

NEW IPPF BOARD CHAIR | TALKING ABOUT YOUR DIAGNOSIS | CLOSER LOOK: FJORD STUDY

Quarterly

Journal of the International Pemphigus & Pemphigoid Foundation

FALL 2021

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Message from the Executive Director



First of all, WOW! We just finished a very successful 2021 Patient Education Conference, with more than 760 registrants from 46 countries and over \$8,000 raised in donations. This was likely the largest pemphigus and pemphigoid patient (P/P) event ever, and it couldn't have been conceived, planned, and delivered without a concerted effort by a large number of people and organizations. I will likely omit someone, for which I apologize, but our sincerest thanks to:

- Our sponsors, whose financial commitment made it possible to make this event free for attendees, and whose presence added to the quality of the conference.
- The medical professionals who spoke, who gave up part of their weekend to ensure that the latest information was delivered in a digestible format to attendees.
- The IPPF volunteers, peer health coaches, local support group leaders, Board of Directors members, and others who spoke passionately about their experiences and provided outstanding advice.
- The IPPF staff—both visible and behind the scenes—who worked hard to deliver for all attendees.

Most of all, we thank our attendees. The level of involvement, quality of questions, and post-event feedback made it all so very worthwhile.

So, what's next? Through our peer health coaching program, Patient Education Series webinars, and publications, we will continue to develop the latest P/P knowledge and information and bring you an outstanding 2022 event.

Connecting with each other during the conference was a reminder of the different ways in which the IPPF community contributes. As I mentioned, many of you made a financial contribution, which is always welcome. Others gave their time or talents. I encourage everyone to think about how you can help to further develop this amazing community as we continue to grow.

Kevin Mead, IPPF Executive Director
kevin@pemphigus.org



Kevin Mead

The 2021 IPPF Scientific Symposium (September 19-21) featured three days of live sessions and abstract discussions. The event, held in cooperation with the Pegasus Project and Philipps-Universität in Marburg, Germany, was a tremendous success. Of course, as with so many things in “the age of COVID,” the planning process was somewhat complex. The symposium was originally envisioned as an in-person event in 2020, and instead it became a virtual event in 2021. But what an event it was! There were nearly 250 participants from across the world and close to 30 sessions, which included the following major research areas:

- **Skin homeostasis and loss of adhesion**
- **Diagnostic criteria of pemphigus and pemphigoid: practice gaps**
- **Epidemiology and genetics**
- **Impaired adhesion: mechanisms**
- **Adaptive and innate immune responses in autoimmune bullous diseases**
- **Treatment: from current evidence to future perspectives**

Additionally, each subject area also saw abstract and poster presentations, many from researchers new to the field. For the posters, there were 46 submissions from 10 different countries.

The feedback was overwhelmingly positive, with new relationships being formed despite the issues created by not being together in person.

A number of abstract and poster prizes were awarded for the presentations seen as the best in advancing knowledge.

Our gratitude to the organizing committee and the leadership of Dr. Michael Hertl, professor in the Department of Dermatology and Allergology at Philipps-Universität in Marburg, Germany, in developing the outstanding program. Also, a sincere thanks for the organizational skills of Dr. Daniel Becker, Project Coordinator in the Department of Dermatology at Philipps Universität in Marburg, Germany, in delivering the program.

Kevin Mead joined the IPPF as the Executive Director in October 2020. He has over 25 years of experience in association management, fundraising, and project management.

SCIENTIFIC SYMPOSIUM AWARDS

BEST POSTERS

JI YEON HONG (SEOUL)

DWP212525, a novel JAK3 and Tec family kinase inhibitor, has the ability to reduce the severity of PV disease through autoantibody inhibition

MICHAEL FUCHS (MUNICH)

Dsg2 is upregulated in pemphigus and undergoes functionally different interactions with desmosomal and classical cadherins

BEST ORAL ABSTRACTS

DAVID CHANG (PHILADELPHIA)

A phase 1 trial of desmoglein 3 chimeric autoantibody receptor T cells (DSG3-CAART) for targeted B cell depletion in patients with mucosal-dominant pemphigus vulgaris

ROBERTA LOTTI (MODENA/MILAN)

PC111, a monoclonal anti-Fas Ligand antibody, blocks blister formation in human pemphigus

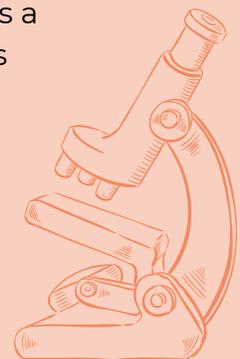


IPPF

Scientific Seminar Webinar Series

In close cooperation with Drs. Ralf Ludwig & Katja Bieber of the Lübeck Institute for Experimental Dermatology at the University of Lübeck, the IPPF Scientific Seminar Series is a scientific educational program with experts from various international institutions who present on their scientific findings on pemphigus and pemphigoid. Content will highlight recently published and/or unpublished data in both basic and translational research.

Find upcoming topics and dates at
www.pemphigus.org



The IPPF Announces New Chair to the Board of Directors



On behalf of the IPPF Board of Directors and staff, we are excited to announce that Carolyn Fota has been appointed as our new Board of Directors Chair. Carolyn has served as an IPPF Board member since this past summer. She has also been active in the IPPF community through peer health coaching, Mid-Atlantic Support Group leadership, advocating on Capitol Hill to our nation's leaders, and participating in various IPPF projects whenever asked. She resides in Stafford, Virginia, with her husband of over 27 years, Francis. She also enjoys golf, walking, church activities, and writing.

Additionally, we would also like to thank Mirella Bucci, PhD, and Mert Eroglu, MD, for their time and service on the Board of Directors as they step down from service.

FUNDING UPDATE



THE IPPF RECEIVES A RARE DISEASE WEEK SCAVENGER HUNT GRANT

Throughout **Rare Disease Week 2021**, the EveryLife Foundation for Rare Diseases challenged advocates to engage and participate by earning points in a scavenger hunt. Throughout the week, advocates were awarded points for attending events, meeting with their legislators, and other actions.

EveryLife recently announced that the top 50-point earners were each awarded a grant ranging from \$1,000 to \$5,000 for the rare disease non-profit organization of their choice! EveryLife awarded a total of \$100,000 to 36 rare disease non-profit organizations in the name of these amazing advocates.

Rare Disease Legislative Advocates appreciates the participation of all rare disease advocates at Rare Disease Week! Not only did the participation of these advocates help to expand the voice and reach of our collective rare community—as well as to raise awareness for their respective disease communities—but their active engagement throughout the week also **RAISED FUNDS** for their organizations.

Together, IPPF rare patient advocates secured a \$5,000 grant from the EveryLife Foundation for their efforts during Rare Disease Week 2021.

IPPF AWARDED \$50,000 OPERATIONAL GRANT FROM THE CHAN ZUCKERBERG INITIATIVE

The IPPF is grateful to share that we have received a one-time, \$50,000 grant from the Chan Zuckerberg Initiative in support of accelerating research and finding treatments and cures for pemphigus and pemphigoid.

Recently, the Chan Zuckerberg Initiative (CZI) announced \$13 million in funding for 40 patient-led, rare disease advocacy organizations that are working alongside researchers and clinicians to accelerate research in their disease areas. These grants are part of CZI's Rare As One (RAO) Project, aimed at supporting and lifting up the work that patient communities are doing to drive progress in the fight against rare diseases.

Rare disease is not rare. As many as 7,000 rare diseases affect 400 million people globally. The Rare As One Project is committed to uniting rare disease patient advocates in their quest for cures.

To read more about the funding and CZI's Rare As One Project, visit <https://chanzuckerberg.com/>



A Closer Look at the Fjord Study

A CLINICAL TRIAL TO STUDY BENRALIZUMAB FOR THE TREATMENT OF BULLOUS PEMPHIGOID



Skylar Sever, RN: Clinical Trial Educator, IQVIA

Catherine Datto, MD, MS : Executive Director, Clinical Development, AstraZeneca

Lila Bahadori, MD: Global Clinical Product Leader, AstraZeneca

Maria Bergquist, PhD: Global Development Scientist Director, AstraZeneca

What is the Fjord Study?

The Fjord study is a Phase 3, global clinical trial being conducted by AstraZeneca AB in the United States, Japan, Australia, Spain, Germany, Italy, Bulgaria, Israel, China, Greece, and France. One hundred twenty participants with symptomatic bullous pemphigoid (BP) will be participating in this study.

What is the main objective of the Fjord study?

The Fjord study is designed to compare how well benralizumab works versus placebo (looks like benralizumab, but is not an active medicine) in participants with symptoms of BP who are prescribed oral steroids. The ability of benralizumab-treated patients to be without signs of BP for two months, while also no longer needing oral corticosteroids, will be compared with that of placebo.

Why is this study being conducted?

While BP is the most common of the pemphigoid family of diseases, it is considered a rare disease with an estimated 2 to 22 diagnosed cases per 1 million people annually. An accumulation of the past 20 years of

research reflects a 1.9-to-4.3-fold increase in BP cases. The trend in the increasing BP incidence could well continue in years to come (Kridin and Ludwig 2018, 1).

The usual age for patients with BP is between 66 and 83 years, and incidence in patients over 80 increases to 190 to 312 new cases per 1 million people (Kridin and Ludwig 2018, 1). Another point worth recognizing is that a growing body of evidence indicates that neurological conditions such as Alzheimer's disease, dementias, multiple sclerosis, and stroke are associated with BP (Schmidt and Zillikens 2013, 321).

The most common signs of BP are chronic and recurring tense blisters, redness, and inflammation leading to erosions and crusting of the skin. Persistent and severe itch is one of the most distressing symptoms (Amber et al. 2018, 1, Schmidt and Zillikens 2013, 321). Standard frontline treatment for moderate to severe, widespread BP starts with oral (taken by mouth) and topical (applied to the skin) corticosteroids (Feliciani et al. 2015, 872). These frontline treatments do not come without the risks of possible adverse effects on various organ systems (Schäcke, Döcke and Asadullah 2002). Adjunctive therapies, treatments that are used in

addition to the frontline treatment, are often prescribed and include immunosuppressants and anti-inflammatory agents. These are prescribed for difficult to treat BP; however, there is limited data demonstrating how well these medicines treat BP, and they come with their own set of side effects (Feliciani et al. 2015, 872).

A major problem in managing BP disease is relapse, or a persistent return of the disease. A relapse generally results in the need to restart frontline corticosteroids again along with their adverse effects.

Combining these factors—unbearable symptoms, a rare disease, an increasing incidence, age, comorbidities, adverse effects of frontline treatment, and recurrent relapse—it is clear there is an unmet need for a better treatment of BP. A treatment is needed that improves patient quality of life, minimizes dependency on oral corticosteroids, and decreases BP relapses.

There is an increasing body of evidence for eosinophils as a potential target for the treatment of BP. An important factor in BP is the role of eosinophils, which are a type of white blood cell that fight infections as part of our immune system. Research supports a role for eosinophils in the blister formation in BP (Amber et al. 2018, 1, de Graauw et al. 2017). Furthermore, a direct correlation between blood eosinophil levels and disease severity has been repeatedly observed and reported (Kridin and Ludwig 2018).

What is benralizumab?

Benralizumab is a monoclonal antibody that lowers the number of eosinophils in the blood and tissue. Benralizumab binds to eosinophils and attracts specific cells in the immune system to remove them from the body naturally. Benralizumab does this by binding to a receptor (Interleukin-5 receptor, or IL-5Ra) (Lavolette et al. 2013, 1087).

Previous clinical studies have demonstrated the effectiveness of benralizumab at depleting levels of eosinophils in patients diagnosed with eosinophilic asthma (Bleecker et al. 2016, FitzGerald et al. 2016). It is approved for use as a medication for the treatment of moderate to severe asthma in the US, Japan, and countries within the European Union.

The Fjord study is designed to study benralizumab to see if it is effective at controlling BP. Benralizumab is not currently approved by the FDA for use in BP treatment.

Who can participate in the Fjord study?

Below are some of the main criteria for participation in this clinical trial.

Adults 18 years or older with clinical features of classic BP who have a confirmed diagnosis of BP either once participating or prior to participation in the study. Participants must meet a certain level of BP disease severity, confirmed through an assessment called the Bullous Pemphigoid Disease Activity Index (BPDAI). Participants must be taking or able to take systemic corticosteroids.

Participants cannot have any other unstable health conditions that the study doctor thinks would impact participant safety or their ability to participate in the study. This includes any known immune deficiency disorder or positive HIV test.

Exclusionary medications and treatments of note include:

- Immunosuppressives within 30 days before participating in the study
- Immunoglobulin or blood products within 30 days prior to participating in the study
- Other biologic within four months (or longer depending on the biologic) prior to participating in the study
- Benralizumab or Fasenra®
- Concurrent enrollment in another clinical trial

Additionally, women cannot be pregnant or breastfeeding during the study.

What does participation in the Fjord study look like?

Interested and potentially eligible patients would attend an initial visit to see if they qualify for the study. If accepted into the study, there is a 36-week treatment period where patients would either receive the study drug or placebo. This study is a 1:1 placebo controlled, double-blinded study. This means that half of the patients will receive benralizumab and half will receive a placebo during a 36-week treatment period. A placebo looks just like benralizumab but does not contain active medicine. Double-blind means that neither the participant nor the study staff will know if a participant is receiving placebo or benralizumab. (Note: The study doctor can easily find out this information if needed for safety reasons.) After the 36-week treatment period,

all participants can enter the open-label period and receive the study drug until the study closes (at minimum one year after the 36-week treatment period) or until participation terminates.

Participants in this study would undergo regular study visits where certain assessments would take place including vital signs, blood samples, urine samples, skin biopsies, skin photography, health history and physical examination, all at no cost to the participant. For safety, women of child-bearing potential will have to agree to certain birth control requirements and will be routinely tested for pregnancy.

A note about clinical trial participation

When considering participation in a clinical trial, it is important to make a fully informed decision. Individuals can ask questions of the study doctor, study staff, their healthcare provider, as well as trusted family and friends. It is important to understand the risks and compare them to any benefits that the study may provide. It is also important to recognize that participation is always voluntary and can be terminated at any time. Participant safety is always a priority, so study doctors may decide that enrolling in a clinical trial is not in a patient's best interest.

To learn more about this study please call +1 855-233-1701 to speak with a clinical trial educator.

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Conflict of Interest

Skyler Sever is a Clinical Trial Educator at IQVIA who is contracted by AstraZeneca on the Fjord Study. Her role is to educate about clinical research participation and build study awareness.





Results of Mental Health and AIBD Study Published in the Journal of the European Academy of Dermatology and Venereology

Marc Yale

In the fall of 2020, the International Pemphigus & Pemphigoid Foundation (IPPF) teamed up with researchers at The University of Illinois at Chicago to conduct a study on assessing the risk factors for anxiety, depression, and post-traumatic stress disorder for patients with autoimmune blistering skin diseases (AIBD) such as pemphigus and pemphigoid (P/P).

The study found that almost half of the patients that responded had both anxiety and depression. It also showed that patients not taking systemic corticosteroids were found to have a lesser risk of developing depression. The survey illustrated that symptoms of AIBD correlate with a higher prevalence of anxiety, depression, and post-traumatic stress disorder in many patients. These could be attributed to pain and discomfort from the chronic disease, associated social stigma, and lifestyle changes needed by patients.

The IPPF would like to thank the 422 patients that participated in the research to help us better understand the impact that P/P have on the mental health and well-being of those affected. We would also like to acknowledge the research community for their continued interest in autoimmune bullous diseases. The study was recently accepted and published in the Journal of the European Academy of Dermatology and Venereology. Please contact the IPPF at info@pemphigus.org for more information.

Marc Yale was diagnosed in 2007 with cicatricial pemphigoid. In 2008 he joined the IPPF as a peer health coach. He became executive director in 2016 and recently started his role as IPPF research and advocacy coordinator. Marc currently resides in Ventura, CA, with his wife Beth and his daughter Hannah.



Anna Lane

IPPF Advocacy Ambassadors, dermatologists, and other advocates met with congressional members during the American Academy of Dermatology Association’s (AADA) Virtual Legislative Conference in September. The AADA Legislative Conference is an important meeting focused on legislative, regulatory, and political issues that affect dermatology. Dermatologists, patient group leaders, practice administrators, and political insiders participated, and the goals of the conference focused on the following:

1. Providing participants with a unique opportunity to learn about the AADA’s advocacy process and how health policy affects dermatology practices and patients every day
2. Boosting attendees’ ability to advocate on behalf of the specialty through participation in interactive training sessions
3. Positioning participants as the experts on issues that are important to the specialty by providing the unique opportunity for attendees to meet with members of Congress

IPPF Advocacy Ambassadors’ congressional meeting goals focused on the support of the Safe Step Act (H.R.

2163/S. 464). Step therapy, also known as “fail first,” is a process used by health insurers to control costs. It requires patients to try one or more medications specified by their insurer to treat a health condition. These medications typically cost the insurance company less. Patients must fail on this medication(s) before receiving approval to “step up” to another medicine that may work to better manage the patient’s health. This often leads to delays in patients receiving the treatment prescribed by their doctor, and this is especially prevalent in dermatology patients.

H.R. 2163 and S. 464 limit, but do not eliminate, step therapy protocols required by health insurance plans. These bills provide a transparent override process for doctors and patients to get answers in a timelier manner and for patients to access necessary treatment.

For more information about step therapy and how to get involved by contacting your congressional members, visit the Derma Care Access Network’s advocacy toolkit available at:

<https://www.dermacareaccess.org/advocacy-toolkit>.

Anna Lane is the IPPF Communications and Marketing Manager. She lives in Denver, CO, with her family.

2021 RareVoice Award Finalists



MARC YALE

Last month, the rare disease community nominated advocates who give rare disease patients a voice on Capitol Hill and in state government for the Rare Disease Legislative Advocates (RDLA) annual RareVoice Awards. After deliberation by the 2021 annual RareVoice Nominations Committee, RDLA has officially announced the finalists for the 2021 RareVoice Awards and opened registration to all who wish to attend this exciting virtual event. At the RareVoice Awards, one nominee from each category will be named the awardee and sent an “Abbey” statuette commissioned specially for the RareVoice Awards, and named for Abbey Meyers, founder of the National Organization for Rare Disorders (NORD).

The awardee for each category will be announced live at the virtual RareVoice Awards on December 15, 2021. The evening will celebrate rare disease advocates who make their voices heard year-round to advance policies that benefit the rare disease community. To join the celebration, visit www.RareVoiceAwards.org.

We are excited to share that Marc Yale, IPPF Advocacy & Research Coordinator, and Hannah Yale, Rare Disease Advocate, were both nominated in separate RareVoice Award categories.

Marc was nominated in the “Federal Advocacy-Patient/Organization” category, which honors advocates or organizations that have worked to create and pass federal legislation.

Marc Yale was diagnosed in 2007 with mucous membrane pemphigoid. Like others with a rare disease, he experienced delays in diagnosis and difficulty finding a knowledgeable physician. This inspired him



HANNAH YALE

to help others with the disease. In 2008, he joined the International Pemphigus and Pemphigoid Foundation as a peer health coach. In 2009, he helped develop a comprehensive disease profile giving experts insight into the patient perspective. In 2016, Marc became the executive director of the IPPF, and he now serves as the advocacy & research coordinator focusing on research and advocacy for all of those affected by pemphigus and pemphigoid. He is a member of the American Academy of Dermatology Drug Transparency Task Force, sits on the Executive Board of Directors for the International Alliance for Dermatological Patient Organizations, serves on the RDLA Advisory Committee, and is a board director for the Haystack Project.

Hannah Yale was nominated in the “Federal or State Advocacy by a Teenager” award category, which honors teens who have advocated for state or federal legislation.

Hannah has been an advocate for the EveryLife Foundation and the International Pemphigus and Pemphigoid Foundation since 2017. She has attended RDLA’s Rare Disease Week on Capitol Hill annually since 2017, and she is also a member of the Young Adult Representatives of RDLA. In 2020, Hannah served on the Funding Committee for Living in the Light’s “I Stay Home for Rare” Emergency COVID-19 Relief Fund. Hannah is living with Ehlers-Danlos Syndrome, although she began her rare disease advocacy to support her father, Marc, and her mother (who also has a rare disorder). Hannah is currently a student at St. Mary’s College of Maryland, where she is majoring in public policy and minoring in English and philosophy.

Four Questions with Dr. Nasser Said-Al-Naief

Our Spotlight section features a medical professional whose work regularly impacts the lives of pemphigus and pemphigoid patients. Get to know a new physician, researcher, or other medical professional who knows these diseases best. This issue, we're featuring Dr. Nasser Said-Al-Naief.

Dr. Said-Al-Naief is an IPPF medical advisory member. He is currently a professor of oral and maxillofacial pathology at the Department of Integrated Biomedical and Diagnostic Sciences at Oregon Health & Science University (OHSU) School of Dentistry and School of Medicine. He recently served as a professor, chair, and laboratory director at the Department of Pathology and Radiology at OHSU. Dr. Said-Al-Naief has also served on the faculty at Loma Linda University School of Dentistry; Loma Linda University School of Medicine; the University of Pacific; the University of Alabama Birmingham School of Medicine; and Marquette University. Dr. Said-Al-Naief earned his DDS degree from Marquette University, his MS degree from the University at Illinois, Chicago. Dr. Said-Al-Naief completed an advanced fellowship at Mount Sinai Medical Center. He has authored and co-authored numerous articles, manuscripts, abstracts, chapters, and posters on various topics in head and neck pathology and three chapters in Maxillofacial / Head and Neck Pathology, including the most recent WHO classification of head and neck tumors in 2016.

How did you become interested in pemphigus and pemphigoid (P/P)?

This is a great question. My interest began when I had practiced general dentistry for only a few years and noticed the extreme difficulty in soft tissue management and in performing routine preventive dental and oral hygiene work and executing tasks in general. My interest and enthusiasm grew when I became a pathologist and further understood the limitations P/P patients have in everyday life, and how we as clinicians



can make a difference in the patient's life, health, and overall wellbeing.

What is one thing you'd want all patients to know early on in their journey with P/P?

You will find difficulty and hardship during flare-ups of the diseases, but treatment, recommendations, and emotional support are available from different resources, including the IPPF. Always be optimistic, and have faith that things will be better.

What can patients do to better advocate for themselves?

[They could become more] involved and engaged within societies, support groups, and forums that serve to spread awareness about the diseases and their complications and management modalities, as well as [help patients learn] how to cope during daily activities. This leads to more awareness and more support from the government and private societies for social, referral, and research support.

What is one fun fact about yourself?

If you are having a bad day, I will make you feel better, always.

Talking About Your Diagnosis

YOU ARE NOT ALONE

Mei Ling Moore

It may have taken weeks, months, or years and countless doctors and tests to find out why you've been experiencing a painful event in your life before receiving a pemphigus or pemphigoid diagnosis. Hopefully you've had support during your diagnostic journey to share in the frustration, anxiety, anger, and fear that you have been going through.

Upon receiving a diagnosis of pemphigus or pemphigoid, you may have gone back to your car and felt numb for what seemed like an eternity. You may have cried due to the relief of finally having a name for your experience, then worried about what might happen next. How do you tell those closest to you about your disease? What about your coworkers or employer?

You may feel anxious about the visual effects of the disease and what friends, family, coworkers, and even strangers think. It's possible that you aren't able to sleep at night because you're so worried about information you found online about pemphigus and pemphigoid (P/P).

Here are some tips for talking with your family, friends, and even employers about your diagnosis:

- When you feel ready to share your diagnosis, you may explain what you've learned, that you are working (or in the process of getting in touch with) an experienced P/P professional, and how you are feeling.
- Ask a family member or friend to accompany you to your appointments. They can provide moral support and assist you with taking notes so that you can focus on getting answers to your questions.
- Let them know about the IPPF. The IPPF has an array of patient resources through the Peer Health

Coach Program, Patient Education Series Webinars, Find a Doctor Map, Peer Support Groups, social media, and more. The IPPF will help you feel less alone during this challenging time.

- If you have blisters in visible areas and don't want to provide a detailed explanation to a friend, it might be easier to explain that you're experiencing issues with your skin similar to an allergy. Autoimmune diseases are complex, and each individual may experience different symptoms.
- If you have concerns about discussing your diagnosis and/or condition with your employer, there are resources available on the US Department of Labor website related to the Family and Medical Leave Act (FMLA) and the Americans with Disabilities Act (<https://www.dol.gov/>). In the USA, individual states have varying laws and guidelines. For example, California provides information about the Fair Employment and Housing Act (FEHA) related to employee rights on the Department of Fair Employment and Housing website: <https://www.dfeh.ca.gov/employment/>.

Dealing with a recent, often complicated, diagnosis is not always easy. You may feel ready to start talking to people about your diagnosis right away, or it may take some time. Regardless of the choice you make, the IPPF is here for you.

Mei Ling Moore was diagnosed with PV in 2002. She has been a peer health coach with the IPPF since 2012. She also organizes the Southern California Support Group with Marc Yale. Mei Ling lives in Los Angeles.

Cabaletta Bio™

Clinical Data from the Third Dose Cohort in DesCAARTes™ Trial in Patients with mPV

Cabaletta Bio, Inc., a clinical-stage biotechnology company focused on the discovery and development of engineered T cell therapies for patients with B cell-mediated autoimmune diseases, recently announced 28-day clinical data from the third dose cohort using 500 million DSG3-CAART cells in the DesCAARTes™ Phase 1 clinical trial for the treatment of patients with mucosal-dominant pemphigus vulgaris (mPV).

As of October 31, 2021, three patient cohorts in the DesCAARTes™ Phase 1 trial have completed DSG3-CAART dosing. The Company observed a dose dependent increase in DSG3-CAART persistence in the third cohort relative to the first two low dose cohorts throughout the 28 days following infusion. In addition, no clinically relevant adverse events or DLTs were observed during the 28-day monitoring period post-infusion. These safety data were observed without preconditioning, and in the presence of circulating anti-DSG3 antibodies.

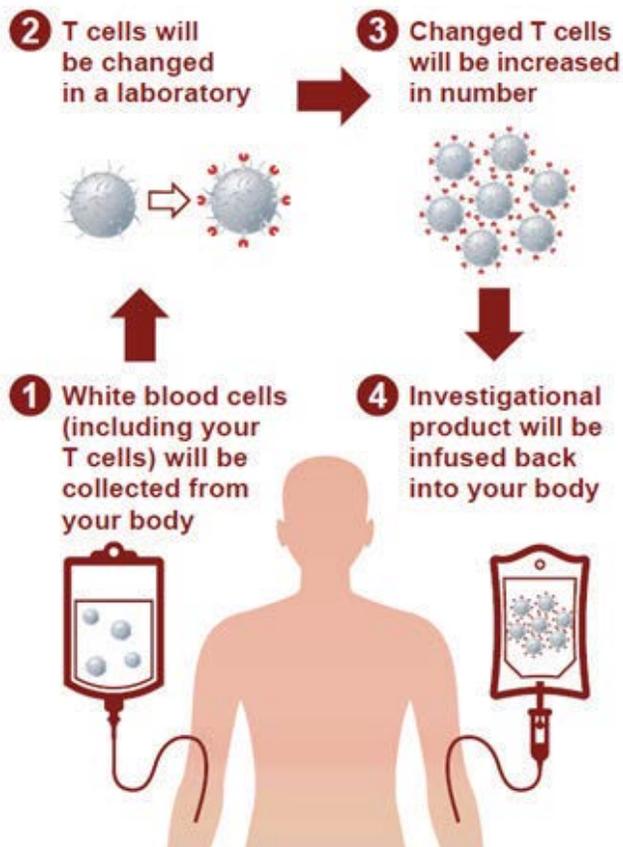
“We are highly encouraged by the observation of dose dependent increases in persistence as well as the continued absence of any DLTs or clinically relevant adverse events for DSG3-CAART across the first three

cohorts, particularly in the presence of circulating anti-DSG3 antibodies and without lymphodepletion,” said David J. Chang, M.D., Chief Medical Officer of Cabaletta. “The rapid pace of the clinical trial has been possible due to the enthusiasm and engagement of patients, investigators and patient advocacy groups. With a 100% manufacturing success rate to date, we look forward to continuing to advance the trial until we identify a maximum tolerated dose and dosing regimen that has the potential to achieve a durable response while maintaining a favorable tolerability profile for patients suffering with mPV.”

As of October 31, 2021, three additional clinical sites have opened for recruitment, doubling the total number of activated DesCAARTes™ trial sites to six. Dosing of patients in the fourth cohort at a treatment dose of 2.5 billion DSG3-CAART cells has been initiated. The Company anticipates announcing 28-day safety data for the fourth dose cohort in the first quarter of 2022.

Top-line biologic activity data for the first two low dose cohorts are anticipated to be announced in the fourth quarter of 2021.

Update on the DesCAARTes™ Trial in Patients with Mucosal Pemphigus Vulgaris



What investigational product is being studied?

- DSG3-CAART is the investigational product used in this study, and it will be made from your own T cells
- A T cell is type of immune cell called a white blood cell. White blood cells fight against invaders that enter the body
- Your T cells will be changed (genetically modified) in the laboratory so that they will attack and kill “bad” B cells that cause mucosal pemphigus vulgaris in your body

DSG3-CAART: An Experimental Targeted Cell Therapy

- DesCAARTes™ is a Phase 1, 1st human trial to determine the highest tolerated dose and evaluate the safety of DSG3-CAART
- We are studying if infused DSG3-CAART can eliminate the “bad” B cells that make DSG3 autoantibodies, sparing normal B cells, and be used to treat mucosal pemphigus vulgaris and how long it might work
- This study is believed to be the 1st cell therapy clinical trial specifically designed to target the disease-causing B cells in patients with an autoimmune disease
- The 1st two groups of 6 patients experienced no safety concerns with DSG3-CAART
- As a targeted therapy, DSG3-CAART has the potential to avoid chronic immunosuppression
- DSG3-CAART has the potential to be a one-time treatment that leads to a long-term response for patients with mucosal pemphigus vulgaris

To Learn More about the Study or Have Questions

Cabaletta Bio Clinical Trials Website at <https://www.cabalettabio.com/patients/descaartes-phase-1-trial>

Patient Story

KATE SPROUL

Kate Sproul

In the fall of 2017, I spat out blood every time I brushed my teeth. I had sores on my gums, and it hurt when I ate hard or spicy foods. In August and September of that year, I had some dental work done, and the dentist had not seen anything wrong with my gums. When I went back to the dentist, he guessed that I had either gingivitis or lichen planus, and he gave me some chlorohexidine to rinse with. When that did not help the problem, he sent me to a periodontist. After both oral and topical steroids failed to heal the sores, I was sent to another periodontist who did a biopsy. I was diagnosed with pemphigus vulgaris (PV) on March 27, 2018.

I hadn't ever heard of PV. None of my family or friends had it. I did a Google search, and www.pemphigus.org came up immediately. I was incredibly relieved to find out that not only did other people have this disease, but many of them were also helping other people get through it. First, I read the concise and scientific description of PV, and I was grateful to learn exactly what I was dealing with. I then read some of the stories of people who had the disease for years before they were correctly diagnosed and was thankful for

my own relatively fast diagnosis. Then I searched the Find a Doctor map to find a physician in the Nashville area. I spent quite a bit of time exploring the website, absorbing what information I could, and making a note to look at the rest of the information when I felt less overwhelmed.

A couple of days later, I received an email from an IPPF peer health coach asking if I had any questions or needed help. I was so impressed and grateful to get that email! Even though I was recently diagnosed and unsure about what to ask, I was so happy to know that there was someone who was ready to talk to me.

The first IPPF Patient Education Conference that I attended in 2018 was a fabulous experience. Even though most in attendance were diagnosed with a serious rare disease, everyone was very happy and welcoming, including the patients who got up in front of all of us and shared harrowing stories about their lives with P/P before they were properly diagnosed. I was amazed at the courage of the people who told us the personal details of being so ill, and I was astounded that they were walking around and looking incredibly healthy. It was a sobering look at what I could have

experienced and a joyful realization that even if my condition worsened, there were people there who had recovered and been in remission for 10, 15, or even 20 years.

Besides real-life stories, we were given a lot of practical advice that I would never have heard from my doctor, such as staying away from toothpastes, shampoos, conditioners, soaps, etc., with sodium laurel sulfate; choosing a Waterpik® with different speeds so that the lowest setting can be used on your gums; using a soft child's toothbrush; and using Coban™ wrap instead of band-aids.

I also heard, formally and informally, that there is not a specific diet plan that will cure P/P, but that each individual has to determine what works for them. Some people can eat onions at any time, while others will get a flare if they even taste one. The peer health coaches suggested keeping a food diary to determine which foods made our symptoms worse. This type of advice showed us what we could do to help control our disease besides just taking our medications.



Even though I was recently diagnosed and unsure about what to ask, I was so happy to know that there was someone who was ready to talk to me.

I have participated in several patient education webinars, and I have learned something from each one. It is great to be able to ask doctors about medications and their side effects. Also, just listening to some of the questions from other patients has provided me a more complete picture of the various aspects of my disease. On one of the webinars, the peer health coaches answered questions about more practical advice on a variety of topics. Food and diet were discussed quite a bit, as well as tattoos. Their advice was not to get one since we have skin diseases! Since the start of the pandemic, there have been webinars with doctors who are studying COVID-19 and how it affects our community. This demonstrates how much work the IPPF does to give everyone up-to-date information about things that affect our health, including what we can do to keep ourselves healthy during this pandemic.

The best part about the IPPF is that they are ready to help at any time. Peer health coaches share their stories—some of them much sadder and more uncertain than mine—and they do their best to make sure that the next person has a quicker diagnosis, better treatment, shorter amount of time on steroids, and a faster return to a life without

blisters. They have created an environment in which each person who goes through either of these diseases and gets to the other side wants to turn around and help someone behind them.

I encourage everyone who has P/P to get involved with the IPPF. You can connect with others who understand your experience as a patient. You can keep up with the latest clinical trials and approved medications. And you can learn what practical things you can do to improve your health.

Kate Sproul has worked for the Nashville Zoo for the last 19 years, taking care of cattle, sheep, donkeys, goats, alpaca, and kangaroos. She lives in Nashville, TN with her cats.

Connecting at the 2021 IPPF Virtual Patient Education Conference

Anna Lane

The IPPF Patient Education Conference is an event that the pemphigus and pemphigoid (P/P) community looks forward to each year. For the IPPF staff, it's remarkable to see the joy and relief that patients feel when they meet with other patients and learn valuable information about their diseases. When we held our first virtual Patient Education Conference in 2020, our goal was to connect with patients despite the inability to be together in person. Immediately following that event, we started planning the 2021 conference that was held this past October. We knew that the planning process would require us to be flexible due to the COVID-19 pandemic, but we hoped that with vaccines on the horizon, we'd be able to hold an in-person event in 2021.

However, it became clear last spring that an in-person conference was unlikely due to ongoing COVID-19 case numbers and the uncertainty of travel guidelines. Nevertheless, the IPPF staff pored over the planning of another great virtual conference with the goal of connecting P/P patients, caregivers, and others affected by these diseases. Luckily, we had the resources and

experience from planning a prior virtual conference, so we focused on the ways we could improve the 2021 Patient Education Conference.

I often talk to my two young children about silver linings, especially since the start of the COVID-19 pandemic. (Spending time at home together, FaceTiming more often with extended family, and Target drive-up are examples I often give to help ease the pain of events, school, and play dates getting canceled.) One of the silver linings about the virtual conference is the ability to reach more attendees across the world. This year, we were able to reach even more conference attendees than last year, with 762 registered attendees from 46 countries.

The 2021 virtual conference started off with a welcome reception that also included regional support group breakout meetings. Since the start of the COVID-19 pandemic, finding creative ways to connect with each other has been imperative. At the start of the pandemic, IPPF support group leaders throughout the US have done an amazing job of hosting virtual meetings in order to maintain connections. Attendees

at this year's conference had the opportunity to gain more information about these support group meetings, as well as details about starting a group in their area.

The first day of the conference continued with Staci White's remarkable story about her journey to diagnosis. Staci is an IPPF Board of Directors member, and her strength is inspiring. Friday's agenda also included an introduction to P/P, a session on the mind-body connection, and a peer health coach panel discussion.

Day two began with sessions focused on oral care, topical treatments, and managing corticosteroids. Pemphigoid gestationis patient Ashton Brown then explained her harrowing experience with the disease and its effect on her health, pregnancy, and the delivery of her baby boy. Ashton reached out to the IPPF after going viral on TikTok about the condition. The day closed with a general Q&A panel hosted by Dr. Aimee Payne, Dr. Neil Korman, and Dr. Nasser Said-Al-Naief; a session on navigating insurance and Medicare; and an informative discussion on COVID-19 and P/P.

The final day of the conference kicked off with sessions on the role of immunosuppressants, rituximab/IVIg and next generation therapies, and research. IPPF Executive Director Kevin Mead discussed the impact of international support groups in the United Kingdom,

France, Germany, and the exciting work Noel Mudibo is doing in Kenya. The final session of the conference focused on advocacy efforts, including recent legislative efforts and ways to get involved.

The willingness to help one another, even through a computer screen, will always be a silver lining I will remember from the COVID-19 pandemic.

As an IPPF staff member who works primarily behind the scenes, I don't often have the opportunity to see patients and physicians face-to-face. This year, more than ever, I was able to experience that connection and strength within our community. Having a rare disease is hard; no one understands that more than P/P patients. The willingness to help one another, even through a computer screen, will always be a silver lining I will remember from the COVID-19 pandemic. We can't wait until we can all be together in person again, but until that day, the IPPF is here for you.

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Anna Lane is the IPPF Communications and Marketing Manager. She lives in Denver, CO, with her family.



Support Groups

Many P/P support groups are meeting virtually during the COVID-19 pandemic. Learn more about local groups at pemphigus.org

IPPF Participates in the Autoimmune Association's Inaugural Autoimmune Summit



Jim Sliney Jr.

The Inaugural Autoimmune Summit hosted by the Autoimmune Association convened with over 2,000 autoimmune patients virtually from across the country and around the world. The event was held October 15-16, 2021. Ninety-four percent of attendees reported a positive experience, with testimonials from attendees remarking that they “felt even more empowered to be my own advocate” and “felt very seen and heard.” Summit goals for attendees were threefold:

1. Discover tips for managing your autoimmune disease
2. Learn about the latest research in autoimmune diseases and gain an increased understanding of future treatments
3. Connect with autoimmune patients and caregivers and build your autoimmune family

The two-day event had 23 educational and empowering sessions and over 50 speakers, including IPPF Outreach Director Becky Strong, who participated in the Dermatology Breakout session.

Dermatology Breakout: Patient Experience Panel

The Dermatology Breakout session kicked off with a Patient Experience Panel featuring Howard Chang, psoriasis patient advocate/blogger; Alisha Bridges, psoriasis patient researcher; Becky Strong, pemphigus vulgaris patient advocate from the IPPF; and Kathryn Shusta, hidradenitis suppurativa patient advocate. The topics covered included mental health and quality of life, access issues, and diversifying clinical trials. Some highlights from the session are included below.

Mental Health & Quality of Life

Becky mentioned, “My experience is a bit different because my disease happened in my early thirties.” Becky was fit and athletic, but the pain of her disease left her feeling severely run down. It took her a long time to stop blaming her body for “betraying” her. But mental health counseling made a big difference. “I’ve heard others in our community relate this to PTSD,” she said.

After processing her complex emotions through therapy, Becky entered a phase of acceptance, which then led her to advocacy. She asked herself, “How can I help somebody else get diagnosed quicker? How can I share my story and the things I’ve learned so they don’t have to go through the trials I went through?”

Access

The panelists spoke about the challenges they have experienced accessing treatment, and Becky spoke about her experience with step therapy. After going through many medications looking for what would best suit her needs, Becky finally came to her final option, a biologic, but insurance wouldn’t cover it, despite having tried the other available options.

Some tips to manage step therapy and other issues accessing medication included:

- Learn what your insurance covers and doesn’t cover
- Learn the appeals process
- Get comfortable talking to your doctor

Diversifying Clinical Trials

Alisha shared how a lack of diversity in clinical trials can result in mainstream therapies that don’t meet the needs of specific communities of color. “We’re not represented in (phototherapy) studies to learn how much sunlight it would take to show improvement for our skin. Maybe it would take more because of the melanin, but we don’t know that because it hasn’t been properly studied in people of color,” she mentioned.

Alisha also works with the National Psoriasis Foundation’s Citizen Scientist program, where patients can share data about their diseases and compare with others. “Patient research has helped me not only advocate for myself, but to advocate for other minority groups, women, and different age groups because a lot of those studies are heavy with men who are white and older.”

Dermatology Breakout: Clinical Trials and Drug Development

In part two of the Dermatology Breakout session, experts came together to discuss clinical trials and drug development. Panelists included Neda Shahriari, MD,

FAAD, dermatology-rheumatology fellow; Stacie Bell, Chief Scientific and Medical Officer, National Psoriasis Foundation; Bret Ringdahl, Behavioral Sleep Medicine Clinical Psychologist, the Insomnia and Sleep Institute of Arizona.

Clinical Trials 101

Stacie Bell, PhD, opened the panel by walking attendees through the role of clinical trials in treatment development and options. She talked about the four phases of clinical trials and shared that the current clinical trial workflow takes approximately 10-15 years and 2-3 billion dollars!

Treatments on the Horizon

Dr. Neda Shahriari, a dermatologist, opened her remarks with excitement about the future of treatment. “I’m here to discuss some of the newer medications that are in the pipeline for different dermatologic autoimmune conditions. It’s really exciting how far research has come in terms of finding agents that help these different conditions. It seems like not long ago where our hands were tied—we were limited with topical medications or a few oral medications. Clinical trials really pushed the edge and allowed us to have more treatments in our armory for our patients.”

Of note, Dr. Shahriari shared that there is a new biologic being studied in pemphigus vulgaris patients who have failed other treatments, 50 percent of whom saw improvement over time. The most common side effect was headache.

Patient experience in clinical trials

Bret Ringdahl, PhD, talked about his personal experience participating in a clinical trial as a psoriasis and psoriatic arthritis patient. Being involved in that study gave him hope. He advocates for serious consideration of clinical trials. It’s a risk, but the rewards can be great for individuals and the community.

The Autoimmune Association is the world’s leading nonprofit organization dedicated to autoimmune awareness, advocacy, education, and research.

Recordings of the Autoimmune Summit sessions are available now on the Autoimmune Association’s YouTube page.



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