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Welcome to the final issue of 2014. It’s been an exciting year for the IPPF and we hope 2015 brings more of the same. I’d like to welcome Todd Kuh to the IPPF Board of Directors. Todd is a PV patient from Southern California and avid cyclist.

Our 2015 Patient Conference will be in New York City at Mount Sinai Hospital, April 25-26, 2015. Dr. Annette Czernik is helping plan the event and Biofusion is sponsoring our Friday Night Conference Kickoff, April 24, 2015, with dinner and refreshments at Yankee Stadium.

You may have seen a letter in the mail from my good friend Dave Baron asking you to support the IPPF. I met his father and sister at our 2006 Annual Meeting because he couldn’t make it (his PV made travel impossible). The IPPF helped him find a doctor, educated him on treatments, and supported him every step of the way. Now in remission, Dave doesn’t want patients to suffer like he did. Join him and his family with a generous gift to the IPPF this Holiday Season at www.pemphigus.org/donate.

William Zrnchik, MBA, MNM
IPPF Chief Executive Officer
will@pemphigus.org

Association and to dental students at Indiana University, conducted focus groups, and was published in the American Dental Hygienists’ Association’s Access Magazine. The Awareness Ambassador Program launched. We unveiled our new website in August and the Awareness Campaigns in December. And I could go on!

“I gave today because you helped me, and tomorrow, someone else is going to need your help.”

A First-time Donor

A Quick Recap

In 2014 the IPPF celebrated our 20th Anniversary. Noelle Madsen and Patrick Dunn joined our team (and Todd with the BOD). The Patient Education Series launched revamped Patient Education Calls averaging 75 registrations each. Our Patient Conference had 125 attendees and raised over $18,000. Social media grew nearly 1,000%. 450+ patient cases were closed. We supported 8 pieces of legislation benefiting patients. A dozen local support group meetings were held. The Physician Referral List grew 5%. The Awareness Campaign (AC) formed a Dental Advisory Council. We finalized patient and physician videos. We are supporting clinical trials. The AC presented at the American Dental

What’s in store for 2015?

Rare Disease Day. National Autoimmune Disease Awareness Month. Patient Conference. Patient Education Calls…and videos! New printed and downloadable information. Awareness Ambassadors. Patient Advocacy tools. New ways to support the IPPF. Dental Professional and Student Education. And much, much more!

It’s an honor to serve this wonderful community of friends and family.

My family, and the entire IPPF family, wish you and yours a safe and Happy Holiday Season!
My pain started four years ago, in April 2010. I distinctly remember the raw feeling in my mouth that appeared unexpectedly as I struggled to eat a meal with my family. Over the next few days, painful sores that were larger than usual mouth ulcers began to form in my mouth. I couldn’t remember ever having mouth sores, ulcers, or any mouth condition in my lifetime. The constant pain and the fact that I was finding it increasingly difficult to eat or drink made me concerned, so I booked an appointment with my general practitioner (GP).

Initially, my GP thought it could be thrush and prescribed basic antibiotics, mouth gels, and other remedies. They seemed to work for a while and eased the pain a little, but once I had completed the treatments, the sores reappeared, only more aggressively.

It was a toothache that led me to book an appointment with my dentist. She strongly suggested I see an oral consultant. At this point I was even more worried about my condition, as the sores were not going away and I was in constant pain. I began to research possible causes for my condition on the Internet, which I am not sure was a good thing to do as it made me imagine I had all sorts of possible diseases.

Near the end of June, at my first appointment with an oral consultant, I was diagnosed with possible geographic tongue. As the condition of the rest of my mouth became progressively worse, I decided to get a second opinion. The diagnosis from the second oral consultant was stomatitis vegetans, and I was prescribed metronidazole, Biotene® Gel and fluconazole. This treatment initially seemed to help, but after a while the oral lesions were widespread in my mouth, which led to my having a biopsy of my tongue.

Eventually, a biopsy confirmed I had pemphigus vulgaris (PV). Like many others, I had never heard of this condition. My oral consultant explained that PV was a rare autoimmune skin blistering disease for which there was no cure, but it was treatable. I was stunned and frustrated, but I also felt some relief that I finally knew what my disease was. The most disappointing news was that there was no cure, but I had faith in my oral consultant and the professor at Guys Hospital in London and believed that they would do everything they could to help me. I am also extremely lucky to have such a wonderful family and friends who have supported me during my suffering, and I cannot thank them enough. Unfortunately, I was unaware that worse was yet to come.

In a strange way, I seemed to be getting used to having this pain on a daily basis and it soon became the norm.

Between November 2010 and March 2011, I was prescribed a steroid mouthwash and continued to take metronidazole with some success. I also had regular reviews with my oral consultant. I continued to suffer on a regular basis with major flare-ups in my mouth and difficulty eating and sleeping. In a strange way, I seemed to be getting used to having this pain
The IPPF Awareness Campaign is excited to announce our new website: pemphigus.org/awareness.

With resources for dental professionals and students, as well as helpful information for patients, the new site is an important step toward achieving the Awareness Campaign’s goal of reducing the amount of time it takes a patient to receive a pemphigus vulgaris (PV) or mucous membrane pemphigoid (MMP) diagnosis. The site is also home to the IPPF’s Awareness Ambassador Program.

Since the majority of PV/MMP patients present their first symptoms orally, the Awareness Campaign website is specifically designed with the dental community in mind. There are diagnostic tools, tips for managing the diseases, and opportunities for continuing education.

Visit the site to learn about all the ways the IPPF Awareness Campaign is spreading knowledge about PV and MMP. We encourage you to “PUT IT ON YOUR RADAR.”

www.pemphigus.org
Three Tales of Awareness

Kate E. Frantz, MPH

The average pemphigus or pemphigoid patient sees five doctors over 10 months to obtain a correct diagnosis. The IPPF Awareness Campaign strives to change this statistic by reducing the amount of time it takes a patient to receive a pemphigus vulgaris (PV) or mucous membrane pemphigoid (MMP) diagnosis.

One way we do this is by sharing your stories. The following three stories highlight this important mission by sharing tales of awareness from a patient, a student, and a dentist.

International Ambassador

The IPPF recently launched its Awareness Ambassador Program with 11 participants attending the first Ambassador Orientation. Carlos Andres Campo filled out his paperwork right away and became the IPPF’s first Awareness Ambassador. Carlos is also our first international Ambassador, volunteering all the way from Bogotá, Colombia.

Diagnosed with PV 10 years ago, Carlos understands the struggle patients go through to obtain a diagnosis. He saw five doctors and two dentists over a period of six months before he was diagnosed. Carlos recognizes the need for awareness and is eager to contribute in whatever ways he can. “I hope to bring a message of hope by teaching and training people how to deal with this disease through the experiences I have lived,” he said. “I felt the need to be a connector between the United States and Colombia, as well as South America, if possible.”

In addition, Carlos will use his language skills to translate awareness materials into Spanish and Portuguese, providing a valuable resource to P/P patients around the world. “If a patient can be diagnosed at an early stage of the illness, they could have a better quality of life,” he added.

Intern Educates Campus

Rendell “Dell” Doctor has been interning with the IPPF for several months, working closely on the Awareness Ambassador Program. One day Dell informed us of an on-campus health fair at his college, Sacramento State. He immediately recognized the opportunity for awareness and proceeded to gather brochures and pamphlets and put together a trifold poster on P/P.

“I may not have any connection with PV or MMP,” said Dell. “I don’t have any of these diseases or know anyone who does, but I definitely agree with the strong need for raising awareness, especially on such rare diseases. The IPPF has taken a chance on me by allowing me to intern with them. I want to show that I can be as passionate about the effort as the people I work with.”

Dell also partnered with the pre-dental society and his pre-health professional fraternity, Delta Epsilon Mu, to showcase this information and get the word out. Several students and staff members visited his booth to ask questions, and a few even expressed interest in volunteering for the Awareness Campaign.

“These people, like me, had no prior knowledge of PV or MMP before learning of the IPPF,” Dell said. “It’s really great.

CONTINUED ON PAGE 14...
December marks the official calendar start of Winter. The good news: holidays, parties, special foods, festivities, time to be with family and friends.

The bad news for much for the Northern hemisphere: snowy, cold, and blustery weather, the shortest days of the year, time for reflection on people we’ve loved and lost, year-end promises made and broken. Sadly, holidays can be difficult for many people and can get the best of even a healthy person.

Seasonal Affective Disorder, also known as SADS, is a potentially debilitating condition that is characterized by depressed mood, particularly among people who live in areas that do not have sufficient sunshine or warm weather. While the symptoms usually improve as the season changes and the days and hours of sunshine get longer, for the many who experience it, it is a very difficult time of year.

I am starting to write this article in October (in Pittsburgh, PA), and the shorter days are already affecting many people. Once Daylight Savings Time goes back to Standard Time, it will be dark by 5:00 p.m. in many regions, meaning many people will go to work in the dark and return home in the dark. Others will go to bed late and sleep well into the day, allowing only a few hours of natural sunlight on a good day!

Years ago it became clear in my own psychology practice many patients became more depressed during winter months to the degree they temporarily required larger doses of antidepressant medications. Others were able to “function” but felt more sad, moody, or agitated, had less energy, and had more difficulty with everyday tasks.

Some people are so sad, irritated, and moody that they deliberately isolate themselves during the holidays and refuse invitations. This is probably the single most detrimental thing you can do. The holidays are not a time to be alone and looking into yourself. It is important to hold onto doable traditions.

I normally ask patients to over-plan their time during the holidays, thereby leaving less idle time to feel more upset or depressed. The period be-
The Immune System at Work

How Complement Functions in Bullous Pemphigoid

Mirella Bucci, PhD
Secretary, IPPF Board of Directors

The immune system is a complex network of different cell types, specialized proteins called antibodies, and cell-to-cell signaling events. There are two basic legs of the immune system: the innate and the adaptive immune systems. Innate immunity takes a shotgun approach to protect us, and is meant to attack anything that alters tissue structure and function, or looks “foreign,” such as a bacterial or viral invader.

The adaptive immune system represents a more long-term defense mechanism, and prepares the body for future challenges with invaders by generating specialized cells called B cells that each make a unique antibody to recognize a single foreign protein. Unfortunately, the adaptive immune system can make mistakes by developing B cells that make antibodies against “self” proteins (called autoantibodies). When this happens, autoimmune conditions arise.

All of the autoimmune mucocutaneous blistering diseases are caused by these antibodies. In bullous pemphigoid (BP), blisters are caused when the dermis and epidermis layers (specifically the lamina lucida) separate due to the action of another set of specialized immune cells, the neutrophils, that recognize that an antibody-protein interaction has occurred and come to perform their part of the adaptive immune system’s work.

In the case of BP, the antibodies bind to a protein called type XVII collagen (or COL17). Neutrophils release enzymes that digest the critical supporting structure that helps to hold the dermis and epidermis together.

Yet another part of the immune system has been implicated in development of autoimmune disease. The complement system is a type of surveillance and amplification system and is actually part of the innate immune system, but it can be called upon by the adaptive immune system to carry out its job of destroying whatever it is that the antibodies recognize.

One of the key players of the complement system is a protein in the blood called C3. Without it, the complement system is crippled. C3 is actually found at the dermal-epidermal junction of skin blistered due to BP, which is the main reason it has been implicated in progression of BP. As well, the complement system is activated in response to antibody binding to COL17 in a mouse model used to study BP. The research group of Dr. Hideyuki Ujiie in the Department of Dermatology, Hokkaido University Graduate School of Medicine, have recently asked whether the complement system is a red herring in the question of what causes BP, guilty only by association with the dermal-epidermal junction.

In their recent publication, Ujiie and colleagues developed mice lacking the complement system to find that autoantibodies against COL17 can still induce blister formation, suggesting that the disease progresses without complement simply by antibody-induced depletion of COL17 from skin cells (Journal of Immunology, www.jimmunol.org/cgi/doi/10.4049/jimmunol.1400095). The researchers had previously developed mice that were “humanized” to contain the human version...
Join the Call!

Noelle Madsen

Having a rare disease can be a scary thing, especially, when there are so few people in the world who know about your disease and what you are going through. Often times you have questions but no one is there to answer them.

At the IPPF we are here to answer your questions. Because the questions we receive from patients are often very similar, we host calls where patients can learn live, directly from other patients and experts, on pemphigus and pemphigoid (P/P) topics.

The Patient Education Series has been designed to bring knowledge and information to patients all over the world regarding P/P. The first project in the series is the monthly Patient Education Calls.

One of the strengths of the IPPF is our ability to reach individuals around the world and share expert-provided information with those affected by P/P. To that end, we host a monthly Patient Education Call with a different expert panelist each time, focusing on a different topic.

Some months will be general question and answer sessions with either a physician or other patients. Other months we focus on specific topics such as oral care, rituximab, IVIG, and nutrition.

It is a challenge to find a good time to accommodate the speaker’s schedule across the multiple time zones of our community. For this reason each month we have the call at a different time and day.

The calls are free to attend but subject to charges according to your phone or Voice over IP (VoIP) provider. Each call is recorded and key points are made available in a PDF for you to listen to or read at your convenience. We understand some calls may be at inconvenient times for some people, but we do everything we can to ensure everyone can still learn from what was discussed on the call.

Registration is open on our website for each call. We promote the calls through our eBlasts, Facebook, Twitter, discussion group emails, and on RareConnect. Hopefully, these reminders will help you check your calendar to see if you can attend. If you aren’t on our eBlast mailing list please email me at noelle@pemphigus.org so that I can add you.

When you register for the call you will receive an email with instructions. In the instructions you will be given the phone number to call, the access code to enter, instructions on how to ask a question, and the etiquette for asking a question of the panel member. We also have some international numbers and online/smartphone apps to help you get connected.

You don’t have to have a question to be a part of the call. Many callers listen in to learn something new. We understand it can be difficult to ask your question during a live call. For this reason, we provide an option when you register to presubmit a question that may be asked during the live call. If time does not permit for all of the presubmitted questions, we have them answered later on and will contact you with the results.

Our first Patient Education Call was in May 2014 as an extension CONTINUED ON PAGE 16...
of COL17 to test the effect of BP (human) patient-derived antibodies. Generally, mice are a great experimental model for studying the human immune system since the mouse and human systems have been found to be mechanistically very similar.

The authors genetically removed C3 from the humanized COL17 mice and showed that indeed, they lack the complement system. The authors also isolated four different autoantibodies from four different BP patients and found they vary in the degree to which they activate the complement system. All of the BP antibodies could induce skin detachment (characteristic of blisters) when injected into either the normal mice or in the complement-deficient mice, demonstrating the complement system is likely not at play in BP blister formation.

They next developed new antibodies that recognize the exact same portion of COL17 and found a correlation between the level of COL17 recognized by the autoantibodies and blister formation. Recent studies have shown COL17 antibodies not only recognize and bind COL17 but also deplete it from cultured cells. Ujiie and colleagues repeated that result showing it is complement-independent. As well, they find the same effect of COL17 depletion in the COL17-humanized mice - the antibodies caused blisters and simultaneously reduced the amount of COL17.

Finally, the authors found that this was due to an induction of the ubiquitin-proteasome system, the machinery of cells that acts as a garbage disposal for unwanted proteins. In this case, the COL17 autoantibodies somehow mark the otherwise normal COL17 for destruction, possibly setting the stage for BP symptoms and disease.

Several mechanisms may still be at play to mediate the effects of COL17 autoantibodies generated by BP patients (see figure). These include a degradation system, as suggested from the current work or COL17 may be internalized into cells upon binding of the autoantibodies, as has been seen in studies from other labs. It is also possible that COL17 gets internalized first and then the intracellular proteasome system degrades it. In any case, COL17 targeting by BP autoantibodies is a probably occurring by a more direct mechanism than if it involved the complement system.

It is possible that BP shares a mechanistic basis with other autoimmune mucocutaneous diseases such as pemphigus vulgaris, where autoantibodies recognize desmoglein proteins Dsg1 and Dsg3 in keratinocytes of the epidermis. Therefore, understanding the underlying mechanisms at play in blister formation in the various P/P diseases will be applicable to all patients being treated for these diseases.

Figure. A possible mechanism of COL17 depletion by autoantibodies. The new data support a model where BP patient antibodies that recognize COL17 generate a (yet unknown) signal for internalization of COL17 into the cell where it is tagged for destruction. This depletes COL17 from the surface of cells, which the authors found to be the critical event (after finding that the complement pathway does not play a role) leading to blister formation in BP patients.

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 newsletter in the “Research Highlights” column.

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IPPF ONE-ON-ONE INTERVIEW EXCLUSIVE

Q&A with Julia Jenkins of the EveryLife Foundation

Badri Rengarajan, MD

Established in 2009 by Dr. Emil Kakkis, a physician scientist and drug developer, the EveryLife Foundation focuses on catalyzing and accelerating the development of drugs for rare and orphan diseases. Before founding EveryLife, Dr. Kakkis was Chief Medical Officer of BioMarin Pharmaceuticals and is currently the CEO of Ultragenyx Pharmaceuticals. IPPF President Badri Rengarajan, MD, interviewed Executive Director Julia Jenkins.

Q: What does the EveryLife Foundation do?
A: Our organization works to create scientific-based public policy to improve clinical development and the regulatory process in rare diseases. The Orphan Drug Act was passed more than 30 years ago. Yet drugs have only been developed and approved for five percent of them (400 diseases among the 7,000 rare diseases). [Author note: In the US, a disease that affects fewer than 200,000 people is considered an orphan disease, and a disease that affects fewer than 50,000 people is considered an ultra-orphan disease.]

Q: What is distinctive about what your organization does relative to other rare disease organizations?
A: Our organization was created specifically to create change and improve the drug development process. We focus on policy issues that no other organization is addressing.

Q: Do you have a focus on certain types of rare disease or certain situations?
A: Our initiatives are beneficial for any rare disease. Improvements in the drug development process will benefit all rare diseases. However, our primary focus is on ultra-orphan diseases, which make up 80 percent of rare diseases. Our motto is “no disease too rare.” We also focus on diseases in which no treatments have been developed (which are most of them). It should be noted that 83% of diseases have fewer than 6,000 patients, yet only 18% of orphan drugs have been approved for ultra-orphan diseases. We are trying to address this lopsidedness.

Q: Who are your constituents or “customers”?
A: We work with patient organizations and, in some cases, parents and patients. We work mainly with smaller patient organizations (so-called “kitchen table organizations”). We also do some work with industry and regulators, and academics. We hold workshops to develop science behind drug development and regulatory process for rare disease drugs.

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Q: What are some significant programs of the EveryLife Foundation?
A: CureTheProcess: We want to accelerate biotechnology innovation and increase the predictability of the FDA’s regulatory review process.

Rare Disease Legislative Advocates: We support legislative advocacy for all rare disease groups with events such as lobby day, Capitol Hill Briefings, and a gala. We want to empower the individual to become an advocate by providing informational meetings, legislative resources, advocacy tools, and special events that support organizations and advocates working to promote rare disease legislation.

CONTINUED ON PAGE 16...
tween the winter holidays and early January is the most busy time of the year for mental health professionals, in part because it is difficult during these times of reflection not to become depressed, if that is a struggle.

It is important for those who are affected in this way to make a special effort to practice positive thinking (mindfulness) and reflect not only on the unpleasant, but also pleasant memories. I’ve suggested imagining that you’re putting negatives into a box and taping it up; then seeing yourself locking that box into a trunk; then imagining wrapping chains around the trunk and leaving it for a later date. You will know where the trunk is and how to retrieve what is inside, but you can also choose to keep all or most of the contents locked up for a while. At this point you may be willing to accept offers of help from others, which is among the healthiest of escapes.

The holidays are also a good time to reach out to others. We’ve all heard that it is truly more satisfying to give than to receive. Inviting lonely people to an event or volunteering at a nursing home or other facility, for instance, will have a positive effect on your mood. As a way to track your progress through the difficult holiday period, keep a record of your mood and activities or lack of activities. As regularly as you can, rate your mood on a 1-to-10 scale with 1 being the worst and 10 being the best. Also track your activities or lack of them as a record of the patterns and both positive and negative triggers. Everyone is different, and how you are in this world and during this time of year will not be the same as for others. Hold onto necessary traditions, but allow yourself to have new experiences. You may surprise yourself.

Besides these mindfulness approaches and techniques related to reaching out to others, there are a number of physical solutions that may help with dealing with SADS. Vitamin D supplementation has been found to be useful. As well, daylight spectrum lamps (or just special bulbs put into existing lamps) can also help counteract the seasonal lack of natural sunlight. Still, many people will need different medications or increased doses of their current medications. Checking with your primary care physician or having an evaluation by a psychiatrist or psychologist can be helpful and may greatly increase the quality of your holiday season.

After eight months of oral symptoms and appointments with doctors and dental specialists, I received my own PV diagnosis right before Thanksgiving. One of the first posts I read on the P/P online discussion group forum was from someone (Hi, Skip!) who recounted feeling lucky to swallow mashed potatoes in his first symptom-filled year. That really helped me to put things in perspective.

I focused on what I could do, not what I could not do. Unless you’re having a flare or going through a particularly rough time physically, get out and do things. Happiness is a choice, and all humans have the ability to be happy.

Happy holidays and my best wishes for a terrific new year.

Terry Wolinsky McDonald, PhD, is a PV patient, licensed clinical psychologist, and former IPPF Board member living in Pittsburgh, Pennsylvania. She is a regular contributor to the Quarterly newsletter in her “Psychologically Speaking” column.
on a daily basis and it soon became the norm. While it was a difficult and stressful time both emotionally and mentally, I tried to keep positive and deal with it as best I could. I took each day one at a time . . . what else could I do?

In December one of the doctors noted that along with the predominantly oral presentation, I had some skin symptoms, but it wasn’t until July 2011 as a result of my skin condition worsening with blisters on my scalp and face that I was referred to a dermatologist.

In September 2011, as my dermatologist had suspected, my PV diagnosis was confirmed and refined as pemphigus with predominant mucous membrane involvement. Unfortunately, my dermatologist moved to another country and I was referred to a new dermatologist. I was now being seen by both an oral consultant and a dermatologist. I continued a variety of different treatments (Dermovate, Protopic ointment, Elidel, Betnesol nasal spray, and prednisolone mouth wash) and drugs (prednisolone, azathioprine, mycophenolate). None of them were particularly effective.

During this time, the erosions had spread all over my face and nose and were particularly severe on the top of my head. I was then, and I still am, obese due to the large amount of steroids. The disease is extremely painful; so much so it affected my sleep because I cannot rest my head on the pillow at night. I remember visiting the hospital one day, when my lesions were particularly bad and the nurse thought I had been in a car crash!

Every time I washed my hair or face I would lose a piece of skin. Every time I ate anything my mouth would start to bleed. I can honestly say at this point I was a broken man with little quality of life. I would hide the pain and discomfort from my family. Many times I said I was okay, but inside I felt so depressed and despondent: there did not seem to be any light at the end of the tunnel. It felt like no one could help me and there was nowhere to go.

In an attempt to seek help and get yet another opinion, my dermatologist arranged for me to see a world expert in dermatology at Guys Hospital who determined that my high daily steroid requirement could not continue. He recommended a drug called rituximab and initiated a request for funding via National Health Service (NHS) of England. Unfortunately, the funding request was declined. I was devastated.

My wife wrote to our local member of Parliament hoping his support would sway NHS to reconsider their decision, but it didn’t. My wife and family decided that we had no alternative but to try and raise the money ourselves, and as a family we began our mission.

In preparation to receive rituximab, I was advised to start weaning off the drugs I had taken for so long. This had disastrous consequences and led to a massive flare up – the worst I have ever had. I had to be taken into hospital as an emergency case, where for over four days I received three pulsed doses of IV methylprednisolone and IVIG infusions.

This made an enormous improvement to my pemphigus and for the first time in years I felt so happy and such a sense of relief as the pain had largely subsided.

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I was aware IVIG is meant to be an interim treatment and a temporary solution. Generally patients remain lesion-free for up to 30 days. In my case only two weeks passed. I continued to receive IVIG every two to four weeks, and it seemed to stop disease progression.

Because I had to be hospitalized, my consultant submitted additional clinical information to the NHS explaining my new circumstances. Sadly, this renewed request for funding of rituximab was declined again.

One of my family’s first fund-raising events was a 100-mile bicycle ride. I am so proud of my son, brothers, and friends who took part. Everyone who donated has been so supportive, kind, and
to let people know as soon as possible, and maybe, by chance, someone contacts us saying that they know somebody who needs help.”

This goes to show no volunteering act is too small. We all have it in us to spread awareness and can start by looking for opportunities in our own communities.

**ADA Presentation**

On October 9, 2014, Dr. Vidya Sankar presented at the American Dental Association’s (ADA) Annual Meeting in San Antonio, Texas. Her presentation, “The Dental Detective: Investigating Autoimmunity,” addressed the common symptoms and referral patterns for several different autoimmune diseases, including PV and MMP. This served as an excellent way to provide continuing education on these illnesses to the entire dental community.

“The oral health care professional will commonly encounter patients with an array of oral and systemic health needs,” Dr. Sankar said. “The ADA offered us a platform to review some of these lesser known conditions in order to identify patients with potentially undiagnosed needs and act as a conduit to aid in diagnosis and management. Additionally, linking up the dental professionals with professional societies such as the IPPF will help to increase awareness and access to care and potentially increase our patients’ quality of life.”

As you can imagine, trying to cope with this disease over a number of years has taken its toll. I have gone from being a very fit and healthy man to being overweight with a painful, incurable disease. This whole period has been a depressing and difficult time for me and my whole family. The most difficult aspect of living with this disease is the excruciating pain I have had to endure, and therefore I am looking forward to returning to a life free of pain where I can enjoy keeping fit and healthy and return to my weekly cycling event with my brothers.

As a volunteer, it has been overwhelming and we have done really well so far. The IPPF has helped us, too, by providing promotional material that we handed out. We also had T-shirts and a banner made, which have definitely helped raise awareness of PV. Please check out the IPPF Facebook page to see photographs of the event.

Thanks entirely to the funds we raised, I received my first rituximab infusion two weeks ago, and it went well. I am due for my second infusion next week, and while I know I have a long wait, I am feeling very positive about the outcome.

I just hope and pray it will work for me. I have been advised that it normally takes at least three months to determine whether or not rituximab is effective, and I understand it is likely I will need further infusions. Over the next few weeks, I will hopefully be reducing the steroids and immunosuppressants.

The IPPF thanks Carlos, Dell, and Dr. Sankar for their collaboration on this project and their devotion to raising awareness.

Together we can raise awareness and promote early diagnosis of PV and MMP. We all have ways we can contribute. These are just three of the many more tales of awareness there are to share.

What’s yours? The IPPF encourages our Community to get involved with the Awareness Campaign. If you are interested in learning more about the campaign or getting involved, please contact Awareness@pemphigus.org.

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Scott is an Operations/Call Center Manager who lives and works in West Midlands, United Kingdom. Scott is happy to raise P/P awareness by telling his story in the Quarterly. Once he is feeling better, he hopes to continue to raise funds for other PV sufferers. If you would like to support Scott’s treatment fund, please visit http://www.youcaring.com/medical-fundraiser/medical-treatment-for-scott-doyle-pemphigus-vulgaris-/210699

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Kate E. Frantz, MPH, CTTS, is the Awareness Program Manager at the IPPF living in Dixon, CA. She is a contributor to the Quarterly newsletter in her “Awareness and You” column. Kate can be reached at awareness@pemphigus.org.
Veltuzumab is an anti-CD20 antibody administered by subcutaneous injection. In a recent study, it was shown to be safe and effective. Results showed one patient to be in complete remission of disease and off therapy. No serious or adverse events occurred during the 35 months of follow-up.

Orphan drug status is granted by the FDA to a drug or biological product to treat a rare disease or condition upon request of a sponsor. Orphan drug designation qualifies the company for various development incentives, including tax credits for qualified clinical testing, a waiver from FDA’s application, and a seven-year period of marketing exclusivity in the United States for veltuzumab, if it is approved by FDA for the treatment of patients with pemphigus.

The granting of an orphan designation request does not alter the regulatory requirements and process for obtaining marketing approval. Safety and effectiveness of a drug must be established through adequate and well-controlled studies before a drug becomes FDA approved.

I wanted to know what this means for the pemphigus & pemphigoid community, so I spoke to one of the authors on this study, Dr. Aimee S. Payne, M.D., Ph.D. Dr. Payne is an Albert M. Kligman Assistant Professor of Dermatology at the University of Pennsylvania.

I asked Dr. Payne what does orphan drug status for veltuzumab mean for pemphigus vulgaris patient right now. She said, “Although there is no immediate effect on pemphigus therapy right now, the orphan drug designation is an important first step toward FDA approval. It helps to encourage the development of treatments for rare diseases such as pemphigus.”

More specifically, I asked if she could elaborate on what this means for pemphigus vulgaris patients in the future. Dr. Payne replied, “We are currently working with the company to design a clinical trial of subcutaneous veltuzumab in pemphigus.” An advantage of subcutaneous dosing is it is more convenient, and requires a shorter post-injection observation time (30-60 minutes) opposed to intravenous dosing that can take several hours to complete.

While not immediate, Dr. Payne is hopeful veltuzumab testing could extend to other pemphigus and pemphigoid patients (i.e. PF, BP, MMP, OCP, PNP, etc.) saying, “Veltuzumab may eventually be tested in patients with other autoimmune blistering diseases in the future.”

To read the study abstract visit http://pemphigus.org/11OQNQ2.
of a discussion during our 2014 Patient Conference in Chicago. There, a panel of expert patients answered questions from the audience on different aspects of P/P. The discussion was incredibly informative. We decided to return to it for our first call in May.

June’s call focused on rituximab (Rituxan®/MabThera®) and featured special guest panelist, dermatologist, and a major influencer behind the founding of the IPPF, Dr. Grant Anhalt. Dr. Anhalt has immense experience in treating P/P patients with rituximab, and was able to provide a lot of insight on the treatment.

MAB member Dr. Neil Korman, also a dermatologist, was featured in July’s call, which was a general Q&A session. Dr. Korman has been treating P/P for years and is a good friend of the IPPF. He was able to provide care tips and advice to those able to ask questions.

A huge topic we get questions on regularly is oral pemphigus and pemphigoid. For example, “What kind of topicals are used for mouth lesions?” “Can I still brush my teeth?” “Can I have dental implants?” Our August call focused on oral care and featured professor and periodontist, Dr. Terry Rees.

September’s call focused on another hot topic here at the IPPF: IVIG. MAB member Dr. Sergei Grando is a dermatologist practicing in Irvine, CA, who has massive experience in treating P/P patients with this infusion therapy.

In October we did another patient panel of expert to answer questions. Peer Health Coach Marc Yale and IPPF founder Janet Segall joined us on the call.

Dr. Susan Cohen Byrne, a PV patient and psychotherapist out of Davis, CA, was on our November call discussing the mind/body connection. She focused on managing mental health while combating a rare disease.

If you were unable to participate in the previous calls and would like to listen to the call recordings or read through the call transcripts, you can go to pemphigus.org/peer-support/town-hall-series/.

If you want to learn more about the series, or have any questions, please contact Noelle Madsen at noelle@pemphigus.org.

Noelle Madsen is the IPPF Patient Services Coordinator and lives in Sacramento, CA. She is dedicated to providing support and education to those affected by P/P. She is a new contributor to the Quarterly and can be reached at noelle@pemphigus.org.

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Q: What is one of your biggest accomplishments?
A: We were successful in passing legislation which directed the US Food and Drug Administration (FDA) to increase access to accelerated approval pathways for rare diseases.

[Author’s note: Accelerated approval can bring down the cost and time of the drug development process allowing for less-stringent criteria (also known as a surrogate endpoint) when determining whether a drug has passed a clinical trial.]

Q: What are the foundation’s future aspirations?
A: A primary aspiration is to create a market incentive to repurpose major market drugs for rare diseases. Companies currently do not have an incentive to develop their current drugs in major diseases for rare diseases. Companies can be incented with market exclusivity.

Q: What are the biggest opportunities and challenges for rare disease community?
A: Thirty million Americans are affected with rare diseases, but the advocacy community is less than one million strong. There is potential to grow the advocacy community. FDA and National Institutes of Health (NIH) budget cuts are significant challenges.

Q: How can organizations and individuals get involved with your foundation?
A: We recently launched the Community Congress, which brings together industry and patient organizations. Additionally, organizations can endorse the CureTheProcess campaign (www.curetheprocess.org). We rely on our patient organization partners to reach out to their members to contact congress. Individuals can also get involved in the Rare Disease Legislative Advocates program (e.g., patients and parents).

Badri Rengarajan, MD, is President of the IPPF Board of Directors and lives in northern California.
Support Group News

The Southern California Pemphigus & Pemphigoid Support Group Meeting was held on Saturday, October 25, 2015 at the Santa Monica Public Library in the Martin Luther King, Jr. auditorium. Dr. Jennifer Haley, Dermatologist, Kaiser Permente and Dr. Vanessa Holland, Dermatologist, University of California, Los Angeles answered questions from patients and loved ones for two hours. There were 31 people in attendance. Patients asked a lot of important questions regarding treatments, nutrition, vaccinations, and on-going research. Also, many engaging discussions with the doctors and patients occurred. Hearing other patient experiences in dealing with this rare disease was healing. Afterwards, a lively social gathering was held outside on the patio with refreshments provided by Biofusion, who sponsored the meeting. Many came away with new friendships and an enthusiasm for next year’s meeting.

If you are interested in hosting a support group meeting in your local area please contact Noelle Madsen at noelle@pemphigus.org.

A group of PEM Friends enjoyed lunch together in London, November 2015. We met to share their experiences and advice. We were particularly pleased to be joined by Dr. Jane Setterfield, reader and honorary consultant in dermatology in relation to oral disease at Guys and St Thomas’ Hospital. Dr. Setterfield's enormous experience and understanding of bullous disease and advice on its treatment, was well received. Everyone had a chance to talk with her and get a lot of questions and concerns answered.

It’s always good to meet others who have faced similar challenges and this was a great opportunity to do so in a relaxed, informal environment.

On October 18, 2014, the Houston Support Group held its FOURTH meeting of the year! Patients gathered at the Bayland Park Community Center and discussed their current disease states, provided feedback and support, and overall had a very nice discussion amongst peers.

The Houston support group meets every 3 months at the Bayland Park Community Center. If you have questions about the support group leader, or want some delicious pralines, contact Mary Lee Jackson at marylee@pemphigus.org.

DO YOU HAVE A GROUP?

LET US KNOW! It doesn't have to be formal to be a group! All you need is another person, a place to sit, and time to talk. The important thing is to be able to share your experiences and get the support you need.

If you want to find others in your area, contact Noelle Madsen at noelle@pemphigus.org.
2015 IPPF PATIENT CONFERENCE
NEW YORK
APRIL 24-26, 2014
MOUNT SINAI HOSPITAL

VENUE
Mount Sinai Dermatology
5 E. 98th St., New York NY

HOTEL
Courtyard by Marriott
Upper East Side
410 E. 92nd St., New York NY

REGISTER TO ATTEND
ONLINE
www.pemphigus.org/2015pc

MAIL
Send in the form on page 19

CALL
Call 855-4PEMPHIGUS (855-473-7447)

COST
Conference & Game: $170/person
Conference Only: $150/person
Game Add-on (limit 1): $40/person

RESERVE A ROOM
ONLINE
www.pemphigus.org/2015hotel

CALL
Call 800-321-2211
Inform the agent you are part of the IPPF room block at the Courtyard by Marriott, Upper East Side

COST
$259/night (single or double)
Must reserve by March 23, 2015

FRIDAY NIGHT CONFERENCE KICKOFF
AT YANKEE STADIUM!
SPONSORED BY BIOFUSION

JOIN THE IPPF AS THE YANKEES HOST THEIR CROSS-TOWN RIVAL METS.

Tickets are just $20 for conference attendees ($40 for one guest). SEATING IS LIMITED TO THE FIRST 100 PEOPLE. Enjoy over 2 hours of food, beverage, and fellowship in our own reserved area before the game. Watch the game from our reserved seats with other patients, caregivers, and members from the IPPF. This special event includes transportation from our conference hotel and back after the game.

*The bus will depart the Courtyard by Marriott approximately 4:00 pm for Yankee Stadium. It will depart the stadium approximately 10:00 pm for the hotel. Times may change based on published game time.
# 2015 Patient Conference Registration Form

**April 24-26, 2015**

**New York, NY**

**Print Name (as you want it to appear on your name badge)**

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<th>Organization/Employer Name (Corporate Sponsors Only)</th>
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**I am a ☐ Patient __________ (PV, BP, etc) ☐ Caregiver ☐ Friend ☐ Other __________________________**

**Mailing Address - Street ☐ Home ☐ Work**

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**Daytime Phone: ☐ Home ☐ Work ☐ Cell ( )**

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**A portion of your registration may be tax-deductible. Please consult with your tax professional.**

**JOIN US FRIDAY NIGHT FOR OUR CONFERENCE KICKOFF AT YANKEE STADIUM SPONSORED BY BIOFUSION.**

## Patient Conference Registration Fees

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**I WANT IT ALL! Complete Conference Package: $170.00 per person**

Enjoy the conference from start to finish! Join the IPPF and Biofusion on Friday night at Yankee Stadium for the Subway classic Mets-Yankees game! Then join your P/P friends at the plenary sessions and workshops on Saturday and Sunday at Mount Sinai Hospital. Also includes light breakfast, breaks, and conference materials. Transportation between Yankee Stadium and the Courtyard New York Manhattan/Upper East Side will be provided. Parking near Mount Sinai is at a reduced rate of $X per day.

**MEETING ONLY! Conference Registration Only: $150.00 per person**

You will be registered for all plenary sessions and workshops on Saturday and Sunday at Mt Sinai. Also includes light breakfast, breaks, and conference materials. Parking near Mount Sinai is at a reduced rate of $X per day.

**BRING A FRIEND! Friday Night at Yankee Stadium Only: $40.00 LIMITED SPACE!**

One per paid complete conference package.

Transportation between Yankee Stadium and the Courtyard New York Manhattan/Upper East Side is provided.

**Scholarship Donation. Please help someone else attend this year's conference.**

$  

**Tax-Free Donation. Help support current and future programs and research.**

$  

**Total Enclosed**

$  

☐ Check/Money Order made payable in U.S. funds to IPPF

☐ VISA ☐ MasterCard   Card # __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ __ ____
PEMPHIGUS & PEMPHIGOID
18th Annual Patient Conference

Mount Sinai Department of Dermatology
April 24-26, 2015

Featuring Bullous Disease expert talks on skin, dental, and psychological issues.

The IPPF Patient Conference highlights the power of community featuring the collective voices of P/P professionals. Join us as we gather, engage and contribute to improving the quality of life for P/P patients everywhere. At this conference, attendees will identify strategies and discover solutions to help start your journey, or move closer towards remission. Come prepared to expand your personal support network, create professional relationships, and leave empowered and inspired.

REGISTRATION NOW OPEN!
MORE INFORMATION AT:
www.pemphigus.org/2015ny or call (855) 4-PEMPHIGUS

Join the IPPF and Biofusion at Yankee Stadium for our 2015 Patient Conference Kickoff!