VOICE OF THE PATIENT REPORT

Understanding the Unmet Needs of the Pemphigus & Pemphigoid Community

IPPF Externally-Led Patient-Focused Drug Development Meeting, held January 25, 2023
Understanding the Unmet Needs of the Pemphigus & Pemphigoid Community

VOICE OF THE PATIENT REPORT

This Voice of the Patient report was prepared on behalf of a coalition of pemphigus and pemphigoid organizations including the International Pemphigus & Pemphigoid Foundation (IPPF) (USA), PEM Friends (United Kingdom), Association Pemphigus Pemphigoïde France (APPF) (France), and the Pemphigus/Pemphigoid Friends Association (Japan). This report is a summary of the input shared by patients during the Pemphigus and Pemphigoid Externally-Led Patient-Focused Drug Development (EL-PFDD) meeting, conducted virtually on January 25, 2023.

Authors and Collaborators: This report was prepared and submitted on behalf of the coalitions of pemphigus and pemphigoid organizations by Marc Yale, IPPF Advocacy and Research Coordinator; Patrick Dunn, MFA, IPPF Executive Director; Rebecca Strong, RN, IPPF Outreach Director; Anna Lane, IPPF Communications & Marketing Manager; Marney White, PhD, MS, Statistical consultant/Academic reviewer; Laurence Gallu, APPF; and by Chrystal Palaty, medical writer.

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The International Pemphigus & Pemphigoid Foundation (IPPF) (USA), Association Pemphigus Pemphigoïde France (France), PEM Friends (United Kingdom), and the Pemphigus/Pemphigoid Friends Association (Japan) extend their deep gratitude to all of the individuals and organizations who made our EL-PFDD meeting a reality.

Thank you to the many individuals who shared their story at the EL-PFDD event or contributed written comments after the event. Although every person's journey is unique, you each spoke for the thousands of people living with pemphigus or pemphigoid. Your bravery is an inspiration, and we dedicate this report to you.

We wish to thank Dr. Shari Targum, MD, MPH, from the Center for Drug Evaluation and Research, US FDA, for her insights into how patient-focused drug development influences the regulatory process. We thank all the members of the FDA who took the time to attend our event on January 25, 2023, and who are taking the time to read this report. We especially thank Will Llewellyn, from the FDA’s PFDD program, for his guidance and support during the entire process.

Our EL-PFDD meeting greatly benefited from the ongoing involvement of four clinical experts, Dr. Pascal Joly, Dr. Dedee Murrell, Dr. Aimee Payne, and Dr. Victoria Werth. We thank you for sharing your disease and treatment insights at our meeting. We also thank you for committing your lives to helping people living with pemphigus and pemphigoid.

Thank you to our generous sponsors for supporting our EL-PFDD meeting: argenx, AstraZeneca, Janssen, and the EveryLife Foundation for Rare Diseases. Without you, this meeting and this report would have not been possible.

We wish to thank all the staff members from our coalition of meeting organizers who worked so hard to plan this meeting. This includes representatives from the IPPF, Association Pemphigus Pemphigoïde France, PEM Friends (United Kingdom), and the Pemphigus/Pemphigoid Friends Association (Japan).

Finally, and most importantly, thank you to all the patients, family, and caregivers whose perseverance is the lifeblood of this community. Our EL-PFDD meeting was the culmination of years of advocacy and awareness work by so many. We send you our deep gratitude.

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**Key Points about Pemphigus and Pemphigoid**

1. **Pemphigus and pemphigoid are severe and life-threatening diseases.** These rare, autoimmune blistering diseases involve both B-cells and antibodies that attack and destroy skin and mucous membranes. Blisters can appear anywhere on the skin, on mucous membranes, as well as in the gastrointestinal tract.

2. **Symptoms often take a long time to be recognized.** Misdiagnoses are common, and many patients feel that their disease symptoms – and their suffering – are not taken seriously. Stigma is pervasive, and many feel isolated and alone.

3. **Symptoms can be severe.** Patients experience pain, trouble eating and swallowing, fatigue, itching, anxiety, depression, lesions, scarring that can include skin, loss of vision, trouble breathing, and trouble swallowing. Immune suppression is a major issue, as many medications suppress the immune system. Left untreated, blisters can spread.

4. **Pemphigus and pemphigoid are life threatening diseases that impose a heavy burden on the day-to-day lives of patients.** Most activities of daily living are impacted, especially eating, personal hygiene, and dental care. These diseases impact relationships and families. Patients have many worries, including worries about infections resulting from prolonged immunosuppression.

5. **Pemphigus and pemphigoid have few approved therapies and an enormous unmet medical need.** The only FDA-approved therapy is rituximab in combination with steroids for pemphigus vulgaris, and steroids for pemphigus foliaceus. There are currently no approved therapies for pemphigoid. Most individuals with pemphigus or pemphigoid try many types of medications – many times these medications are administered off-label and often in combination – to find something that alleviates their symptoms.

6. **Not all therapies for pemphigus and pemphigoid work for all patients, and only half of patients have experienced remission.** Even in remission, many worry about flares and relapses, and some still take medications to control symptoms. Remission, if achieved, is relatively short for most, and when it ends, most patients are offered corticosteroids.

7. **Medication side effects can be as challenging as the disease itself, and patients are forced to balance the burden of disease with the burden of therapy.** Current treatment options such as long-term systemic steroids can have drastic and harmful side effects. The risk of chronic B-cell depletion and immunosuppression is a real concern for many patients.

8. **The pemphigus and pemphigoid community needs better treatment options and “more tools in the toolbox.”** This includes more targeted and efficacious steroid-sparing treatments with less side effects and that start working much faster than current options. In absence of a cure, some described how they would settle for a remission, lesions that resolve, or even a period of minimal disease activity. Patients need faster approval of medications that they are already using off-label, access to medications already approved for other indications, and access to innovative new drugs that may not have yet made it through the approval process. The risk tolerance of those living with pemphigus and pemphigoid is very high and needs to be taken into consideration by regulators.
Pemphigus and pemphigoid are severe and life-threatening diseases. Pemphigus involves IgG4 autoantibodies which directly target adhesion proteins in the skin, resulting in intraepidermal (skin) blisters. The lesions are painful and can be extensive and disfiguring. Different autoantibodies cause lesions in different locations: anti-desmoglein-1 antibodies target the skin, while anti-desmoglein-3 antibodies target the mucosa. Pemphigus may involve both genetic predispositions and environmental triggers. The disease is rare, 1-6 cases per million, but up to 20 cases per million in endemic areas, with a one-year mortality rate of 10%. The two major types of pemphigus are:

- **Pemphigus vulgaris (PV)** is the most common, accounting for about 70-80% of cases in North America and Europe. PV often starts with painful mucosal lesions in the mouth, esophageal, or genital areas. Skin involvement is secondary (often involving the scalp and the head), resulting in substantial and painful erosive areas. Wounds can be extensive and life-threatening, due to the risk of septic shock and renal failure.

- **Pemphigus foliaceus (PF)** accounts for 15-20% of pemphigus cases and has a more relapsing course. While PF patients are generally older, the disease can affect a wider range of individuals including children. Skin lesions can occur on areas of the upper trunk and may be extensive. PF can be severe; the mortality rate was 90% prior to steroids.

Pemphigoid autoimmune pathways are complex, involving many different antibody subsets including IgG1, IgG4 and IgE. These autoantibodies target the adhesion proteins in the dermal-epidermal junction. This leads to inflammation and separation of the dermal-epidermal layers, causing deep blisters, pain, and itch and can result in permanent scarring and disfigurement.

- **Bullous pemphigoid (BP)** is the most common type of pemphigoid and may be induced by medications, light, radiation, and medical conditions. It often starts with severe

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1 This summary was based on presentations made at the January 25, 2025 EL-PFDD meeting by Dr. Pascal Joly, Rouen University, France, Dr. Aimee Payne, University of Pennsylvania, Philadelphia, PA, Dr. Dedee Murrell, University of NSW, Sydney, Australia, and Dr. Victoria Werth, University of Pennsylvania, Philadelphia, PA.
itching then large blisters occur with urticarial lesions (hives). Blisters can occur in all parts of the body surface, and may be very large, leading to skin erosion. BP is often associated with debilitating neurological conditions including stroke, dementia, and Parkinson’s disease. BP is rare, with an incidence rate of 13 new cases per million inhabitants per year in Europe. The disease especially targets the elderly, in whom the incidence rate is 500 cases per million per year. This severe disease has a one-year mortality rate between 20-30%.

- **Mucous membrane pemphigoid (MMP)** can attack the mouth, esophagus, nasal cavity, eyes and other mucosal areas of the body and cause difficulties speaking, swallowing, breathing, and seeing. Patients can also have very painful involvement of their genitalia and anal areas. Ocular cicatricial pemphigoid (OCP) is considered a subtype of MMP affecting the eyes and can lead to blindness.

- **Non-bullous atypical pemphigoid** accounts for approximately 20% of cases. This can include hives, intensely itchy spots especially on the palms of the hands, the soles of the feet, and at the edges of the fingers and toes. The disease causes itching and pain related to erosive skin, mucosal, and genital-anal lesions.

- **Additional types of pemphigoid** include linear IgA bullous dermatosis (LAD), pemphigoid gestationis (PG), and anti-p200 pemphigoid. These are less frequent, possibly because many cases remain undiagnosed.

**CURRENT PEMPHIGUS AND PEMPHIGOID TREATMENT LANDSCAPES ARE SPARSE.**

First-line therapies for pemphigus and pemphigoid include oral or topical steroids. These are accompanied by a plethora of serious, long-term side effects including immunosuppression, high blood pressure, and diabetes. Additional immunosuppressive therapies include anti-inflammatory drugs (doxycycline, tetracycline, minocycline) and immunosuppressive drugs (methotrexate, mycophenolate mofetil (Cellcept), azathioprine (Imuran)). Intravenous immunoglobulin (IVIg) is not maximally immunosuppressive but can dilute out autoantibodies and suppresses harmful immune reactions.

The only FDA-approved therapy for pemphigus vulgaris is corticosteroids plus rituximab, an intravenous monoclonal antibody infusion. The only FDA-approved medication for pemphigus foliaceus is prednisone. There are no clinically approved medications for pemphigoid, so medications are prescribed off-label.

Treatment objectives for pemphigus and pemphigoid are three-fold: (1) to immunologically suppress or eliminate disease-causing antibodies and B-cells; reduce the inflammation contributing to pain, itch, and blistering; and facilitate healing of the blistering eruption; (2) to resolve the functional impairments associated with the disease such as pain, dysphagia, weight loss, and infection; and (3) to hopefully improve the quality of life by preventing or limiting the appearance of recurrences while also limiting treatment side effects related to corticosteroids and immunosuppressants.

Ideally, treatment will lead to remission of these diseases. Unfortunately, disease relapse often results in patients enduring long-term cumulative doses of steroids and related side effects.

Several new medications are in the pipeline.

- For pemphigus, efgartigimod (FcRn inhibitor) is in clinical trials and desmoglein 3 chimeric autoantibody receptor T cell therapy is under investigation.

- For bullous pemphigoid, several phase II and III clinical trials are in progress, including dupilumab and FcRn inhibitors.
Meeting Summary


The meeting was an important opportunity for the pemphigus and pemphigoid community to identify areas of unmet needs of patients, to identify the need to develop tools to assess the benefits of patient therapies, and to raise disease awareness and engage the patient community in sharing their disease experiences. The meeting was co-moderated by Marc Yale, IPPF Advocacy and Research Coordinator, and Becky Strong, IPPF Outreach Director. The January 25, 2023, meeting had 463 registrants and was attended by 498 attendees and panelists.

The meeting was opened by Patrick Dunn, IPPF Executive Director, who welcomed all the meeting attendees including the US Food and Drug Administration (FDA). Patrick described the meeting objectives, outcome expectations, and presented the agenda, Appendix 1.

Dr. Shari Targum, Deputy Director of the Division of Dermatology and Dentistry, Office of Immunology and Inflammation, Centre for Drug Evaluation and Research, FDA, provided opening remarks on behalf of the FDA. She explained how Patient-Focused Drug Development helps to ensure that patient experiences, perspectives, needs, and priorities are captured and meaningfully incorporated into drug development and evaluation.

Dr. Pascal Joly, Professor of Dermatology and Head of the Department of Dermatology at Centre Hospitalier Universitaire Rouen, France, introduced and described the background of pemphigus and pemphigoid. Dr. Aimee Payne, Professor of Dermatology and Director of the Clinical Autoimmunity Center of Excellence at the University of Pennsylvania gave a presentation about current treatments and limitations.

The meeting was organized into two sessions. Session 1: Disease Symptoms and Treatments: How They Impact the Daily Lives of Patients, and Session 2: Patients’ Perspective on Available Treatments for the Disease(s), Side Effects, and How to Improve Them.

The first session featured patient story videos and a patient panel, followed by a facilitated group discussion of patients and physicians, moderated by Marc Yale and Becky Strong. Patients described their symptoms and the daily impacts of living with pemphigus and pemphigoid. Marc and Becky invited all patients living with pemphigus and pemphigoid to contribute their voices through online polling, participating by Zoom, calling in by phone, and by submitting written comments by email. The group had a short break for lunch.
The second session was opened by Patrick Dunn. **Dr. Dedee Murrell**, Chair of the Department of Dermatology at St. George Hospital at University of New South Wales in Sydney, Australia, discussed the lack of healthcare treatments, awareness, and education for pemphigus and pemphigoid. **Dr. Victoria Werth**, Professor of Dermatology at the Hospital of the University of Pennsylvania, provided a presentation about the clinical trial experience and meaningful benefits to patients.

The session continued with patient story videos, and a panel of patients, caregivers, and clinicians. This was followed by a facilitated group discussion, moderated by Marc Yale and Becky Strong. Patients described the many different treatments that they had tried, how well they worked, and the challenges and downsides of each treatment. They shared their thoughts on what ideal treatments for pemphigus and pemphigoid would entail. Patients were again invited to contribute their voices through online polling, participating by Zoom, calling in by phone, and by submitting written comments by email. Patrick Dunn closed the meeting by thanking all of the meeting attendees for sharing their perspectives. Names of meeting panelists and discussants are in **Appendix 2**.

**ONLINE MEETING POLLING AND THE POST-MEETING SURVEY**

Online polling was used during the meeting to capture the voice of the patient. These same questions were sent out as a post-meeting survey. The post-meeting survey had a much higher response rate (550 respondents, with 437 complete responses, and 113 incomplete responses), so it was included in this VOP report instead of the online poll results. Post-meeting survey responses are either included as figures or narratively integrated throughout this report.

According to the survey results, 73% of survey respondents were female, 26% were male, and 1% preferred not to answer. The community members represented the following disease types: 45% with pemphigus vulgaris, 24% with bullous pemphigoid, 21% with mucous membrane pemphigoid/ocular cicatricial pemphigoid, 7% with pemphigus foliaceus, and 3% with ‘other’ including pemphigoid gestationis. The meeting was truly international, with 75% of the attendees from the US, 9% from the UK, 7% from Canada, and representation from many other countries. Demographics are in **Appendix 3**. Patient comments submitted by email are in **Appendix 4**. Expert reflections and recommendations are included in **Appendix 5**.

**PEMPHIGUS AND PEMPHIGOID VOICE OF THE PATIENT REPORT**

This Voice of the Patient report is provided to all pemphigus and pemphigoid community members including the US FDA, government agencies, regulatory authorities, medical product developers, academics, clinicians, and any other interested individuals. The input received from the January 25, 2023, EL-PFDD meeting reflects a wide range of pemphigus and pemphigoid experiences, however not all symptoms and impacts may be captured in this report.

The final report, the meeting transcript, and a video of the meeting are available on the IPPF website at www.pemphigus.org/el-pfdd/.
SESSION 1

Disease Symptoms and Treatments – How They Impact the Daily Lives of Patients

During the EL-PFDD meeting, patients shared their perspectives on the direct and indirect impacts of their diseases on their health and overall wellbeing. Selected patient quotes are included below. This section is organized by primary themes identified in the post-meeting survey, however several key points emphasized throughout the EL-PFDD meeting were not captured in the survey.

Symptoms often take a long time to be recognized. Many living with pemphigus and pemphigoid underwent long diagnostic journeys, visited many specialists, and endured painful biopsies. Many felt that their disease symptoms – and their suffering – were not taken seriously. Misdiagnoses are common and include fungal infections, psoriasis, cancer, seborrheic keratosis, epiglottitis, tonsillitis, strep throat, shingles, eczema, and vascular blisters.

“IT took over 10 years to identify my complaint. I think it originally started with many referrals to the dental hospital due to inflamed gums and they could not identify a cause. Then I had problems with stinging eyes and blepharitis and pterygium. I saw a number of eye specialists over 10 years and eventually was diagnosed with OCP/MMP after a biopsy.”
– David, living with ocular cicatricial pemphigoid/mucous membrane pemphigoid

Stigma is very pervasive, and many feel isolated and alone.

“Feeling alone is one of the worst factors in having a rare autoimmune blistering disease. …I knew I wasn’t infectious, but people stared and some even asked. It’s very hard to deal with, and I wasn’t always polite in my response.”
– Ingrid, living with bullous pemphigoid
Left untreated, pemphigus and pemphigoid can often progress to other parts of the body.

“The pemphigus progressed quickly from my mouth to almost every part of my body, my esophagus, my ears, my nostrils, chest, torso, back, belly button, legs, and my areas below the belt. And the areas on my scalp that one dermatologist had told me were eczema, they started to peel off like glue along with much of my hair. I was incredibly frightened, and I felt my body was betraying me.”

– Ellen, living with pemphigus vulgaris

In the post-meeting survey, individuals living with pemphigus and pemphigoid selected the top three symptoms with the most significant impacts on their lives. Survey results are shown in Figure 1 and illustrated with patient quotes below. Some of the closely related symptoms are clustered together.

### Figure 1: Of all the symptoms that you experience because of your condition, please select the top 3 symptoms that have the most significant impact on your life? (Pick only 3)

Pain: 51%

Trouble eating or swallowing: 39%

Fatigue: 34%

Itching: 31%

Anxiety: 24%

Wound care: 22%

Bleeding from lesions: 19%

Depression: 12%

Infection due to immunosuppression: 10%

Sleep disorder: 10%

Impaired vision: 8%

Bruising: 7%

Difficulty speaking: 6%

Infected skin lesions: 4%

Mood swings: 4%

Trouble breathing: 3%

Other mental health issues: 3%

**Pain is a top symptom, followed by trouble eating and swallowing, anxiety and depression, fatigue, and itch. Most individuals experience multiple symptoms.**

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*Figure 1: The number of individuals who responded to this polling question are shown below the X axis (n=467). Because participants selected up to three symptoms, percentages will not sum to 100.*
Pain is a top symptom, followed by trouble eating and swallowing, anxiety and depression, fatigue, and itch. Most individuals experience multiple symptoms.

**PAIN**

Pain was selected as the most impactful symptom by survey respondents. Individuals living with pemphigus and pemphigoid experience extreme pain from raw skin; blisters on all parts of the body, including under finger and toenails, in the oral cavity, and around genitals; and rectal fissures.

“I was in constant pain from raw patches on my back and scalp and especially in my mouth. … Those lesions are about on a level with having touched a hot stove usually. Manageable, but something I wouldn’t wish on anyone.”

– Fred, living with pemphigus vulgaris

“What started out as a tiny pinpoint (like an insect bite) quickly spread to large burn-like lesions that covered my entire upper back and were so painful, I was scared to death.” Any contact with water, clothes, sheets and even hair on her lesions caused her pain. “It would take me over 10 minutes to lie down on the bed because I dreaded the pain of the contact of my skin against the sheets.”

– Mei Ling, living with pemphigus vulgaris

*Initially treated in a burn unit, Mark was “debrided and scrubbed, a practice no longer done. That amount of pain is incomprehensible and no amount of pain drug can protect you from the horror of it.”*

– Mark, living with both pemphigus vulgaris and pemphigus foliaceus

Additional survey results: 89% of respondents reported that their skin/mucosa burns, stings, or hurts (n=239).

**TROUBLE EATING OR SWALLOWING DUE TO BLISTERS IN THE MOUTH, NOSE, AND/OR ESOPHAGUS**

The second most impactful symptom of pemphigus and pemphigoid was trouble eating and swallowing. Blisters in the mouth and esophagus also cause difficulty speaking and trouble breathing.

“For many weeks I couldn’t really eat properly. There were days when I couldn’t breathe very well, and I had a lot of difficulty swallowing.”

– Andy, living with pemphigus vulgaris

“Painful lesions and erosions started to appear on my gums and inside my mouth, peeling off like sheets of tissue paper or like layers of onion skins. And I began to lose weight quickly, about 10 pounds over two months. I panicked because as much as I didn’t want to trust my instincts, I knew something was terribly wrong.”

– Ellen, living with pemphigus vulgaris

“It started in my mouth. It was all over my mouth. I couldn’t eat. It went down my esophagus. It was closing up. I could barely breathe. And then it was all over my body, and my armpits.”

– Kristina, living with pemphigus vulgaris

“A large blister blew up and blocked my epiglottis, and I spent three days intubated and sedated in intensive care.”

– Isobel, living with mucous membrane pemphigoid

Additional survey results: 77% reported having painful blisters or erosions in their mouth (n=438); 73% reported that their gums bleed easily (n=440).
ANXIETY, DEPRESSION, MOOD SWINGS AND OTHER MENTAL HEALTH RAMIFICA TIONS INCLUDING STRESS AND TRAUMA

The mental anguish of pemphigus and pemphigoid is as challenging as the physical burden and stress contributes to the disease, creating a vicious cycle. Anxiety and depression were selected as the most impactful symptoms by many, along with mood swings and other mental health issues. Throughout the meeting, patients described their stress and trauma.

“Aside from the physical pain I had to endure, the emotional stress caused by this rare disease really took a toll on my mental health. Stress is a huge factor in aggravating any physical challenge (ulcers, high blood pressure, heart attacks). The stress caused not only emotional problems for me but also brought on more blistering activity.”
– Mei Ling, living with pemphigus vulgaris

“The worst part for me having this disease was the impact on me mentally. … I became depressed. I was embarrassed. I was ashamed. I didn’t want to be around other people. … I couldn’t sleep as well. I had the itching, so the mood has affected that. Then I felt like no one would want me. It was an awful, awful way to feel.”
– Wanda, living with linear IgA bullous dermatosis

“I think we all have some degree of PTSD from the whole pandemic, and then those of us who’ve been afflicted with this condition, I believe, also have PTSD from it. I know that I was somebody who didn’t know the word ‘fear’ until this happened, and now it’s a big part of my life and impacts every decision that I make.”
– Naomi, living with bullous pemphigoid

Additional survey results: 89% of participants reported depression (n=440); 85% reported anxiety (n=437).

FATIGUE

Fatigue was also a highly reported impactful symptom selected by survey respondents. Fatigue is also a treatment side effect.

“As the blisters began to occupy more real estate on my body, my energy quickly depleted and the pain rapidly ensued.”
– Staci, living with pemphigus vulgaris

“Tiredness is still a constant reminder that I’m living with an autoimmune disease.”
– Ingrid, living with bullous pemphigoid

“If the PV progresses quickly before treatment is received (which is often the case) it can present challenges with eating and extreme fatigue. Fatigue is one of the biggest challenges as it makes my day unpredictable. Work and family obligations can suffer.”
– Alison, living with pemphigus vulgaris

Additional survey results: When asked if their disease treatment made them tired or lethargic, 78% of patients responded “yes” (n=430).
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ITCHING
Itching was selected by survey respondents as one of their top three most impactful symptoms.

“Bullous pemphigoid attacks the underlayer on your skin, so you can’t necessarily see it. You can be itching all over and only see red simple signs. You don’t even see bumps.”
– Eric, living with bullous pemphigoid

“The symptoms that I experienced began with itchiness of my skin and then the appearance of blisters. At first, they began in my ears and my lower back, but then progressed to my entire back, my stomach, chest, breasts, underarms, shoulders, neck, face, and scalp.”
– Doreen, living with pemphigus foliaceus

Additional survey results: 77% of respondents reported that their skin/mucosa itches (n=437).

SKIN LESIONS, INCLUDING WOUND CARE, BLEEDING, AND INFECTION
Skin lesions are an impactful symptom of pemphigus and pemphigoid along with wound care, bleeding from lesions, and infected lesions.

“I had a serious flare, but the symptoms started gradually. They started on my forehead, my scalp, my chest, in my mouth, inside my nose, and a little bit in my eyes and the back of my throat. A few weeks later, overnight I suddenly had a hundred blisters all over my body.”
– Andy, living with pemphigus vulgaris

After developing lesions in her vaginal area and crusting blisters on her chest and face, “The scab would fall off, but then the process would begin again. The blisters did not heal but regenerated in the same spots while slowly spreading across my body. … The extent of damage to my skin was so severe that it required me to receive the same level of wound care as a burn victim.”
– Staci, living with pemphigus vulgaris

“I experienced lesions on my scalp that would not heal. My hairdresser told me that she could no longer cut my hair because of the open wounds.”
– Mindy, living with pemphigus vulgaris

INFECTION DUE TO IMMUNOSUPPRESSION
Prednisone, rituximab, and immunosuppressive drugs cause prolonged immunosuppression, which was especially a concern during the COVID-19 pandemic. Patients mentioned how they experienced different bacterial and fungal infections.

“Patients who take these medications are immunocompromised and are at risk of serious illness or death if they get COVID-19. They are also at high risk for flu, pneumonia, melanoma, and other diseases and infections. Although most people have put the COVID-19 pandemic behind them, many patients who take these medications continue to live somewhat isolated lives and are unable to engage in their usual work, recreational, or other activities.”
– Renee, living with mucous membrane pemphigoid

“I can live with the constant nose drips and illnesses. … The regular lung infection caused by the mucous also causes a problem.”
– Isobel, living with mucous membrane pemphigoid
“For many of us, [immunosuppression] isn’t a temporary thing as a lot of our patients cannot stop taking the immunosuppressants without a flare of the disease. COVID only increased the significance of this concern and this problem. In fact, I’m getting over a bout of COVID right now, and I was very glad that I was not on any immunosuppressants when the pandemic hit.”
– Scott, living with pemphigus vulgaris

**IMPAIRED VISION**

Impaired vision was selected both as a top symptom and as a top impact. Pemphigus and pemphigoid blisters in or around the eyes can cause visual impairment, and even blindness. Many experience cataracts and secondary glaucoma from long-term steroid treatment.

“In terms of my day-to-day life, I’m registered as blind. The MMP took my sight, all of my right and my left eye, within six months of my first blister. In my right eye, I’ve got cataracts from the steroids, secondary glaucoma. There’s quite heavy scarring on my cornea, so my vision even in that eye is really quite poor.”
– Mark, living with mucous membrane pemphigoid

“I had a blister in my eye about a month ago. And I’m beginning to think that maybe that was a bullous pemphigoid blister. I’m concerned about that.”
– Eric, living with bullous pemphigoid

**OTHER SYMPTOMS**

The other most impactful pemphigus and pemphigoid symptoms selected in the post-meeting survey include sleep disorder and bruising.

Many other symptoms were described in the meeting but are not captured in the survey results. These include comorbid autoimmune diseases, including rheumatoid arthritis. Rectal and genital complications such as mucositis and fissures impact excretion and can interfere with sexual relations. Patients described hearing loss, damaged salivary glands, dental issues including gum inflammation, blepharitis, pterygium (a raised, wedge-shaped growth of the conjunctiva that extends onto the cornea), and heavy mucous clots in the throat.

“Others had warned me that those who have autoimmune disease often face a second autoimmune disease or health condition. Yet I was shocked when it happened to me. Since remission, I have developed a condition where my body overreacts to many, many foods, medications, and even scents. Symptoms include erratic blood pressure that causes fainting, severe intestinal pain, and anxiety. If I eat the wrong food, I pass out, and it’s very scary. I’m now being treated with antihistamines and a special diet by my primary care doctor for what is suspected to be mast cell related issues.”
– Sharon, living with pemphigus vulgaris

“I also suffered – and I can’t stress the word ‘suffered’ enough – from blisters. … In my anal area, you talk about a mess, this was a mess. I couldn’t see it. It was hard for me to treat it and it was so painful to sit and lay down.”
– Vivian, living with pemphigoid

“I have scarred salivary glands, which generate lots of saliva or none, causing teeth problems. I use an eye bag and a steam inhaler every day to counteract the effects, particularly the frequent blepharitis. I can pluck out most of my ingrowing eyelashes myself fortunately, but they can be extremely painful and irritating until they’re plucked.”
– Isobel, living with mucous membrane pemphigoid

“I woke up one morning and could not open my eyes and was admitted to the hospital where I was observed by the dermatology department as well as ophthalmology.”
– Doreen, living with pemphigus foliaceus
Pemphigus and pemphigoid impose a heavy burden on the day-to-day lives of patients, with most activities of daily living impacted.

Using the post-meeting survey, individuals living with pemphigus and pemphigoid selected all the specific daily activities that are important to them and that they are unable to perform at all, or as fully as they would like, because of their condition or because of medication side effects. Although almost one-third of survey respondents experienced no impacts on their daily activities, the rest selected eating, studying/concentrating, personal hygiene, and chores around the house as their top impacts. Survey results are shown in Figure 2 and illustrated below with patient quotes. A key point emphasized throughout the meeting was that:

Pemphigus and pemphigoid are life-changing diseases that impose a heavy burden on the day-to-day lives of patients.

“For the last decade, I’ve been dealing with this on a daily basis.”
– Daphne, living with pemphigus vulgaris

“I was diagnosed with pemphigus vulgaris in July of 2018. In that one phone call, I went from being a healthy person who took the occasional aspirin to someone who needed access to lifesaving drugs.”
– Lori, living with pemphigus vulgaris

Figure 2: Which specific daily activities that are important to you are you unable to perform at all or as fully as you would like because of your condition? (Check all that apply)

<table>
<thead>
<tr>
<th>Activity</th>
<th>Percentage</th>
</tr>
</thead>
<tbody>
<tr>
<td>Eating</td>
<td>40%</td>
</tr>
<tr>
<td>None, I am able to perform all my daily activities</td>
<td>29%</td>
</tr>
<tr>
<td>Personal hygiene</td>
<td>25%</td>
</tr>
<tr>
<td>Study/concentrate</td>
<td>25%</td>
</tr>
<tr>
<td>Chores around the house</td>
<td>22%</td>
</tr>
<tr>
<td>Walking</td>
<td>19%</td>
</tr>
<tr>
<td>Mobility issues</td>
<td>17%</td>
</tr>
<tr>
<td>Talking</td>
<td>15%</td>
</tr>
<tr>
<td>Complex decision-making</td>
<td>15%</td>
</tr>
<tr>
<td>Impaired vision</td>
<td>12%</td>
</tr>
</tbody>
</table>

Percentage of respondents who selected each option (n=468)
Each respondent selected an average of 2.2 responses

Figure 2: This survey question and the response options were taken from the validated Autoimmune Bullous Disease Quality of Life Questionnaire1 and the Treatment of Autoimmune Bullous Disease Quality of Life questionnaire2, which when designed, didn’t account for patient input. The number of individuals who responded to this polling question are shown below the X axis (n=468). Because participants selected an unlimited number of response options, percentages will not sum to 100%.

DIFFICULTIES EATING

Eating was the top activity of daily living impacted by pemphigus and pemphigoid. Many patients experience mouth pain from lesions and blisters, which interferes with chewing and swallowing. Some described profound weight loss, and some described how certain foods triggered oral lesions.

Debra’s husband helped her cut all her food up and made sure she had soft food to eat. “[Pemphigoid] is a burden on your family. And thankfully I had that support from [my husband].” Pemphigoid changed her relationship with food. “I still, to this day, feel differently about hard food, when I see fresh vegetables or hard food, I flinch.”

– Debra, living with mucous membrane pemphigoid

“I didn’t experience the weight gains so many people do on steroids because I couldn’t really eat. So many foods hurt my mouth, including anything spicy, anything crunchy, anything with preservatives or acidic, like orange juice. I still can’t eat apples. And alcohol was, and still is, something that is occasionally a trigger for oral lesions.”

– Lori, living with pemphigus vulgaris

Additional survey results: 82% reported needing to avoid some types of foods because of their condition (n=439).

82% reported needing to avoid some types of food because of their condition (n=439)

IMPACTS ON PERSONAL HYGIENE, INCLUDING CHALLENGES WITH DENTAL CARE

One in four of those living with pemphigus or pemphigoid indicated that their personal hygiene was impacted due to difficulties showering and bathing (pain with water touching the skin), not being able to wear clothing on lesions and blisters, having to change clothing frequently, needing help applying topical medications, and issues with dental care. Some even require pain medications prior to dental care.

“I can remember being in the shower and sort of flinching like a fish when the water hit my back. And that’s a very different experience.”

– Naomi, living with bullous pemphigoid

“I had blisters in 14 areas of my body. … The blisters were noticeable if I wore sleeveless tops. I had to wear loose clothing because anything that touched my legs or groin would irritate the skin.”

– Wanda, living with linear IgA bullous dermatosis

“The open sores, mobility from open sores becomes a problem. … wearing clothes on your raw body. I wore a sarong bottom and a T-shirt top with no sleeves, split down the middle just to get it on.”

– Mark, living with both pemphigus vulgaris and pemphigus foliaceus
Despite using steroid cream in her mouth, “I was still experiencing new outbreaks and still having difficulty eating and brushing my teeth.”  
– Lori, living with pemphigus vulgaris

Additional survey results: 52% of patients reported difficulty bathing (n=434); and 55% reported limitations in clothing and/or fabrics that they can wear (n=436).

COGNITIVE INTERFERENCE: CHALLENGES WITH STUDYING, CONCENTRATING, AND COMPLEX DECISION-MAKING

Pemphigus and pemphigoid disease symptoms and medications interfere with cognition, and can make activities like studying, concentrating, and complex decision-making difficult.

“I have brain fog. I can’t function on a daily basis for a week after I have Rituxan.”  
– Doris, living with mucous membrane pemphigoid

Additional survey results: 65% of respondents reported experiencing some form of cognitive impairment (brain fog, difficulty concentrating) as a result of their treatment(s) (n=423).

CHALLENGES WITH CHORES AROUND THE HOUSE

For many, living with pemphigus or pemphigoid impacts their independence. In the survey many selected chores around the house as a daily activity impacted by their diseases.

“It was difficult to just take care of myself. ...In my late twenties. I had to move back in with my parents because I needed full-time care. I just couldn’t do pretty much anything on a day-to-day basis.”  
– David, living with pemphigus vulgaris

“I’m registered as blind. ...so, in terms of my day-to-day life, it’s obviously taken my independence quite a lot. I live my life as most visually impaired people would. I don’t go out very much.”  
– Mark, living with mucous membrane pemphigoid

“I am my own caregiver. While I love cooking and nutrition, I struggle with household chores. Since I am retired and living on Social Security, I cannot afford to hire helpers.”  
– Judith, living with mucous membrane pemphigoid

WALKING AND MOBILITY ISSUES

Walking and mobility are both impacted by pemphigus and pemphigoid. Some patients described being unable to sit or to wear a seat belt. Others described how medication-induced balance issues, dizziness, vertigo, and blood clots interfered with walking.

“I couldn’t exercise or walk even though prior to incurring BP, I was walking two to three miles a day. ... The prednisone was incredibly debilitating, and I fell three times. I experienced dizziness and vertigo on a daily basis.”  
– Lou, living with bullous pemphigoid

“My husband had to push me around in a wheelchair because I couldn’t walk.”  
– Sharon, living with pemphigus vulgaris

“I couldn’t drive for several months because the chest wounds didn’t allow me to use a seatbelt.”  
– Andy, living with pemphigus vulgaris
“The open sores, mobility from open sores becomes a problem. Sitting in a car, riding in a bus, even sitting or walking in a mild breeze can be extremely painful.”

– Mark, living with both pemphigus vulgaris and pemphigus foliaceus

DIFFICULTIES SPEAKING

Many lost their voice or were unable to speak due to blisters and lesions in the mouth and throat. The inability to speak has enormous social impacts.

“My voice would sound raspy whenever the blistering was active, and I often had to refrain from talking.”

– Mei Ling, living with pemphigus vulgaris

“By far the worst effect was the 15 life-changing years without a voice. … I’m a pretty robust person and could easily soldier on even without being fully functioning, but the isolation and social challenges created by not being able to speak were hard to bear, and I did contemplate suicide on many occasions.”

– Isobel, living with mucous membrane pemphigoid

SLEEP DISORDERS/DISTURBANCE

Pain, open lesions, and anxiety interfere with sleep.

“I had to go on a soft diet and couldn’t sleep. My clothes and sheets were destroyed by blood blisters bursting.”

– Lou, living with bullous pemphigoid

“I call my side effects mild, but my house was never cleaner, because I never slept. All my personal relationships were strained because of my lack of sleep, and I was extremely moody, jumpy, and reactive.”

– Mindy, living with pemphigus vulgaris

Itching kept Naomi awake at night. “And when it goes on all night and keeps you from sleeping, it’s very, very difficult.”

– Naomi, living with bullous pemphigoid

SOCIO-ECONOMIC AND PSYCHOLOGICAL IMPACTS

Many additional impacts of pemphigus and pemphigoid were mentioned during the meeting and include social and relationship impacts including social isolation; family member and caregiver impacts; changes to appearance and self-image; employment, career, and retirement impacts; and interference with hobbies and travel. These are illustrated below with quotes.

Social and relationship impacts including social isolation. Many described how their immunosuppression vulnerability to severe illness caused anxiety, depression, PTSD and led to social isolation, even for their families. Some are unable to speak and socialize freely, while others are worried about their appearance.

Even after his scalp lesions were treated, “The scalp remained very painful for six weeks. For the first two weeks, I couldn’t lay my head down at all, so I wasn’t able to sleep.”

– Andy, living with pemphigus vulgaris
During COVID, Marney missed spending precious time with her ailing mother. “My 10-year-old child was unable to attend school for an entire year because Connecticut provided no remote school alternative. My husband’s work also requires face-to-face interactions, so he was unable to work for the duration of our isolated period and his was the primary income.”
– Marney, living with pemphigus vulgaris

“My family now has to take extra precautions to avoid infections. This has been difficult for my teenage children who are often the only ones wearing masks, and for my husband who is exposed to so much in his work in the emergency room.”
– Janeal, living with pemphigus vulgaris

Some saw their marriages dissolve. “I’ve also lost my marriage because I couldn’t perform sexually.”
– Doris, living with mucous membrane pemphigoid

“I just didn’t feel comfortable being in public. I don’t know, maybe it’s just vanity, but I just didn’t feel that’s myself, my smile. I just couldn’t be myself. I did feel that inward feeling and it wasn’t pleasant.”
– Debra, living with mucous membrane pemphigoid

“I’m really a very outgoing person. … I have depression, I have insomnia. I feel isolated. I’m no longer the outgoing, bubbly person. When I’m around people, my personality is generally outgoing, so because of this disease, I tend to isolate myself.”
– Doris, living with mucous membrane pemphigoid

Additional survey results: The majority of respondents (79%) reported that their disease affects their social life (n=439), 45% reported that relationships have become more difficult (n=434), and 61% reported experiencing sexual difficulties (n=430).

Family member and caregiver impacts. Pemphigus and pemphigoid put enormous amounts of stress and anxiety on caregivers and family members. Many times, doctor appointments are centered around the patient and their experience, which leaves family members and caregivers to be the unacknowledged collateral damage of pemphigus and pemphigoid.

“It’s not just the disease that affects the person with the disease, but those close around them as well. It causes severe anxiety.” Trina’s flare impaired her ability to breathe, and as the paramedics took her away, her husband was afraid. “He didn’t know if he’d see me again and that caused him extreme anxiety. And sadly, my 17-year-old son has got several anxiety disorders, and one of them being a separation anxiety disorder. And I’m absolutely certain it is because of his fear of the paramedics arriving and taking me to the hospital and not knowing what was going to happen next.”
– Trina, living with pemphigus vulgaris

“My parents were pretty sure that I was dying. They always had a strong face in front of me, but they would leave the room and go cry somewhere because their son was in such bad shape. And the amount of stress and the burden on them having to pretty much take care of me full time, deal with that, I mean, that was beyond difficult. … So, it put a huge amount of stress on my caretakers.”
– David, living with pemphigus vulgaris
Changes to appearance and self-image. Many described scarring, disfigurement, and hair loss. For some, these diseases changed who they are on a fundamental level.

“The very important feature of MMP in my view is that it leaves scars; living with the damage done by the scarring is life changing.”
– Isobel, living with mucous membrane pemphigoid

“The scars on my back, stomach, arms, and legs still bear witness to pemphigus vulgaris’ path on my body and remind me that I am a warrior, especially on the days that I don’t feel like one.”
– Staci, living with pemphigus vulgaris

“I have the hair loss, which to me, it takes away from my quality of life, of the things that I have to do just to make myself look presentable to go out and to go to work every day. As I get older, I feel my energy depleting from me.”
– Odette, living with pemphigus vulgaris

Additional survey results: The majority of respondents (72%) reported some embarrassment about their appearance because of their disease (n=435).

Employment, career, and retirement impacts. Numerous medical appointments, fatigue, fear of infection, and increased anxiety all contributed to employment, career, and retirement changes.

“My normal schedule would involve interacting with patients, having a lot of research-related meetings, and traveling frequently. But I’ve had to avoid almost all of that and all indoor social and recreational activities while on rituximab because of the risk of COVID, flu, and RSV.”
– Joel, living with pemphigus vulgaris

“The disease has had a significant impact on my lifestyle, including the early retirement. It’s very hard when you no longer have blisters to explain to some people – including my work’s occupational health – that it’s a bit more than just a bit of a skin complaint.”
– Ingrid, living with bullous pemphigoid

“My life has changed dramatically. I have not been able to resume my daily activities including my job as a banker. It is frustrating and depressing.”
– Josie, living with pemphigus vulgaris

Additional survey results: The majority (71%) reported that the disease has affected their work life or study (n=432), with 59% reporting needing to miss work (n=423). Those who responded affirmatively were asked why they had missed work. A total of 45% missed work due to initial disease activity, 13% missed work due to disease relapse/flare activity, and 42% missed work due to both initial and relapse activity (n=241).

Interferes with hobbies and travel. Many described how pemphigus and pemphigoid interfered with their hobbies, spending time outdoors or in nature, and travel.

“I did not see my family or grandchildren for three years because they live 1300 miles away, and it was not safe for me to travel due to being immunocompromised.”
– Renee, living with mucous membrane pemphigoid
“Over the summer, my family had been on a vacation in Europe, and we had to cut it short. I was not able to even carry my own pocketbook. Most days I was too weak and nauseous to get out of bed, and upon arriving home customs asked if I needed a paramedic.”

– Daphne, living with pemphigus vulgaris

Patients have many worries, including the worry that the disease will persist because it is chronic. They worry about prolonged immunosuppression from medications.

In the post-meeting survey, individuals living with pemphigus and pemphigoid were asked to select what worries them most about their condition. Survey respondents each selected an average of 3.5 responses, with the top selected response, “that I will have this for the rest of my life because it is chronic.” Post-meeting survey responses are shown in Figure 3 and described with patient quotes below.

**Figure 3:** What things worry you most about your condition? (Check all that apply)

<table>
<thead>
<tr>
<th>Worry</th>
<th>Percentage</th>
</tr>
</thead>
<tbody>
<tr>
<td>That I will have this the rest of my life because it’s chronic</td>
<td>85%</td>
</tr>
<tr>
<td>That my condition will not improve</td>
<td>52%</td>
</tr>
<tr>
<td>That nobody understands what I am experiencing</td>
<td>50%</td>
</tr>
<tr>
<td>That I can’t physically do things that I used to do or enjoy doing</td>
<td>42%</td>
</tr>
<tr>
<td>That it affects my social life with family/friends</td>
<td>41%</td>
</tr>
<tr>
<td>That it affects the ability to be intimate with others</td>
<td>30%</td>
</tr>
<tr>
<td>That I can’t afford treatments</td>
<td>24%</td>
</tr>
<tr>
<td>That it affects my ability to earn a living</td>
<td>23%</td>
</tr>
</tbody>
</table>

Figure 3: The number of individuals who responded to the polling question are shown below the X axis (n=465). Because participants selected an unlimited number of response options, percentages will not sum to 100%.
Understanding the Unmet Needs of the Pemphigus & Pemphigoid Community // Voice of the Patient Report

WORRY THAT I WILL HAVE THIS THE REST OF MY LIFE BECAUSE IT’S CHRONIC

Patients in remission worry about experiencing relapse. Patients with active disease worry that blisters will migrate to other parts of the body.

“The fear that it’s never going to go away. That the illness is always going to be there.”
– Trina, living with pemphigus vulgaris

“I’ll partially echo what Trina said. The fact that there is no cure, that this disease is always going to be there. And even after spending a long time in remission, it was always in the back of my mind that it was there and that it could come back. I used the analogy that it was a predator stalking me, at times it was far away but I could still hear it out there.”
– David, living with pemphigus vulgaris

WORRY THAT MY CONDITION WILL NOT IMPROVE

This was the second most selected worry and is related to worries that the medication won’t work or will stop working.

“My biggest fear is that at some point, rituximab is going to stop working. That was the only treatment that worked for me. And I worry that if that stops working, what other treatments are out there that I could use? So far it still works, but it’s always in the back of my head, and that’s probably my biggest fear associated with this.”
– David, living with pemphigus vulgaris

“My biggest fear right now is that I will have a pemphigus relapse and when they try to treat me with CellCept and rituximab, my body will reject both. And then what?”
– Sharon, living with pemphigus vulgaris

WORRY THAT NOBODY UNDERSTANDS WHAT I AM EXPERIENCING

This worry ties into the social isolation that individuals with pemphigus and pemphigoid experience and the feeling of being alone.

“During the last eight years without remission, “my friends and family have helped very little. My best friend dropped our relationship, and my son seems to think the knowledge I have is all I need. … Most people haven’t heard of MMP, so they minimize the seriousness and think you are exaggerating.”
– Judith, living with mucous membrane pemphigoid

Additional survey results: The majority of respondents (70%) reported that they believe their friends and family find their disease tiresome (n=437).

WORRIES THAT I CAN’T PHYSICALLY DO THINGS THAT I USED TO DO OR ENJOY DOING, THAT IT AFFECTS MY SOCIAL LIFE WITH FAMILY/FRIENDS, THAT IT AFFECTS MY ABILITY TO BE INTIMATE WITH OTHERS

These worries selected in the poll reflect the impacts of pemphigus and pemphigoid including interference with hobbies and travel, social and relationship impacts, and social isolation and relationships with family members.

“Every day, I worry that the little sores I have will turn into a full-blown flare and that I will experience that pain again.”
– Janeal, living with pemphigus vulgaris
WORRY THAT I CAN’T AFFORD TREATMENTS AND THAT IT AFFECTS MY ABILITY TO EARN A LIVING

Many worried about the financial impacts of the disease: both the expensive treatment costs as well as reduced earning potential because of employment impacts.

“Because of the fatigue and the time commitment of the treatments themselves, I backed out of several job commitments and opportunities including canceling a book contract, opting to delay my own promotion because I was too fatigued to go through the process. And so, these factors combined with the out-of-pocket costs of treatment had very significant financial implications.”
– Marney, living with pemphigus vulgaris

OTHER WORRIES

Other worries not included in survey question Q3, but mentioned by patients include worries about infection related to chronic immune suppression, worries about being unable to breathe, and worries about medication side effects.

Worries about infection because of chronic immune suppression. This worry was expressed in many different ways throughout the meeting.

When Trina encountered a fellow congregant who had acute tonsillitis, “I sort of ran from them and I actually went and burst into tears because I thought, ‘I don’t want to be here’. So yeah, just generally day-to-day fear of infection.”
– Trina, living with pemphigus vulgaris

“We pulled my child from school before the lockdown. …My family and I remained isolated in our home for 706 days from March 2020 to February 2022 because I remained maximally B-cell depleted, and I could not generate antibodies to the COVID vaccine. My physician determined that if I were to be exposed to COVID at that degree of B-cell depletion, it could be fatal.”
– Marney, living with pemphigus vulgaris

Additional survey results: When asked if they were afraid of getting sick (flu or COVID-19) because of the immunosuppressive therapy prescribed for their disease, 87% of respondents said “yes” (N=428). The majority of participants (75%) reported that their activities are limited due to treatments or fears of becoming ill (n=427).

Worries about being unable to breathe. For those with blisters in their airways, this fear is very real.

“The fear of every day, every minute, not being able to breathe. It’s the most awful experience, like a light switch.”
– Trina, living with pemphigus vulgaris

Worries about medication side effects. Many individuals with pemphigus and pemphigoid worry about medication side effects as well as medication interactions.

“I also am very reluctant to take on any new medication for anything going on in my life just because of the fear I have of the interactions between all the medications and all the pills.”
– Daphne, living with pemphigus vulgaris

Additional survey results: Most (83%) are always or sometimes worried about adverse effects of their disease treatments(n=433).
SESSION 2

Patients’ Perspective on Available Treatments, Side Effects, and How to Improve Them

During the EL-PFDD meeting, individuals living with pemphigus and pemphigoid described the different treatments that they had tried and how well they worked to alleviate symptoms, as well as downsides and side effects. Finally, they described their wishes for future medications.

Most individuals with pemphigus or pemphigoid try many types of medications, often in combination and some off-label, to try to find something that works. This was a key point emphasized throughout the second session of the EL-PFDD meeting. Some described how managing and taking their medications became a full-time job. Several patients mentioned how they always feel they have a lot of drugs in their body, a comment that resonated with many.

“I started with high dose prednisone, high dose mycophenolate and doxycycline. Because I was on these medications, my doctor also prescribed Fosamax, Omeprazole, calcium, vitamin C, and vitamin D, in addition to blood tests and regular bone density scans to lessen any possible long-term side effects. When I think back, I realize I went from being a person on no medications, to needing a spreadsheet to keep track of all my medications and their doses. Can you imagine if I had severe disease?”
– Mindy, living with pemphigus vulgaris

“The first few years of my treatment are what I called my ‘trial and error pill popping phase,’ trying to determine the most effective medication along with the appropriate dosages while minimizing the adverse side effects.” In addition to methotrexate, Cellcept, antibiotics, intravenous immunoglobulin, rituximab, and other medications, “I have tried the majority of systemic meds that are used to treat pemphigus, including mega doses of prednisone, a steroid, dapsone, an antibacterial drug used to treat leprosy, sulfasalazine, an anti-rheumatic drug used to treat rheumatoid arthritis and other autoimmune diseases.”
– Ellen, living with pemphigus vulgaris

65% of respondents reported that they feel that they are taking a lot of medication for their disease (n=355)
Additional survey results: 65% of respondents reported that they feel that they are taking a lot of medication for their disease (n=355) and 87% reported that they were afraid of a relapse or their illness worsening when offered lower doses of treatment (n=349).

**Most take prescription medications to help treat their condition or its symptoms.**

Survey respondents indicated all the approaches that they used to help treat their condition or its symptoms. Poll results are shown in Figure 4. The results of the post-meeting survey indicated that prescription medications were the top approach to treating pemphigus and pemphigoid.

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**Figure 4:** What are you currently doing to help treat your condition or its symptoms? (Check all that apply)

- Taking prescription medicines: 82%
- Using vitamins/supplements: 45%
- Diet modification: 41%
- Wound care: 26%
- Taking over-the-counter products: 22%
- Nothing at this time: 9%

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76% of poll respondents reported that they take two or more medications to treat their disease (n=365)
Survey respondents were asked to select all the prescription medications that they tried for their condition. Results are shown in Figure 5 and are described below.

**Figure 5: What prescription medications have you tried for your condition?**

(Check All That Apply)

<table>
<thead>
<tr>
<th>Medication</th>
<th>Percentage</th>
</tr>
</thead>
<tbody>
<tr>
<td>Prednisone or other oral steroids</td>
<td>82%</td>
</tr>
<tr>
<td>Topical steroids</td>
<td>75%</td>
</tr>
<tr>
<td>Mycophenolate mofetil (Cellcept)</td>
<td>42%</td>
</tr>
<tr>
<td>Rituximab (Rituxan / Truxima / Ruxience)</td>
<td>38%</td>
</tr>
<tr>
<td>Tetracycline, doxycycline or minocycline</td>
<td>36%</td>
</tr>
<tr>
<td>Dapsone</td>
<td>23%</td>
</tr>
<tr>
<td>Other*</td>
<td>19%</td>
</tr>
<tr>
<td>Azathioprine (Imuran)</td>
<td>17%</td>
</tr>
<tr>
<td>IV Ig</td>
<td>15%</td>
</tr>
<tr>
<td>Cyclophosphamide (Cytoxan)</td>
<td>0%</td>
</tr>
</tbody>
</table>

Figure 5: The number of individuals who responded to this polling question is shown below the X axis (n=449). Because participants selected an unlimited number of response options, percentages will not sum to 100.

Additional survey results indicated that the number of prescription medications needed to treat their disease is burdensome and that having to take less medications would be an improvement. The majority (76%) of poll respondents take two or more medications, 46% take three or more medications, and 23% take four or more medications (n=365).

**PREDNISONE OR OTHER ORAL STEROIDS**

High initial doses of steroids are usually necessary for the initial management of pemphigus and pemphigoid symptoms and are administered continuously until the disease is under control. A total of 82% indicated that they had taken prednisone and other oral steroids. Sometimes, prolonged administration is required to prevent relapse.

“My doctor spent a lot of time making sure I understood that while prednisone would help, it was far from an ideal solution. … At that point, my PV symptoms were so significant that I didn’t really care what the side effects were going to be. It had to be better than what I was experiencing, and that’s what most of us deal with. We use prednisone because it’s the only drug we have that begins to work quickly and it works for most patients. But as soon as it starts to work, we begin to look for the drug that will allow us to get off of the prednisone before those serious side effects come.”

– Scott, living with pemphigus vulgaris
“I was diagnosed in 1983. It’s been a long 40 years. And prednisone was the only thing available to me at the time and I couldn’t get under 30 mg of prednisone without breaking out, and that was considered a mild disease. I couldn’t even go to 28 mg without breaking out, and I mean breaking out from head to toe, and in my mouth, and other areas.”
– Janet, living with pemphigus vulgaris

Chronic steroid use includes many side effects that become worse the longer patients are on their medications. Side effects that were described throughout the EL-PFDD meeting include insomnia, psychological changes (agitation, psychosis, and hypomania), weight gain, high blood pressure, bone loss and fractures, infections from immunosuppression, diabetes, glaucoma, and cataracts.

“The prednisone was the worst experience of my life. I’m not going to say it’s worse than the disease, because that was pretty brutal, but I went from 130 pounds up to 160 pounds, and I walked every day, so it was crazy. My blood pressure skyrocketed. I lost my hair.”
– Kristina, living with pemphigus vulgaris

“To mitigate the effects of the steroids, I took a handful of other pills. I take at times up to 20 pills a day, every day. I have a spreadsheet just to keep track of all the information. But every time I adjusted my dosage, I would erupt in blisters. I felt sick beyond description from adrenal fatigue. The steroids have such an unpredictable effect on the user. I never know if I’m experiencing a symptom or a side effect. Side effects include uncontrollable outbursts, often inappropriate, having an insatiable appetite, moon face, muscle weakness, joint pain. I experienced all of those and then more.”
– Daphne, living with pemphigus vulgaris

In addition, steroid downsides include the need to slowly taper, which is very difficult.

“Even today, I don’t think I could put a prednisone pill in my mouth and swallow it. I just had a terrible experience with it. I dealt with moon face, insomnia, depression, and crankiness. However, once I began to taper, the side effects got 10 times worse. I had terrible muscle pain in my legs, so much so that I had to use a cane to walk for six months.”
– Sharon, living with pemphigus vulgaris

“A pharmacist friend of mine put prednisone in perspective this way. He said, ‘Everyone knows how bad cancer drugs are, that’s common knowledge, but steroids are so bad they give you cancer drugs to get you off the steroids.’”
– Robert, living with pemphigus vulgaris

**TOPICAL STEROIDS**

Topical corticosteroids (including drugs such as clobetasol) are considered first-line therapy. These can include topical creams applied to the body as well as oral rinses and steroids injected into lesions.

“I was given high-dose, 80 mg of prednisolone and mycophenolate, and I used topical steroids in the mouth and ointments and creams and a shampoo, which I couldn’t use because my scalp was so bad.”
– Andy, living with pemphigus vulgaris
“I have been finding a lot of success with the dexamethasone oral rinse.”
– Debra, living with mucous membrane pemphigoid

Chronic use of topical steroids causes similar side effects as oral steroids. In addition, topical steroids can cause skin thinning or atrophy, which can lead to tears, stretch marks, and bruises. Many patients need to apply very large amounts of medication and require assistance to do so.

IMMUNOSUPPRESSIVE MEDICATIONS INCLUDING MYCOPHENOLATE MOFETIL (CELLCEPT), AZATHIOPRINE (IMURAN), METHOTREXATE AND CYCLOPHOSPHAMIDE

Immunosuppressive medications were not specifically developed for, nor do they have FDA approval for use in pemphigus or pemphigoid. These medications are used off-label for these diseases due to clinical research. Mycophenolate mofetil is FDA-approved as a solid organ antirejection drug, azathioprine is FDA-approved for autoimmune diseases. Methotrexate and cyclophosphamide are chemotherapy drugs also used to treat autoimmune diseases. Often these are used in addition to prednisone, and other times they are used as steroid-sparing medications.

Sharon took mycophenolate mofetil in combination with prednisone and rituximab. “Now CellCept, I never really had any side effects to that until almost the end. And just before remission, I took CellCept briefly this summer and I had a little relapse, during my relapse and it bothered my stomach. So, I’m not sure if I could take it again, but it did work for five years.”
– Sharon, living with pemphigus vulgaris

“At this point, I guess I’m in what I call chemical remission. I’m on a maintenance dose of methotrexate, which keeps new lesions to a minimum to be one or two per month. My doctors and I have tried over the past five years to lower or stop the dosage from time to time and turns out that’s a bad idea. I experience significant flares every time, the worst of them lasting eight months or so.”
– Fred, living with pemphigus vulgaris

“The mycophenolate didn’t work.”
– Andy, living with pemphigus vulgaris

After diagnosis, David “was then put on a course of cyclophosphamide which helped.”
– David, living with ocular cicatricial pemphigoid/mucous membrane pemphigoid

These immunosuppressive medications work for some patients but not others. These medications include significant side effects including immunosuppression, fatigue, nausea, and diarrhea. Kidney and liver toxicity as well as abnormal blood cell counts necessitate frequent monitoring and blood work. Methotrexate can interfere with pregnancy, and mycophenolate (CellCept) has increased risk of miscarriage and higher risk of birth defects.

“I was put on a wheelbarrow full of drugs, including steroids, which helped the blisters to disappear quickly, but have side effects. I was given steroid creams, but the pharmacist at the hospital did not realize the extent of my blisters and thought that one 20 milligram tube was enough to cover all my body.”
– Ingrid, living with bullous pemphigoid
“Imuran, a short course of that, gave me smoldering liver failure.”
– Jody, living with pemphigus vulgaris

“When I’m on the methotrexate, I get sick a lot. I end up in the emergency room, because of breathing and all that.”
– Debra, living with mucous membrane pemphigoid

RITUXIMAB (RITUXAN)
Rituximab is a monoclonal antibody targeted against the B-cell CD20 receptor. Although rituximab is FDA-approved for moderate to severe pemphigus vulgaris, other patients receive rituximab off-label and it does not work as well for pemphigoid. Biosimilar or generic versions including Truxima, Ruxience, and Riabni are not FDA-approved or specifically tested for pemphigus or pemphigoid but are sometimes substituted for rituximab for cost reasons. Often low doses of prednisone, topical steroids, or dapsone are maintained during rituximab therapy. Efficacy varies: some patients experience success, and others do not.

“The game changer: I had my first two infusions of rituximab in late 2006, and that went some way to calming the disease progression.”
– Mark, living with mucous membrane pemphigoid

“I received the Rituxan infusion in September and then again in October, and I am scheduled for one more in March. The infusions and medications have made my life a lot easier with regards to the blisters as they have disappeared, but the pigmentation to my skin still exists. I still have headaches, dizziness, and light-headedness as well. … I have since been able to drive and now I’m able to go to the supermarket on my own where I wasn’t before.”
– Doreen, living with pemphigus foliaceus

“I had eight rituximab infusions. In the end, I believe the rituximab is what healed me. … I really never had any reactions after the treatments, but during the treatments, it was always a little bit shaky each time because I would always have some kind of reaction then they’d have to give me more meds and then I’d have to wait an hour. And they also found by having the drip slower, it helped, but it always took like seven hours, so it was a good part of the day.”
– Sharon, living with pemphigus vulgaris

“I actually had Rituxan for three and a half months and it didn’t seem to be working for me and my blood levels were a little wacky, so we decided to stop it.”
– Eric, living with bullous pemphigoid

Rituximab has numerous downsides. The medication is transient, requiring repeated infusions every 6-12 months. Patients are immunosuppressed for the 6-9 months (or longer) it takes for their body to replace the B cells. Rituximab takes several months to demonstrate an effect, and sometimes patients are maintained on steroids during that time. Pre-authorization approval, which happens with rituximab, biosimilars and any expensive therapies that insurance companies don’t want to cover (tier 3-6 drugs), often causes delay and confusion for patients. Some patients described side-effects including persistent migraines, brain fog, and immune reactions requiring co-administered medications. In addition, the long-term side effects of rituximab are not fully known.

“Rituximab is an effective treatment, but it is certainly a double-edged sword. Importantly, it takes several months to start working, during which one often needs to take prednisone, which with its numerous and potentially severe side effects is another double-edged sword.”
– Joel, living with pemphigus vulgaris
“Rituximab therapy was effective, but it has the unfortunate side effect of killing off your CD-19 and -20 active B-cells, making it very difficult to develop immunity to new pathogens. In today’s environment, with COVID and so forth, that is actually quite dangerous.”
– Andrea, living with pemphigus foliaceus

“My first round of Rituxan was in February 2020. My immediate side effects were uncomfortable, but also manageable. I experienced fatigue and nausea. This required that I take off a few days from work following each infusion. I’m a professor, and for a few classes I had to sit down in the middle of lecture due to dizziness and exhaustion. This fatigue and nausea were intermittent but prolonged, lasting several weeks to months, but I was able to continue working through this time. I was, however, a lot less productive.”
– Marney, living with pemphigus vulgaris

**ANTIBIOTICS**

Many patients are prescribed antibiotics for infections. Some antibiotics are also immune modulators, including tetracycline, doxycycline, and minocycline (tetracycline antibiotics), and dapsone (a sulfone antibiotic). Usually, they are prescribed along with many other medications.

In addition to Kenalog and methotrexate, Ellen has tried “several different antibiotics for wound infections and a variety of topical treatments for my mouth and skin.”
– Ellen, living with pemphigus vulgaris

After waking up and being unable to open her eyes because of her disease, Doreen was “prescribed 80 mg of prednisone, 1500 mg of CellCept twice daily, doxycycline, niacinamide, as well as various topical ointments for the blisters on my body.”
– Doreen, living with pemphigus foliaceus

“The amount of dapsone I was taking has caused permanent peripheral neuropathy in parts of my feet.”
– Jody, living with pemphigus vulgaris

**INTRAVENOUS IMMUNOGLOBULIN (IVIG)**

Intravenous immunoglobulin (IVig) is a therapy consisting mainly of IgG1 and IgG2 antibodies as well as IgA or IgM antibodies used to treat many inflammatory and autoimmune diseases. IVig works by adding extra antibodies that dilute or out-compete harmful autoantibodies, with the goal of suppressing harmful autoimmune reactions. Many described receiving this along with rituximab. A clear IVig advantage is that it is not immunosuppressive.

IVig downsides can be significant. Side effects can include debilitating headaches and blood clots. Obtaining and administering the medication can be a challenge; it is an expensive medication and patients must receive three to five daily infusions that are repeated every six weeks to start. Some patients described challenges in finding a homecare nurse to administer the drugs.

“I’ve had about 150 infusions of intravenous immunoglobulin and rituximab. … And that necessitated my having to get a portacath implant.”
– Ellen, living with pemphigus vulgaris

“Unfortunately, I was not able to tolerate [IVig], and I ended up in the emergency room owing to extreme side effects that I experienced.”
– Marney, living with pemphigus vulgaris
"Everything I do from day-to-day is around managing my airway through the use of a nebulizer, keeping my airway clear, and lubricating my eyes. I still have to use eyedrops every few minutes. I rarely sleep more than a couple of hours without waking up to hydrate them as well."

– Mark, living with mucous membrane pemphigoid

"I also receive monthly IVIg, which I am hopeful will help with long-term remission. There’s a major time commitment, with each round taking about 16 hours per week. I’m fortunate to be able to work from home during the infusions, but I still end up missing important meetings, and it’s difficult to be fully productive with an IV running."

– Robert, living with pemphigus vulgaris

OTHER MEDICATIONS AND OTHER MEDICAL APPROACHES

All survey respondents who chose “other medications” in the post-meeting survey were invited to write the types of medications using free-form text. They provided a very long list of both prescription and over-the-counter medications: anti-osteoporosis medications; eye ointment and eyedrops; anti-inflammatory medications;omalizumab (anti-IgE antibody); dupilumab (Dupixent) (anti interleukin 4 and interleukin 13 receptor antibodies); anti-gout medications; antihistamines; antibiotics, antivirals and antifungal medications; proton pump inhibitors; anti-anxiety and antidepressants; psoriasis medications; blood pressure medications; cannabis; experimental medications (efgartigimod); anticoagulants; and pain medications.

"I found the rare dermatologist extremely knowledgeable about BP and its treatment, and she prescribed Dupixent to help concerning BP and my psoriasis, and they appear to have been mitigated by use of the drug."

– Arthur, living with bullous pemphigoid

"I was able to be started on dupilumab, and it has done a wonderful job at controlling my disease, at least for right now. ... I appreciate that it works really well. It comes with its own complications."

– Naomi, living with bullous pemphigoid

"I was hospitalized for a GI bleed after taking Eliquis (an anticoagulant) because I developed a blood clot and couldn’t walk. Remember, I was walking every day. I was immunocompromised from the infusions, which were very frightening during the pandemic."

– Lou, living with bullous pemphigoid

"The pain was unbearable at times, especially if the blisters had the opportunity to dry up, so I was prescribed oxycodone for those times."

– Doreen, living with pemphigus foliaceus

In addition to other medications, individuals with pemphigus and pemphigoid required other medical approaches, including surgical interventions to address their symptoms.

"I’ve had over 30 operations on my eyes and my airway. I had an operation a few years ago to unfuse my epiglottis that was sealing my windpipe shut and restricting my breathing. I’ve been to Accident and Emergency at least 200 times since then, and I still, even now, despite all of the operations, still have to go every few weeks, once the pain is unbearable, for some treatment."

– Mark, living with mucous membrane pemphigoid

"Because of constant ingrown eyelash problems, I had an operation to have the follicles removed on my lower left eye. I have had two cataract operations and the first one involved a three-day visit to the hospital as I had to have infusions before and after the operation."

– David, living with ocular cicatricial pemphigoid/mucous membrane pemphigoid
Out of sheer desperation, some try unconventional approaches.

“In desperation for a cure, I tried a number of off-label approaches to treat my lesions, including hyperbaric oxygen therapy, which is a high-pressure oxygen used to treat chronic non-healing wounds, typically from diabetes and radiation therapy. Now, hyperbaric oxygen therapy is not used to treat pemphigus, but I was desperate and willing to pay out of pocket and a lot of my time for this very unorthodox and experimental treatment. So, I spent two hours per day, five days a week for six weeks, lying in an oxygen chamber, breathing 100% oxygen to see if my lesions would clear up.”
– Ellen, living with pemphigus vulgaris

In addition to prescription medications, individuals with pemphigus and pemphigoid use other approaches to manage their symptoms including over-the-counter options.

Besides prescription medications, other approaches that survey respondents selected to currently help treat their condition or its symptoms were shown in Figure 4 (page 26). These include vitamins, supplements and diet modifications, wound care, and over-the-counter products.

**VITAMINS AND DIET MODIFICATIONS**

Vitamins and diet modifications were selected by many post-meeting survey respondents. Diet modifications included avoiding foods that would irritate the mouth.

“Over the last 10 years I’ve had to supplement with Vitamin B12 tablets and at one stage specific iron deficiency tablets.”
– David, living with ocular cicatricial pemphigoid/ mucous membrane pemphigoid

“I’ve also tried nutrition (AIP or autoimmune protocol diet), which has helped somewhat.”
– Judith, living with mucous membrane pemphigoid

**WOUND AND SKIN CARE**

Some described magic mouthwash, non-steroidal anti-itch cream, special toothbrushes, lifestyle changes, and simply avoiding anything touching their wounds.

“I was still experiencing new outbreaks and still having difficulty eating and brushing my teeth.”
Lori found a knowledgeable specialist. “I was told about magic mouthwash, which can be made with a variety of ingredients and can be found on the internet. I was given soft toothbrushes that didn’t hurt my gums and non-steroidal anti-itch cream, and I was given hope.”
– Lori, living with pemphigus vulgaris

“When [skin lesions] get bad, my clothes will stick to them. It makes it difficult to sleep. I have to go through a lot of non-stick bandages to just try to function on a day-to-day basis.”
– David, living with pemphigus vulgaris

Ingrid’s bullous pemphigoid was so severe, that, “nurses had to aspirate the blisters, meaning I needed a change of sheets every few hours. …The itch is so hard to deal with, but I found cool pads helped and once home, I laid on sheets on my sofa, which I got especially as the cream and weeping blisters meant that wearing clothes was very difficult.”
– Ingrid, living with bullous pemphigoid

“Two years ago, I changed to a vegan lifestyle as a way to continue to keep pemphigus vulgaris at bay.”
– Staci, living with pemphigus vulgaris
“Avoiding sun damage, getting enough sleep, and avoiding negative stressors are important in controlling my symptoms. Diet isn’t really a factor for me as it seems to be for others. I pretty much eat what I want, although I’ll avoid foods that irritate the inside of my mouth when I have active oral lesions.”
– Fred, living with pemphigus vulgaris

OVER THE COUNTER PRODUCTS

Many patients use over-the-counter products, some of which were described on page 32 of this report.

NOTHING AT THIS TIME

A small number of individuals indicated that they are not using any treatments, including some who are currently in remission. However, “nothing” is a relative term, and many of those living with pemphigus and pemphigoid regard non-systemic therapies as “nothing.”

“I’m in remission now and off all medication. And my path through remission, it’s pretty close to a best-case scenario for somebody diagnosed with pemphigus.”
– Scott, living with pemphigus vulgaris

After using Imuran for five years, “Now I have been in ... I don’t know if you call it complete remission because I have lesions that I get in my mouth from time to time but a little clobetasol makes it go away. But I basically have not been on drugs since 2002. So, it’s been a long time since I’ve been pretty clear.”
– Janet, living with pemphigus vulgaris

Half of patients have never experienced remission, and for those who have, remission is relatively short.

Survey respondents indicated how well their current treatment regimen treats the most significant symptom of their disease. Poll results are shown in Figure 6.

Figure 6: How well does your current treatment regimen treat the most significant symptoms of your disease?

<table>
<thead>
<tr>
<th>Percentage of respondents who selected each option (n=449)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Very well</td>
</tr>
<tr>
<td>Mostly</td>
</tr>
<tr>
<td>Somewhat</td>
</tr>
<tr>
<td>Very little</td>
</tr>
<tr>
<td>Poorly</td>
</tr>
<tr>
<td>N/A</td>
</tr>
</tbody>
</table>

Figure 6: The number of individuals who responded to this polling question are shown below the X axis (n=449).
In the case of pemphigus and pemphigoid, there isn’t a preferred treatment that works better for all or most patients, and often a multitude of medications at differing doses must be tried in order to alleviate the most significant symptoms of the diseases. As previously mentioned, some treatments do work, but most are off-label and have serious side effects that must be considered. Even when current treatments are working, many patients took many medications over many months to determine the medication that works best for them. In the meantime, people suffer debilitating disease everyday and long-term side effects from all these medications. Patients’ experiences with specific therapies are described in the section starting on page 25.

“There is no one-size-fits-all for our community. Some of us are eligible due to comorbidities to take some medications and not be able to take others, and some of us respond and we wouldn’t have these diseases if we colored inside the lines, right? We’re not always going to respond as doctors think that we should.”
– Becky, living with pemphigus vulgaris

“I have been treated with virtually every medication recommended for MMP, and so far, all have failed or had too many negative side effects.”
– Judith, living with mucous membrane pemphigoid

According to post-meeting survey responses, half of patients had never achieved remission of their disease. Results are shown in Figure 7. Only 14% of participants had experienced full remission with no relapses, but this figure is confounded by the length of time since treatment, as well as ongoing treatment(s).

**Figure 7: Have you had remission of your disease?**

<table>
<thead>
<tr>
<th>Response</th>
<th>Percentage</th>
</tr>
</thead>
<tbody>
<tr>
<td>No, I have never been in remission</td>
<td>50%</td>
</tr>
<tr>
<td>Yes, and I have had multiple relapses/flares of my disease</td>
<td>20%</td>
</tr>
<tr>
<td>Yes, and I have had one relapse/flare of my disease</td>
<td>16%</td>
</tr>
<tr>
<td>Yes, I am in remission with no relapses</td>
<td>14%</td>
</tr>
</tbody>
</table>

**Figure 7:** The number of individuals who responded to this polling question are shown below the X axis (n=449).

“I’m so very happy to report that the combination of the steroids and Rituxan day one, day 15, and again six months later, has put me in near remission. I have few sores, no full flareups, and with appropriate immunocompromised caution am living normally.”
– Jaime, living with pemphigus vulgaris

“I did 10 rounds of Rituxan, and that, I didn’t really have too many side effects from that. I do IVlg every month, and now tapering down to every other month. I am now in remission, so it worked, the Rituxan and IVlg.”
– Kristina, living with pemphigus vulgaris
It is also evident from the patient reported data collected in the post-meeting survey, that just because a patient reaches remission, there is no guarantee how long they will remain in remission or how quickly or severely the disease will return.

“I had been living with pemphigus vulgaris for eight years. After a two-year IVIg infusion treatment, I am currently in remission from the pemphigus vulgaris. However, the lab results show the antibodies are active.”
– Josie, living with pemphigus vulgaris

Those who are able to achieve remission may still need to take medications to maintain remission or to control symptoms.

“The rituximab works, and I now meet the technical [specifications] for being in remission, but I still have a blister in my mouth today and one on my perineum. And I’m treating these with topicals.”
– Andy, living with pemphigus vulgaris

“I am in clinical remission even though I am continuing to be on a low dose of mycophenolate. I wonder if I might be on mycophenolate for the rest of my life because even with the rituximab that I’ve gotten, my disease has basically stayed the same.”
– Mindy, living with pemphigus vulgaris

“Even though I’ve gone into a fairly stable remission, I do get flares occasionally, and I’ve been able to handle those with the topicals and with intralesional injections.”
– Rebecca, living with pemphigus vulgaris

Individuals with pemphigus and pemphigoid face the threat of disease relapse, which emphasizes the need for new, more effective therapies. Those who indicated that they had experienced a remission in the previous question, were asked how long they were in remission before having a relapse. Poll results are shown in Figure 8. The majority experienced a relapse within the first few years, and a minority of patients achieved remission for more than five years.

**Figure 8: How long were you in remission before having a relapse?**

<table>
<thead>
<tr>
<th>Duration</th>
<th>Percentage</th>
</tr>
</thead>
<tbody>
<tr>
<td>Within 6 months</td>
<td>24%</td>
</tr>
<tr>
<td>Within the 1st year</td>
<td>21%</td>
</tr>
<tr>
<td>Within 2-3 years</td>
<td>27%</td>
</tr>
<tr>
<td>Within 3-5 years</td>
<td>3%</td>
</tr>
<tr>
<td>Longer than 5 years</td>
<td>11%</td>
</tr>
<tr>
<td>Not applicable</td>
<td>14%</td>
</tr>
</tbody>
</table>

Figure 8: The number of individuals who responded to this polling question are shown below the X axis (n=x).
Fred experienced a three-year remission, “I let my guard down a bit and my hopes that my PV was gone disappeared when I went from zero lesions to around 70 in the space of three or four days. … That flare felt like a failure, as well as interfering with my activities. … It was kind of a wake-up call that I needed to reestablish a more vigilant approach.”
– Fred, living with pemphigus vulgaris

“I spent 13 years in remission, which I consider myself very fortunate for. But then in 2018, I had a flare again. I knew what to look for, I caught it early, went immediately back to rituximab, but I haven’t been able to get back into remission again. Every eight months or 12 months, it’ll pop up again and we’ll hit it with rituximab again. But so far, it’s just kind of kept disease activity at a very low level and fingers crossed they’ll be able to get back to remission.”
– David, living with pemphigus vulgaris

Additional survey results: Those who responded that they had experienced a relapse were asked if their doctor had recommended corticosteroids at the time of relapse. Just over half, or 54% said “yes, and I took steroids for my relapse”; 8% said “yes, but I decided against using corticosteroids”; and 5% said “yes, but I chose to use another medication”. Only one-third, or 33%, said “no, my doctor did not recommend using corticosteroids for my relapse” (n=159).

Side effects are the most significant downside of current treatments.
In addition to the lack of consistent efficacy, as discussed above, individuals living with pemphigus and pemphigoid selected the most significant downsides of their current treatments in the post-meeting survey. Results are shown in Figure 9 and described below.

**Figure 9: What are the most significant downsides to your current treatments, and how do they affect your daily life? (Check all that apply)**

- Side effects of treatment: 60%
- The length of time it takes to start working: 30%
- Cost of the treatments: 29%
- Time devoted to treatment each day: 24%
- Having to go to the clinic for treatment: 22%
- Route of administration (topical/oral versus intravenous): 16%
- N/A: 16%

“Having a rare disease is very, very challenging, but honestly, the side effects can be equally as challenging.”
– Sharon, living with pemphigus vulgaris

*Figure 9: The number of individuals who responded to this polling question are shown below the X axis (n=450). Because participants selected an unlimited number of response options, percentages will not sum to 100.*
SIDE EFFECTS OF TREATMENT
For many individuals living with pemphigus and pemphigoid, the side effects are as challenging as the disease itself. Many side effects were described in previous sections and include fatigue, cognitive impairment, and immunosuppression. Some also experienced bleeding, bruising, and sensitivity to hot and cold water.

“Oftentimes the accompanied side effects of these meds were worse than the lesions and blisters that covered my body. I experienced elevated liver function tests that were off the chart, lower than normal hematocrit levels that impacted my energy level, jaundice, neuropathy, fluid buildup, infections, and wound abscesses to name a few.”
– Ellen, living with pemphigus vulgaris

“I have just been diagnosed with diabetes. My A1C level is 7.3. I believe that’s a cause of the medication that I’m presently on. I am very grateful for the medication; however, the side effects are very debilitating to me.”
– Doreen, living with pemphigus foliaceus

Additional survey results: When asked if they had noticed any bleeding or a tendency to bruise easily since starting their disease treatment, the majority (69%) reported experiencing this problem (n=430). When asked if contact with hot or cold water bothered them since starting disease treatment, the majority (54%) reported having this issue (n=429).

THE LENGTH OF TIME IT TAKES FOR THE MEDICATION TO WORK
Many immune-modulating medications can take a very long time to get the disease under control, which creates a significant burden on the daily lives of patients. This waiting time is often six to eight weeks or as long as three months, and this time is spent worrying if the medications will work while trying to live through pain and suffering from skin coming off the bodies. Some described how sometimes it took years of treatment to achieve remission.

David received off-label rituximab with IVlg for his severe disease. “And that took a couple years to get under control. ... It took a few years to heal.”
– David, living with pemphigus vulgaris

Scott tapered off prednisone over 18 months. “Why did it take so long? Because CellCept and Imuran, as well as any of the other drugs I might have tried, don’t work for everybody and they can take a long time to work when they do.”
– Scott, living with pemphigus vulgaris

COST OF THE TREATMENTS
Almost all medication used to treat pemphigus and pemphigoid are used off-label. Insurance companies often don’t cover the costs for off-label, yet necessary medications, leading to high out-of-pocket costs for pemphigus and pemphigoid patients. As a result, some patients receive sub-optimal treatment due to insurance restrictions and delays related to the lack of FDA approval. Some insurance companies insist on biosimilars and unapproved treatment regimes, despite a lack of clinical data demonstrating the effectiveness of the treatments or protocols specifically for pemphigus and pemphigoid.

Mindy’s insurance approved one 500 mg treatment every 12 months instead of the recommended two 1000 mg treatments every six months. “My doctor’s advice was to get the treatment I can and not fight with the insurance company. With my antibody levels remaining
unchanged, this makes me think that I can have a flare in any minute and stress can also lead to a flare.”
– Mindy, living with pemphigus vulgaris

“The FDA has not approved rituximab for treatment of pemphigus foliaceus. … I have top-of-the-line health insurance through [my work], and yet it took five weeks to get insurance approval for the therapy. Those five weeks almost killed me. By the time I got it, I had about 800 lesions all over my body.”
– Andrea, living with pemphigus foliaceus

“There are no approved treatments for MMP. All drugs are prescribed off-label and therefore are not covered by any Medicare Part D Prescription Drug plans.”
– Renee, living with mucous membrane pemphigoid

Additional survey results: When asked if their disease treatment ever caused any financial difficulties, almost half (47%) responded “yes”. While 22% had to cut down on their expenses a little, 18% had to cut down on expenses a lot, and 6% can’t afford to buy their treatments (n=438).

“There are a lot of people who do not have the ability to pay their share cost. When you have a $20,000 Rituxan treatment and you have to pay $5,000, or $2000, ... If I had to go back on medication, I’d worry about it every day.”
– Janet, living with pemphigus vulgaris

AMOUNT OF TIME DEVOTED TO TREATMENT EACH DAY AND HAVING TO GO TO THE CLINIC FOR TREATMENT.
Many patients have complex medication regimens, spend a lot of time receiving treatment and wound care, and still need to travel to the clinic for treatment.

Daphne described how much time her medications took. “Honestly, it became a job. Once a month I sit, and I do pill boxes and I lay them out because every day to start opening all those things. I spend thousands [of dollars] on medicine. … I also try not to take those pills in front of my kids because I think it’s a lot for them to see all those pills.”
– Daphne, living with pemphigus vulgaris

“The Rituxan takes a whole day away from me, and then the recouping period takes a whole week, so it’s almost like a whole week of my life. It’s like there are four weeks in a month, but I only get to live three weeks.”
– Doris, living with mucous membrane pemphigoid

“Between the pills for pemphigus and the pills to manage the side effects of prednisone, I was taking 15 to 20 pills throughout the day. Some mornings it took a lot of willpower to take that first fistful of pills. Additionally, there were oral gels, rinses, and lozenges, all of which had to be juggled to not interfere with each other. I also spent as much as 90 minutes per day doing twice daily dressing changes on my back and armpits. You can’t put ointments and dressings on your own back. Therefore, my spouse had to help. So wound care was also time consuming for her.”
– Robert, living with pemphigus vulgaris
Additional survey results: The majority (64%) felt that taking medications interferes with their time/daily routines” (n=354). The majority (56%) of those undergoing treatments reported frustration over needing to take their treatment at a set time (n=432).

ROUTE OF ADMINISTRATION (TOPICAL/ORAL VERSUS INTRAVENOUS)

Patients described administration challenges including: intralesional injection pain; needing assistance to apply topical medications; sheer amounts of medications required; and the difficulties in swallowing pills. In addition to route of administration, frequent monitoring is an issue.

Rebecca described intralesional injections. “I have to get multiple injections around the site of the lesion and my lesions are always in my mouth and often in my throat. … So just imagine 6 to 12 shots in the back of your throat and don’t scream because your toddler is next to you. … Could you even do it without gagging or screaming?”

– Rebecca, living with pemphigus vulgaris

“I cannot take it in one swallow. … It’s one pill at a time, so I have a whole morning routine based around getting those pills down and started.”

– Daphne, living with pemphigus vulgaris

Additional survey results: 30% responded that blood tests for their disease bother them (n=429).

OTHER TREATMENT DOWNSIDES

Some patients find it hard to access medical treatment for such a rare disease as knowledgeable physicians are located at major medical teaching institutions. Some patients report having a hard time finding a physician to administer rituximab and finding infusion centers and specialty pharmacies while others discussed how medication storage requirements inhibit travel.

Ideal future treatments for pemphigus and pemphigoid should have little or no side effects and would be more efficacious. Until then, patients want anything that will improve their symptoms.

Individuals living with pemphigus and pemphigoid were first asked to select the specific things that they would look for in an ideal treatment for their condition, and then were asked what factors they consider when making decisions about selecting a course of treatment. The top selection for both poll questions, was “little or no side effects.” Post-meeting survey results are shown in Figures 10 and 11. Other specific qualities that individuals with pemphigus and pemphigoid hope for in an ideal treatment include ease of treatment administration (i.e., “easy to take”), cost, ease of access, and medication effect on daily activities. Besides side effects, other factors they would consider when making decisions about selecting a course of treatment include efficacy, cost, treatment administration, and peer feedback.
Understanding the Unmet Needs of the Pemphigus & Pemphigoid Community // Voice of the Patient Report

Figure 10: What specific things would you look for in an ideal treatment for your condition? (Check all that apply)

- Little or no side effects: 91%
- Easy to take: 58%
- Cost: 55%
- Ease of access: 51%
- Affect on my daily activities: 50%

Percentage of respondents who selected each option (n=448)
Each respondent selected an average of 3.0 responses

Figure 11: What factors do you consider when making decisions about selecting a course of treatment? (Check all that apply)

- Side effects: 89%
- Efficacy: 76%
- Cost: 41%
- How treatment is administered (oral, IV, topical, etc.): 31%
- Feedback from peers: 21%

Percentage of respondents who selected each option (n=448)
Each respondent selected an average of 2.6 responses

Figures 10 and 11: The number of individuals who responded to each polling question are shown below the X axis (n=x). Because participants selected an unlimited number of response options, percentages will not sum to 100%.

LITTLE OR NO SIDE EFFECTS INCLUDING IMMUNOSUPPRESSION

Patients want pemphigus and pemphigoid medications that are safer and better tolerated, especially without chronic immunosuppression and organ toxicities.

“Pemphigus patients need more precise therapy options, ones that are less immunosuppressant and have a better side effect profile on the current crop of shotgun-like immunosuppressants.”

– Robert, living with pemphigus vulgaris

Scott described what he wanted. “Drugs with less side effects, so that the side effects don’t undo so much of the benefit of the drugs.”

– Scott, living with pemphigus vulgaris

“Just something with minimal side effects. … my body has been through torture. I currently have prednisone-induced diabetes. I’ve had a heart attack. I’ve had a stroke. And it’s just all those things, all those – the medicines – that have caused the side effects.”

– Doris, living with mucous membrane pemphigoid
ADDRESSING PATIENTS’ UNMET TREATMENT NEEDS WITH RESPECT TO EFFICACY

Patients want steroid-sparing treatments that address their symptoms more rapidly and more completely. They asked for treatments that are more targeted, including CAAR-T (chimeric autoantibody receptor T cell) therapy. Although it wasn’t a survey response option, many patients asked for a cure, and some just want “more tools in the toolbox”.

“We need better medications that work faster and target just anti-desmoglein antibodies.”
– Joel, living with pemphigus vulgaris

“We need drugs that work or if they’re not going to, don’t work, faster, so that we don’t have to waste a year on a treatment that isn’t going to do it for us. … I had close to a best-case scenario, and it still took three years until I got to remission.”
– Scott, living with pemphigus vulgaris

“[My ideal treatment] would be anything where I didn’t have to take steroids or be on a very low dose of steroids because it destroyed me.”
– Janet, living with pemphigus vulgaris

EASY TO TAKE/DIFFERENT ROUTES OF ADMINISTRATION

Patients would like oral medications due to the ease of administration and the fact that they can be carried easily to be taken throughout the day.

Additional survey results: Patients who indicated that the route of administration would influence their treatment decisions were then asked what route of treatment administration they would prefer. Oral administration was the top response, selected by 54%; followed by topical, selected by 24%; intravenous, selected by 16%; and infusion, selected by 7% (n=138).

“An ideal treatment for me would be … something that I could take orally.”
– Doris, living with mucous membrane pemphigoid

“It would be a drug that I could take orally so that it would be simpler for me, and I would not have to live my life around, ‘Am I here? Am I there? Is it time?’ I would like to be able to use my alarm clock again to wake me up for work instead of waking me up to take my medications. And that would be really good.”
– Naomi, living with bullous pemphigoid

“In a perfect world, … rituximab or a biosimilar easily delivered into the system. Perhaps a low dosage, pre-dosed injectable pen administered at home or in an easy environment over a longer period of time could be very helpful to pemphigus people.”
– Mark, living with both pemphigus vulgaris and pemphigus foliaceus

COST

Cost influences treatment selection as well as the frequency of treatment, in some cases.

“Just, it’s got to be affordable somehow.”
– Eric, living with bullous pemphigoid
“How often the drug has to be administered, how is it being administered, whether it’s [topical], IV, or oral. And…is it affordable?”
– Doris, living with mucous membrane pemphigoid

“How often the drug has to be administered, how is it being administered, whether it’s [topical], IV, or oral. And…is it affordable?”
– Doris, living with mucous membrane pemphigoid

“Drugs that are prescribed off-label by providers who are experienced at treating MMP should be covered by Medicare Part D Prescription Drug plans.”
– Renee, living with mucous membrane pemphigoid

EASE OF ACCESS
Patients want faster approval of medications that they are already using off-label, access to medications already approved for other indications, and access to innovative new drugs that may not have yet made it through the approval process. A point made by many was that the risk tolerance of those living with pemphigus and pemphigoid is very high and needs to be taken into consideration by regulators.

“The drugs that we have aren’t effective enough, and we need more options. So, we need things like FcRn drugs, and Bruton Kinase inhibitors. We need these drugs because they’re not cancer drugs and we’re not treating these diseases with cancer drugs … [which] really underscores the seriousness of the disease.”
– Marc, living with mucous membrane pemphigoid

“What if that treatment wasn’t such a struggle to get? We need more treatments, and we also need the approvals to go much faster than they do, allowing for off-label use of promising treatments without having to endure the failure ladder… We need more treatments with less red tape so that those of us who are already felled by side effects and who may lack coverage have some choices that allow us to be productive members of our society.”
– Rebecca, living with pemphigus vulgaris

“When you flare, access to drugs in a timely manner so that your condition doesn’t degrade as you’re waiting for an insurance company to approve you. Approval of orphan drugs for pemphigus people.”
– Mark, living with both pemphigus vulgaris and pemphigus foliaceus

“I just wanted to make a comment about risk tolerance. I think what a lot of us have shared today is that our risk tolerance for side effects is probably much higher than the people who are making decisions about drug approval, and that it would, I think, be great if that was taken into consideration.”
– Naomi, living with bullous pemphigoid

EFFECT ON MY DAILY ACTIVITIES
Patients want medications that don’t negatively impact daily activities and mental health. This includes less time spent in the ER and at the doctor’s office.
“For me, the ideal drug is the drug that keeps me out of the emergency room and out of my doctor’s office for an acute visit and lets me go about my daily activities. … [It] is one that makes me feel like I’m a well person instead of a sick person.”
– Naomi, living with bullous pemphigoid

“I would say that anything that would make any person’s life better.”
– Eric, living with bullous pemphigoid

OTHER IMPORTANT TREATMENT GAPS

During the EL-PFDD meeting, individuals living with pemphigus and pemphigoid identified other important treatment and research gaps. These include faster diagnosis and time to treatment, more information regarding treatment protocols, treatment coaches and better mental health support, the inclusion of patient reported outcome measures in studies, more research into the influence of diet, and greater awareness of gender bias and the inclusion of female patients.

“What do I wish for? Until there is something better, [protocols to administer] rituximab first before steroids. We need the best infusion protocol for patient outcomes. We need more drug options specifically targeting our disease, and we need better dental protocols and drugs for our gums and tongues and mouth washes and cleaning protocols. We need the information online and available to all dermatologists and dentists.”
– Lori, living with pemphigus vulgaris

“I strongly believe that more patient reported outcome studies assessing both health status and health utilities are needed to explore the full impact of pemphigus and treatment of pemphigus.”
– Joel, living with pemphigus vulgaris

“Moving forward, front line physicians across an array of specialities, especially dermatology and dentistry, need to be better educated not just about this disease but about how their own biases against women are causing malpractice. And finally, clinical trials should prioritize natal female subjects. Science based on predominately male subjects should be re-examined and potentially rejected.”
– Cara, living with ocular cicatricial pemphigoid/mucus membrane pemphigoid

Defining meaningful improvement: acceptable reduction in disease activity

An ideal future treatment for pemphigus and pemphigoid would be a curative treatment with minimal side effects from treatment. However, during the meeting patients emphasized that even modest improvements in disease activity can greatly improve quality of life.

In the absence of a cure or total remission, patients were asked what they would consider to be an acceptable improvement in their condition that a treatment could provide. Post-meeting survey results are shown in Figure 12. Note that each poll respondent could select as many response options as they would like, which is why the total adds up to more than 100%. Although most (72%) respondents would ideally like to have no lesions, over half of patients (54%) said that they would consider either a 90% improvement or to have up to 1 blister/erosion/lesion an acceptable improvement of their disease. Importantly, patients selected all the poll response options; the message is that meaningful improvement needs to be significant, but it doesn’t need to be perfect.
Figure 12: What would you consider to be an acceptable improvement in your condition that a treatment could provide? Check all that apply.

<table>
<thead>
<tr>
<th>Improvement Description</th>
<th>Percentage of Respondents</th>
</tr>
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<tbody>
<tr>
<td>To have no lesions</td>
<td>72%</td>
</tr>
<tr>
<td>To have at least 90% improvement in the number of lesions or disease activity</td>
<td>37%</td>
</tr>
<tr>
<td>To have up to 1 blister/erosion/lesion</td>
<td>17%</td>
</tr>
<tr>
<td>To have at least 75% improvement in the number of lesions or disease activity</td>
<td>12%</td>
</tr>
<tr>
<td>To have up to 2 blisters/erosions/lesions</td>
<td>7%</td>
</tr>
<tr>
<td>To have at least 50% improvement in the number of lesions or disease activity</td>
<td>5%</td>
</tr>
<tr>
<td>To have up to 3 blisters/erosions/lesions</td>
<td>5%</td>
</tr>
<tr>
<td>To have up to 5 blisters/erosions/lesions</td>
<td>2%</td>
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</tbody>
</table>

Percentage of respondents who selected each option (n=443). Each respondent selected an average of 1.6 responses.

In absence of a cure, some described how they would settle for remission, lesions that resolve, or even a period of minimal disease activity. Participants expressed a variety of opinions on what the ideal outcome of treatment would be.

*Measuring bullous pemphigoid disease activity is difficult. “You can be itching all over and only see red simple signs. You don’t even see bumps. So, it’s hard to measure that…I would take anything that’s an improvement over what you have. … a little bit of improvement is better than no improvement at all. And so, I don’t think that there ought to be a limit like it has to be 25% better than the previous treatment.”*

– Eric, living with bullous pemphigoid

“The disease for pemphigus vulgaris just gets worse. So how do we decide what is the best amount of disease activity that you can live with because for pemphigus, there is no best way to live with it unless I am, with occasional lesions that go away.”

– Janet, living with pemphigus vulgaris
Incorporating Patient Input into a Benefit-Risk Assessment Framework

The pemphigus and pemphigoid EL-PFDD meeting provided an opportunity for the FDA and others to hear the patient voice and to better understand the unmet needs of the pemphigus and pemphigoid community.

The FDA is interested in two particular categories of the patient experience. First, the burdens of the disease and impacts on patients’ daily lives, and second, patients’ perspectives on the adequacy of available therapies. These two categories of information help the FDA understand what types of benefits will matter most to patients. Patient and caregivers are in a unique position to help the FDA calibrate how much risk a patient population would tolerate for a given set of benefits.

Table 1 (shown on next page) speaks to the challenge of having a lifelong disease burden that patients living with pemphigus and pemphigoid endure. It serves as the proposed introductory framework for the Analysis of Condition and Current Treatment Option to be adapted and incorporated in the FDA’s Benefit-Risk Assessment. This may enable a more comprehensive understanding of these disorders for key reviewers in the FDA who would be evaluating new treatments for pemphigus and pemphigoid. The data resulting from this meeting may help inform the development of disease-specific, clinically meaningful endpoints for current and future clinical trials, as well as encourage researchers and industry to investigate better treatment.

The information presented captures the perspectives of patients living with pemphigus and pemphigoid presented at the January 25, 2023, EL-PFDD meeting. The collective hope of pemphigus and pemphigoid patients is that this meeting will encourage future research and successful new product development for people living with these diseases who urgently need treatment options.

Note that the information in this sample framework is likely to evolve over time.

“I live with the freedom derived from gratitude for where I am on this journey, while also feeling like my toes are on the edge of a cliff most days. Because I know remission is not a cure, and I fully understand that pemphigus vulgaris is a chronic and unpredictable condition, and therefore my journey is far from over.”

– Staci, living with pemphigus vulgaris
### Table 1: Benefit-Risk Table for Pemphigus and Pemphigoid

#### Evidence and Uncertainties

Pemphigus and pemphigoid are severe and life-threatening diseases. These rare, autoimmune blistering diseases involve both B-cells and antibodies that attack and destroy skin and mucous membranes. Blister can appear anywhere on the skin, mucous membranes, and in the GI tract.

Symptoms often take a long time to be recognized. Misdiagnoses are common, and many patients feel that their disease symptoms – and their suffering – are not taken seriously. Stigma is pervasive, and many feel isolated and alone.

#### Conclusions and Reasons

Symptoms can be severe. Patients experience pain, trouble eating and swallowing, fatigue, itching, anxiety, depression, lesions, scarring that can include skin, loss of vision, trouble breathing, and trouble swallowing. Immune suppression is a major issue, as many medications suppress the immune system. Left untreated, blisters can spread.

Pemphigus and pemphigoid are life threatening diseases that impose a heavy burden on the day-to-day lives of patients. Most activities of daily living are impacted, especially eating, personal hygiene, and dental care. These diseases impact relationships and families. Patients have many worries, including worries about infections resulting from prolonged immunosuppression.

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#### Analysis of Condition / Impacts on Daily Activities

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<th>Conclusions and Reasons</th>
</tr>
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<tbody>
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#### Current Treatment Options / Prospects for Future Treatments

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<thead>
<tr>
<th>Evidence and Uncertainties</th>
<th>Conclusions and Reasons</th>
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<tbody>
<tr>
<td>Pemphigus and pemphigoid have few approved therapies and an enormous unmet medical need. Most individuals with pemphigus or pemphigoid try many types of medications – many times these medications are administered off-label and often in combination – to find something that alleviates their symptoms. Not all therapies for pemphigus and pemphigoid work for all patients, and only half have experienced remission. Remission, if achieved, is relatively short for most. Medication side effects can be as challenging as the disease itself, and patients are forced to balance the burden of disease with the burden of therapy. Current treatment options such as long-term systemic steroids can have drastic and harmful side effects. Risks of chronic B-cell depletion and immune-suppression is a real concern for many patients in today’s COVID environment.</td>
<td>The pemphigus and pemphigoid community needs better treatment options. They need steroid-sparing treatments with less side effects that are more targeted and efficacious, and that start working much faster. In absence of a cure, some described how they would settle for remission, lesions that resolve, or even a period of minimal disease activity. Patients need “more tools in the toolbox.” Patients need faster approval of medications that they are already using off-label, access to medications already approved for other indications, and access to innovative new drugs that may not have yet made it through the approval process. The risk tolerance of those living with pemphigus and pemphigoid is very high and needs to be taken into consideration by regulators.</td>
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See the Voice of the Patient report for a more detailed narrative.
APPENDIX 1: AGENDA

IPPF VIRTUAL EL-PFDD MEETING - JANUARY 25, 2023

11:00 am – 11:05 am  Welcome  
Patrick Dunn, IPPF Executive Director

11:05 am – 11:10 am  Opening Remarks  
Marc Yale, IPPF Advocacy and Research Coordinator

11:10 am – 11:20 am  Patient-Focused Drug Development at the FDA  
Dr. Shari Targum

11:20 am – 11:40 am  Pemphigus & Pemphigoid: Unmet Needs  
Dr. Pascal Joly, Rouen, France

11:40 am – 12:00 pm  Current Treatments and Limitations  
Dr. Aimee Payne, Philadelphia, USA

12:00 pm – 12:10 pm  Patient Story Videos

SESSION 1: DISEASE SYMPTOMS AND TREATMENTS: HOW THEY IMPACT THE DAILY LIVES OF PATIENTS

12:10 pm – 12:40 pm  Session 1: Panel of patients, caregivers, clinicians will provide comments concurrent with live polling of the audience

12:40 pm – 1:25 pm  Session 1: Facilitated Group Discussion by Patients

1:25 pm – 2:00 pm  Break

2:00 pm – 2:05 pm  Welcome Back Remarks  
Patrick Dunn, IPPF Executive Director

2:05 pm – 2:25 pm  Burden of Disease, Outcome Measures, and Endpoints  
Dr. Dedee Murrell, Sydney, Australia

2:25 pm – 2:45 pm  Clinical Trial Experience and Meaningful Benefit  
Dr. Victoria Werth, Philadelphia, USA

2:45 pm – 2:55 pm  Patient Story Videos

SESSION 2: PATIENTS’ PERSPECTIVE ON AVAILABLE TREATMENTS FOR THE DISEASE(S), SIDE EFFECTS, AND HOW TO IMPROVE THEM

2:55 pm – 3:25 pm  Session 2: Panel of patients, caregivers, clinicians will provide comments concurrent with live polling of the audience

3:25 pm – 4:15 pm  Session 2: Facilitated Group Discussion by Patients

4:15 pm – 4:25 pm  Public Questions/Comments by Patients (Pre-submitted)

4:25 pm – 4:30 pm  Closing Remarks  
Patrick Dunn, IPPF Executive Director
APPENDIX 2: PATIENT STORY AND PANEL PARTICIPANTS

PATIENT STORY VIDEOS
- Andy, living with pemphigus vulgaris
- Janeal, living with pemphigus vulgaris
- Doreen, living with pemphigus foliaceus
- Wanda, living with linear IgA bullous dermatosis

SESSION 1: PANEL OF PATIENTS, CAREGIVERS, CLINICIANS
- Trina, living with pemphigus vulgaris
- David, living with pemphigus vulgaris
- Debra, living with mucous membrane pemphigoid

SESSION 1: FACILITATED GROUP DISCUSSION BY PATIENTS
- Fred, living with pemphigus vulgaris
- Daphne, living with pemphigus vulgaris
- Mei Ling, living with pemphigus vulgaris
- Gina, living with pemphigus vulgaris
- Arlene, daughter of a pemphigus patient
- Bryon, living with pemphigus vulgaris
- Ellen, living with pemphigus vulgaris
- Marney, living with pemphigus vulgaris
- Staci, living with pemphigus vulgaris
- Ingrid, living with bullous pemphigoid

PATIENT STORY VIDEOS
- Kristina, living with pemphigus vulgaris
- Andrea, living with pemphigus foliaceus
- Mark, living with mucous membrane pemphigus
- Jody, living with pemphigus vulgaris

SESSION 2: PANEL OF PATIENTS, CAREGIVERS, CLINICIANS
- Naomi, living with bullous pemphigoid
- Doris, living with mucous membrane pemphigoid
- Eric, living with bullous pemphigoid
- Janet Segall, living with pemphigus vulgaris

SESSION 2: FACILITATED GROUP DISCUSSION BY PATIENTS
- Mindy, living with pemphigus vulgaris
- Arthur, living with bullous pemphigoid
- Sharon, living with pemphigus vulgaris
- Lori, living with pemphigus vulgaris
- Joel, living with pemphigus vulgaris
- Rebecca, living with pemphigus vulgaris
- Mark, living with both pemphigus vulgaris and pemphigus foliaceus
- Robert, living with pemphigus vulgaris
- Scott, living with pemphigus vulgaris

PUBLIC QUESTIONS/COMMENTS BY PATIENTS
- Jaime, living with pemphigus vulgaris
- Isobel, living with mucous membrane pemphigoid
- Lou, living with bullous pemphigoid
- Odette, living with pemphigus vulgaris
APPENDIX 3: DEMOGRAPHIC RESULTS

The graphs below include patients and caregivers who chose to participate in the post-meeting survey after the January 25, 2023, meeting. The number of individuals who responded to each polling question is shown below the X axis (n=x).

Please indicate your gender

- Female: 73%
- Male: 26%
- Prefer not to answer: 1%

Please indicate which disease you have:

- Pemphigus vulgaris (PV): 45%
- Bullous pemphigoid (BP): 24%
- Mucous membrane pemphigoid/Ocicular cicatricial Pemphigoid (MMP)/(CP): 21%
- Pemphigus foliaceus (PF): 7%
- Other, including pemphigoid gestationis (PG): 3%
- Linear IgA pemphigus: 0%

Please indicate the country in which you reside

- United States: 75%
- United Kingdom: 9%
- Canada: 7%
- Other*: 4%
- Australia: 2%
- India: 1%
- Singapore: 1%
- Bulgaria: 1%
- South Africa: 1%
- New Zealand: 1%

*At least one individual attended from each of the following countries: Cambodia, Cyprus, France, Greece, Israel, Italy, Japan, Kenya, Mexico, Portugal, Qatar, Russia, Saudi Arabia, Sweden, Switzerland, Trinidad, West Indies, Türkiye, Ukraine.
APPENDIX 4: SUBMITTED PATIENT COMMENTS

To ensure that as many voices as possible were heard, patients or caregivers of individuals with pemphigus and pemphigoid were invited to submit written comments through the online portal. Submitted comments are presented in this document with some excerpts included in the main body of the Voice of the Patient report.

David, living with ocular cicatrical pemphigoid/mucous membrane pemphigoid

It took over 10 years to identify my complaint. I think it originally started with many referrals to the dental hospital due to inflamed gums and they could not identify a cause. Then I had problems with stinging eyes and blepharitis and pterygium. I saw a number of eye specialists over 10 years and eventually was diagnosed with OCP/MMP after a biopsy. I was then put on a course of cyclophosphamide which helped. I was down for monthly hospital visits and at every one I had to have eyelashes removed. I have lost most of my teeth due to the gum inflammation. I was then put on a course of mycophenolate. I had a dental operation for broken teeth which inflamed the OCP/MMP and there was resultant more scarring. I have regular blood tests and over the last 10 years have had to supplement with Vitamin B12 tablets and at one stage specific iron deficiency tablets. Because of constant ingrown eyelash problems, I had an operation to have the follicles removed on my lower left eye. I have had two cataract operations and the first one involved a three-day visit to the hospital as I had to have infusions before and after the operation and for the second on just the one infusion

My situation seems to have now stabilized but I do suffer from tiredness and heavy mucous clots down the back of my throat which has caused bad choking fits on occasions. The one plus point from taking mycophenolate is the fact that my asthma has almost disappeared so perhaps there is a connection?

Alison, living with pemphigus vulgaris

My PV typically begins in my mouth and spreads throughout my mucosa. Blistering is all internal. If the PV progresses quickly before treatment is received (which is often the case) it can present challenges eating and extreme fatigue. Fatigue is one of the biggest challenges as it makes my day unpredictable. Work and family obligations can suffer. I no longer take prednisone - although it was helpful, the symptoms from the steroids were intolerable. Withdrawal of prednisone was also very difficult. I now receive infusions of rituximab. I generally tolerate this well (Benadryl is required during infusions), but I experience extreme achiness and fatigue after several days. It also takes up to four months to work, which is frustrating when dealing with prolonged symptoms. The oral rinses provided to help alleviate symptoms do not work. Having oral rinses or other medication to help alleviate symptoms/pain while the Rituxan is working would be wonderful.

Cara, living with ocular cicatrical pemphigoid/mucous membrane pemphigoid

The number one issue facing patients is medical misogyny. Most bullous autoimmune disorder patients are women AND yet, we are not believed by healthcare providers and our symptoms are diminished, resulting in delayed diagnosis. Additionally, [clinical] trials often do not account for the very real biological differences between natal women and men. Moving forward, front line physicians across an array of specialities, especially dermatology and dentistry, need to be better educated not just about this disease but about how their own biases against women are causing malpractice. And finally, clinical trials should prioritize natal female subjects. Science based on predominately male subjects should be re-examined and potentially rejected. If the male/female incidence ratios were reversed (aka Ankylosing spondylitis) male experiences and needs would be prioritized.
Renee, living with mucous membrane pemphigoid

I was diagnosed with mucous membrane pemphigoid (MMP) in December 2019. It causes blisters in the mucous membranes such as the mouth, the eyes, the nose, the esophagus, the genitals, etc., that leave permanent scars after they heal. This is such a rare disease that little research has been done on it. There are no approved treatments for MMP. All drugs are prescribed off-label and therefore are not covered by any Medicare Part D Prescription Drug plans. I initially took mycophenolate mofetil to control my disease. My disease has recently been diagnosed in my eyes, and I now get rituximab infusions. Both mycophenolate mofetil and rituximab are strong immunosuppressants.

This is a problem because patients who take these medications are immunocompromised and are at risk of serious illness or death if they get COVID-19. They are also at high risk for flu, pneumonia, melanoma, and other diseases and infections. Although most people have put the COVID-19 pandemic behind them, many patients who take these medications continue to live somewhat isolated lives and are unable to engage in their usual work, recreational, or other activities. I did not see my family or grandchildren for three years because they live 1300 miles away, and it was not safe for me to travel due to being immunocompromised. I would like to see better drugs to treat MMP that do not force the patients into isolation. Drugs that are prescribed off-label by providers who are experienced at treating MMP should be covered by Medicare Part D Prescription Drug plans. It should be disclosed in writing to people before they sign up for a Medicare Prescription Drug plan that drugs that are prescribed off-label by experienced physicians will not be covered by the plan for which they are paying premiums every month.

Judith, living with mucous membrane pemphigoid

I am a 78-year-old grandmother who is battling diabetes, glaucoma, skin cancer, chronic pain, and mucous membrane pemphigoid (MMP), a disease I have had for eight years without remission. During that time my friends and family have helped very little. My best friend dropped our relationship, and my son seems to think the knowledge I have is all I need. As you know, people with rare diseases have unique challenges. Most people haven’t heard of MMP, so they minimize the seriousness and think you are exaggerating. Bottom line: I am my own caregiver. While I love cooking and nutrition, I struggle with household chores.

Since I am retired and living on Social Security, I cannot afford to hire helpers. Moreover, it has required seeing four dermatologists and five dentists over the past few years until I finally found ones who understand my illness, have the resources to support the disease, and care enough to help. I have been treated with virtually every medication recommended for MMP, and so far, all have failed or had too many negative side effects. I’ve also tried nutrition (AIP or autoimmune protocol diet), which has helped somewhat. It’s been important to leave any medical providers who were not helpful or understanding.

To give you an idea about my symptoms, here are the main ones: itchy blistering of the skin and mucous membrane including nose, oral cavity, and sinuses. Blistering of the esophagus and stomach as well as gums and scalp. Squamous gingivitis with nine cavities and four dental extractions. Extreme fatigue, depression, and anxiety.

I have been homebound for most of the past eight years, only leaving the apartment for medical appointments. Fortunately, I’m a computer person, so I do all my shopping, including groceries, online. My one volunteer helper picks up my groceries and prescriptions and takes me to doctor’s appointments. I’m blessed by his generosity and tremendous caring.

At present I am feeling hopeful because I recently found a new dermatologist and a new dentist who is willing to work with this condition. That’s good because it’s hope that keeps me going. Chatting with other patients online is probably the most elevating activity I do because only they understand what I am experiencing. Thanks for the help your organization is providing.
Vivian, living with pemphigoid

I first broke out with pemphigoid in the fall of 2021. I had no idea what was happening. I knew that I had huge blisters on the top of my feet. I went to see a doctor and was incorrectly diagnosed as having vascular blisters. I’ve then looked up my symptoms to satisfy my mind about what was really happening. I am a nurse and it didn’t look like vascular blisters to me. I then went to see a dermatologist and she is and was a great dermatologist. She did a biopsy on one of my blisters and the answer came back pemphigoid.

I was treated immediately with high doses of prednisone. I then developed secondary problems from the medication. I had very high blood pressure. It was not being controlled and I’ve gained 40 pounds within a few months. I felt horrible. I didn’t feel like myself. I had the energy, I just wanted this to go away. We finally decided on clobetasol on the wound, and that seemed to help. I also suffered, and I can’t stress the word “suffered” enough from blisters. They’re in my anal area; you talk about a mess, this was a mess. I couldn’t see it. It was hard for me to treat it and it was so painful to sit and lay down. I caught COVID in January 2022 and ended up in the hospital. My body was so miserable from the outbreaks of blisters along, not to mention the COVID, which hit when I was very weakened from the pemphigoid. This went on until May 2022. I started on Rituxan and in May I begin to heal. I had one large blister that formed in October 2022 on my groin. That was it as far as the symptoms went, I have just now this week broke out in new lesions in the anal area again. I am once again feeling weak, tired, and in pain (that area affected is small). However, the pain from it takes over my entire hip, causing me to walk funny, sit funny, and feel like crap again all over. We need some research to find out how to prevent these outbreaks, and how to take care of them safely. If I can be of any assistance, let me know. Thank you for being there.

June, living with pemphigus vulgaris

I am very upset that the FDA apparently does not approve of giving mycophenolate for pemphigus vulgaris patients. As a result, Medicare and my secondary insurance Anthem does not cover it for my pemphigus.

Mycophenolate works for me without too many side effects. In the past, I’ve taken prednisone which resulted in me becoming a diabetic. I was given dapsone which resulted in life threatening anemia. I ended up having 12 months of IVlg which, with mycophenolate, resulted in a remission for a period of time. After some flare-ups, I’m back on mycophenolate which I have been buying from GoodRX at a reasonable price, but I always worry that it might not be available when I need it.

It’s outrageous that the FDA does not support the use of mycophenolate for my pemphigus vulgaris. It’s the only drug without horrible side effects that works for me. I feel angry and think that it is unfair that I am harmed by this FDA policy.

FDA: please change this policy.

Josie, living with pemphigus vulgaris

I had been living with pemphigus vulgaris for eight years. After a two-year IVlg infusion treatment, I am currently in remission from the pemphigus vulgaris disease. However, the lab results show the antibodies are active.

My life has changed dramatically. I have not been able to resume my daily activities including my job as a banker. It is frustrating and depressing.

More research needs to be done to treat rare diseases, including pemphigus vulgaris.

My life has changed dramatically.
APPENDIX 5: PATIENT DIALOGUES WITH EXPERTS

The January 25, 2023 EL-PFDD meeting featured leading experts in Autoimmune Bullous Diseases.

• Dr. Pascal 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

During the panel discussions, these experts reflected and responded directly to what they heard patients say. Many important recommendations emerged during their dialogues with patients and are summarized below.

### Pemphigus and pemphigoid patients said: Diagnosis can take a long time; existing treatment options have many downsides including length of time required to work or side effects such as sustained immunosuppression.

**Expert reflections:**
These diseases are rare, patients are highly stigmatized, and the impacts on day-to-day life, especially employment, are significant. Most medications take three months or more to have a therapeutic effect, during which time patients can experience severe symptoms, including mouth blistering or total body blisters.

**Expert recommendations:**
1. Increase awareness about the severity of these rare diseases in the medical community as well as among the general public. Create awareness and education around the lack of treatment options for patients. Disseminate information and increase awareness regarding treatment complications and comorbidities.
2. Develop faster-acting, better tolerated treatments for pemphigus and pemphigoid. New treatments will ideally avoid chronic immunosuppression or perhaps can be easily discontinued if necessary.
3. Consider self-administration options including oral, topical, or subcutaneous formulations.

### Pemphigus and pemphigoid patients said: Steroid side effects are devastating.

**Expert reflections:**
Pemphigus and pemphigoid are relapsing diseases, and some patients require longer term cumulative steroid doses. Chronic steroid side effects, in most cases, can have major quality of life impacts.

**Expert recommendations:**
4. Consider minimal therapy, that can often provide significant disease control, which is often a primary goal for patients; complete remission is often a secondary consideration. New international consensus definitions of minimal therapy for pemphigus, pemphigoid, and mucous membrane pemphigoid were recently established and are in the process of being published. These new consensus recommendations are for prednisone per kilogram of body weight per day, in contrast to the original guidelines which defined minimal therapy as less than or equal to 10 milligrams per day of prednisone, independent of body weight.

CONTINUED
5. Use the recently published consensus definitions of minimal dose therapy, complete remission, and partial remission on minimal therapy or off therapy. Note that these new definitions pertain to standard of care and do not necessarily dictate clinical trial endpoints.

6. New therapies will require specific clinical trial endpoints and minimal therapy definitions, based on half-life and mechanisms of action.

Pemphigus and pemphigoid patients said: **Even modest improvements in disease activity can greatly improve quality of life.**

**Expert reflections:**

Clinical trials for rare diseases have fewer patients, and so require carefully chosen endpoints that are validated, sensitive, and meaningful from a patient perspective. Many pemphigus and pemphigoid clinical trials are using the Investigator Global Assessment (IGA), which is inappropriate for the following reasons:

- IGA is too simplistic, insensitive, and not specifically validated for diseases with such wide manifestations and severity as pemphigus and pemphigoid.
- IGA requires patients to achieve an endpoint of “clear” or “almost clear” disease, which is inappropriate for diseases that cause skin and mucosal fragility and where even the slightest trauma (for example, eating lunch) can induce a blister.
- IGA scores may include skin damage, making it more difficult to demonstrate that treatments are working for this patient population.

**Expert recommendations:**

7. Meaningful improvement needs to be defined in a way that is beneficial to pemphigus and pemphigoid patients. The community needs clinical trial endpoints that are appropriate relative to the burden of disease and the risks and limitations of current therapies.

8. Experts would appreciate consideration of validated disease specific metrics for clinical trials: the Pemphigus Disease Area Index (PDAI); Autoimmune Bullous Skin Disorder Intensity Score (ABSIS); Bullous Pemphigoid Disease Area Index (BPDAI); Mucous Membrane Pemphigoid Disease Area Index (MMPDAI); and the Epidermolysis Bullosa Disease Activity and Scarring Index (EBDASI). These can scientifically measure the disease, severity of the disease, and the responses to the treatment better than IGA scores.

9. Clinical endpoints for pemphigus and pemphigoid need to separate disease activity, which is reversible, versus skin damage and scarring, which is left behind by these diseases and that new drugs are unlikely to influence.

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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.

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