Yale was diagnosed in 2007 with cicatricial pemphigoid. In 2008 he joined the IPPF as a peer coach. He was the executive director from 2016-2020 and is now the research and advocacy coordinator. Marc currently resides in Ventura, CA with his wife Beth and his daughter Hannah.
**My Experience at the Global Genes 2022 RARE Patient Advocacy Summit**

Iva Rauh

**After more than two years** of living in isolation due to the COVID-19 pandemic, I was thrilled about traveling to San Diego and attending the 2022 RARE Patient Advocacy Summit organized by Global Genes for the first time. Attendees came from all over the US, Europe, and New Zealand. I was really glad to see the familiar face of Patrick Dunn, IPPF Executive Director.

The first day included the following sessions and keynote speakers:

**Keynote Speakers:**

**Emily Rapp-Black**, a poster child for the March of Dimes and bestselling author.

In 2007 she published her first memoir *Poster Child*, detailing her life as an amputee.

She started her presentation with the poem *Boats on the Water* (Rainer Maria Rilke).

“Love wins because the purest form of love is to reach out to other people, to tie our boats to piers. To rise or sink always together.” From an early age, Emily had fantasies of being healed of her disability through a miracle.

Don’t we all who are affected by rare diseases hope for a miracle? She is the author of a book about my favorite painter, *Frida Kahlo and My Left Leg*.

**Gail Devers**, a three-time Olympic gold medalist, was successful in both sprinting and hurdling, and she was selected for the 1988 Olympics. Devers was later diagnosed with Graves’ disease, an autoimmune disease that affects the thyroid. Devers recovered and she is still running. The 5’3” dynamo was recognizable by her 1.5-inch nails and her boots. She entertained attendees, but her message was clear: “If you have knowledge, what good does it do you if you keep it to yourself?” Gail emphasized the importance of mentoring younger people who will continue to make a difference and show the world that our voices need to be heard.

From the many informative sessions where we could learn how to manage our everyday life challenges I chose:

**Mental Health for Rare Adults**

Many rare disease patients need mental health treatment, but high costs (including financial) of...
mental health treatment keep them from getting help. Pemphigus and pemphigoid (P/P) patients often reach out to the IPPF for support, but some of them may need to seek professional help.

It’s important to research a mental health professional’s credentials prior a visit to be sure they are qualified and have experience with chronic illness.

Caregivers Track

Some of us are not only patients, but are caregivers too. How can we avoid feeling burned out and depressed? Finding the right kind of support from people who are willing and able to help is an important part of the treatment. The IPPF peer coaches can help, and some patients might join a support group or find a helpful podcast.

The Emergency Guide

Many times, rare disease patients are in a difficult situation when they deal with a new provider. They know more about their disease than most doctors and dentists. Thanks to the IPPF Patient Education Series webinars, P/P patients are well-informed about options for different treatments. We just have to be kind and educate our providers.

Following the day’s sessions, attendees had just enough time to dress up before the evening’s program. During the Blue Carpet and Cocktail Reception hour there were a lot of opportunities for networking and to take a few pictures with new and old friends. The evening’s main event was the 2022 RARE Champions of Hope Celebration, where individuals were recognized for their extraordinary service to the rare disease community. Alice Payne, a standup comedian, speaker, and rare disease advocate and Madison McLaughlin, a well-known actor, were perfect hostesses for this unforgettable evening. Cris Jacobs enchanted with virtuoso guitar playing.

It was inspiring to listen to the stories of the award recipients:

RARE Champion in Advocacy (Individual):

Adrian Goretzki: rare patient, lawyer, professional patient advocate, and founder of the Healthcare Education Institute. Since the Russian invasion of Ukraine, Adrian has been engaged in helping Ukrainian refugees with rare diseases to find shelter and specialized treatment in the European Union.

RARE Champion in Advocacy (Foundation):

Lysosomal Storage Disorders Support Society was established in 2009. It is the first and largest rare disease organization in India, and has grown to include 600 patient families.

RARE Champion in Medical Care and Treatment:

Yuriy Stepanovskiy, PhD, is the Executive Director of the Ukrainian Association of Pediatric Immunology and is a member of the NGO Rare Immune Disease. His professional interests include primary immunodeficiencies, autoinflammatory diseases of unknown origin in children, and Kawasaki disease.

RARE Champion in Industry:

Arthur A. Levin, PhD, is a key opinion leader in the RNA therapeutics field. He played key roles in developing the first approved antisense drugs and the first microRNA-targeted therapeutic in clinical trials.

RARE Rising Star:

Nell Choi became a junior ambassador to the Sumaira Foundation. At the age of nine, Nell was diagnosed with a rare autoimmune disease. She wrote My Hospital Story to share her experience and help other children.
RARE Champion in Science and Technology:

Richard Horgan is the Founder and President of Cure Rare Disease. He formed an interdisciplinary collaboration of world-class researchers and clinicians to pioneer the rapid development of customized therapy for rare genetic diseases.

RARE Founder’s Award:

Joe and Cynthia Lang

The Lang’s daughter Jordan is affected with a very rare genetic mutation which has been identified in six other children in the world. A team of research universities around the world are working to learn more about this mutation and are preparing to begin testing on three different approaches to treat and cure what has been named “Jordan’s Syndrome.”

(In October 2018 Global Genes recognized Marc Yale, IPPF’s Executive Director as Rare Leader!)

On the last day of the summit, the keynote speaker was Jim Geraghty. His speech focused on “The Past and Future of Patient-Centered Rare Disease Innovation.” Jim has been a passionate participant in the orphan drug revolution since its inception forty years ago.

Advances in medicine have made better treatments possible for cancer, diabetes, and heart disease. Yet there is still no treatment for the thousands of rare diseases because they afflict only a small number of people. Scientists and researchers are starting companies where promising new treatments for rare diseases will be developed. Many will fail a first, second, or third time.

The IPPF has done a spectacular job of raising awareness about P/P. It’s important to continue striving for faster diagnosis and more effective treatments. Patient involvement has, and can, make a difference, and patients should continue to advocate for research.

I had the opportunity to represent the IPPF at the summit and share their story with numerous representatives of other rare disease organizations. I look back at the conference with gratitude because these personal connections lead to more understanding about each other. Thank you to everybody from Global Genes!

Iva Rauh is a pemphigus vulgaris patient. She lives with her husband on the Eastern Shore of Maryland.
Diagnosed with Bullous Pemphigoid? (BP)

Learn about a study of a potential new treatment for BP

With your participation, you can help researchers investigate a potential treatment for BP

Who Can Take Part?
• 18 years old+
• Confirmed diagnosis of moderate to severe BP
• Active blisters
• There are additional study requirements you must meet to take part in this study. A study representative will discuss these with you.

What Will The Study Involve?
• Screening period: 2-3 weekly visits
• 43 weekly visits – most at the study center, some at home
• 1-2 minute subcutaneous injection (under the skin)

Are There Any Costs?
• No costs to participant
• Travel, accommodations, food and drink expenses reimbursed by study sponsor

How Do I Participate?
The first step is to talk with your doctor and share the study listing:
https://clinicaltrials.gov/ct2/show/NCT05267600

Speak to a Know Rare Patient Advocate who can help you connect to a study center to discuss the details of the study and if you are eligible to participate.

Go to balladstudybp.com

A Look at the Impact of the Coalition of Skin Diseases and the American Academy of Dermatology Association Legislative Conference

Becky Strong

On a bright and cheery weekend last September, I traveled to Washington, DC, with IPPF advocate Doris Chenier. We had the opportunity to advocate alongside almost 200 dermatologists and patients as IPPF representatives to the Coalition of Skin Diseases (CSD) at the American Academy of Dermatology Association (AADA) Legislative Conference.

We learned about the legislation’s impact on the dermatologists who treat many of us in the pemphigus and pemphigoid (P/P) community during the three-day conference. While there has always been a day-and-a-half of great sessions that cover legislative asks, Hill Day looked a little different this year. In-person meetings always garner the largest impact on Senators and Representatives, but not all offices were meeting back in person on Capitol Hill. So, attendees of the conference had the opportunity to have both in-person and virtual meetings. No matter how we met with congressional representatives, we felt our voice was important.

This year, the AADA had two significant areas of focus: supporting physician practices and removing barriers to treatment and care.
Supporting Physician Practices

We learned during the legislative conference that it is essential to go to the Hill and ask Congress to ensure Medicare stability for patients and physicians. Medicare physician reimbursement has failed to keep up with inflation, threatening the viability of medical practices. Since 2001, the cost of operating a medical practice has increased by 39%, while inflation has risen by 51%. During this time, Medicare physician reimbursement has risen by only 11%, while Medicare hospital and nursing facility reimbursement has increased by 60% or more, a significantly greater amount than what physicians are being reimbursed. When adjusted for inflation in practice costs, Medicare physician reimbursement declined by 20% from 2001 to 2021.

The current payment structure has contributed to the consolidation and increased hospital ownership of more costly physician practices, reduced competition, and tends to be less patient centered. Year-over-year cuts to Medicare reimbursement jeopardize physicians’ ability to keep the doors open and care for patients in our communities.

Please understand we are not asking for money to line doctors’ pockets. Doctors rely on reimbursements to cover multiple practice expenses. This includes staff salaries and benefit costs, state and federal regulatory compliance costs, and associated insurance mandate costs like step therapy and prior authorization, making it more challenging to keep their practices open to treat patients like you and me. Fewer physicians mean longer wait times for patients to receive care, and as patients, we will suffer in the end.

Again, as in past years, the Centers for Medicare & Medicaid Services (CMS) has proposed a fee schedule that would offset increased payments for evaluation and management services with payment reductions to the other services. These cuts to the Medicare Physician Fee Schedule were made because of CMS’s budget neutrality requirement, meaning cuts to other services must offset the increased payments. CMS is proposing a cut of nearly 4.5% to the Medicare conversion factor, which is the critical component in determining Medicare physician payment.

On top of this, due to budget rules created by the Pay-As-You-Go Act of 2010 (PAYGO) and the implications of using the reconciliation process to enact the American Rescue Plan of 2021, CMS will cut Medicare payments by another 4% in 2023. This will come on top of any required cut under the Medicare Physician Fee Schedule. Unless Congress acts, dermatologists could see their Medicare reimbursement cut by 8.5% or more.

So how does this affect patients? The cuts affect budgets to keep practices running. Due to cuts, doctors may be forced to reduce their hours of practice or close the doors altogether because they cannot afford to keep staff in the office. Patients like you and me may be forced to travel further to find a knowledgeable provider to take care of us. If they stay open, prior authorizations are often needed for many of our medications since so many are used off-label. With less staff in the office, we face a longer wait time to submit the proper paperwork, resulting in increased pain and suffering while waiting for treatment for our disease. How far must we travel, and how long must we wait to find relief?

Patients like you and me may be forced to travel further to find a knowledgeable provider to take care of us.

You may not have Medicare, so why should this matter? Because patients with other types of insurance see doctors who may also accept Medicare. In the past, when CMS decided to alter its payment schedules, other insurance companies followed suit. So, doctors’ cuts could be seen by all health insurance companies since they base their pay schedule off CMS processes.

Specific asks to the House and Senate:
• Replace the scheduled and anticipated cuts in Medicare physician payments with positive, inflationary-based updates for at least one year.
• Co-Sponsor H.R. 8800, Supporting Medicare Providers Act of 2022, and eliminate the nearly 4.5% cut in reimbursement in CMS’s proposed rule for the 2023 Medicare Physician Fee Schedule.
• Waive the 4% PAYGO sequester necessitated by the passage of legislation unrelated to Medicare.

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Remove Barriers to Treatment and Care

**Step Therapy**

As many in the IPPF community can testify, step therapy strategies can negatively impact patient outcomes and quality of life by requiring patients to try one or more drugs before coverage is provided for the treatment regimen our physicians recommend. This practice by insurance companies can affect our quality of life and jeopardize our health by risking adverse and potentially life-threatening reactions or situations due to taking inappropriate or inferior drugs. Step therapy can ultimately cost insurance companies and patients more in the long run through additional trips to physician’s offices, emergency room visits, copays, and out-of-pocket expenses for ineffective treatments.

It has been shown that step therapy implemented by health plans does not consider the following:

- A patient’s medical history
- Whether the patient has already tried the drug and failed in the past
- If a patient has a medical condition that would interfere with the drug’s efficacy
- If the drug’s side effects would interfere with the person’s ability to perform their job
- If the best treatment for the person is a drug with a different ingestion method or dosage.

Essentially, patients like you and me must have access to alternative treatments if the first line is not optimal or is contraindicated. Switching therapies can cause the medication to lose effectiveness if a patient resumes it later, potentially affecting the quality of our lives.

**Specific Asks to the House and Senate:**

The Safe Step Act (H.R. 2163 / S.464) would ensure that step therapy protocols used by health plans preserve the doctor’s right to make treatment decisions on behalf of their patients. Our doctors are the ones who know our medical histories, which means they are the ones who should be making the prescribing recommendations based on their education, training, and medical judgement. In the end, it saves costs because it prevents our healthcare dollars being spent on ineffective treatments. It also protects us from a long process that delays us from getting the medical treatment we need by forcing us to try ineffective medications first.

To accomplish this, the Safe Step Act would amend the Employer Retirement Income Security Act (ERISA) to require group health plans to provide an exception process for any medication step therapy protocol. This also includes a straightforward exemption process that is transparent and will provide patients and doctors a way to request an exemption to step therapy protocols by providing the process on their websites with all accompanying forms, documents, and contact information. This legislation would also require group health plans to respond to an exemption request within 72 hours in all circumstances, and within 24 hours if a person’s life is at risk.

There are five exceptions that this legislation is asking to be included in the exemptions to the protocols. The exemption would be granted if an application clearly states that:

- The person has already tried and failed on the required drug, meaning that they have taken it and it has failed before.
- Delayed treatment will cause irreversible consequences, meaning the drug is reasonably expected to be ineffective, and a delay of effective treatment would lead to severe or irreversible consequences.
- The required drug will cause harm to the patient: the treatment is contraindicated, or the person has already demonstrated an allergy or an adverse reaction.
- The required drug will prevent a patient from working or fulfilling activities of daily living (ADL). This means that the treatment will prevent a person from fulfilling their occupational responsibilities at work or performing ADL, including but not limited to basic everyday activities such as eating, toileting, bathing, dressing, grooming, or moving.
The IPPF works with many different organizations to make sure that the patient’s voice is heard by Congress.

Reduce Prior Authorization Burden
Prior authorization policies requiring advance approval before performing a service to qualify for coverage can negatively impact patient outcomes and quality of life. It interferes with the patient and physician relationship and their ability to determine the best course of treatment. This process typically requires the doctor’s staff to spend the equivalent of two or more days negotiating with insurance companies to approve a medicine or procedure.

This can be an expensive and time-consuming undertaking that takes away resources from patient care and often baselessly questions a doctor’s clinical judgment. For the patient, it can have adverse outcomes due to the added work associated with processing and responding to prior authorization requests. According to the AADA 2020 Prior Authorization Survey, dermatology offices have spent an average of $40,000 on additional staff to help manage the prior authorization process, which takes up to 3.5 hours per day. This time could have been better spent by seeing at least five additional patients per day.

Specific Asks to the Senate:
Sponsor the Improving Seniors Timely Access to Care Act (S. 3018) that would:
- Establish an electronic prior authorization (ePA) program and require Medicare Advantage plans to adopt electronic prior authorization capabilities.
- Require the secretary of Health and Human Services (HHS) to establish a list of items and services eligible for real-time decisions under the Medicare Advantage ePA program.
- Standardize and streamline the prior authorization process.
- Ensure prior authorization requests are reviewed by qualified medical personnel.
- Increase transparency around Medicare Advantage prior authorization and its use.

Specific Asks to the House:
Co-sponsor the Getting Over Length Delays in Care as Required by Doctors (GOLD CARD) Act of 2022 (H.R. 7995). GOLD CARD will exempt providers from requiring prior authorization for a Medicare Advantage plan year if the provider has at least 90% of the prior authorization requests approved in the preceding year. The 90% threshold for this approval includes those granted after appeal.

It will also allow the Medicare Advantage plan to revoke a provider’s GOLD CARD status if at least 90 days of such plan year has elapsed. It would also be revoked if less than 90% of claims submitted would have been approved for prior authorization, and in the case of fewer than ten claims being submitted, less than 90% of the last ten claims submitted would have been approved for prior authorization. This bill would also allow physicians to appeal the GOLD CARD revocations they believe were wrongly decided.

Overall, it was a very educational and busy three-day conference for the 35 patient advocates of the CSD and the 158 dermatologists of the AADA. The IPPF works with many different organizations such as the CSD and the AADA to make sure that the patient’s voice is heard by Congress on issues that matter and affect our community. If you are interested in advocating for the IPPF community at the federal level, either in Washington, DC or in your Congressional member’s home district, we would love to help you! Please email Marc Yale, IPPF Advocacy and Research Coordinator at marc@pemphigus.org, or Becky Strong, IPPF Outreach Director at becky@pemphigus.org for more information.

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.
The goal was to hear from patients about what it’s like to live with rare, autoimmune bullous skin diseases, so the FDA and pharmaceutical companies can understand the patient experience.
2023. The meeting was recorded and is available here pemphigus.org/el-pfdd/.

Specific goals of the meeting were to:
1. Help to identify areas of unmet need of patients
2. Identify the need to develop tools to assess the benefits of patient therapies
3. Raise disease awareness and engage the patient community in sharing their disease experience

The meeting agenda included presentations on P/P by disease experts, remarks from the FDA, patient panel testimonials, patient perspectives, and extensive audience discussion sessions. The topics included:
• Patient-focused drug development at the FDA
• Background: pemphigus and pemphigoid
• Current treatments and limitations
• Disease symptoms and treatments: how they impact the daily lives of patients
• Lack of healthcare treatments, awareness, and education
• Clinical trial experience and meaningful benefit
• Patients’ perspective on available treatments for the disease(s), side effects, and how to improve them
• In addition to patients, speakers included:
  • Dr. Shari Targum, Deputy Director of the Division of Dermatology and Dentistry, Office of Immunology and Inflammation, Office of New Drugs, Center for Drug Evaluation and Research (CDER), US Food and Drug Administration.
  • Dr. Pacal Joly, Rouen University, France
  • Dr. Dedee Murrell, University of NSW, Sydney, Australia
  • Dr. Aimee Payne, University of Pennsylvania, Philadelphia, PA
  • Dr. Victoria Werth, University of Pennsylvania, Philadelphia, PA

In Patrick Dunn’s (IPPF Executive Director) closing remarks, he summarized the emotional meeting and said:

“So, what did we learn today? We learned that both pemphigus and pemphigoid can be severe, life-threatening diseases that cause significant burden on the day-to-day lives of patients. It’s evident that there is a need for treatments that address both the long-term and short-term effects of these diseases.

The current treatment landscape is sparse, with only corticosteroids and rituximab approved for pemphigus vulgaris, and no approved therapies for pemphigoid. This creates access issues for many patients, as drugs must be prescribed off-label.

Perhaps even more significant are the challenges faced by patients in balancing the burden of disease versus the burden of therapies, as the side effects from chronic use of steroids often have a debilitating effect on patients.

It’s evident that there is a need for treatments that address both the long-term and short-term effects of these diseases.

Along with the validated measures, patient experiences can help inform researchers about the physical and psychological impacts of these conditions to help guide investigations into new treatments.

As new therapies are developed and we learn more about these diseases, it is also important to refine the meaning of words like ‘remission’ so that they accurately reflect the lived realities of patients. Due to the side effects of steroids, minimal therapy is often the primary goal in the minds of patients, with the long-term goal of ‘complete remission’ being secondary. Because steroids have such profound effects on people’s daily lives, therapies that are ‘steroid-sparing’ are of great interest to patients. In other words, new therapies don’t have to be perfect, as long as they provide meaningful benefit to a person’s quality of life. Additionally, since we already have sensitive validated assessment tools that measure this, any new tool must include the patient’s perspective into what is most important to them.

It is our hope that [the EL-PFDD] meeting not only clarified the unmet needs of pemphigus and pemphigoid patients, but also highlighted the importance of
the patient voice in the drug development process. It’s imperative that regulators, industry, researchers, clinicians, and advocacy groups hear directly and continually from those who are most impacted by their decisions: the people living with these diseases. Though the challenges are many, there is more reason than ever to be optimistic about the future treatment landscape for pemphigus and pemphigoid. At the IPPF, we’ve observed a steady increase in research in recent years, with more studies and trials in the pipeline. And though [the EL-PFDD] meeting is coming to an end, this can really be the beginning of future collaboration under a common goal: to improve the quality of life for all people affected by pemphigus and pemphigoid.

Over the next several months, we will be preparing a Voice of the Patient Report that will not only summarize the EL-PFDD meeting, but will also publish additional written comments we receive. The report will be sent to the FDA to be used as a reference point for their decisions concerning the approval of potential therapies and treatments for P/P. The report will be available for the public to read on the IPPF website. If you would still like to share your story or make a comment about the EL-PFDD meeting, please email pfdd@pemphigus.org.

Thank you to the patients that bravely shared their stories, our speakers, and our coalition of meeting organizers: PEM Friends from the UK, the Association Pemphigus & Pemphigoïde France, the Pemphigus/Pemphigoid Friends Association from Japan, and the IPPF staff. Additionally, thank you to our sponsors for making the meeting possible: argenx, AstraZeneca, Janssen, and the EveryLife Foundation for Rare Diseases.

Though the challenges are many, there is more reason than ever to be optimistic about the future treatment landscape for pemphigus and pemphigoid.
Patients with Autoimmune Blistering Diseases (AIBD) may require multiple oral, topical, and intravenous drugs to obtain the best clinical outcomes. Obtaining this drug therapy requires the patient to interact with multiple physicians, pharmacists, pharmacies, drug wholesalers, and insurance companies. What is the pharmacist’s role in this process?

In many respects, the pharmacist acts as the coordinator of this process since they function at the intersection of the physician’s prescription, the drug makers’ FDA-approved product, the drug wholesaler, the insurance company’s authorization and payment for the medication, and the patient, for whom all these actions are taken.

CONTINUED
Relative to the patient experience, the pharmacist’s main duties can be categorized into five main areas. Let’s discuss each separately to see how it impacts the patient’s experience and clinical outcome.

1. **Prescription processing:**

This is the main coordinating function the pharmacist manages for the patient. When the prescription is received (usually electronically nowadays), the pharmacist compares the drug ordered to the patient’s diagnosis, history, and physical to see that there is a match and that there are no major issues such as the patient’s allergies, the inventory in the pharmacy or the drug wholesaler at that time, the insurance companies formulary and approved diagnosis that would allow coverage for the drug for the patient that is for their disease. The pharmacist also verifies what the patient’s coverage would be and what the patient’s out-of-pocket costs would be, and verifies that the prescriber is licensed to prescribe in the patient’s state of residence. They also check to verify that the physician is not on any state or federal list of prescribers who are barred from prescribing for many reasons. Finally, the pharmacist tabulates the time needed to accomplish all of these events to assure the availability of the drug and how rapidly the patient needs to receive the drug.

In some instances, for oral generic drugs, this whole process may take 15 minutes or less. But, in other more complex cases that involve costly oral, topical, or intravenous medications, this process may take days or weeks. This may depend on the availability of the drug, the possible need for more patient clinical data to be submitted, additional laboratory testing, etc., that may be required to obtain authorization for the ordered therapy from the insurance company. This protracted process may involve written appeals by the physician/pharmacy or a telephone consultation with the prescribing doctor and the insurance company’s doctors.

2. **Patient support and safety:**

During the many steps in the above process, the pharmacist analyzes multiple issues and data points to assure that the likelihood of clinical, temporal, logistical, and financial harm to the patient is minimized. This may involve checking for allergic responses to ordered medications based on their history, or checking to see that the time needed to complete the above process is consistent with the urgency of the patient’s needs and other planned activities (i.e., other medical procedures or tests, vacations, etc.), and that the out-of-pocket costs that the patient will be facing as a result of the ordered medications are minimized.

3. **Patient education:**

This is an important function where the pharmacist compiles information from various sources and shares with the patient details about drug dosage and dose frequency, expected rapidity of drug effect, drug storage, expected drug side effects, and who to call if an issue occurs. Education may also include issues surrounding home safety, fall prevention, and tips to better manage the other diseases the patient may have relative to the newly ordered drug therapy.

4. **Billing and associated actions with the insurance company:**

One of the most important responsibilities of the pharmacy and pharmacist is their “customer service” functions which mostly encompass the insurance company’s billing on behalf of the patient and adjudicating the patient’s out-of-pocket costs. On the one hand, this function may be completely managed electronically in a few minutes for inexpensive oral medications. Alternatively, this function may take days or weeks for more expensive, infused medications. Interaction between the pharmacy, the payor, and the ordering physician is typically required initially, and maybe in an ongoing fashion if the authorization is denied and an appeal is needed, to obtain the payor’s authorization for payment.
Pharmacists are extremely knowledgeable about the medications you have been prescribed and are traditionally underutilized by patients.

While this is happening, the pharmacist is evaluating the out-of-pocket exposure to the patient for the drug(s) ordered to see if there is anything clinically equivalent that is available that may result in a lesser out-of-pocket cost to the patient. Suppose there is a less costly, clinically equivalent product available. In that case, the pharmacist discusses it with the ordering physician to get the order changed, and then reaches out to the payor for authorization reflecting the new lowered out-of-pocket costs.

Finally, suppose the patient cannot afford their out-of-pocket costs. At the patient’s request, the pharmacy may explore drug company-based and nonprofit foundation-based financial assistance programs for which the patient may qualify. The sum of these programs may make the drug affordable to the patient.

5. Regulatory compliance:
This is a complex concept. It ensures that all the data collected from each patient’s prescription processing and dispensing is properly collected, stored, and reported to the inquiring party. These parties include:
- The state board of pharmacy
- The patient’s insurance company
- Medicare
- Medicaid

The pharmacist must also assure that the prescriber is licensed to prescribe the exact products ordered in the patient’s state and is not on any lists of suspended or disbarred prescribers.

Any of the organizations above may audit the pharmacy’s records. These audits may be known and planned for or may be a surprise in nature. Poor performance in these audits may result in the pharmacy being sanctioned, removed from the payor contract, fined, or closed.

As you can see, there are a lot of actions behind the scenes that make the pharmacist a key player in the healthcare team. Pharmacists are extremely knowledgeable about the medications you have been prescribed and are traditionally underutilized by patients. Their role is often misunderstood. I hope this information has helped you better understand the pharmacist’s important role in your healthcare and well-being.

Dr. Mike Rigas is now retired and was the Chief Clinical Officer, cofounder, and principal at KabaFusion, a nationwide infusion therapy company started in 2010, which is dedicated to managing IVIg, other chronic, and acute therapies.

In 2008, he joined Geisinger Health System as its Associate Chief Innovation Officer for Pharmaceutical Care and Chief Clinical Officer for VITALine CareSite Pharmacy Services.

Before moving to Geisinger, Dr. Rigas served as the Senior Vice President of Clinical Affairs at Crescent Healthcare in Anaheim, CA, a large alternate site pharmacy company specializing in infusion and specialty pharmacy therapies.

He graduated from USC School of Pharmacy with his Pharm.D. degree and completed his residency at UC San Francisco Hospitals and Clinics in 1982. He spent the first 10 years of his career in the hospital arena serving as the Chief Clinical Pharmacist in three different 200+ bed community hospitals. From 1998 to 2002, Dr. Rigas managed pharmacies and nursing operations for a nationwide infusion company with 32 pharmacies in 22 states at the Vice President level.

His areas of expertise include Antimicrobial therapy, Immunoglobulin therapies, nutritional support, inotropic therapies, pain control, improving clinical and financial outcomes, and payor and contract relations. He is also active in the High-Cost Biologic/Specialty Pharmacy and infusion industries. He has worked on standardized coding for infusion billing, universal access via Patient Assistance Plans and managing Medicare Part D issues in front of the California Board of Pharmacy and CMS regional managers. Dr. Rigas holds memberships in the American Society of Hospital Pharmacists, the National Home Infusion Association, the American Academy of Neurology, and the Clinical Immunology Society. He is on the IPPF Board of Directors.
We all know how extremely difficult it is to manage a rare autoimmune disease. Besides experiencing painful and difficult symptoms, we must deal with doctors' offices, insurance companies, pharmacies, and the side effects of drugs. Sometimes, having pemphigus or pemphigoid (P/P) feels like a full-time job.

But what happens when you are actually working full time? The stress of balancing work and illness can be completely overwhelming. For those working P/P patients, it is important to understand what rights exist and what resources are available, especially for those who need to continue to work full time while they are ill.

In the United States, having an autoimmune disease can be considered a disability. Under the American with Disabilities Act (ADA), any medical issue that severely impacts a person’s quality of life or life function is considered a disability, and a person with a disability is entitled to a reasonable accommodation to help assist them in their job.

What is an accommodation?

A reasonable accommodation is any change or adjustment to a job or to the work environment that allows a person with a disability to perform the essential functions of their job. In other words, a reasonable accommodation is when the employer changes the way things are customarily done that allows an individual with a disability to enjoy equal employment opportunities.
Am I Sick Enough for an Accommodation?

An individual can qualify for a reasonable accommodation if they have “a physical or mental impairment that substantially limits one or more major life activity.” So, if you have symptoms of your illness that interfere with a major life activity, such as work, sitting, standing, talking, etc., you may qualify for an accommodation.

A person does not have to “look sick” to be sick. A person may suffer from symptoms like fatigue, brain fog, joint pain, blistering, swelling, unexplained weight changes, inability to concentrate, rapid heart rate, abdominal pain, poor coordination, and other symptoms that are not visible to their employer. Your illness does not need to be visible to be accommodated.

Am I Qualified to Request an Accommodation?

First, an employee with a disability must be able to perform the essential functions of their job with or without an accommodation. Essential functions are job duties that are fundamental to the position. For example, a teacher must be able to work with children, a bus driver must be able to drive, and a butcher must be able to use a knife.

Second, under the ADA, not all employers are legally required to provide accommodations, although smart ones do! Employers who have 15 or more employees are generally legally required to provide reasonable accommodations. Some state and local laws may require that employers with fewer employees provide reasonable accommodations. If you work for a smaller company, check your state or city laws to see what applies in your area.

Nervous about Requesting an Accommodation?

In many cases, requesting an accommodation, for any reason, and especially for an autoimmune disease, can be a difficult process to start. If you are not comfortable with sharing personal information, the process of requesting an accommodation may seem daunting or scary. However, there are two significant benefits to sharing. First and foremost, you may get the accommodation you need to make your job easier and your life better. Second, the pure act of disclosing your health information and requesting an accommodation provides some legal protection, as your employer cannot legally retaliate against you for making such a request or having a disability.

Preparing to Request an Accommodation

There are several ways that you can prepare for requesting an accommodation which will make the whole process easier to manage.

Consider what limitations you are currently experiencing. Do you have difficulty sitting? Getting dressed? Are you in pain? Do you have limited mobility? Do you need time to take care of your blisters? Make a list of these limitations.

Ensure you have the necessary medical documentation from your medical provider. Certain medical providers may be uncomfortable with providing such letters because of the Health Insurance Portability and Accountability Act (HIPAA). However, with your authorization, medical providers can release limited information. Ensure that your medical documentation is sufficient. Make sure your medical documentation is on office letterhead, dated, and signed by your medical provider. You can always supplement any documentation with test results. Make sure that your diagnosis/diagnoses and your limitations are also clear. You may ask your medical provider to include your diagnosis, your current medication, and your limitations. For example, your note could say, “Ms. Smith has pemphigus vulgaris and is being treated with Rituxan®. Ms. Smith is experiencing fatigue, aches, and is managing blistering and open wounds. Ms. Smith also needs to attend weekly physical therapy.”

Don’t overshare. If you have pemphigus, but also have asthma, irritable bowel syndrome (IBS), and diabetes,
you do not need to mention the other diseases unless they are also impacting your ability to work.

Don’t keep relevant details to yourself. If you have multiple illnesses and all of them are impacting your ability to successfully work, be forthcoming about the aspects of those diseases that are impacting your work.

Know what kind of accommodation(s) you need before you put in your request. Possible accommodations could be remote work, an adjusted schedule, a different chair, a private area to work, time off for doctor appointments, breaks, late arrival, access to a bathroom, change in uniform requirements, medical leave, etc. Sometimes, a small accommodation such as the ability to take a later train or wear more comfortable clothes will make a huge difference in your success at work.

Put In the Application!

If your job has a specific process to apply for accommodations, follow that process. If not, you are advised to submit your request in writing, although a verbal request is also considered to be valid.

Once your request is received, your employer should schedule an interactive meeting. This means that your employer is required to meet with you and discuss your request. (Of course, if you request a specific accommodation and your employer is happy to approve it, a meeting is not necessary to effectuate that accommodation.)

When your employer schedules an interactive meeting, try not to stress about it (it might make your disease worse, anyway!). You’re prepared! Attend the meeting with your request, your documentation, and any other information that may be necessary (e.g., medical tests, list of appointments, etc.). If you are uncomfortable with speaking about these details, you can simply hand your employer the request and let them start the meeting.

Your employer will likely offer accommodations that are reasonable for the worksite and your job title. If specific aspects of your job demand that you must be in-person, such as a nurse, waiter, or truck driver, then it would generally be unreasonable to give you remote work. But your employer may be able to provide you with a lighter schedule, time off for appointments, a change in uniform, or another accommodation that will make it easier to do your job with your condition.

If you request a specific accommodation and your employer refuses, they are required to explain why they are refusing. If you are aware of another employee with a similar accommodation to your request who is doing the same job, you may want to reference that accommodation. While each person and situation are different, an employer cannot deny a request without a reasonable explanation for such a denial (e.g., having both employees out at once will be a burden to the employer, there is only one private office in the building, etc.)

Unfortunately, there may be a situation where no reasonable accommodation will provide you with the relief you need to do your job during your current stage of illness. During this interactive meeting, you can also request (or they can offer) a medical leave of absence. You may be eligible for a leave under the Family Medical and Leave Act (FMLA) or through your office or union. Your employer can also discuss disability retirement options if you inquire.

Once the meeting is over, your employer should follow up, in writing, with a summary of the reasonable accommodation, the length of the accommodation, and any other guideline(s) related to the accommodation. If you do not get this, it is advisable to make a specific request for it, also in writing.

Congratulations, You’ve Been Accommodated!

Once you have your accommodation, there are a few things to remember to ensure that you get the most out of your accommodation.

Set a reminder for when your accommodation will expire. If you will need to extend your accommodation, you should seek to request an extension about two to four weeks before your initial accommodation expires.

Do your job to the best of your ability and keep your employer informed of any changes. Employers have no reason to disrupt your accommodation if it makes you a more effective employee!

Be prepared to work with your supervisor if coworkers have questions or concerns. Colleagues may be jealous or annoyed that you now have a better parking spot or get to wear jeans to work. It is not your responsibility to share why, but you are welcome to do so if you feel comfortable. Your employer can explain that you have
an accommodation, but they should not be sharing your medical information.

Not all accommodations work. Maybe you were granted a flexible schedule, but you need more time off. Or maybe you got a new chair, but you also need a new desk. Maybe the prednisone has weakened your legs and you now have mobility issues. Or maybe you are working remotely for six months after your infusion, but the Rituxan® starts working in one.

If your accommodation isn’t working for you or you need a change for any reason, you can ask your employer for a review or another interactive meeting to discuss the accommodation. Again, be prepared with any updated medical documentation, a list of your current limitations, and your updated request.

If your employer feels that the accommodation isn’t working, they can also ask for a review. For example, if your employer feels that you are not getting your work done or that the accommodation is overly burdensome to the employer, they can ask to review it at any point. If they do, try to breathe and make sure you have the documentation to support your current needs.

Issues or Questions?

You can find a wealth of information on accommodations specific to each disease or disability through the US Department of Labor’s Job Accommodation Network (JAN) External Site (https://askjan.org/) or the ADA National Network (https://adata.org/). You can also seek guidance through your employer’s Equal Employment Opportunity (EEO) office.

If you are not getting the support you need within your company or believe you have been the victim of disability discrimination, you may want to seek out the EEO office in your city or state, or the Equal Employment Opportunity Commission (EEOC) on the federal level.

You can file claims through most of these offices. You can also seek guidance from an employment attorney in your area.

At the end of the day, small changes to your work environment can greatly increase your quality of life if you need to continue working with an autoimmune disease.

Katherine R. has been an employment attorney for 20 years and is a member of the NY and DC bars. She is also a pemphigus vulgaris patient.
Thank you to all the IPPF volunteers who have supported our mission of improving the quality of life for all those affected by pemphigus and pemphigoid.
Dr. Fairley is the John S. Strauss Professor and Chair of the Department of Dermatology at the University of Iowa. She completed medical school, residency training, and a research fellowship at the University of Michigan. Dr. Fairley’s interests include autoimmune skin blistering diseases, such as bullous pemphigoid. She has published over 130 articles and book chapters. Dr. Fairley is a diplomate of the American Board of Dermatology and a member of the American Academy of Dermatology and the Society for Investigative Dermatology, for which she serves as a member of the Board of Directors. Her certifications are with the American Board of Dermatology, Iowa Board of Medicine, Michigan Board of Medicine, and the National Board of Medical Examiners. Dr. Fairley also completed an NIH-sponsored research fellowship in cell physiology at the University of Michigan, Ann Arbor.

**How did you become interested in P/P?**

One of my mentors was working in the area of pemphigus and pemphigoid, and we collaborated on a lab project. I realized it was a lot more fun to work on something in the lab that translated to improving patient care, so [I] made the switch to studying P/P almost 30 years ago now!

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

The earliest part of the journey is oftentimes the worst. Stick with it and work with your doctors, and it will get better. While it may take a while to achieve remission you will get there, and you will control your disease rather than having it control your life.

**What can patients do to better advocate for themselves?**

The best way you can advocate for yourself is to communicate with your provider. Write out your questions if doctors’ visits make you nervous, or use one of the online portals like MyChart. If a medicine or procedure makes you nervous, let your provider know so that they can explain things thoroughly. On the other hand, if you want to stop a medication, let your provider know and give them your reasons. Most of the medications we have for P/P don’t provide immediate relief, so on the other side don’t stop prematurely.

**What is one fun fact about yourself?**

I grew up in Michigan on ice skates. I still love to skate with my grandsons, and my goal is always to be the oldest person on the ice.
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