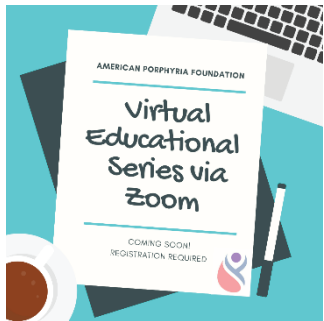




AMERICAN PORPHYRIA FOUNDATION

3rd Quarter, 2020

VIRTUAL EDUCATION MEETINGS



The APF has replaced our typical in-person Patient Education and Support Meetings with a virtual education series via ZOOM! This experience includes patient education meetings, live sessions with Porphyria Experts on various topics and video sessions.

Discussions include an overview of

Porphyria, medical explanations, information about current and emerging treatments, and an extended time for Q&A to answer specific questions.

The sessions to date have been popular! Dr. Cynthia Levy (University of Miami) led a packed patient education meeting in July. Next up was a Patient-to-Patient Resources for Acute Porphyria session in August which including useful resources used by patients and offered by the APF. In August, Dr. Manisha Balwani (Icahn School of Medicine at Mt. Sinai) also led an informative patient education meeting. EPP Shadow Jumpers gathered to learn and engage

at the end of the month, including sessions for <18, young adults and a special session for parents to learn from Dr. Amy Dickey (Massachusetts General Hospital.)

If you are interested in joining one or more of the next scheduled session, please email Edrin Williams, APF Director of Patient Services, at edrinw@porphyriafoundation.org to receive the registration link.

UPCOMING DATES AND TOPICS:



- ✓ **Heme Basics with Dr. Phillips**
September 10, 2020 | 7:00 PM EDT
- ✓ **Nutrition and the Acute Porphyrias**
October 7, 2020 | 7:00 PM EDT
- ✓ **Virtual Patient Education Meeting**
October 26, 2020 | 8:30 PM EDT

PORPHYRIA NURSE ADVOCATES (by Member Candace Colbert)



Being a nurse with a desire to help advocate for all those with Porphyria, I was encouraged by Kristen to explore the possibility of creating a group of nursing

advocates. After recruiting interested nurses on Facebook, a dynamic and diverse team of passionate nurses responded. With the guidance of the APF, this group of nurses recently formed the Porphyria Nurse Advocates. (PNA). We had our first meeting June 2020 with a mission of increasing Porphyria education within the healthcare community. More specifically, we hope to educate nursing professionals on the signs/symptoms of Porphyria and about the resources and support the APF can provide. Porphyria Nurse Advocates members include Candace Colbert, Sally Davidson, Chrissy Dirnbeck, Erin Janiak, Amanda Jordan and Chan-an Stauffer. If you are a nurse and interested in joining this team, please contact the APF.

APF IS RECIPIENT OF SECOND ANNUAL ADVOCACY FOR IMPACT GRANTS PROGRAM

Anylam Pharmaceuticals, Inc., announced that the APF is one of seven patient advocacy groups that will



receive supportive funding as part of the company's second annual Advocacy for Impact grants program. The global competitive grants program aims to inspire patient advocacy groups to develop solutions that address critical unmet needs among the hereditary transthyretin-mediated (hATTR) amyloidosis, acute hepatic porphyria (AHP) and Primary hyperoxaluria type 1 (PH1) rare disease communities. The 2019-2020 Advocacy for Impact grants program was open to patient advocacy groups around the world requesting funding for up to \$50,000 for new projects. The American Porphyria Foundation will implement a Porphyria Grand Rounds educational program that will be created by porphyria experts, bolstered by the voices of patients, and delivered by qualified physicians. The program will be translated into multiple languages. We look forward to implementing this series throughout 2021.

COMPLEMENTARY AND ALTERNATIVE MEDICINES (CAMs) AND CANNABIS STUDY

Patients with Acute Intermittent Porphyria and Erythropoietic Protoporphyrin have anecdotally reported using Complementary and Alternative Medicines (CAMs) and cannabis to alleviate their symptoms, while others have reported that these may worsen symptoms. However, this has never been formally evaluated. This study is trying to assess what CAMs and types of cannabis patients with AIP and EPP use (if any), or have tried in the past, and how it affects their symptoms. This will provide useful, preliminary, information on patients' experiences with CAMs and cannabis for healthcare providers. Over one hundred EPP patients and fifty acute intermittent porphyria patients responded to the initial survey. We look forward to sharing the data after it is analyzed and reported.

SCENESSE APPEAL PROCESS GUIDELINES

The appeal process to access a new treatment can be a daunting process. In support of patient access to SCENESSE, the APF initiated a patient task force to develop a document to aid in developing a comprehensive appeal in the event that insurance has denied coverage of this life-altering treatment. Over multiple ZOOM meetings, this team created a guideline that includes the access process, helpful tips, sample support letters and important publications. If you would like to receive this guideline, please contact the APF at info@porphyriafoundation.org.



IF YOU RECEIVE MEDICAID AND MEDICARE, YOU CAN RECEIVE TREATMENT FOR ACUTE PORPHYRIA!!!

Many people with porphyria receive Medicare and Medicaid benefits. The Medicaid program covers one in five Americans, including many with complex and costly needs for care, like people with the acute porphyrias. Medicare primarily provides health insurance for Americans aged 65 and older, as well as some younger people with disability status as determined by the Social Security Administration. Since it covers only a percentage of the costs of treatment, remaining costs are covered by taking additional private insurance and/or by joining a public Part C or Part D Medicare health plan.

People with acute porphyria (AIP, HCP, VP, ADP) on Medicaid and Medicare often have been told at their clinics that these government programs do not pay for costly treatments, like Panhematin or Givlaari. Panhematin is prescribed to halt acute attacks and to prevent acute attacks. Givlaari is given to reduce the number of attacks patients suffer. Study data showed Givlaari reduced attacks by 70 percent.

If you need treatment for acute porphyria and have Medicaid and Medicare benefits, **you are eligible** for treatment and can insist upon being treated. You and your doc-

tor should discuss your situation and your right to receive treatment. If you are denied treatment because of the cost, please contact the APF.

The APF can also provide guidance in receiving Medicare/disability benefits. In addition, ...

The **Recordati Rare Diseases Patient Support Program** can help with verifying and understanding your insurance benefits. If your insurance does not cover Panhematin, the representatives can help determine your eligibility for the patient assistance program. You can reach the Recordati Rare Diseases Patient Support Program by calling **866-209-7604**, Monday – Friday, 9am-5pm Eastern time.

Anylam Assist offers support throughout your treatment with Givlaari including understanding insurance coverage, options for financial support, and educational materials and resources. Call **1-833-256-2748**.

Clinuvel is committed to facilitated treatment access with SCENESSE for eligible patients with a confirmed diagnosis of erythropoietic protoporphyria (EPP). Go to www.scenesse.com to request support.

TREATMENT TESTIMONIALS

The American Porphyria Foundation (APF) regularly gathers testimonials from members about their experience with FDA-approved treatments (**Panhematin®**, **GIVLAARI** and **SCENESSE**.) These testimonials are shared with other patients who can learn from your experience as they assess a treatment. If you are interested in sharing your experience with the APF, please send a written testimonial to us at info@porphyriafoundation.org. Sharing your experience may help future patients!

PATIENT AND PROVIDER FACT SHEET: INFUSIONS OR INJECTIONS DURING COVID-19 PANDEMIC

The APF recently hosted a webinar on new guidelines issued by CMS (Centers for Medicaid and Medicare Services) regarding access to treatment during the COVID-19 pandemic crisis. Patients have options to receive infusions and injections at home. With the support of Dr. Wang and a policy specialist, the webinar and a subsequent fact sheet share important information regarding reimbursement policy. To view the webinar and access the fact sheet, please see the special COVID-19 and PORPHYRIA section of the APF website at www.porphyriafoundation.org.

YOU DID IT!



Thanks to you, a July 17th one-day click campaign was a success! We

are grateful to Recordati Rare Diseases (RRD) for helping us continue our mission to spread porphyria awareness and educate our communities about this rare disease! Nearly 1,500 individuals clicked on a link that brought them to the APF website and access to educational materials on porphyria. RRD offered a donation for each of those "clicks." Thank you to all members, families, physicians, and others who took time to participate. Most important, thank you for spreading the word about Porphyria to your community! **#PorphyriaStrongerTogether #PorphyriaAwareness #AskMeAboutPorphyria**



NLB DONATES THOUSANDS... OF LIGHTBULBS!

The National Lighting Bureau (NLB) has delivered several thousand incandescent light bulbs to the American Porphyria Foundation (APF) for our CEP community. With an initial goal of 500 bulbs, the NLB was able to easily surpass this, with donations from LEDVANCE, Imperial Lighting Maintenance, Signify, SATCO, and Lambda 530 Consulting, and GE Lighting.

Congenital Erythropoietic Porphyria (CEP) causes extreme photosensitivity to light. Individuals with CEP develop acutely painful scars and blisters if exposed to sunlight or artificial light. Incandescent bulbs emit less short wavelength blue light than other types of bulbs and they can also be easily dimmed so that only longer wavelength green, yellow and red light is emitted — virtually eliminating the wavelengths that damage patients’ skin. Sadly, these bulbs are virtually impossible to find.

APF Executive Director, Kristen Wheeden reached out to Randy Reid, Executive Director of the NLB, to request the bulb donations. “The NLB has a rich tradition of working with many organizations,” said Reid, “we were honored that the APF called us for assistance and that our industry quickly stepped up to assist.”

Reid reached out to the NLB’s members, and word quickly spread. In less than a week, the NLB had collected 1,200 incandescent light bulbs for the cause. Then GE Lighting joined the cause and offered a tremendous donation included several thousand bulbs.

Our deepest thanks go out to the NLB for their generous donation and support of Porphyria.

The National Lighting Bureau (NLB) exists to create demand for High-Benefit Lighting® — efficient lighting designed to optimize human performance, health, safety, and commerce — by educating those who make and influence decisions about lighting.

THANK YOU, MR. BROWN!

Good things come in small and large packages. Our deepest thanks go out to Mr. Ron Brown for his generous donation of eight cases of light bulbs for our CEP bulb drive. Mr. Brown’s commitment to the APF cause and resourcefulness exemplifies the caring and generous spirit of our APF members. Thank you, Mr. Brown, for making the life of those with CEP a lot brighter!

ROAD TRIP!

Traveling in a special APF donned van, Kristen Wheeden delivered bulbs to several CEP member families on a special road trip. We were able to get the lightbulbs in the hands of several families - and we hope to get more bulbs out to as many in our CEP patient community who want them! Should you wish to receive a supply of bulbs via mail, please contact Iany at: iany@porphyriafoundation.org.



UPCOMING NORD SUMMIT FEATURES EPP

APF member Jennifer Beck has been tapped to help lead the opening session of the upcoming NORD summit on October 8, 2020, entitled “Entering a New Era.” With scientific innovation driving progress toward transformational and curative treatment, the keynote address, **Innovation Transforms Lives**, will include a panel of patients and caregivers that will describe how their lives have been changed by medical capabilities that didn’t exist until recently. We are excited about this patient-centered focus for the two-day conference that underscores why every patient advocate does the work he or she does. Jennifer will do a wonderful job describing her transformation on treatment with SCENESSE.

DIA DIA CONFERENCE

The DIA 2020 Global Annual Meeting was held virtually in late June. DIA is a global association that mobilizes life science professionals from across all areas of expertise to engage with patients, peers and thought leaders. Kristen Wheeden, APF Executive Director, was invited to join another patient leader to discuss the impact of the patient perspective and the importance of the patient voice in drug development during a panel session. Held virtually, this conference has focused on patient-centered programming. We were delighted to share our message at this important event!



Each year, the Patients as Partners program delves deep into patient involvement ideas, strategies, and implementation processes that biopharma can utilize in order to help advance patient participation for better clinical outcomes.

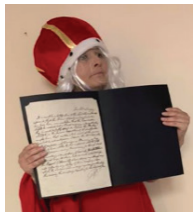
Barry Green, President of Alnylam Pharmaceuticals, led a keynote address alongside Desiree Lyon, APF Global Director. Together,

they demonstrated the synergy of the relationship between Alnylam and the APF and how collaboration on research, diagnostic and treatment projects helped create better health for porphyria patients.

Helping other companies learn how synergistic such a collaboration can be with patient organizations is important to the APF, as this is a message we have shared with other rare disease groups for over thirty years. Thank you, Patients as Partners.

OLDEST KNOWN APF MEMBER

Since our last Newsletter story about Joseph Hoss, our eldest known porphyria member, we have been notified by a few more members with bragging rights. EPP member, Mr. Darrell Klampe, will be celebrating his 89th birthday this September! We send our **Happy Birthday** wishes to Mr. Klampe and invite all other record breakers to contact us with their longevity news.



HEARKENING KING GEORGE!

Making the best of COVID at home, member Amanda Boston (VP) channeled her inner King George after ordering the King's porphyria letter. The letter, available on the APF online store, provides provocative insight into

the King's state of mind – during what may have certainly been an acute porphyria attack.



AWARENESS GONE VIRAL!

Meet Charlotte Hayes, our creative APF member who lives with EPP. A few weeks ago, Charlotte decided to post a short video to a popular on-line platform. That 15 second video has now been watched over 165K times!! With that response

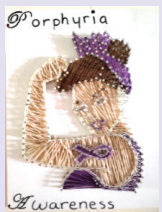
came many questions that she has been responding to with the help of the APF. Way to go, Charlotte!



EDRIN WILLIAMS, DIRECTOR OF PATIENT SERVICES

Please meet Edrin Williams, MHA, who serves as Director of Patient Services at the APF. Edrin has been committed to the health and well-being of porphyria patients for over three years. You may hear his kind voice when you contact the office, and you will quickly learn that he will follow through with any request. Edrin always goes above and beyond to serve our community. Hailing from Ridgeland, Mississippi, he attended Mississippi College where he earned his Master of Health Services Administration (while making the Dean's list!) Edrin enjoys the daily interaction that he has with patients. Often via phone or social media, he engages with patients, listening to their personal stories. The challenge to understand the science behind a group of eight ultra-rare blood disorders motivates him. "I

truly enjoy learning continuously from our group of Porphyria Experts," Edrin said. He is strengthened by his faith, living by a favorite verse in Philippians 4:13, "I can do all things through Christ which strengthened me." Thank you for being a valuable part of the APF team!



RARE ARTIST CONTEST

APF member Jennifer Safrit (AIP) submitted a stunning string art submission for the Rare Artist contest. Her piece, like Rosie the Riveter, is an inspiration to fight in the war against porphyria. Jennifer has gifted

her masterpiece to the APF office – where it will hang as a constant inspiration to our staff and visitors.

Jennifer entered the contest because she wanted everyone to see that a rare disease can't beat you. That you can continue to do what you love. "The string and nails represent that struggles can come from different ways, but in the end the picture can show how strong you are. It shows that you can overcome anything. That we are strong," she said.

NIH RARE DISEASE CLINICAL RESEARCH NETWORK COVID SURVEY

For the millions of people living with a rare disease, the novel coronavirus disease COVID-19 presents challenges, from potential reduced access to needed medical care to possible heightened anxiety and stress. A new online survey launched by the National Institutes of Health-supported Rare Diseases Clinical Research Network (RDCRN) aims to find out how the COVID-19 pandemic is impacting individuals with rare diseases, their families and their caregivers. Results will help the rare disease research community shed light on the needs of people with rare diseases during the COVID-19 pandemic and other potential health crises, in addition to informing future research efforts. The survey is available in both English and Spanish. Many porphyria patients have responded to the survey.... Our voice will be included in this important research.

GLOBAL PROGRAM by Desiree Lyon

The work of APF reaches across the world. Since the last newsletter, we have assisted patients in 30 countries and YOU have made our work possible. We grateful to our friends around the world for their help. There are many examples of how the APF collaborates with our board of experts, industry, and other patient organizations to help people who suffer from porphyria globally. Please know that you, too, are part of this process when you support APF programs with your finances, prayers, and loving outreach to others.

Patients around the world have the same complications and challenges we have in the US. In fact, most have greater challenges and less access to diagnosis and treatment. To participate with our international program, join the Porphyria International Support and Education Facebook group and meet new friends worldwide. Experience the joy of linking your life with people who have porphyria and together make a better porphyria world.

Lastly, I know you join me in thanking our Porphyria experts who every day give of themselves to save our lives. Thank you!!!



MASK UP!

As COVID was making a big impression on our world, the APF

team came together to think of how to help keep patients safe while doing necessary things such as attending medical appointments. We came up with masks because everyone needs one (or more!), right?

As quality masks were difficult to find, a friend of Amy Chapman came to the rescue. A seamstress for an NBC show by day and COVID RN by night, she agreed to make 300 masks in just 2 weeks. All we had to do is provide the material. We quickly selected over 17 styles, patterns, and designs. Outside upholstery was used which does not fade and keeps its shape. They can be washed by hand and line dried, with a firm grip and a HEPA filter inside. They keep you 25-30 degrees cooler. Since then, dozens of masks have been shipped to porphyria patients across the US. Amy says, "I hope everyone takes a cool photo of themselves in their mask and submits it to amy@porphyriafoundation.org. Get yours at the APF store at www.porphyriafoundation.org.



MITSUBISHI TANABE PHASE THREE CLINICAL TRIAL FOR EPP!

Are you interested in learning more about participating in this clinical trial for EPP/XLP?



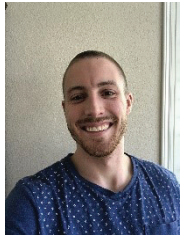
Participants ages 12-75 are needed at several sites for a Phase 3, Global, Multicenter, Randomized, Double-Blind, Placebo-Controlled Study to Evaluate Efficacy, Safety, And Tolerability of MT-7117 in Subjects with Erythropoietic Protoporphyrinemia (EPP) or X-Linked Protoporphyrinemia (XLP).

This is for oral medication (pills), once daily in the morning with or without food. Duration is 26 weeks plus optional 26-week double-blinded extension. The goal is to assess increased pain-free light exposure in adults and adolescents with a history of phototoxic reactions. There are three sites currently in the US to be followed by several more activating in September/ October, as well as global study sites across multiple countries.

Please contact the American Porphyria Foundation for more information and we will connect you with a study site nearest you. Email info@porphyriafoundation.org OR call 866-APF-3635 (301-347-7166).

MTPA – PHARMA LUNCH AND LEARN ABOUT EPP

Mitsubishi Tanabe Pharmaceutical America, the commercial arm of the organization, invited Kristen Wheeden, APF Executive Director, along with three patient panelists to do a virtual presentation including on the work of the APF and an overview of porphyria. The focus was on the patient perspective on the impact of living with EPP, including diagnosis and burden of disease. Three excellent panelists participated in the conversation. Thank you to Jay Goddu, Ginger Honiker, and Colleen McKillop for bringing the impact of EPP to life for the Mitsubishi staff in a poignant and personal conversation. Nearly 200



staff members attended and learned about porphyria. Our hope is that they were inspired by our stories to continue their focus on the investigational treatment MT-7117 for the treatment of EPP and XLP. (pictured l-r) Jay Goddu, Ginger Honiker, Colleen McKillop (caregiver for Morgan McKillop)

SUNSENSE

Dr. Amy Dickey, Massachusetts General Hospital, is currently conducting a study that is investigating methods for measuring light exposure and symptoms in EPP. The study may eventually help patients predict symptoms using a wearable smartphone-based device. This could help patients better manage the disease with reduced anxiety. Patients are answering a brief symptom and light exposure survey every day for two months. For one month, patients will wear a small light sensor. Please contact the APF at 866-APF-3635, or info@porphyriafoundation.org for more information and to volunteer.



APF PET PARADE

Congratulations! Nothing stirs people's sense of fun like showing off our pets. Porphyria friends love their pets and always enjoy participating in the annual APF pet events. A ZOOM Pet Parade was hosted August 8. Congratulations go to pet Milo Futo who won the contest!!! Milo is a four-year-old Jackemo who lives with his mom, Marianne, and family. Marianne crocheted Milo's entire adorable costume, mask, booties, cape and hat!

COALITION OF PATIENT ADVOCACY GROUPS



As the co-chair of the NIH RDCRN Coalition of Patient Advocacy Groups, Kristen Wheeden recently helped moderate the first in a webinar series titled **“An Introduction to Clinical Trials.”** This educational webinar helped train other advocacy groups who are embarking on clinical trials for their patient communities. The next in the series will include **“Educating the Patient Community and Collecting Patient Data.”** It is an honor to share your work and success in bringing treatments through clinical trials and to FDA approval with other patient communities.

RESEARCH IS THE KEY

Are you interested in participating in research? If so, we need you! Below is a list of current studies, clinical trials, and surveys that need participants. Click on the APF website for more information. This will be updated as new opportunities arise. “Remember...Research is the Key to Your Cure!”

1. Panhematin Prevention Study – Acute Porphyria
2. MT-7117 Phase 3 Clinical Trial for EPP/XLP
3. Acute Porphyria Survey – Patients with 1-2 attacks per year
4. SunSense - Short Term Study for EPP



MEET THE BOSTON TEAM

The research coordination team at the Mitsubishi Tanabe Boston study site is hard at work supporting EPP patients! Please meet (l-r) Megan Cheng, Akshata Mathur and Stella Schandorf. Research coordinators are the backbone of a clinical trial as the direct patient contact for appointments, screenings, follow-ups, and issues. They ensure compliance and that each patient has the best possible clinical trial experience. Thank you to the team in Boston for taking care of our brave participants!



DR. BISSELL – WORDS OF WISDOM

The APF honors Dr. D. Montgomery Bissell, who has served on the APF Scientific Advisory Board for decades and has excelled as

Director of the UCSF Porphyrin Center in San Francisco, California. Dr. Bissell is a world-renowned expert in the porphyrias, has advanced critical research and has provided expert clinical care to countless porphyria patients. Through the APF Protect the Future program, which attracts and trains the next generation of doctors and future specialists in the field of porphyria, Dr. Bissell has trained Dr. Bruce Wang to now manage the UCSF Porphyrin Center. We are grateful to Dr. Bissell for his service to the porphyrias and for his dedication to his patients. We asked Dr. Bissell to share some words of wisdom with us:

Did you always want to be a doctor?

My father was a physician, which meant that medicine was on my list from an early age. However, I was encouraged to explore, and at Harvard College I majored in English literature while taking the minimum pre-med coursework. In the end I went on to Harvard Medical School.

What led to your interest in porphyria?

I had enjoyed research as a medical student, and when I finished residency, I applied to Rudi Schmid's program at the University of California, San Francisco (UCSF) for a research fellowship. Schmid's interest in porphyria had been seeded when he was a trainee with Cecil Watson (University of Minnesota), a man regarded as the godfather of porphyria in the U.S. Watson had trained with Hans Fischer in Munich, who had received a Nobel in 1930 for deter-

mining the structure of heme. Entering the Schmid lab, I did not see myself becoming a 4th-generation porphyria specialist, particularly since Dr. Schmid was doing the patient care. Then one day, he called me into his office to tell me that I was the new porphyria doctor. "You do it," he said. "I'm too busy." That was the start of an interest that lasted more than 40 years.

What do you consider your contributions to porphyria?

My priority was serving as a clinical resource for patients. However, I did some porphyria research even while my main lab effort was focused elsewhere. As heme treatment of acute attacks emerged in the 1970's, it turned out that the IV solution was unstable. I began preparing it fresh for our patients while thinking about a way to stabilize it. I consulted with UCSF pharmacy colleagues on a formulation that could be freeze-dried, and came up with one that was stable for months in dry form and fully active after being reconstituted with mildly acidic water. When Congress passed the Orphan Drug Act of 1983, Abbott Laboratories (Chicago) was interested in the rare disease area and acquired the rights to our formulation, which became Panhematin®. It was the first *orphan drug* to be launched.

Where do you see porphyria research heading?

The symptoms in acute porphyria are neurological in nature and likely caused by chemical changes in the blood during an acute attack. While several of the changes are known, our understanding of their effects on neuronal cells is limited. Filling this knowledge gap could lead to new and improved therapies for symptoms.

THANK YOU, DR. BISSELL (From Patient Mary Schloetter)

I would like to take a moment to honor one of the leading lights in porphyria research and treatment, Dr. Montgomery Bissell. Dr. Bissell is a Professor of Medicine in the Division of Gastroenterology, Director of the UCSF Liver Center, and runs the Porphyrin Center at the University of California, San Francisco.

I had the great luck to be referred to Dr. Bissell in 1997 when my local PCP and specialists were at a loss on how to treat

my monthly hospitalized attacks of Acute Intermittent Porphyria. Though I only saw him twice a year, Dr. Bissell wrote detailed letters to my doctors regarding my care and was always available to them for consultation. He and another porphyria specialist, Dr. Anderson, were instrumental in allowing me to do several clinical trials for Normosang and Lupron in the late 1990's, and Dr. Bissell arranged for me to get genetic testing way back in 1998! He was definitely a doctor ahead of his time!

A subgroup of acute porphyria patients has chronic neurological injury, liver disease or kidney disease. Studies to document the time-course of these complications are under way in the Porphyrin Consortium (U.S.) (<https://www.rarediseasesnetwork.org/cms/porphyrias>). The goal is to identify patients who are at risk of chronic organ injury and to define the role of preventive treatment.

Do you have any words of wisdom for the patient community based on your decades of providing care to porphyrics?

I'll mention two things:

1. Training the next generation: Over a period of 30 years (roughly 1980-2010), the number of porphyria experts in the U.S. dwindled down to a precious few as the older generation retired and were not replaced. This led in 2010 to a joint project of the American Porphyrin Foundation and the Porphyrin Consortium, called the Protect the Future Campaign. The project has attracted a number of new people, reversing the downward trend. Sustaining this progress, however, will require the ongoing support of the porphyria community.

2. Social media: Use with caution. Affinity groups can combat the isolation that many patients with a rare disease feel, and that's a strong plus. For medical advice, however, the internet is a minefield. Blog commentary gets passed around as if it were proven fact, and treatments may be promoted that are inappropriate or dangerous. Patients are advised to consult science-based sites and to ask questions. The Porphyrin Consortium and the APF both provide the sober facts on porphyria as well as a list of bona fide experts across the country who will gladly take questions.

In the last several years, as he's been preparing for retirement, he has devoted his time to the *Protect the Future Program* and has trained a wonderful young doctor, Dr. Bruce Wang to take over UCSF's Porphyrin Center.

I will forever be grateful for all Dr. Bissell has done for me and countless other porphyrics now living better lives because of his dedication. Thank you, Dr. Bissell!

NEW PUBLICATION: HEPATOCELLULAR CARCINOMA IN ACUTE HEPATIC PORPHYRIA

A new publication by physicians of the Porphyrias Consortium was recently released in **Hepatology**, a peer-reviewed medical journal of hepatology. The risk for hepatocellular carcinoma (HCC) is increased in the acute hepatic porphyrias (AHP). The aim of this study was to explore the clinicopathologic characteristics, outcomes, and frequency of HCC in patients with AHP in the US through analysis of patients who have participated in the Longitudinal Study, supported by NIH. The study of 327

patients concluded that 1.5% of AHP patients had HCC. HCC in AHP occurred in the absence of cirrhosis, which contrasts to other chronic liver diseases. These results prompted the guideline that patients with AHP, regardless of clinical attacks, should be screened for HCC, beginning at age 50. For the full article, please search:

Hepatocellular Carcinoma in Acute Hepatic Porphyrias: Results from the Longitudinal Study of the U.S. Porphyrias Consortium.

Saber B, Naik H, Overbey JR, Erwin AL, Anderson KE, Bissell DM, Bonkovsky HL, Phillips JD, Wang B, K Singal A, McGuire B, Desnick RJ, Balwani M. *Hepatology*. 2020 Jul 18. doi: 10.1002/hep.31460. Online ahead of print.



In coordination with the APF, the National Institutes of Health (NIH) will be highlighting this important research in an upcoming social media campaign!



COVID CREATIVITY

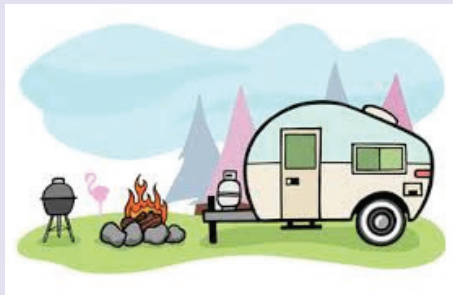
Dutch EPP patient and advocate, Jeroen Verheul, had a great idea! Combining the surgency of covid face masks and the need for protection, Jeroen applied a protective film to a mask intended for safety from Covid-19. The result – protection from the sun and covid. Thank you for sharing your excellent idea from across the world.



NEW PORPHYRIA SATELLITE CENTER

The APF extends a warm welcome to the newest addition to our team of Satellite Porphyria Centers. Dr. Marshall Mazepa and Dr. Gregory Vercellotti join the Porphyrias Consortium team from the University of Minnesota.

Dr. Mazepa (pictured left) is a clinical researcher in rare blood disorders with a medical degree from the University of Wisconsin, School of Medicine and Public Health, Madison, Wisconsin. Dr. Vercellotti (pictured right) is a Professor of Medicine, Division of Hematology, Oncology and Transplantation. He brings 44 years of experience after attending medical school at University of Illinois, Chicago, Illinois. We are thrilled to harness their research and clinical expertise and to offer another option for porphyria patients in the US. Welcome!



SUMMER FUN WITHOUT THE SUN

The Knauff family is no stranger to protecting their family from the ill affects of light, as their daughter Kasey lives with Congenital Erythropoietic Porphyria. They recently ventured on a summer camping trip that proved to be a fantastic family trip – and proof that we can find fun without the sun! Camping in the mountains in Pennsylvania, they trekked their RV across the state. Animals abounded with sites of deer, elk, bear, groundhogs, fox, squirrels, chipmunks, turkey, skunks, porcupines, and many fish! They launched their small boat around 7:30-dark so Kasey could catch some fish...and she even got up at 5:30am to fish before the sun came up in the creek between two mountains. We are always inspired by the creativity of our members to do all the things that life offers...in your own time and space.

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IN MEMORY & IN HONOR

We thank the families and friends who memorialized their loved ones with a generous gift to the APF.

IN MEMORY:

Henry Franzysen for **Etta Louise Insley**; Kay Lucas for **Clinton R. Lucas**; Marie Sidoti for **Frank Niccnio**; The Al & May Lou D'Augusta Charitable Fund, Anonymous, Mr. & Mrs. William Carvey, Mr. & Mrs. Earl Collins, Mr. & Mrs. Frank Cutko, Mr. & Mrs. Landweber, Mr. & Mrs.



Richard Vogelsang, Ms. Sheila Tersteg for **Dr. Richard Wheeden.**

IN HONOR:

Amy Chapman for **Porphyria Consortium Physicians**; Gaetana Iacono for **Maria Hensinger**; Sharon Koch for **Jagger Liguori**; Pietrina Scaraglino for **Mary Mistretta**; George Rusnak Jr for **Diana Sabella**; Lawrence Lutz for **Jason Sunney**; Anonymous, Don & Amy Hershey for **Brady Wheeden.**



SAFE / UNSAFE DATABASE – UPDATE

Acute Porphyria members have requested expanding the ability to print the Safe/Unsafe Drug Database alphabetically according to drug class, generic name, or brand name. As always, our expert physicians were consulted to identify any concern with implementing this update. With their input, this capability is now available.



AMERICAN PORPHYRIA FOUNDATION

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During a recent call, when the APF supported a patient with an issue related to access to treatment, the APF did that! A hospital had denied access to GIVLAARI, even though insurance had approved and was ready to pay. APF stepped in to pull the right players together to make this happen for her. Patients are constantly being tested by situations related to diagnosis, management, and treatment. We are here to support you through them all. Give us a call... there is a need to suffer in silence or accept a situation that could be helped. We are here for YOU, too!

"I DIDN'T KNOW THE APF DID THAT!"

