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Message from the Executive Director

Dear Reader,

It’s once again the season when we spend a lot of time thinking about... time. We’re looking back to assess if and how the IPPF achieved its goals for 2023. We’re looking forward to 2024 and beyond, planning how we can best serve this community. We’re also wishing we could simply pause time, even for a moment, to catch our collective breath! I’m sure this is a sentiment that many people reading this letter also share.

Today, as I write this, the calendar just flipped to December. As I look back over the past year, I believe the work the IPPF has done can be summed up using the slogan from October’s Patient Education Conference: “Empowering the Patient Voice.” From landmark events, such as our Externally-Led Patient Focused Drug Development meeting held in January for the US FDA, to our annual programming and Patient Education Conference, elevating the voice of pemphigus and pemphigoid patients to a place of prominence is one of the most important things the IPPF can do.

But what, exactly, do we mean when we say this? The “patient voice” can be both the literal voice of individual patients, as well as the collective voice—or opinions—of the broader patient community. As the largest pemphigus and pemphigoid (P/P) patient advocacy group in the world, the IPPF is in a unique position to gather the opinions of a significant number of patients. This is especially important when dealing with rare diseases like P/P, where patients in any one area are few and far between.

Over time, the IPPF has collected enough information through surveys, patient stories, and direct conversations, that we are able to identify the top concerns of people living with P/P. This information not only helps us create better resources, it also gives us a tremendous amount of credibility among doctors; researchers; pharmaceutical companies and other industry; legislators; and regulatory agencies like the FDA. In the past, organizations like the IPPF had to fight especially hard to be considered partners by such entities. Thankfully, times have changed, and many of these entities now recognize how essential it is to include opinions from patients in their work. However, there is still much to be improved on this front. Because of this, the IPPF spends a lot of time reminding its partners that true progress for patients can only occur when patients are engaged every step of the way. After all, what good is a new treatment if it doesn’t address the things that actually matter to the people living with these diseases?

Elevating the patient voice can take on many forms. It’s publishing patient stories in this magazine. It’s inviting patients to give lectures to dental and medical students about their experience. It’s making sure legislators are aware of potential bills that could have dramatic impacts on the health and finances of P/P patients. It’s publishing educational resources that patients can use to become self-advocates. It’s assisting industry partners in recruiting patients to provide feedback in the drug development process before potential new therapies reach the clinical trial stage. Most of all, it’s making sure that improving the quality of life for all people affected by P/P is the reason we take on this work day in and day out.

With that as our north star, our direction is true, our path is clear, and time is on our side.

Patrick Dunn, IPPF Executive Director
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www.pemphigus.org
Events Update

Rare Across America
Virtual and in-person advocacy meetings were held between August 7-18 with Members of Congress for Rare Across America 2023. The IPPF and Rare Disease Legislative Advocates helped organize meetings with congressional representatives in support of advocating for rare disease legislation and policies. During the week, there were 268 meetings held and a total of 447 advocates from 48 states, Washington DC, and Puerto Rico.

American Academy of Dermatology Association (AADA) Legislative Conference
IPPF Outreach Director, Becky Strong, attended the annual American Academy of Dermatology Association Legislative Conference and Hill Day from September 10-12 in Washington, DC. The meeting gathered dermatologists, residents, patient advocates, healthcare policy experts, and political insiders to advocate for the specialty and fight for Medicare reform on Capitol Hill.

Global Genes RARE Advocacy Summit
Each year, Global Genes convenes one of the world’s largest gatherings of rare disease patients, caregivers, advocates, healthcare professionals, researchers, partners, and allies at the RARE Advocacy Summit. This year, Patrick Dunn, IPPF Executive Director, attended the event in San Diego, CA from September 19-21. The sessions provided attendees with insights about the latest in rare disease innovations, best practices for advocating on an individual and organizational level, and actionable strategies to accelerate change.

Chan Zuckerberg Initiative (CZI) Science in Society Annual Meeting
The Chan Zuckerberg Initiatives’ Science in Society program works with patient communities, scientists, policymakers, advocates, and philanthropic partners to foster meaningful integration of science and society. Patrick Dunn, IPPF Executive Director, attended this year’s annual meeting from September 20-23 which focused on the role of patients driving scientific progress in rare disease. The meeting was described by CZI as “an opportunity for researchers, patients, and stakeholders to pool knowledge, forge collaborations and address challenges and opportunities in diagnosis, equity, and the development of treatments and cures.”

European Academy of Dermatology and Venereology (EADV) Congress
The EADV annual international Congress was held in Berlin, Germany from October 11-14. Marc Yale, IPPF Research and Advocacy Coordinator, and Patrick Dunn, IPPF Executive Director, both attended the event, and the IPPF was asked to permanently join the Autoimmune Bullous Disease Taskforce. The meeting focused on dermatology and venereology, and provided a platform for researchers, clinicians, and industry professionals to exchange knowledge, network, and present their latest research findings.

National Organization for Rare Disorders (NORD) Breakthrough Summit
Becky Strong, IPPF Outreach Director, attended this year’s event from October 16-17 in Washington, DC. NORD described the event as “an unparalleled opportunity to discuss complex, multi-faceted topics with all rare disease stakeholders—medical and academic experts, regulators and policymakers, innovators, patients, caregivers, and industry leaders.”
Patrick Dunn, IPPF Executive Director, attended the Chan Zuckerberg Initiative (CZI) Science in Society Annual Meeting in September.

Marc Yale, IPPF Advocacy and Research Coordinator, at the EADV Congress in Berlin, Germany.

Becky Strong, IPPF Outreach Director, attended the AADA Legislative Conference in Washington, DC.

Patrick Dunn, IPPF Executive Director, and Marc Yale, IPPF Advocacy and Research Coordinator, attended the EADV Congress in October.
Empowering the Patient Voice at the IPPF Patient Education Conference

Anna Lane

Each year, when the weather cools down and my children have gone back to school, it means two things: fall is here and it’s time for the IPPF Patient Education Conference. It’s one of the most beautiful seasons where I live, in Colorado, and it coincides with the excitement and anticipation of the annual conference. All the IPPF staff take on a variety of roles for the preparation of the conference, and we come together as a strong team knowing that the impact of the conference can be vital to patients’ lives and well-being.

The conference is an action-packed event with speakers that are experts in the pemphigus and pemphigoid (P/P) field, and this year patient panelists were invited to share their perspectives during the Q&A section of the presentations. The patient perspective was one of the focus areas of the conference to go along with this year’s theme: Empowering the Patient Voice.

The patient voice has been one of the most consistent themes for the IPPF in 2023. In January we held an Externally Led Patient Focused Drug Development (EL-PFDD) (www.pemphigus.org/el-pfdd/) meeting with the Food and Drug Administration (FDA). The goals of the meeting were to help identify areas of unmet need of patients, identify the need to develop tools to assess the benefits of patient therapies, raise disease awareness, and engage the patient community in sharing their disease experience. We are currently working on the finalized report that will be sent to the FDA and serve as a reference point for their decisions concerning the approval of potential medicines for pemphigus and pemphigoid (P/P).

In addition to the EL-PFDD meeting in January, we have spent the year focusing our efforts on amplifying the patient voice by encouraging patients to participate in clinical trials and become rare disease advocates through legislative policies; holding monthly Patient Education Series webinars; raising awareness at universities through the Biopsies Save Lives program; traveling in the United States and internationally to advocate for P/P with partner organizations, and more. We recently published the revised IPPF Guide to Pemphigus and Pemphigoid and released a new search function for our Find a Doctor webpage. These resources are available on our website: pemphigus.org. It is our hope that these initiatives help patients feel empowered to use their voice, share their story, and impact the future of living with a rare disease.

This year’s three-day conference kicked off on October 27 with
a powerful patient story by Marc Friedman, a pemphigus vulgaris (PV) and pemphigus foliaceus (PF) patient. Throughout the day there were presentations on P/P Disease Overview and Subtypes, Being Newly Diagnosed, the Trauma of Skin Disease and an Embodied Approach to Healing, Steroids and Managing Their Side Effects, Biologic Agents: IVIg and Rituximab, Nutrition, and an IPPF Peer Coach panel.

Saturday started with a session on Caring for Your Skin and Wounds, followed by another busy day of presentations including Making Informed Decisions with Your Healthcare Provider, Antibody Recycling for Potential Treatment of Pemphigus Vulgaris, Immunosuppressive and Anti-inflammatory Agents, Biosimilars, Oral Disease and Care, and New Treatment Strategies for Ocular Disease. The final session of the day on Saturday was a participatory event that connected attendees on Zoom!

The sessions on the final day of the conference included Emerging Therapies in P/P, Understanding the Role of Patient Data in Drug Development, Patients and Clinical Trials, Co-Pay Assistance Programs, and Advocating for Policy Change with Decision-Makers. Thank you to our wonderful attendees from around the world that interacted with us on the conference platform and joined each session.

As we look forward to 2024 and the IPPF’s 30th anniversary, we are hopeful that we will be able to welcome attendees in person at next year’s conference. We will be working on a hybrid event that will also include a virtual option for attendees that aren’t able to attend. Please be on the lookout in the upcoming months for announcements about next year’s conference.

Anna Lane is the IPPF Communications and Marketing Manager. She lives in Denver, CO with her family.
We’re excited to share a new resource with our community: The IPPF Guide to Pemphigus and Pemphigoid (https://www.pemphigus.org/patient-resources/). The guide is intended to provide medically-reviewed information relevant to the most common questions people have when first diagnosed with pemphigus and pemphigoid (P/P), as well as educational information about ongoing disease management and treatment options.

Through this guide and other IPPF resources, we hope to empower the community with essential knowledge that can make living with P/P much more bearable. In addition to English, Spanish and French translations of the guide are available to read and download on our website.

“At this stage in your disease journey, there’s a good chance you are feeling many emotions and worrying about what this all means for your future. This is normal, as it’s common for people diagnosed with any chronic illness to experience depression, stress, anxiety, anger, confusion, and/or fear. Trying to understand pemphigus and pemphigoid and their treatments can be overwhelming, not to mention figuring out how factors like diet, sleep, and lifestyle may play a part in your individual experience. With this in mind, I assure you that the IPPF is here to support you on this journey,” wrote Patrick Dunn, IPPF Executive Director, in the publication’s introduction.

*The information in the guide has been reviewed by the Education and Patient Support working group of the IPPF’s Medical Advisory Council. The IPPF does not endorse any drugs, treatments, or products in this guide. Information is provided for informational purposes only. Because the symptoms and severity of pemphigus and pemphigoid vary among individuals, discuss all drugs and treatments with a patient’s physician(s) for proper evaluation, treatment, and care.
Diagnosed with Bullous Pemphigoid? (BP)

Learn about a study of a potential new 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

Becky Strong is the Outreach Director of the IPPF and is living (not just surviving) with pemphigus vulgaris (PV), a life-threatening autoimmune blistering disease. I first met Becky on a Google Meet video call after we were paired together through the Global Genes RARE Compassion Program, which provides an opportunity for medical students to connect and partner with individuals living with rare diseases. The goal of the program is to help support and develop compassionate physicians around the globe. By emphasizing personal experience and human connection, the program facilitates a constructive dialogue about what happens on both sides of an exam room. This experience has given me invaluable insight into the stressors that individuals with rare diseases must endure during their diagnostic journey. Behind a diagnosis, there is potential physical and emotional pain, medical trauma/gaslighting, and social ramifications of the disease that physicians need to be aware of and address. My conversations with Becky have helped ensure that in the future I will always enter an exam room being patient-oriented, humble, and open-minded.

During our partnership, I learned a lot about PV; statistics on risk factors, mortality, morbidity, treatments, and what is happening to the skin at a cellular level. The most important thing I’ve learned is how this disease and its diagnosis can impact an individual’s life. Apart from being rare, PV can mimic other diseases, and its involvement of multiple body parts can make it extremely difficult to diagnose. This often leads health professionals down a seemingly never-ending rabbit hole of tests and procedures.

In medical school, we are often taught about rare diseases, but it is easy to put a mental asterisk by them and assume that we’ll either never see them in practice, or when we do, they will be a textbook presentation. Physicians sometimes fall into heuristic autopilot and rely on what the most likely diagnosis is. In other words, “when you hear hoofbeats, think horses not zebras.” During the preclinical years of medical school, it can be easy to get lost in a sea of statistics and buzzwords. This experience has helped lift me out of the textbooks and expose me to the human side of medicine. It is important to remember that every picture we see in a textbook is a picture of a person that has a story behind them. In addition, we need to remember that patients are not just statistics, and that reality has a way of smudging or completely ignoring the tidy boxes we try to draw around diseases.

Navigating medicine should be a marriage between Occam’s Razor: “The simplest explanation is usually the best one,” and Hickam’s Dictum: “A patient can have as many diseases as they damn well please.” Providing patients with the correct diagnosis and treatment is important, but it is also important to let our patients teach us about their disease. A strong patient-physician partnership benefits everyone.

After participating in discussions with other student members in the RARE Compassion Program, I am extremely excited to join these future physicians as they begin entering the healthcare system, and to see everything that we can accomplish for, and with, our patients. Thank you to Becky, the IPPF, and the Global Genes Team, in particular Rachel Barron (Senior Manager, Health Equity) and Nicholas Ehly (Program Coordinator), for their support throughout this experience.

Harrison Loftus is a second-year medical student at Central Michigan University School of Medicine. In his free time, he enjoys gardening, playing tennis, going to the movies, and traveling with his dog, Oliver.
Congressman Dunn (R-FL), Congresswoman Doris Matsui (D-CA) and Reps. Mike Thompson (D-CA), Mike Kelly (R-PA), and Markwayne Mullin (R-OK) introduced the Providing Realistic Opportunity to Equal and Comparable Treatment for Rare (“PROTECT Rare”) Act, legislation to support patients with rare and ultra-rare diseases in getting evidence-based, medically necessary care covered by their health insurance.

The bill permits Medicare and Medicaid to use clinical guidelines and peer-reviewed literature to allow for coverage of rare disease treatments.

“We look forward to the enactment of this important legislation, and for the work the sponsors have put into this so far. We are confident that this will make a significant difference in the lives of the rare disease patients we represent and work with every day,” said Haystack Project CEO, Chevese Turner.

This bill is supported by more than 60 rare disease organizations.

“When I needed my off-label drug for Immune Thrombocytopenia, I had to jump through several hoops. Insurance kept denying the treatment, not even sending it to the proper third-party reviewers. They kept trying to push me to take IVIG infusions every other week, which were not working and were billing at $175,000 per treatment! There was also no formal PA or appeals process my doctor’s office could leverage. I ended up almost in critical care because my platelets dropped low enough to put me at serious risk of potentially fatal internal bleeding. Because this process took so long, it caused disease progression in a way that made recovery more difficult. Totally avoidable if we had proper protocols in place for rare disease patients to access off-label treatments. I applaud Dr. Dunn for the work he is leading on the PROTECT Rare Act. It can’t come soon enough! When I learned he was going to tackle this, it made me so proud to call Dr. Dunn my Congressman!” said Candace Lerman, from Tallahassee.

The bill will also require private payers to create an expedited review pathway for formulary exception, reconsideration, and/or appeal of any denial of coverage for a drug or biological prescribed for a patient with a rare disorder.

“Although there is only one FDA-approved therapy for my rare condition, pemphigus, there are several other off-label drugs that are used as standard of care for patients like me,” said Janet Segall, a constituent of Congresswoman Matsui. “Unfortunately, the off-label treatments are not covered. Also, there isn’t even an appeal process that’s easily accessible, even though my doctors tell me these treatments are standard of care for my disease. In the meantime, all the delays make my symptoms worse, cause a lot of pain, and make things near impossible financially. The PROTECT Rare Act will give rare disease patients like me access to the treatments my doctors think is best for me and are considered medically acceptable for pemphigus. This will help me and so many other patients, so thank you for tackling this important issue for all of us. Living with a rare disease is hard enough without having to be denied access to the treatments I need.”

Ask your Representative to co-sponsor the bill: https://www.votervoice.net/HaystackProject/Campaigns/108649/Respond.
Biosimilars 101 for Pemphigus and Pemphigoid Patients

Michael Rigas, PharmD
There are states and patient support groups advocating to end these practices so that more patients can experience lower out-of-pocket costs, as was intended to be part of the biosimilar concept.

**History: Biosimilars are also known as “follow-on biologics” or “subsequent entry biologics”**

The Biologics Price Competition and Innovation Act (BPCI) was introduced by Senator Edward Kennedy in 2007. It was passed by Congress and signed by President Obama in 2010 as part of the Patient Protection and Affordable Care Act (ACA) (Center for Drug Evaluation and Research, 2016). The intent of the Act was to lower prescription costs to patients by having biosimilars priced 25-35% less than innovator drugs.

Biosimilars include large molecule drugs that are most commonly injectables or infused drugs. They are Food and Drug Administration (FDA)-approved copies of the original “innovator” drug product that are allowed to be licensed and approved by the FDA once the innovator product’s patent expires. Well-known oral generic drugs are small molecule drugs and typically taken orally. Biosimilars are large molecule drugs and are typically given via the intravenous or muscular route. The first drug that was FDA-approved as a biosimilar was a version of Neupogen® called ZARXIO in 2015. The most significant biosimilar available for pemphigus and pemphigoid (P/P) patients is the drug rituximab (innovator named Rituxan®), which now has three available approved biosimilar products.

3. Must be similar in their pharmacokinetics (how the drug is absorbed and eliminated by the body).
4. Must be similar in their pharmacodynamics (how the drug affects the body).
5. Must be similar in their safety, purity, and potency.
6. Must be FDA-approved for one or more of the indications that the FDA approved for the innovator product.
7. Most commonly, the biosimilar product’s acquisition cost is 20-30% less than the innovator product, which is supposed to have a cost-reducing impact on insurance companies and patients.
8. Currently, there are over one hundred biosimilars approved or in the process of approval for use in the US. Please see this link (https://www.fda.gov/drugs/biosimilars/biosimilar-product-information) for a list of the currently FDA-approved biosimilars and which ones are in the application process with the FDA. (Center for Drug Evaluation and Research, 2023)

**The concept of “similarity” includes these principles which were enacted and are enforced by the FDA:**

1. Any differences between the biosimilar and the innovator products must not be clinically significant.
2. Must be similar in their ability to cause immunogenicity (meaning they have the ability of therapeutic protein products to stimulate an immune response in the patient).

**Cost reduction to the American healthcare system**

As of late 2022, there were 39 approved biosimilars of 11 previously approved innovator products, with 22 biosimilars currently available on the market. It is estimated that biosimilars saved over $7 billion in 2021. It has also been shown that patient access to medications has improved for every drug with an available biosimilar. (Association for Accessible Medicines, 2022)
Patient out-of-pocket costs

The cost savings impact of biosimilars in the US has not had the expected reducing impact on prices (due to many factors, which will be discussed below). Thus, many regulatory updates to the ACA have been proposed. The main issue has been the creativity shown by the innovator product’s drug makers and the insurance companies to manipulate the market pricing structure to make the most money by forcing patients to use the innovator product or a more expensive biosimilar product, meaning patients may be forced to pay more out-of-pocket than if they were able to use the least expensive biosimilar product.

Traditionally, generic and biosimilar drugs are priced 25–35% less than the innovator product. Insurance companies then pass these lesser acquisition prices to the patients in the form of lower out-of-pocket costs to the patient. However, many innovator product companies offer significant rebates to the insurance company that cause them to prefer these innovator products to the less costly biosimilar products. Patients are then forced to pay higher out-of-pocket costs with the innovator’s product. This innovator product rebate program circumvents the original intent of the biosimilar model concept. There are states and patient support groups advocating to end these practices so that more patients can experience lower out-of-pocket costs, as was intended to be part of the biosimilar concept.

Prescribing physician

Biosimilars may adversely impact physicians since they may not have control over which products are approved for their patients (innovator products vs. biosimilar products) since individual insurance companies’ strategies vary dramatically. For example, a prescribing physician office with 20 patients that may need rituximab (the innovator product is Rituxan®) as part of their autoimmune blistering disease treatment plan may wind up with five patients on each of the four (one innovator and three biosimilars) available products. This presents a complex situation for the prescribing physician, who may face various concerns and responses from their patients about the drugs they are required to take by their insurance company.

Insurance companies

Insurance companies have an excellent opportunity to create strategies that can save money for patients by encouraging them to use biosimilars. Alternatively, insurance companies can choose to make more money for themselves by obtaining rebates from innovator brand drug makers. This may then require patients to use the innovator product at a higher cost to the patient. Either way, the process is complex and time consuming for insurance companies and patients, alike. These policies are also very transient and may need to change whenever a new biosimilar product hits the market or as contracts and negotiations change yearly.

Patients and caregivers

The biosimilar concept significantly impacts patients and caregivers since they may be able to save out-of-pocket costs by using the least expensive biosimilar product instead of using the brand-name drug. However, based on some of the above-mentioned strategies, patients may have to pay higher out-of-pocket costs than expected if they are prescribed a drug other than the lowest cost biosimilar. Also, since patients might be on these drugs for a significant period of time, they may be forced to switch between products and experience a change in out-of-pocket costs as insurance companies update their formularies each year. (Note: Each payor has a drug formulary listing the drugs they prefer their patients to use, and which also explains the various extra or lesser costs
For patients and their families involved with drugs for which biosimilars are available, it is important to consider these issues with their physician, insurance company, and pharmacy.

the patient may qualify for if they do or don’t use the payors preferred formulary drugs.)

**Pharmacies**

Biosimilars challenge pharmacies since they must order and stock many versions of the same drug. They must arrange for the proper cost-effective purchasing of each product, have the ability to sell it to the insurance company at a reasonable profit, and be able to clinically manage patients on multiple versions of the same drug. Also, Boards of Pharmacy have enacted rules for the use of biosimilars. Typically, most states require a pharmacy to notify and obtain consent from the doctor when a biosimilar is substituted for an innovator product at the request of their payor or as a result of product availability to the pharmacy.

**Suggestions for navigating a biosimilar future**

For patients and their families involved with drugs for which biosimilars are available, it is important to consider these issues with their physician, insurance company, and pharmacy. The best way to obtain the best financial outcome from the biosimilar concept is to always be on the offensive. This involves knowing these critical facts about biosimilars that may be available. Creating a spreadsheet that includes the following information may be the best way to coordinate this complex data:

1. Which innovator drugs are currently available as biosimilars for your disease?
2. Contact your insurance company to see which biosimilars or innovator products are on their preferred formulary for your diagnosis.
3. Look up typical acquisition pricing for these drugs online so you know the acquisition cost differences between the innovator product and biosimilars. Less acquisition cost usually equates to less out-of-pocket costs for patients.
4. For each biosimilar and innovator product that might be an option for your disease and with your payor, determine available financial assistance program options by checking the drug’s website.
5. Ask your prescribing physician which innovator brand or biosimilar they prefer so you can compare with what your payor prefers and its cost.
6. Ask your pharmacist whether these innovator products or biosimilars require special purchasing actions, purchasing contracts, payor contracts, training, or Risk Evaluation and Mitigation Strategy programs. The presence of any of these items may make the innovator or biosimilar product hard to get or not obtainable by your pharmacy.

For more information about biosimilars, visit the following IPPF patient resources:


**References**

The oral cavity is the gateway to the rest of the body and is reflective of our overall health, both mentally and physically. It allows us to communicate, smile, laugh, and properly nourish ourselves. In most pemphigus and pemphigoid (P/P) patients this becomes compromised. While every individual with P/P has uniquely different symptoms and severity, in a significant number of cases, oral manifestations are the first sign of disease—with painful blistering and lesions being the primary complaint. Learning that you have P/P and adjusting to your new lifestyle can be a stressful and discouraging experience. When you aren’t able to speak or chew in ways that you could before, you may have no choice but to cut your favorite foods from your diet, or avoid eating meals with friends and family due to the debilitating presentation of symptoms. Through the guidance and tips outlined below, I hope to alleviate some of the burden associated with P/P oral symptoms.

Optimal management of P/P involves a combination of medical treatment, oral care, and continuous monitoring by dental professionals familiar with P/P. In addition, it’s beneficial to consult with other members of your healthcare team such as a dermatologist, primary care physician, and/or oral medicine specialist. Good oral hygiene practices are critical. It is recommended to use “extra soft bristle” toothbrushes and mild, nonalcoholic mouthwashes to minimize irritation and prevent infections. Patients should brush, at a minimum, once in the morning after breakfast and at night before bed. Of course, regular visits to the dentist are important as well for monitoring, but daily at-home care plays a large role in managing the risk of secondary infection, cavities, and gum disease (periodontal disease).

Sometimes even with proper oral hygiene, patients find themselves prone to cavities. This can be due to a lack of saliva present in the oral cavity (called xerostomia) which can be the result of taking certain medications. The first line of defense in many P/P cases is the prescription of corticosteroids and immunosuppressive medications for systemic treatment. These medications specifically target the body’s immune system and diminish the response since P/P are autoimmune diseases.
While this has an overall healing effect on the condition, it can further exacerbate the presentation of oral disease. Corticosteroids and other immunosuppressive medications can lead to dry mouth and impact the body’s ability to heal. Ways in which patients can address these subsequent challenges include using saliva substitutes as lubricants, as well as frequently drinking water to maintain oral moisture. The body’s weakened immune system further emphasizes the importance of regular visits to the dentist and consistent, gentle, at-home care.

Consuming foods and fluids can be a challenge with P/P. The most helpful and basic ways to minimize development of mucosal ulcerations and erosions are to select soft and liquid nutrient-rich foods like protein shakes, smoothies, yogurt, lukewarm soups, and beverages, while it may be best to avoid hot, crunchy, and/or spicy foods and fluids. Rinse your mouth with water and a nonalcoholic fluoride rinse after meals to minimize your risk of caries development (a common chronic bacterial infection in the tooth) and brush within 20 minutes of a meal.

Overall, patients should anticipate a multidisciplinary approach to their care. It’s important to understand that while none of this is a “quick fix,” and it may take some time to figure out what works best for you, with diligence and patience people with P/P are able to get their conditions under control.

Madison Costello is a third-year dental student at the Rutgers School of Dental Medicine (RSDM). She graduated from Lafayette College in 2021 with a B.S. in Neuroscience and a minor in Art. Her time spent in the research lab, working in private practice, and in the classroom demonstrated to her the deeply touching impact that case outcomes can have on patients when a dentist has a strong dedication to service, delicate handiwork, and esthetic sensibility. To Madison, dentistry embodies both her interdisciplinary interests and passion for helping others.
Whether you are newly diagnosed with pemphigus or pemphigoid (P/P) or you’ve been living with it for a while, medical visits can be overwhelming. There are many options to manage and treat P/P, so two patients with the same disease may receive different recommendations. Doctors learn in medical school that medicine is an art, and there are many factors, such as age, other medical problems, allergies, and support at home, that may help your doctor tailor the best treatment for you. It is important to also remember that you know your skin and body better than anyone else. As a person with P/P, you have the right to make fully-informed choices about your health. Most doctors are happy to provide any information you need to make those decisions, called patient-centered care. We’ve put together a list of ways to advocate for yourself with your doctor.

Come prepared with all your health information.

Make sure to get any lab orders completed before you come to your appointment. Bring any medical records such as office visit notes, lab results, or pathology results from your previous doctors who have treated you. Write down and/or take pictures of your symptoms (and when they occurred) and all the past and current medications (pills, creams, shots) you have taken or used. This information is important for the doctor’s assessment.
of your condition and any decision-making that needs to happen.

**Bring a written list of questions.**

Appointments can go by quickly and sometimes you are so busy talking to your doctor, you may forget to ask all of your questions. Write your questions down as they come up prior to your visit on a piece of paper or electronically on your phone so you can go over them during your visit. Here are some common questions that may help you get started:

- What is the natural course of this disease?
- What may have caused this disease?
- How many patients with my disease have you treated?
- What are the risks and benefits involved with each treatment option?
- For each treatment option, when should I expect to start noticing improvement and how much improvement is expected? How can I best contact you if my disease gets worse or I have a question?
- What are signs of infection or disease worsening that I should be aware of?
- What symptoms warrant immediate attention by a medical professional?

**Don’t be afraid to take charge of your visit.**

Slow your doctor down to ask follow-up questions or to process any information they’ve shared. If something comes up after your appointment, many doctors’ offices have an online patient portal that you can use to contact your doctor in-between appointments. Oftentimes, you can even send photos of your skin through the portal. Ask the office staff to help set this up for you.

**Use your doctor to better understand your disease.**

Ask your doctor to explain the disease in a simple way. You can even ask them to draw or show you a picture of what is happening with your skin. They may recommend a website or print material for you to do some additional reading at home. Doctors not only treat disease, but also serve as valuable resources for patients to gain a better understanding of their disease.

**Bring a trusted friend or family member to the appointment.**

There is so much to talk about at your appointment, and research shows that people only remember 20-50% of everything they talk about after a doctor’s visit. If you don’t have a friend or family member to bring, there are professional patient advocates that you can hire to help you at the appointment, too. Ask your friend, family member, or advocate to take written notes for you so you can focus on the conversation with your healthcare provider. Here are some suggestions to write down:

- Names of medications and their side effects.
- How will you receive the medications? Will you pick them up at the pharmacy? Will they be mailed to you? Will you have to go to an infusion center to receive the drug?
- Labs needed before the next appointment.
- Date of your follow-up appointment if needed.

It is important to also remember that you know your skin and body better than anyone else. As a person with P/P, you have the right to make fully-informed choices about your health.

**Try the repeat-back method.**

Ask your doctor if you can repeat your instructions back to them as you understand them. Research shows that this may help you remember the information later, and you can make sure that both of you are on the same page.

**Understand wound care and optimal skin protection.**

Pemphigus and pemphigoid often lead to wounds and breakage in the skin. Ask which products you can use to help your skin heal and protect it from infection. Share which cleansers, moisturizers, and ointments you are using at home with your doctor and make sure they approve of them.

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Talk about insurance coverage and the ability to pay for treatments (including wound care).

Pemphigus vulgaris (PV) has one Food and Drug Administration (FDA)-approved first-line medication: Rituxan® (rituximab). Bullous pemphigoid (BP) doesn’t currently have any FDA-approved medications, however there are certain treatments that have worked for patients. Your doctor’s office can try to work with your insurance to try to get these medications covered.

Set expectations for follow-up.

You may wonder what you can expect with your disease in the next two weeks, months, or years. Asking these types of questions can help you and your provider set reasonable expectations and help you feel more confident in your treatment plan. Depending on where you live and your transportation options, it may be easier to ask your doctor if you would be able to have a telehealth visit. If you are waiting to hear back from your insurance about a specific treatment, ask when you should follow up. You should also clarify what homework you may need to do, such as completing certain labs before the next visit or prior to beginning or continuing a medication.

Don’t be afraid to ask for a second opinion.

Ask if your primary care doctor can refer you to a skin doctor (dermatologist). Sometimes, another dermatologist may have ideas about how to treat the disease, or another doctor’s personality might be a better fit for you. Your doctor will not feel offended if you seek a second opinion, and they may even help find you another doctor to see.

Speak up about your mental health.

Having a rare disease can feel very isolating. Pemphigus and pemphigoid have been associated with worse mental health, and conditions like depression. The IPPF holds virtual support group meetings in the US, which have been shown to improve quality-of-life. If you are feeling lonely, sad, or angry; tell your doctor. They can work with your primary care physician or refer you to counseling services. If you are having thoughts of suicide or harming yourself, please call 1-800-662-HELP (4357), or call or text 988.

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Racial Disparities in Immunobullous Diseases

Emily F. Cole MD, MPH

What are health disparities?

Health disparities are differences in the health of different populations. These differences can happen for many different reasons and are a consequence of inequities, meaning that certain populations have greater access to health resources than others. These inequities can include health-related resources, such as access to culturally appropriate high-quality health care, but can also include social and economic factors like employment, housing, food insecurity, community support, and exposure to violence. Despite ongoing research and changes in practice aimed at achieving health equity, these disparities stubbornly persist.

One important set of health disparities is related to race and ethnicity. Certain racial and ethnic groups are consistently shown to have poorer health outcomes as well as greater inequities in social, economic, and health-associated factors. For example, in the United States, Black and American Indian/Alaska Native people have a shorter overall life expectancy compared to white people (70.8 years and 65.2 years versus 76.4 years, respectively) (Arias et al. 2022). The reasons for these disparities are complex and likely enmeshed in many social and economic inequities that have existed for centuries.

Health disparities in immunobullous diseases

Although pemphigus and pemphigoid (P/P) are rare diseases (and research is always more difficult on rare diseases), racial disparities exist in these conditions. For example, non-white patients with P/P admitted to the hospital were more likely to have a serious infection than white patients (Ren et al. 2018, 1768-76). Additionally, inpatient medical care is more costly for non-white pemphigus patients compared to white pemphigus patients (Hsu et al. 2016, 645). Finally, although rare, mortality in pemphigus is influenced by race (Hsu et al. 2016, 1290-98). Given the shortage of research studies on P/P, these statistics may just be scratching the surface of racial disparities in immunobullous diseases.

Why don’t we know more?

One of the reasons why we don’t have more information about racial disparities in P/P is that researchers often do not report race at all. A recent review by the author on studies published to Pubmed in the first five months of 2023 found that more than three-quarters of studies published on pemphigus, bullous pemphigoid (BP), mucous membrane pemphigoid, and dermatitis herpetiformis did not report any information about the race or ethnicity of study participants.

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We see a similar trend in clinical trials. Clinical trials are an important tool to bring promising new therapies to patients suffering from P/P. However, it is critical to include a diverse group of patients in clinical trials to ensure that these therapies work for all patients in the community. A recent review by the author on completed or terminated clinical trials with results posted to clinicaltrials.gov found that three-quarters of clinical trials on BP and just under half of clinical trials on pemphigus did not report any information about race or ethnicity of the participants. For the studies that did report data on race, all the participants with BP and more than three-quarters of the participants with pemphigus were white.

Finally, the tools that we use to measure disease activity in research and clinical trials may not be appropriate in patients with darker skin tones. For example, the most common tool to measure disease severity in bullous pemphigoid is called the Bullous Pemphigoid Disease Area Index (BPDAI) (Murrell et al. 2012, 479-85). The BPDAI is a tool that was developed by an international panel of experts in BP. There are three components to the BPDAI score—a measurement of skin blistering, a measurement of skin damage (like scarring and hyperpigmentation), and a measurement of erythema, or redness. However, using color change to measure inflammation across skin tones has been challenged as color will appear different across different skin tones, and changes in color may be more difficult to detect in patients with darker skin tones. One recent study from a racially diverse group of BP patients in Atlanta, Georgia supported this idea, as Black patients and those with darker skin tones demonstrated greater skin blistering, damage, itch, and higher antibody titers than white patients but less erythema (Cole et al. 2022, 606-7). This is especially important as these disease severity measures are the most common outcome measure in clinical trials for new therapies.

Where do we go from here?
There is still much work to be done to define racial health disparities in P/P and to determine the best ways to tackle these gaps. I encourage all patients to participate in research studies and clinical trials, but particularly those from understudied racial and ethnic groups (Black, Hispanic, and American Indian/Alaska Native specifically). I also encourage physicians and researchers to consider multi-institutional collaborations which would facilitate research across all races, ethnicities, and skin types. Ultimately it will require all of us—patients, caregivers, and physicians, among others—to attain racial health equity in P/P.

References

Dr. Emily Cole is an Assistant Professor of Dermatology and health researcher at Duke University in Durham, North Carolina. Her clinical and research interests include inflammatory skin diseases and itch.
Peer Coach Spotlight: Janet Segall

Our Peer Coach Spotlight section features one of our IPPF Peer Coaches, volunteers who have learned to manage living with pemphigus and pemphigoid (P/P). Peer Coaches share their personal tips and tricks, as well as IPPF resources and educational materials that help people affected by pemphigus and pemphigoid to have meaningful discussions with their healthcare teams. Learn more at pemphigus.org/peer-coaches. This issue, we asked Janet Segall to share more about herself.

Janet Segall is the Founder of the IPPF and a pemphigus vulgaris (PV) patient since 1983. She is an IPPF Peer Coach and the leader of the Northern California Support Group.

How did you become involved with the IPPF?

In 1983, I was diagnosed with PV. After living with PV on and off for 11 years, I went looking for other patients. Unfortunately, I couldn’t find anyone else, so in 1994 I started the IPPF.

What is something that our community members with P/P can do to better advocate for themselves?

They need to ask questions of their doctor. I encourage patients to write down their questions that they want answers to. Try not to be afraid to talk to doctors: They are just people. Doctors may not know the answers to your questions, or there might not be an answer. Check with the IPPF if you need information. Also, talk to a pharmacist about medications. Communication is key.

What treatments did you take in your rare disease journey?

When I was diagnosed, there were not many drugs I could take. I was on and off prednisone for the first 10 years of living with my disease. We tried methotrexate, but it didn’t work well for me. So, the only medication I took was prednisone and I was in and out of remission during that time. In 1993, after my second remission was over, immunosuppressives were being used to treat PV, so my doctor put me on azathioprine (Imuran). I was on azathioprine and prednisone for about four years. I went into remission in 1997, and I stayed on 5 mg of prednisone every other day for about two years without experiencing any outbreaks at all. I stopped taking all medication and I have been in remission since 2002. I get a mouth sore from time to time, but for now they go away without treatment.

What is something fun or interesting about yourself?

I enjoy playing games. I play cards and like to go to the casino occasionally to play blackjack. (I don’t do it very often, though.) I am also a Mahjong player.