RESEARCH HIGHLIGHTS | NEW IPPF OUTREACH MANAGER | ADVOCATING ON CAPITOL HILL

Quarterly
Journal of the International Pemphigus & Pemphigoid Foundation

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This issue of the Quarterly has great significance to me and the team as we get to share the success of the 19th Annual Patient Conference in Austin, Texas. This year’s conference would not have been possible without the tireless effort of Noelle DeLaney, Patient Services Manager. Our community owes her our gratitude. Although Noelle has left the foundation, she will continue to volunteer for the IPPF in the future. The conference brought together many key individuals of the pemphigus and pemphigoid community. Patients from across the country, expert dermatologists, oral medicine specialists, disease researchers, IPPF board members, staff, and our pharmaceutical partners all assembled to learn from one another and celebrate another successful year. I would like to extend a very special thank you to the conference host, Dr. Terry Rees from Texas A&M School of Dentistry, whose support was unwavering.

As you can see by many of the articles in this edition, pemphigus and pemphigoid research is beginning to take front-and-center in our quest for a cure. The IPPF is continually looking for ways to promote research opportunities, improve diagnostic delays, advocate for treatments, and support each and every one of you. As you will discover in this edition, recent research by the Japanese group at Tohoku University, led by Dr. Taku Fujimura and Dr. Setsuya Aiba, as well as work being done at the University of Pennsylvania by Dr. Aimee Payne and Dr. Michael Milone, are just two examples of the possibilities for learning about disease triggers and treatments for our patients.

Pemphigus and pemphigoid awareness and advocacy opportunities continue to grow. Patient participation and physician recognition are taking center-stage. Becky Strong is our new Outreach Manager, and she will be working with all of our stakeholders to improve patient and physician education, community engagement, and support services. In this issue, you will learn how patients are getting involved in clinical trials, advocating with their congressional members, and submitting artwork to help create awareness. Let us know how you want to get involved and become empowered!

We are in an extraordinary time. Science and medicine are moving at an incredible pace. I am confident that the opportunity to find a cure for pemphigus and pemphigoid could be within our reach. I continue to be inspired by all of you and your commitment to improving the quality of life of all people affected by pemphigus and pemphigoid.

Thanks for your support,

Marc Yale
IPPF Executive Director

Message from the Director

Marc Yale
IPPF Executive Director

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Hello all! Working for the IPPF has been something I have been interested in doing for about five years, since my pemphigus vulgaris finally got under control. I knew from my first contact with the Foundation that this is an amazing group of people. I am proud of the way our community pulls together and rallies for each other; it’s amazing how we sincerely care about one another.

The position of Outreach Manager is new to me, as well as to the IPPF. This position was created to be a link between Patient Support and the Awareness Campaign. While these programs have different focuses, they are united by the common goals of education, early diagnosis, and promoting the best treatments for P/P patients.

As a result, half of my time will be spent supporting patients. If anybody touched by our diseases has a question, comment, or concern, I am their first point of contact. It is my responsibility to provide all who contact me with clear, precise information and resources. I will also be creating and revising material for print and online, as well as helping to facilitate local support groups, meetings, and patient education calls.

The other half of my job will be spent raising awareness of P/P in the medical community with the goal of reducing patient suffering and diagnostic delays. Currently, the Awareness Campaign focuses on dental outreach. I will be coordinating patient presentations at universities and looking to recruit, train, and supervise other patient speakers and volunteers. I will also take over coordinating exhibit booths at conferences for dentists and medical professionals.

It sounds like a lot of work — and it is — but I am up for the challenge! Because of my status as a PV patient and my past work in the healthcare field, I feel that I am qualified to work with both communities simultaneously. I promise to work tirelessly to promote quality of life for the members of our community, as well as in the dental, medical, and nursing communities.

I know I would not be where I am today if it weren’t for the IPPF community. So many of you provided me with support, compassion, and encouragement during one of the lowest periods of my life. I was young when I was diagnosed. I was scared and felt isolated. But my IPPF friends changed all of that. I want to do that for others. It is my goal to pass along what I’ve learned and use my knowledge to make a difference in the lives of P/P patients. And the IPPF has given me this wonderful opportunity to do so.

Please know you can contact me by email at becky@pemphigus.org or by phone at (916) 992-1298 x105. I will work to get you the answers you need. I am here for you.
On Thursday, September 22, I joined 350 advocates on Capitol Hill for the Rally for Medical Research. Our main goal was to encourage members of Congress to continue robust, sustained, and predictable funding for the National Institutes of Health (NIH). Because I live in Washington, DC, a city without a vote in Congress, I was assigned to the group from Montana.

Along with a University of Montana biologist and a cancer research advocate, I met with Sens. Jon Tester and Steve Daines, and Rep. Ryan Zinke. All three members were supportive of our efforts to maintain NIH funding, especially Sen. Tester, who seemed to have a vested interest in medical research. In addition to our general request for NIH funding, I stressed the importance of cutting edge research of rare diseases and emphasized the significance of the Open Act, which incentivizes pharmaceutical companies to make treatments available off-label.

Of my three visits to Capitol Hill as a rare disease advocate, this is the first time I met with the members themselves rather than only their staff. It was a humbling experience, but also a powerful one. I truly felt that our representatives were listening closely when I told them my story of being diagnosed with, and treated for, pemphigus vulgaris. Meeting our lawmakers in person and telling our stories makes all the difference!

Sarah Gordon is an art historian and author. She teaches at American University and Photoworks at Glen Echo, and she lectures at the National Gallery of Art. Sarah was diagnosed with pemphigus vulgaris in 2007. She lives in Washington, DC, with her husband and two sons.
This past September, I had the opportunity to travel to Washington, DC, to represent the IPPF at the 2016 American Academy of Dermatology (AAD) Legislative Conference. It was a great experience, and if you ever get the chance — no matter how intimidated you feel — you should do it!

From the moment I arrived, I felt like history was about to be made. The conference was held at the Willard InterContinental Hotel. Urban legend proclaims that the term “lobbying” was coined at the Willard by citizens tracking down President Grant while he smoked cigars in the lobby. The League of Nations was formed in this same hotel, and many famous people have stayed there — including George Clooney the same weekend I was there!

The conference started with a Coalition of Skin Diseases Development Day. It was a fantastic opportunity to get to know other rare — and not-so-rare — disease advocates to learn what their organizations are doing, how partnerships can turn the tables, and tips to make our organizations more effective. It was amazing to see just how cutting edge the IPPF is and how many other organizations feel we are leading the way in advocacy and education. It really brought to the forefront how talented our staff and volunteers are and how much they have accomplished.

The AAD boot camp began on day two. Attendees were educated
on the issues dermatologists face and how current and future legislation will affect doctors and patients. We all know the patient part of healthcare. We live it, and it is our role. It was fascinating to get a glimpse into the lives of doctors and the impact legislation can have on their ability to treat patients.

As patients, it is extremely important that we have accessible healthcare options and medications. We need to have competition, even among generic companies, in order to keep prices within the financial reach of patients. Speakers helped patients and doctors see how these issues directly affect them. This helped us prepare for meeting our senators and representatives.

We were instructed about the importance of the people we would meet from our senators’ and representatives’ offices. While not everybody would meet with his or her senator or representative, it was also critical to convince the aides and assistants of the importance of the issues. These are the people who typically do the research and help senators and representatives decide how they will vote. If you move the staff, it will get back to the boss.

The conference speakers also pointed out how important follow-up would be, as it’s not enough to simply state your case. It is every bit as important to stay on their radars. My personal follow-up technique was to ask senators and representatives to take a photo with me while wearing the IPPF’s orange #healourskin sunglasses. Then, a few days later, I sent the picture and reiterated the importance of their support to the acts and bills we discussed.

Capitol Hill Day was the best day ever. Walking through the different office buildings on the Hill quickly brought to mind the fact people have walked those halls for 200 years. They shaped this country to what is today.

During meetings in those same offices, great men and women have wheeled, dealed, and pleaded to make this country a better place for their children. I knew patients like me could truly make a difference.

My time on the Hill was scary and fun at the same time. It was hard being away from my family and my job, but I was there for a cause much greater than me. It was greater than the IPPF. It was for each and every patient who has had to suffer, lacked health care, or couldn’t afford necessary medications.

There was a real sense of the power we have as patients. We are the constituents — the voters — who hold re-election power and have stories that tug on heartstrings. We have the knowledge of what it is like to live with a rare disease. We are the reminder that bills and acts aren’t just words on a page; they affect real people. A “yea” or “nay” could have a lasting impact not only on our lives, but on the lives of everybody affected by pemphigus, pemphigoid, or other diseases. “Yea” or “nay” could be the difference between gaining access to lifesaving treatments and not even being able to see a doctor. One patient’s mere presence and story can have a profound effect on how a senator or representative will vote.

While it will be a while before I find out how effective we were that day, we lobbied. We made our voices heard with the conviction of seasoned professionals. We the people came together to stand for the issues that affect us most. We stated our case as to why we require our senators and representatives to stand with us. Each and every one of us there made a difference that day. We spoke for those who cannot always speak for themselves. It was powerful. It was fun. It was American.

A research group in Japan has been studying the molecular basis of autoimmune mucocutaneous blistering diseases and, in a recent study, they’ve identified a link between a protein, POSTN, that has already been implicated in inflammatory skin diseases, and a particular immune cell type that is elevated in pemphigus vulgaris (PV) and bullous pemphigoid (BP). Their results also serve to help distinguish PV and BP at a molecular level.

The immune system is made up of several types of specialized cells that are involved in a cascade of events that lead to execution of foreign cells, such as bacteria, or that promote an inflammatory response, such as during an injury. During inflammation — including inflammation that occurs at pemphigus and pemphigoid lesions — one type of immune cell, macrophages, recruit other immune cells, T cells, which ultimately lead to lesion formation and inflammation. These cells communicate with each other through immune-modulating proteins called cytokines, chemokines, and matrix metalloproteinases (MMPs) that are secreted by macrophages and sensed by T cells. It has been shown that a specific subset of macrophages, those expressing a protein called CD163, are elevated in PV and BP. Yet, these two diseases have different sets of T cells, suggesting that communication between macrophages and T cells differ at the level of production of the immune-modulating proteins.

The Japanese group at Tohoku University, led by Dr. Taku Fujimura and Dr. Setsuya Aiba and driven by a desire to understand PV and BP at a molecular level, predicted that POSTN, which is already known to be involved in maturation of macrophage cells, might be at play in these diseases and that it might function at the level of a specific class of macrophage cell, the CD163-positive (CD163+) macrophages, which are known to be increased in PV and BP. In their recently published work in the journal Experimental Dermatology (http://onlinelibrary.wiley.com/doi/10.1111/exd.13157/abstract) the researchers found by immunohistochemical staining that dense deposits of POSTN were formed in the inflamed tissue from PV and BP patients, specifically in the superficial dermis, where these macrophages are known to reside. Following the cascade of signals known to take place downstream of POSTN, they narrowed in on the chemokine CXCL5, since this has been implicated in other autoimmune diseases including multiple sclerosis and rheumatoid arthritis. Not only were they able to show that the disease-linked CD163+ macrophages were activated in disease, they also found that while CXCL5 was significantly elevated in the blood of PV patients, it was unchanged in BP compared to non-diseased patients.

Since POSTN has been shown previously to stimulate CD163+ macrophages to increase the levels of the MMP, MMP12, the authors looked at this protein as well and found differences again in PV compared to BP. In PV, they saw MMP12 deposits in the upper layer of epidermis and superficial dermis in PV lesions but...
only in the superficial dermis in BP lesions. Finally, the researchers identified several cytokines that are known to be correlated to pathogenesis of autoimmune bullous diseases and looked for the induction of these by POSTN and for their production in lesion skin in the two diseases. From this analysis, they found the cytokine IL-36γ in epidermal keratinocytes and infiltrating leukocytes (another type of immune cell) in PV lesions and in epidermal keratinocytes, but only weakly in infiltrating leukocytes in BP.

These results suggest that POSTN stimulation of CD163+ macrophages could trigger PV via secretion of CXCL5 as well as IL-36γ, which is known to interact with the very cells, keratinocytes, whose adhesion to one another is corrupted in PV skin.

Mirella Bucci, PhD, is Secretary of the IPPF Board of Directors and a scientific journal editor living in San Mateo, California. She is a regular contributor to the Quarterly in the Research Highlights column.
In a study with potentially major implications for the future treatment of autoimmunity and related conditions, scientists from the Perelman School of Medicine at the University of Pennsylvania have found a way to remove the subset of antibody-making cells that cause an autoimmune disease, without harming the rest of the immune system. The autoimmune disease the team studied is called pemphigus vulgaris (PV), a condition in which a patient’s own immune cells attack a protein called desmoglein-3 (Dsg3) that normally adheres skin cells.

Current therapies for autoimmune disease, such as prednisone and rituximab, suppress large parts of the immune system, leaving patients vulnerable to potentially fatal opportunistic infections and cancers.
The Penn researchers demonstrated their new technique by successfully treating an otherwise fatal autoimmune disease in a mouse model, without apparent off-target effects, which could harm healthy tissue. The results are published in an online First Release paper in Science. “This is a powerful strategy for targeting just autoimmune cells and sparing the good immune cells that protect from infection,” said the study’s co-senior author Aimee S. Payne, MD, PhD, the Albert M. Kligman Associate Professor of Dermatology. Payne and her co-senior author Michael C. Milone, MD, PhD, an assistant professor of Pathology and Laboratory Medicine, adapted the technique from the promising anti-cancer strategy by which T cells are engineered to destroy malignant cells in certain leukemias and lymphomas. “Our study effectively opens up the application of this anti-cancer technology to the treatment of a much wider range of diseases, including autoimmunity and transplant rejection,” Milone said.

The key element in the new strategy is based on an artificial target-recognizing receptor, called a chimeric antigen receptor, or CAR, which can be engineered into patients’ T cells. In human trials, researchers remove some of patients’ T cells through a process similar to dialysis and then engineer them in a laboratory to add the gene for the CAR so that the new receptor is expressed in the T cells. The new cells are then multiplied in the lab before re-infusing them into the patient. The T cells use their CAR receptors to bind to molecules on target cells, and the act of binding triggers an internal signal that strongly activates the T cells — so that they swiftly destroy their targets. The basic CAR T cell concept was first described in the late 1980s, principally as an anti-cancer strategy, but technical challenges delayed its translation into successful therapies.

Since 2011, though, experimental CAR T cell treatments for B cell leukemias and lymphomas — cancers in which patients’ healthy B cells turn cancerous — have been successful in some patients for whom all standard therapies had failed. B cells, which produce antibodies, can also cause autoimmunity. Payne researches autoimmunity, and a few years ago, a postdoctoral researcher in her laboratory, Christoph T. Ellebrecht, MD, took an interest in CAR T cell technology as a potential weapon against B cell-related autoimmune diseases. Soon Payne’s lab teamed up with Milone’s, which studies CAR T cell technology, in the hope of finding a powerful new way to treat these ailments.

“We thought we could adapt this technology that’s really good at killing all B cells in the body to target specifically the B cells that make antibodies that cause autoimmune disease,” said Milone. “Targeting just the cells that cause autoimmunity has been the ultimate goal for therapy in this field,” noted Payne. A more disease-specific receptor In the new study, for which Ellebrecht was first author, the team took aim at pemphigus vulgaris. This clinically serious condition occurs when a patient’s antibodies attack molecules that normally keep skin cells together. When left untreated, PV leads to extensive skin blistering and is almost always fatal, but in recent decades the condition has been treatable with broadly immunosuppressive drugs such as prednisone, mycophenolate mofetil, and rituximab.

To treat PV without causing broad immunosuppression, the Penn team designed an artificial CAR-type receptor that would direct patients’ T cells to attack only the B cells producing harmful anti-Dsg3 antibodies. The team developed a “chimeric autoantibody receptor,” or CAAR, that displays fragments of the autoantigen Dsg3 — the same fragments to which PV-causing antibodies and their B cells typically bind, as Payne’s laboratory and others have shown in prior studies. The artificial receptor acts as a lure for the B cells that target Dsg3, bringing them into fatal contact with the therapeutic T cells. Testing many variants, the team eventually found an artificial receptor design that worked well in cell culture, enabling host T cells to efficiently destroy cells producing antibodies to desmoglein, including those derived from PV patients. The engineered T cells also performed successfully in a mouse model of PV, killing desmoglein-specific B cells and preventing blistering and other manifestations of autoimmunity in the animals. “We were able to show that the treatment killed all the Dsg3-specific B cells, a proof of concept that this approach works,” Payne said. T cell therapies can be complicated by many factors. But in these experiments, the Penn scientists’ engineered cells maintained their potency despite

“...the word “cure” is potentially within our reach with this technology.”
the presence of anti-Dsg3 antibodies that might have swarmed their artificial receptors. In addition, there were no signs that the engineered T cells caused side effects by hitting the wrong cellular targets in the mice.

The team now plans to test their treatment in dogs, which also can develop PV and often die from the disease. “If we can use this technology to cure PV safely in dogs, it would be a breakthrough for veterinary medicine, and would hopefully pave the way for trials of this therapy in human pemphigus patients,” Payne said. Also on the horizon for the Penn scientists are applications of CAAR T cell technology for other types of autoimmunity. The immune rejection that complicates organ transplants, and normally requires long-term immunosuppressive drug therapy, may also be treatable with CAAR T cell technology. “If you can identify a specific marker of a B cell that you want to target, then in principle this strategy can work,” Payne said. In addition to Payne, Milone and Ellebrecht, co-authors of the study include Vijay G. Bhoj, Arben Nace, Eun Jung Choi, Xuming Mao, Michael Jeffrey Cho, John T. Seykora and George Cotsarelis, all of Penn; Giovanni Di Zenzo of the Istituto Dermopatico dell’Immacolata in Rome; and Antonio Lanzavecchia of the Institute for Research in Biomedicine in Bellinzona, Switzerland.

Researchers have engineered T cells to target and kill a malfunctioning component of the immune system responsible for autoimmune disease, while sparing healthy immune cells that still protect the body. The work brings scientists closer to targeting only the disease-causing cells in autoimmune diseases, which isn’t possible now. Some autoimmune diseases occur when a subset of B cells, which respond to specific signatures of pathogens, incorrectly see a person’s own tissue as foreign, prompting the rest of the immune system to attack. Currently, strategies to treat autoimmune diseases involve wide-sweeping immunosuppression, which can leave a patient more susceptible to infection; what’s more, patients often experience relapse following such treatments. Now, initial results in mice by Christoph Ellebrecht et al. show that a more targeted approach may be viable for treating autoimmune disease. Inspired by a technique that has recently shown success for treating leukemia, the researchers explored how chimeric antigen receptors (CARs) may be used to target rogue B cells. CAR techniques involve harvesting the antibodies that trigger an immune response and fusing them to pathogen-killing T cells. By tweaking this technique, researchers can create an arsenal of T cells that target a specific pathogen — or in the case of autoimmune diseases, the abnormal B cells. Pemphigus vulgaris (PV) is a life-threatening autoimmune disease that results in blistered skin. Here, the team harvested the key protein, Dsg3, that disease-causing B cells recognize, and fused it to signaling proteins that activate T cells. When the researchers infused mice with the engineered T cells, their levels of Dsg3-targeting B cells decreased, as did the occurrence of blisters. Furthermore, these engineered T cells can divide and proliferate, the researchers show, suggesting that CAR techniques to treat PV, and perhaps other autoimmune diseases, could have long-lasting effects.

What does this mean for our community?

Noelle DeLaney asked Dr. Payne.

Noelle DeLaney (ND): Can you speculate about how this treatment might be translated to clinical practice?

Dr. Aimee Payne (AP): CAR-T cells have already been used in humans to treat cancer. At this point in time, we have generated just about the same amount of preclinical data as our cancer colleagues showed prior to starting clinical trials in humans, but pemphigus is not as quickly fatal as the cancers were in the initial patients they treated. Thus, we want to set a “higher bar” of evidence that these CAAR-T cells will work in pemphigus. So we are taking a two-pronged approach: we are initiating discussions on how we would design and gain approval for a first-in-human trial for CAAR-T cells, and at the same time we are seeking to open clinical trials for dogs with pemphigus. Dogs are one of the only animals other than humans that spontaneously develop pemphigus. If we can show that we can safely treat and potentially cure dogs with pemphigus, we think that will convince both doctors and patients to begin enrolling for human clinical trials of CAAR-T therapy.

ND: Is there a role for Pharma? If not, how will clinical trials be funded?

AP: Yes. The right partnerships will be essential. Pharmaceutical companies, universities, the National Institutes of Health (NIH), and philanthropic donors have funded the CAR-T cell work in cancer. We need
to make sure we have adequate resources to support the lab-based scientists, clinical treatment teams, and overall research infrastructure to safely and effectively move forward this technology to human clinical trials.

**ND**: How is it that this could qualify as an actual cure?

**AP**: The unique feature of CAAR-T cells is that they are a “living therapy.” Unlike an antibody-based therapy, in which a defined dose is infused, CAAR-T cells can expand over a thousandfold in vivo when they see their target (they can spawn new soldiers to fight off the enemy, if you will.) Additionally, we know from prior studies that they can also make memory T cells that can persist for decades. So you only infuse the CAAR-T cells once, and they will kill all pemphigus B cells, then go dormant. If a pemphigus B cell should recur at any point in the future, the memory CAAR-T cells can expand again and kill them. This exciting aspect is why CAR-T cells were named by both the director of the NIH and Vice President Joe Biden as part of the “moonshot” to cure cancer — the word “cure” is potentially within our reach with this technology.

**ND**: Do you have any information for patients wanting to participate in future trials?

**AP**: Stay tuned! We are still some amount of time away from human trials.

**ND**: Are there implications for other diseases or types of diseases/disorders this treatment could be used for (autoimmune and other)?

**AP**: Yes, this therapy could theoretically be used for any antibody-mediated disease, including other autoimmune blistering diseases like bullous pemphigoid, epidermolysis bullosa acquisita, and even non-skin autoimmune diseases such as myasthenia gravis and many others.

**ND**: What do you want pemphigus and pemphigoid patients to take away from this?

**AP**: We are optimistic that research will lead to better therapies and options for pemphigus and pemphigoid patients, but it’s by no means easy! Research support from the NIH and other sources has been dwindling over the last several years, and due to the rarity of pemphigus and pemphigoid, it has become increasingly difficult to advocate for research into mechanisms of disease and potential treatments for these diseases. Our community is in danger of losing outstanding research programs, and once that infrastructure is gone, there won’t be the intellectual capital remaining to make sure that future generations of doctors and researchers continue to focus on these devastating diseases. Please keep up all the great work you are doing at the IPPF in regard to advocacy and meetings to bring together doctors, researchers, and patients to talk about their disease and what the future may hold.

**More about Dr. Payne**

Aimee Payne, MD, PhD, is the Albert M. Kligman Associate Professor of Dermatology at the University of Pennsylvania. Her career interest has been in pemphigus: diagnosing and treating patients with this potentially fatal autoimmune disease, and performing research to better understand disease, with the goal of improving therapy. Dr. Payne received her BS in Biology from Stanford University and her MD/PhD from Washington University School of Medicine, followed by residency and postdoctoral fellowship training in dermatology at the University of Pennsylvania. Her laboratory has cloned B cell repertoires from pemphigus patients to better understand why disease occurs, research that has revealed common features of the immune response among patients. Her laboratory has also focused on patient-oriented research to improve pemphigus therapy, which has led to a better understanding of how rituximab works in pemphigus, as well as on the development of novel targeted therapies.

Dr. Payne’s work in the field has been recognized with the American Academy of Dermatology Young Investigator Award, the Charles and Daneen Stiefel Award in Autoimmune Diseases, the Sanofi Innovation Award, and election to the American Society for Clinical Investigation. Dr. Payne is also active in mentoring the next generation of physician-scientists through teaching medical students, graduate students, and dermatology residents. She serves as Associate Director of the Medical Scientist Training Program at Penn and faculty advisor for the Association of Women Student MD-PhDs (AWSM).

Now a volunteer, Noelle DeLaney was the IPPF’s Patient Services Manager for two-and-a-half years. She lives in Dixon, CA with her husband.
Wow! The 2016 Patient Conference has already come and gone. It was an amazing experience for attendees and presenters alike. This year’s event focused on the importance of peer support and expert research, and there was a special emphasis on oral care. The conference was full of learning, laughs, and the formation of life-changing bonds.

The Hilton Garden Inn Downtown Austin is located close to some of the best food and entertainment in the city. Austin’s 6th Street has been a famous entertainment hub since the 1970s. It’s home to South By Southwest — Austin’s famous music and film festival — as well as the Pecan Street Festival, which attendees got to catch a bit of after the conference. The street itself is closed to vehicle traffic on Friday and Saturday nights and becomes a pedestrian paradise filled with lights, music, and bustling people.

Thursday, 9/22

Terry Rees, DDS, MSD, Director of the Stomatology Center at Texas A&M University College of Dentistry, and I started the conference by giving a joint continuing education presentation to local dentists and patient attendees on pemphigus and pemphigoid (P/P) and the reason why early diagnosis is so important. Dr. Rees covered the academic content of managing patient care, while I gave a personal testimony of my journey to obtain a diagnosis. Together, we presented the whole picture of diagnosing, treating, and living with an autoimmune blistering disease.

Thursday night’s Cocktail Hour, Awards Dinner, and Casino Night were all held at Eighteenth Over Austin — a beautiful venue with 180 degree views of the city skyline. Aimee Payne, MD, PhD, Associate Director of the Medical Scientist Training Program at the University of Pennsylvania, gave the keynote speech. This included an exciting glimpse into the potential breakthroughs of her current research. The IPPF Awards Dinner recognized many people who have worked hard, both in front of and behind the scenes, at the Foundation.

At Casino Night, patients, doctors, and researchers alike tried their hand at games of skill and luck to
see who could gather the most chips by the end of the night. Marc Yale, IPPF Executive Director, served as MC, calling out the numbers of winning raffle tickets. Prizes included a Samsung Galaxy Tablet, FitBits®, Sonicare™ electric toothbrushes, Waterpik® flossers, and more!

Friday 9/23

Friday began with opening remarks from Dr. Rees and Marc Yale. Todd Kuh, IPPF Board Chairman, introduced the Board of Directors and staff.

Sergei Grando, MD, PhD, and a leading P/P expert from UC Irvine, gave a lecture on IVIG. Next, Victoria Werth, MD, Professor of Dermatology and Medicine at the University of Pennsylvania, and Member of the IPPF’s Medical Advisory Board, spoke of the treatments that are commonly used to treat our diseases. She covered why steroids and immunosuppressants work to treat P/P, as well as how they work. Dr. Werth also covered many of the side effects and complications that come with using such strong medications.

Kim Yancey, MD, Professor and Chair of the Department of Dermatology at the University of Texas Southwestern Medical Center in Dallas, covered the use of topical treatments in the care of patients with ocular, nasal, and oral disease involvement. He stressed the importance of regular checkups with eye doctors, dentists, and ENT doctors, as well as the role of dermatologists in managing lesions.

These lectures were followed by a Q&A session where patients and caregivers were given the chance to ask questions that were not answered in the lectures. Sessions like these give patients power over their diseases because they give them a chance to have an active dialog with experts.

After a short break, Animesh Sinha, MD, PhD, professor and dermatologist at SUNY Buffalo, gave a lecture on genetics. He explained certain genetic characteristics that most P/P patients share with one another and how blood samples collected at the IPPF’s Patient Conferences over the years have helped him in his research.

Dr. Rees participated in an oral care panel with Nancy Burkhart, RDH, EdD, adjunct associate professor at Texas A&M College of Dentistry and member of the IPPF Dental Advisory Council; Paul Edwards, MSc, DDS, FRCD(C), professor at Indiana University and member of the IPPF Awareness Committee and Dental Advisory Council; and Michaell Huber, DDS, professor at the University of Texas HSC San Antonio. This panel gave the patients a chance to ask questions that would help them improve their overall oral health. Topics covered included mouth rinses, brushing techniques, and the best kinds of toothbrushes for P/P patients.

After lunch, participants had the opportunity to choose from different breakout sessions.

The first group of sessions included topics as diverse as “below the belt” with A. Razzaque Ahmed, MD; clinical trials with Diana Chen, MD, MBA, FAAD; disease-specific patient reported outcomes with Badri Rengarajan, MD; and ophthalmology with Dennis Kay, MD.

The next group included a sessions on insurance from BioFusion’s Dinesh Patel, IVIG from Dr. Ahmed, oral care from Dr. Rees and Dr. Burkhart, and Rituximab and next generation therapies from Dr. Payne.

The final breakout sessions featured Dr. Payne’s lecture, “Future Targeted Therapy of Pemphigus,” as well as “Mindfulness Based & Positive Psychology with P/P” by Terry Wolinsky-McDonald, PhD; Dr. Ahmed’s “Pemphigoid Q&A”; and Dr. Sinha’s “Pemphigus Q&A.”

The day ended with Dr. Kay, Dr. Ahmed, Dr. Sinha, and Dr. Payne sitting for a Q&A session, followed by cocktails and cupcakes. Many attendees then made their way out to explore the best that downtown Austin had to offer.

Saturday, 9/24

Saturday was a day for patients by patients. The morning started with breakfast and another opportunity to bond with fellow patients. Camaraderie is so important with P/P. Our diseases can be isolating, and it was awesome to meet others who have walked the same path, many of whom are well on their way to remission.
Mei Ling Moore started the morning sessions with a guided meditation and de-stressing presentation, helping us all to be open to the day ahead.

Next, Valhalla Holeman led us through the emotional story of her son Laten’s struggle with pemphigus foliaceous. There were not many dry eyes during the presentation. Laten’s quiet strength — along with the support of his sister and brother, Myles and Coale, sitting next to him — was truly inspirational.

IPPF Peer Health Coaches Mei Ling Moore and Janet Segall, IPPF Board Member Dave Baron, and I led a patient-to-patient panel. There were so many good questions and comments, and it really seemed to bond our community more tightly together once again.

Roy Vongtama, MD, reinforced the mind-body connection in his lecture. He explained how we might not have control over the stressors in our lives, but we do have control over how they affect us. Dr. Vongtama then showed how posture, breathing, and meditation affect overall health.

IPPF Awareness Ambassador Coordinator Bryon Scott discussed the Awareness Ambassador program and simple ways we can all spread awareness in our networks and with our own dentist. Anyone interested in getting involved should email ambassadors@pemphigus.org.

Marc Yale then spoke of the work he is doing to advocate at the state and federal level. Marc is a true gem to the foundation. He has lobbied for Rare Disease Day in California and has stormed Capitol Hill with the National Organization on Rare Disorders and the American Academy of Dermatology. Marc broke down barriers and empowered everybody in the room. He made us aware that we, as patients, have the power to change laws in this country. It was truly inspiring.

Todd Kuh then gave a lecture on his “Chasing Down Pemphigus” fundraiser. Todd shared the story of how he felt after being diagnosed with PV and how a single conversation inspired him to regain the active lifestyle he led before his diagnosis.

After Todd, it was my turn to present “The Power of You.” There was a time when I felt I had lost my voice to PV, the treatments, and the side-effects of the medication. But somebody told me that patients are the true experts because we live with the disease. That inspired me to find my voice again, and my presentation focused on the ways we can all be advocates and share our expertise.

After the final presentations, Dr. Rees and Marc gave their closing remarks and hopes for all P/P patients. Everybody was touched. After two days of intensive and inspiring lectures, attendees left feeling united. It felt like we mattered. Doctors cared about the plights of patients like us. Patients felt like they had renewed hope. Some reinforced old friendships from past conferences, while others forged new relationships. I’m looking forward to what the IPPF has to offer in 2017!

Becky Strong is a PV patient and the IPPF Outreach Manager. She was diagnosed in 2010, but is currently in remission. She lives in Michigan with her husband Tim and her young family.
IPPF Awards

PHYSICIAN OF THE YEAR
Aimee Payne, MD, PhD

DENTAL PROFESSIONAL OF THE YEAR
Paul Edwards, MSc, DDS, FRCD(C)

STAR AWARD EDUCATION
Rebecca Strong
Hannah Heinzig

STAR AWARD OUTREACH
Rudy Soto

STAR AWARD SUPPORT
Mei Ling Moore
Mary Lee Jackson
Rudy Soto
Nancy Corinella
Ellen Levine
Sam Iwamoto
Esther Nelson

BRIGHT STAR AWARD
Noelle DeLaney

FOUNDERS AWARD
Marc Yale
Walter F. Lever, MD was one of the prominent dermatologists in the United States, during his professional life and thereafter. In the course of his career he made landmark contributions. His discoveries affected patients with blistering diseases then and even today. His legacy will last forever.

Dr. Lever was born in Germany in 1909. He initially studied in Heidelberg and got his medical degree from the University of Leipzig. Many young professionals were immigrating to the US because of political tensions in Europe. Dr. Lever was one of them. In 1936 he joined the Massachusetts General Hospital to do a residency in dermatology. He stayed there for 20 years working with a prominent focus in dermatopathology. In 1959, he became the Chief of the Dermatology Service at the Tufts University Medical School and subsequently the Chairman of the Department of Dermatology. He developed a strong research program that trained many young dermatologists who eventually became chairmen of many dermatology departments. In 1983, Dr. Lever returned to Germany. He died on his birthday in 1992.

He wrote many books, but the one that was most widely read and translated into 16 languages was Lever’s Textbook of Histopathology. Dr. Lever was the first person to separate pemphigoid from pemphigus, thus creating a new blistering disease. Hence, from a clinical perspective and a scientific viewpoint Walter Lever laid the foundation stone for what subsequently emerged as a subspecialty of dermatology, namely immunodermatology, which is mainly autoimmune blistering diseases.

To commemorate this enormous milestone in the history of modern dermatology, and as a token of respect for one of the most illustrious Chairmen of the Department of Dermatology, the Memorial Lecture Award was created. It is given once in 25 years for the following reason. It takes one or more decades to prove that any major advancement can be readily reproduced and the benefits from it are verified by physicians across the globe. Dr. Ahmed was cited for discovery of antibodies to beta 4 and alpha 6 integrins in mucous membrane pemphigoid and for identifying the ability of the combination of intravenous immunoglobulin and rituximab in producing long-term, sustained remissions in autoimmune blistering diseases.
About Bullous Pemphigoid

Bullous Pemphigoid (BP) is an acquired autoimmune skin blistering disease. It is a very rare disease that usually occurs in the elderly, but is actually the most common of the autoimmune skin blistering diseases. BP affects a lower layer of the skin, between the epidermis and the dermis, creating blisters that do not break easily. Lesions present predominantly on the abdomen, groin, back, arms and legs, but occasional patients may have involvement of the mucous membranes. The first symptoms are usually patches of itchy skin followed by blisters in as early as one week. The blisters may itch and be painful.

Bullous Pemphigoid Facts

- Bullous Pemphigoid generally responds to systemic corticosteroids (alone or combined with other oral agents), with most patients improving on prednisone at high dosages but there is no consensus on optimal dosing.
- High-potency topical corticosteroids are also commonly used for the management of BP, and may be used as monotherapy for patients with mild disease.
- ~70% of patients treated with systemic corticosteroids suffer from toxicities and major short and long-term side effects including but not limited to diabetes, glaucoma, peptic ulcers, skin atrophy and psychosis.
- Because of this toxicity profile of systemic corticosteroids, there is tremendous need for new and better therapeutic options.

For more information, please visit www.Immunepharma.com or www.pemphigus.org

Circulating autoantibodies bind to antigen BP 180 in the skin

A high density of autoantibodies induce complement binding and production of cytokines and chemokines in mast cells, including eotaxin-1

Eotaxin-1 binds to CCR3, the main chemokine receptor expressed on eosinophils to induce eosinophil migration

Migration of eosinophils into the skin leads to secretion of large amounts of eosinophil-derived proteases

Immune Pharmaceuticals is conducting a Phase 2 clinical trial (BP-01) to study the effects of bertilimumab in patients with newly diagnosed moderate to extensive BP.

- Bertilimumab is an experimental therapy targeting eotaxin-1, a pro-inflammatory protein which is believed to play a role in BP.
- Eotaxin-1 is found elevated in the serum and in the blister fluid. Eotaxin-1 attracts specific cells from the blood stream into the skin, including the eosinophils.
- The study treatment consists of three infusions of bertilimumab (the study drug) along with oral corticosteroids that will be tapered according to the clinical response as assessed by the study physicians.

For more information about the trial visit www.clinicaltrials.gov and search for NCT02226146.

For more information on Immune visit www.immunepharma.com

For more information on IPPF visit www.pemphigus.org