What’s New at the APF

www.porphyriafoundation.org

Is Your Membership Up to Date? The APF is able to maintain our physician and patient education programs and many other services because of your support. We do not receive government funding to run the APF, rather we receive donations from you, your friends, your family and people interested in the porphyrias. Now we need your support for several programs that are very special.

First, our Protect the Future program to train future experts is important to our future health. Without experts, doctors have nowhere to turn for advice and to learn about porphyria. This is a serious problem that we are trying to prevent by training young doctors, but where do we receive funding to do this except for help from our members.

Next, we have an enormous physician education program that distributes exceptional educational materials to doctors. Please help us produce these materials.

INTERNATIONAL CONGRESS OF PORPHYRINS AND PORPHYRIA 2017

BORDEAUX, FRANCE

The International Conference is a gathering of porphyria experts and researchers from around the world and is held every two years. This conference provides them with the opportunity to share their research and discuss the present and future therapies, case studies, etc. The official Patient Day is held on Sunday, June 25, 2017, followed by Congress Days 1-4, which conclude Wednesday, June 28, 2017. View the agenda below.

SESSION 1: Fundamentals on Heme Biosynthesis in relation to Porphyrias
SESSION 2: Acute Intermittent Porphyrias
SESSION 3: Bullous Acute and chronic Porphyrias
SESSION 4: Erythropoietic Porphyrias
SESSION 5: Clinical Complications of the Porphyrias
SESSION 6: Clinical evaluation and laboratory investigations in the Porphyrias
SESSION 7: Miscellaneous and Emerging Therapies in the Porphyrias

PLENARY LECTURES

Experts from around the world will be presenting lectures on a host of porphyria topics:

PL1: Tissue specific regulation of heme biosynthesis, Barry PAW (USA)
PL2: Cellular transport, trafficking, and recycling of heme, Izuki HAMZA (USA)
PL3: Acute Intermittent Porphyria, pathophysiology of acute attacks and recurrence, treatment options, overview and update, Robert J. DESNICK (USA)
PL4: Results from Clinical Trial using ALN-AS1, an Investigational RNAi Therapeutic Targeting ALAS1 for the Treatment of Acute Porphyrias (ALNYLAM), Elane SARÐ (Sweden) (for EU/USA Porphyria Reference Centers)
PL5: Vanierage Porphyria, update on pathophysiology and management of cutaneous symptoms, P. MEISSNER (SA)
PL6: Clinical aspects, pathophysiology and management of PCT, Jorge FRANK (Germany)
PL7: Pharmacological chaperones as a potential therapy for congenital erythropoietic porphyria, Oscar MILLET (SP)
PL8: Splice modulating therapy in EPP, state of the art, Laurent GOUYA (France)
PL9: Aflamaloides for the treatment of protoporphyria-induced phototoxicity, Janneke LANGENDONK (Netherlands)
PL11: Porphyria associated kidney disease, Nicolas PALLET (France)
PL12: Porphyria associated liver disease, Staffan WAHLIN (Swedend)
PL13: The value of long term longitudinal clinical and laboratory observations in porphyria, Karl ANDERSON (USA)
PL14: Measurement of therapeutic effect in the porphyrias: Balancing the needs of professionals, patients and the pharmaceutical industry, Elisabeth MINDER (Switzerland)
PL15: Diagnosing and monitoring porphyrias: benefits and disadvantages of centralizing porphyria dedicated laboratory tests, Sverre SANDBERG (Norway)
PL16: EPNET and EMON EQAS impact on porphyria diagnosis, Sharon WAHTLEY (UK) and A. AARSAND (Norway)
PL17: Gene correction in erythropoietic porphyrias by the CRISPR/Cas9 technology, F. MOREAU-GAUDRY (France)
PL18: Inducing iron deficiency improves erythropoiesis and photosensitivity in CEP, John PHILLIPS (USA)

The Social Program begins with the Patient Day gathering, the Welcome Receptions, Tours of the City and ends with a Gala Dinner for all the Conference attendees. See: https://icpp2017.org/

PATIENT ACCESS TO CARE TOOLKIT

A downloadable Access to Care Toolkit is a resource designed to help patients living with Acute Intermittent Porphyria (AIP), Hereditary Coproporphyria (HCP), and Variegate Porphyria (VP) or their caregivers, loved ones and healthcare providers secure access to Panhematin at their preferred health facility. We have recently learned of patients who are being denied this treatment from some hospitals and directed to secure another healthcare provider. If this has happened to you or someone you know or care for, please use these tools to request help from your state and local representatives and health advocacy organizations. We understand the debilitating effects of acute porphyria and hope these resources will help you access Panhematin when you need it most.

The Toolkit contains the following materials:

- Healthcare Conversation Tracker is a simple form to record your conversations with doctors, insurance agents, etc.
- Customizable letter templates to record your details to use for doctors, state departments, insurance, etc.
- AIP Access to Care Fact Sheet defines AIP, its symptoms and why it’s important for patients to get immediate care
- Patient Bill of Rights can be used to support your appeal for access to treatment

This Toolkit can be found on the APF website: www.porphyriafoundation.org

4900 Woodway Drive, Suite 780, Houston, Texas 77056-1837 • 866-APF-3635 • www.porphyriafoundation.org

The information contained on the American Porphyria Foundation (APF) Web site or in the APF newsletter is provided for your general information only.

The APF does not give medical advice or engage in the practice of medicine. The APF under no circumstances recommends particular treatments for specific individuals, and in all cases recommends that you consult your physician or local treatment center before pursuing any course of treatment.

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APF MEETS WITH FDA FOR ACUTE PORPHYRIA

The new air in Washington, DC is one of PATIENT INVOLVEMENT in health issues, particularly with drug development. In fact, before a drug is approved, FDA is encouraged to participate in Patient Focused Drug Development Meetings. The Patient Focused Drug Development (PFDD) initiative is a part of FDA commitments under the fifth authorization of the Prescription Drug User Fee Act (PDUFA V). The patient perspective is critical in helping FDA understand the context in which regulatory decisions are made for new drugs. PFDD meetings give FDA an important opportunity to hear directly from patients, patient advocates, and caregivers about the symptoms that matter most to them, the impact the disease has on patients’ daily lives, and patients’ experiences with currently available treatments. This input can inform FDA’s review of drug development and during the review of a drug application.

The APF secured one of these coveted meetings for EPP in October of 2016. Now we have also secured a PFDD meeting for the acute porphyrias on March 1, 2017 at the College Park Marriott Hotel & Conference Center in Maryland. Over 70 patients, families, physicians, and industry partners attended this meeting. Participants included experts, Herbert Bonkowsky, MD, John Phillips, PhD, Karl Anderson, MD, Robert Desnic, MD, PhD, and patients Lisa Keheberg, MD, Ariel Lager, Tara Cantley and husband, Shane, Amy Chapman and husband, Craig, Rose Jeans, Nichol Kirby, Candace Johnson, Lashiesha Johnson, Michael Boone, and wife, Sandra, Terri Witter, Evelyn Jacabucci, Colin McEwen, Mary Schlotter, Tracey Kelly, Lina Rebeiz, Louise Schloesser Braun, and MANY more.

If you missed the webinar, a full recording is available on the APF YouTube Channel at www.youtube.com/porphyriafoundation. The APF is also soliciting your comments. Please send them to the APF and we will share them with the FDA. A Voice of the Patient Report will become available after the conclusion of the meeting, along with submitted patient experiences.

NATIONAL PORPHYRIA AWARENESS WEEK (NPW) April 22-29, 2017

The NPW is the time each year for YOU to bring porphyria awareness to your community. We, at The American Porphyria Foundation, encourage YOU to help raise awareness and provide accurate information about porphyria where you live. Over the years, we have made great progress. However, we still need to improve porphyria awareness among other medical professionals and the public in general. During National Porphyria Awareness Week (NPW), participants can choose from a large number of opportunities to be proactive and enhance awareness among your family, friends, and community. Attracting media attention is one major means to accomplish this goal. YOU have a story to tell.

Ask your local newspaper or community newsletter to include a story about your porphyria or just write an article on your experience with porphyria and submit it. You can be involved in any way that works with your schedule, resources, etc. Even if you can only share one story.

The APF will help you accomplish your activity by providing: porphyria brochures, Porphyria Live DVDs, fact sheets, PowerPoint presentations and materials for medical seminars, press releases for local newspapers and television and other suggestions. Even if you cannot organize an event, the materials are invaluable to mental health professionals, colleges, hospitals, employee assistance programs and all types of health organizations.

Tell your story to local media. Help others by spreading your experience. Television, newspapers, and community magazines are looking for people who have undertaken the challenge with a rare illness.

Help others by sharing knowledge about porphyria with your community, including your family members, friends, and the physicians in your local hospitals. Suggest that they host a seminar, grand rounds on porphyria or a local meeting where you can hand out materials.

Help your story on social media. It is an easy and effective way of getting porphyria in the public eye. Assist with press releases, or in any form to provide information. Ask your hospital or doctor if there is a local meeting where you can hand out materials or tell your experience. Befriend your physicians, and they will share their newfound knowledge of the disease.

Volunteer your talents or skills to help achieve the educational programs of the APF. You are talented, let’s use it for our common good, for example, donate your paintings, sculptures, computer expertise, business acumen, etc. for our fall raffle or to help APF.

Purchase an APF T-Shirt or Cap and wear it. See the website for shirts and other APF products.

Learn how to be an advocate in your daily life and share your knowledge everywhere.

THE RAREST PORPHYRIA DID YOU KNOW DELTA-AMINOLEVULINIC ACID DEHYDRATASE; ALAD or ADP is the rarest of all the porphyrias with only 8 people diagnosed worldwide, mostly in Europe. ALAD is the enzyme that converts ALA to Porphobilinogen; the first step in the pathway leading to heme, a component of hemoglobin. A deficiency of the enzyme delta-aminolevulinic acid (ALA) dehydratase. Deficiency of this enzyme leads to the accumulation of the porphyrin precursor ALA, which can potentially result in a variety of symptoms, which vary from one person to another. Affected individuals experience intense and neuropsychological symptoms. Like other acute porphyrias, affected individuals may experience severe abdominal cramping or pain accompanied by vomiting and constipation and neuropsychiatric symptoms. During infancy, gastrointestinal abnormalities may cause an affected child to fall to grow and gain weight as expected. In severe cases, the motor nerves are involved, resulting in loss or partial impairment of the ability to use arms or legs. This can also be associated with severe biological changes during an acute attack.

In severe cases, loss of contact from reality (psychosis) has been reported.

ALAD porphyria is caused by mutations in the ALAD gene. The disease is inherited as an autosomal recessive disorder. This means that both copies of the ALAD gene have a mutation. Recessive genetic disorders occur when an individual inherits two copies of an abnormal gene for the same trait, one from each parent. If an individual inherits one normal gene and one gene for the disease, the person will be a carrier for the disease but usually will not show symptoms. The risk for two carrier parents to both pass the altered gene and have an affected child is 25% with each pregnancy. The risk to have a child who is a carrier is 50%.

The chance for a child to receive normal genes from both parents is 25%. The risk is the same for males and females. Parents who are close relatives (consanguineous) have a higher chance than unrelated parents to both carry the same abnormal gene, which increases the risk to have children with a recessive genetic disorder.

The ALAD gene contains instructions for creating the enzyme aminolevulinic dehydratase (ALAD), which is necessary for the production of heme, the part of hemoglobin, which is the oxygen-carrying component of red blood cells. Like AIP, VP and HCP, a number of agents can precipitate attacks, including alcohol, certain drugs, physical and psychological stress, infection, reduced calorie intake, dehydration and estrogen and progestogen.

The onset of ALAD porphyria is usually during infancy or childhood, but late-onset of the disorder has been reported. The treatment of ALAD porphyria is directed toward the specific symptoms that are present in each individual. Because there have been so few cases of ALAD porphyria, aside from avoiding triggering factors, there is only limited information about therapeutic options. Two drugs that are used in the treatment of ALAD porphyria (metyrapone and dolasetron) and glucose, have not been universally effective in treating ADP. Biochemical test results have an increase of 5% aminolevulinic acid (ALA) in the liver, other tissues, blood plasma, and urine. Urine coproporphyrin and erythropoietic protoporphyrin are increased. DNA testing is the most effective means for diagnosis.

IN MEMORY AND IN HONOR

We send our sincere sympathy to the families and friends who honored their loved ones with their generous gifts to the APF. We join them in thanking you for their donations.


In Honor We also thank those who donated in honor of a friend or family member: The Maggie for Melissa Nagic, Patricia Hoover for Cassandra Hooper, Steven A. Andrade for Josephine Dzygala, Carole F. Garay, Anna and Bill for Linda Ewing, Lisa M. Kancsar, Sharon I. Koch for Marty Krovetz, Robert G. Goddu for Jay Goddu, Michael Leppert for Nicole and Craig Leppert, Dr. Bonnie L. Katz and Dr. Carl L. Tisher for Dr. Peter Tisher, Florence Kirshoff for Ruth Taffe, David W. Shepherd for Margaret Shepherd, Elaine Smuczynski for Smuczynski Family, Mary Crown for Mary and Virginia, John F. Godley for Fred and Doris, Alice Gray for Alice Gray, Eileen Pritchard, Bill and Nancy Gray, Ruth Wilson, Grayed Gray, Lori Hanson, Paula Hendrix, Robert Hendrix, Myrna and Donald Cartledge, Mary Frances Donnelly, Sara Elaine and Douglas J. Coffier for Ralph M. Gray, Connie Hellesson for Jennifer Streets, Dale and Kitty for Alicia Zdzinski, Michael Pagano for Andrea M. Pagano Reyes, David Russell for Craig and Nicole Leppert, Cheryl Harriman for Jeffery Pradovic, Sharon Dill for Desiree, Jessica and Amy, Fred Cerkoney for Fred, Stephanie and Jason.
Although Amy Chapman (pictured with husband, Craig) lives in Florida, she hosted the Patient Education Meeting in Denver. It is one of the most enjoyable meetings you will ever attend and you can learn about porphyria, too. It is also not difficult to host a meeting. The APF helps a lot. We send out a nice invitation to all the patients who live near you. We also send the pamphlets, the DVD and arrange for an expert either to be there or to make a presentation via conferencing into the home or venue. Contact Edrin at the APF today!

ROBERT DOYLE
The first time I got overexposed was on a trip to Florida at age five. My reaction was so severe that my knuckles broke open because they had swollen so badly. The ER doctors said I had "sun poisoning." I went to many doctors of different specialties, including the dermatologist. They all suggested I was allergic to the sun, and I would outgrow it. The doctor ordered a test and almost 20 years ago, at the age of 38, I finally received a diagnosis of EPP. It took me 33 years to get a diagnosis.

At the age of 54, I noticed that I was getting weaker and weaker. I coughed a lot and had pain in my chest. They ran a blood test and said, "No, you've had a major coronary!" On Thursday, November 10, 2016, my surgeon told me I didn't have a major coronary, but my aortic valve was only functioning at 10% of the normal capacity, and my heart was so overworked trying to push so much blood through such a small opening that my blood levels showed that I had had a major coronary. He then told me that I must have surgery now, or I will die, plain and simple.

While completing pre-operation procedures, I finally got the attention of the anesthesiologist, who decided to have a little chat with me. He informed me that he thought I had AIP and that the operating rooms would not be a problem, but because I have EPP, the surgery needed to be postponed in order to get the right type of lights in the operating room. This hospital, which is one of the major hospitals in the county, did not have the appropriate filters for the lights on hand, so they had to be ordered.

My main goal in telling my story is to inspire others with any of the porphyrias to make sure that their hospital has the necessary tools, specifically filters for the operating room lights, available for them in case of an emergency. Speaking with the anesthesiologist a week later, he informed me that if I hadn't alarmed his attention, that I could be dead! In short, the operating room lights could have damaged my heart. What if I had needed an emergency operation to treat my heart, because it was so debilitated? Please, please talk to your doctor! Have him/her check to see if these filters are available and are at your local hospital, now; YOU just might need them! You never know what is going to happen.

Visit the Member Stories section of the APF Website to read the full version!

JESSICA IS OFF TO MEDICAL SCHOOL
If you have had any dealings with the APF, you will most assuredly have met Jessica Hungate, Patient Services Director. We are sad Jessica is leaving but overjoyed that she has been accepted into medical school in El Paso, Texas. Jessica says that her experience at the APF was key to her acceptance. She also expressed that she is grateful for all the patients have taught her about being a good doctor and for having the experience of meeting and working with the greatest porphyria experts in the world. Describing Lyon, Executive Director, said, "There are not enough superlatives to describe Jessica, nor enough words to say how she will be missed, nor enough blessings to heap on her for her goodness." Congratulations!!!

SAN DIEGO PATIENT MEETING
Although Amy Chapman (pictured with husband, Craig) lives in Florida, she hosted the Patient Education Meeting in San Diego recently. Amy has hosted a number of such meetings around the country. Dr. John Phillips made the educational presentation on all of the porphyrias, as well as conducted the Question and Answer portion of the meeting. Amy has also hosted several patient meetings around the country. These meetings provide YOU with the opportunity to meet other patients with different types of porphyria while learning about the disease from an expert. The agenda most often includes a Question and Answer session. This is one of the most important parts of the meeting, because most patients have never had the chance to meet a "real" expert who not only knows the answers but also is one of the researchers who discovered the answers and is continuing to find treatments for us and ultimately a CURE. Thank you Amy and Dr. Phillips!!!

We hope to have a meeting each month in 2017, but we can't do it without YOU. Our members host these meetings and the APF sends all the supplies and provides the speakers. If you are interested in hosting a meeting in your area, please contact the APF at 713.266.9617 or porphyrus@porphyriafoundation.com.

Over the past few years our members held meetings or fundraising events in almost every state, including Florida, California, New York, Maryland, Texas, Alabama, Missouri, Illinois, North Carolina, Massachusetts, Washington, Oklahoma, Tennessee, Nevada and Colorado. Join Families like the Jacobucci family (photo left) who hosted a meeting in Denver. It is one of the most enjoyable meetings you will ever attend and you can learn about porphyria, too. It is also not difficult to host a meeting. The APF helps a lot. We send out a nice invitation to all the patients who live near you. We also send the pamphlets, the DVD and arrange for an expert either to be there or to make a presentation via conferencing into the home or venue. Contact Edrin at the APF today!

PROTECT THE FUTURE
We are very proud to have Dr. Bruce Wang as one of our Protect the Future (PTF) doctors. Dr. Wang has been involved in the PTF program since the beginning and is now one of the best experts in the country. He is a brilliant clinician who works with Dr. Montgomery Bissell at the University of California, San Francisco Porphyria Center. Dr. Wang has participated in the many porphyria research projects of the Porphyria Consortium and has also participated in their publications in major medical journals and textbook chapters.

He completed his medical education at the University of California, San Francisco School of Medicine in 2007. He went on to complete his Residency here in 2009, and his Fellowship in 2013. Dr. Wang is a valued member of the Protect the Future program and we look forward to working with him for many years to come.

Some of his porphyria publications include:

For those of you who are new and don't know about our PTF program, it is our most important program. Without porphyria experts and their expertise in this group of diseases, expertise would be lost as each of the experts approach retirement. Patients and doctors would remain in the dark about the porphyrias. Therefore, we train young physicians as the next generation of porphyria experts. The training takes place at Porphyria Centers under the guidance of the current Porphyria Consortium of experts. The funding to train these young experts, like Dr. Wang, comes from you, the people whose lives are affected now and in the future.

Contact the APF to make your donation today!
(Pictured above is the Wang family, Tina, Bruce, Teddy and Andy.)

ALNYLAM
invited Amy Chapman, Rose Jeans and Colin McEwen to present their experiences with acute porphyria to their entire company so that the employees better understand the porphyrias. It is important for people to know why they are working hard to develop a small molecule drug for a group of patients. These three people did a brilliant job sharing their experience with porphyria at a companywide meeting. While Amy Chapman flew from Boston to San Diego the next day to facilitate a Patient Education Meeting, Rose and Colin filmed an outstanding video on their porphyria experience.

To understand the treatment that Alnylam is developing for the acute porphyrias, see: https://www.youtube.com/watch?v=Z6qMBQ9vm9A&feature=youtu.be

COLOMBIAN FOUNDATION FOR PORPHYRIA
The Colombian Foundation for Porphyria is one of many groups around the world that the APF has helped become a thriving organization. We thank Dr. Andrade for her message from our Colombian friends.

I am Dr. Marcellana Avila Andrade. I am a medical doctor in physical medicine and rehabilitation, and also a patient with Acute Intermittent Porphyria. In addition, I serve as the President of the Colombian Foundation for Porphyria. Our most recent meeting in California, San Francisco Porphyrria Center.

We are planning to meet in Bogota, Colombia in the next few months. We have organized a meeting for over 150 patients. The Colombian Foundation for Porphyria has a mission to help others who are affected by porphyria.

The Colombian Foundation for Porphyria is one of many around the world that the APF has helped become a thriving organization. We thank Dr. Andrade for her message from our Colombian friends.

I am Dr. Marcellana Avila Andrade. I am a medical doctor in physical medicine and rehabilitation, and also a patient with Acute Intermittent Porphyria. In addition, I serve as the President of the Colombian Foundation for Porphyria. Our most recent meet-

ing was attended by 13 patients from Bogota. In Colombia we have about 250 patients in the database of the Foundation from different cities of the country. Next year we plan to resume meetings with doctors and specialists to heighten awareness of the porphyrias, as well as patient meetings. We will carry out the genetic study of the pa-
tients of the Foundation to determine the type of porphyria and to look for the passive carriers in each family mem-
ber besides to identify those patient in the numbers common in our population. Our new website page is under construc-
tion but the address is www.funcolpor.org.

Best regards,
Dr. Marcellana Avila Andrade, President

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**JESSI BRILL**

Lives in Northern Virginia. I was diagnosed with EPP about 5 years ago, but my first episode was when I was 18 months old. As, I'm sure, many of you can relate, I ran the gauntlet when I was 18 months old. As, I'm sure, many of you can relate, I ran the gauntlet.

**WHY SO MANY GIRLS?**  
ACUTE PORPHYRIAS ARE 50/50 BUT WOMEN GET MORE ATTACKS DUE TO THE MENSES.

**APF WELCOMES EDRIN WILLIAMS**

After completing his Master of Health Services Administration, Edrin Williams relocated to Houston, TX from Ridgeland, MS to take on the position Director of Development for the APF. Aside from assisting with the daily operations of the APF office, Edrin will also head the APF fundraising projects and new educational programs. Edrin represented the APF at all porphyria patients at funding the Porphyria Week in Washington, DC and facilitated APF involvement in legislation that affects rare diseases. As a former healthcare provider, Edrin is high experienced with patients and the impact a patient organization can make in a patient’s life and on their hope for the future. During his spare time, Edrin enjoys travel and spending time with family and friends. When you contact the APF, give him a hearty welcome.

**FDA ATTENDEES**

A few of the attendees at the FDA meeting: Row 1 and 2, left to right

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1. Louise Braun, Terri Witter, Lisa Keihberg, Michael Boone, Tara Cantley, Evelyn Jaccobucci, Lina Rebeiz, Candace Johnson
2. Ariel Leger, Amy Chapman, Sharon Dill, Mary Schloetter, Lakeda Johnson, Rose Jeans, Danielle Frizzoli Tatarka

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**PANHEMATIN®**

To date, there are 6000-7000 rare diseases that have been identified, however, there are only 400 plus drugs to treat rare diseases. Unlike most rare diseases, we have a treatment for the acute porphyrias. Panhematin was the first Orphan Drug and has been saving lives now since 1983 when the Orphan Drug Act was signed into law by President Ronald Reagan. Without Panhematin, many people with acute porphyrias would not be alive today. At the APF, we receive many questions about Panhematin from newly diagnosed patients. One question often asked is when to start the treatment. Although patients improve with treatment, an attack can still be life-threatening. Respiratory insufficiency may require use of an artificial respirator. Therefore, experts agree that porphyria attacks should be treated as early and effectively as possible. 

**Dr. Lisa Kehrberg**

Very early after my diagnosis, a PICC line was inserted in my arm. I felt much better after only a few infusions. I was close to death by the time I was diagnosed and certainly would not be alive today had I not met my internist and had not received Panhematin timely.

**Charles Johnson**

... the frequency of the attacks rendered me unable to keep a schedule and the pain left me unable to work. Now that I am on Panhematin every two weeks and the attacks have been under control, I am hopeful that I can return to work soon.

**Cheryl Black-Blair**

“The procedure itself is similar to IV glucose treatment—and no more uncomfortable. I chatted on the phone through my Panhematin treatment, and by the time I fell asleep I felt a definite lessening of the pain.”

**Amy Chapman**

“I was a new person. Panhematin works so well for me!”

**Desiree Lyon**

“Once a month, I would have a critical attack which put me in the ICU. Finally, I was treated with hemin therapy, which later became Panhematin. Panhematin saved my life over and over.”

**Judy Snyder**

“I have not had to be hospitalized for an attack in a long time! The Panhematin treatment works for me. I don’t believe I would be here today if it weren’t for this drug.”

**Steve Stevens**

“My hematologist suggested that I have a regimen of monthly Panhematin treatments. I am 55 and have learned not to take life for granted. I know there is no cure for VP, but I’m not letting it dictate my life.”

**Amanda Boston**

“I had the most amazing privilege of participating in the 2013: A double-blind, randomized, placebo-controlled, parallel group trial on the efficacy and safety of Panhematin® in the treatment of acute attacks of porphyria research study.”

Editor’s note: Panhematin saved Amanda’s life when she was in a coma and paralyzed.

**RARE DISEASE DAY**

On 28 February 2017, the tenth edition of Rare Disease Day saw thousands of people from all over the world came together to advocate for more research on rare diseases. Over the last few decades, funds dedicated to rare disease research have increased. But it can’t stop there. Rare Disease Day 2017 provided an opportunity to call upon researchers, universities, students, companies, policy makers and clinicians to conduct more research and to make them aware of the importance of research for the rare disease community. The APF participated in Rare Disease Day. Edrin Williams represented the APF during the Capitol Hill project to visit key Congressmen’s offices to request greater funding for rare disease research.

Patient involvement in research has resulted in more research, which is better targeted to the needs of patients. Patients no longer solely reap the benefits of research; they are empowered and valued partners from the beginning to the end of the research process. Patients:

- Advocate for research on a specific disease or across diseases. They know where research is needed and work to influence research bodies and companies to prioritize these areas in their research.
- Fund research. Individuals or patient organizations, such as the APF, raise money for clinical trials or research projects, on their own or in partnership with private funding initiatives.
- Partner in research projects and are included in the governance of research.
- Participate as subjects in clinical trials and also in the design of clinical trials. They, therefore, help to ensure that the development of a medicine takes into account their real needs, so that the patient perspective is not overlooked.

**DON’T FORGET TO DONATE**

The APF maintains extensive patient and physician education programs with comprehensive publications and curricula, as well as regular newsletters, quarterly newsletters, support systems, social media networks, major media campaigns, research projects, brochures for each porphyria, APF products and many other services to assist patients, families, care takers and doctors.

Call Today: 866.APF.3635