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As we begin a new year and new decade, I am excited about what the future holds for the IPPF and all those affected by pemphigus and pemphigoid (P/P). As demonstrated in our 2019 Year in Review Infographic, our community made great strides in our main areas of focus: outreach and patient services, research and treatments, awareness, and advocacy. We have made progress but there is still work to be done!

By sharing our stories about living with these diseases with doctors, researchers, other disease groups, decision-makers, and one another, our community will continue to grow. P/P are rare diseases, but collectively we have a loud voice.

When we band together, our stories will:

- Accelerate basic research and drug development through IPPF initiatives that collect vital disease information, like the IPPF Natural History Study and IPPF Biobank
- Improve access to life-saving treatments and quality healthcare by advocating with law-makers and regulatory agencies
- Create even more disease awareness by educating practicing and future medical professionals
- Provide hope and support for each other when they are most-needed

In 2020, the IPPF will gather key members of the P/P community to raise disease awareness, advocate for unmet medical needs, and accelerate the path for new treatments. Globally, we must assess the current state of treatments, steroid burdens, the role of clinical research, and our ability to overcome treatment barriers. To meet these challenges, we need everyone in our community to participate. You can make a difference by letting us know how we can help you, pledging to join a study that advances research, or sharing your story with policy makers. Although there are many obstacles ahead, we can improve the lives of all those affected by pemphigus and pemphigoid! Together we are going to make 2020 a milestone year!

Marc Yale
IPPF Executive Director and MMP Patient
marc@pemphigus.org
Pemphigus and pemphigoid (P/P) are close to my heart, as my mother has struggled with pemphigus vulgaris (PV) for most of my adolescence and early adulthood. Watching her live with PV and listening to her ask why? inspired me in my pursuit to become a physician, seek a career in dermatology, and work toward enhancing the research of external factors contributing to the onset of P/P.

The last time I reached out to the IPPF community, I asked for participation in my research project. Thank you to everyone who took the time to complete the online survey. We received responses from the United States, United Kingdom, Canada, Europe, the Middle East, Australia, South America, and India, with a total of 397 participants. Thank you for helping us to take a step forward and hopefully inspire more research on the causes of P/P.

The survey asked participants about their lives before the onset of P/P, as well as the age of disease onset, allergies, diet, exercise, occupation, pre-selected stressors, and significant life events and moments. Survey results have been published in the December 2019 issue of the Journal of the American Osteopathic Association. I also had the opportunity to present the research at the Osteopathic Medical Education (OMED) Conference in Baltimore, MD, last October.

Data Analysis

After participants completed the survey, the data was exported to statistics software for further analysis. The responses for food in the diet were charted and found to be near equal across most responses, so further analysis was not completed. Specific allergies mentioned were reviewed. The most frequently mentioned allergies were selected, but were not further analyzed as they were not found to be significant.

Written responses for major, stressful life events were reviewed and tallied for the mention of stress as a result of divorce, work, home life, romantic relationships, friendships, finances, school, illness in family/friends/pets, death in family/friends/pets, caregiving, illness in self, multiple or frequent surgeries, recent dental cleaning or procedures, physical or emotional trauma, pregnancy, birth, difficult pregnancy, medications, new or lost home/job, major moves to new city or state, and retiring.

Written responses of significant moments were reviewed and recategorized into personal/social events, health events, and occupational events. The optional written responses were reviewed and were not analyzed.

Results

The survey had a 91.4% completion rate. Females predominated at 73% with 51.85% of participants with a diagnosis of PV, 21.43% bullous pemphigoid (BP), and 19.05% mucous membrane pemphigoid (MMP). Disease onset increased with age; onset was highest over the age of 60 at 34.31%, and second highest between the ages of 51 and 60 at 26.60%.

The notable occupation categories were management/business/finance (30.77%), health care/allied health (14.06%), and education/social services (12.79%). The pre-selected stress events with the most positive responses were 31.71% ever having surgery, 22.41% having a minor bacterial or viral infection, and 15.43% using tobacco products.

Exercise frequency was highest at 34.76% for one to two times per week, followed by 28.88% for three to four times per week, 20.86% never exercising, and 15.51% exercising five or more times per week. Stressful major life events before disease symptom onset were
experienced by 68% of respondents, with an average of 2.16 events per respondent.

The most commonly mentioned events were stress from work (13.23%), stress from the death of a family member/significant other/friend (9.62%), stress from home/family life (9.22%), and stress from an ill family member/significant other/friend (8.42%). The descriptors “intense,” “severe,” “constant,” “crisis,” and statements including “life in general” were often used to describe stress.

Significant moments near symptom onset were experienced by 55% of respondents, with mention of sudden stress/anger; exposure to extremes in heat; travel to hot, tropical, dry locations; and recent illness with antibiotic or anti-fungal use.

Conclusion

Autoimmune bullous diseases are complex and difficult to study with multiple potential factors that could play a role in the onset of disease symptoms. These diseases have genetic components and are thought to be multifactorial, meaning that one or more external factors can cause the body enough insult to trigger disease in people with certain genetic mutations. The goal of this study was to ask patients with P/P about their life before disease symptoms to find areas that could be studied in more depth in the future.

There may be a relationship between experiencing multiple stressful events—particularly extremes in emotion, environmental changes, and persistent stress events—with onset of disease, especially in conjunction with stressful occupations.

We cannot claim that stress is causal in autoimmune bullous dermatoses, but the body’s response to stress may play a role. Future studies focused on the physiological response to stress and coping mechanisms to stress in individuals with genetic susceptibility could be promising, but we cannot exclude other external factors.

Thank you again for your participation. With each research project, we learn more and can hopefully reach an even better understanding of P/P.

Mandy Alhajj is a fourth-year medical student at Lincoln Memorial University-DeBusk College of Osteopathic Medicine in East Tennessee.
In 2018, PEM Friends, the UK support group for people with immune-bullous diseases, was invited to participate in a Priority Setting Partnership (PSP) by the UK Dermatology Clinical Trials Network (UK DCTN). After eagerly agreeing to something we knew nothing about, we discovered that this PSP was intended to establish the top issues related to the treatment of pemphigus vulgaris, bullous pemphigoid, and mucous membrane pemphigoid that required investigation or research.

A PSP enables clinicians, patients, and caregivers to work together on an equal footing to prioritize particular areas of health and care that could benefit from research. Facilitated by the James Lind Alliance, this PSP is funded by the Nottingham Hospitals Charity and coordinated by the UK DCTN at the Centre of Evidence Based Dermatology. It is also organized by the PSP partner organizations.

Our work started by refining the communications and the wording for the first survey. This task was surprisingly tough. We wanted to speak to as many medical professionals, patients, and caregivers affected by these diseases as possible, and it was a challenge to clearly explain our goals to these groups in a simple way.

Despite the fact that the PSP includes PEM Friends, patients, dermatologists, ophthalmologists, and dermatology nurses, our biggest challenge has been finding contributors for the survey. Clinics run by experts in these diseases in the UK are few and far between. They hand out information and copies of the survey; however, we need to reach as many people as possible, and unfortunately there are no lists of patients with our
rare diseases available. We are hoping to get as many UK-based patients, caregivers, and healthcare professionals as possible to participate by spring 2020.

Once enough participants have contributed to the first survey, we will be able to carry out a second survey that will ask people to prioritize questions about treatments. We will then create a shortlist. Finally, the patients and clinicians who express interest will be invited to a workshop to establish the top 10 issues related to treatment.

Currently, we’re halfway through the process of collecting responses from the first survey and need to step up our efforts to get as many respondents as possible. The survey includes up to five questions about disease treatment and will provide valuable data on what patients and medical experts see as the most important aspects of pemphigus and pemphigoid treatment.

In conjunction with the PSP, members of PEM Friends are also involved in another project led by researchers at the Centre of Evidence Based Dermatology. This work will help design studies to address the results of the PSP. One study will show how many people in England have developed bullous pemphigoid in the last 20 years, and whether the number of people affected has changed or varies by geographical region. We will also describe the dose and length of time that people are prescribed steroids, as well as any reasons why patients might not be able to take part in a future trial.

We hope that our work with the PSP is the start of something important for generating a deeper understanding of these diseases. If you are based in the UK, please consider providing your input to the survey. Every contribution makes a difference, and the more participation we can get, the more informed future research will be. To access the survey, go to www.surveymonkey.co.uk/r/PEM-PSP.

More information about the UK DCTN and the James Lind Alliance can be found at www.ukdctn.org and www.jla.nihr.ac.uk/priority-setting-partnerships.

Isobel Davies runs PEM Friends, a support group for P/P patients and caretakers in the UK. Isobel was diagnosed with MMP in 1994 and still struggles with the effects of the disease. She lives in Buckinghamshire with her husband, Phil.
Rare Disease Legislative Advocates (RDLA), a program of the EveryLife Foundation for Rare Diseases, will bring together over 800 patients, caregivers, and others in Washington, DC for a week of events dedicated to empowering patients, families, friends, and healthcare professionals to become legislative advocates. During the week of February 25-28, 2020, rare disease advocates will have an opportunity to meet with members of Congress and to learn about policy updates and best practices for successful advocacy.

Rare Disease Week on Capitol Hill 2020 kicks off on February 25th with a Rare Disease Congressional Caucus lunch briefing followed by a documentary screening and cocktail reception in the evening.

Rare disease advocates will attend the all-day Legislative Conference on February 26th to learn about federal legislation and policies that affect the rare disease community. Policy experts from Capitol Hill, nonprofit organizations, and industry will share their expertise with advocates. In addition, advocacy professionals will provide opportunities for participants to refine techniques for effective advocacy on the Hill and build strong relationships with their Members of Congress.

On February 27th, a Hill Day breakfast will be held with keynote speakers. After breakfast, 500 rare disease advocates will go to Capitol Hill to meet with their Senators, Representatives, and congressional staffers to discuss key legislation, policies, and the Rare Disease Congressional Caucus. Later in the evening, the Rare Artist Reception will feature the 2019 Rare Artist contest winning artwork and highlight the importance of art as therapy for rare disease patients.

We will close out the week at the National Institutes of Health (NIH) in Bethesda, Maryland. On February 28th, the NIH will host Rare Disease Day at NIH. This event aims to raise awareness about rare diseases, the people they affect, and NIH research collaborations to advance new treatments. The EveryLife Foundation will be there to meet patients and provide information on their programs as well.

In addition, the Food and Drug Administration (FDA) has announced a Rare Disease Day public meeting at the FDA White Oak Campus in Silver Spring, MD, on Monday, February 24th.

Information on Rare Disease Week on Capitol Hill 2020, the week’s events and locations, travel and hotel accommodations, and registration for the event can be found at rareadvocates.org/rdw.

We are excited for this powerful week of events and to continue to advocate with the rare disease community.

Shannon von Felden is a healthcare policy and advocacy professional with experience on Capitol Hill and with national nonprofit organizations. She joined the EveryLife Foundation in 2018 and is the Director of the Rare Disease Legislative Advocates Program.
IPPF Launches
2020 Corporate Council

On January 1st, the IPPF launched the 2020 Corporate Council, a collection of top stakeholders that are committed to facilitating collaboration and information exchange among patients, the IPPF, key opinion leaders, health-related organizations, and industry. We believe that only through an open and cooperative collaboration among all stakeholders will we effectively be able to address issues facing the pemphigus and pemphigoid (P/P) community.

The IPPF Corporate Council will focus on three areas of emphasis: access, advocacy, and awareness. Within these focus areas, the IPPF Corporate Council will develop and support activities and programs that ensure the IPPF fulfills its mission of improving the quality of life for all people affected by P/P through early diagnosis and support.

Membership in the IPPF Corporate Council will demonstrate an organization's leadership and commitment to addressing the challenges facing our patients. Involvement in the Council will also provide the IPPF with an extended network of expertise and insight to ensure that we continue to address the unmet needs of all patients.

The Corporate Council will provide hope and support by:

• Enhancing awareness, knowledge, and attention of P/P in society and among key audiences
• Organizing the IPPF community to drive progress in advocacy, awareness, research, drug development, and access to care
• Encouraging an industry, academic, and community collaborative investment in, and development of, new/improved treatments and therapies
• Promoting more effective identification, diagnosis, and treatment of P/P to improve patient experiences/outcomes

Please join us in welcoming our newest members to the 2020 IPPF Corporate Council:
The IPPF Meets with the Centers for Medicare and Medicaid Services

Marc Yale

On December 19th, the International Pemphigus and Pemphigoid Foundation (IPPF) and the American Academy of Dermatology Association (AADA) met with Jeet Guram, MD, MBA, who is the Senior Advisor to Seema Verma, the administrator of the Centers for Medicare and Medicaid Services. The purpose of the meeting was to share problems with drug compendia and highlight access to treatment issues related to both Medicare Part B and Part D. According to MJH Life Sciences, “drug compendia are defined as summaries of drug information that are compiled by experts who have reviewed clinical data on drugs. According to the Centers for Medicare and Medicaid Services (CMS), a compendium should include a summary of pharmacological characteristics for each drug/biological that may include dosage and recommended uses.”

In attendance at the meeting were Joerg Albrecht, chair of AADA’s Drug Transparency and Access Task Force; Marc Yale, IPPF Executive Director; and AADA staff; as well as representatives from the American Society of Transplantation, American Society of Clinical Oncology, American College of Rheumatology, American Gastroenterological Association, and the American Society for Mohs Surgery.

The meeting began with an overview of the problems with the compendia and how they impact access to treatments across multiple specialties. CMS’s reliance on the compendia is restricting the ability of physicians to treat orphan, rare, and even common diseases. These limitations prevent patients from getting medically necessary treatments. We explained how the compendia often deviate from the standard of care for treatment, or worse, how there is simply no treatment listed in the compendia for a disease. In some cases, a physician has to prescribe a more costly or less effective treatment. As a result of this barrier, a subsection of Medicare Part D beneficiaries does not
have adequate drug coverage. Unfortunately, there is no exception process, and CMS was encouraged to consider developing one.

The lack of inclusion of necessary treatments in the compendia, the high costs resulting from barriers to lifesaving and life-enhancing drugs, and the lack of a process for exemptions, were discussed. CMS was very interested in these concerns and indicated a willingness to continue a dialogue and seek a solution.

During the meeting, I described the IPPF’s observation of an increasing number of pemphigus and pemphigoid patients who are experiencing issues related to the lack of inclusion of treatments in the compendia. Patients, and even medical facilities, reach out to the IPPF for help in accessing treatments for these diseases. We described a case in which a patient went through a legal battle for an entire year just to be told they still could not access the necessary treatment. It was also highlighted that there are access issues with both Medicare Part B and Part D.

Overall, the compendia are not keeping up with the rate at which science is progressing. There are breakthrough treatments patients should be able to access to improve their overall quality of life, or even increase their chances of survival. A process for exemptions could be a solution to this access issue. It was reiterated that finding a solution would meet the CMS administration’s goal of putting patients first.

If you are experiencing access issues with your Medicare coverage or other insurance, contact the IPPF at info@pemphigus.org for assistance. The IPPF advocates with, and on behalf of, the IPPF patient community with congressional representatives, regulators, and other support organizations to encourage legislation and policies that provide better healthcare.

Marc Yale was diagnosed in 2007 with cicatricial pemphigoid. In 2008, he joined the IPPF as a Peer Health Coach and was promoted to Executive Director in 2016. Marc currently resides in Ventura, California, with his wife Beth and daughter Hannah.
OUTREACH & PATIENT SERVICES

- 7 Patient Education Webinars
- Find a Doctor Map accessed 2,207 times
- 938 Patient/Caregiver Cases Closed
- Celebrated the IPPF’s 25th Anniversary at the Patient Education Conference with 208 attendees
- 13 Support Group Meetings
  - New meetings in Chicago and Southern New Jersey/Philadelphia

RESEARCH & TREATMENTS

- PUBLICATIONS
  - 3 peer-reviewed articles written by IPPF research grant participants
  - IPPF research collaborations published in the British Journal of Dermatology and Frontiers in Immunology
- MabThera granted positive opinion by the European Medicine Agency (EMA) for pemphigus vulgaris (PV) treatment
- IPPF NATURAL HISTORY STUDY
  - 140 new participants
  - Presented preliminary scientific results at 4 major U.S. conferences
Launched Biopsies Save Lives campaign aiming to accelerate diagnosis times and reach 41% of practicing U.S. dentists.

14 print ads in dental journals and exhibit conferences, and 8 social media ad campaigns.

5 dental exhibits, reaching 2,153 dental professionals.

16 lectures to 1,296 faculty and dental students.

Presentations at 60% of the top 10 largest dental schools in the U.S.

Signed 16 letters to legislators and regulators supporting rare disease research and patient care.

In-person international consortium meetings with PemFriends UK, the Association of Pemphigus Pemphigoid France (APPF), the National Association of Pemphigus Pemphigoid Italy (ANNPI), and China.

Patients advocated to over 60 congressional members.
What is advocacy?

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

What is an umbrella organization?

The IPPF is a member of umbrella organizations, including the National Organization for Rare Disorders (NORD), Rare Disease Legislative Advocates (RDLA), the International Alliance of Dermatology Patient Organizations (IADPO, also known as Global Skin), the Coalition of Skin Diseases (CSD), Global Genes, and the American Academy of Dermatology (AAD). Umbrella organizations are larger than the IPPF and often include smaller organizations that support patients of specific rare diseases or dermatological conditions. Umbrella organizations often dedicate a portion of their operating budget to research and advocacy priorities that affect their member organizations. They reach out to patient organizations to promote new legislation through “sign-on letters,” where organizations pledge their support and encourage their members to reach out to their congressional representatives.

The IPPF uses the reach of these umbrella organizations to plan large events, like Rare Disease Week on Capitol Hill and Rare Across America, and open doors to political offices through appointments on Capitol Hill and in district offices. Many times, the IPPF is able to meet directly with voting members of Congress on both Capitol Hill and in their district offices. Since it is important to reach as many members of Congress as possible, the IPPF notifies our community about these opportunities to advocate for rare diseases. Your voice is a vital part in getting bills passed that affect P/P patients.

You may hear that the IPPF has “signed on” to support a letter, recommendation, act, or piece of legislation moving through Congress. This means that IPPF Executive Director, Marc Yale, has added the IPPF to the list of organizations that support that
particular issue. An organization may represent thousands of patients, so the number of constituents it can affect can add up very quickly.

How does it fit together?

An example of IPPF advocacy in action is in its support of the Rare Disease Congressional Caucus. This bipartisan caucus meets on Capitol Hill four times per year and includes members of the US Senate and House of Representatives. The IPPF supports the efforts of the RDLA to encourage all members of Congress to be part of the Rare Disease Congressional Caucus, since healthcare should not be a partisan issue.

The IPPF periodically asks patients to attend In-District Lobby Days and Rare Disease Day on Capitol Hill, and to ask their legislative member to join the Rare Disease Congressional Caucus. At these events, patients explain how rare diseases have affected their lives and how members of Congress have an opportunity to work together to help the 1-in-10 Americans living with a rare disease. RDLA reports that these efforts have convinced over 35 congressional members to join the Rare Disease Congressional Caucus. In total, there are currently 103 House of Representative members and 6 Senate members on this Caucus.

Moving forward

The IPPF is always looking for ways to be involved in advocacy efforts that benefit our community by promoting access to affordable healthcare and encouraging robust funding for the National Institutes of Health (NIH) and the Federal Drug Administration (FDA). If you would like to learn more, or to notify us about specific legislation, email advocacy@pemphigus.org.

Becky Strong is the IPPF Outreach Director. She was diagnosed with PV in 2010 and is currently in remission. She lives in Michigan with her family.

Umbrella Organization Highlight: The Coalition of Skin Diseases

The Coalition of Skin Diseases (CSD) advocates on behalf of individuals with skin disease. Currently, there are 16 organizations and 7 affiliate organizations in the coalition. Each patient organization is invited to bring two members to CSD meetings, and they are encouraged to bring a patient.

The IPPF participates in the CSD by supporting basic scientific and clinical research, fostering physician and patient education, generating awareness of skin diseases, and supporting the growth of member organizations through the sharing of mutual concerns. The CSD meets twice a year, once at the American Academy of Dermatology Association (AADA) Legislative Conference in late summer in Washington, DC, and once at the American Academy of Dermatology (AAD) Annual Meeting during the late winter or early spring. This year the AAD meeting will be held in Denver, CO.

There are a number of task forces within the CSD, and the IPPF is a member of two of them. These committees meet throughout the year to accomplish specific goals. This year, Marc Yale (IPPF Executive Director) served on the CSD Advocacy Task Force, where he helped develop the advocacy goals and priorities of the coalition. In 2019, this messaging was delivered to over 60 congressional offices.

Becky Strong (IPPF Outreach Director) served on the Marketing Task Force. She met with select patient organization staff and directors to successfully redesign the CSD logo and develop a new CSD booth display for use at the AAD Annual Meeting Exhibit Hall. The task force also created a new brochure that defined the CSD, listed member organizations, and outlined CSD services that are beneficial to dermatologists and their patients. With the new professional exhibit, the CSD is able to showcase how patient organizations and the CSD can act as a clearinghouse of information to support a dermatologist’s effort to educate and support their patients. The exhibit also includes information about locating experts who can consult on cases in order to give patients the best possible care.

Marc Yale and Becky Strong are happy to bring any issues to the CSD. Email them about the issues that are important to you (marc@pemphigus.org or becky@pemphigus.org). The IPPF also encourages you to connect with the CSD on Facebook (@CSD4You). These efforts are very important to the IPPF as we strive to help all of those affected by P/P.
Perfect Complements:
COMBINING THE PHYSICIAN’S PERSPECTIVE WITH PATIENT EXPERIENCE AT AMERICAN ACADEMY OF DERMATOLOGY MEETINGS

Donna Culton, MD, PhD

As a dermatologist who specializes in treating patients with pemphigus and pemphigoid (P/P), I am humbled every day by how challenging these diseases can be—not only for the patients that suffer from them, but also for the physicians who treat them. I am fortunate to have had excellent training and mentorship from Dr. Luis Diaz, one of the world’s experts in autoimmune blistering diseases. I have also had the privilege to connect with other P/P experts with whom I can share difficult cases. In my own specialty clinic, I see numerous P/P patients and continue to learn from every patient. As with most things in life, the more I see, the more I learn.

Several years ago, I began to share what I have learned about the diagnosis and treatment of P/P with other dermatologists by giving educational lectures at regional and national meetings. Due to the rare nature of these diseases, most dermatologists may only encounter a handful of P/P patients in their entire career. I am well aware that these dermatologists are the “front line” and have the incredibly difficult task of confirming the diagnosis for these rare conditions that they may not have seen since their residency training.

For five years now, I have led a session titled, “Pemphigus and Pemphigoid: Evidenced-Based Updates” at the American Academy of Dermatology (AAD) meeting. The annual AAD meeting attracts over 18,000 international attendees each year and offers over 200 sessions for dermatologists to learn from their colleagues. I developed my session specifically for dermatologists in busy practices with the goal of passing along practical tips for effectively diagnosing...
and treating P/P. The first year, my focus session fell on a Sunday morning at 7:00. I fully expected to give my talk to one or two individuals. To my surprise, the room was nearly full. As it turns out, treating P/P patients is so challenging, it motivated dermatologists to wake up bright and early just to learn more.

Three years ago, I was approached by the AAD Patient Advocacy Task Force about the possibility of having a patient as part of the session. This novel idea was meant to bring the patient experience to life for attendees, thereby highlighting the importance of early diagnosis and proper treatment. What better patient to share their story than one of the IPPF’s own, Outreach Director and pemphigus vulgaris (PV) patient, Becky Strong? Becky’s story is like that of many P/P patients—a story of multiple doctors, many months of suffering, and great desperation before a diagnosis was ultimately made and treatment was begun. Becky’s story has had a profound impact on the physicians who have heard it, with session attendees finding it to be powerful and a helpful way to better understand what it is like to be a patient struggling with these diseases.

Becky’s participation in these sessions has also allowed for hundreds of dermatologists to learn more about the IPPF and the resources it provides to both patients and physicians.

I am pleased to say that our session has grown each year. It is now a forum with an expanded time allotment and additional expert physician speakers. As our session grows, we continue to raise awareness and knowledge of P/P within the dermatology community. We cover a lot of information in our session; however, if attendees only remember one thing, I want it to be the profound impact they can make on one patient’s life and the integral role the IPPF plays in unifying and supporting patients and physicians.

Donna Culton, MD, PhD, is an Associate Professor of Dermatology at the University of North Carolina, Chapel Hill, and the Director of the Clinical Immunofluorescence Laboratory at UNC. She sees pemphigus and pemphigoid patients from North Carolina and neighboring states in her specialty autoimmune clinic.

Find a Doctor: IPPF Physician Map

The IPPF’s Find a Doctor P/P physician map provides patients with contact information for medical and dental professionals familiar with P/P.

Access the map online at: www.pemphigus.org/find-a-doctor
Last year, I was fortunate enough to take part in Rare Disease Week on Capitol Hill, a gathering of hundreds of patients, caregivers, researchers, and other people from all over the United States who share an interest in shaping healthcare policy. Participants spent an entire week learning about healthcare and funding issues affecting the rare disease community, the legislative process, and how citizen advocates can present their stories in ways that are more likely to hold the attention of legislators and staff members.

One of the first things I learned at Rare Disease Week was that the term rare disease is a misnomer of sorts. By definition, any condition affecting fewer than 200,000 people in the United States is considered a rare disease. There are an estimated 7,000 rare diseases, and the total number of patients in the United States is estimated at 25 to 30 million. When you add the number of caregivers, researchers, and practitioners who deal with these diseases, one out of every seven or eight Americans are a part of the rare disease community. Taken in that light, we’re hardly rare at all.

As a pemphigus vulgaris (PV) patient, I was aware of the work the IPPF had been doing to raise awareness and advocate for more research and treatment funding. However, I was not aware that a whole group of similar organizations, along with university medical research departments, had teamed up with Rare Disease Legislative Advocates (RDLA, a program of the EveryLife Foundation for Rare Diseases), to encourage our senators and members of Congress to support efforts to address the challenges of rare diseases. Rare Disease Week on Capitol Hill is one of the primary ways that RDLA draws the national legislature’s attention to those challenges.
Attending Rare Disease Week was an easy decision for me. I had the time and energy available to devote to something I support and the willingness to leave the comforts of my home for a good cause. I also had a curiosity about the process. I had done a little legislative outreach (okay, lobbying) for the Pharmaceutical Manufacturers Association in the late '80s and early '90s, but had never taken part in a large, concentrated effort such as RDLA’s. Could the participating organizations and individuals really expect to influence the thinking of the House and Senate on healthcare policy?

Part of the answer became clearer when I attended the opening reception. Over 800 people from all over the country were there, each with a story about a struggle with rare disease. Anyone present that evening couldn’t help but sense the commitment and positivity of the crowd. Although I met several eager first-time participants, I was mostly impressed by the enthusiasm of people who were there for the fifth or sixth year. These veterans helped reassure me that the effort was more than just an awareness-raising exercise.

The following day consisted of the Legislative Conference, an all-day session starting at 7:00 a.m. It spelled out specific goals for participants’ rounds of congressional offices, outlined proven approaches to presenting our specific issues, and provided practical information such as the protocols and courtesies expected when meeting with our representatives.

Not surprisingly, we were told that meeting with an actual member of Congress was unlikely, and we should anticipate meeting with a staff member specified task with addressing healthcare issues. We were not to consider it a slight or a sign of lack of interest. Staff members would be more conversant with our concerns, would be able to direct their bosses’ focus toward the issues presented, and would be our primary contacts over the next several months to follow up about progress.

The Legislative Conference also provided time for participants to meet with others from their state or legislative district (New Jersey 4th, in my case) to plan the next day’s encounters, select a group leader, and, if needed, determine a “batting order” of which participant would speak and when.

We also received contact information to help us stay aware of schedule changes and to report any difficulties with the appointments. For example, suppose we were to meet with Senator A at 2:00 p.m. Then, Member B was running late for our meeting at 1:00 p.m. and couldn’t meet with us until 1:35 p.m., and it was a 20-minute walk between their offices. If something like this happened, we would get a notice via a dedicated phone app, along with instructions on how to resolve the problem. Likewise, if there was an extended discussion with a staffer that would cut into our next meeting, we had a number to call to help reschedule.

Overall, I came away from the Legislative Conference with confidence that the team I was on was ready to meet with our assigned legislators or staff members, tell our individual stories concisely, and do an effective job of starting to build a relationship with them.

The next day was Lobby Day, which started with another early breakfast and keynote address. One of the most useful items of the entire conference came out of the Lobby Day breakfast: a map of Capitol Hill showing the various House and Senate office buildings and their lesser-used entrances (security lines vary greatly in length), as well as the rabbit warren of individual legislative offices. Capitol Police Officers are accustomed to out-of-towners getting lost and are extraordinarily helpful, but the map was an essential part of the toolkit supplied by RDLA.

The New Jersey contingent of 18 people, in one configuration or another, met with representatives of both our Senators while visiting the offices of the entire New Jersey congressional delegation. I met with staffers from Representatives Chris Smith’s (CD-4) and Josh Gottheimer’s (CD-5) offices, as well as a representative from Senator Cory Booker’s office.

My day on Capitol Hill, aside from reminding me that “I’m Just a Bill” from Schoolhouse Rock just scratched the surface, reinforced my contention that much of the work of government, with oversight by our elected officials, is carried out by a vibrant, talented group of staffers. The congressional staff members we met all shared a few traits. They were young, averaging in age somewhere in their mid-to-late twenties; they were genuinely interested in what we had to say; and they were all really smart.

These encounters also made it clear that the personalized approach is effective in bringing attention to the shared concerns of the rare disease community. It is one thing to say, “We need to maintain adequate funding for the FDA so that it can continue to streamline its
approval of new pharmaceuticals.” It is quite another to 
say, “I am a patient with PV, a potentially life-threaten-
ing disease. Advances in treatment that could save my 
life and those of thousands of others will be delayed if 
FDA appropriations are cut. I am requesting the mem-
ber’s support of the bill that would assure continued 
funding for this critical matter.”

Telling our own stories made a difference. In those 
meetings with several of us talking about our per-
sonal situations, I could see the staffer paying greater 
attention, I suspect, than if we had come in with just a 
recitation of facts.

The following day, I attended a briefing of the Rare 
Disease Congressional Caucus, a growing bipartisan 
group of Senators and Representatives who have com-
mitted to giving a voice to the needs of those affected 
by rare diseases. The session brought in experts and 
interested parties to highlight the current state of both 
policy and legislative matters of concern to the rare dis-
ease community.

The final day of Rare Disease Week included a 
tour of the National Institutes of Health in Bethesda, 
Maryland, a world-class medical research facility in 
its own right, and a major grantor of funds for other 
non-governmental research projects.

I could come up with plenty of excuses to make my 
first involvement in Rare Disease Week my last. Just 
for starters, late February is about the dreariest time 
of year in the DC area. A PV flare that began in June is 
still not under control. As a confirmed night owl, I’m 
accustomed to sleeping late instead of getting to 8:00 
a.m. meetings (or worse, 7:00 a.m. breakfasts). I’ll 
miss a session of guitar playing with my jam group at 
Maloney’s Pub and possibly my monthly poker game. 
The traffic in DC is worse than ever.

Yet, when somebody from the IPPF asked me if I 
planned to participate in the 2020 Rare Disease Week 
on Capitol Hill, my immediate response was, “Of 
course I am.” I found last year’s sessions inspiring and 
invigorating, and I truly got more out of it than I ever 
thought I would.

So, in February I’m going to again uproot my com-
fortable life at the Jersey Shore and join the hundreds 
of patients, caregivers, and other advocates to push for 
legislative action that can improve the lives of millions. 
I’d encourage anyone with an interest to register for 
this year’s session.

Fred Wish is a PV patient who lives at the Jersey Shore. 
He retired from full-time work in 2010 and owns a 
writing and editorial service company with his wife, 
Loretta. He enjoys being a member of a classic rock 
band, plays a possibly competent game of poker, and 
looks forward to spoiling his grandson at every chance.
National Coalition of Autoimmune Patient Groups Successful Summit

Carolyn Fota

The American Autoimmune Related Diseases Association (AARDA) successfully conducted its strategic National Coalition of Autoimmune Patient Groups Summit this past fall. The summit brought together senior leaders from the autoimmune, health policy, and research communities to discuss patient advocacy at state and federal levels. AARDA is the only national nonprofit dedicated to bringing a national focus to autoimmunity, a major cause of serious chronic diseases. Approximately 50 million Americans (20 percent of the population) suffer from an autoimmune disease. AARDA raises awareness and funding for critical research aimed at solving the underlying immune issues that link autoimmune disorders. The 2019 summit meeting focused on building partnerships and involving patients and patient advocacy organizations in medical research.

During the panel discussion, Jeff Allen, President and CEO of Friends of Cancer Research (Friends), shared that the breakthrough for Friends was shifting their focus from awareness to “an engine driving cures.” Although awareness still remains an important value, their focus is on representing patient needs in terms of medical research, clinical trials, supporting medical grants, the Federal Drug Administration (FDA) approval process, and health policy. Both patients and patient advocacy groups bring critical perspective, and as a result, the FDA is now developing pathways to directly involve patients.

Step therapy reform is another active topic in the autoimmune community. In theory, step therapy appears to be sound; however, patients first need to fail a treatment protocol before being approved for the next step of treatment. This fail-first therapy results in delay of care, increases the risk of complications, and adds to the cost of care. Doctors know the most effective therapies and treatments for their patients, but are forced to follow a policy that requires an ineffective protocol. AARDA supports the position that a patient and their doctor should determine the best course of treatment, rather than healthcare policy.

I attended the summit as a representative of the IPPF and provided updates on the following topics:

• the 25th anniversary celebration and IPPF Patient Education Conference in Philadelphia, PA in October
• The Awareness Program’s focus on the Biopsy Saves Lives campaign
• Research and drug development partnerships
• The IPPF’s growing network of peer health coaches and local support group meetings
• IPPF Executive Director Marc Yale’s trip to an international bullous disease meeting in Shanghai, China
• IPPF Outreach Director Becky Strong’s visit to Capitol Hill advocating for patients, research, policy, and legislation
• My attendance at the National Institutes of Health (NIH) conference prior to the summit meeting

I left the summit meeting galvanized because it validates the IPPF’s focus on patient advocacy; research involvement through the Natural History Study, clinical trial support, and research grants; representing our community on Capitol Hill concerning legislation, health policy and funding; and collaborating with other patient advocacy organizations. I am proud of the fact that we are a compassionate organization focused on patients, research, and—one day—finding a cure.

Carolyn Fota, MHR, lives in Stafford, VA, with her husband, Frank and their three crazy cats. Carolyn enjoys walking, yoga, church, and writing.
In January 2020, biotechnology company argenx announced positive proof-of-concept data for efgartigimod in pemphigus vulgaris (PV). Twenty-three patients were evaluated for efficacy in an adaptive Phase 2 trial aiming to establish optimal treatment. Recently, IPPF Executive Director Marc Yale talked to CEO Tim Van Hauwermeiren about the trial.

Tim Van Hauwermeiren cofounded argenx in 2008 and has served as the Chief Executive Officer since April 2008. He has served as a member of their board of directors since July 2014. Mr. Van Hauwermeiren has more than 20 years of general management and business development experience across the life sciences and consumer goods sectors. He holds a Bachelor of Science and Master of Science in bioengineering from Ghent University (Belgium) and an Executive MBA from the Vlerick School of Management.

What sets argenx apart from other biotech companies?

Tim Van Hauwermeiren (TVH): argenx refers to the ancient story of the argonaut, which talks about the collective power of the team instead of individual heroes. In our company we collaborate as equals, both within and beyond our walls in a humble fashion. But let there be no misunderstanding—our ambition level is high: we want to achieve the unthinkable and revolutionize the way the world treats really bad diseases like PV.

What is the therapy that argenx will use for the clinical trial called, and how does it work?

TVH: Efgartigimod targets FcRn, a tiny structure in our bodies which enables our antibodies (of the IgG type) to persist for a long time. Most often this is highly beneficial to mobilize our immune system, but in cases where these antibodies cause disease, we don’t want them to persist. So by blocking FcRn, we hope to actively eliminate them from the body so they can no longer do harm.

Is argenx’s novel therapy for PV designed to be steroid-sparing, or will it eliminate the need for steroids altogether?

TVH: In our Phase 2 proof-of-concept trial, we demonstrated fast onset of action of efgartigimod as a monotherapy and demonstrated likely synergy with low dose of steroids. In addition to efficacy and safety, we hope to also evaluate steroid dosing in our registrational trial.

How is efgartigimod administered to patients, and how often would patients need to take it (daily, weekly, etc.)?

TVH: We studied a one-hour IV infusion product given on a weekly basis in our Phase 2 proof-of-concept trial in PV patients. We are also developing a subcutaneous injection which could be dosed less frequently.

When and why did argenx decide to work on a clinical trial for pemphigus?

TVH: We built our drug candidate, efgartigimod, on the basis of the seminal work of our collaborator, Professor Sally Ward at Texas A&M University. Efgartigimod has the unique ability to rapidly and substantially eliminate disease-causing antibodies from the body. Severe autoimmune diseases which are driven by disease causing antibodies—like PV—are potentially an ideal target.

You recently showed data from the argenx Phase 2 proof-of-concept clinical trial of efgartigimod in PV. Can you describe what you have learned so far?

TVH: Efgartigimod showed everything we were hoping to see in the trial. We saw fast onset of action where patients achieved disease control after one or two infusions, deep responses including complete clinical remission within 2-10 weeks, and a favorable tolerability profile consistent with our experience with the drug.
in other clinical studies. This is a unique drug profile and very encouraging for PV patients.

**Was this trial done in a single site?**
*TVH:* The study ran in a handful of clinical sites across Europe and Israel, conducted by some of the best pemphigus experts.

**Is argenx considering a trial in the US? If so, how many patients will you be enrolling?**
*TVH:* The US will definitely be part of the global study. We will disclose more trial details as soon as it starts in the second half of 2020. We look forward to working with the IPPF to share this information with PV patients.

**What impact does argenx feel this therapy will have on patients’ lives?**
*TVH:* Our goal is to develop a therapy that provides rapid control of the disease, fast skin healing together with strong steroid sparing, and a promising tolerability profile. If we achieve this goal, efgartigimod has the potential to change the way we treat pemphigus.

**How can the IPPF community help argenx in the drug development process?**
*TVH:* Marc, we appreciate your guidance and partnership to date. We want to continue this collaboration with IPPF by incorporating patients’ voices and treatment desires into the design of our registrational trial and by working together to share information with patients. In addition, we look forward to getting to know the PV community and bring additional awareness of the patient journey with this rare diagnosis.

argenx attended and participated in the 2019 IPPF Patient Education Conference. Can you share one important takeaway from the conference?
*TVH:* We met many PV patients and were inspired by their fighting spirit. This is a tight knit, collaborative community with a strong belief that tomorrow will bring more solutions. argenx wants to be part of that journey.

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**Marc Yale was diagnosed in 2007 with cicatricial pemphigoid. In 2008, he joined the IPPF as a Peer Health Coach and was promoted to Executive Director in 2016. Marc currently resides in Ventura, California, with his wife Beth and daughter Hannah.**
Rare Together in China: MY VISIT TO THE INTERNATIONAL BULLOUS SKIN DISEASE SYMPOSIUM

Marc Yale

On October 16, 2019, I boarded a 13-hour flight to Shanghai, China, to represent the IPPF at the 7th Shanghai International Bullous Skin Disease Symposium. The IPPF was invited to attend by Meng Pan, MD, PhD, and Chairman at Rui Jin Hospital, Shanghai Jiao Tong University School of Medicine.

Held every two years, the symposium has achieved great success and earned a reputation among doctors and physicians. This year, a number of well-known scholars from around the world in the field of bullous diseases were invited to present the latest research findings and share diagnosis and treatment progress. Additionally, I was invited to address Chinese patients directly at the first ever patient-doctor discussion at the symposium. Prior to the symposium, I was aware that there were doctors and patients in Shanghai that were interested in starting a patient support organization in China. I felt honored to represent the IPPF and hoped that I would have the chance to meet another patient with mucous membrane pemphigoid.

After a long flight, I arrived in Shanghai and was greeted at the airport by a very cheerful, young medical student named Yen-chi. From the outskirts of Shanghai, we headed to the bustling city of 24 million people to meet the other invited symposium guests for dinner. As we entered the city, I was amazed by all of the large, modern buildings decorated by beautiful lights. After some incredible food and hospitality, I excitedly headed back to my hotel to prepare to meet patients like me from halfway around the world.

When I arrived at the symposium, I was surprised to find approximately 100 patients, medical students, and doctors gathered for the patient forum in a large lecture hall. I had never worked with a translator before, so I had to remind myself not to rush through my presentation and to allow Yen-chi enough time to convey my message in Chinese to the attentive audience. As I shared my story of how long it took to be diagnosed after seeing six doctors, I was astonished to see many heads nodding in agreement. I spoke about the high dosage of corticosteroids I was on and the side effects I experienced as the patients in the hall whispered...
to each other. I recalled all of the treatments that my doctors prescribed and challenges that I faced in my everyday activities as I realized that everyone in the room knew exactly what I was talking about. At that moment, I understood that it doesn’t matter what part of the world someone lives in, all people with these diseases share common experiences. I told the attendees about the IPPF, the services we offer, the support we give, and how the hope we provide is crucial to all those affected by pemphigus and pemphigoid (P/P).

After my presentation, both a pemphigus and pemphigoid patient got up and spoke about their disease experience. Although Yen-chi was translating to English for me, I could see the familiar anguish on their faces as they described their journeys. As they spoke, I could also see the relief they were experiencing as they finally had the opportunity to share their stories with people who knew exactly how they were feeling. When I asked Yen-chi how many people they saw in the hospital at the bullous disease clinic each day, she told me they see about 200. My jaw dropped, as I couldn’t believe the large number of people who are served. She went on to tell me that many people travel great distances from villages far from Shanghai to see an expert. It reminded me of the patients I’ve spoken to in the United States who had to travel five hours to see an experienced physician. Again, I was reminded that as rare disease patients, we have so much in common. Our language and customs may be different, but together we are all rare.

Attending this important symposium taught me a great deal about the patient experience. I now understand that all patients with this disease can benefit from the support of a patient organization like the IPPF. I also realized that even though we have access challenges in the United States, we still have more therapies available. Most importantly, I learned that the IPPF can be a bridge to patients from around the world and help them communicate their stories to accelerate diagnosis and treatments.

Marc Yale was diagnosed in 2007 with cicatricial pemphigoid. In 2008, he joined the IPPF as a Peer Health Coach and was promoted to Executive Director in 2016. Marc currently resides in Ventura, California, with his wife Beth and daughter Hannah.
Support Group Spotlight: SOUTHERN CALIFORNIA

Mei Ling Moore

In 1994, when Janet Segall discovered that she was unable to find support or information on the internet about pemphigus, she started her own quest to find answers. When she started the Pemphigus Foundation that year, she also started an email discussion group which was used by patients who communicated daily. It was a great way to find other pemphigus and pemphigoid (P/P) patients and for patients to ask Janet questions directly. Before social media took off, this was the main way for patients to communicate online.

Several years after starting the Foundation, Janet organized the first Los Angeles support group meeting, held at the University of California, Los Angeles (UCLA). She brought people together in person who were experiencing the same diseases, and she felt it was important for patients to meet others with similar experiences to realize that they weren’t alone.

The meetings eventually became an annual event. Sonia Tramel and Lee Heins, both members of the IPPF Board of Directors at the time, took over organizing the Southern California meetings, since Janet was, and still is, based in Sacramento. Marc Yale also helped organize the first meetings, and he and I continue to organize them today.

We currently meet at the Santa Monica Library; however, we’d also like to establish meetings in Orange County and the San Fernando Valley in order to meet the needs of all P/P patients in the region. Typically, our meetings start with catered refreshments provided by our sponsors, followed by a presentation by a guest speaker. The group has had many leading disease experts as speakers, such as Dr. Vanessa Holland and Dr. Sergei Grando. Once in a while, IPPF peer health coaches hold a question and answer session as well. Topics have included blistering issues, nutrition, and oral care. Attendees are able to meet with the guest speaker after the meeting to privately ask questions. After the meeting, attendees often visit the Santa Monica Pier or Third Street Promenade.

I enjoy being involved with the organization of these support group meetings—it’s like a reunion for those of us who regularly attend. We also try to make new patients feel special by offering support, inspiration, and plenty of our own experiences. Patients who are in remission offer hope to everyone going through treatment, which is reassuring to those who are still taking medications and trying to get through each day. These meetings remind all attendees that we share similar experiences and that no one is alone.

Mei Ling Moore was diagnosed with PV in 2002. She has been a peer health coach with the IPPF since 2012. She also organizes the Southern California support group with Marc Yale. Mei Ling lives in Los Angeles.
IPPF 2019 Volunteers

Thank you to all the volunteers who have supported the IPPF’s mission of improving the quality of life for all those affected by pemphigus and pemphigoid.

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Nasser Al-Naief
Kyle Amber
Grant Anhalt
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Vanessa Baron
Rachel Barsha
Silvia Beam
Marieke Bolling
Tara Brandt
Mirella Bucci
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