

RITUXIMAB & CLINICAL TRIALS | ADVOCATING FOR DENTAL RESEARCH | SURVEYS & STUDIES

Quarterly

Journal of the International Pemphigus & Pemphigoid Foundation

**Research &
Advocacy
Issue**

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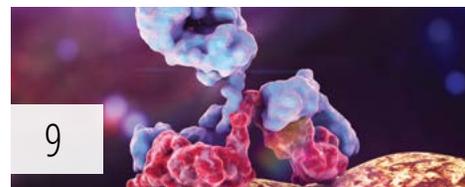
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Message from the Executive Director



Welcome to the second annual advocacy and research edition of the *Quarterly*! The IPPF has certainly come a long way since last year. Thanks to your support, our advocacy and research efforts have as well.

This issue includes inspiring stories from Kevin Kreutner (North Carolina) and Iva Rauh (Connecticut), who advocated with the IPPF at Rare Disease Week on Capitol Hill last year. In 2017, the IPPF visited over 60 congressional offices while building a community of passionate advocates like Kevin and Iva. We also signed onto 20 letters regarding issues that are critical to our community, and we joined the Friends of the National Institute of Dental and Craniofacial Research Patient Advocacy Council.

I am excited to share that our advocacy efforts will continue to grow in 2018. The IPPF will continue to develop relationships with congressional representatives and other support organizations in order to advocate for favorable state and federal legislation, research funding, and policies that benefit our community. Similar to past years, IPPF advocates will storm our nation's capital the last week of February for Rare Disease Week to make sure all our voices are heard.

In 2017, the IPPF also worked hard to promote pemphigus and pemphigoid research. Some highlights include holding a scientific conference in Lübeck, Germany, with over 225 attendees from 13 different countries; funding seven new disease research projects; and collaborating on multiple surveys to gather crucial information about treatments, quality of life issues, and unmet medical needs. The IPPF community is fortunate to have expert clinicians, researchers, and scientists from around the world striving to understand the mechanisms that cause these diseases, develop better therapies, and address the need for more clinical trials. Collectively, we understand that **no disease is too rare for a cure.**

In 2018, the IPPF will continue to encourage the research of pemphigus and pemphigoid and provide the most current information to patients by collaborating with stakeholders that both treat these diseases and search for a cure. **The IPPF team is honored to support you!**

We hope that you enjoy this issue of the *Quarterly* and reading about our collective accomplishments and plans. Whether we are supporting research, advocating for better policy related to patient care, or educating the medical community, **our work is designed to improve the lives of all people affected by pemphigus and pemphigoid.**

Gratefully,

Marc Yale
IPPF Executive Director and MMP Patient

marc@pemphigus.org



BANDING TOGETHER

New Awareness Ambassador Activity

SPREADING AWARENESS ON FACEBOOK



If you are looking for an easy way to spread awareness of pemphigus and pemphigoid, look no further! The IPPF's newest Awareness Ambassador activity can be done without even leaving your home.

The IPPF is looking for volunteers to share P/P messages on Facebook. We'll provide training and sample messages you can use. The training is online, self-paced, and available

to all registered Ambassadors. It's been released prior to Rare Disease Day and FNIDCR/AADR Advocacy Day (see article on page 20 for more details) to prepare Ambassadors for sharing research and advocacy messages.

Ambassadors are volunteers who spread awareness of pemphigus and pemphigoid locally. At various times throughout the year, the IPPF will release new activities. Our first

activity trained Ambassadors on how to distribute educational materials to local dental offices. If you would like to distribute educational materials or participate in the newest Facebook outreach activity, you will first need to become a registered Ambassador. Thank you to all of our Ambassadors for spreading awareness of P/P!

Sign Up Today at www.pemphigus.org/awareness/ambassadors.

IN THE TREATMENT OF PEMPHIGUS VULGARIS

WE'RE LOOKING AT THE BIG PICTURE



At Principia, we believe that patients are part of the solution. They help us connect the dots that reveal the best way to treat autoimmune diseases like Pemphigus Vulgaris. We're dedicated to finding a treatment that combines the convenience of a small-molecule with the focus of an antibody, for titration of efficacy and safety to meet individual patient's needs. To learn more, visit us at our booth, or <http://www.principiabio.com>

PRINCIPIA
BIOPHARMA



New Year Revelations

Terry Wolinsky McDonald, PhD

Many people make New Year's resolutions, but I decided to write about "revelations" this year. At this time last year, many people with serious preexisting conditions were wary of possible negative health care and insurance changes. That wariness has now been replaced with a healthy dose of reality-based fear. What will actually happen? No one knows; however, we do know that all of us are much stronger together than alone. The IPPF, administrative leaders, expert doctors, and patients continue to work together and with other rare disease organizations to make our voices heard. Letters, petitions, and emails to our congressional representatives are weekly, and sometimes daily, activities.

Many are also concerned about whether or not they

will be able to purchase personal health care insurance. Others are struggling to pay increased premiums they cannot afford. As someone who grossly overpaid for group insurance coverage for many years just to make sure any and all of my own preexisting conditions would be covered for expensive, medically necessary treatments, I do not want others to be put in that untenable position. No one asks to be diagnosed with an incurable disease, but not being able to receive necessary medical treatment is criminal. Now I am on Medicare and wondering if and when that could also change. Last year, I was shocked to learn that Medicare approved services for different conditions vary from state to state. How can this be? It makes absolutely no sense, except from a government monetary perspective: I call it the "cents

versus sense” logic. My great hope is that those of us with these lesser known diseases and disorders will never have to fear receiving medically necessary services due to a lack of wealth or connections.

The staffs of my senators and representatives (both local and national) know who I am because I have never shied away from visiting their offices, calling, emailing, and working behind the scenes to both educate and help others who may be suffering. Please know that your elected officials can often help you receive necessary care and treatments that even your doctors may have trouble getting authorized by insurance companies. I even contacted my state governor once (and received an unexpected positive result), when my doctors were unable to get necessary authorizations, which were held up for more than a year. The sad part is that this is not unusual and is actually standard operating procedure. The good news is that if a patient can get politicians involved, there is an excellent chance that negative insurance decisions may be reversed. However, there are no guarantees.

Because pemphigus vulgaris is not my only autoimmune disease (as with many of you), I am challenged

every day. Facing adversity head on has made me stronger and more resilient. Are there days that seem too challenging? Yes, of course—there always will be. Are there days when I feel so exhausted that I am not sure how I will get through them? Again, yes. However, it helps me to know that my positive and goal-oriented behaviors help and encourage others. So does watching others whom I look up to. The friendships, bonds, and kinships formed along the way will always be there. We all support each other as we hope for improved treatments and cures.

As I write this article, I am not focusing on New Year’s resolutions, but on what I have learned about these illnesses over the past 17 years. Much of that learning has been through the kindness, generosity, and friendship of so many. Yes, it takes a community, and we have that community.

.....
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 in her “Psychologically Speaking” column.



2018 IPPF Pre-International Investigative Dermatology Satellite Meeting

Pemphigus and Pemphigoid: A New Era of Translational and Clinical Science

May 15-16, 2018

Rosen Shingle Creek Resort
Orlando, FL

Register at www.pemphigus.org

Unmet Needs in Pemphigoid Diseases:

A SURVEY AMONG PATIENTS, CLINICIANS, & RESEARCHERS

Aniek Lamberts, MD

All who have been professionally or personally involved with pemphigoid know the great influence this disease can have on a patient's life. As for me, I first came in contact with autoimmune blistering diseases during my medical training. After graduating, I was given the great opportunity to start a PhD on pemphigoid diseases.

Over the last few years there has been a considerable increase in knowledge on pemphigoid. For example, laboratory tests for diagnosis have improved, and

By listing the most urgent unmet needs, we aim to highlight topics on which future research can focus.

there is more information on the disease's mechanism. Therefore, new drugs can be developed. On the other hand, many uncertainties, points of improvement in patient care, and gaps in knowledge still exist. This gave us the idea of exploring and prioritizing these so called "unmet needs."

In collaboration with the IPPF and a small panel of international experts on pemphigoid diseases, a survey was developed and spread amongst patients, clinicians, and researchers. In this survey, we provided participants with a list of unmet needs and asked them to pick the top three needs that they think are most important. Patients were asked about their opinion

on delay in diagnosis, awareness amongst caretakers, self-assessment tools, treatment options, availability of information, guidance, health impact, and treatment accessibility. Clinicians were asked about unmet needs concerning disease recognition, availability of diagnostic tests, accessibility of new drugs, and the need for multidisciplinary teams. Researchers were asked for their opinions on gaps in knowledge regarding disease mechanisms, drug development, and the need for more clinical trials.

By listing the most urgent unmet needs, we aim to highlight topics on which future research can focus. In our project, patients can express which high priority topics should deserve the most attention. By sharing thoughts and reflecting on current care, patients, clinicians, and researchers can learn from each other and together will be able to make important improvements in patient care.

The international experts working together on this project are: M.F. Jonkman, MD; D. Zillikens, MD; S.A. Grando, MD; B. Horváth, MD; and Marc Yale.

Aniek Lamberts, MD, graduated medical school in December 2014. She worked for the Dutch Society for Dermatology and Venereology on the development of national dermatology guidelines. She is currently a PhD candidate studying pemphigoid diseases at the University Medical Center Groningen in the Netherlands.

Rituximab and Clinical Trials

In 2017, the United States FDA granted Breakthrough Therapy Designation status for the use of Rituxan® (rituximab) to treat pemphigus vulgaris based on the results from an investigator-sponsored study. This study was independently organized by Professor Pascal Joly, and Roche (pharmaceutical company) provided the rituximab. This multicenter, open-label, randomized controlled study in newly-diagnosed pemphigus patients demonstrated that rituximab in combination with short-term, low-dose prednisone treatment provided substantial improvement in complete remission rates and the successful tapering of prednisone therapy

when compared to standard, long-term prednisone treatment of pemphigus patients.

Breakthrough Therapy Designation allowed Roche to discuss an accelerated regulatory pathway for approval with the FDA that would enable patients to receive access to rituximab therapy as soon as possible.

Roche collaborated with Professor Joly and his institution to independently analyze the data collected from this study and prepared a formal submission to the FDA to seek approval for rituximab use in moderate to severe cases of pemphigus vulgaris.

Current Trial Highlights

Inclusion does not imply endorsement by the IPPF. For more information on these and other clinical trials, visit <http://clinicaltrials.gov>

Syntimmune

Title: A Safety Study of SYNT001 in Subjects with Pemphigus (Vulgaris or Foliaceus)

Estimated Primary

Completion: January 2018

Estimated Study

Completion: March 2018

Disease Type: Pemphigus

Locations: Palo Alto, California: Clinical and Translational Research Unit, Stanford University; Atlanta, Georgia: Emory University School of Medicine; Buffalo, New York: University at Buffalo, Clinical and Translational Research Center; Chapel Hill, North Carolina: University of North Carolina; Durham, North Carolina: Duke University Medical Center; Cleveland, Ohio: University Hospitals Cleveland Medical Center; Philadelphia, Pennsylvania: University of Pennsylvania Perelman Center for Advanced Medicine

Principia BioPharma, Inc.

Title: A Study of PRN1008 in Adult Patients With Pemphigus Vulgaris

Estimated Primary

Completion: December 2019

Estimated Study

Completion: December 2019

Disease Type: Pemphigus

Locations: Osijek, Croatia: Clinical Hospital Osijek; Bobigny, Siene-Saint Denis, France: Hôpital Avicenne; Rouen, France: Rouen University Hospital; Ioánnina, Ioannina: Greece: University General Hospital of Ioannina; Larissa, Thessaly, Greece: University Hospital of Larissa; Athens, Greece: Hospital of Venereal and Skin Diseases A.Syggros; Thessaloniki, Greece: Papageorgiou General Hospital of Thessaloniki; Ramat-Gan, Israel: Chaim Sheba Medical Center; Tel Aviv, Israel: Tel Aviv Sourasky Medical Center

Immune Pharmaceuticals

Title: Evaluation of Safety, Efficacy and Pharmacodynamic Effect of Bertilimumab in Patients With Bullous Pemphigoid

Estimated Primary

Completion: April 2018

Estimated Study

Completion: July 2018

Disease Type: Pemphigoid

Locations: Iowa City, Iowa: University of Iowa; Buffalo, New York: University at Buffalo; New York, New York: Icahn School of Medicine at Mount Sinai; Durham, North Carolina: Duke University Medical Center; Cleveland, Ohio: University of Cleveland; Salt Lake City, Utah: University of Utah; Ramat Gan, Israel: Chaim Sheba MC, Tel-Hashomer; Tel Aviv, Israel: Sourasky-Ichilov Tel Aviv Medical Center

Bioverativ

Title: Safety, Tolerability and Activity of TNT009 in Healthy Volunteers and Patients With Complement Mediated Disorders (TNT009-01)

Estimated Primary

Completion: June 17, 2018

Estimated Study

Completion: October 2018

Disease Type: Pemphigoid

Location: Vienna, Austria: Medical University of Vienna

New Study Seeks to Improve Treatment of Pemphigus Vulgaris



Dr. Nicole Fett



Dr. Brittney Schultz

Brittney Schultz, MD

Pemphigus vulgaris (PV) is tough. And for those patients living with the pain and challenges of PV on a daily basis, we know we don't have to tell you twice. We are two dermatologists practicing at different institutions across the country working together towards the same goal: improving the treatment of PV. As dermatologists, we are always looking for the best way to help our patients while providing treatment options with the most benefit and the least harm .

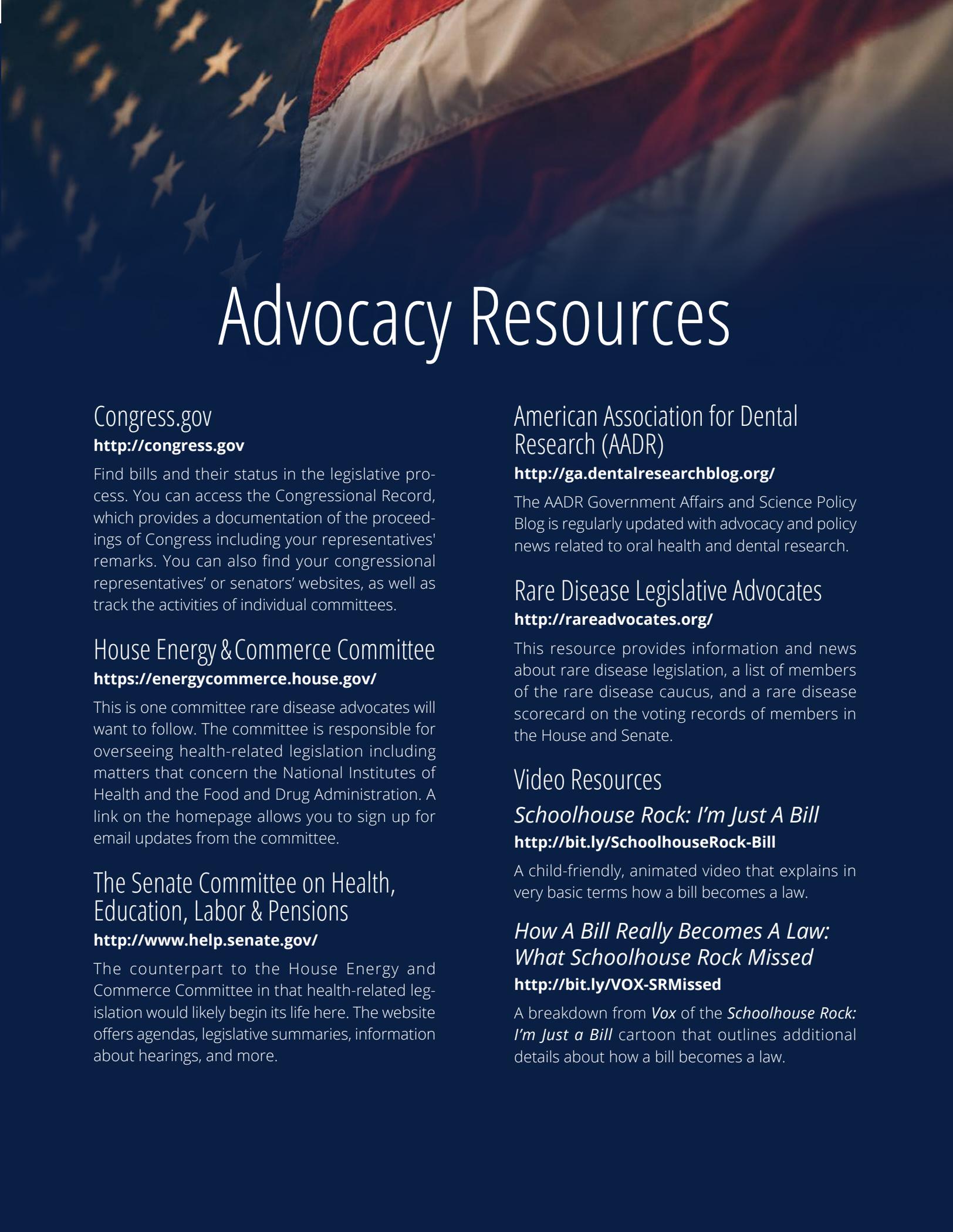
Dr. Nicole Fett is an Associate Professor of Dermatology currently practicing at Oregon Health and Science University (OHSU). She completed her education at the University of Wisconsin after which she joined the University of Pennsylvania for several years before joining the faculty at OHSU. She is board certified in both internal medicine and dermatology and has interests in complex medical dermatology, including autoimmune and blistering diseases.

Dr. Brittney Schultz is currently in her fourth year of a five-year combined internal medicine-dermatology

residency program at the University of Minnesota. She is a lifelong Minnesota native and has completed all of her training to date at the University of Minnesota. Additionally, she has interests in complex medical dermatology and hopes to care for patients with blistering diseases in her future practice.

Together, we are working on a study designed to help us understand the effects that various treatment regimens for PV have had on quality of life. The goal of our study is to determine if certain treatment options have more benefit and less harm than other treatment options. If you have PV, please consider participating in our survey. Your responses will be completely anonymous and you may refuse to answer any of the questions you do not wish to answer. We appreciate your involvement and hope we can continue working towards the goal of improving treatment of PV.

(Principal Investigator, Dr. Nicole Fett- OHSU IRB# 17670)



Advocacy Resources

Congress.gov

<http://congress.gov>

Find bills and their status in the legislative process. You can access the Congressional Record, which provides a documentation of the proceedings of Congress including your representatives' remarks. You can also find your congressional representatives' or senators' websites, as well as track the activities of individual committees.

House Energy & Commerce Committee

<https://energycommerce.house.gov/>

This is one committee rare disease advocates will want to follow. The committee is responsible for overseeing health-related legislation including matters that concern the National Institutes of Health and the Food and Drug Administration. A link on the homepage allows you to sign up for email updates from the committee.

The Senate Committee on Health, Education, Labor & Pensions

<http://www.help.senate.gov/>

The counterpart to the House Energy and Commerce Committee in that health-related legislation would likely begin its life here. The website offers agendas, legislative summaries, information about hearings, and more.

American Association for Dental Research (AADR)

<http://ga.dentalresearchblog.org/>

The AADR Government Affairs and Science Policy Blog is regularly updated with advocacy and policy news related to oral health and dental research.

Rare Disease Legislative Advocates

<http://rareadvocates.org/>

This resource provides information and news about rare disease legislation, a list of members of the rare disease caucus, and a rare disease scorecard on the voting records of members in the House and Senate.

Video Resources

Schoolhouse Rock: I'm Just A Bill

<http://bit.ly/SchoolhouseRock-Bill>

A child-friendly, animated video that explains in very basic terms how a bill becomes a law.

How A Bill Really Becomes A Law: What Schoolhouse Rock Missed

<http://bit.ly/VOX-SRMissed>

A breakdown from *Vox* of the *Schoolhouse Rock: I'm Just a Bill* cartoon that outlines additional details about how a bill becomes a law.

INTERNATIONAL PEMPHIGUS & PEMPHIGOID FOUNDATION

2017 YEAR IN REVIEW

IPPF PATIENT ADVOCATES



made over **60**
congressional
visits

signed onto **20** advocacy letters

LAUNCHED HEALING HEROES



recurring giving program

Scientific Meeting in Lübeck, Germany

225 attendees from **13** countries



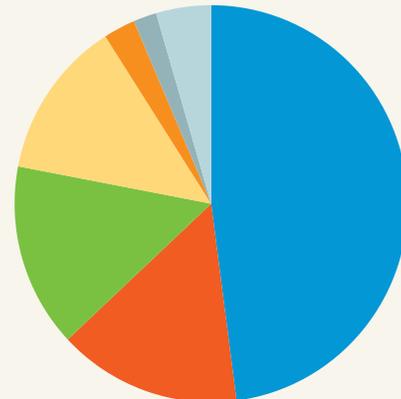
PATIENT EDUCATION SERIES

hosted **8** calls
with **836** attendees

10% increase since 2016

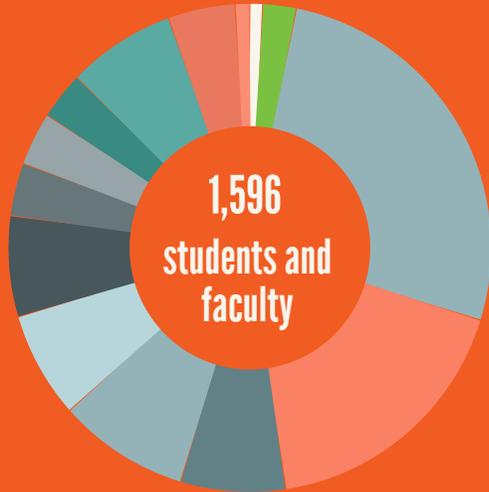
1,252 PATIENT/CAREGIVER CASES CLOSED

63% increase since 2016



■ Physician Referral (47.94%) ■ Treatment Options (15.08%) ■ Peer Support (15%)
■ General (12.94%) ■ Symptoms (2.62%) ■ Feedback (1.90%) ■ Other (4.52%)

PATIENT EDUCATORS presented
16 lectures at 13 schools reaching...



Faculty lectures reached **107** faculty

Interdisciplinary lectures reached **32** healthcare students

Reached an estimated **3,500** dental professionals through in-person events:

8

Dental Exhibits

5

Continuing Education Course Presentations



New professional courses video



Online CE module with Paradigm Medical Communications

LAUNCHED AWARENESS AMBASSADOR PROGRAM



BANDING TOGETHER

34 registered Ambassadors

17 SUPPORT GROUP meetings in **8** CITIES

30% increase since 2016



new groups started in
Buffalo, NY and **Washington, DC**

2017 PATIENT CONFERENCE

Newport Beach, CA

158 attendees



A Rare Disease Advocate's Story

Iva Rauh

For years I was told that I had allergies. The dermatologist became my best friend. My general practitioner gave me injections with herbal supplements to boost my immune system. So when I was diagnosed with pemphigus vulgaris (PV), I was in denial. Thankfully, my daughter is a medical doctor at the University Hospital in Erlangen, Germany. She had the right connections and I got an appointment to see Michael Hertl, MD, at Uniklinik at Philipps University in Marburg, Germany. It was there that the diagnosis was confirmed.

With a preexisting thyroid problem, I could not get health insurance in the United States. I was a self-paying patient in the US, and I flew to Germany for prednisone-induced cataract surgeries, dental treatments, and so on. I was so happy when the Affordable Care Act (ACA) was passed. I no longer had to fly to Europe to visit hospitals.

I lobbied against the repeal of the ACA. For us patients with rare diseases, it is extremely important that we have access to affordable healthcare. I lobbied for research funding. As the mother of an obstetrician-gynecologist and breast cancer research doctor, I realized how many new options are available for women fighting for survival after being diagnosed with cancer, but I cannot understand why 40,000 PV patients don't have a choice of approved treatment specially developed for them.

My son is a microbiologist in a biotech company in Gaithersburg, MD, and I am aware of how expensive and time consuming the development of new drugs are. I am pleased that the Orphan Drug Tax Credit was saved. We rare disease patients expect that the Food

and Drug Administration (FDA) will approve new drugs without delays because the insurance companies refuse the payment for not-yet-approved treatments.

Last year I attended Rare Disease Week on Capitol Hill. During meetings with the staff members of Senator Ben Cardin (D-MD), Representative Andrew Harris (R-MD), and Representative C.A. Ruppertsberger (D-MD), our team had the opportunity to tell our stories, including the challenges we face every day dealing with our rare diseases. Senator Chris van Hollen (D-MD) greeted us in person. He had just joined the Rare Diseases Caucus, and we should be really grateful to have him on board. I didn't have any prior experience dealing with senators and representatives on Capitol Hill. I jumped in the cold water, and Marc Yale and the IPPF gave me the life jacket and the compass I needed to explore these new possibilities.

The opportunity to be a patient advocate during Rare Disease Week on Capitol Hill was not just an honor, but a responsibility to remind senators and representatives that they have been elected to serve the people. I will continue to write emails to Representative Harris and remind him that as a medical doctor and member of the House of Representatives, he could do more for his constituents on the Eastern Shore of Maryland to get the healthcare they need.

I will be on Capitol Hill again during the 2018 Rare Disease Week. I am looking forward to meeting the politicians and some of the amazing people who inspired me to become engaged.

Iva Rauh is a PV patient. She has lived with her husband on the Eastern Shore of Maryland since 2001.



RAREDISEASEDAY.ORG

RARE DISEASE WEEK ON CAPITOL HILL



Rare Disease Week on Capitol Hill brings rare disease community members together to be educated on federal legislative issues, meet other advocates, and share their unique stories with lawmakers. This week-long series of events gives advocates the tools they need to shape healthcare policy to better meet the needs of the rare disease community.

SUNDAY, FEBRUARY 25TH

- 8th Annual Cocktail Reception and Documentary Screening

MONDAY, FEBRUARY 26TH

- Legislative Conference

TUESDAY, FEBRUARY 27TH

- Lobby Day Breakfast
- Lobby Day on Capitol Hill

WEDNESDAY, FEBRUARY 28TH

- Rare Disease Congressional Caucus Briefing
- Rare Artist Reception

THURSDAY, MARCH 1ST

- Rare Disease Day at the National Institutes of Health

ALL EVENTS ARE FREE, BUT ADVANCE REGISTRATION IS REQUIRED.

**FOR MORE INFORMATION, VISIT
WWW.RAREADVOCATES.ORG/RDW**

Finding Community at Rare Disease Week

Kevin Kreutner



Prior to being diagnosed with pemphigus vulgaris (PV), I was fundamentally ignorant about the world of rare diseases. Besides learning about my own condition, things changed considerably last year when I was fortunate enough to attend Rare Disease Week on Capitol Hill with the IPPF. It proved to be an amazing experience for me not only in trying to make a public policy difference, but also in finding community and understanding the challenges faced by those with rare diseases.

At the time, I was a few months post-infusion and about 90 percent back to normal physically. More importantly, I was in the midst of tapering off prednisone. The PV battle had been a rough one. I'm one of those people who doesn't take well mentally to prednisone, but the fog in my head had literally subsided two days before Rare Disease Week. Nonetheless, the experience left me with many of the normal emotions we go through. I had no idea what to expect from the week, but I was looking forward to meeting others with PV, especially Becky Strong, who was my IPPF Peer Health Coach.

My week began with a cocktail party and film showing, which very much suited my beer-drinking style. It was a bit surreal at first. I noticed a lot of well-dressed people who mostly "looked healthy." I'm used to walking around a trade convention staring at name badges to see who someone works for, but somehow that took on a whole different meaning as I read the names of the various rare diseases represented. Suddenly I felt out of my element. I wasn't sure how to talk about it with people. I didn't even know what most of these ailments were. How much would be okay to ask?

After grabbing a beer and some food I sat down at a table occupied by three people. One was an advocate whose sister had a rare disease. The other was a married couple and the gentlemen, who clearly did not "look healthy," was suffering from amyotrophic lateral sclerosis (ALS). He was an inspiration and made me realize that as much as PV sucks, it could be worse. Suddenly I understood why Becky once told me that I'm not a "victim" of PV. The man told me that he thinks he was chosen for ALS because he thought he was better suited to handle it after his experiences in combat. Sadly, he wasn't able to finish all of his activities for the week because he fell at his hotel. I think I subconsciously made sure not to get too many names, connect on Facebook, etc. As I write this today, I realize that the odds are likely that this hero of war and life is not among us. We can't always put a pretty face on reality.

The more formal activities were centered around learning about relevant legislation so that we could gather in small groups based on geography and meet with the staff of our elected officials. It was pretty cool to wander the halls of the Senate and House office buildings. I even set up a makeshift office in the halls

of the Russell Building to do some work while there. For me, the actual meetings were a mixed experience. While I saw the value of them hearing our stories, the realities of political lines had the efficacy in question. In my case, I have two senators that support all the tasks at hand and a US Representative who does not and likely never will.

I also learned an important thing during these meetings. People with rare diseases are just a subset of the population. We had some awesome people in my group. People who had persevered, struggled for a diagnosis, and refused to surrender. Despite that and not related to it, we also had some grandstanders who seemed to want to use their disease to promote themselves personally.

Getting to meet some of the IPPF team and others with our rare disease was amazing for me. I think it's a key part to our healing process and something to do via support groups, the Annual IPPF Patient Conference, or, as was the case here, Rare Disease Week. It helped me see the light of hope, to see that while we may not come out of our first outbreak exactly the same as we went in, we do get through it. At the time, I was just starting to understand that the PV experience goes beyond physical symptoms. As such, the full weight of the experience didn't bear on me until months after.

For me, it's clear as day that the real value in attending Rare Disease Week was community. I realized that we are all part of a community and family. It has its nuclear side with pemphigus and pemphigoid, extending to those with other autoimmune diseases (there are many!), and then goes even farther to the world of all rare diseases or those of us with "preexisting conditions." We share many tangible battles like off-label treatments, getting diagnosed, and modern medicine knowing how to treat the disease but not the person. We also share many battles that go more to the heart and soul. I learned from those with other ailments, and they learned from me. I felt simultaneously lucky and unlucky. But there was a sense that we're all in this together and need to support one another. And that was what Rare Disease Week meant to me.

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Kevin Kreutner is an executive recruiter, proud father of two, youth soccer coach, and self-proclaimed chef. He lives in Salisbury, MD, and was diagnosed with pemphigus vulgaris in August 2016.



Technology & Communication

AN INNOVATIVE SOLUTION

Lisa Ann Krutzik

What if there was an app that enabled pemphigus and pemphigoid (P/P) patients to communicate in real time with doctors? Or at least enabled patients to document day-to-day realities in an electronically transferable format before an appointment so that doctors had real data about daily challenges? What if we were able to provide valuable data to research doctors on a continual basis that could either stop the progress of this disease or alleviate some of the suffering patients may face in the future?

On November 30, 2017 a group of doctors, patients, and app developers from Copenhagen gathered at the University at Buffalo Research Facility. Dr. Animesh Sinha, Dr. Kristina Seiffert, Dr. Alba Posligua, Joe Janca, David Tsujimura, and I met with Kasper Juul, Christian Sejersen, and Aleksander Eiken from LEO Innovation Lab. Although LEO Innovation Lab was

established by LEO Pharma, they are not a part of the pharmaceutical industry. They exist to create electronic solutions for doctors and patients. Their current focus includes skin diseases and providing solutions to some of the most common issues.

The communication system within the app would give patients the ability to provide clear information about their situation . . . to almost every doctor they visit.

This meeting was a brainstorming opportunity that represented both parties and their needs. The following statement was made to us prior to the discussion: “We are here to understand the problems in the doctor/patient journey for the rare disease pemphigus, and to start thinking about solutions to test.” Our ideas are as follows.

- Everyone agreed that it takes patients too long to obtain a correct diagnosis. The diagnostic process makes the patient feel lost, unimportant, and frustrated. For doctors, this typically means that the disease has progressed much further than it should have and may require a longer treatment plan to get it under control. This increases costs for both insurance companies and patients.
- It may be difficult for patients to find reliable and reputable online information to provide to their primary care providers.
- Because of misdiagnoses, patients may be subjected to various drugs that have their own side effects. Once they see a dermatologist, the communication between doctors can be difficult to coordinate. Patients have to tell their stories numerous times, which can be frustrating.
- It is often difficult for patients to remember every detail about their ongoing symptoms and various treatments.
- Simplifying treatment/drug information into everyday language is a major stumbling block.
- On a global basis, there isn't a common protocol for less experienced doctors to follow. Patients may not live close to a doctor experienced in treating P/P, so there is a strong need for easily accessible information on both sides.
- In between doctor visits, patients may experience flares, illness, side effects, and triggers. These should be documented, but most patients don't have a good way of doing this.

This is where an app may be beneficial. The communication system within the app would give patients the ability to provide clear information about their situation—including a list of ongoing medications and other pertinent health information—to almost every doctor they visit. The app could potentially allow patients to communicate directly with doctors outside of visits, provide helpful information when it comes to research on both the physical and emotional aspects of P/P, and track (with time-stamped photos) disease progression and regression.

We also discussed the emotional aspects of P/P, including the fact that it isn't always helpful for newly

diagnosed patients to have everything explained in clinical terms, especially when they are extremely sick. Doctors are familiar with P/P and their schedules are tight, so it isn't always easy for them to recognize a patient's inability to absorb information. We came to the basic realization that communication was the key factor that needed a solution.

It was also interesting, as a patient, to hear the doctors' side of the situation. They too are frustrated by the lack of knowledge about P/P by common first step doctors such as, dentists, ENTs, and general practitioners. It was reassuring to realize that although they need to be compensated for their time, they do want to know what is going on with patients between appointments. As doctors, they are looking for a more efficient way to provide quality care for those of us struggling with rare diseases.

There will need to be additional meetings in order to create an app that provides usable scientific data for research. Additionally, the app must be supported by the insurance industry. But the first steps have been taken to understand the problems that may occur in the doctor/patient P/P journey, and it's time to start thinking about solutions to test.

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Lisa Ann Krutzik is a photographer, writer, entrepreneur, religious education director, and student of the world. PV shifted her perspective onto health and wellness. Find her blog at LARoxLife.wordpress.com.



From top left: Dr. Kristina Seiffert, Dr. Animesh Sinha, Christian Sejersen, Kasper Juul, Aleksander Eiken, Lisa Ann Krutzik, Joe Janca, and David Tsujimura



Advocating for Dental Research

Kate Frantz

In February 2017, the IPPF joined the Friends of the National Institute of Dental and Craniofacial Research (FNIDCR) Patient Advocacy Council (PAC). This group is made up of patient advocacy organizations whose patient members' conditions and diseases include an oral component and have a stake in research supported by the NIDCR. The group is supported by the American Association for Dental Research (AADR).

The AADR and FNIDCR will hold an Advocacy Day on Capitol Hill on Tuesday, February 27. Members and patient advocates will meet with members of Congress and Hill staff to advocate for dental, oral, and craniofacial research. They will emphasize how important investments in biomedical research and oral health programs are in impacting the people in their home states and beyond.

The IPPF had the pleasure of interviewing Lindsey Horan, AADR's Assistant Director of Government Affairs about their 2018 Advocacy Day and important legislative issues.

What is your goal for your advocacy day? What do you hope to achieve?

Lindsey Horan (LH): Our overarching goal for Advocacy Day is to educate on and raise awareness for dental, oral, and craniofacial research with members of Congress and congressional staff.

As oral research advocates, we know that oral health is integral to overall health, but it's critical to stress that to policymakers who are weighing competing priorities and responsible for divvying up federal funds across government agencies and programs.

The Hill visits our members conduct on Advocacy

Day have the opportunity to demonstrate the far-reaching nature of oral health research and, most importantly, to share their personal stories—whether it is the story of a patient whose life has been impacted by an oral disease or condition, or a researcher whose work is positively shaping the trajectory of dental and oral care we provide in this country.

Which legislative issues are of priority for the AADR/FNIDCR this year? Why are they important?

LH: Our legislative priorities for AADR and the Friends of NIDCR will be consistent with the priorities from 2017, and they largely relate to securing the highest possible federal funding for oral research and oral health programs. While this certainly includes the National Institutes of Health and the National Institute of Dental and Craniofacial Research (NIDCR), we also champion agencies whose work touches oral research in some capacity, such as the Centers for Disease Control and Prevention and its National Center for Health Statistics and the Agency for Healthcare Research and Quality.

Heavily focusing our legislative portfolio on appropriations is reflective of the fiscal and political environment in which we're operating. Congress has to make difficult decisions about how to tackle a mounting federal debt and deficit, and we want to ensure that shortsighted cuts aren't made in the name of savings. In the absence of our community speaking out—loudly—about these federal agencies and programs, lawmakers will see a win-win scenario: being able to cut funding with little to no pushback.

What can those who are unable to attend Advocacy Day in DC do to advocate locally?

LH: There is so much that can be done locally—even from home—to champion oral research throughout the year.

First, it's important to remember that members of Congress are not in Washington, DC, year round. They regularly return home to meet with their constituents, and these visits are great opportunities for people to voice their priorities or concerns. Sign up for your elected officials' email listservs to learn about upcoming town halls or other events where you might have an opportunity to speak with them. At the end of the day, constituents are the people members of Congress want to hear from most!

Additionally, don't underestimate the power of social media. Virtually all Senators and Representatives are active on Twitter, Facebook, and other social media platforms—and they pay attention to them. While seemingly inconsequential, research has shown that it doesn't take many Tweets on a given topic for staff to pay attention, especially if the Tweets come from constituents (and constituents should identify themselves as such in their Tweets).

Do you have advocacy alerts or ways to stay updated throughout the year on important legislative issues?

LH: We want to make sure our community knows how developments at the federal level may impact our field and the research enterprise more broadly.

The first resource I would recommend is our Government Affairs & Science Policy Blog which is regularly updated with advocacy and policy news. To make it easy for readers, there is an option to subscribe to the blog via email: <http://ga.dentalresearchblog.org>

We also post information to our Twitter account: [@DentalResearch](https://twitter.com/DentalResearch).

These are great places to learn about any new action alerts or opportunities for engagement.

There are also a number of opportunities available through AADR membership for those who want to further engage, such as the potential to serve on committees like our Government Affairs Committee or joining us on Capitol Hill for Advocacy Day. www.iadr.org/AADR/Join-Renew/Join-Us

Is there anything else you'd like to share?

LH: I know people often shy away from advocacy for a variety of reasons—they worry about bringing politics into the workplace, they are put off by the term, or they don't see the point. To this I would say:

1. *As an American citizen, you have a right to petition your government as outlined in the Constitution. It's correct that many employers do have rules related to advocacy, but they do not prohibit you as an individual citizen from being able to advocate. To clarify what is and is not allowed, talk to the government or public affairs staff at your organization or institution.*

2. *For those who don't quite understand or are put off by the term "advocacy," think of it as education. When you reach out to members of Congress, you are sharing your story, explaining your work, or demonstrating how a program is making a difference in your community. Members of Congress and congressional staff are grappling with information overload. Meeting with them provides an opportunity to share what you know so that they might better understand the issue and how it fits into their legislative priorities.*

3. *Finally, advocacy does make a difference. While a phone call, a Tweet, or an email seem too small to be significant, they add up—and we have seen this demonstrated time and again. Just recently, a provision in the House's first version of the tax bill calling to tax graduate students' tuition waivers as income received so much pushback from the community that it was removed in the final legislation. Speaking up and speaking out matter!*

A huge thank you to Lindsey Horan for taking the time to answer our questions!

IPPF Awareness Ambassador Coordinator, Bryon Scott, will attend Advocacy Day on Capitol Hill this year. We look forward to updating you on his experience and the outcome of his advocacy.

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Kate Frantz is the IPPF Awareness Program Director. She lives in Michigan with her husband.

The Benefits to Bionutrition

Kelly J. Calabrese

This article originally appeared in the Colorado Springs Business Journal (April 2004). It is reprinted here with the author's permission.

Many of us do not realize the aging process starts when we are in our twenties. We believe, at a young age, that we are resilient to any infection, chronic dysfunction, or disease. For some, this may be true, but for many of us this is where a slow and subtle progression of symptoms begins to occur.

I would like to use the analogy that Dr. Ray Strand has used in his book, *Bionutrition: Winning the War Within*. You are sitting in front of a wood-burning fireplace in your living room watching the flames and hearing and seeing the popping cinders. Now, imagine there is no protective screen preventing the cinders to pop onto the nice rug that lies in front of the fireplace. As time goes on, without the protective screen, eventually the cinders will burn many holes in the rug. The popping cinders are the free radicals and the rug is the DNA in our body. The protective screen is the antioxidant vitamins in the form of fruits, vegetables, and supplements that will help prevent oxidative stress.

By getting adequate daily vitamins and minerals greater than the recommended daily allowance, we will prevent chronic disease and we will live a long, optimal life. By focusing on the nutritional aspect of our diet, we will change the behavioral expression of the genetic (phenotype) DNA. If we support our cell dysfunction due to our genetic disposition, we will combat the aging and disease process. We will slow down the process and create an environment that will not influence our genetic disposition. We will do this by changing the biochemistry in our bodies with nutritional supplementation.

By preventing free radical damage in our DNA cells, we will prevent cancer, diabetes, heart disease, and immune and neurological dysfunction diseases. I encourage all of you to educate yourself, seek professional guidance through a Certified Clinical Nutritionist and be persistent! Best wishes in your quest for optimal health!

Kelly Calabrese, MS, CCN, is an Integrative Biochemical Nutritionist. She utilizes an 80-marker blood test to determine nutrient deficiency and get to the root cause of health disorders. She may be reached at (719) 590-9879 or fitfocus@questoffice.net. Her website is www.optimalwellnessLLC.com.



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WE ARE RECRUITING FOR A NEW PEMPHIGUS CLINICAL TRIAL

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- For more information, please visit www.clinicaltrials.gov and refer to study identifier NCT03075904

At Syntimmune, our mission is to develop targeted and effective therapies for IgG-mediated autoimmune diseases, such as pemphigus. Pemphigus is characterized by the presence of IgG-type auto-antibodies that bind to proteins called desmoglein 1 and desmoglein 3. The neonatal crystallizable fragment receptor, or FcRn, is thought to enable these desmoglein auto-antibodies to cause disease. SYNT001 has been designed to target FcRn and interrupt its interaction with IgG, including potentially disease-causing auto-antibodies, which we predict will dismantle and eliminate the inflammatory activities of these auto-antibodies.

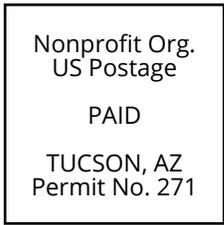
With industry-leading expertise in FcRn biology, our team of executives, scientists and advisors brings world-class leadership to the development of innovative drugs for autoimmune diseases.

For more information, visit www.syntimmune.com

SYNT001 is an investigational product and has not been approved for use outside of clinical trials.



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Healing Heroes go above and beyond to support the IPPF community by making sustaining, monthly gifts to support our mission of improving the quality of life for all those affected by pemphigus and pemphigoid.

Whether they be patients, family, friends, caretakers, medical professionals, or rare disease advocates, our Healing Heroes share the same vision:

No disease is too rare for a cure.

Monthly gifts allow us to not only sustain current programs, but also expand our key areas of operation: patient support, education, awareness, research, and advocacy.

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