

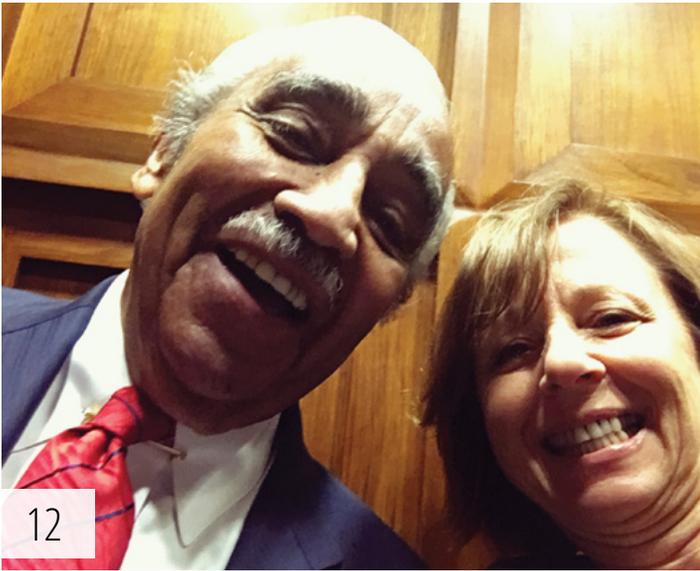
IPPF RESEARCH GRANT | NATURAL HISTORY STUDY | RARE DISEASE DAY 2017

# Quarterly

Journal of the International Pemphigus & Pemphigoid Foundation

The Research &  
Advocacy Issue

# WINTER 2017



12

14

## FEATURES

- 5 Meditate, Visualize, Affirm for Stress Relief  
*Janet Segall*
- 10 IPPF Year in Review: 2016
- 12 Where Patients Have a Voice  
NO EXPERIENCE REQUIRED  
*Debra A. Levinson*
- 14 Your Blood Donation  
IMPACT THE FUTURE OF P/P RESEARCH  
*Kristina Seiffert-Sinha, MD*
- 16 Why the Bertilimumab Bullous Pemphigoid Trial is So Important  
*Daniel Teper, PharmD, MBA*

## DEPARTMENTS

- Foundation**
- Message from the Director 3  
*Marc Yale*
- IPPF Research Grant 7
- Natural History Study 19
- Awareness**
- Awareness Update 4  
PATIENT EDUCATORS BY THE NUMBERS: 2014-2016
- Psychologically Speaking**
- Living in a Political World 6  
*Terry Wolinsky McDonald, PhD*
- Advocacy**
- Rare Disease Day 2017 8  
*Becky Strong*



5



8



6

# Quarterly

The Journal of International Pemphigus & Pemphigoid Foundation

ISSUE #87 | WINTER 2017

The IPPF is a U.S. 501(c)(3) nonprofit organization. EIN: 94-3207871

## BOARD OF DIRECTORS

Todd Kuh, *Chairman*  
Mirella Buccì, PhD, *Secretary*  
Sonia Tramel, *Treasurer*  
David Baron

Mert Eroglu  
William Gerstner  
David Sirois, DMD, PhD  
Mindy Unger

## MEDICAL ADVISORY BOARD

Sergei Grando, MD, PhD, DSci, *UC Irvine, Irvine CA*  
Neil Korman, MC, PhD, *Case Western Reserve University, Cleveland OH*  
M. Peter Marinkovich, MD, *Stanford University, Stanford CA*  
Dedee F. Murrell, MD, *University of New South Wales, Australia*  
Animesh A. Sinha, MD, PhD, *University of Buffalo, Buffalo NY*  
David A. Sirois, DMD, PhD, *NYU College of Dentistry, New York NY*  
Victoria Werth, MD, *University of Pennsylvania, Philadelphia PA*

## QUARTERLY STAFF

Patrick Dunn, *Editor-in-Chief*, [patrick@pemphigus.org](mailto:patrick@pemphigus.org)  
Toby Speed, *Copyeditor*

## CONTRIBUTING WRITERS

Debra A. Levinson  
Terry Wolinsky McDonald, PhD  
Janet Segall  
Kristina Seiffert-Sinha  
Rebecca Strong, RN  
Daniel Teper, PharmD, MBA  
Marc Yale

## CONTENT REVIEW TEAM

Vanessa Baron  
Sandra Gittlen  
Janet Segall

## IPPF STAFF

Marc Yale, *Interim Executive Director*  
Kate E. Frantz, MPH, *Awareness Program Manager*  
Patrick Dunn, MFA, *Communications Manager*  
Rebecca Strong, RN, *Outreach Manager*  
Monique Rivera, *Administrative Assistant*  
Mei Ling Moore, *Peer Health Coach*  
Janet Segall, *Peer Health Coach*  
Jack Sherman, *Peer Health Coach*  
Hannah Heinzig, BNS, *Patient Educator*  
Bryon Scott, *Volunteer Awareness Ambassador Coordinator*  
Debra Fuentes, *Cristo Rey Intern*  
Niko Pretelin, *Cristo Rey Intern*

## INTERNATIONAL PEMPHIGUS & PEMPHIGOID FOUNDATION

1331 Garden Highway, Suite 100, Sacramento, CA 95833  
toll free: 855-4PEMPHIGUS tel: 916-922-1298  
[info@pemphigus.org](mailto:info@pemphigus.org) | [www.pemphigus.org](http://www.pemphigus.org)

**POSTMASTER:** Please send address changes to  
IPPF, 1331 Garden Highway #100, Sacramento, CA 95833, USA

The *Quarterly* is published four times a year and provided free to donors as a thank you for their support. **The material presented is not intended as medical advice or to promote one product or service over another. Readers should consult their physicians before making changes to their health regimen.** The contents of the *Quarterly* cannot be reproduced or copied without written permission from the IPPF. Inquiries should be directed to: IPPF, 1331 Garden Hwy #100, Sacramento CA 95833, USA. **The opinions of contributors are not necessarily those of the IPPF.** We can reproduce articles and provide electronic copies of issues. If you would like to submit a story for consideration, please contact the Editor-in-Chief prior to submitting your story: [patrick@pemphigus.org](mailto:patrick@pemphigus.org)

© 2017 International Pemphigus & Pemphigoid Foundation

Printed in the USA by our friends at **SUNDANCE PRESS**, Tucson, AZ.  
[www.sundancepress.com](http://www.sundancepress.com)

# Message from the Director



**Happy New Year!** My last six months as Executive Director of the IPPF has truly flown by. Sitting down and reviewing our accomplishments, I realize how much our organization has changed since my diagnosis of MMP in 2007. However, one thing that has remained constant is the IPPF's continued pursuit of improving the lives of people affected by pemphigus and pemphigoid (P/P). At the forefront of this quest are *advocacy* and *research*.

P/P are not well recognized, understood, or researched. This is why advocacy is so important. We encourage you to get involved in advocating on Rare Disease Day—the last day of February every year. Rare Disease Day gives all P/P patients the opportunity to raise awareness of our rare diseases among lawmakers and share how they impact our lives. Coincidentally, the theme of this year's event is *research*.

Getting involved can be as simple as sharing your story. Thanks to generous sponsorships by Biofusion and Immune Pharmaceuticals, the IPPF is sending ten patient-advocates to Washington, DC, this year to hand deliver patient stories. To have your story included, please submit it online by February 24th at <http://pemphig.us/pemstories>

In recent years, the IPPF has placed an increased emphasis on encouraging the development of new therapies. We continue to collaborate with pharmaceutical companies on the leading edge of disease treatment. We also provide information about current clinical trials and research to P/P patients. This edition of the *Quarterly* includes articles about the impact of blood donations on research from Kristina Seiffert-Sinha, MD, and the importance of clinical trials from Daniel Teper, PharmD, MBA, and CEO at Immune Pharmaceuticals, Inc.

The IPPF recently announced its first research grant program, as well as a natural history study beginning in March to learn more about the progression and burdens of P/P. Later this year, we will be sponsoring the IPPF Scientific Conference in Lubeck, Germany. This conference will follow in the tradition of previous scientific meetings held in 2001, 2005, and 2010, bringing together as many as 150 clinical and scientific experts in the fields of pemphigus and pemphigoid.

As you can see, 2017 will be an exciting year for the IPPF. I look forward to sharing our story with you.

Thank you for your continued support,

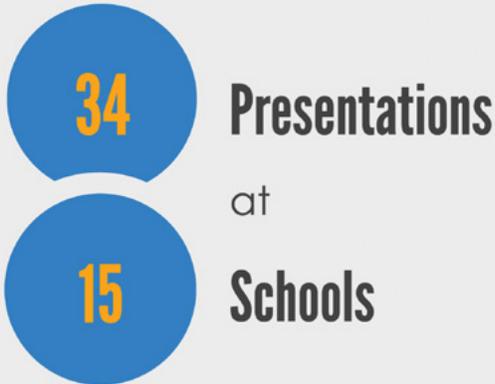


Marc Yale  
IPPF Executive Director

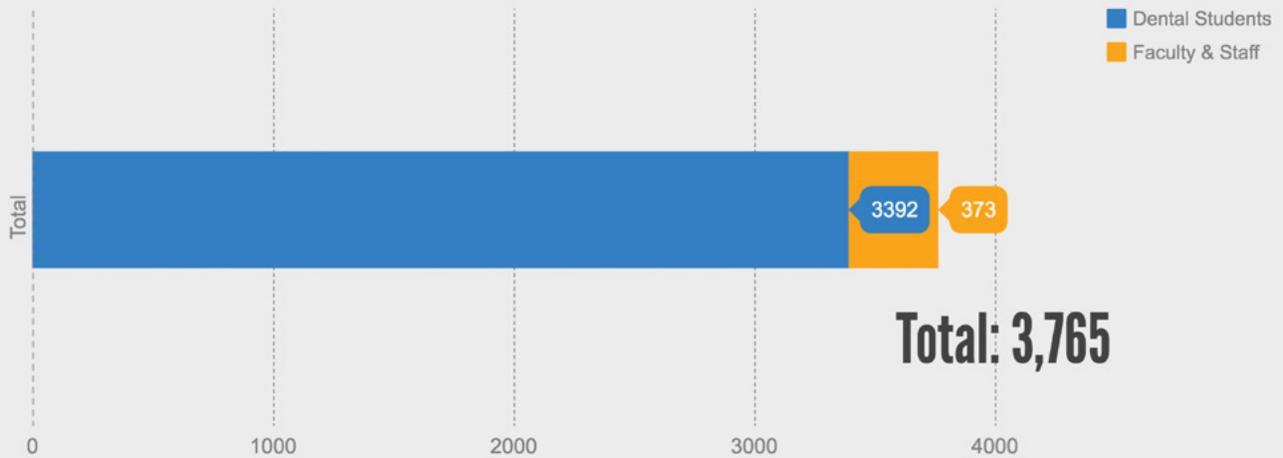
[marc@pemphigus.org](mailto:marc@pemphigus.org)

# Awareness Update

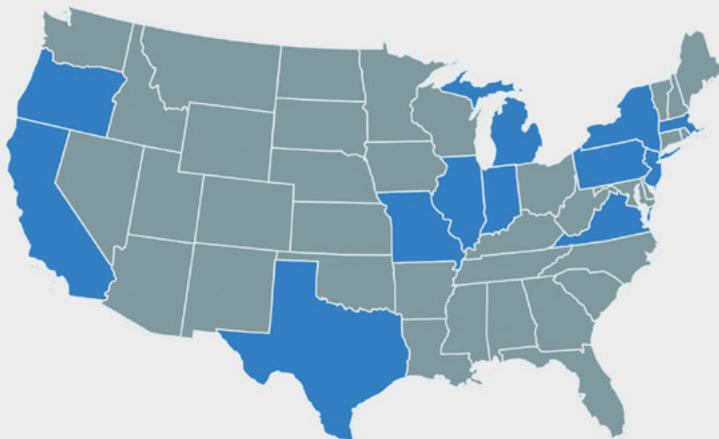
PATIENT EDUCATORS BY THE NUMBERS: 2014-2016



## Attendance



## Schools



- University of Michigan
- Indiana University
- Temple University
- UMKC
- Western University
- Loma Linda University
- Tufts
- Virginia Commonwealth
- Midwestern University-IL
- Boston University
- SUNY Buffalo
- NYU
- Rutgers University
- University of Pennsylvania
- Texas A&M

# Meditate, Visualize, Affirm for Stress Relief

Janet Segall

**W**hen I was first diagnosed with pemphigus vulgaris (PV) in 1983, the only available drug was prednisone. I investigated many alternative treatment methods, hoping to find something else that would help me deal with my disease. I ate soybean patties and had a tablespoon of linseed oil every day. I even drank some awful smelling and tasting Chinese tea three times a day.

Besides trying to find the right foods to eat, I checked out meditation and biofeedback. The biofeedback therapist taught me a meditation sequence that I've been using to this day. In 2009, I decided to learn hypnosis. I've now incorporated the meditation with the hypnosis, and it really helps with stress. When I was on medication, the meditation I learned from biofeedback helped me to deal with the side effects of prednisone.

I thought I would pass along the meditation sequence I learned and give you a visualization used in hypnosis. These meditations are best used twice a day, but even if you can do it once, it might be a great way to relieve stress.

Repeat each phrase three times. Between each repetition, count to seven. After the third repetition, go on to the next phrase.

- *My right arm is heavy.*
- *My left arm is heavy.*
- *My arms and legs are heavy and warm.*
- *My neck and shoulders are heavy and warm.*
- *My breathing is calm and regular.*
- *My heartbeat is calm and regular.*
- *My solar plexus is warm.*
- *I am at peace.*

You can repeat this exercise as many times as you need until you feel calm and quiet. Once you've found your peace, you can go into a deeper state of relaxation by counting slowly from 1 to 10.

You can then go one step further by using visualization and/or affirmations and your imagination. For example, imagine your stress (or pain or whatever else) as an object. Give the object a shape and then a color. Put the object in your hand and imagine it smaller and smaller. Then throw it in the garbage, or bat it into space, or whatever works for you to get rid of it. Feel how relaxing that can be.

You can also find a helpful phrase, such as, "I am a positive, healthy person," and repeat it. Or you can visualize a color that engulfs your body in a healing light. There are many ways to use these techniques.

I have found this very helpful for dealing with stress and pain. I've been doing it for so long, I can often (but not always) go to that place automatically and relax almost immediately—even before completing the instructions. I have even recorded these instructions so I can just sit in a comfortable position and listen in a quiet space. I work part-time with people who have mental health issues, and sometimes it can get quite stressful, so I'll often meditate during my breaks.

Meditation, and even hypnosis, has been studied a great deal. Both have been found to help with the issues we deal with as people living with pemphigus and pemphigoid. Caregivers can also benefit from these techniques. I know how they've helped me as both a patient and a counselor. If you would like more information, please feel free to contact me: [janet@pemphigus.org](mailto:janet@pemphigus.org).

---

*Janet Segall is the founder of the IPPF and served as executive director until 2008. She has been living with PV since 1983 and has been in remission since 2002. She is a Certified Hypnotherapist and Health Coach and currently works as a part-time administrative assistant/counselor helping people with mental health issues.*

# Living In A Political World

Terry Wolinsky McDonald, PhD

**W**hat a year! So much happened all around us all the time.

No one could keep up. Frustrations and strong emotions mounted. Overwhelming, 24/7 media coverage of political news became a constant. Terrorism; fear; acting out; Brexit. Divisive campaigns became daily fodder during an election year that felt more like a bad reality TV show. Then came the actual election, and all the predictions and polls became history.

My response: What do we expect while living in a world where more people know the names of reality TV personalities than their Vice President, Secretary of State, senators, and representatives? This is the reality of the world in which we are living. The political climate of the USA and the rest of the world has been unpredictable at best. As for rare and orphan diseases, who has been thinking much about them except for those of us directly affected? What will happen to people with preexisting conditions and rare, chronic conditions? Change remains the only constant, and we must adapt to all kinds of a “new normal.”

Can one voice make a difference?

Meanwhile in Pittsburgh, PA, there was a late fall with beautiful foliage remaining when I started to write this column. I don't have

a crystal ball, but I am pretty sure we will all be trying to get on with our lives after a such a divisive year. What does this mean for those of us with serious illnesses who are just trying to get life-saving medical treatments and protect the quality of our lives? We need to be proactive fighters for ourselves. Being proactive and appropriately assertive are very different from aggression.

A very good MD friend once told me that there are more politics in medicine than anywhere else, and that dermatology is one of the most political specialties. I know that when I needed life-saving medical treatment and went through traditional insurance channels (i.e., requests for treatment and timely insurance appeals), I was not particularly successful. Unfortunately, insurance companies do not grant medically necessary treatments (including FDA-approved treatments) just because the best medical experts prescribe them.

After more than a year of appeals and efforts to get potentially life-saving biologic medical treatment (non-FDA approved because of a lack of research), I wrote to the president and chief medical officer of my insurance company, with copies to my state governor, senator, and congressman. I provided written HIPPA permission with the letters,

so that the politicians could use their initiative if they chose to do so. Two of the three politicians did get involved. This made me, a patient trying to receive a medically necessary biologic treatment, a “high profile” patient—not because of the expert doctors who had signed my

A very good MD friend once told me that there are more politics in medicine than anywhere else, and that dermatology is one of the most political specialties.

last appeal, but because of a couple of politicians who “got involved.” So, the physician administrators of the insurance plan reversed their previous decisions. I received treatment in less than two weeks, after so many rejections of requests and appeals.

That continued for nine years, but now that I have Medicare, there are no guarantees, no matter how much extra I pay for additional coverage. The biologic I was approved for in 2007 is okay in Pennsylvania, but not in Florida. And here I thought Medicare would be the same in all states. It is a government program, right? Apparently, different states have different rules. And now, no one knows if Medicare and other

government plans will even exist or what they may cover in the near future.

Yes, politics can—and often does—have a major impact on the treatment of our rare illnesses, and the only certainty is that things will be changing. Unfortunately, I am unable to better understand and explain this phenomenon. Why are political voices more important than those of expert physicians? I do not know, and I do not believe they should be. I want to believe that at some point our expert professionals will once again be in charge of our well-being, but while my own glass remains half full, I currently have more questions than answers.

If you also feel this way, allow yourself to be validated at this time. Can one voice make a difference? Absolutely, and with the IPPF running on fast forward and more solid ground, we have more support behind each of us. Get to know your own elected representatives; this may become even more important than ever. It certainly cannot hurt.

*Happy New Year.*

---

*Terry Wolinsky McDonald, PhD, is a PV patient, clinical psychologist, and former IPPF Board member living in Pittsburgh, PA and Sarasota, FL. She is a regular contributor to the Quarterly newsletter in her “Psychologically Speaking” column.*

# IPPF Research Grant Program

The International Pemphigus and Pemphigoid Foundation (IPPF) is pleased to announce the availability of a seed grant program to encourage and support patient-oriented research of pemphigus and pemphigoid. The intent of these grants is to support well-defined research projects that have a specified benefit for improving the treatment, health, disease management, or diagnosis of persons with pemphigus and pemphigoid. Consideration will also be given to studies that contribute to the body of medical knowledge of pemphigus and pemphigoid.

“The IPPF Grant Program will help provide seed grant funding for research of diseases whose patients have unmet medical needs,” said Marc Yale, Executive Director of the IPPF. “We hope this program will encourage advancement in patient-oriented research in pemphigus and pemphigoid.”

The IPPF anticipates awarding 4-8 one-year grants in 2017 with values ranging from \$25,000 to \$50,000. IPPF Research Grants are currently open to applicants based in the U.S.

## Application Schedule

- Letter of Intent deadline for FY 2017 is February 28, 2017
- Application deadline for FY 2017 is March 31, 2017
- FY 2017 award announcements will be made on June 30, 2017
- FY 2017 funds will be awarded by August 1, 2017

Each research proposal is reviewed by the IPPF's Research Committee, which recommends funding for the highest scoring proposals. The grant review process is based on the peer review system used by the National Institutes of Health (NIH). Applications are individually scored by each member of the Research Committee, and scores are added together to determine the final ranking of each proposal. Any reviewer with a conflict of interest is excused from voting on that specific proposal. The grant selection process is unbiased and independent; awards are based solely on scientific merit.

## Application Process

1. Download and complete the IPPF Research Grant Guidelines and Application Form from the IPPF website:  
[www.pemphigus.org/research/ippf-research-grant-program/](http://www.pemphigus.org/research/ippf-research-grant-program/)
2. Attach CVs and/or bio-sketches, letters of support/recommendation, and letters from collaborators agreeing to participate
3. Submit the completed application with attachments to  
[grants@pemphigus.org](mailto:grants@pemphigus.org)

The application, along with the attachments, must be assembled and submitted electronically as a single PDF to [grants@pemphigus.org](mailto:grants@pemphigus.org)



## The IPPF wants to share your story on Capitol Hill

Thanks to generous sponsorships by Biofusion and Immune Pharmaceuticals, we are sending 10 patient-advocates to hand-deliver stories from pemphigus and pemphigoid patients to representatives in Washington, DC, for Rare Disease Day.

Submit your story online at [pemphig.us/pemstories](http://pemphig.us/pemstories)

# RARE DISEASE DAY 2017

## Becky Strong

**T**he first Rare Disease Day was first launched in Europe by EURORDIS, The Voice of Rare Disease Patients in Europe, and its Council of National Alliances in 2008. The USA joined the cause in 2009. By 2016, Rare Disease Day has become a worldwide event with over 80 countries participating.

Rare Disease Day is a pretty cool thing for those of us with rare diseases. According to the National Organization for Rare Disorders (NORD):

*Rare diseases are not so rare: there are 7,000 rare diseases & disorders that combined affect 30 million Americans—1 in 10 of us—and more than half are children.*

*People with rare diseases have tremendous unmet needs, including misdiagnosis, a long time to finally receive a correct diagnosis, and when they do, 95% have no treatment with ZERO CURES.*

*Rare Disease Day takes place on the last day of February each year. The main objective is to raise awareness with the general public and decision-makers about rare diseases and their impact on patients' lives.*

This year's global theme is research. It is our chance to give lawmakers, industry leaders, researchers, and healthcare professionals a glimpse of the impact a rare disease and its treatments may have. It is a local, national, and international awareness event.

Rare Disease Day is a day to find strength and power in your weakness, your disease.

Rare Disease Day is a day of unity for us patients. I can't speak for you, but I felt very alone and isolated when I found out I had pemphigus vulgaris. Then I found community in the IPPF. It was very comforting to know there were others out there who had gone through what I was living through. Now imagine being with hundreds of others who have a variety of rare diseases. Like us, they are patients looking not only for effective treatments, but also cures. On Rare Disease Day, we are a united front advocating for increased funding for research. It's a time of inclusion. A time for uniting as one. A time to make a difference.

It's also a time to network with other patients and learn about their diseases, how those diseases affect them, and what their disease organizations do to support their needs. It's a way to find out what we—as members of the IPPF—can do better to help and support all who are affected by rare diseases. It is a time to find community and strength in knowing that we are not alone in a search for better treatments and cures.

Rare Disease Day is a day to find strength and power in your weakness, your disease. You have the power to share your story. Your story has the power to impact the leaders of your city, state, and country. Your story can evoke changes in law, government, healthcare, and medical education.

I encourage you to look for opportunities where you can get involved. If no opportunity exists nearby, you can create your own. You could share your story with co-workers or local government officials, organize a bake-sale and donate the proceeds to the IPPF, or organize a run/walk to raise awareness. The list of possibilities is endless. Just be sure to explain what pemphigus and pemphigoid are and refer people to the IPPF if they have questions.

Learn more about Rare Disease Day and Rare Disease Week events at [rarediseaseday.org](http://rarediseaseday.org) and [rarediseaseday.us](http://rarediseaseday.us).

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

“A rare day to put rare diseases in the spotlight!”



Rare Disease Day

# RARE DISEASE DAY®



## When?

The last day of February every year

## What?

Rare Disease Day is to raise awareness for rare diseases and their impact on the lives of patients and families.

## Where?

Thousands of events around the world! EURORDIS organises the international campaign and National Alliances and other rare disease patient organisations hold events locally.

> [Visit rarediseasesday.org](http://Visit rarediseasesday.org) and find an event near you!

## Who?

For everyone!

The Rare Disease Day campaign targets the general public and policy makers and anyone is welcome to join: patients and their families, patient organisations, health professionals, researchers, drug developers, public health authorities - the more, the better!

## How?

Since Rare Disease Day was created by EURORDIS in 2008, thousands of awareness-raising activities have taken place throughout the world reaching hundreds of thousands of people and generating local and international media attention.

We hope many more will continue to join us!



[rarediseaseday.org](http://rarediseaseday.org)

# INTERNATIONAL PEMPHIGUS & PEMPHIGOID FOUNDATION

## 2016 YEAR IN REVIEW

### IPPF PATIENT ADVOCATES



made **32**  
congressional  
visits



**13** SUPPORT GROUP  
meetings in **6** CITIES

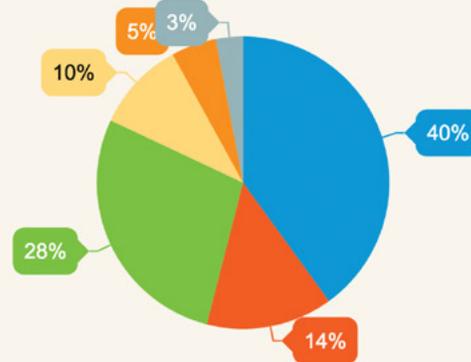
**IPPF signed onto 7 advocacy letters  
and helped to pass the 21st Century Cures Act**



### PATIENT EDUCATION SERIES

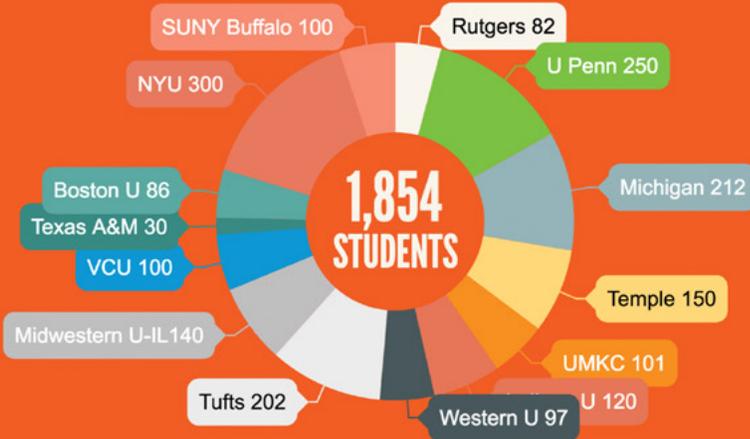
hosted **7** calls  
with **761** attendees

### 768 PATIENT/CAREGIVER CASES CLOSED



■ Physician Referral (40%) ■ Treatment Options (14%) ■ Peer Support (28%)  
■ General (10%) ■ Symptoms (5%) ■ Feedback (3%)

**PATIENT EDUCATORS** presented at  
**14 DENTAL SCHOOLS** reaching...



Reached an estimated **3,740**  
dental professionals through  
in-person events:

**5** dental exhibits

**9** continuing education  
course presentations

**34 Articles Published on**



pempress

**2016 PATIENT CONFERENCE**  
**AUSTIN, TX**  
**137 attendees**

19<sup>th</sup> ANNUAL PEMPHIGUS & PEMPHIGOID  
PATIENT CONFERENCE





Former U.S. Representative Charles Rangel (NY) and Debra Levinson

# Where Patients Have a Voice

NO EXPERIENCE REQUIRED

Debra A. Levinson

February 2013:  
Pre-Patient Status

**M**y foray into advocacy was at legislative boot camp on behalf of my profession as a court reporter. Shootings had been reported monthly in rural and suburban courtrooms since 2010, and court reporters representing their state associations completed basic training in lobbying to seek support of critical legislation that would protect them on the job.

The Local Courthouse Safety Act of 2013 provided metal detectors, wands, and baggage screening equipment the federal government no longer used. It ensured

protection not only for court personnel, but for all Americans who use the services provided at courthouses.

For me, that was the beginning of a new interest in government and policy change—and reading the *Washington Post* daily. It felt good to get involved, adding my voice to the process.

February 2016:  
Washington, DC, Rare Disease Day

A different advocacy opportunity presented itself when I was diagnosed with mucous membrane pemphigoid (MMP) and became a rare disease patient. At an IPPF support group meeting in November 2015, I met other pemphigoid and pemphigus patients. There I learned of upcoming legislative training for Rare Disease Day. “The chance to promote disease awareness and lobby for treatment and research?” I thought. “Where do I sign up?”

In Washington, DC the following February, I learned about the challenges and unmet needs of a staggering number of rare disease patients. The 21st Century Cures Act was significant legislation that could improve over 7,000 lives by providing access to better healthcare.

My fellow advocates and I attended biomedical lectures at the National Institutes of Health (NIH), my favorite organization, and all-day advocacy sessions, culminating in our collective voice being heard on the Hill. It was energizing and exhausting—and we kept up the pace. There were meetings in the Hart Building followed by meetings in the Longworth House Building half a mile away.

There were many sidebar highlights, from shaking hands with Senator Charles Schumer (and posing for a photo op with our New York contingency) to taking a selfie in an elevator with former New York Congressman Charlie Rangel—a day in the life of an advocate!

Government Lobbying:  
Same Concept, Different Hat

Regardless of the ask, advocacy may also be healthful; it can positively influence the quality of life of many patients suffering from chronic disease. The shift in

mood is palpable just walking the marble halls of Congress. Recent research supports this theory. See this article from the NIH that summarizes recent research findings focusing on links between social support and physical health: [www.ncbi.nlm.nih.gov/pmc/articles/PMC2729718](http://www.ncbi.nlm.nih.gov/pmc/articles/PMC2729718).

I recommend becoming an advocate. It will make a difference in your life and those of rare disease patients worldwide. Save the Rare Disease Week dates: February 27 to March 2, 2017!

### November 2016: Greater New York Dental Meeting

This past November, I advocated for disease awareness at the 92nd Greater New York Dental Meeting. Exhibitors at this giant event promoted the latest technology in operatory equipment, digital restorations, and practice management systems from manufacturers all over the world.

The Jacob K. Javits Convention Center hosted over 1,500 exhibitors—whose booths ranged from simple design to exceedingly elaborate—competing for the attention of nearly 55,000 attendees.

This event is the premier buying location for the domestic and international dental communities.

I recommend becoming an advocate. It will make a difference in your life and those of rare disease patients worldwide.

The IPPF's assigned booth was off to the side, tucked in the back of the hall. But we had great display posters and informational brochures. Best of all, it had us, the volunteer patients. We shared our stories for nearly eight hours a day. It was quality—and quantity—disease awareness time!

This was a huge opportunity to educate dental professionals about the existence of our diseases. "Do you know about pemphigus and pemphigoid?" We must have said that hundreds of times. Some attendees kept walking, shaking their heads. We weren't always sure what they were gesturing. But relentless we were. If



(From left to right) Miriam Weinberg and Debra Levinson volunteering at the Greater New York Dental Meeting with IPPF Outreach Manager Becky Strong.

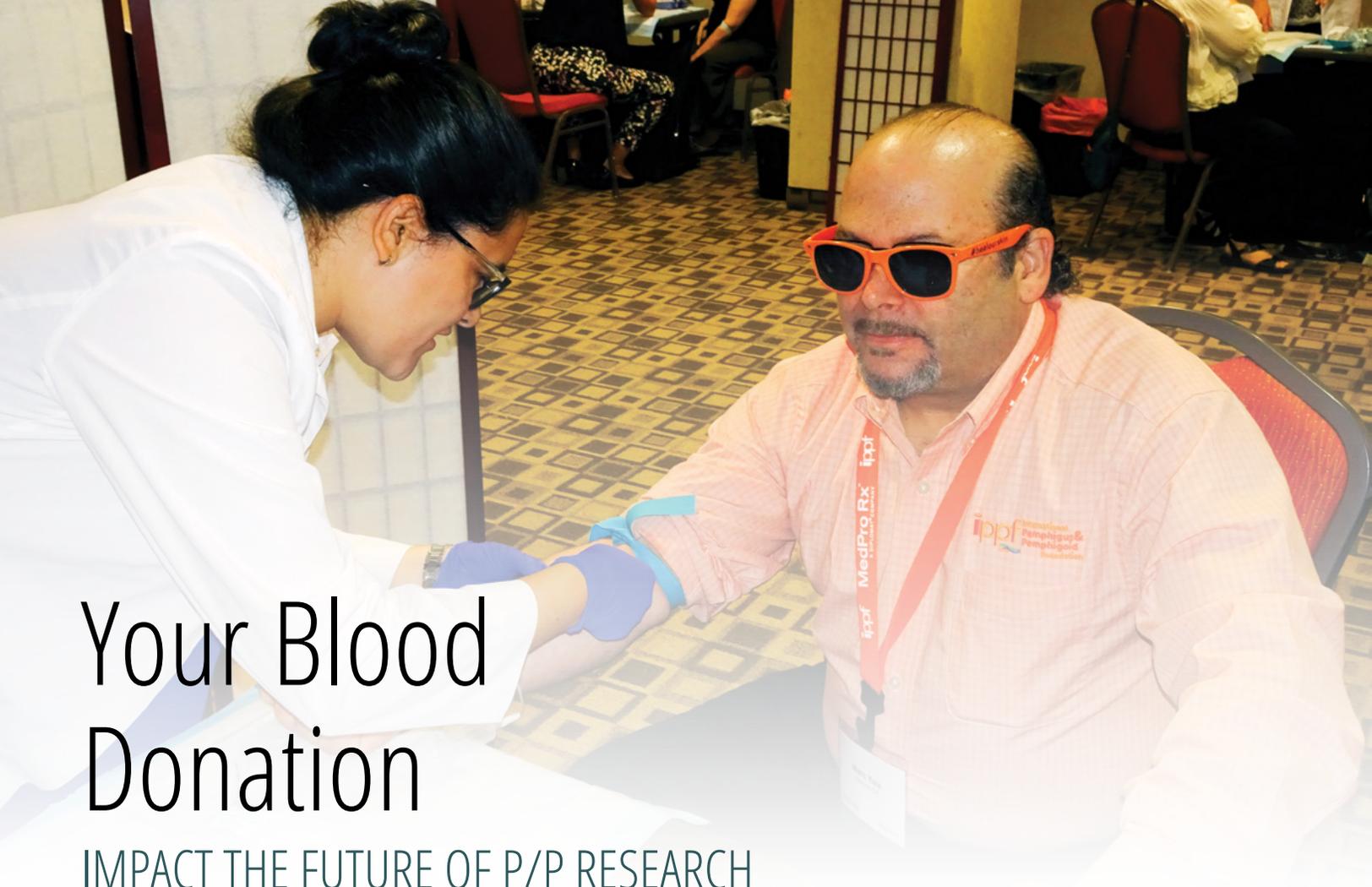
they kept walking, we'd follow up with, "The IPPF's services are free!" Then, some who weren't inclined to stop, did.

We conveyed short, personal stories and created relevant conversations. Many dental professionals had little to no hands-on experience with pemphigus and pemphigoid, having just read about the diseases in textbooks. We stressed the importance of early diagnosis, shared the IPPF's comprehensive resources, and added many new contacts to the IPPF database. Those who completed our iPad survey received the iconic #healourskin orange sunglasses.

Advocacy is not effortless, nor is it anxiety-free. I still doubt my ability to communicate the proper "elevator pitch" effectively. I am a court reporter, used to memorializing the words of others for over 40 years. Now I'm using my own words as a patient advocate, and with no prior experience.



Debra A. Levinson is an MMP patient, diagnosed in 2015. She lives in New York with her husband, Bruce.



# Your Blood Donation

IMPACT THE FUTURE OF P/P RESEARCH

**Kristina Seiffert-Sinha, MD**

**Tremendous advances** have been made over the past decades both in understanding as well as treating pemphigus and pemphigoid (P/P). Once considered a group of poorly understood and often fatal blistering conditions of the skin and the mucosal surfaces, pemphigus and pemphigoid have now been stratified into numerous well-defined entities.

While proper and timely diagnosis remains a challenge, a number of therapeutic options now exist. However, current therapies are often associated with unpleasant and detrimental side effects. Moreover, the cycling between disease remission and flares follows an unpredictable pattern in individual patients. Clearly, the "holy grail" of targeted, safe, and individualized therapies for P/P has not been reached yet. Much more work is needed in terms of understanding disease mechanisms and developing the next generation of treatment strategies in order to improve the lives of people affected by these blistering conditions.

Scientists use many different models to investigate

disease in order to learn about their underlying causes, far too many to list here. A few examples within P/P research would include:

- *questionnaires* to address clinical features of disease, e.g. how common is disease in general; are there any populations preferentially affected; on which body surfaces does disease typically present, among others
- *tissue culture models* in which human or animal cells are grown in the laboratory to investigate the role of various components of the immune system and effect of autoantibodies on cell-cell adhesion and downstream signaling mechanisms within individual cells
- *animal models* to test the effects of immune cells and autoantibodies and/or therapies; these effects are often tested on mice to see how an intact organism responds

While many of these experimental approaches have brought deep insights into disease manifestation and

mechanisms, they are not always applicable to what is happening to you as a patient. Both tissue culture and animal models can be biased, and what works in a culture dish or in a mouse will not necessarily be applicable to humans.

That is why it has been so important to have actual patients participate in research. Through the donation of your blood and other tissues, researchers can get a glimpse of what is happening to an individual on a given day, and then try to correlate this information with carefully curated information about your disease activity, other conditions you may have or that run in your family, medication use, or events that may have triggered your current flare.

Using this invaluable resource—your blood collected in large parts at the yearly IPPF meetings—the research team led by Dr. Animesh A. Sinha at the University at Buffalo and previous institutions has published many studies over the past nearly two decades that shed light on disease mechanisms and presentation (see sidebar, references 1-14) and add to the ever growing mosaic that represents the clinical, genetic, and immunological underpinnings of pemphigus & pemphigoid disease.

However, a major challenge to P/P research is that these conditions are exceedingly rare. Newer scientific techniques that specifically assess genetic markers and immune alterations require large numbers of participants, often in the range of hundreds to thousands of patients. This fact makes it even more important to have a long-term commitment to collecting patient samples and a patient population willing to share their blood and information.

The road ahead of us, both for researchers and patients, is still long, but we can walk it together in the hope of improving our understanding of disease and being able to ultimately find a way to better the lives of all affected. Thank you, and please participate—today!

Contact us:

Kristina Seiffert-Sinha, MD  
Department of Dermatology, Univ. at Buffalo  
[krs2002@buffalo.edu](mailto:krs2002@buffalo.edu)

## REFERENCES

- 1: Senger P, Abidi N, Lin DM, Seiffert-Sinha K, Sinha AA. Positive correlation of anti-herpes simplex type I virus antibody levels with pemphigus vulgaris disease status and activity in a large patient cohort. *Eur J Dermatol*, in press
- 2: Shah AA, Dey-Rao R, Seiffert-Sinha K, Sinha AA. Increased oxidative stress in pemphigus vulgaris is related to disease activity and HLA-association. *Autoimmunity*. 2016 Jun;49(4):248-57
- 3: Sajda T, Hazelton J, Patel M, Seiffert-Sinha K, Steinman L, Robinson W, Haab BB, Sinha AA. Multiplexed autoantigen microarrays identify HLA as a key driver of anti-desmoglein and -non-desmoglein reactivities in pemphigus. *Proc Natl Acad Sci U S A*. 2016 Feb 16;113(7)
- 4: Naseer SY, Gill L, Shah J, Sinha AA. Gender-based variability in disease presentation in pemphigus vulgaris. *J Drugs Dermatol*. 2014 Oct;13(10):1225-30
- 5: Naseer SY, Seiffert-Sinha K, Sinha AA. Detailed profiling of anti-desmoglein autoantibodies identifies anti-Dsg1 reactivity as a key driver of disease activity and clinical expression in pemphigus vulgaris. *Autoimmunity*. 2015 Jun;48(4):231-41
- 6: Parameswaran A, Attwood K, Sato R, Seiffert-Sinha K, Sinha AA. Identification of a new disease cluster of pemphigus vulgaris with autoimmune thyroid disease, rheumatoid arthritis, and type I diabetes. *Br J Dermatol*. 2015 Mar;172(3):729-38
- 7: Dey-Rao R, Seiffert-Sinha K, Sinha AA. Genome-wide expression analysis suggests unique disease-promoting and disease-preventing signatures in Pemphigus vulgaris. *Genes Immun*. 2013 Dec;14(8):487-99
- 8: Shah AA, Seiffert-Sinha K, Sirois D, Werth VP, Rengarajan B, Zrnchik W, Attwood K, Sinha AA. Development of a disease registry for autoimmune bullous diseases: initial analysis of the pemphigus vulgaris subset. *Acta Dermatol Venereol*. 2015 Jan;95(1):86-90
- 9: Bhanusali DG, Sachdev A, Rahmanian A, Gerlach JA, Tong JC, Seiffert-Sinha K, Sinha AA. HLA-E\*0103X is associated with susceptibility to pemphigus vulgaris. *Exp Dermatol*. 2013 Feb;22(2):108-12
- 10: Dhandha MM, Seiffert-Sinha K, Sinha AA. Specific immunoglobulin isotypes correlate with disease activity, morphology, duration and HLA association in pemphigus vulgaris. *Autoimmunity*. 2012 Nov;45(7):516-26
- 11: Gupta VK, Kelbel TE, Nguyen D, Melonakos KC, Murrell DF, Xie Y, Mullard A, Reed PL, Seiffert-Sinha K, Sinha AA. A globally available internet-based patient survey of pemphigus vulgaris: epidemiology and disease characteristics. *Dermatol Clin*. 2011 Jul;29(3):393-404
- 12: Sachdev A, Bhanusali DG, Patterson KC, Zamora MB, Ghuman A, Gerlach JA, Sinha AA. PTPN22 1858T is not a risk factor for North American pemphigus vulgaris. *Exp Dermatol*. 2011 Jun;20(6):514-9
- 13: Lee E, Lendas KA, Chow S, Pirani Y, Gordon D, Dionisio R, Nguyen D, Spizuoco A, Fotino M, Zhang Y, Sinha AA. Disease relevant HLA class II allele isolated by genotypic, haplotypic, and sequence analysis in North American Caucasians with pemphigus vulgaris. *Hum Immunol*. 2006 Jan-Feb;67(1-2):125-39
- 14: Rizzo C, Fotino M, Zhang Y, Chow S, Spizuoco A, Sinha AA. Direct characterization of human T cells in pemphigus vulgaris reveals elevated autoantigen-specific Th2 activity in association with active disease. *Clin Exp Dermatol*. 2005 Sep;30(5):535-40

# Why The Bertilimumab Bullous Pemphigoid Trial Is So Important

Daniel Teper, PharmD, MBA

## **B**ullous Pemphigoid

**(BP)** is an autoimmune blistering disease of the skin that affects 30,000 Americans and an estimated 90,000 people worldwide (one-third with moderate to severe disease). Most of the patients are over 60 years old, require several visits before being diagnosed, and have limited therapeutic alternatives. The mainstay of treatment is oral prednisone at high doses for several months with risk of relapse when steroids are tapered down or discontinued. Europeans also use topical steroids on large areas of the body. The chronic use of steroids is responsible for a worsening of multiple conditions, including osteoporosis, bone fractures, severe infections, and cardio-metabolic diseases. As a result, hospitalization is frequent and mortality is double that of people the same age without the disease.

Most patients with BP have high levels of eosinophils, white blood cells that secrete pro-inflammatory substances in the blood stream and locally in the skin. Eosinophil migration and activation is regulated by eotaxin-1, a protein targeted by Immune Pharmaceuticals' first-in-class monoclonal antibody, bertilimumab. This is the reason why the International Pemphigus and Pemphigoid Foundation (IPPF) approached Immune and connected the company with leading physicians treating BP.

Immune formed a Scientific Advisory Board and



IMMUNE  
Pharmaceuticals

designed a phase II open label trial with the following goals:

- assessing the safety of multiple doses of intravenous (IV) bertilimumab administered every two weeks for one month
- evaluating the clinical response to bertilimumab on a background of 30mg of oral prednisone tapered down as the patient shows symptom improvement
- evaluating the number of patients successfully tapered down to 10mg of oral prednisone or less without short term disease relapse

The Bullous Pemphigoid Disease Activity Index (BPDAI) is a comprehensive score that has been validated by leading dermatologists. A score between 50 and 70 is high and reflects moderate to severe disease. A score below 10 suggests the patient is achieving a meaningful response with no or very limited symptoms.

After a delayed initiation and an assessment of the first few patients to complete treatment, patient recruitment is accelerating in Israel (two centers) and now in the United States (six centers). This is encouraging, as a meaningful clinical response in a majority of

the approximately 10 planned patients would support further development of bertilimumab in BP with additional trials looking not only at induction treatment, but also at maintenance and ability to control the disease with bertilimumab on a background of a minimal

Bertilimumab is finally approaching the limelight and creating hope for patients with severe inflammatory diseases.

dose of prednisone, as in many other autoimmune and inflammatory diseases.

Such an outcome would be game changing for BP patients and would create

an opportunity for Immune Pharma estimated at a value globally of \$500 million to over \$1 billion. This would also be the first proof of clinical relevance for

bertilimumab and further support development in other dermatological indications such as atopic dermatitis which affects over one million adult Americans. Development in Ulcerative Colitis (UC) and Crohn's Disease is ongoing with a phase II double blind placebo controlled UC trial which is also accelerating patient recruitment toward completion. Other indications, including NASH, a severe inflammation of the liver, are supported by the over 1,500 publications on eotaxin-1, and will be considered for further development in 2017 and beyond.

Bertilimumab, which was discovered by Cambridge Antibody Technologies, the originator of Abbvie's blockbuster Humira, is finally approaching the limelight and creating hope for patients with severe inflammatory diseases.

.....  
*Daniel Teper, PharmD, MBA, is the CEO at Immune Pharmaceuticals Inc. For more information, visit [www.immunepharma.com/](http://www.immunepharma.com/)*

WIRB 20142233  
#13046405.0

# PEMPHIX

## Pemphigus Vulgaris Clinical Research Study - NOW ENROLLING

**If you have been diagnosed with pemphigus vulgaris within the previous 24 months and are 18-75 years old, you might be eligible for a research study testing two different investigational medications to see if one is better than the other and is safe in treating pemphigus vulgaris.**

For more information, please see [ClinicalTrials.gov](http://ClinicalTrials.gov) (NCT02383589)

*If you are interested and would like more information, please call Genentech's Trial Information Support Line at 1-888-662-6728 (6:00 AM – 3:00 PM PST), or see [ClinicalTrials.gov](http://ClinicalTrials.gov)*



## TRUE NORTH THERAPEUTICS

At True North Therapeutics, we are guided by the needs of patients as we discover life-changing medicines for rare diseases. We combine scientific and clinical expertise in diseases of the Complement system so that we can rapidly develop new drugs for rare diseases with few, if any, treatment options. We concentrate on therapies that can intervene upstream in the Complement system, selectively targeting the pathway underlying the disease. Our most advanced product candidate, TNT009, is a first-in-class, anti-C1s antibody that selectively targets the Classical pathway of the Complement system. This novel antibody is currently in clinical development for several rare diseases, including bullous pemphigoid, in a Phase 1b safety/tolerability study. True North Therapeutics is located in South San Francisco, California.

For more information, please visit [www.truenorthrx.com](http://www.truenorthrx.com).



## Have a Support 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 share your experiences and get the support you need. To find others in your area, contact Becky Strong: [becky@pemphigus.org](mailto:becky@pemphigus.org).

Here's the Southern California Support Group at a meeting in Santa Monica, CA!

# Natural History Study

**The IPPF** will soon be launching the largest-ever study of pemphigus and pemphigoid. "The IPPF Natural History Registry will provide a complete picture of each patient's experience with pemphigus or pemphigoid," said Marc Yale, Interim Executive Director of the IPPF. "We are launching this initiative to collect missing information researchers and medical experts need to advance research and find to a cure."

To help drive awareness and participation, the IPPF will provide information about the registry via email, advertisements, flyers, its Quarterly journal, and at its annual patient meeting to members of the pemphigus and pemphigoid community.

"Our goal is to enroll as many patients, or their parents or legal guardians, as possible," said Yale. "The success of the registry is dependent upon community participation."

The IPPF Natural History Registry is a natural history study that consists of electronic surveys to collect information about the patient experience and disease progression. Patients, or their caregivers or guardians, can enter information from anywhere in the world. The data is made anonymous and stored securely in an online portal called a registry. The IPPF may share the data, but not your personal identifying information, with individuals or institutions conducting

research or clinical trials, as approved by the study's governing board that includes scientists, doctors, and patient advocates.

The IPPF is launching the study in collaboration with the National Organization for Rare Disorders (NORD), an independent charity that built its natural history study platform as part of its mission to help identify and treat all 7,000 rare diseases. Funding is supported by a cooperative agreement between NORD and the U.S. Food and Drug Administration (FDA). The FDA has praised NORD's program as a helpful tool "that protects the security and privacy of personal information, while making valuable information available to a researcher or drug developer interested in creating a new therapy for a rare disease."

NORD President and CEO Peter L. Saltonstall said, "NORD's natural history studies platform empowers patients and families to drive research and eliminate some of the unknowns that still exist in rare diseases. We are glad to be working with our Member Organization on this project and thank the FDA for its support and ongoing commitment to help people with rare diseases."

More information will be available at [pemphigus.org](http://pemphigus.org) soon.



International Pemphigus & Pemphigoid Foundation  
 1331 Garden Highway #100 • Sacramento, CA 95833  
 855-4PEMPHIGUS • 916-922-1298 • Fax: 916-922-1458  
[info@pemphigus.org](mailto:info@pemphigus.org) | [pemphigus.org](http://pemphigus.org)

Nonprofit Org.  
 US Postage  
 PAID  
 TUCSON, AZ  
 Permit No. 271

RETURN SERVICE REQUESTED



UNIVERSITÄTSKLINIKUM  
 Schleswig-Holstein

# IPPF Scientific Meeting

LÜBECK, GERMANY  
 JUNE 21-23, 2017

The IPPF is pleased to announce this international meeting of clinical and scientific experts in the field of autoimmune bullous disorders.

More information available at [pemphigus.org](http://pemphigus.org)

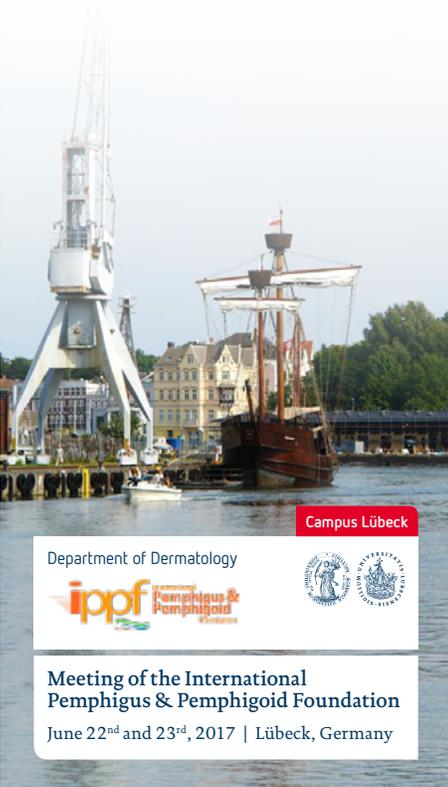
Supported by:



CLINICAL RESEARCH UNIT 303  
 Pemphigoid Diseases - Molecular Pathways  
 and their Therapeutic Potential



Titelbild: Die Kremwellen von Lübeck. Schenke/Ina/ingegre Kommunikation, G. Weinberger, Stand August 2016



Quarterly

Journal of the International Pemphigus & Pemphigoid Foundation