

Quarterly

Journal of the International Pemphigus & Pemphigoid Foundation



FALL 2020

Facts about Bullous Pemphigoid

- 1 in 1000** adults aged **over 80 years old** lived with bullous pemphigoid in 2017
- 8 in 100,000** adults develop bullous pemphigoid each year and this number has **risen 1% every year** for 2 decades
- People with bullous pemphigoid are **3x more likely to die** in the first **2 years** after diagnosis.
- 4** need to better understand the causes and how to prevent this
- Bullous pemphigoid is **not rare in older people**

12 **2020**
IPPF Virtual Patient Education Conference Recap

10 **RARE**
ACROSS AMERICA
EVERY VOICE, IN EVERY DISTRICT, MATTERS



SECTIONS

	Foundation
3	Message from the Executive Director <i>Kevin Mead</i>
	Research & Treatments
4	Bullous Pemphigoid Research Update in the UK <i>Monica SM Persson</i>
6	What Is Cell Therapy? <i>Aimee Payne</i>
	Advocacy Update
10	Rare Across America Goes Virtual <i>Katelyn Laws</i>
11	Virtual AADA Legislative Conference <i>Sandra Ring</i>

FEATURES

2020 IPPF Virtual Patient Education Conference Recap <i>Janet Fairley and David Woodley</i>	12
My Call to Action at the Annual IPPF Patient Education Conference <i>Staci White</i>	14
Looking to the Future IPPF LEADERSHIP <i>Kevin Mead and Marc Yale</i>	18
Inspiring Hope NOEL'S STORY <i>Noel Mudibo</i>	20
Connecting Virtually at the South Florida Support Group <i>Daphna Smolka</i>	22



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CONTRIBUTING WRITERS

Janet Fairley
Katelyn Laws
Kevin Mead
Noel Mudibo
Aimee Payne
Monica SM Persson
Sandra Ring
Daphna Smolka
Staci White
David Woodley
Marc Yale

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INTERNATIONAL PEMPHIGUS & PEMPHIGOID FOUNDATION

915 Highland Pointe Dr, Suite 250, Roseville, CA 95678
Toll free: 855-4PEMPHIGUS tel: 916-922-1298
info@pemphigus.org | www.pemphigus.org

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Message from the Executive Director



Three months might not seem like much, but we've seen a lot of change in a short period of time. After a brief summer respite, many locations around the world are again in the grips of a resurgent pandemic and the reactions to it that dislocate families, education, and livelihoods. The fears for life and health, coupled with the restrictions that are either placed on us or that we place on ourselves, are taking an increasing toll on mental and physical health.

Things have also changed at the IPPF. Three months ago, I was in early discussions with the Board of Directors about succeeding Marc Yale as your executive director. Now, I am well into my second month as a staff member. In the last edition of the *Quarterly*, Marc wrote that registration for our 2020 Patient Conference was about to be announced—that conference is now in the record books.

And what a conference it was! There were over 600 registrants, and we received overwhelmingly positive feedback from patient attendees, medical professionals, presenters, and corporate partners. Led by Marc, the ability of the staff to build an effective platform was incredible. That platform not only provided the virtual space to present the sessions, but it also provided a way for attendees to interact. I'm aware that no platform, no matter how robust, will replace the coffee-line conversations that are a part of a real-life conference experience, but we got as close as possible! The generous support from our corporate sponsors meant that the conference was available at no charge to attendees, and this resulted in a great increase in attendance.

So where do we go from here with conferences and meetings? First, your input is crucial—tell us how we can support your needs. Your suggestions will be taken into account as we develop plans for 2021. One thing is clear, though: with the diverse global attendance, it will be difficult to return to an exclusively in-person format. There seems to be a growing consensus (not only within the IPPF, but also among other patient advocacy groups, associations, and societies) that a return to in-person events needs to be accompanied by a strategy to retain newly expanded digital offerings.

In my first two months, I have started my journey of learning about pemphigus and pemphigoid, the patient community, and the IPPF. I was fortunate to be able to meet with the IPPF founder, Janet Segall, and learn about what drove her to found this unique organization. I have been incredibly fortunate that Marc Yale is not leaving the Foundation, just taking on new responsibilities. Included in this issue, you will find a conversation between Marc and me where we discuss the transition and our thoughts about where we go next.

The Board, staff, and volunteers have made this transition easy so far. With your active input and support, we will continue to develop a patient-centered foundation that is truly an example of what can be accomplished to support and advocate on behalf of those with rare diseases.

Kevin Mead, IPPF Executive Director
kevin@pemphigus.org

Bullous Pemphigoid Research Update in the UK

Dr. Monica SM Persson on behalf of the full study team: Dr. Sonia Gran (principal investigator), Dr. Karen Harman, Dr. Yana Vinogradova, Professor Sinead M Langan, Professor Julia Hippisley-Cox, and Professor Kim Thomas

I do not need to start off by explaining the impact of pemphigus and pemphigoid (P/P), or why there needs to be more research into these devastating diseases. It seems sufficient to say that my colleagues and I agree: more research is needed. And that is exactly what we have been doing over the last year-and-a-half at the Centre of Evidence Based Dermatology at the University of Nottingham, UK. Working closely with experts from the University of Oxford and the London School of Hygiene, as well as patient partners from PEM Friends UK, we have embarked on a journey aiming to improve our understanding of P/P in the UK.

Faced with the inherent difficulty of studying a rare disease, we decided that the best approach would be to use electronic health records. When a person visits their general practitioner in the UK, the information about the consultation is recorded electronically. For a subset of practices in the UK encompassing approximately 17 million people, the anonymised version of that information is available for research purposes in the form of the Clinical Practice Research Datalink (CPRD) (Herrett et al., 2015, 827-836). Within the database, there is useful information, such as diagnoses, prescriptions, vaccinations, and basic personal information (e.g., age, gender). Such a rich resource is invaluable for examining rare diseases, but only if the diseases in question can be accurately identified from a person's health records.

We first set to investigate whether we could accurately identify people with bullous pemphigoid (BP) and pemphigus vulgaris (PV) from their general practice records (Persson et al., 2020). To do so, we compared the diagnosis recorded for each person in their general practice records to any blistering disease diagnosis they may have received while hospitalized. Of course, not everyone in the database with BP or PV had been hospitalized, but we were able to compare the diagnoses for 797 people with BP and 85 with PV. We found that for every 100 people with a record for BP,

approximately 93 had the disease according to hospital records. This was excellent news—we could use the CPRD to examine BP.

Unfortunately, we did not find the same results for patients with PV. For every 100 people with a record for PV, only 59 had PV according to hospital records. The other 40 had another blistering disease diagnosis (25 had a BP diagnosis). Here we identified an inherent flaw in using electronic health records—it seemed that, too often, not enough care was taken when entering the diagnosis into patient records. This was disappointing, but perhaps not surprising.

The diagnosis of BP or PV is made in dermatology departments and consequently fed back to the patient's general practitioner, where it is manually entered into the patient's electronic records. It seemed that the term pemphigus was used interchangeably with other terms, likely because of a lack of awareness about the differences between these similar-sounding diseases. The devastating bottom line was that we could not use electronic health records in the UK to conduct much-needed research into PV. So, we turned our focus to BP.

The next step was to understand how many people were diagnosed with BP for the first time each year, how many people were living with it, and what the consequence of a diagnosis was in terms of mortality (Persson et al., 2020). Between 1998 and 2017, we found that for every 100,000 people, approximately 7 to 8 were diagnosed with BP for the first time each year in England. This is the highest reported incidence in the world, and it had increased by approximately 1% each year. We found that BP increased dramatically at older ages, and that older men in particular were most likely to be affected. Our most recent estimate (in 2017) was that about 48 in every 100,000 adults were living with BP. Focusing on the older age groups, about 141 in every 100,000 adults aged over 60 were living with BP, while 375 per 100,000 adults aged over 80 were affected. For reference, the European definition of a

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People with bullous pemphigoid
are **3x more likely to die** in the first
2 years after diagnosis.

We need to better understand the
causes and how to prevent this

Bullous pemphigoid is
not rare in older people
particularly older men

rare disease is one affecting less than 50 per 100,000 people. As such, we argue that BP should not be considered rare in older age groups.

Finally, we wanted to examine what happened after a patient was diagnosed. We found that in the two years after being diagnosed, those with BP were approximately three times more likely to die than their peers. Even after this initial period, they remained at about a 50% higher risk of death. This is a startling number and should be followed by a caveat: Due to the type of information we have, we could not specifically attribute the increased risk of death to the disease alone. Our finding may be influenced by factors such as differences in who gets BP (perhaps BP affects those that are already at higher risk of dying because of other health conditions) and the effects of the treatment for BP (for example, oral prednisolone). We call for greater awareness of the disease, improved recording of blistering diseases in health records, and further research into the causes and prevention of excess deaths from the disease.

We took our research one step further and provided our information to researchers and clinicians that might help plan clinical trials of new treatments. Oral prednisolone has long been the mainstay of treatment for BP, but it is often regarded as a double-edged sword. We therefore sought to understand the extent

of the problem. We wanted to know how many patients were prescribed oral prednisolone, the dosage, and length of time. In order to replace oral prednisolone with safer alternatives, clinical trials of new treatments are necessary. We want to understand how many people might be eligible to take part in a clinical trial like this in the UK, as well as to provide information that could be used to design such a trial. Currently, our work is being reviewed for publication to the scientific community. Stay tuned for an update once the work is finalized. In the meantime, I will share that the extent of prednisolone prescribing for BP is worrisome, but it looks like we might have enough people in the UK to conduct clinical trials of new treatments.

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What Is Cell Therapy?



Aimee Payne, MD, PhD

An introduction to regenerative medicine

Capitalizing on its compelling rationale to use the body's own ability to heal itself, regenerative medicine has gained increasing public and commercial interest in recent years. "We're at the beginning of a paradigm change in medicine with the promise of being able to facilitate regeneration of parts of the human body, where cells and tissues can be engineered to grow healthy, functional organs to replace diseased ones; new genes can be introduced into the body to combat disease; and adult stem cells can generate replacements for cells that are lost to injury or disease. This is no longer the stuff of science fiction. This is the practical promise of modern applications of regenerative medicine," said former United States Food and Drug Administration (FDA) Commissioner Scott Gottlieb, MD, in a 2017 press release. "But this field is dynamic and complex. As such, it has presented unique challenges to researchers, health care providers, and the FDA as we seek to provide a clear pathway for those developing new therapies in this

promising field, while making sure that the FDA meets its obligation to ensure the safety and efficacy of the medical products that patients rely upon." (www.fda.gov/news-events/press-announcements/fda-announces-comprehensive-regenerative-medicine-policy-framework)

How does regenerative medicine impact the IPPF community?

Cell therapy is a rapidly growing sector of regenerative medicine that involves the transplantation of human cells to replace or repair damaged tissue and cells. Three different types of cell therapy have entered into clinical evaluation for the treatment of pemphigus, so it's worthwhile to review a brief history of cell therapy, what types of cell therapy have been approved by the FDA for clinical use to date, and the data supporting the use of cell therapy in pemphigus so far. (Apologies to the pemphigoid community—so far, no cell therapies have been clinically evaluated for pemphigoid.)

A brief history of cell therapy

The first cell therapy, blood transfusion, has been in clinical use for nearly two centuries. The earliest published record of blood transfusion in humans dates back to 1825, describing the work of British physician James Blundell, who rescued a woman with severe bleeding after childbirth using blood collected from her husband as the donor (Waller 1825, 273-177).

Another major category of cell therapy is stem cell or bone marrow transplant, pioneered by US physician and Nobel Laureate E. Donnall Thomas (Thomas, 1957, 491-496). There are two major types of stem cell transplant: autologous (using a patient's own stem cells) or allogeneic (using a donor's stem cells). During stem cell transplant, the patient's or a matched donor's blood or bone marrow is collected, and the stem cells are isolated and frozen down for storage. The patient then undergoes intensive chemotherapy and/or radiation to eradicate their blood cells (both diseased and normal), and the stem cells are subsequently infused, with the goal of restoring the body with healthy blood cells. Although such therapy has led to cures of otherwise deadly blood disorders, the overall procedure comes with potentially fatal complications, including liver and kidney damage from chemotherapy, infection, recurrence of the original disease if the chemotherapy and radiation was insufficient, and, in the case of allogeneic stem cell transplant, graft-versus-host disease (where the transplanted cells attack the body's own tissues.)

FDA-approved cell therapies

In addition to blood transfusion and stem cell transplant, several other cell therapies are approved for clinical use in the United States ([fda.gov/vaccines-blood-biologics/cellular-gene-therapy-products/approved-cellular-and-gene-therapy-products](https://www.fda.gov/vaccines-blood-biologics/cellular-gene-therapy-products/approved-cellular-and-gene-therapy-products)).

Allogeneic cell therapies are being grafted to restore damaged gums. Autologous cartilage cells can be collected, expanded, and reinjected to repair injured knees. Collagen-producing fibroblasts can be harvested from skin biopsy samples taken from behind the ear and injected along smile lines to improve unsightly wrinkles. Cell therapies are also being used to fight cancer, including a prostate cancer vaccine and chimeric antigen receptor (CAR) T cells for B cell leukemias and lymphomas. CAR T cells are manufactured by harvesting the patient's immune cells from the blood,

and a subset of cells known as T cells are genetically engineered to express an anti-CD19 CAR that programs the T cells to specifically kill CD19-expressing B cells. Three CAR T cell therapies are approved for clinical use today, supported by impressive clinical data indicating a 58-81% short-term rate of cancer remission and a 40-57% rate of long-term remission (Maude 2018, 439-448; Locke 2019, 31-42; Wang 2020, 1331-1342), including patients who are thought to be cured of their B cell leukemia or lymphoma. Risks of anti-CD19 CAR T cell therapy include immune suppression, since both healthy and cancerous B cells express CD19 and are eliminated. Life-threatening side effects are also possible, including cytokine release syndrome due to the immune activation and massive cell death that occurs in association with high tumor cell burden, as well as neurologic toxicities that may occur either in conjunction with cytokine release syndrome or independently, potentially due to expression of the CD19 target in brain tissue (Parker et al 2020, 126-142).

Cell therapy for pemphigus

Autologous stem cell transplant was first reported for the treatment of a refractory pemphigus foliaceus patient (Oyama 2004, 1097-1098). The patient's course was complicated by neutropenic fever, which can occur during the vulnerable period when the patient's immune system has been ablated by chemotherapy, but the stem cell transplant has not yet restored normal blood counts. Fortunately, no serious infections occurred, and the patient achieved complete remission. A minor relapse 10 months after transplant was treated with topical steroids, and the patient remained in complete remission off all systemic therapies at 15 months after transplant. A subsequent study described eleven pemphigus vulgaris patients pretreated with chemotherapy, followed by allogeneic stem cell transplant. No serious adverse events were reported, and all patients achieved complete remission of disease lasting an average of eight years (Vanikar 2012, 9-11). A third study (Wang 2017, 296-301) reported 12 pemphigus patients (vulgaris, foliaceus, and erythematosus subtypes) treated with autologous stem cell transplant. Seventy-five percent of patients maintained clinical remission at five years after transplant, and one patient remained in complete remission for over 11 years. Three patients developed severe but temporary liver damage, and one

patient developed transient infertility due to chemotherapy toxicity. Two patients developed a severe blood infection known as sepsis, one of whom subsequently died. Collectively, these studies indicate the promise of stem cell transplants to provide lasting remissions of pemphigus, although the risk of the overall transplant process, particularly the intensive chemotherapy regimen, is generally thought to be too toxic to warrant widespread use.

Subsequently, researchers have refined strategies for cell therapies based on mechanisms for how autoimmunity occurs. In many autoimmune diseases, including pemphigus, a specific type of immune cell known as a regulatory T cell is deficient. Regulatory T cells can suppress immune responses and are part of the immune system's natural defense against autoimmunity; thus, several groups have investigated the potential to expand and reinfuse autologous regulatory T cells to treat a variety of autoimmune diseases. In type 1 diabetes, regulatory T cells have been shown to be safe, and they can persist up to a year after reinfusion (Bluestone 2015, 315ra189). Clinical benefit was most apparent in patients treated with repetitive doses, with 2 of 12 treated patients no longer requiring daily insulin injections (Marek-Trzonkowska 2014, 23-30). Regulatory T cells entered clinical trials for pemphigus in October 2017 (NCT03239470), and the trial has enrolled four patients to date. The study remains active, but it is no longer recruiting as of July 2020. Study results have not yet been posted.

Inspired by the cures of B cell cancer induced by CAR T cells, our research group developed a novel cell therapy known as desmoglein 3 chimeric autoantibody receptor T cells, or DSG3-CAART. By replacing the anti-CD19 targeting domain of the CAR (which targets all CD19-expressing B cells for elimination, both healthy and cancerous) with the DSG3 protein that is attacked by autoantibodies produced by anti-DSG3 B cells, we created a precision cellular immunotherapy designed to eliminate the anti-DSG3 B cells that cause disease while sparing healthy B cells, thus potentially providing lasting remission or potential cure of mucosal pemphigus vulgaris without the risks of global immune suppression. We demonstrated the preclinical efficacy and safety of this approach in cell culture studies, mouse models of pemphigus vulgaris, and broad toxicology screens against human cells and proteins

(Ellebrecht 2016, 179-184; Lee 2020, in press), resulting in FDA clearance of the Investigational New Drug application for DSG3-CAART in late 2019. This cleared the way for the opening of a phase 1 trial to evaluate the safety, dosing, and preliminary efficacy of DSG3-CAART in patients with active anti-DSG3 mucosal-dominant pemphigus vulgaris (NCT04422912). The trial is sponsored by Cabaletta Bio and is currently recruiting at the University of Pennsylvania under investigators David Porter, MD; Robert Micheletti, MD; and Victoria Werth, MD; and at University of California-Davis under investigators Mehrdad Abedi, MD; and Emanuel Maverakis, MD, PhD.

In conclusion

By enhancing the body's ability to correct its own mistakes, the field of regenerative medicine, and more specifically, cell therapy, holds promise to restore normal immune function in patients suffering from pemphigus and other autoimmune diseases. We look forward to the results of the phase 1 trials to determine the safety and preliminary efficacy of these novel cell therapy approaches.

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Dr. Payne is a professor of dermatology at the University of Pennsylvania. She received her BS in biology from Stanford University and her MD and PhD from Washington University School of Medicine, followed by dermatology residency and fellowship training at the University of Pennsylvania. Her clinical practice specializes in the diagnosis and treatment of pemphigus and pemphigoid. Her laboratory research has investigated how autoimmunity occurs in order to develop precision medicine therapies for disease. At Penn, Dr. Payne also serves as Director of the Clinical Autoimmunity Center of Excellence, Core Director for the Skin Biology and Diseases Resource-based Center, Associate Director of the Medical Scientist (MD-PhD) Training Program, and faculty advisor for the Association of Women Student MD-PhDs. Outside of Penn, she serves on the Medical Advisory Council of the International Pemphigus & Pemphigoid Foundation, chairs the NIH/NIAMS Board of Scientific Counselors, and is co-founder and co-chair of the Scientific Advisory Board at Cabaletta Bio, Inc., a biotechnology company focused on targeted cellular immunotherapy of pemphigus and other B cell-mediated diseases. This article is a follow up to a talk presented at the IPPF's annual Patient Education Conference on October 4, 2020. (Conflict of interest statement: Dr. Payne is an inventor on cellular immunotherapy patents licensed by Novartis and Cabaletta Bio. She has received equity, payments, and research funding from Cabaletta Bio.)

Fund the Future: #GivingTuesday & Beyond



The IPPF has been an active participant in the international movement known as #GivingTuesday for some time, and we are pleased to join again this year and ask for your participation. Giving Tuesday, held on the first Tuesday after the US Thanksgiving (December 1 this year) is a day that brings nonprofits, donors, businesses, and communities together to celebrate generosity and the impact of giving back.

This year, our participation will add to our resources and enable us to fund our important work in supporting the pemphigus and pemphigoid patient community. We are excited about participating again as a kickoff to our final fundraising push of 2020. With your help, we are confident that we can meet our goal of raising \$40,000 between Giving Tuesday and the end of 2020.

To help us achieve our goal, we've received a generous matching gift from the Unger Family Foundation. This match will double all new or increased donations through the end of the year. That means your donation can have twice the impact!

Your support is critical to our success, and donating is only one way to get involved. For the month of December and beyond, share why you support the IPPF on social media. Use the hashtag #healourskin and encourage your friends and family to support the IPPF, too.

If you haven't already, connect with us on Facebook, Instagram, or Twitter: [@healourskin](#)

RARE ACROSS AMERICA

EVERY VOICE, IN EVERY DISTRICT, MATTERS

RARE ACROSS AMERICA GOES VIRTUAL

Katelyn Laws

Each year, Rare Disease Legislative Advocates (RDLA), a program of the EveryLife Foundation for Rare Diseases, holds Rare Across America during the month of August. Rare Across America gives rare disease advocates the opportunity to meet with their members of Congress and staffers at their local district and state offices to discuss important rare disease issues and policies. This year, due to COVID-19, rare disease advocates met with their senators and representatives virtually. More than 540 rare disease advocates participated in 286 meetings with their member of Congress or their staff in 48 states, plus the District of Columbia and Puerto Rico.

During the virtual meetings, advocates shared their stories and rare disease experiences, and discussed different important pieces of legislation. Advocates asked their members to join the Rare Disease Congressional Caucus, which ensures rare disease patients have an impactful voice on Capitol Hill. The Rare Across America meetings resulted in 13 new caucus members, bringing the total to 184 members. If you couldn't participate but would still like to ask your member to join, you can do so at www.rareadvocates.org/take-action.

Many advocates also spoke about the Creating Hope Reauthorization Act (HR4439 and S4010), which would reauthorize the Pediatric Priority Review Voucher program at the Federal Drug Administration to spur innovation in rare and neglected diseases that disproportionately impact children. Due to the advocates'

hard work, 17 new cosponsors signed onto HR4439, which passed in September. A short-term reauthorization for the program was ultimately included in the continuing resolution passed on September 30, but Senate action on S4010 is urgently needed before the December 11 deadline. You can ask your Senators to act here: <https://rareadvocates.org/take-action>

Rare Across America participants from across the country spoke to their legislators on the phone or over video conferencing to make a personal connection and to make positive change for the rare disease community. Many advocated for other bills, such as the Medical Nutrition Equity Act, Lymphedema Treatment, Ensuring Lasting Smiles Act, and the Helping Experts Accelerate Rare Treatments Act. Through advocacy, sharing stories, and asking legislators to support these causes, participants helped build champions and move the needle even further!

All rare disease patients, caregivers, and community members are welcome to join virtually from home for Rare Across America in 2021. Rare Across America will take place from February 22 to March 5, 2021. Registration for Rare Across America opened on November 16, 2020. You can find more information at RareAcrossAmerica.org. Follow RDLA on Facebook and Twitter for updates @RareAdvocates.

Katelyn Laws is the RDLA Program Coordinator at the EveryLife Foundation for Rare Diseases.



Virtual AADA Legislative Conference

Sandra Ring

The American Academy of Dermatology Association (AADA) held its first ever virtual Legislative Conference from September 10-15, 2020. The conference broke attendance records, with more than 330 participants from 44 states and Washington, DC. The Coalition of Skin Diseases (CSD) joined the conference by recruiting 48 patient advocates to participate in state groups with AADA members as well. On the last day of the virtual conference, participants met with almost 270 members of Congress and their staff to discuss Medicare reform, medical liability protections, telemedicine, and patient access to care.

Conference attendees included physicians, residents, select medical students, and patient advocates. Among those attending were:

- 260 Physicians, including 132 first-time attendees and 59 residents
- 48 Attendees from the CSD, including dermatology nurses
- 21 medical students invited by AADA physician members

Participants learned about this year's legislative priorities during an overview webinar hosted by Bruce Brod, MD, MHCI, FAAD, chair of the Government Affairs and Health Policy Council, and Kelley Pagliai Redbord, MD, FAAD, chair of the Congressional Policy Committee. The webinar also featured information on the SkinSerious campaign from Seemal Desai, MD, FAAD, board member and chair of the Specialty

Positioning Work Group, as well as best practices for virtual meetings with members of Congress from Christopher Kush, CEO of Soapbox Consulting.

Participants met with 183 House of Representatives offices and 86 Senate offices. Of the 269 congressional meetings, 60 were member-level meetings that included either the senator or representative and 102 meetings included senior-level congressional staff.

Meetings focused on supporting dermatologists and dermatology patients during and after the COVID-19 public health emergency (PHE). Participants asked their legislators to delay pending Medicare cuts by waiving budget neutrality before the end of the year and to support medical liability protections for physicians working during the PHE. We have been very encouraged by the feedback received from members regarding congressional interest in the various issues our members raised.

Attendees were encouraged to share their advocacy experiences and connect with legislators via social media using the hashtag #AADAOnTheHill. The hashtag was used 56 times on Twitter and generated over 286,000 impressions. The @AADmember Instagram account posted a total of 41 stories (a combination of "ambassador" Dr. Sara Moghaddam's testimonial videos, registration/attendee graphics, and shared stories from members), which resulted in over 32,000 views.

Sandra Ring is the Senior Manager, Constituent Relations and Strategic Planning, at the AADA.

2020

IPPF Virtual Patient Education Conference Recap

// CREATING A BRIGHTER FUTURE TOGETHER //

Janet Fairley, MD, FAAD, and David Woodley, MD

Hosting this year's IPPF Virtual Patient Education Conference was a different experience from the usual in-person event. Prior to the start of the conference, we had concerns about technical errors with our laptops and coordinating our audio and visuals from two different sites, so we decided to co-moderate from the same location. This ended up being a good decision, since Janet's computer couldn't run the IPPF's platform due to the University firewall! Thank you and congratulations to Becky Strong, IPPF Outreach Director, and Amethyst Yale, IPPF Outreach Assistant, who smoothly ran the technical aspects of the three-day virtual conference.

Before the start of the conference, we were introduced to the IPPF's new executive director, Kevin Mead. Marc Yale has done an outstanding job in the role for many years—under his leadership the IPPF has grown substantially and has brought increasing visibility and usefulness to patients with pemphigus and pemphigoid (P/P). We are delighted that Marc is not leaving the IPPF. Instead, he is starting a new role that focuses on research and advocacy. We would like to welcome Kevin to the IPPF and to his new role.

On the first evening of the virtual conference, there was a panel discussion on the burden of living with

pemphigus that featured both patients and disease experts. This included the founder of the IPPF, Janet Segall, who now continues to work as a peer health coach. Jennifer Mangone was also on the panel. She is the Manager of Congressional Policy for the American Academy of Dermatology and was able to provide broad political context. When panelists discussed the burden of their diseases and the hurdles in the way of receiving optimum and affordable care (such as lack of health insurance), Jennifer was able to discuss potential solutions at the local, state, and national levels in the US.

As physicians, it is always eye-opening to hear about the effects of blistering diseases on the lives of our patients. In the course of their medical visits, patients are more likely to share how they are tolerating their treatments, or the level of their current symptoms, but they do not always share the details of how hard these diseases can make their lives. The number of people who mentioned the loss of a job or inability to find a job due to their disease was startling. It seemed like it was an exception if a patient had an understanding employer. There is more work to be done, not only to improve treatments for P/P, but also to continue educating the public about these disorders. This is why the IPPF is so valuable.

Another highlight from the conference include a presentation by Dr. Ronald Feldman from the Department of Dermatology at Emory University on the use of immunosuppressive agents for patients during the COVID-19 pandemic. He mentioned that, so far, it appears that the use of such medications in this patient population has not been associated with an advanced spread of the disease or untoward outcomes. This is a question on the minds of both P/P patients and physicians amid the pandemic.

A new strategy developed in Europe for quickly diminishing the autoantibodies circulating in the blood of pemphigus patients called “immunoabsorption” was discussed. The presentation was given by one of the pioneers of this treatment in Europe, Dr. Michael Kasperkiewicz. In brief, the patient’s blood is filtered through a machine that separates the blood cells from the plasma, where the autoantibodies reside. The plasma is then filtered through a column that pulls out the IgG antibodies from the patient’s plasma. Then, the plasma (minus the IgG antibodies) and the cells are given back to the patient. The advantage to immunoabsorption is that it causes a rapid decline in the disease-causing antibodies while leaving more beneficial parts of the plasma. Many treatments of P/P can take several months to be fully effective, such as rituximab. Immunoabsorption may provide a bridge treatment during that time to give rapid improvement without additional immunosuppression. Dr. Kasperkiewicz said a European company is interested in bringing immunoabsorption technology to the US and is currently working toward this end with the Food and Drug Administration. Dr. Kasperkiewicz will be involved in the initial studies using immunoabsorption in the US.

Jennifer Harmon and Dr. Donna Culton, both from the University of North Carolina in Chapel Hill, presented on how to address oral mucosa blisters and erosions in P/P patients. Their presentation was filled with many great practical tips for both patients and physicians. For example, they recommended that patients with oral lesions avoid using products in their mouths that contain detergents, such as sodium laurel sulfate (SLS), which is in many products. They gave examples of specific products that do not contain SLS, such as Biotene, Sensodyne, Kiss My Face, Jason’s Toothpaste, and Toms of Maine Whole Care. (Note:

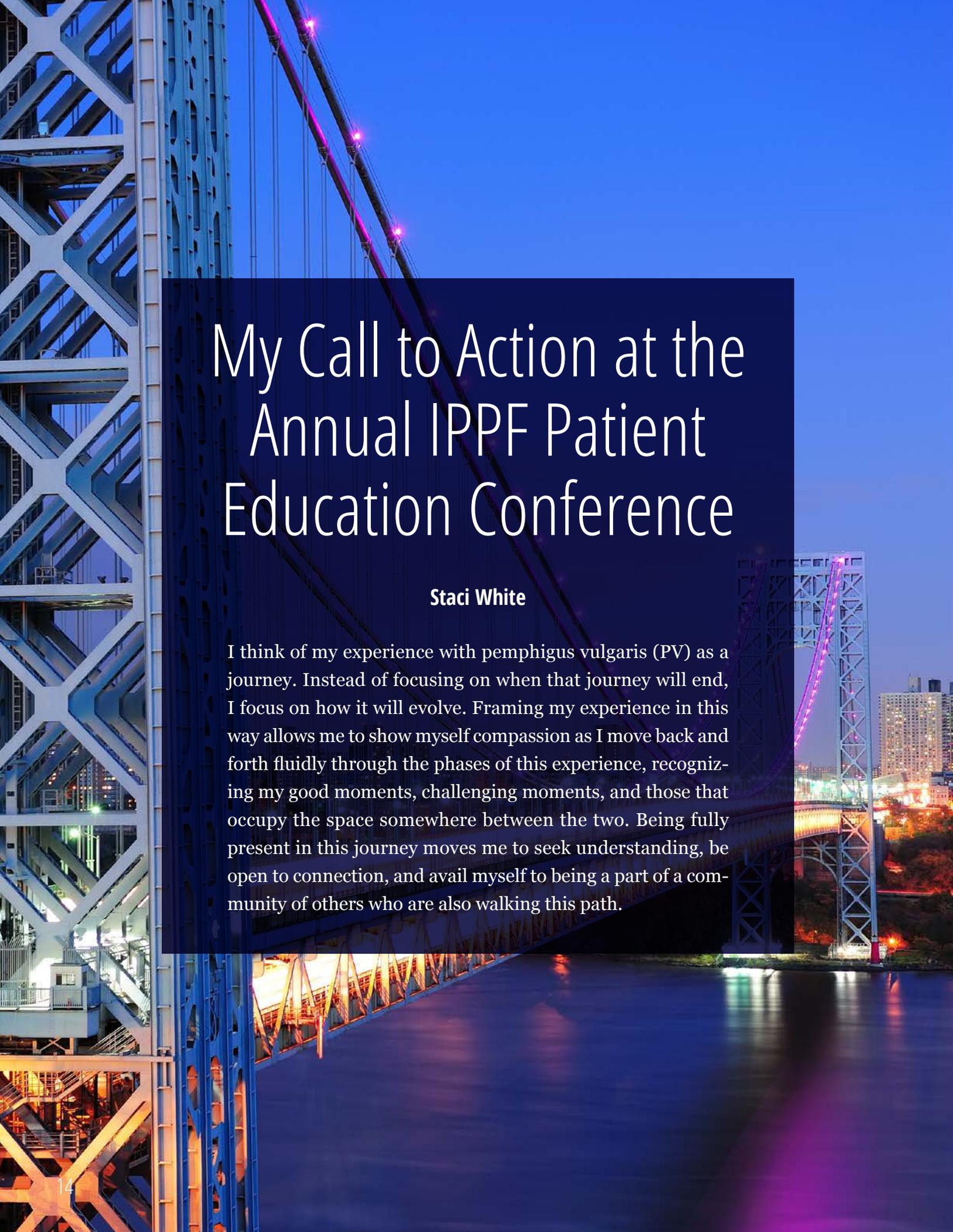
always check product labels to determine ingredients.) They also suggested avoiding mouth washes that contain alcohol and recommended Listerine Cool Mint and Listerine Sensitivity. A tip they provided for cleaning teeth was to use a water flosser device on the lowest setting. As physicians know, these are the sort of practical tips that can be extremely helpful for our patients.

The conference included three days of excellent presentations from patients, advocates, and health care providers. Another unique aspect of the virtual conference was the various opportunities for patient participation, such as attendee questions and polls that were conducted between presentations. The poll responses were often surprising and very interesting. The results highlighted the broad participation from across the world for this first virtual conference, as well as the large number of first-time attendees. A silver lining of being forced to meet virtually is that people who might not be able to take time away to travel to the event could participate from home.

Although the virtual conference was a resounding success, we missed the opportunity to get to know (or renew our acquaintance) with attendees in person. The ability to meet P/P patients and hear their stories at the annual conference is always motivating for those of us who treat and conduct research on autoimmune blistering diseases. Plus, who can forget the party with the Elton John impersonator? We are looking forward to the next time we can be together in person, as well as retaining the virtual components for those who cannot make the trip to our next destination.

Dr. Janet Fairley is Professor of Dermatology and Chair at the University of Iowa Carver College of Medicine. Her current research is to better understand the pathogenesis of the organ-specific autoimmune blistering diseases, such as bullous pemphigoid. Dr. Fairley is currently the president of the Society for Investigative Dermatology as well as a member of the IPPF Medical Advisory Council.

Dr. David Woodley is Professor of Dermatology at the University of Southern California Keck School of Medicine. Dr. Woodley is the author of over 250 original articles and is a clinician-investigator with continuous National Institutes of Health funding since 1982.



My Call to Action at the Annual IPPF Patient Education Conference

Staci White

I think of my experience with pemphigus vulgaris (PV) as a journey. Instead of focusing on when that journey will end, I focus on how it will evolve. Framing my experience in this way allows me to show myself compassion as I move back and forth fluidly through the phases of this experience, recognizing my good moments, challenging moments, and those that occupy the space somewhere between the two. Being fully present in this journey moves me to seek understanding, be open to connection, and avail myself to being a part of a community of others who are also walking this path.

The annual IPPF Patient Education Conference meets patients where they are on their journeys. The conference, whether in-person or virtual, is a thoughtfully curated space for patients. It welcomes us with compassion and builds a sense of community by grounding us in a story that is woven together by our diverse, yet shared, experiences on our walk through pemphigus or pemphigoid (P/P). The conference empowers us with education facilitated by clinicians who present in a way that shows respect for our humanity. It inspires pathways for action that give us permission to nurture ourselves, discover our voice in the face of our health journeys, and advocate for this community.

I have attended three conferences so far. Each time, I leave with a personal call to action. My first was in 2015 in New York City. I was at the beginning of my journey, and uncertainty and fear abounded within me. I was nervous when I walked into the conference space, but that feeling quickly faded. I met patients from across the country, and I learned about the clinical underpinning of my disease. Prior to the conference, I knew the basic science of PV, but I was too busy trying to manage my day-to-day health to focus on it. During the conference, I listened as patients courageously shared their stories. In them, I saw myself. I also was able to participate in a study through blood donation. I was strengthened by having an avenue to do my part to help find a cure and improve treatment. That conference gave me a moment to feel at ease in the company of what was now my community.

Four years later in 2019, I attended my next Patient Education Conference in Philadelphia, PA. Philadelphia is a place with deep roots for me, as it's where I was born. The long gap was purely a reflection of where I was on my journey. I needed time to focus on my health and find the new me. During the conference, I acknowledged that it was OK to move through my journey in a way that made sense to me. This acknowledgment gave me the courage to share my story when I felt ready. It also laid the foundation for me to become more engaged with the IPPF. Most importantly, I felt hopeful.

I attended the 2019 conference with my Aunt Cheryl, someone who has been a part of my health journey from the beginning. It was her first time attending the annual conference. By that time, I had been in remission for three years. I had also written my first article

in the Quarterly a year before. It was the first article I had ever written and published! I was in a different phase of my journey than I was in 2015, but the impact of the conference was no less powerful. This time, I didn't feel nervous—I was home. I experienced a quiet comfort when I was able to identify elements of my own journey as Becky Strong, IPPF Outreach Director, shared her story. I attended a workshop on mindfulness and managing stress with Mei Ling Moore, IPPF Peer Health Coach. I had interacted with Mei Ling on social media, but experiencing her authenticity and compassion in person was grounding. I also attended a session presented by Dr. Aimee Payne, where she shared information on current studies and related treatments. I listened carefully and was in awe of how artfully she discussed the underlying science of pemphigus. My Aunt Cheryl also commented on how much she learned from Dr. Payne. We also both participated in a blood donation study again.

I moved through that conference with a hunger to learn, a joy to connect with those I knew, and an effort to create new connections with members of the P/P community. I left with a commitment to elevate my engagement with the IPPF. I joined the Healing Hero recurring donor program (www.pemphigus.org/hero). I also decided to apply to join the IPPF Board of Directors, thanks in part to a gentle nudge from my aunt.

This year, I had looked forward to the 2020 Patient Education Conference in Las Vegas, NV. As it had done to most other events of 2020, COVID-19 caused the conference to become a virtual event. However, in moving to a virtual setting, the real-time reach of the conference was widened. Patients and attendees from Africa, Europe, Asia, Australia, the US, and more were able to attend together. It was extraordinary to be part of the IPPF global community amid a pandemic. In the face of an unthinkable worldwide health crisis, the conference created an opportunity for patients to connect, regardless of where they were on their journey. Our community remained strong, and because of the solid foundation that was intentionally built over the years, the conference was well-positioned to shift to an online event.

At this year's conference, I was humbled by the opportunity to be part of a patient forum with patients who are also leaders within the IPPF, such as Janet



me in the patient forum because as a Black woman, it made her feel seen. It was humbling. This was her first patient conference, and 2020 has been steeped in a bolder conversation about racial justice. I have spent the past several months seeking avenues to find a voice in the current discourse. This year's conference was an unexpected channel for that voice that simply involved telling my story. It was a great lesson about the impact that sharing my story can have on another person as well as myself.

On the final day of the virtual conference, I attended the clinical trial sessions with a notebook in-hand, ready to be a student. I learned more about T cells and B cells. I also learned about the clinical pathways for treatments that were not available at the start of my PV journey. The physicians presented the information in a way that not only deepened my knowledge, but also made me curious to learn more. By the end of the presentations, this year's personal call to action became evident. I've decided that I will spend

time becoming more knowledgeable about the clinical aspects of PV. Being able to talk about the clinical side of PV will be a tool that will allow me to feel more empowered in my advocacy work.

If you have not attended a patient conference, I encourage you to consider it in the future. You will hear stories that are similar to your own. You will acquire empowering knowledge. You will be wrapped in compassion and love. Regardless of where you are in your journey, the patient conference will provide a sense of comfort and a community that will be there for you throughout your own journey with P/P.

Segall, the Foundation's founder; Rudy Soto, a peer health coach; and Marc Yale, the former executive director and current advocacy and research coordinator. We shared our stories while answering questions about finding our voices as patient advocates and the road to remission. I was especially inspired as I listened to Janet and Rudy's stories. At one point, I sat back to take in the moment. In 2015, I could not have imagined that I would sit on a panel to share my perspective on PV. I was proud, humbled, and in utter disbelief that this experience was now a part of my journey.

After the patient forum, I joined the Principia Biopharma (a Sanofi Company) virtual lounge, where there was a lively discussion about the process for moving new treatments forward. During the discussion, a question was asked about diversity throughout the process. I was struck by the candor of the response from Principia's clinical representative. She spoke eloquently about the complicated history of the healthcare experience of African Americans, specifically in clinical trials. She spoke with knowledge and sensitivity, while focusing on owning the reality of the past as part of moving forward. The discussion came on the heels of a patient sharing with me that she appreciated seeing

Staci White is the Operations Manager for the Center for Genetic and Genomic Medicine and the Institute for Child Development at Hackensack Meridian Health. Staci became a member of the IPPF Board of Directors in 2020. She currently resides in Hackensack, New Jersey. Staci was diagnosed with pemphigus vulgaris in 2011 and celebrated four years in remission in July 2020.

2020 Virtual Patient Education Conference

// CREATING A BRIGHTER FUTURE TOGETHER //

629

registered attendees

from

25

different countries



Thank you to all of the patients and caregivers who joined us from all over the world. Though we were only able to connect online this year, **the strength of our community has never been so strong.**

Looking to the Future

IPPF LEADERSHIP

Kevin Mead and Marc Yale

Over the past several months, the IPPF performed an intensive search for a new executive director to lead our community going forward. We were fortunate to find an experienced individual who is equipped to meet our unique challenges. The IPPF is in good hands with Kevin Mead, who has taken over as the Executive Director of the IPPF. Former Executive Director Marc Yale has transitioned to a new role as the IPPF Research and Advocacy Coordinator. Kevin and Marc connected virtually to interview each other about the future of the IPPF.

Part 1

Kevin Mead (KM): Can you walk us through the reasons for the leadership change and what you will be doing next?

Marc Yale (MY): I have struggled with a pemphigoid relapse that started in April 2018. I had to step away from the executive director role in order to focus on my health, but I didn't want to leave entirely as I have such a strong connection with the Foundation and its work. The IPPF Board of Directors wants to expand our work in both advocacy and research, and I thought that taking this on as a volunteer while also retaining my peer health coach responsibilities was critical to our future.

KM: So where does advocacy go from here? Both in your work and in mobilizing the patient community?

MY: Well, that's it! We need to mobilize our patient community. Developing our Awareness Ambassador program and making sure that patient voices are heard so that we can influence the political and regulatory processes is a critical part of building awareness.

KM: Moving to research, what excites you about current and future research that would assist the patient population?

MY: We have seen an expansion of research, which is exciting. This is because we have the biomarkers that enable researchers to know which antibodies are present and to develop therapies based on this knowledge. Medicine is expanding rapidly, but it is really important that patients are involved. Through initiatives like the IPPF Natural History Study, we can provide a route for effective patient involvement. In the future, we should be able to use technology to give patients the ability to track their disease journeys through apps, and then use this knowledge to enable research through engaged patients.

KM: What will you miss the most as you transition to your new role?

MY: Since I'll still be involved in the areas I'm most passionate about, there won't be much to miss! I will be building the research and advocacy initiatives and staying involved with the patient population and other stakeholders. However, one area that has changed are my administrative duties. For example, last month we lost the lease on our office space, and now that's your problem, Kevin!

KM: There have been great advances made by the Foundation recently. What do you consider as the IPPF's biggest accomplishments over the last five years?

MY: I think the five biggest accomplishments from the past five years are:

1. The launch of the new IPPF website
2. The expansion of the Peer Health Coach program

3. The 2020 Virtual Patient Education Conference
4. Our continued efforts to send patient advocates to Washington, DC, to advocate on behalf of all rare disease patients
5. Working with researchers to develop therapies, expand scientific conferences, and build a relationship with the US Food and Drug Administration

Part 2

Marc Yale (MY): Kevin, we are excited to have you on board. What initially made you think about joining the Foundation?

Kevin Mead (KM): When I first saw the job advertised, like other people, I had not heard of pemphigus and pemphigoid. As I did some research, I discovered that some of the therapeutic treatments were similar to ones that I had received for a different autoimmune condition. Having established that connection, and after talking with you and the Board of Directors, I was convinced that this was an organization making a difference in the lives of patients, and that becoming a part of it would be both challenging and inspiring.

MY: How do you see the Foundation growing in the future?

KM: You mentioned our new website. One issue that we face is increasing both the number and quality of visits to this important resource. This is indicative of a number of things that we must do. We need to be the first place patients go for peer support, both before and after being diagnosed. As we expand information and services, we must always remember to put patients at the center of everything we do.

A further area of growth for us will be expanding internationally. As access to medical technology increases across the world, more P/P patients will need access to information and support. We have already received a grant to assist in the establishment of a patient support group in Kenya, and I see this as an example of future expansion.

MY: These are strange times, and all organizations are facing challenges. What do you think are the biggest challenges facing the IPPF right now?

KM: First, let's talk about the things that are not a problem: enthusiasm, the demand for information, a vocal and active patient group interested in new therapeutics and clinical trials, and the need for patients who feel isolated with their disease to connect with

peers. All of these things are strengths for us. They are the building blocks of the Foundation's future.

Now, one of the things that is problematic is funding. We need to expand our sources of funding and, bluntly, convince the patient population of the impact they can have through the IPPF on peer patients, governments, the pharmaceutical industry, and the medical community. That impact then needs to be translated into successful grant applications and fundraising appeals.

A further challenge is to make the diseases and their effects better known outside of our stakeholder communities.

MY: After a successful virtual patient conference in October, what is the future for IPPF meetings and conferences?

KM: Going virtual with the Patient Education Conference in 2020 was a great success. Nearly two-thirds of attendees were first-time participants. The message there is that we have to expand and enhance our education and communication efforts using technology, virtual events, and additional opportunities for information sharing.

We are looking at the number and timing of events, the platforms that we use to support enhanced experiences, and the ability to host hybrid events with both in-person and virtual components.

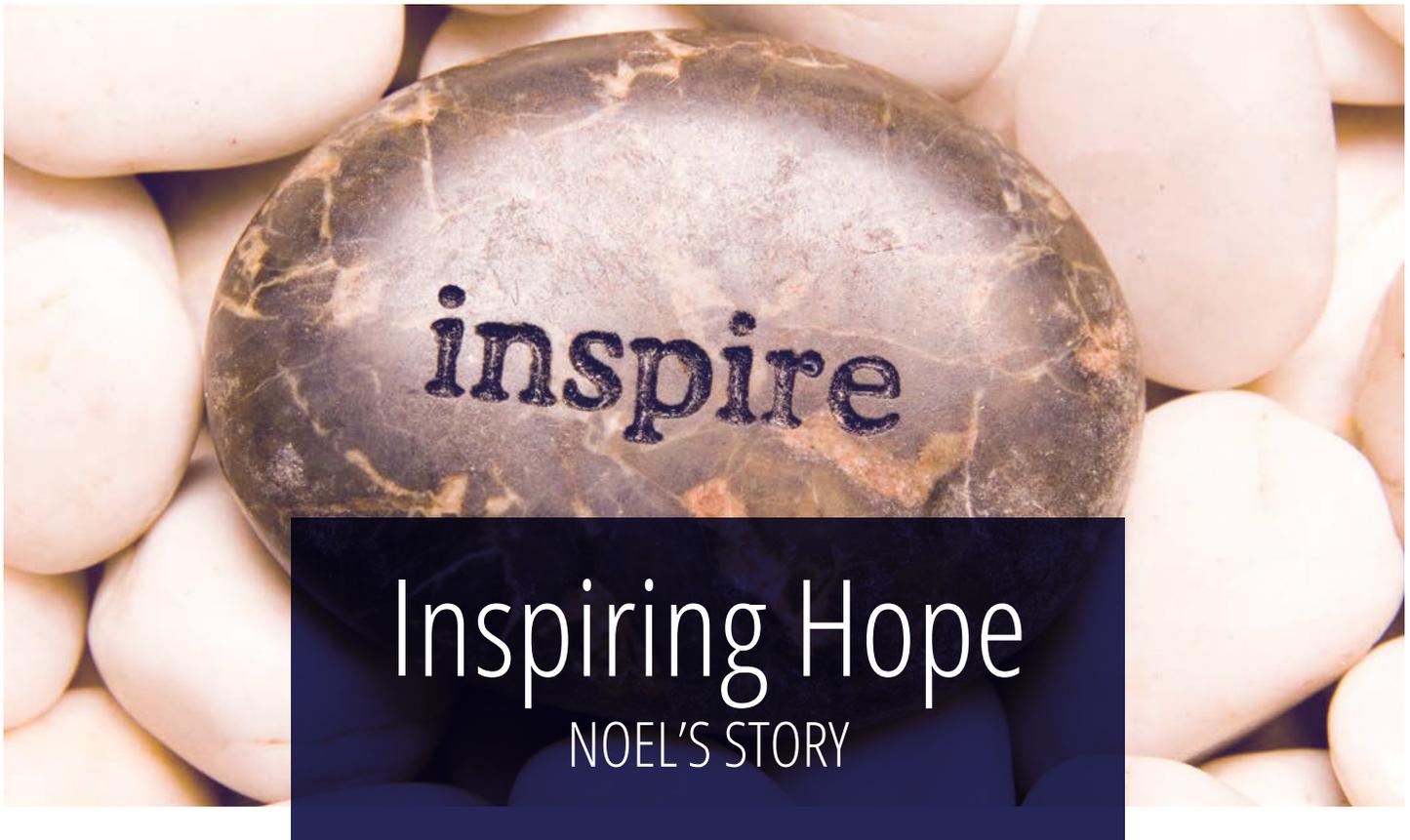
MY: What surprised you the most during your first few weeks?

KM: The commitment and enthusiasm of the Board of Directors, volunteers, and staff. You expect competence, but when you get people willing to sacrifice their evenings and weekends in order to enhance the effectiveness of the Foundation, you realize that this is a very special organization.

MY: What should readers know about the future of the Foundation?

KM: Let's not minimize the challenges we face. Demand for services and information is increasing, and many of the individuals and organizations that support us face uncertain times, at least partly due to COVID-19.

Despite this, the difference that the IPPF makes in the lives of patients every single day is so important that the board, staff, volunteers, and the entire patient universe need to commit to stability and expansion in order for the work to continue, so that we can build a brighter future together.



Noel Mudibo

Since childhood, I have had several health challenges. I had intestinal surgery at the age of two. At the same time, doctors discovered that I had an eye problem. Immediate action was not taken due to my parents' financial constraints, which caused me to pay a heavy price. Years later in 2010, an ophthalmologist discovered that I had dual keratoconus, and they performed corneal transplants on my left eye in 2012 and my right eye in 2013. In 2014, I embarked on a transformative academic journey.

The turning point of my life happened in January of 2016. I developed lesions all over my body, including painful lesions on my tongue and groin, and blisters appeared in my mouth. I also suffered from severe constipation and bloody stools, and I needed to take strong painkillers. I experienced fevers, body weakness, and fatigue. Whenever I swallowed certain food, I felt a lot of pain. My skin became pale, and I developed brownish spots all over.

At the time, I was still in college. People thought I had HIV, but my friends took me for testing, and I was negative. I was still in a lot of pain and surviving on pain medication. I went to a clinical officer who

examined me and told me I was suffering from an autoimmune condition called pemphigus vulgaris (PV). I was prescribed prednisone, but it did not make much of a difference as I continued to get lesions. At one point, my flare up was so bad that I had to be admitted to the hospital for two days.

In 2018, I saved 40 dollars and went to see a skin specialist who also told me that I had PV and that I needed an urgent treatment. He prescribed a combination of steroids. I felt better when I started the medications, but not completely. I still had frequent, severe flare ups. These flare ups would keep me grounded for several days and out of class.

I started researching pemphigus conditions online. I read about the research that had been done on treatments, including testimonies of people who have had pemphigus. Some patients died, some went into remission, and some of their conditions were still very active. During this research, I came across the IPPF. I also started searching for other people in Kenya who were suffering from PV, and I found quite a few. Some of them had very serious conditions that they succumbed to. I was very sad about that.

I continued my efforts to connect with the IPPF, and I started reading PV articles and testimonies with positive stories about people who had gone into remission and whose lives were almost back to normal. This information encouraged me a lot. I started communicating with the IPPF Outreach Director, Becky Strong—herself a PV patient in remission.

The articles on the IPPF website became my daily religion. I started reading them routinely and sharing them with other PV patients in Kenya. Some patients are not educated, so I have had to interpret the information for them. There are also some people in Kenya who believe PV is due to witchcraft.

Even though medication is quite expensive here, and I sometimes go months without getting my required steroids, I live a positive life full of hope and faith. I hope that one day, this condition will go into remission like Becky Strong.

During the COVID- 19 pandemic, I have had challenges, but knowing that my immune

system is low, I adhere to directives to stay indoors. In addition, I have recently filled out surveys provided by the IPPF. My most important request is for the IPPF to open an African office in Kenya. So many people here are suffering from PV, but they don't have proper information.

.....
Noel Mudibo is a passionate volunteer social worker from Kenya with 11 years of experience. He has a BS in Financial Economics, Diploma in Petroleum Geoscience and Accounting Level 2. Additionally, he is a fiction writer currently writing about his childhood friend that was killed by the police in a Nairobi ghetto. He recently finished writing a compilation of traditional African children stories.



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Connecting Virtually at the South Florida Support Group

Daphna Smolka

I will always remember the feeling when I was first diagnosed. The confusion and disbelief were soon replaced by feeling alone and overwhelmed. Luckily, I discovered the IPPF and its website, which provides access to so much information. I have been sifting through that information ever since I was diagnosed. Attending the annual Patient Education Conferences (seven so far) and being active in my local support group meetings have helped me change my perspective about this disease.

When first diagnosed, I accepted that I had a rare disease and spent my time researching and learning, but in a vacuum. Fortunately, Nancy Corinella felt motivated to start the South Florida Support Group in 2014. The first meeting was in her home, and three of us showed up.

Since then, the group has grown and welcomed numerous patients and caregivers to our meetings. I am always amazed at the number of newly diagnosed patients who find us. The opportunity to meet with other patients dealing with similar challenges is such a relief. More than once, I have felt a burden lifting when another patient can share their experiences with me.

Sadly, our March 2020 meeting was canceled at the last minute due to COVID-19. I was disappointed that

I wouldn't have the chance to catch up with the regular attendees, and I was sorry that I wouldn't be able to meet with some newly diagnosed families in person after several phone calls. The worst part was that it happened so suddenly, and we had to cancel the already long overdue meeting. When newly diagnosed patients reach out to us and attend a support group meeting, it provides a profound comfort that cannot be given through a phone call or text message.

Experiencing the lockdown and all of the unknowns of the pandemic has created many stressors for pemphigus and pemphigoid (P/P) patients. Pivoting to a virtual platform meeting in June felt like the least we could do to keep the group connected.

Luckily, Mindy Zimmerman has stepped up and been the most incredible host. I know that shifting gears and mastering technology is not for everyone. We do have some members who struggle with the technology, and we are working to find a solution. Part of the benefit of



a support group is that when something needs to happen, there's usually someone who knows what to do.

So far, we have held four support group meetings via Zoom, and each one has been a lifeline. Newly diagnosed patients and their families can communicate with some of the regulars and those who are lucky to be in remission. Even virtually, we can see their relief. We have had a patient from South America join our meetings, as well as attendees from all over Florida. Snowbirds (Florida winter residents) spending time up North were able to join, and what used to be a long drive for some to participate has now become a journey of a few clicks. Though Florida is large, we are the only IPPF support group that currently exists in the state. This virtual option, which we did not use widely before, now feels normal. It allows more accessibility to patients who might have had obstacles to attending a meeting in person.

Currently, we are trying to formalize a meeting agenda that also includes the opportunity to talk, catch up, and share our stories. At one of our virtual

meetings, we had a guest speaker, Iris Cohen, MSW, discuss mindfulness and lead us in a short meditation. I think everyone who joined that day felt better. Her presentation discussed the chemical brain response to stress and how breath work can help.

At another virtual meeting, a newly diagnosed patient attended with several family members who were located around the world. We discussed that the erratic behavior that may coincide with taking prednisone isn't imaginary, and we provided collective responses with tips for the patient and family members.

Personally, these chances to meet with fellow patients have kept me motivated and hopeful. I care about connecting with others, regardless of the manner. I believe that in the future, even after the pandemic, it will be beneficial to hold meetings with a virtual element.

Daphna Smolka is a patient with PV. She was originally misdiagnosed in 2013 and then properly diagnosed in 2014. She lives in South Florida with her children and dog, Milo.

Do you have mucosal pemphigus vulgaris?

You may be able to take part in the DesCAARTes Study

The study will help find the highest dose of an investigational product that can safely be given to people with mucosal pemphigus vulgaris. The investigational product will be made by collecting white blood cells from your own body and changing (genetically modifying) them in a laboratory. These same cells will then be given back to you to potentially treat your condition.

You may be able to take part if **you are 18 years of age or older** and **you have been diagnosed with mucosal pemphigus vulgaris.**

Want to know more?

If you are interested in learning more about the DesCAARTes study, please go to <https://cabalettabio.com/clinical-trials/descaartes-phase-1-trial/>, or contact:

- Email (Cabaletta Study Team): clinicaltrials@cabalettabio.com
- Phone (Patient Recruitment Center): 800-711-4906
- Email (Patient Recruitment Center)*: cabalettatrials@iqvia.com

**Please include only your name and telephone number in the email. Someone from the recruitment center will contact you to discuss the study.*



International Pemphigus & Pemphigoid Foundation
915 Highland Pointe Dr. #250 • Roseville, CA 95678
855-4PEMPHIGUS • 916-922-1298
info@pemphigus.org | pemphigus.org

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