

ELEVATING AWARENESS OF THE PRESSING NEEDS OF THE PEMPHIGUS COMMUNITY



EXECUTIVE SUMMARY

The International Pemphigus & Pemphigoid Foundation (IPPF) held an inaugural Patient Forum on October 2, 2020, as part of the 2020 IPPF Virtual Patient Education Conference: “Creating a Brighter Future Together.” A total of 541 people from around the world registered for the event and either attended the live session or viewed the recording. For 70% percent of them, this was the first IPPF Patient Conference they attended.

The Patient Forum brought together pemphigus patients, caregivers, patient influencers, healthcare professionals, thought leaders, and advocates to discuss the most important disease-related topics affecting the pemphigus community.

At the conclusion, the speakers and panelists reached a consensus on the most urgent needs facing the pemphigus community: 1) improving the speed and accuracy of diagnosis; 2) developing improved treatment options; and 3) improving the affordability of and access to current and future therapies.

Based on these needs, they agreed on the following calls to action:

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

Speakers and Panelists

The Patient Forum was moderated by **Kevin Mead**, IPPF Executive Director, with support from **Marc Yale**, IPPF Advocacy & Research Coordinator. Two physicians with clinical expertise in the treatment of pemphigus served as speakers, and people living with the disease, as well as a representative from the American Academy of Dermatology (AAD), were featured on a panel. **Janet A. Fairley, M.D., FAAD**, Chair, John S. Strauss Professor and Head, Professor of Dermatology, Department of Dermatology, Carver College of Medicine, University of Iowa Health Care, and **David T. Woodley, M.D.**, Professor of Dermatology, Keck School of Medicine at the University of Southern California (USC), introduced the speakers:

Speakers:

- **Dedee Murrell, M.D.**, Dermatologist and Professor, University of New South Wales (UNSW) Medicine; Director of Dermatology, St George Hospital, Sydney, Australia

POLL OF PATIENT FORUM REGISTRANTS



79%
patients



8%
family/friends



3%
clinicians

- **Victoria P. Werth, M.D.**, Chief, Dermatology, Philadelphia V.A. Medical Center; Professor of Dermatology, Hospital of the University of Pennsylvania and V.A. Medical Center; Professor of Medicine, Penn Medicine; member, IPPF Medical Advisory Council

Panelists:

- **David Baron** – living with pemphigus vulgaris; Chairperson, IPPF Board of Directors
- **Jennifer Mangone** – Manager, Congressional Policy, American Academy of Dermatology (AAD)
- **Janet Segall** – living with pemphigus vulgaris; IPPF Founder and Peer Health Coach
- **Rudy Soto** – living with pemphigus foliaceus; IPPF Peer Health Coach
- **Staci White** – living with pemphigus vulgaris; Member, IPPF Board of Directors

Overview of Presentations and Discussion

The speakers presented an overview of the current state of pemphigus treatment and limitations of current therapies, including side effects, limited options for patients who become refractory to treatment, and the need for steroid-sparing therapies. They also covered advances in pemphigus care and the potential role of new investigational therapies in the pipeline, which range from an anti-FcRn antibody to a BTK inhibitor to CAAR T cells. The panelists spoke about their personal experience with pemphigus and its effect on their quality of life, specifically the physical, psychological, and financial burdens posed by this autoimmune disease. They described how their disease was diagnosed, treated and, for some, put in remission. They also provided their perspective on critical needs faced by the pemphigus community and practical solutions to address them.

The Patient Forum was sponsored by Principia Biopharma, a Sanofi company.

OVERVIEW OF PEMPHIGUS

Severe autoimmune blistering disease

Pemphigus is a group of rare, serious, and potentially life-threatening autoimmune disorders characterized by painful blistering of the skin and mucous membranes. The blistering occurs because of an immune response that results in autoantibodies attacking the proteins that bind the cells of the skin and mucous membranes. This process, called acantholysis, is not fully understood. Pemphigus patients develop mucosal erosions and/or blisters that fill with pus or fluid on the skin and in the mouth, nose, throat, eyes, and/or genitals. Open sores left by the blisters increase the risk of infection, which can be fatal if it spreads through the bloodstream (sepsis). As Dr. Murrell pointed out, despite current treatment, pemphigus has a mortality rate of up to 10%.

There are four major types of pemphigus, distinguished by their clinical features, associated autoantigens, and laboratory findings: **pemphigus vulgaris**, **pemphigus foliaceus**, **IgA pemphigus**, and **paraneoplastic pemphigus**. Pemphigus vulgaris is the most common form. It usually involves many erosions and blisters that frequently occur in the mouth, and is generally more severe than pemphigus foliaceus, in which blistering is more superficial.

Most common in people middle-aged or older

Typically, pemphigus vulgaris and nonendemic pemphigus foliaceus occur in adults ages 40 to 60. Pemphigus is rare in children, except for endemic pemphigus foliaceus, which affects children and young adults in endemic areas. Neonatal pemphigus is a rare form of pemphigus that occurs when an affected mother's autoantibodies are transferred to the fetus.

Epidemiologic information on IgA pemphigus is limited, but it may occur at any age and may be more common in females. Paraneoplastic pemphigus is very rare and more common in middle-aged adults, although some children have been diagnosed with it.

CHALLENGES IN BEING DIAGNOSED AND LIVING WITH PEMPHIGUS

Misdiagnosis and delays in diagnosis are common

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

People with pemphigus are most commonly misdiagnosed as having erythema multiforme (a skin condition considered to be a hypersensitivity reaction to infections or medications), herpes simplex virus (a viral infection that causes oral and genital herpes), or candida (a fungal infection caused by yeast).

Affects small proportion of the worldwide population

According to Dr. Murrell, pemphigus affects approximately 40,000 people in the United States and more than 200,000 worldwide. Pemphigus vulgaris occurs all over the world, and its frequency is influenced by geographic location and ethnicity. It occurs in 0.1 to 2.7 per 100,000 people per year. Studies have found that certain populations are at a greater risk for pemphigus vulgaris. These include people of Jewish ancestry, particularly Ashkenazi Jews, and inhabitants of India, Southeast Europe, and the Middle East. In North Africa, Turkey, and South America, pemphigus foliaceus is more common than pemphigus vulgaris.

Underlying cause not well understood

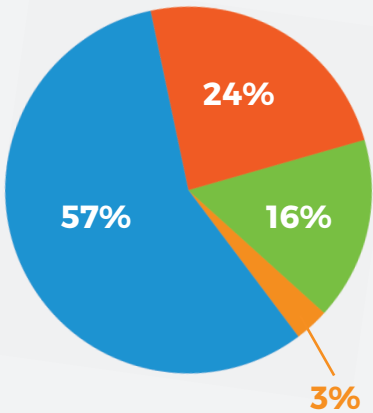
Researchers believe genetic and environmental factors may influence pemphigus. Some have suggested that ultraviolet radiation could lead to pemphigus vulgaris and pemphigus foliaceus activity. In some patients, pemphigus has developed following burns or electrical injury. Viral infections, certain food compounds, ionizing radiation, pesticides, and psychological stress may trigger or worsen the disease.

Why autoantibodies attack cell adhesion is intensively debated. Several reasons have been proposed, including events that cause cell separation and damage to adhesive molecule function. One theory suggests that acantholysis results from autoantibody-caused disruption of cellular signals leading to a cell's structural collapse and shrinkage. Autoantibodies that attack a variety of cell surface antigens have been identified in patients with pemphigus. Desmogleins are the autoantigens most studied in pemphigus vulgaris and pemphigus foliaceus. They are components of desmosomes, key components of cell-to-cell adhesion.

POLL OF PATIENT FORUM REGISTRANTS

Diagnosis by specialty

- Dermatologists
- Dentists
- Other
- PCPs



Laboratory studies, including a biopsy and immunofluorescent studies, are needed to distinguish pemphigus from other blistering and erosive diseases and achieve a correct diagnosis.

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

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

During the Patient Forum, the panelists discussed their own experiences with delayed diagnoses. Staci White mentioned that she did not receive an accurate diagnosis until her

disease had become so severe that she required a trip to the emergency room. She and other panelists also shared recommendations for how the pemphigus community can elevate awareness about the need for faster and more accurate diagnoses:

- Keep in mind that some healthcare professionals have only heard of pemphigus in medical school. Raise awareness of the disease by sharing your experience. Be an open book and talk about it. Share IPPF posts on your social media platforms. – *David Baron*
- Advocate for yourself. Share your story with others. Don't deny that you have the disease. Rather, embrace it. – *Janet Segall*
- Tell your personal story on all platforms available to you. Encourage your family and your network to tell their story as well. Make it personal. – *Staci White*
- Get in front of lawmakers, including members of Congress, and tell your story. Talk about the patient perspective and the science. Partner with industry. – *Jennifer Mangone*

Pemphigus impacts quality of life

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



"I had wounds on 75% of my body. I had to move back in

with my parents in my 30s. After trying every drug, I went into remission. The physical wounds healed, but the mental ones stayed."

- David Baron
living with pemphigus vulgaris;
Chairperson, IPPF Board
of Directors



"The disease took a big toll on me. I was covered with

lesions, including on my face, and I had a customer-facing job. The disease led to anxiety and depression. I didn't want to go out except for work. I missed family functions and my kids' soccer games."

- Rudy Soto
living with pemphigus foliaceus;
IPPF Peer Health Coach



"I had a severe case of pemphigus with wounds

on 80% of my body. From the steroid treatment, I gained a tremendous amount of weight and experienced moon face and joint pain. I've been in remission for four years, and I'm trying to reclaim my body. It's important not to dismiss that, even with remission, the disease still has a physical and mental impact."

- Staci White
living with pemphigus vulgaris;
Member, IPPF Board of Directors

CURRENT STATE OF PEMPHIGUS TREATMENT

Pemphigus is a chronic disease and, with rare exceptions, does not improve without active treatment. According to Dr. Murrell, clinicians have tried many different therapies over the years to manage their patients with pemphigus, but most are not optimal—they take too long to be effective, they cause severe side effects, they fail to induce long-term remission, and patients relapse or become refractory. She provided an overview of the most commonly used treatment approaches over the years, including corticosteroids, immunosuppressants, and rituximab.

High doses of **corticosteroids**, which suppress the immune system and limit inflammation, are considered the standard of care for pemphigus in most countries around the world. Although they are effective (taking effect after just a few weeks), they can cause severe and even life-threatening side effects, especially in elderly patients. These complications include weight gain, Type 2 diabetes, hypertension, and osteoporosis with increased risk of fracture. Additionally, there is no consensus on the use of corticosteroids. One study found that high-dose intravenous (IV) corticosteroids were effective. Another study that evaluated the Lever regimen (i.e., medium doses of oral corticosteroids) in 22 pemphigus patients with mild disease who were treated for a decade showed this regimen was effective with few side effects, but it took four months for patients' skin lesions to heal. Yet another study used a tapering regimen of corticosteroids that reduced prednisone by 10 mg every 4 weeks until 40 mg/dL, but took 4 months for patients to completely taper off the steroid (0 mg).

In 2009, Drs. Murrell and Werth, along with Janet Segall and others, conducted a literature review and found results from only 11 published randomized, controlled clinical trials in patients with pemphigus. Results showed minimal evidence for a steroid-sparing benefit with the immunosuppressive agents **azathioprine**, mycophenolate mofetil (MMF) or **cyclophosphamide**.

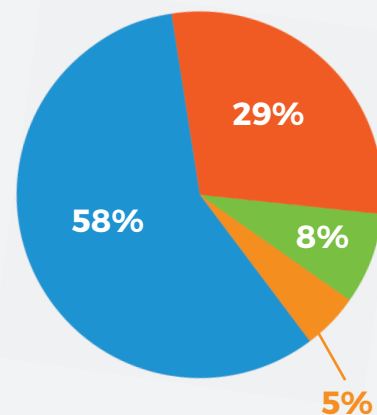
A retrospective analysis published in 2002 showed that all 15 patients responded to intravenous immunoglobulin (IVIG). However, only one randomized, double-blind trial of IVIG—the gold standard—has been conducted. That study of a single cycle of high-dose IVIG for 5 days versus placebo plus a tapering regimen of steroids, showed that only 2 of 21 patients relapsed, providing evidence of the utility of short-term use of high-dose IVIG. However, there is no evidence that IVIG is beneficial in inducing long-term remission.

A double-blind, placebo-controlled, international study of 96 patients conducted at 21 centers assessed the treatment response of steroids plus placebo versus steroids plus MMF. After one year, little difference was seen between the two groups, with 69% of patients in the MMF group and 63.9% in the placebo group having a treatment response. However, MMF-treated patients showed faster and more durable responses with a trend toward quicker remission and longer time to relapse.

POLL OF PATIENT FORUM REGISTRANTS

Management of pemphigus by specialty

- Dermatologist at academic center
- Dermatologist at clinic
- Other
- Dentist



Rituximab, a B-cell antibody given by infusion, was the first drug to be approved by the U.S. Food and Drug Administration (FDA) for pemphigus. That regulatory approval was based on results of a prospective, open-label, randomized trial that compared rituximab plus short-term oral prednisone with short-term prednisone alone in 90 patients with new-onset pemphigus. The primary endpoint was complete remission off therapy at two years. Results showed that 90% of patients in the rituximab group achieved the primary endpoint versus 34% in the standard treatment group. The rituximab treatment group experienced 50% fewer side effects and less corticosteroid use. Based on the findings, which demonstrated a major steroid-sparing benefit, the FDA approved rituximab for pemphigus.

A later study published in 2019 showed rituximab reduced the median prednisone dose, allowing more patients to be on minimal or no prednisone at four months. Subsequently, a Phase 3 clinical trial, called PEMPHIX, compared treatment with steroids plus rituximab or MMF in adults with moderate to severe pemphigus vulgaris. Results, presented at the 28th Congress of the European Academy of Dermatology and Venereology (EADV) in Madrid in October 2019, demonstrated that rituximab was superior to MMF, with 40.3% of patients treated with rituximab achieving a sustained complete remission without the use of steroids for 16 consecutive weeks or more, compared with 9.5% of patients treated with MMF. Patients in the rituximab group required a lower cumulative oral corticosteroid dose, and they had fewer flares and a greater improvement in quality of life than patients taking MMF.

In 2018, international guidelines on the treatment of pemphigus published in the *Journal of the American Academy of Dermatology* listed rituximab as first-line therapy for pemphigus.

New treatment options are needed

Yet, even with the availability of rituximab, Dr. Murrell pointed out, new treatment options for pemphigus are needed that are corticosteroid-sparing, have a rapid onset, are convenient to use, are safe for chronic use, and target the underlying cause of the disease because:

- Corticosteroids remain the mainstay of treatment but are associated with toxicities, and they cannot be tapered off until four months after treatment starts.
- Some patients do not respond to corticosteroids, and others relapse. More trials of investigational compounds are needed

in relapsed patients, and not just in patients with new-onset disease.

- Some patients experience B cell suppression or depletion with pemphigus treatment—a particular concern given the COVID-19 pandemic because this makes them more susceptible to infection.

In her presentation at the Patient Forum, Dr. Werth noted that the medical community has come a long way in treating pemphigus, and a lot has been accomplished in a short period of time.

ADVANCES IN PEMPHIGUS CARE AND POTENTIAL ROLE OF NEW THERAPIES

Reiterating what Dr. Murrell said, Dr. Werth noted that, following the introduction of corticosteroids in the 1950s, pemphigus became a treatable disease, and the mortality rate fell. Next, immunosuppressive agents, such as mycophenolate mofetil (MMF), showed faster and more durable responses than placebo. IVIG showed significantly better efficacy than placebo. Rituximab, which works by removing the B cells that produce the autoantibodies involved in pemphigus, received FDA approval for pemphigus and changed the way pemphigus is treated. Yet, Dr. Werth pointed out, shortcomings remain with each of these. Corticosteroids are associated with high morbidity due to serious side effects and relapses are frequent with both corticosteroids and rituximab. Additionally, while rituximab is rapidly becoming first-line therapy for pemphigus and its use is recommended in international guidelines, more data is needed to determine its optimal use.

In response to the need for new treatment options, scientists in academia and the biopharmaceutical industry are working hard to refine and expand therapeutic options that are more tolerable and more targeted. Dr. Werth provided an overview of the numerous innovative scientific approaches that are being studied.

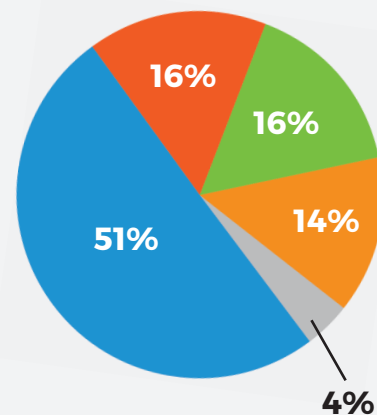
Investigational pemphigus treatments in the pipeline

One approach in development involves clearance of the autoantibodies involved in pemphigus with the use of **immunoabsorption** or an anti-FcRn antibody. Immunoabsorption involves removing the antibodies from the bloodstream and then treating the patient with rituximab within five days of the last immunoabsorption treatment to remove the cells that make the antibodies. Results to date have shown improvement after only one month, with decreases in skin score, antibody levels, and steroid dose. The biopharma company Argenx is working to block FcRn from binding to immunoglobulin G (IgG) with its investigational drug efgartigimod, which is being evaluated in a Phase 2 proof-of-concept trial. To date, efgartigimod has been shown to have favorable tolerability and to reduce IgG, resulting in improvements in study participants' Pemphigus Disease Area Index (PDAI) score.

POLL OF PATIENT FORUM REGISTRANTS

Primary consideration when deciding on/getting new treatment

- To achieve remission
- To help with symptoms
- Reduced side effects/toxicity
- Reduced steroid use
- Pill (not infusion)



Principia Biopharma is taking a different approach with its **Bruton's tyrosine kinase (BTK)** inhibitor. The enzyme BTK is involved in activating B cells and inducing antibodies. BTK regulates the antibody-mediated activation of macrophages and other immune cells involved in pemphigus. Inhibiting BTK rapidly blocks inflammatory immune cells and the destruction associated with autoantibodies, which halts inflammation. Principia's investigational oral therapy, rilzabrutinib, has been engineered to specifically target select immune pathways and attach to or block the BTK target for a longer time, requiring less drug to be effective. It has also been engineered to be reversible to clear more quickly from the patient's system. Results of a Phase 2 study of rilzabrutinib showed it was well tolerated; PDAI scores fell 70% over 12 weeks, with reductions seen as early as 2 weeks, and autoantibodies fell 65% over 12 weeks. Patients' daily use of corticosteroids also was reduced. Rilzabrutinib is currently being studied in a Phase 3 clinical trial.

Dr. Werth explained that another exciting approach to treating pemphigus involves engineering human T cells to express a chimeric autoantibody receptor (CAAR) consisting of the pemphigus vulgaris autoantigen, desmoglein (Dsg) 3, fused to CD137-CD3ζ signaling domains. These **Dsg3 CAAR T** cells are intended to destroy the pathogenic B lymphocytes that produce the autoantibodies involved in pemphigus. An ongoing study is evaluating whether this approach is safe and could permanently remove the pathogenic B cells.

Dr. Werth summarized her talk by saying that the scientific approaches she outlined are highly sophisticated. As such, they require international, multicenter clinical trials to evaluate the investigational therapeutics resulting from these approaches. Because pemphigus is considered by the FDA to be an orphan drug indication (i.e., it affects fewer than 200,000 people in the United States), participation in clinical trials is critical to advance the development of these medicines and eventually bring them to patients in need.

CRITICAL TOPICS COVERED DURING PANEL AND INTERACTIVE Q&A

During the **panel discussion and interactive Q&A**, the speakers and panelists provided insight into the following key topics of interest and concern to the community:

Living with pemphigus and finding support to address the challenges

David Baron talked about the challenges he faced when pemphigus limited his ability to work. An airline pilot, he was out of work for two months on disability because he was not able to fly with active disease,¹ but was able to obtain social security disability. He eventually became Chairperson of the IPPF Board of Directors because the organization played such an important role in helping him cope with the burdens he faced with this disease.

Staci White was out of work for two months on disability, a particular challenge because she supports her household financially and the impact to her salary made her feel an urgency to get back to work. While she had health insurance to cover her medications and an immediate family that provided support, she recommends that others in this situation turn to the IPPF Peer Health Coaches for support and resources beyond that provided by your family and community. As Staci explains, “Pemphigus gave me the humility to get the help I needed.”

Janet Segall had just taken a new job in customer service when she was diagnosed with pemphigus. With sores on her face from the disease and facial swelling due to the corticosteroids she was taking, she was fired from her job without any health insurance. She went on disability, had to file for bankruptcy, and moved her 6-year-old daughter and herself in with a sibling. When given the advice that “you can decide to live or die,” she decided to live and founded what is now the IPPF. It was a difficult journey and took a long time before she could look at herself in the mirror.

Rudy Soto had a different experience in that he worked for a small family-owned business and was able to work as

much as he could and take off as much time as he needed for rituximab infusions and blood draws. He did not need to apply for disability. Because of his positive experience and desire to help others who were not as fortunate, he decided to become an IPPF Peer Health Coach.

Achieving remission

Staci White explained that she was diagnosed and treated with pemphigus before IVIG or rituximab were introduced, so she was treated with a combination of prednisone and MMF by a dermatologist who took a holistic approach. She eventually achieved a long-lasting remission and has not been on any medications for four years. Staci decided to try to get her body in the healthiest shape possible. She made important lifestyle changes, including becoming a vegetarian and cutting back on salt in her diet. She also started journaling and was fortunate to be supported by a large family. Most importantly, she came to accept that having pemphigus is a part of who she is. She recommends finding a doctor who supports you, managing your stress, implementing lifestyle and nutritional changes, and considering counseling with a therapist who is an expert in chronic illness.

Getting a flu or pneumococcal vaccine

Dr. Werth explained that it is very rare for a flu or pneumococcal vaccine to activate pemphigus. She recommended that people be immunized with the injected influenza vaccine, which is based on an inactivated (killed) virus and not with the nasal influenza vaccine, which is based on a live or attenuated vaccine. This is especially important for those taking rituximab.

Navigating pemphigus during the pandemic

Janet Segall pointed out that she is at increased risk of infection because she is a senior and two of her co-workers in the mental health field were diagnosed with COVID-19. She recommends that other people with pemphigus take the same precautions she does, including following public health recommendations to wear a mask, maintain social distancing by staying six feet apart, using hand sanitizer, and being tested as necessary.

URGENT NEEDS AND CALL TO ACTION

During the panel discussion and interactive Q&A, the speakers and panelists agreed that the most pressing needs facing the pemphigus community are as follows:

- Improve the speed and accuracy of **diagnosis**.
- Develop improved **treatment** options to address the limitations of current therapies. New options should have such features as a rapid onset of action, convenience and the ability to be taken orally at home rather than by injection or infusion, be well tolerated for chronic administration, target the underlying cause of the disease, and spare patients from having to take corticosteroids.
- Improve the affordability of, and **access** to, current and future therapies.

Call to Action: Elevate Pemphigus Awareness

Working in partnership with the IPPF, the pemphigus community can help address these critical needs by taking action at the grassroots level to:

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

Among the most impactful ways discussed to achieve this is to take the following steps:

ESTABLISH A RELATIONSHIP WITH CONGRESS

- Check [Senate.gov](https://www.senate.gov) and [House.gov](https://www.house.gov) to find your members of Congress and reach out to them; share your personal story and issues/concerns.
- Collaborate with the IPPF, which has been on the Hill advocating about issues with access.
- Conduct grassroots action alerts.
- Find a champion on the Hill, in a government agency, or in the political sphere, especially one who has personally dealt with a blistering disease as a patient or as a caregiver, family, or friend to a patient.

ADVOCATE FOR THE HEALTHCARE INDUSTRY TO:

- Fund studies that show the significant impact pemphigus and blistering diseases have on patients' quality of life.
- Eliminate prior authorization.
- Make the business case for the cost-benefit of therapies.
- Eliminate step therapy (i.e., health plan requirement to start with prednisone before rituximab even if your physician recommends initiating treatment with rituximab).
- Recruit diverse patients.

Following the conclusion of the Patient Forum, the IPPF conducted a poll of attendees and found an overwhelmingly positive response:

- More than **ninety percent (90%)** said they found the sessions helpful.
- **Seventy-two percent (72%)** said attending the sessions will help increase their quality of life.
- Nearly **ninety percent (90%)** indicated that the topic of advances in pemphigus care and the potential role of new therapies **should be included in a future Patient Forum**.

ABOUT THE IPPF

The International Pemphigus & Pemphigoid Foundation's most important objectives are to provide patients and doctors worldwide with information about pemphigus and pemphigoid, and to provide patients and their caregivers much needed comfort and support so they can continue to live active, productive lives. Our mission is to improve the quality of life for all people affected by pemphigus and pemphigoid through early diagnosis and support. Our vision is to find a cure for pemphigus and pemphigoid.



The Four Pillars of the IPPF

Patient Support

The IPPF offers a variety of free patient services to help patients live an active, productive life. These include our Peer Health Coach program, Patient Education Webinars, local and regional support groups, Find a Doctor map, annual Patient Education Conferences, and educational resources.

Awareness

The IPPF Biopsies Save Lives program seeks to accelerate diagnosis time of pemphigus and pemphigoid patients. Since the majority of patients present oral symptoms first, the Biopsies Save Lives program focuses on pemphigus vulgaris (PV) and mucous membrane pemphigoid (MMP)—two forms of pemphigus and pemphigoid with increased presentation in the mouth. PV/MMP patients often see a dentist as one of their first clinician contacts. Therefore, outreach through student and continuing education is directed to dental professionals, who have the unique opportunity to shorten diagnosis times.

Research

Before new treatment therapies can be developed, investigators need to better estimate the prevalence of pemphigus and pemphigoid, study their pathophysiology, understand treatment patterns, and assess the burden of these diseases on patients. The IPPF encourages research by conducting our Natural History Study, hosting scientific symposia, periodically offering research grants, and maintaining a library of clinical trial information for researchers and healthcare professionals.

Advocacy

Advocacy is defined as public support or recommendation for a particular cause and can take place on local, state, or federal levels of government. The IPPF mainly advocates for patients at the federal level, but we encourage our community to support grassroots efforts through various umbrella organizations.

For more information, visit pemphigus.org.

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