

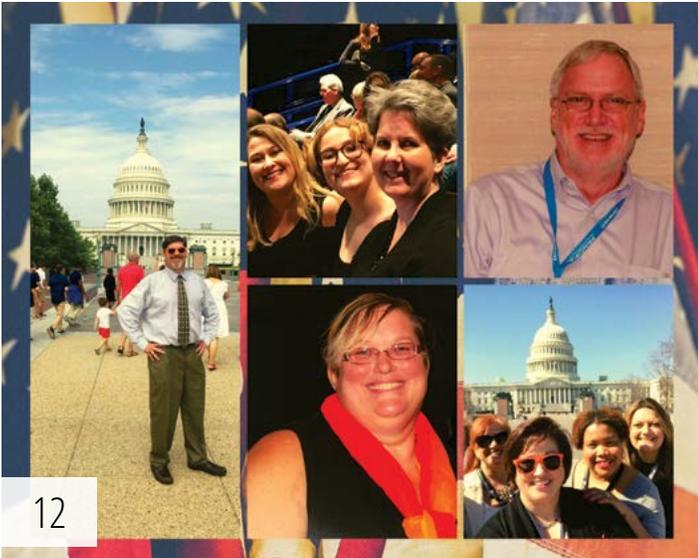
# Quarterly

Journal of the International Pemphigus & Pemphigoid Foundation



The  
Research & Advocacy  
Issue

# WINTER 2019



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# Message from the Executive Director



Welcome to the 2019 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 continue to grow.

One thing I have learned since being diagnosed in 2007 is that there is a clear link between our advocacy initiatives and accelerated research of pemphigus and pemphigoid (P/P). Our community's advocacy work continues to create disease awareness that spawns new research. Increased research brings with it the promise of new and improved treatments. These treatments provide hope for all those affected by P/P, which in turn promotes better health outcomes and well-being.

I'm excited to share that our advocacy in 2019 continues to be strong. We are committed to fostering relationships with congressional representatives and other rare disease organizations. This allows the IPPF to advocate for favorable state and federal legislation, research funding, and policies or regulations that benefit our community at large. IPPF advocates will once again head to our nation's Capital during Rare Disease Week at the end of February to make sure all our voices are being heard.

The IPPF had a banner year in the areas of research and drug development in 2018. Highlights include the first FDA drug approval in 60 years for pemphigus (rituximab); the first IPPF meeting with the FDA on disease treatment strategies; a scientific research symposium in Orlando, Florida, with over 175 attendees from 13 different countries; two papers published as a result of our research projects; collaboration on multiple surveys to learn more about P/P; almost 100 new patients enrolled in the Natural History Study; and a scientific poster based on the data collected from the Natural History Study. The IPPF community is fortunate to have expert clinicians, researchers, and scientists from around the world working diligently to increase our knowledge about what may cause P/P.

## "No disease is too rare for a cure."

In 2019, the IPPF will continue to encourage the development of P/P research and provide the most current information to patients by collaborating with stakeholders who are both treating these diseases and searching for a cure. **The IPPF is working hard to support you.**

Please enjoy the *Quarterly*; our successes are your successes. Together, we are supporting research, advocating for better policies related to patient care, and spreading disease awareness to the medical community. **Our work improves the lives of all people affected by P/P!**

Marc Yale  
IPPF Executive Director and MMP Patient  
[marc@pemphigus.org](mailto:marc@pemphigus.org)

# The Impact of Insurance Companies on Patient Access to Off-Label Drugs

Rebecca Krain, BA; Olivia Jew, BA; and Victoria Werth, MD

**P**emphigus and pemphigoid (P/P) are rare diseases that can have devastating consequences if not treated. Despite advancements in our understanding of the diseases, physicians consistently struggle to treat these patients effectively. While this struggle is in part due to the complexity of the disease, more often than not physicians are aware of which medications to prescribe, but face barriers to approval by third-party payers. Because the appropriate medications are off-label, insurance companies reject physicians' prescriptions. This leads to physicians feeling frustrated and patients feeling helpless.

In an effort to control healthcare spending and potentially inappropriate prescriptions, insurers maintain formularies that define which drugs they will or will not cover and for which indications. An "off-label" drug is one that is not approved for an indication on an insurer's formulary. Before approving an off-label drug for use, insurers will typically require patients to first go through trials of on-label drugs in a step-wise

manner. Beyond time consuming, especially in cases when physicians already know their patients will require a different approach from what is mandated by insurers, this delay to appropriate care can cause significant distress, worsened quality of life, and poor clinical outcomes for patients. When it comes to rare diseases such as P/P, insurers' formularies often fall short, and the covered medications can be both limited in efficacy and constrained by significant side effects. Without insurance coverage, patients have to pay out-of-pocket, which can cost thousands of dollars per year depending on the medication.

While this issue is not unique to patients with P/P, it is common in this patient population, as there are few drugs approved to treat such diseases. One patient in particular encapsulates the frustration that medical providers face when simply trying to relieve patient suffering.

Mrs. S\*, an elderly woman, came to the clinic with a recent diagnosis of urticarial bullous pemphigoid.

Because of her long-standing history of type 2 diabetes mellitus, a steroid-sparing approach was decided with the goal that she be administered omalizumab, an anti-IgE monoclonal antibody. Although IgG antibodies are the predominant antibody in bullous pemphigoid, it is known that IgE levels are often elevated and associated with its pathogenesis. Administration of omalizumab would therefore decrease IgE levels and disease activity, respectively. However, according to her insurance company's drug formulary, urticarial bullous pemphigoid is not an indication for omalizumab, and they denied medication coverage.

Mrs. S continued to suffer, with worsening pruritus, pain, and discomfort, all of which significantly impaired her quality of life. Without many alternatives, she was started on prednisone, leading to only mild improvement in her pemphigoid while exacerbating her diabetes. With a rising blood glucose level of over 400 mg/dL, she began to complain of worsening vision. Because of her hyperglycemia and poorly controlled pemphigoid, she was admitted to the hospital with the hope that, as an inpatient, she could be approved to receive omalizumab. A week after discharge and many months of suffering later, her insurer finally approved omalizumab. Although omalizumab has not yet been administered, we look forward to carefully monitoring and evaluating its effects, which we expect will improve Mrs. S's physical and mental health.

In addition to this patient's long ordeal to obtain omalizumab, patients with pemphigoid have faced significant issues with securing mycophenolate mofetil. Mycophenolate mofetil, an immunosuppressive indicated for patients with transplants, is a medication both well-tolerated and highly effective for the treatment of pemphigoid. Historically, although classified as an off-label drug, mycophenolate was often approved by insurers, but recently, insurers have started to withdraw approval even for patients who have used this medication for years.

Fortunately, medications that were once off-label are getting approved by the Food and Drug Administration. For instance, rituximab, an anti-CD20 monoclonal antibody, is now indicated for the treatment of adults with moderate to severe pemphigus vulgaris. This medication, known to drastically improve the signs and symptoms of blistering diseases, is finally becoming more affordable and accessible. While rituximab is

currently only indicated for pemphigus, due to a similar mechanism of disease, it should logically follow that rituximab be approved for pemphigoid as well.

Our argument is not that insurance formularies should not exist, but that they should be used judiciously given the clinical circumstances. Practically speaking, insurance formularies are beneficial for regulating standard drug prescriptions. These formularies may curb larger costs to the healthcare system by requiring patients to trial lower-cost medications first before resorting to more expensive medications.

However, patients with rare diseases receive the worst end of the bargain, as less is known about how to best treat their conditions, and thus this specialized clinical acumen is rarely incorporated into formularies. When physicians have the knowledge and expertise to identify appropriate and efficacious treatments for these rare diseases, they should be granted the freedom to do so by engaging in a more open-minded conversation with insurers to secure these appropriate medications for patients. Otherwise, while physicians and insurers battle for drug approval, patients with rare diseases such as pemphigus and pemphigoid will continue to suffer.

*\*Mrs. S's name has been changed for the purpose of this article.*

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# Efficacy and Safety of the Multidrug Protocol of Treatment of Pemphigus Combining Intravenous Immunoglobulin with the Cytotoxic Immunosuppressor and Mitochondrion-Protecting Drugs

**Sergei A. Grando, MD, PhD, DSc**

*This is a short version of the published article "Grando SA: Retrospective analysis of a single-center clinical experience toward development of curative treatment of 123 pemphigus patients with a long-term follow-up: efficacy and safety of the multidrug protocol combining intravenous immunoglobulin with the cytotoxic immunosuppressor and mitochondrion-protecting drugs. Intl J Dermatol 2019; 58:114–125."*

## INTRODUCTION

A systematic review and meta-analysis of randomized controlled trials evaluating the available evidence regarding efficacy and safety of interventions for pemphigus vulgaris (PV) concluded that despite widespread use of non-steroidal immunosuppressive agents, it is not known if steroid-sparing agents are beneficial. Although introduction of rituximab (RTX) provided for a favorable outcome, the high relapse rate, i.e., up to 80%, precludes successful use of RTX as a monotherapy. Intravenous immunoglobulin (IVIg) is being increasingly utilized as off-label therapy for a variety of autoimmune and inflammatory diseases, including PV and pemphigus foliaceus (PF). In contrast to RTX and conventional ISA, IVIg does not cause immunosuppression that endangers patients with infectious complications. It has been demonstrated that co-administration of a cytotoxic immunosuppressive drug (ICD) improves the ability of IVIg to lower serum levels of pathogenic autoantibodies in pemphigus.

This report summarizes clinical outcome of 117 PV and 6 PF patients treated with a combination of IVIg, an ICD and mitochondrion-protecting drugs in the Blistering Disease Clinic at University of California Irvine from 2007 to 2017. To the best of my knowledge, this is the largest cohort of pemphigus patients reported to be treated with IVIg in a single academic center and followed for a sufficiently long period of

time to justify meaningful conclusions. The results have demonstrated a success rate of 100%, suggesting that permanent remission off drugs (or cure) may be possible.

## TREATMENT PROTOCOL

All treatment modalities included in the multidrug protocol described below were initiated simultaneously. All patients were treated with oral prednisone or methylprednisolone starting at approximately 1 mg/kg/day dose, minocycline (or doxycycline) at 200 mg/day and niacinamide (nicotinamide) at 1.5 g/day. In case of disease progression, the dose of prednisone was increased by approximately 20% in a 2-week interval until disease control was achieved. In patients who did not respond to approximately 1.5 mg/kg per day, prednisone was changed to the equivalent in activity dose of methylprednisolone, taking into consideration that the activity of 5 mg or prednisone is equivalent to that of 4 mg of methylprednisolone. After disease control was achieved, the therapeutic dose of prednisone/methylprednisolone remained fixed until complete remission of disease, at which point, the daily dosage was reduced by 15-20% every 15-20 days down to zero. IVIg, 2 g/kg/month, was given on 4-5 consecutive days as a slow, approximately 4-hour infusion, hereinafter referred to as "IVIg cycle." Prior to each infusion, the patients were premedicated with 50 mg diphenhydramine and 500

mg acetaminophen. To prevent a rebound effect from compensatory overproduction of pemphigus antibodies by the autoantibody-producing cells stimulated via a negative feedback due to rapid clearance of pathogenic IgGs, IVIg was combined with the ICD mycophenolate mofetil, 2 g/day, azathioprine, 200 mg/day, or cyclophosphamide, 150 mg/day, chosen in the above order depending on the patient's insurance allowance as well as counter indications and adverse reactions (*scenario "A" on the graph*). The patients who could not tolerate ICDs were switched to RTX, which was administered using the modified Dr. Ahmed's IVIg+RTX protocol, according to which RTX was given at 375 mg/m<sup>2</sup> body surface area once per week for 3 weeks during the first and the second months followed by four monthly infusions (10 infusions total; *scenario "B" on the graph*). Pemphigus patients with disease relapse were treated exactly the same way as new patients, ie, the current dose of prednisone/methylprednisolone was increased by 50 to 100% until disease control, and then tapered off per. After prednisone/methylprednisolone was tapered off, all other medications were continued without a change for 6 months. If no relapse was observed during that time, the frequency of IVIg was decreased to every other month for 3 cycles, and then to every three months for 2 cycles. Thus, in the absence of relapses, the post-steroid treatment continued for 18 months. All patients also received 1 g of calcium supplement and two age/gender-specific multivitamin tablets per day, as well as medications required to control coexisting medical conditions and complications, if any. The treatment ended, ie, all oral medicines were discontinued, two weeks after the last IVIg cycle. The patients were counseled about the need to avoid factors known to precipitate pemphigus onset and exacerbations, such as emotional stress, overheating, ultraviolet radiation, as well as the drugs angiotensin-converting enzyme inhibitors, and penicillin and its derivatives.

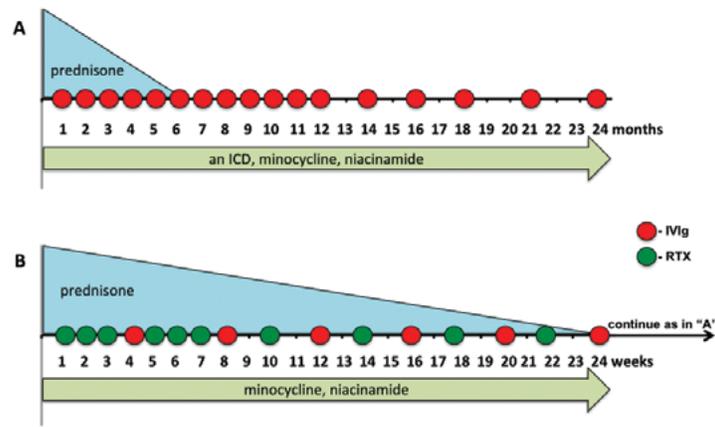
## RESULTS

The retrospective analysis of medical records of 123 pemphigus patients who had completed the course of treatment with IVIg demonstrated that the mean time to disease control was 0.2 month and time to complete remission was 1.7 months. Duration of complete remission on drug until relapse or end of treatment was 19.3 months. The mean duration of complete remission off

drugs until relapse was 15.8 months. That until end of follow up was 48.4 months, with a minimum of 9 and a maximum of 97 months. The overall complete remission rate off all drugs was 100% with 12% overall relapse rate. Most relapses, 8.1 vs. 3.3%, occurred during the time of treatment, compared to post-treatment. No patients had more than a single relapse. Current maximum duration of complete clinical remission off all drugs is 91 months or ~7.5 years. The minimal duration of therapy without a relapse was approximately 26 months, but the calculated mean value for all patients, including those who had a relapse, was 29.4 months. Total number of IVIg cycles ranged from 26 (in patients without a relapse) to 37 (in patients with a relapse).

Mycophenolate mofetil was used as the first line ICD in 63 PV and 4 PF patients and azathioprine in 29 and 2 PV and PF patients, respectively, and 11 PV patients were initially treated with cyclophosphamide. Transient transaminitis developed in 7 patients taking mycophenolate mofetil and 2 patients taking azathioprine (all had PV). Instead of reducing the standard dose, all patients were switched to cyclophosphamide. There was no evidence that one ICD was tolerated better or associated with a different outcome than others. All patients remained on an ICD throughout the entire duration of and discontinued two weeks after last IVIg cycle together with the mitochondrion-protecting drugs. RTX was included in the IVIg treatment protocol of 23 PV patients who could not tolerate other ICDs due to liver and/or kidney problems or specifically requested RTX. The clinical outcome in patients that received IVIg with RTX or another ICD were similar.

Overall, adverse effects of IVIg therapy, i.e. those developed during or within 48 hrs after IVIg infusion, were observed in 72 patients (58.5%). These complications included mild-to-severe headache (22.1%), nausea/vomiting (14.6%), fever/chills (25.2%), fatigue/lethargy (15.4%), increased or decreased blood pressure (17.9%) and cutaneous symptoms, such as pruritus, erythema and urticaria (9.8%). These events usually resolved due to slowing the infusion rate, pausing infusion and/or using symptomatic treatments. Severe intractable headache that required stopping the infusion and switching the IVIg batch or brand was observed in 7 patients (5.7%), all of who had a history of migraine. Self-limited anemia, which might be induced by an ICD and/or IVIg (due to natural



Two scenarios of treatment: without (A) and with (B) RTX

antibodies to red blood cell antigens), was observed in 12 (9.8%) patients. Rare adverse events requiring hospitalization were observed in 5 (4.1%) patients. These included pulmonary embolism (1 patient), deep vein thrombosis (1 patient), stroke (1 patient) and hemolytic anemia requiring blood transfusion (2 patients). These complications did not re-occur after IVIg treatment was resumed. All patients with a history of thromboembolic events received anticoagulant therapy.

## DISCUSSION

The results of comprehensive treatment of our patients with PV and PF using the multidrug protocol that consisted of the initial loading dose of prednisone and prolong administration of IVIg together with an ICD and mitochondrion-protecting drugs provided for rapid control of pemphigus symptoms and stable disease remission and allowed an overall safety of treatment. Although our therapeutic approach did not ultimately resolve the principal problem of pemphigus treatment because of remaining issues with adverse events from systemic corticosteroids and ICDs as well as a relatively long duration of treatment, it allowed to achieve a prolonged, possibly lifetime clinical remission in a vast majority of pemphigus patients.

A relatively small relapse rate of 12% eliminated the need for additional courses of systemic corticosteroids allowing to avoid most typical adverse effects of long-term systemic corticosteroid therapy. This was achieved by designing the regimen that optimizes treatment efficacy due to synergy of the drugs included in the treatment protocol. Indeed, each drug had been used in the past for treatment of pemphigus patients,

but not in such unique combination.

The IVIg dose was based on the reports about the most effective doses of IVIg and the benefit of combining IVIg with an ICD. A trial of different doses of IVIg ranging from 0.5 to 2 g/kg per cycle and different infusion schedules has determined the optimal dose of 2 g/kg per cycle and frequency of cycles under 4 weeks. This frequency is apparently mandated by the need for maintaining the therapeutic dose of IVIg, which depends on the IVIg half-life of ~3 weeks. The rationale for concurrent administration of IVIg and an ICD was based on observations that, on the one hand, depletion of pathogenic antibodies brings about a reciprocal increase in their serum levels, potentially leading to a flare of the disease, and, on the other hand, agents that suppress antibody synthesis can prevent such rebound and thus improve the effectiveness of IVIg therapy of pemphigus. This physiological mechanism provides an opportunity to selectively suppress production of pathogenic antibodies, because only antibodies that have been depleted are resynthesized. The plasma cell producing depleted antibodies thus become a selective target for cytotoxic agents, because the ICDs such as azathioprine and mycophenolate mofetil were shown to be highly efficient against activated, compared to quiescent, lymphocytes. Hence, co-administration of IVIg and an ICD leads to "suicidal" proliferation of pathogenic antibody-producing cells. Indeed, it has been documented that pemphigus patients treated with such combined regimen had a greater decline in pathogenic autoantibodies, faster clinical improvement, and required a smaller cumulative dose of systemic corticosteroids.

The concurrent administration of IVIg and RTX eliminates the major concern of using RTX in the treatment of pemphigus, such as the high incidence of systemic infections that can lead to fatal septicemia. As documented in this study and reported in literature, combination of RTX with IVIg brings RTX-related mortality down to zero.

The combination of niacinamide and minocycline have been empirically shown to be effective steroid-sparing agents in pemphigus. In addition to their well-known mechanism of pharmacological action, both drugs exhibit the mitochondrion-protecting effects by protecting the mitochondrial oxidative-phosphorylation function (niacinamide) and the outer membrane impermeability (minocycline), both of which are altered by anti-mitochondrial antibodies produced in PV patients.

## CONCLUSIONS

The multidrug treatment regimen allowed to achieve the following therapeutic goals:

- abrupt cessation of development of new and rapid healing of existing lesions;
- prompt disappearance of the functional impairment associated with the disease;
- complete clinical remission;
- prevention of flares; and
- lack of serious side effects.

The most important outcome is improvement of the quality of life of the patients. As such, this regimen appears to be superior to the treatment modalities that do not include IVIg. A reasonable expectation that current complete remission in 100% of patients will extend indefinitely, makes patients less apprehensive about possible flares and the need for additional therapy. Indeed, the possibility that our patients develop a relapse in the future cannot be completely ruled out. The efficacy of the multidrug IVIg protocol does not depend on serological and clinical differences of individual patients, because a combination of IVIg with an ICD eliminates all kinds of pathogenic autoantibodies equally efficiently, and the mitochondrion-protecting drugs uniformly prevent keratinocyte apoptosis triggered through various autoantibody-activated signaling pathways. Future head to head prospective studies should ultimately clarify efficacies of different therapies in defined groups of patients.

# Meet the Doctor: Sergei Grando

Dr. Sergei Grando is an immunodermatologist affiliated with UC Irvine Health who specializes in the treatment of pemphigus and pemphigoid (P/P). He has published more than 225 research documents and has been awarded numerous grants from the National Institutes of Health. Dr. Grando is on the IPPF Medical Advisory Board and has even helped spread awareness in the dental community by volunteering with the IPPF at a California Dental Association meeting.

Recently, the IPPF asked Dr. Grando about his work.

## How did you become interested in P/P?

SG: Back in the USSR in the 1980s, where P/P were the most disadvantageous groups of dermatological patients. [It was] based in my interest in immunology emerging as a new science.

## Why is research important, especially for rare diseases?

SG: Because P/P patients suffer the most from side effects of treatment.

## What do you want the IPPF community to know about your specific research project(s)?

SG: Based on my studies of non-desmoglein autoimmunity in pemphigus, I have developed a multi-drug treatment protocol of pemphigus that allows an 88 percent drug-free relapse period of greater than five years, which is probably a cure.

## What can patients and caregivers do to promote research of rare diseases?

SG: Support research efforts by participating in surveys and donating blood [to researchers].

## What should patients do if they are interested in participating in research studies or clinical trials?

SG: Find one close to their home.

Note: see the clinical trials article in this Quarterly and find more information at [www.pemphigus.org/research/clinical-trials](http://www.pemphigus.org/research/clinical-trials) or [www.clinicaltrials.gov](http://www.clinicaltrials.gov)

Thank you, Dr. Grando, for all that you do to help our community. We are grateful to have knowledgeable doctors who continue to focus on P/P research and to work for better treatments and cures.



# A New Year's Perspective

Terry Wolinsky McDonald, PhD

**I**began writing this article during the week between Hanukkah and Christmas. Holiday lights and decorations were everywhere, and I was hard-pressed to find any non-holiday music on the radio. People were busy with holiday parties and family gatherings. They were watching holiday movies, many guaranteed to bring back memories (and maybe even tears). On the surface, it was a beautiful and festive time. For those of us who work in the mental health field, it's also a busy time of the year.

As I write, it is almost the last day of 2018, and the countdown to New Year's Eve and 2019 is under way. For people with loved ones and those enjoying traditions old and new, the holidays may be relatively smooth. For others—especially those mourning the loss of loved ones, unable to be with family, or living with a

chronic illness—it can be a difficult time. My pager has been going off almost nonstop for most of the month of December and this seasonal phenomenon is showing no signs of slowing down.

The beginning of the New Year can go in different directions. For most people, the rhythm of everyday life returns when the holidays are over. Some settle into old routines—ones that may or may not work well. Some people make many New Year's resolutions and dive into them. In my experience, this doesn't tend to work very well. I've found that making small changes is often more effective and satisfying.

By the time this article is published, most big resolutions will have fallen by the wayside. That's fine; it's normal. It's okay to hope for big changes. However, another way to approach New Year's resolutions is to

view them as long-term goals. After this, think about at least five smaller, more manageable changes that can help you reach your long-term goal. Decide on a measurable way to achieve each of the smaller changes. For example, if your long-term goal is to increase your social network despite being shy or introverted, your smaller goal may start with leaving your home more often. You can make it a measurable behavior by leaving home two to three times per week. After a couple of weeks of success, you may make another small goal of making eye contact with at least three people when you're out of the house. The next goal may be to say "hello" or "good morning" to at least three people that you make eye contact with on each day you leave home. You will achieve small successes on your way to your larger goal, instead of trying to do it all at once.

There are other ways to move more slowly (but surely) toward other types of goals. Let's say your goal is to clean and organize your home or closet. For many, this goal may feel overwhelming. It can lead to procrastination and a feeling of dread that it may never even get started. Another impediment to completing the task may be the sheer amount of time it will take. It's okay to allow yourself to complete a task over time instead of all at once. Breaking up your work into 15 minutes each day is more manageable. Set a timer for 15 minutes

every day and see how much you can accomplish (you may even find yourself working longer). If you do this every day for one week, you will have completed almost two hours toward your goal. Breaking up your time into smaller, daily intervals allow you to make steady progress toward your larger goal. It also sets you up for

## Be a kinder *you* toward yourself.

success. The positive feelings about yourself and what you can accomplish allow you to move forward. You are teaching yourself a new way to get things done. This will help in achieving your current goals and in basic life and time-management skills.

I want to wish everyone a happy and healthy New Year. Try not to be too hard on yourself. Be a kinder *you* toward yourself. As you move forward in 2019, you may find that some of your resolutions will become a reality—and if not, at least you will be progressing in a positive direction.

---

*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.*



## Looking for a Support Group?

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)**

Find more  
Patient Support  
Group locations  
and dates at  
**[pemphigus.org](http://pemphigus.org)**

# We the People



## Becky Strong

**W**hen I first started to share my journey as a pemphigus patient, it wasn't too intimidating to educate my family and friends. I even advocated to the medical and dental communities, encouraging them to learn more about autoimmune blistering diseases so others didn't have to go through what I went through. But the thought of knocking on the doors of my elected state and federal representatives? Terrifying. I needed an opportunity and the courage to make the jump.

Speaking to our legislators can be intimidating. When asked to describe her own experience with rare disease advocacy, IPPF Mid-Atlantic Support Group Leader Carolyn Fota said the following: "At first, I was very shy in participating. I was very new to having a serious disease—what did I know? What I learned is that people really do care, and they want to know about

your experience in having a rare disease, not that you're an all-knowing expert on your illness. Once I understood this, telling people my story was easy."

Bryon Scott, IPPF Awareness Ambassador, offered his own advice. "Take the opportunity to spread your audience base. You are already advocating as you talk to family members, friends, and possibly neighbors. Just take what you are doing and make the circle a little larger."

**But how? I'm just one person with a very rare disease!**

### **You are also:**

- A registered voter who is part of the election process. Whether or not you voted for a particular candidate, they are supposed to work for you.

- You are one of more than 30 million Americans living with a rare disease. There are over 7,000 known rare diseases.
- You are one of the over 50,000 Americans living with an autoimmune disease.
- You are part of the one-third of our country affected by a skin condition.

We each hold a great power, and we are part of a large community that can speak up. We can work together to make sure the government passes fair legislation that supports the rare disease community. Our stories make the debates personal. We have the power to move a congressional representative to vote in favor of laws that make it easier for rare disease patients to get treatments, help the Food and Drug Administration (FDA) approve drugs for our disease, or encourage the funding of research at the National Institutes of Health (NIH). Advocacy can be quiet and subtle, or it can be loud and brave. It's just important that we participate. There are many types of advocacy opportunities, and the IPPF can help you get started.

Jeff Weisgerber, pemphigus vulgaris (PV) patient, wasn't sure that he could have an impact before he became an advocate. "I was skeptical at first about not knowing enough about PV and was scared that I wasn't informed enough to really help," he said. "But that was quickly replaced with the fact that no one knows my body and the effects PV has had on it better than myself. And that is how we as patients help with the cause, by the knowledge we have about our disease and the input we can give. I'm totally on board with helping any way I can and no longer do I feel hesitant about saying 'Yes, I would love to help.' Now I look forward to these advocacy chances."

Lisa Ann Krutzik, IPPF Buffalo Support Group Leader, shared, "My first experience [with advocacy] was going into dental offices, but in reality, it was with my general practitioner. Although he had learned about PV from textbooks, I am his first patient. He has been on this learning journey with me, but I started giving him issues of the IPPF *Quarterly*, and he started asking more questions. We've talked through the drug options, about the NIH and the NIH budget, and about clinical research. We've talked about the need to get more ENTs educated and even general practitioner offices."

Bryon's first taste of advocacy was during In-District Lobby Days, sponsored by Rare Disease Legislative Advocates (RDLA). "I am an introvert by nature—a behind the scenes person," Bryon said. "The truth is that I went into the first meeting with the attitude of listening. After I told my story about PV and the diagnosis, the congressional aide and the two mothers in the meeting came up and thanked me. They told me I placed a personal perspective on rare disease."

The more advocacy events Bryon has attended, the easier it has become. "I am more willing to open up about PV. People are listening, even though it may not seem like they are. When I returned to a senator's office this year for a follow-up meeting, the staffer met me at the door wearing the IPPF orange sunglasses that I gave him the year before. I thanked him for remembering. He responded that it was not the sunglasses that he remembered, it was my personal story of the pain I experienced with PV. He stated that he keeps looking for opportunities to help."

Lisa Ann shared, "So much about having a chronic autoimmune disease is that I feel so much is not controllable. By learning, teaching, sharing, researching, and interviewing, it gives me comfort to know I can make a difference in someone else's life. That I might be the person that teaches the right doctor what they need to know to identify this in someone else much quicker than it happened with me."

When asked about providing advice for someone thinking about becoming an advocate, Jeff was quick to say, "If we could get everyone to help a little, we could help out our cause a lot." Carolyn said, "It's been a wonderful experience advocating for the IPPF community and I recommend for anyone considering advocacy to give it a try." Lisa Ann added, "If you even have a spark of interest, take the opportunity to learn more. A spark can only ignite something that is able to be lit on fire. If by taking the time to learn more about becoming an advocate a fire is not lit, you've taken the time for personal development. If you light up like a fireplace with dry wood then you can help others deal with their situation, or you can teach people how to recognize it faster, thus helping new people indirectly."

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*Becky Strong is the IPPF Outreach Manager. She was diagnosed with PV in 2010 and is currently in remission. She lives in Michigan with her family.*

# My Journey with Pemphigus Vulgaris



## Maria Hernandez

**M**y journey started in the spring of 2017. I started suffering from non-healing blisters that began on my scalp and ultimately covered the majority of my body. I made endless visits to doctors, urgent care, and the hospital with these persistent blisters, and I was primarily treated with topical gels, creams, and washes. I didn't have any relief whatsoever.

By June of 2017, the blisters had spread like wildfire with the intense summer heat. I was referred to a dermatologist for the first time and was prescribed oral steroids (prednisone). The dermatologist also suggested I have a biopsy done to get an accurate diagnosis. The results came back inconclusive, so a second biopsy was done. On June 28, 2017, for the very first time, this rare disease of mine had a name: pemphigus vulgaris (PV). The dermatologist let me know that she was unable to continue with my treatment and advised that I seek university-level dermatology care. She told me there was no cure for PV, but there was treatment

that may lead to remission or control of the disease. I was in shock and had so many questions. I felt lost and afraid of what was to come.

I was in shock and had so many questions. I felt lost and afraid of what was to come.

In July of 2017, I received approval from my insurance company to continue with treatment and to be referred to a specialist. I started my own research, which only led to more fear and questions until I joined a Facebook support group for PV. I scrolled through endless posts. Some were comforting, and others were not. In this group, I was approached by Mei Ling Moore, a Peer Health Coach with the International Pemphigus



*Photo courtesy of Maria Hernandez*

& Pemphigoid Foundation. She was so helpful in guiding me to the Foundation, which provided me with a list of local PV specialists.

Mei Ling kept in contact with me until I had my first appointment on August 28, 2017, at the University of California, San Francisco Medical Center, where I was treated by Dr. Tiffany Scharschmidt and Dr. Chen Chen. I received so much information on this first visit. The care was amazing from beginning to end. I continued taking prednisone, but after two months it caused side effects like blurred vision, anxiety attacks, depression, extreme weight loss, nausea, and others. It prevented new blisters from forming, but it wasn't clearing up the existing ones. I was prescribed prednisone in a tapering method; a scalp oil to loosen up the crust that was forming from the blisters; and, ultimately, rituximab.

At the time, I was informed that rituximab was not yet approved by the FDA as a treatment for PV. But at this point, my disease had reached a very deteriorating stage, so I was willing to give it a try.

I received my first rituximab infusion on September 26, 2017, and I received a second round on October 12, 2017. I reacted well to the treatment without experiencing side effects. Then, it was only a matter of time and a lot of patience before I started to see results.

By December of 2017, I continued tapering my prednisone dosage as the blisters slowly healed. The blisters on my scalp were really bad with this disease, and as they started to dry and lift, my hair fell out in patches until I lost it all. As a woman, this was one of the hardest parts of my journey. On January 10, 2018, I went to my first follow-up appointment after the rituximab infusions. Physically and clinically, I was doing quite well. My doctors and I were very impressed with the amazing progress. By the time summer came, all that was left of my blisters were very faint scars. I was able to enjoy the outdoors without the heat bothering me like it did when I was in full flare. I was also blessed with a full head of healthy and curly hair!

By September 10, 2018, I was finished taking prednisone. At another follow-up appointment, my labs came back negative for any PV activity. I was super excited to hear the word "remission" and cried tears of joy. I am still in remission and try to live as normal a life as I can. I do not know how long I will remain in this stage, but I do know that if I ever relapse, I am more prepared to fight.

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*Maria Hernandez is a PV patient who has been in remission since September 2018. She resides in California and continues to enjoy life with all its new changes.*



Find other stories and the latest info on the IPPF news site: [pempress.com](http://pempress.com)

# A Family Matter

## ADVOCATING ON CAPITOL HILL

Amethyst Yale

**L**ast year was the second year I attended Rare Disease Legislative Advocates' (RDLA) Rare Disease Week on Capitol Hill. My dad and younger sister attended as well. The first year I attended I was nervous and unsure that I even needed to go. After all I am not a patient, my father is.

My dad and I were both California constituents during my first trip, and we were able to attend the same meetings. During one of our meetings, tears filled my eyes as I shared my perspective of watching my dad struggle with finding a diagnosis and getting insurance company approval for the drugs he needed to live. As I told my story, the congresswoman, her staffer, and all of the other advocates listened intently. I stressed that if legislation existed that allowed patients better access to "off-label" drugs and provided support for rare disease research, the outcome could have been very different for my dad. Perhaps he wouldn't have gone permanently blind, or he wouldn't have had to travel thousands of miles to see a doctor who actually knew how to treat his disease. Perhaps he wouldn't have had to fight insurance companies for a life-saving drug in the midst of knocking on death's door.

After sharing his experience, I felt empowered. I had a voice. I was amazed that my representatives cared enough to listen to me. That weekend on Capitol Hill, I learned that my story is important to my representatives because I am one of their constituents. They represent me, and if I don't advocate for the things that matter, how will they know what legislation to support?

Last year when I attended Rare Disease Week, I was a constituent of Colorado. I was able to share my story with Colorado representatives while my dad and sister met with their representatives from California. Our family's rare disease story was not only being shared with three or four representatives, we were able to reach



Hannah, Marc, and Amethyst Yale

twelve different representative's offices! Throughout the weekend, I realized how important it is for our representatives not only to hear from rare disease patients, but also from their family members. RDLA shared with us that one in ten people is affected by a rare disease, which in reality makes rare disease patients more common. Imagine the impact the rare disease community would have if more family members and friends of rare disease patients became involved.

Your story is important, too. I encourage you to consider getting involved in advocacy. If traveling to Washington, DC, for Rare Disease Week is too far, you can participate in RDLA's In-District Lobby Days. In-District Lobby Days are a great opportunity to gather your friends and loved ones to make your stories known to your senators and representatives in your home district. You can also inform your representatives of specific legislation that supports the rare disease community by writing, emailing, or calling their offices. By advocating and sharing your stories, you can help the entire rare disease community overcome the many challenges we currently face.

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*Amethyst Yale is the IPPF Outreach Assistant. She currently lives in Colorado Springs and is pursuing her master's degree in public administration at the University of Colorado. Her father was diagnosed with pemphigoid 10 years ago.*

Thank you to our corporate sponsors, Argenx and Kroger, for helping us send advocates to Capitol Hill for Rare Disease Week 2019.

# The IPPF Natural History Study is Accelerating Drug Development

**Marc Yale**

**T**he IPPF Natural History Study was launched in March of 2017 in conjunction with the National Organization for Rare Disorders (NORD) and the US Food and Drug Administration (FDA). The study is helping pemphigus and pemphigoid (P/P) researchers gain knowledge that is essential to building the scientific foundation for accelerated drug development. Since P/P are poorly understood, the study tracks the course of these diseases over time while identifying patient demographics and quality of life issues that influence the drug development process.

Before new treatment therapies can be developed, investigators need to better estimate the prevalence of P/P, study their pathophysiology, understand treatment patterns, and assess the burden of these diseases on patients. The IPPF Natural History Study collects, stores, and provides patient disease data for analysis in future drug development. According to Pamela Gavin from NORD, "Patients who have a rare disease, or whose family members do, have the most direct interest in generating data about the natural progression of the disease. Patients have played a direct role in the development of a number of drugs but what is lacking is a systematic and uniform approach to using patient experiences and knowledge in developing data about the natural history of diseases that patient organizations can afford to acquire and maintain over time." (Gavin, 2017, 855-857)<sup>1</sup>

1. Pamela Gavin, "The importance of natural histories for rare diseases," *Expert Opinion on Orphan Drugs*, 3:8, (2015): 855-857, <https://doi.org/10.1517/21678707.2015.1063415>.

The IPPF has almost 300 patients currently participating in the Natural History Study, and we want to empower more patients to contribute. Together, we can develop the kind of information that will make it easier for drug developers and researchers to understand the effects of treatments so we can speed up the process. Currently, the IPPF is in the process of creating two new surveys to better understand the delay in disease diagnosis and the effects that systemic corticosteroids have on P/P patients. We are planning to launch these surveys in the upcoming months.

To stimulate interest in basic, clinical, and translational research, the IPPF has begun to analyze the data from the Natural History Study. We are developing scientific posters, abstracts, and manuscripts to create even more awareness of the study. However, we need more patient participation. The more we can collect vital disease data from participants, the more we can accelerate drug development to improve patient health.

**For information on how you can join the IPPF Natural History Study, visit:**

[pemphigus.org/research/natural-history-study](http://pemphigus.org/research/natural-history-study)

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*Marc Yale, IPPF Executive Director, was diagnosed in 2007 with Cicatricial Pemphigoid. In 2008, he joined the IPPF as a Peer Health Coach and was promoted to Executive Director in 2016. Marc currently resides in Ventura, California, with his wife Beth and daughter Hannah.*



## Iva Rauh

**T**he 7th Annual Rare Voice Awards was held on September 12, 2018, at the Arena Stage in Washington, DC. The event was a widely attended public event with congressional, government agency, and National Institutes of Health staff, as well as industry executives and patient advocates.

Award recipients were chosen by a committee from nominations received from the rare disease community. Awardees received an “Abbey” statuette named for Abbey Meyers, the founder of the National Organization for Rare Disorders (NORD). The statuette is specially commissioned for the Rare Voice Awards from renowned sculptor, NOBE. Dr. John Whyte, a popular physician and writer, was the master of ceremonies.

Rep. Cathy McMorris Rodgers (WA) and Rep. Jackie Speier (CA) were recipients of the Rare Voice Award for Congressional Leadership for their support of the rare disease community. Patient advocates and congressional staffers who work behind the scenes were also recognized. Sadie Keller received the Advocacy: Young Adult award. She was diagnosed in 2011 with

acute lymphoblastic leukemia. Instead of feeling sorry for herself, she started the Sadie Keller Foundation that helps kids smile and not think about cancer. The acceptance speech of this amazing 11-year-old moved me and others in the audience to tears.

Thank you to the generous sponsors for the delicious food that was served. With a glass of champagne, it was easy to make some new friends. Heartbreaking stories were shared. I had the opportunity to tell my story, raise awareness about skin disorders, and praise the work of the IPPF.

This was my second year attending the event, and it was an unforgettable evening. To be around many other rare disease advocates truly gives me hope—especially younger people who continue to make a difference and show the world that our voices need to be heard. I’m thankful for my friends in the rare disease community who inspire me and remind me that every single one of us can make a difference.

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*Iva Rauh was diagnosed in 2005 with PV. She and her husband live on the Eastern Shore of Maryland.*

# In Memoriam: Dr. Marcel F. Jonkman

DECEMBER 19, 1957 - JANUARY 14, 2019

**Barbara Horváth and Marieke Bolling**

**“There is luck and there is arbitrariness.**

**T** You should not have the pretention that you can change everything,” said Marcel Jonkman a few months ago, after he learned that he had metastasized pancreas carcinoma. Over the past 30 years Marcel Jonkman has profiled as a doctor, dermatologist, scientist, teacher, trainer, mentor, professor, and head of the department of dermatology at the University Medical Center Groningen (UMCG) in the Netherlands. He was a thinker and an inventor.

During his specialization beginning in the 1990s, he became interested in blistering diseases. In 1995, he described how deficiency of BP180 is the cause of a subtype of the hereditary skin fragility disorder epidermolysis bullosa (EB). In 1996, he described the concept of revertant mosaicism in EB, and he published in *Cell*, one of the best-rated medical journals.

For Marcel, his patients always came first. He established the multidisciplinary EB team at UMCG, consisting of 19 specialists. He always maintained good contacts with patient associations, both for EB and other autoimmune blistering diseases.

In the 1990s, the Center for Blister Diseases in Groningen was established. It was and is the only center of expertise in autoimmune and genetic blistering diseases in the Netherlands focusing on patient care and science. It is internationally recognized.

Marcel’s inauguration as a professor took place in 2002. His inaugural lecture on blistering diseases was entitled “Broken Contacts.” In 2003, Marcel also



*Dr. Marcel\_F.\_Jonkman, photo by Marjolein Annegarn*

became head of the department. Marcel was a very inspiring person. His innovative knowledge, drive, and intelligence is an example for many.

Marcel was integral in making rituximab treatments available to patients with autoimmune blistering diseases in Groningen. He organized several patient information days where patients and professionals came together. He also recently described a new form of pemphigoid called nonbullous pemphigoid—a blistering disease without blisters. Together with the IPPF, he investigated the unmet needs in pemphigoid patients. His goal was to achieve early recognition and effective treatment for patients with autoimmune blistering diseases.

We (the Department of Dermatology at UMCG) will miss him deeply. It is almost inconceivable that the door of his room is no longer open, and we can no longer walk in and ask the question, “Marcel, I have a difficult diagnostic/therapeutic problem in a patient, what would you do?”

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*Barbara Horváth and Marieke Bolling are dermatologists at the Center of Blistering Diseases, University Medical Center Groningen, the Netherlands.*

# Rare Disease Day 2019

Becky Strong

**F**or some of us, living with a rare disease is a full-time job. We go to doctors, specialists, pharmacies, dentists, therapy, and more. It would be exhausting if you were healthy, but when you are sick, sore, and tired it can be overwhelming. This year, Rare Disease Day will highlight the struggle many patients face when trying to manage a rare disease and the importance of self-care.

Rare Disease Day is February 28, 2019. According to the European Organization for Rare Diseases (EURODIS), “Rare Disease Day takes place on the last day of February each year. The main objective of Rare Disease Day is to raise awareness amongst the general public and decision-makers about rare diseases and their impact on patients' lives.”

Raising awareness is important because pemphigus and pemphigoid are only two out of over 7,000 rare diseases that affect over 30 million people in the United States alone. Worldwide, rare disease affects one in 20 people. There are only a few rare diseases that have a known cure or Federal Drug Administration (FDA) approved treatment. With these statistics, you can see that as a whole, rare diseases aren't so rare.

Currently, there are 23 events planned in 48 countries around the world for Rare Disease Day 2019. The easiest way to find an event close to you is by visiting [rarediseaseday.org](http://rarediseaseday.org). Organizations like the National Organization for Rare Disorders (NORD), Rare Disease Legislative Advocates (RDLA), and EURODIS have events planned for Rare Disease Day.

You can still get involved even if there isn't an event near you. You can host a fundraiser to raise awareness, or talk to your elected officials and ask them to make a Forever Proclamation that the last day in February is always Rare Disease Day. This can be done at both the state and federal levels by speaking with your representatives. You

can also ask for a meeting to speak to your representatives in their district offices or write them a letter to share your story and where the rare disease community needs their support. You can even reach out to local universities to see if they are having events you can participate in.

Don't forget to use the Rare Disease Day hashtag [#ShowYourRare](https://twitter.com/ShowYourRare) on social media. This social media campaign will be active during the entire month of February. More information is available at [www.rarediseaseday.org](http://www.rarediseaseday.org)



# Current Clinical Trial Highlights

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

## [Principia BioPharma, Inc.](#)

**Title:** A Study of PRN1008 in Patients with Pemphigus

**Estimated Primary Completion:**  
December 2021

**Estimated Study Completion:**  
March 2022

**Disease Type:** Pemphigus

**Locations:** United States (FL, NC), Australia (Melbourne, Sydney), Bulgaria

## [Argenx BVBA](#)

**Title:** A Study to Evaluate the Safety, PD, PK, and Efficacy of ARGX-113 in Patients with Pemphigus

**Estimated Primary Completion:**  
March 2019

**Estimated Study Completion:**  
August 2019

**Disease Type:** Pemphigus (Vulgaris and Foliaceus)

**Locations:** Germany, Hungary, Israel, Italy, and Ukraine

## [Incyte Corporation](#)

**Title:** A Study of the Safety and Tolerability of INCB050465 in Pemphigus Vulgaris

**Estimated Primary Completion:**  
August 2020

**Estimated Study Completion:**  
October 2020

**Disease Type:** Pemphigus Vulgaris

**Location:** TBA

## [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 Vulgaris

**Locations:** Australia (NSW, Victoria), Croatia, France, Greece, Israel

## [National Institute of Allergy and Infectious Diseases \(NIAID\)](#)

**Title:** Polyclonal Regulatory T Cells (PolyTregs) for Pemphigus

**Estimated Primary Completion:**  
September 2020

**Estimated Study Completion:**  
September 2020

**Disease Type:** Pemphigus

**Locations:** United States (CA, IA, NC, TX)

## [University of Zurich](#)

**Title:** Observational Study of the Genetic Architecture of Neutrophil-Mediated Inflammatory Skin Diseases (NEUTROGENE)

**Estimated Primary Completion:**  
January 2020

**Estimated Study Completion:**  
January 2020

**Disease Type:** Pemphigus

**Location:** Switzerland

## [University Hospital, Limoges](#)

**Title:** Pharmacokinetics Study of Mycophenolic Acid in Patients with an Autoimmune Bullous Dermatose, Pemphigus or Cicatricial Pemphigoid

**Estimated Primary Completion:**  
March 2019

**Estimated Study Completion:**  
July 2019

**Disease Type:** Pemphigus/Cicatricial Pemphigoid

**Location:** France

## [University of Pennsylvania](#)

**Title:** Autoimmune Blistering Diseases Study (AIBD)

**Estimated Primary Completion:**  
December 2018

**Estimated Study Completion:**  
August 2019

**Disease Type:** Pemphigus Vulgaris/Foliaceus, Bullous Pemphigoid

**Location:** United States (Pennsylvania)

## [Mayo Clinic](#)

**Title:** Ixekizumab in the Treatment of Bullous Pemphigoid

**Estimated Primary Completion:**  
July 2019

**Estimated Study Completion:**  
December 2019

**Disease Type:** Pemphigoid

**Location:** United States (Arizona)

## [Assistance Publique Hopitaux De Marseille](#)

**Title:** Dipeptidyl Peptidase-IV Inhibitors, Risk Factor for Development of Bullous Pemphigoid?

**Estimated Primary Completion:**  
March 2019

**Estimated Study Completion:**  
December 2019

**Disease Type:** Bullous Pemphigoid

**Location:** France

## [TWi Biotechnology, Inc.](#)

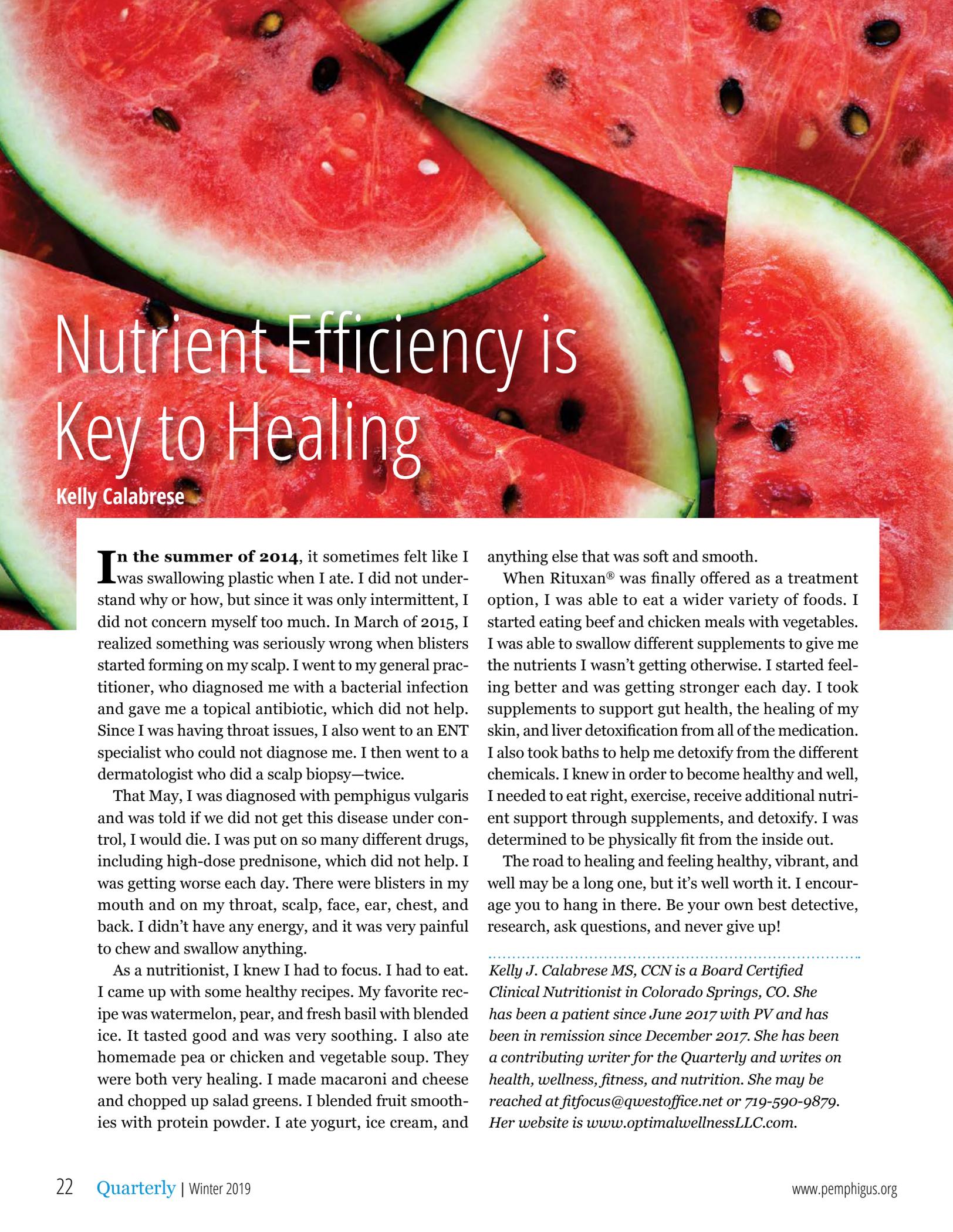
**Title:** A Proof-of-Concept Study of Topical AC-203 in Patients With Bullous Pemphigoid

**Estimated Primary Completion:**  
March 2019

**Estimated Study Completion:**  
May 2019

**Disease Type:** Bullous Pemphigoid

**Location:** Taiwan

A close-up photograph of several slices of watermelon, showing the bright red flesh, dark seeds, and green rind. The slices are arranged in a slightly overlapping manner, filling the entire background of the page.

# Nutrient Efficiency is Key to Healing

Kelly Calabrese

**I**n the summer of 2014, it sometimes felt like I was swallowing plastic when I ate. I did not understand why or how, but since it was only intermittent, I did not concern myself too much. In March of 2015, I realized something was seriously wrong when blisters started forming on my scalp. I went to my general practitioner, who diagnosed me with a bacterial infection and gave me a topical antibiotic, which did not help. Since I was having throat issues, I also went to an ENT specialist who could not diagnose me. I then went to a dermatologist who did a scalp biopsy—twice.

That May, I was diagnosed with pemphigus vulgaris and was told if we did not get this disease under control, I would die. I was put on so many different drugs, including high-dose prednisone, which did not help. I was getting worse each day. There were blisters in my mouth and on my throat, scalp, face, ear, chest, and back. I didn't have any energy, and it was very painful to chew and swallow anything.

As a nutritionist, I knew I had to focus. I had to eat. I came up with some healthy recipes. My favorite recipe was watermelon, pear, and fresh basil with blended ice. It tasted good and was very soothing. I also ate homemade pea or chicken and vegetable soup. They were both very healing. I made macaroni and cheese and chopped up salad greens. I blended fruit smoothies with protein powder. I ate yogurt, ice cream, and

anything else that was soft and smooth.

When Rituxan® was finally offered as a treatment option, I was able to eat a wider variety of foods. I started eating beef and chicken meals with vegetables. I was able to swallow different supplements to give me the nutrients I wasn't getting otherwise. I started feeling better and was getting stronger each day. I took supplements to support gut health, the healing of my skin, and liver detoxification from all of the medication. I also took baths to help me detoxify from the different chemicals. I knew in order to become healthy and well, I needed to eat right, exercise, receive additional nutrient support through supplements, and detoxify. I was determined to be physically fit from the inside out.

The road to healing and feeling healthy, vibrant, and well may be a long one, but it's well worth it. I encourage you to hang in there. Be your own best detective, research, ask questions, and never give up!

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*Kelly J. Calabrese MS, CCN is a Board Certified Clinical Nutritionist in Colorado Springs, CO. She has been a patient since June 2017 with PV and has been in remission since December 2017. She has been a contributing writer for the Quarterly and writes on health, wellness, fitness, and nutrition. She may be reached at [fitfocus@qwestoffice.net](mailto:fitfocus@qwestoffice.net) or 719-590-9879. Her website is [www.optimalwellnessLLC.com](http://www.optimalwellnessLLC.com).*

# The PEGASUS Study in Pemphigus is now enrolling.

Principia is conducting a Phase 3 clinical research study of a novel investigational drug (PRN1008, an oral pill.) This study is being done to see if PRN1008 plus corticosteroid is safe and effective in helping PF/PV patients to achieve remission, and to test the ability of PRN1008 to reduce corticosteroid use.

PRN1008 is a Bruton's tyrosine kinase (BTK) inhibitor and is an investigational drug. PRN1008 may work within your immune system to block the autoimmune process that leads to pemphigus vulgaris (PV) and pemphigus foliaceus (PF).

PRN1008 has been tested in approximately 140 healthy volunteers (up to 11 days of treatment), and in 27 patients with PV or PF (up to 3 months of treatment) in an ongoing Phase 2b study.



## STUDY DETAILS

Over 18 years of age.

Newly diagnosed or relapsing PV and PF patients with moderate to severe disease.

Length of the study for each patient is about 17 months.

For more information, talk to your dermatologist or contact:

EMAIL: [clinicaltrials@principiabiotech.com](mailto:clinicaltrials@principiabiotech.com)

PHONE: 1-833-477-6700



**PRINCIPIA**



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The *Quarterly* is made possible in part by a grant from Incyte



# #ShowYourRare

## for Rare Disease Day 2019



[RAREDISEASEDAY.ORG](http://RAREDISEASEDAY.ORG)

28 February