International support is more than emails and Facebook. Read about positive changes in Italy, the keys to Canada’s successful meetings, and an opportunity to be heard in the UK.

Life is filled with complicated things. Which shoes to wear? Getting the DVD player’s clock to stop blinking. The FDA orphan drug process. Well, we can help clarify the last one.

Weekends are for mowing the lawn, picnics in the park, and grocery shopping. Read how one patient’s weekend at the Annual Meeting in Boston changed her life.

www.pemphigus.org
Spring is in the air and the IPPF is busy planting the seeds of initiatives and enhancements to benefit patients everywhere.

Awareness is a big part of what the IPPF does. The more people know about our diseases, the more people can do about it. On February 28, 2013, the IPPF led a group of six rare disease organizations in our first-ever Rare Disease Day awareness session at the California State Capitol. More than 40 people were on hand to hear talks from rare disease patients and caregivers, including Rare Disease Day Resolution author Assemblymember, Marc Levine (D-CA 10th District), and IPPF Founder, Janet Segall. That evening, another successful international Town Hall took place, with Dr. Takashi Hashimoto from Japan as he battled technology and a 17-hour time difference to answer questions from more than 20 attendees. And this month, March, is Autoimmune Disease Awareness Month.

Still not enough awareness for you? The IPPF is making progress on its Awareness Campaign that targets dentists in training and in practice. This campaign will provide a consensus-based outcome paper on diagnosis and treatment and fund a two-year fellowship program. Patients seeing five physicians over 10 months before being diagnosed is a problem! Because the majority of pemphigus patients have some oral involvement, we see this as part of the solution.

The annual Patient Conference is coming up and San Francisco is THE place to be! Join us April 26-28, 2013 at the Hilton SFO Bayfront (register online at www.pemphigus.org/2013sf or use the form on page 15).

I want to thank Marc Yale for his work in making Rare Disease Day a success. Marc coordinated RDD events 500 miles away and a Town Hall with a guest speaker 5,000 miles away. He also joined me on a 2,700-mile, in-person trip to Miami to meet with our Medical Advisory Board and the Coalition of Skin Diseases. Marc is a man of many talents and the IPPF is better because of him. Thank you, Marc!

Inside you will find some great patient stories. Look for Toby Speed’s article on her 2012 Patient Conference experience and why she is coming back this year. Dr. Terry McDonald talks about pacing yourself in Psychologically Speaking. IPPF BOD President Dr. Badri Rengarajan interviewed U.S. Food and Drug Administration (FDA) officers. Internationally, Italian Pemphigus & Pemphigoid Association Board member Filippo Lattuca has good news for pemphigus patients in Italy; Dr. John Dart is holding a meeting for pemphigoid patients in London; UK patient Sue Bishop tells her story about PHC Sharon Hickey traveling from the U.S. to the U.K. to visit; and Canada’s Dan Goodwill is Still Going Strong.

I hope you enjoy this issue -- our community helped write it!
From the Top

Rituximab Approved in Italy

UK Patient Open Day

Still Going Strong in Canada!

Life is a Journey: Pace Yourself

get more online

Let’s Talk Orphan Drugs

3,500 Miles for a Hug and a Thank You!

What Good Can a Weekend Do?

Patient Conference Registration Form

Registry Tracker

Our Goal is 1,000!

15,000 Companies offer Matching Gift Programs.

Does Yours?

Contact your Human Resources Manager and ask if they have a Matching Gift Program you can participate in.
In recent years, rituximab has demonstrated to be a generally well-tolerated and effective treatment that could be beneficial for a high number of pemphigus patients. Pharmaceutical companies are not interested in investing in this drug for a rare disease like pemphigus. Therefore, this treatment is not directly available for pemphigus patients, even though scientific literature demonstrates its efficacy.

To get this treatment accessible to Italy’s pemphigus patients, the Italian Pemphigus and Pemphigoid Association (ANPPI), issued a formal request to the Italian Medicines Agency (AIFA) to include rituximab in the list of medicines to administer at public hospitals.

On November. 20, 2012, AIFA approved the request! The updated list of drugs included in law 648/96 has been officially published in the Official Gazette of the Italian Republic (the official journal of record of the Italian government) n. 286 on 7/12/2012: “Insertion of Rituximab within the list of medicinal products that can be administered at total NHS expense under law 23 December 1996, n. 648, for treatment of patients with severe pemphigus unresponsive to common immuno-suppressant treatments.” (You can read the Italian document at http://pemphig.us/ippf-nl72-anppi)

This effort was possible thanks to the support and sponsorship by some of the most important Italian doctors and medical scientists involved in autoimmune bullous diseases research and clinical practice. The ANPPI thanks Dr. Ornella De Pità, Dr. Giuseppe Cianchini, and Dr. Biagio Didona (Istituto Dermopatico dell’Immacolata in Rome), Dr. Camilla Vassallo (San Matteo Hospital in Pavia), and Dr. Emilio Berti (Ospedale Maggiore Policlino di Milano).

This is not going to solve all the problems related to rituximab administration, but it is the first time that this treatment is officially available to doctors treating pemphigus patients. The decision whether to use rituximab or other treatments has to be taken into consideration by the doctor regarding the stage of the pathology, history of the patients, co-morbidities, etc.

As its final consideration, ANPPI evaluated the financial impact of a rituximab base treatment of the disease vs. the costs of conventional therapies based on steroids and immunosuppressants.

The interesting, but not surprising, results show how the total cost for the National Health Service is, in the case of rituximab, much lower than in the use of steroids and immunosuppressants, if all cost factors are taken under consideration (for instance, preventing, monitoring, and treating the high percentage of side effects of immunosuppressant therapy in the medium to long term).

In the business case used by the ANPPI, based on forecasted numbers of new patients per year in Italy, the results show that rituximab-based treatment is more cost-effected than conventional immunosuppressant treatment. With these results, it would be best to use resources to further research of this class of drugs in the treatment of pemphigus and pemphigoid.
UK’s Patient Open Day for Pemphigoid

A forum for pemphigoid patients with lectures and lunch!

by Dr. John Dart
Consultant Ophthalmologist
Moorfields Eye Hospital, UK

U.K. pemphigoid patients are encouraged to attend an Open Day on Pemphigoid April 19, 2013, at the Clinical Tutorial Complex, Moorfields Eye Hospital. The purpose of this event is to find out from patients what they think the major problems are that relate to pemphigoid such as:

• deficiencies seen in health service provision for pemphigoid-related problems.
• how the health service and its staff could help pemphigoid patients more.

Attendees may speak for 10 minutes on a topic of their choosing, however, there is no guarantee everyone will be accommodated. Persons who want to speak should contact Dr. Dart with their topic.

Researchers are being invited and will give 10-minute presentations on their areas of research. There will be some scientific posters displayed which can be discussed with the research staff.

Expenses are non-reimbursable, but lunch and refreshments will be provided. There is space for 40 patients. For those who cannot attend, a summary will be available for those interested.

Please contact Dr. Dart if you are interested in attending, speaking, or for a copy of the proceedings. Elaina Reid, a Research Nurse, is assisting.

Professor John Dart, Consultant Ophthalmologist
Moorfields Eye Hospital
162 City Road, London EC1V 2PD
Tel: +44 (0)20 7566 2320 / Fax: +44 (0)20 7566 2019
email: j.dart@ucl.ac.uk

Still Going Strong in Canada!

by Dan Goodwill, PV
Toronto, ON, Canada
Former IPPF Board of Directors
Former CPPF Board Chair and Treasurer
www.dantranscon.com

I live in Toronto, Canada, and was first diagnosed with Pemphigus Vulgaris in 1994. The process of obtaining a diagnosis was quite typical of many patients. I saw five physicians over an eight-month period until two experienced doctors looked in my throat and whispered to each other, PV. I then began taking my initial dose of prednisone (60 mg). It was a horrible time in my life and I committed to try to help other patients in my area.

Shortly after that I founded a support group. To be honest, I did it initially for somewhat selfish reasons. I thought I could learn from other patients and it would provide comfort and support. Five years later, my disease went into remission but I maintained my involvement in the Toronto Support Group.

We continue to have two or three meetings a year and they provide great value to patients, their families, and their caregivers. Here are some of the keys to the longevity of our support group.

You need a passionate leader or leaders who really care about patients in their community. You need close ties with the dermatologists in your community. They typically refer patients to the group. You need to run well-planned, patient-centric meetings. Most of the time we try to have a doctor (who has some
Life can sometimes feel like training for (or actually running in) a marathon. It’s not a 5K walk. When people sprint, they run as fast as they possibly can for the entire race. Life isn’t just about the destination; there is a lot to see, feel, experience, and learn along the way. Life is a long and winding road, and you don’t know what might happen next without a crystal ball.

With chronic illnesses, some days will always be more challenging than others, but you are in it for the long haul. Learn to not feel frustrated every time you have problems doing things that used to be easy. Recognize that it’s part of your new way of being in the world, but not in any way the end of your world. Some days will be more challenging than others. There will be activities, hobbies or work that might not be possible any more - or maybe only on certain days. You might need help along the way to learn new coping skills. If you are very determined, you might be able to get through it, but with considerably more difficulty. However, if you “flare,” listen to your body and make necessary changes. When people ask how you feel, feel free to say that it is a more or less challenging day vs. labeling it "good" or "bad."

People get frustrated over both big and small inconveniences. Constantly fighting a war takes a lot of energy, even for the healthiest of people, and it is not physically or psychologically healthy to be in a constant state of conflict. With autoimmune diseases, your body is already at war with itself. Responsibilities might not change much, but how people choose to tackle them will always need to change. If you can stay flexible, you will do fine. Knowing your limitations (as well as your strengths) helps you view your world and how you choose to live in it.

Personally and professionally, I don’t believe that human beings were ever meant to be running on fast forward all the time. I often ask patients to do a simple exercise: I ask them to close their eyes, take themselves out of the picture, and just imagine everyone else on "fast forward." After a while, I ask them to imagine themselves in the picture and to pace themselves as they move forward, not getting trampled or left behind. We do this exercise until they feel comfortable with it. The image does not remain static. It can and will change multiple times as people adapt to their own comfort levels. Patients often ask me how fast or slow they need to be moving. I explain that this is not my “journey” but rather theirs. I personally work on my own "speed" and slow down regularly. It becomes second nature when you listen to your body, and you do need to listen to your body.

Pacing comes in all shapes and sizes and can have different definitions or interpretations. Negative pacing can be defined as walking around in mindless circles or back and forth with no real purpose. Pacing, in its most positive sense, is knowing how much you are capable of, feeling the difference, and then pacing how and what you do in a healthy way for you. It is a process not just a decision.

Continued on page 13...
Forum posting on high costs of rituximab
Recently there was an article published in TIME magazine that has been sweeping the Internet. Not just among pemphigus and pemphigoid patients, but in every disease community where patients receive rituximab. What are your thoughts?

Read more at http://pemphig.us/169DqYH

You should be in pictures!
The IPPF’s facebook page is filled with information and assistance. Our communities are more than friends, we’re family! And we’ve managed to add a few pictures along the way ;-)

See them at http://pemphig.us/ippf-facebook-photos

Health management is a team effort!
The Pemphigus & Pemphigoid Health Management Program was developed by the IPPF and Centric Health Resources using a Patient Centered Health Management® process, and is available to pemphigus and pemphigoid patients and caregivers!

Read more at http://pemphig.us/ippfhmp

Others need your advice!
Go online and help others understand their disease and medications, or share "pearls of wisdom" you have learned over the years. You know more than you think and sharing really is caring!

IPPF Forums http://www.pemphigus.org/forums
on Facebook http://www.pemphigus.org/facebook
Email Group http://www.pemphigus.org/emd

The IPPF website offers patient and caregiver support and information in one location. Creating a FREE account shares access to additional resources like the Patient Forums. Visit today at:

www.pemphigus.org
Recently, Dr. Badri Rengarajan sat down with two members of the FDA and said:

Let's Talk Orphan Drugs

by Dr. Badri Rengarajan
IPPF President

A few weeks ago, I had the opportunity to interview Dr. Anne Pariser (US FDA Office of New Drugs, Rare Disease Program) and Dr. Gayatri Rao (US FDA Office of Orphan Products Development). We talked about the role of their departments, orphan drug development, repurposing drugs, and what the IPPF and our members can do to help.

BADRI RENGARAJAN: You work in the FDA’s Office of Orphan Products Development. What is the Office’s mandate, and what is its relationship with the rest of the FDA?

FDA: The Office was created over 30 years ago. At that time, there was very little focus on developing products for rare diseases. The primary mission of the Office is to promote the development of products for rare diseases. Prior to the passage of the Orphan Drug Act, companies did not have enough incentives to develop products for the rare disease space. The Orphan Drug Act was created to provide those incentives, including a designation and grants program. Our office administers these programs. We are not in the review divisions of the FDA that review marketing applications [i.e., applications that seek approval to market a drug], however, we work closely with them.

FDA: We deal with companies early on. We review products for purposes of orphan designation. There is a corollary designation program for devices. We also have two grants programs to stimulate research for rare diseases and pediatrics – the Orphan Products Grants Program and the Pediatric Devices Consortia Grants Program. Rare diseases have become a focus internally and externally, so, in addition to administering the designations and grants programs, we also serve as a cross-cutting function among all different parts of Agency. We encourage collaboration.

FDA: In addition, we are often the first stop for patients with rare diseases.

BR: It seems to be common wisdom that pharmaceutical companies do not develop drugs for orphan or rare diseases because the revenue opportunity is not attractive (due to small market)? Is the common wisdom correct? Why or why not?

FDA: This wisdom has evolved over time. When the Orphan Drug Act was initially passed, this was the case. Even after the Act was initially passed, we did not see a whole lot of designations come through. But this has changed over time. Trial costs are rising, and exclusivity has been a good incentive. These days companies will generally see a return on their investment. The rare disease area is becoming a more attractive space – not just for small biotechnology companies but also for large pharmaceutical companies.

BR: How has the FDA made it easier or more attractive for drug companies to develop products in orphan/rare diseases?

FDA: With orphan designation, a company gets tax credits for clinical trial costs (up to 50%). If your product is the first to be approved for a particular rare disease indication [i.e., authorized use], you get 7 years of marketing exclusivity. Orphan designation also gets you a waiver of the FDA’s user fee ($1.9M), which is a fee companies who submit a marketing application to FDA must typically pay.

BR: How are the approval requirements for orphan drugs different?

FDA: To be approved in the U.S., all drugs must demonstrate substantial evidence of efficacy and safety, which is usually done through the conduct of at least one adequate and well-controlled clinical trial. There is no requirement that all drugs go through Phase 1, Phase 2, and two Phase 3 trials. This is often the case for common diseases, but each development program is different, and there has been considerable flexibility shown for rare disease development programs. The FDA can exercise flexibility and scientific judgment. It is important to work Continued on page 9...
closely with FDA to discuss the design of clinical development programs for rare diseases that are able to demonstrate substantial evidence of efficacy and safety.

**FDA:** Most (about two-thirds) orphan drugs are approved based on one adequate and well-controlled clinical trial and supportive information. What constitutes substantial evidence of effectiveness and safety will depend on what is known about the disease and population studied, the drug, and several other factors.

**BR:** Are there even less strict requirements for drugs aimed at ultra-orphan diseases?

**FDA:** There is no official term of “ultra-orphan” diseases. All are rare (also known as orphan) diseases. In the U.S., an orphan disease is defined by law as one that has a prevalence of less than 200,000 in the U.S. Most rare diseases are low prevalence (10,000-20,000 patients or fewer). Most approved products are for low prevalence diseases.

**BR:** Are requirements for orphan/rare drugs the same in other countries? (for instance, Europe and Japan) If not, what are the key differences?

**FDA:** This is outside of our authority. The regulatory requirements are not completely harmonized. Most of the time, FDA and other International Conference on Harmonisation (ICH) regulatory agencies, such as the European Medicines Agency (EMA), agree on approval decisions for orphan drug applications, and orphan designations. Many programs are multinational and we do collaborate quite a bit with authorities in other countries.

**BR:** From a regulatory perspective, are all orphan/rare diseases the same? If not, what are the different categories?

**FDA:** There are 7,000 rare diseases. They affect different age groups, have widely varying symptoms, exhibit different disease severities, etc. We talk about rare diseases like they are monolithic, but they are highly diverse. When thinking about orphan designations...

...continued from LET’S TALK, page 8
tion, understanding the disease is very important (e.g., is it one single disease or two diseases?) We even give orphan designation for subsets of common diseases.

**FDA:** With rare diseases, there is limited opportunity for study so you have to understand what is feasible, but other factors about the disease, the drug, and the expected effects of intervention are very important. The general principles of clinical research still apply.

**FDA:** Product candidates that come to the FDA for review are routed by disease or therapeutic area to the review divisions. For instance, a drug for skin diseases would generally be reviewed by the Division of Dermatology and Dental Products (DDDP).

**BR:** Are pemphigus and pemphigoid different from other orphan diseases from a regulatory perspective?

**FDA:** We searched our orphan designation database. We haven’t seen a lot of orphan designations for pemphigus. Ultimately, the same fundamental regulatory, scientific and clinical research principles would apply to drug development for pemphigus as for other diseases; however, the specific considerations for clinical development of a drug for pemphigus should be discussed with the review division.

**BR:** For ultra-orphan diseases like pemphigus and pemphigoid, what happens if there just are not enough patients to enroll in a trial? For instance, people may be too frail to participate in the trial, or they may not live close enough to a clinical trial site such as an academic medical center.

**FDA:** Most of these diseases are low prevalence. Most rare diseases are serious disorders, and many have patients who are very ill and medically vulnerable. This is where the concept of flexibility comes in. There is considerable diversity in approaches to developing drugs for rare diseases. For instance, in two-thirds of situations, only one adequate and well-controlled (A & WC) trial or another non-traditional study design is done. In contrast, for most common diseases, two A&WC trials are usually done. In unusual cases for some diseases, a case series is submitted. There is an example of a drug development program in which a clinical study of 8 people supported the approval of a drug. The important thing is to collaborate and talk to the FDA early and to arrive at a good trial design. Through this, we can often be quite successful.

**BR:** The FDA approves drugs for particular uses or “indications.” However, physicians can prescribe drugs for non-approved uses. For instance, Rituximab is approved for several uses (e.g., rheumatoid arthritis, certain types of lymphoma) but not for pemphigus, yet some doctors use it to treat pemphigus. How is this possible?

**FDA:** Drugs are prescribed by practitioners off label all the time. Medication choice is governed by the practice of medicine. The FDA does not regulate clinical practice. Based on what a physician knows about a patient, he or she does what is in the best interests of that patient.

**FDA:** Whether a drug is on-label or off-label does have implications for reimbursement.

**BR:** If Rituximab™ were studied in a pemphigus trial, would it have a faster path for being approved for use in pemphigus?

**FDA:** It’s a complicated question. You are re-purposing the drug. If you have a new drug with no prior use in humans, you have a long path with toxicology and other preclinical work that needs to be done. With a repurposed drug, you may already have that early work completed. You may then be able to jump in right at phase 2 and phase 3, but it depends on the circumstance. One should contact the relevant FDA review division to discuss trial de-
July 10, 2012 -- I will remember this date for years to come, echoing with the words, "You have pemphigus vulgaris." This meant nothing to me. I did an Internet search and was shocked. It looked grim. I couldn't believe this was happening!

I was unable to eat a normal diet and large areas of my mouth, throat, and nose became affected. I am a professional clarinetist so this was bad news. My consultant, aware of the impact, stepped up treatment from a mouth rinse to systemic medication. The prospect of high levels of powerful drugs filled me with dread.

I found the IPPF web page and contact a Peer Health Coach. I was soon contacted by Sharon Hickey. She patiently listened to my story, sent information describing various treatment options. It was very reassuring when my dermatologist suggested a similar plan. It was even more reassuring when Sharon told me that my consultant, Dr. Richard Groves at Guy's Hospital, London, is highly respected in the field of immunobullous diseases.

The treatment came just in time for my disease was progressing in strength and scope. Lesions cropped up on my back and scalp. The sores in my mouth were distressing and painful and for a time all but derailed my career and profession. I found the early days of medication, almost worse than the disease itself. Now, several months into treatment, I see the benefits! I can eat a wider variety of food again and recently gave my first concert ... pain free!

The IPPF Peer Health Coach Program has been wonderful! For a while, I felt I had no recognizable future. However, Sharon has been with me all of the way, offering reassurance and information. Her sheer joy and zest for life has been a great encouragement and helped me to get beyond that point. Sharon put all of this into perspective and I have been able to accept the seriousness of the disease while learning not to let it totally dominate my life.

I was amazed when Sharon announced she was coming to England and that we should meet. I had never met anyone with pemphigus before. Sharon enjoys music, so we arranged to attend an Advent service at St. Paul's Cathedral in London where I teach clarinet, sax, and recorder. The service was beautiful and dramatic with the choir singing superbly. The journey from darkness to light (represented by plunging the Cathedral into darkness) is a potent symbol of hope. It was such a pleasure to introduce Sharon, her family, and friend to Father Michael, the school chaplain. It brought two parts of my life together in an unexpected and happy way.

I am lucky in many ways. I was diagnosed quickly; have mild disease that is slow to progress; receiving treatment from a very able dermatologist; and healing well. I am beginning to look beyond pemphigus to a more balanced life. This sense of balance and calm has come from the generosity of other people with the disease. Siri Lowe of the Pemphigus Vulgaris Network, and the many kind people on the Facebook Pemphigus Vulgaris page who have been a constant support. The IPPF website has provided me with a reliable source of information. The IPPF Peer Health Coach program -- and Sharon -- have brought a sense of security back to my life. I am so glad I had the chance to meet Sharon in person to say thank you!
sign. If Rituximab were studied in pemphigus or pemphigoid, it may be eligible for orphan drug designation and all the incentives, including exclusivity.

BR: Given off-label usage is possible, what would be the utility of conducting a trial of an already approved drug in a new disease indication like pemphigus?

FDA: If you are deliberately measuring outcomes in a group of people (versus simply prescribing the drug to single patients), it becomes more of a research situation and you need to consider conducting this under an investigational new drug application, which is a type of authorization for doing investigational work.

BR: What can patient organizations do to support and accelerate the development of drugs?

FDA: You can do a lot. For rare diseases, one of the biggest problems is that patients are sparsely dispersed. It can be difficult to enroll trials. Describing natural history is very important, and patient organizations can help here. Also, many physicians may not be trained to treat patients with this disease. Patient groups can start registries (with type of disease, geographic location, etc.). Some organizations have initiated treatment centers – so if a treatment becomes available, they would have expertise and best practices located at one site.

BR: Is there a particular stage of the drug development and regulatory process where patient organizations can be most impactful?

FDA: All phases. Early on, trying to establish research registries, centers of excellence, and clinical endpoints is helpful. With slow trial enrollment, patient organizations can turn around the situation. Patient groups can help all the way through.

BR: Is there anything a patient advocacy group can do to assist the FDA’s review and approval process?

FDA: The FDA has a patient representative program. Through this program, patients can provide perspective at FDA advisory meetings. It is also important to partner with sponsors [e.g., drug manufacturers]. Sponsors may be willing to share information from trials, whereas the FDA cannot provide such data.

BR: Are there particular orphan drug policy efforts at play in Washington, DC that we should be aware of?

FDA: It is hard for us to comment on legislative activities.

BR: Are there particular orphan/rare disease organizations or groups we should collaborate with?

FDA: More experienced and larger groups are always willing to mentor smaller groups (e.g., cystic fibrosis group will talk to you and give you advice). NORD and the Genetic Alliance also do a lot of mentoring. The Genetic Alliance has boot camps. Get in touch with the Office of Rare Diseases Research at NIH. They can be very helpful. Rare Disease Day seminars, web casts, and events can also be helpful.

Anne Pariser, M.D. is the Associate Director for Rare Diseases in the Office of New Drugs at the US Food and Drug Administration’s Center for Drug Evaluation and Research. She established the Rare Diseases Program in OND in 2010, where she is currently working to support, facilitate and accelerate the development of therapeutics for rare diseases. The Rare Diseases Program concentrates on the development of biomedical and regulatory science, rare disease-specific training and education, and policy and guidance generation for rare disease product review and regulation. Dr. Pariser is also actively involved in numerous collaborations within FDA and with drug developers, other governmental agencies, advocacy groups and other stakeholders to further the development of treatments for rare diseases. Dr. Pariser has worked at FDA since 2000. Prior to founding the Rare Diseases Program, she was a Medical Officer and Team Leader in OND where she worked almost exclusively on the review and regulation of products for rare genetic disorders.

Gayatri R. Rao, M.D., J.D. is the Director for the Office of Orphan Products Development (OOPD) at FDA. The office’s mission is to advance the development of promising products, including drugs, devices, biological products, and medical foods, for rare diseases. As Director, she oversees a number of programs created to promote the development of such products, including the Orphan Drug Designation Program, the Humanitarian Use Device Designation Program, the Orphan Products Grants Program, and the Pediatric Devices Consortia Grants Program. In addition, she oversees the office’s extensive outreach efforts to patients, sponsors, and other stakeholders. Through her role in OOPD, she works to promote communication, coordination, and collaboration within FDA on rare disease issues as well as with other agencies and stakeholders. Prior to joining OOPD, Dr. Rao worked in FDA’s Office of the Chief Counsel where she provided advice on a wide range of issues related to medical devices, combination products, clinical trials, and human subject protection.
...continued from PSYCHOLOGICALLY SPEAKING, p. 6

Why is this so important? You may look healthier than you actually are and others will think that, too. It’s a lot like seeing someone who appears perfectly healthy using a "Handicapped" parking space. We all know heart problems and other diseases are not always visible, but they can be just as deadly. Being misunderstood might be part of your current scenario, but if you are doing too much or moving through life at a speed not conducive to a positive lifestyle, then it is time to take inventory.

At the upcoming Patient Conference (April 26-28, 2013 in San Francisco), the scientific and practical information will be covered by some of the best experts in the field. This year, stress reduction and psychological and emotional aspects of chronic illness are among the plenary sessions and workshops. I highly encourage attendees to take advantage of these sessions.

Until then, work on pacing yourself. Start with closing your eyes and visualizing things as described in this article. Sometimes it will be easier than others. Take the time to work on it. It will be time well spent.

Dr. Terry Wolinsky McDonald is a licensed clinical psychologist in the Pittsburgh, PA. area. She is a member of the IPPF Board of Directors, co-chair of the Patient Conference Committee, and regular contributor to the Quarterly. Her articles focus on the psychological aspects of living with P/P in patients, caregivers, and family members.

...continued from STILL GOING STRONG, page 5

involvement with pemphigus or pemphigoid) talk about an issue that concerns many patients. This may include oral care, wound care, meditation, the latest research and treatments, or diet and exercise. We try to limit these presentations, including questions and answers, to one hour.

In the second hour, we break up into small groups with each group led by a Canadian Pemphigus & Pemphigoid Foundation board member. The intent is to let patients talk about their illness and share their stories on how they are coping. While the small group leaders are not doctors, they provide information and support. We encourage everyone to speak. A significant part of the group consists of patients and their families who have been coming to the meetings for years. The others are typically newly diagnosed patients. It is very important they have an opportunity to speak and ask questions.

The meetings are held at one of Toronto’s leading dermatological centers, Sunnybrook Hospital. The hospital supplies us with an excellent conference room. One of the key supporters of the CPPF supplies refreshments and drinks. The support group has been providing a valuable service to people in the Toronto area and in southwestern Ontario for 18 years. I am very proud to be associated with this support group and I am pleased that I am able to help patients cope with this challenging group of diseases.

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I want to give the gift of Support this Holiday Season!

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www.pemphigus.ca

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What Good Can a Weekend Do?

by Toby Speed
PV patient, Mom, Author
New York Times Children’s Best Selling List
www.tobyspeed.com

A year before I went to the IPPF’s fabulous 2012 Annual Meeting in Boston, I had no interest in gathering for a week-end with doctors, researchers, Peer Health Coaches and other pem-pals. What good could it do?

Since being diagnosed with PV in December 2010, I’d been under the care of a terrific dermatologist. While the initial diagnosis had scared me, I’d read enough on the Internet to feel sufficiently informed. I’m a writer, not a scientist, and, frankly, all that stuff about proteins and B cells scrambled my brain. Did I really need to understand what was happening in my body? My doctor did, and that was good enough.

All I wanted was to go about my life without those nasty sores. So I took my medicine, went for labs, and waited for my condition to clear up.

Only it didn’t clear up.

I flared – badly. Clearly there was more going on than I realized. It was time to accept the fact that I had a chronic disease, one that was still in the early stages of being unraveled by scientists. If I was going to learn how to live with pemphigus over the long haul, I needed the standard tools:

• basic knowledge about the immune system to communicate with my doctor better
• a connection to the medical community to begin to grasp what researchers already know and where current research is headed
• shared experiences with others to better understand how diet, sleep, and stress affect me
• a community for ongoing support

The announcement about the meeting in Boston promised all that so I signed myself up. My daughter, Zoe, accompanied me. With a background of science, Zoe not only was great company and support but translated the molecular biology stuff. Handy!

As we checked in for the weekend, we were warmly welcomed by IPPF staff. Friday’s evening reception was a cozy mingling of patients, family members, and doctors in a setting suited to making new friends and getting to know some of the weekend presenters close up in a casual environment.

Saturday’s workshops covered the gamut of topics related to pemphigus and pemphigoid, their diagnoses and the choices doctors make when prescribing treatment regimes. My favorite talk was the one led by pioneer Dr. Sam Moschella on “Pemphigus Before Prednisone.” He described corn starch baths and treatment with an arsenic derivative called carbosone. Listening to him, the gravity and complexity of the disease hit home.

There was a lot of talk about rituximab, a B-cell targeting drug that has signified a real change in pemphigus treatment. This was particularly relevant to me, as I had just undergone Rituxan™ therapy. In one of the smaller breakout groups, I learned about Dr. Animesh Sinha’s work in genetics. In another, Dr. Vikki Noonan, a dentist, gave us great tips on oral hygiene.

One of the best parts of the weekend was making new friends. Zoe and I sat with a woman from Canada and her daughter at the Saturday night gala dinner and had a wonderful time sharing our lives and many laughs.

Oh, and the delicious food and the generosity of the event sponsors was beyond compare!

What good can a weekend with the IPPF do? It can educate, entertain, and inspire. It can bring to life the truth of the IPPF motto, “A common hope, an uncommon bond”. In short, a weekend can do lots of good!

I will be back again this year in San Francisco. I hope to see you there.
**DISEASE INFORMATION HOW TO REGISTER**

<table>
<thead>
<tr>
<th>Disease</th>
<th>Code</th>
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1. Register Online: [www.pemphigus.org/2013sf](http://www.pemphigus.org/2013sf)
2. Call our offices: (916) 922-1298
3. Contact your Peer Health Coach
4. Mail this completed form with payment to:
   IPPF 2013 Patient Conference
   1331 Garden Highway #100, Sacramento CA 95833

Registration cannot be processed without payment. Payment must be in US funds. All cancellations must be received in writing or by email to info@pemphigus.org. For cancellations postmarked on or prior to March 15, 2013, we will refund registration costs less $25 administrative fee. Cancellations postmarked between April 16 and April 10, 2013 may be refunded up to 50% of the registration fees. We may be unable to make refunds after April 10, 2013 due to food, beverage and material costs. We will gladly transfer your fees to another person as a scholarship, or credit you with a tax-free donation.

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**Patient Conference Registration Fees**

<table>
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<th>Fee Type</th>
<th>Regular</th>
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<tr>
<td>SINGLE Registration</td>
<td>$225</td>
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**GROUP Registration** if you are attending with one or more individuals

Your Name: __________________ Name: __________________
Name: __________________ Name: __________________
Name: __________________ Name: __________________

Registration Fees include attendance, materials, food and beverage during meeting times, Friday’s Welcome Reception, Lunch on Saturday, and Saturday Evening’s Social Mixer! *Subject to change without notice.*

**Scholarship Donation.** Can’t make it? Help someone else attend this year’s meeting.

**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
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  - Exp. Date: __ __ / __ __ Security Code: __ __
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Billing Address (if different from above): _______________________  
Signature: ______________________  
Indicates you agree to have your card charged.
Donate online or download a mail-in form at www.pemphigus.org/donate

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