

NATIONAL PORPHYRIA AWARENESS WEEK APRIL 20-26, 2014

The APF members did an awesome job this year engaging local and national media, raising funds and getting porphyria awareness into doctor's offices and local hospitals. Thank you all! We would like to feature all of your efforts but can only present the following:



Amanda Boston set a great example of educational efforts. She ordered fact sheets, brochures and pamphlets from the office to distribute to nurses, doctors and others in her community. She also made a Rare Disease poster about porphyria to display it in the Norton Cancer Institute in Louisville, KY where she receives Panhematin™ treatment. She is currently a research patient with Dr. Anderson at UTMB in Texas, participating in a very important study *7203: A double-blind, randomized, placebo-controlled, parallel group trial on the efficacy and safety of Panhematin™ in the treatment of acute attacks of porphyria* to evaluate the effectiveness of glucose and Panhematin™ compared to glucose alone treatment for acute attacks of porphyria.

Lorri Stewart got in touch with Public Relations department of Caldwell Memorial Hospital, Lenoir, NC. PR representative let Lorri present information and hand out materials at their monthly meeting. She also presented brochures and talked about porphyria with Emergency Department personnel of the hospital. Besides, Lorri is donating paintings on behalf of all porphyria sufferers in an effort to raise awareness of the porphyrias throughout the year.

Jeanine Keith established a Porphyria Research Fundraising Page to heighten awareness about HCP. Please show your support to help Jeanine with the research efforts to find a cure for the many patients who also suffer from HCP on a daily basis.

JoAnna Floyd also administers a fundraising page. She raises funds for the APF research and hands out materials to promote awareness of HCP porphyria. For every dollar you donate, JoAnna did a burpee! For those of you who do not know, a burpee is a full body aerobic exercise used in strength training. It is a fun a creative way to raise awareness! The challenge was 30 days long. It ended April 26, on the last day of Awareness Week. You may still support JoAnna and her effort!

Cindy Craig visited hospitals and medical professionals in her area to distribute awareness materials. The ER team of her hospital ordered an Emergency Room kit with the guidelines for the porphyrias. Cindy is also inquiring if the National Porphyria Awareness week can be acknowledged at the state level in Missouri.

Amy Chapman is managing a t-shirt fundraising campaign. Our hope for the T-shirts is that you all will buy and wear them to help promote and raise questions about porphyria. The orders can be submitted online; the funds will be used on research and educating new porphyria experts.

Amanda Rich was making purple bracelets for survivors. The bracelets were handmade and could be personally designed. You could reach Amanda through our APF Facebook group to submit orders.

Mark Abeloff will install an informational presentation desk in Berkshire Mall in Wyomissing, PA. He will show a film about porphyria and hand out brochures and fact sheets educating people in his community.

Evelyn Jacobucci's (R) daughter made purple arm bands for National Awareness Week. She presented them at science fair at her school to spread the knowledge and understanding of porphyria among young kids.



The Cook brothers (L), Cason and Caul, continued a HAT DAY tradition for Porphyria

Awareness Week 2014. Both brothers have EPP and have set a great example about enhancing awareness of the disease in their home town of Vernon, TX. The boys raised funds for the APF by hosting HAT DAY, where everyone at their schools who wanted to participate, could wear a hat in honor of the brothers and bring one dollar for the APF.

Donna Caldwell was volunteering for the APF. She received educational materials from the APF to distribute in her community. She was also contacting the local news companies and newspapers to publish her interview during the Porphyria Awareness week.

Emily Carmen created several videos on her YouTube channel about her experience dealing with Hereditary Coproporphyrinemia (HCP) during the NPAW. The video is not intended to give any medical advice, but rather increase awareness and share experience:

<https://www.youtube.com/watch?v=aTVtdfr-Pjw&feature=youtu.be>

Alberta Porphyria Society. Derry Wilcox and his family did a great job at organizing the first fundraising event for the Alberta Porphyria Society in Canada. The event was a success, the participants had a lot of fun, live music from Marty Satre, Pete McConnell and others and door prizes for all who attend.

Jodi Everlith also has a story to share. She and her husband took the sons to their very first major league baseball game on 4/12 this year. Red Sox vs Yankees @ Yankees stadium. They had purchased the tickets through a group and couldn't select the seats. Jodi has EPP, so she contacted the Yankees about switching the seats to a place with shade explaining her condition. The game was close to sold out and they didn't have any seats in the shade in the area where their \$22 seats were, so they upgraded the seats from the 3rd level (highest you can go) to field level MVP seats, in the shade, right behind home plate! At no additional cost! This is an encouraging story, never be afraid to ask and educate people around you at the same time!



Liberty Belle and her husband made a wonderful Nighttime Easter egg hunt for their daughter Sian, who has EPP. Parents wanted something memorable and unique for the daughter. Liberty got the finger LED lights and played with different types of plastic eggs. Sian was getting so anxious that the Easter bunny left her out, which made it harder to wait until dark to hide the eggs. As she was getting ready for bed, Liberty hid the eggs in the garden. Sian saw them glowing from inside the house and went running outside. An amazing idea for Easter celebration, no child should be left behind during Easter egg hunt fun!



Many-many APF members: Jennifer Mitchell, Kimberlee Kasper, Babi Gonzalez, Emily Carmen, Angie Fischer, Patti De Groot, Victoria Harrold, Andrea Carson Fulkerson and others have received awareness supplies and informational materials from the APF to help raise awareness.

We would like to thank all the APF members for their legislative efforts, for educating physicians and others in their communities and for raising porphyria awareness! Let's keep up a great awareness activity not only during the NPAW, but throughout the year. Share your knowledge of the disease at your doctor's office or local hospital, tell your story to local media, join the APF, support research with the donation and encourage family and friends to do the same! Together we are making a big difference in educating the public and medical professionals about the disease and helping each other to overcome the difficulties with a smile and enthusiasm☺



phyria, as well as learn from experts. We thank all who attended and a special thanks to the doctors who volunteered their entire weekend to help us. Throughout the year the APF will be hosting meetings in the following cities: Houston, TX, Atlanta, GA, Santa Rosa Beach, FL, Los Angeles, CA, San Francisco, CA and Boston, MA. Other meetings will be added if possible.



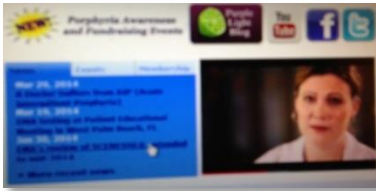
them photos of what occurs when the skin is damaged in EPP, as well as all the protective measures severely affected individuals must take to protect themselves from the light.

PATIENT EDUCATION MEETING The APF hosted a terrific patient education meeting in West Palm Beach, FL. Over forty people attended the meeting, including five porphyria experts: Dr. John Phillips (photo L) and Dr. Charles Parker from the University of Utah; Dr. Robert Desnick from Mount Sinai Medical School in NYC; Dr. Karl Anderson from the University of Texas Medical Branch, Galveston, TX and Dr. Maureen Poh-Fitzpatrick from Columbia University Hospital in NYC. Since five experts were assisting, we were able to break into small groups after a short presentation by Dr. Anderson. This gave everyone the opportunity to ask questions and have time to chat with one another. Dr. Desnick and Dr. Phillips set up a testing station and performed DNA tests on a number of the attendees. APF member Amy Chapman also attended and helped us with the meeting. As usual, Amy is a special friend to patients and a huge help to the APF. It was a wonderful experience for all. These meetings are the best means to meet others with porphyria face to face and exchange their experiences with every type of porphyria

EXPERTS MEET FOR EPP Porphyria experts recently met to review the EPP research with Afamelanotide, an implant to improve photosensitivity and phototoxicity, and hear updates on the drug approval process. Desiree also commented on her visit with the EMA in London. The European Medicine Agency (EMA), which is the counterpart of the FDA, was anxious to hear about the experiences of EPP patients, why they needed a treatment and how it affects their work, play and daily living. In preparation of the EMA meeting, the APF staff sent all of your letters to the EMA representatives. Your letters were very powerful and were well received by the FDA and EMA. We also showed

According to the update, the EMA will be meeting with patients in Europe and doctors soon to assess their opinions about the treatment. It is our hope that the Afamelanotide treatment will be approved soon and that patients who were in the trials and received the placebo would have the opportunity to have the implant soon on a compassionate basis. In Europe, a number of patients have been fortunate to receive the implant on a compassionate basis for years. Patients have been waiting all of their lives for a treatment. We hope this long awaited dream of a treatment will become true in time for the summer. (Photo men l to r) Drs. Joseph Bloomer, Montgomery Bissell, Charles Parker, Robert Desnick, Karl Anderson, John Phillips and Phillipe Wolgen (Clinuvel). Women (l to r) Emily Rodenburger (Clinuvel), Linda Tang (Clinuvel), Dr. Maureen Poh-Fitzpatrick, Desiree Lyon Howe.

AIP PATIENT, DR LISA KEHRBERG VIDEO



As soon as Dr. Kehrberg joined the APF, she offered to help. When Desiree suggested a huge undertaking to Dr. Kehrberg, and she agreed to video her experience as an AIP patient and explain her attacks, she gladly accepted the challenge. She was also asked to speak on the extreme pain of porphyria and what a patient endures to receive appropriate pain medication, particularly since physicians rarely understand the intensity of porphyria pain. The irony of the situation is that Dr. Kehrberg is a Primary Care Physician who was serving as a pain management physician at her nearby hospital.

Unfortunately, Dr. Kehrberg experienced repeated attacks and was hospitalized frequently. It was quite some time before she was able to travel to Houston to film the video, because of her repeated attacks. In fact, she was not well when the video was filmed but chose to press on. Despite her illness, Dr. Kehrberg's video is outstanding. You can view the video on the Home Page and throughout the pertinent sections of the APF website: www.porphyrifoundation.com and on the APF YouTube channel: www.youtube.com/user/Porphyrifoundation

We ask that you encourage your doctors to view this short, but very powerful video. It is also a good tool for those, whose families do not understand the pain of porphyria or the need for appropriate treatment. Remember too, that the APF will send your doctors a very comprehensive educational packet on the diagnosis and treatment of acute porphyria. Just contact the APF and a packet will be sent immediately.

CONGRATULATIONS PIERRE



APF member and EPP advocate, Pierre Mouledoux and his wife Alyse are new parents of Pierre Mouledoux, Jr. who was born January 2, 2014. After participating as a patient volunteer in the Afamelanotide research, he also became a Facebook Administrator for EPP. We are grateful to Pierre for travelling to Washington, DC along with ten others to speak with the FDA and enlighten them on the success of the EPP treatment, Scenesse/Afamelanotide. Most recently, Pierre approached the state legislature in his home state, Louisiana, about the need for film for automobile windows for people with EPP. Driving is a big problem for EPP as the sunlight pouring through the windows can cause pain and swelling. Some states have already recognized this situation and

have granted EPP patients permission to use auto film to cover the windows. With the help from his physician and State Representative Joseph Lopinto, Pierre initiated House Bill HB 1127 to gain permission to use protective auto window film in Louisiana. We thank Pierre for his advocacy work and ask that you contact Pierre to help him in this endeavor. You can be an advocate in your state for this legislation. If so, please contact Pierre to ask about his experience via the APF Facebook EPP group. We also have five FACEBOOK groups for different types of porphyria.

See: <https://www.legis.la.gov/legis/BillInfo.aspx?i=225492>.

ALICIA'S TRANSPLANT



My name is Alicia Moczynski and I'm 22 years old. I had my first porphyria episode at age 2. However, as a child, my episodes were infrequent and mild. It wasn't until I turned about 16 that I started getting worse and attacks more frequent. No one in my family has porphyria, so the doctors could never diagnose me. I just accepted the fact that I would never find out what is wrong with me. Then, when I turned 21 (May 2012), I had the worst episode ever. Not only were my face, hands, arms and legs swollen, my stomach was also in a lot of pain and I had jaundice. I went to the emergency room and received a misdiagnosis of Hepatitis A. The doctor said, Hepatitis A heals on its own and I should just give it time.

However, I never truly recovered and was bedridden for about three months. Unfortunately, during December 2012 I got very sick again with the same symptoms: severe stomach pain and jaundice. I was admitted to the University of Alabama Hospital, where I met porphyria expert, Dr. Joseph Bloomer. As some may know, Dr. Bloomer is very involved with patients who have porphyria and the research. Dr. Bloomer diagnosed me with XLP porphyria. Finally, after 21 years, I can put a name to my misery. I had a liver biopsy, which showed that I already had some scarring

in my liver. Dr. Bloomer told me I had two options: I could either wait and see what happens and possibly one day have a liver transplant (very likely in my case), or I could have a bone marrow transplant instead and be "cured." It took me a couple of months to make my decision, but I finally decided to have a bone marrow transplant. So on October 15, 2013, I received my transplant from an unrelated donor from Denmark (I have two sisters and they were unfortunately not a match). I will not lie. A bone marrow transplant is hard. I had to go through chemo, I was very weak, and I needed a caregiver 24/7. After 4 months passed, I gained a lot of my strength back and I feel good. My road to recovery is still not over, but the thought of never having an episode again is really amazing. I'm also very blessed to have such a great donor who was willing to help me, even though she has no idea who I am. To show you the difference, during December 2012 I had a porphyrin level of 10,000 (I believe 80 is normal). Before the transplant, I had a porphyrin level of 600 and now I have a porphyrin level of 113. Thus far, the bone marrow transplant is working and I highly encourage individuals with porphyria and need a transplant, to read about it and Dr. Bloomer who is famous in the field.

BECOME A MEDICAL HERO as a research volunteer. Porphyria patients who volunteer for



porphyria research are definitely medical heroes. Patient volunteers change the course of our lives, so they deserve our thanks. You have the opportunity to volunteer. If you have porphyria, participating in a clinical trial/research to evaluate a new treatment or gain more understanding of the disease offers YOU a chance to improve and extend your life, as well as those of your family.

What is a clinical trial? It is a research study in human volunteers to answer specific health questions, including testing a new treatment. Carefully conducted clinical trials are the fastest and safest way to find treatments that work in people and ways to improve health.

Why participate in a clinical trial? Participants can play a more active role in their own health care, gain access to new research treatments before they are widely available, and help others by contributing to medical research. This is a very fulfilling experience, which affects your family now and in the future.

What happens during a clinical trial? Trial teams include doctors and nurses as well as social workers. They check the health of the participant at the beginning of the trial, give specific instructions for participating in the trial, monitor the participant carefully during the trial, and check after the trial is completed. Some clinical trials involve more tests and doctor visits than the participant would normally have for an illness or condition. For all types of trials, the participant works with a research team. Clinical trials that are well-designed and well-executed are the best approach for eligible participants to play an active role in YOUR own health care. YOU can gain access to new research treatments before they are widely available. You can help others by contributing to medical research.

The Porphyria Research Consortium needs your help. There are seven clinical trials ongoing. Some require that you only answer extremely pertinent questions over a period of five years. This Longitudinal Study is open to all types of porphyria patients. Then there are individual studies for the acute porphyrias, EPP and X linked EPP, PCT and family studies where your family is needed even if they do not have porphyria. There is also a study being conducted using Panhematin™ to prevent attacks. Although Panhematin™ has been used to stop attacks for over twenty years, it is now being used widely to prevent attacks. If you have frequent predictable attacks of acute porphyrias, you are needed for this study, too. Please contact the APF office if you want to volunteer. Be a medical HERO.

CARBOHYDRATES IN ACUTE PORPHYRIAS For some individuals, who have the "acute porphyrias" (AIP, VP, HCP and ALAD), attacks can be brought on if carbohydrates and calories are restricted for prolonged periods of time. For example, during the Atkins diet craze, many people with porphyria were diagnosed when their reduction of carbohydrates precipitated attacks. This is why fasting or major dieting is not recommended. Thus, to prevent and treat attacks, carefully monitoring one's diet can be especially important for these types of porphyria.



Why are these three porphyrias more sensitive to diet? The pathway in the liver that makes heme from porphyrins and other substances is very sensitive to carbohydrates in particular. Therefore, when less carbohydrate is taken, it appears that porphyrin production is stimulated, and the body can't use them all effectively. This porphyrin overflow is what creates the symptoms of an attack. Carbohydrates are the foods that contain starches or sugars. They are important in everyone's diet, because they provide us with fuel for our bodies, as well as a wide variety of vitamins and minerals in many of the carbohydrates. Starches are "complex carbohydrates" (I) and tend not to be as sweet as sugars. Starches are called "complex" because they are larger molecules and take longer (compared to sugars) for our bodies to break them down for use as an energy source. Some starchy types of foods include: potatoes, pasta and bread. Sugars are "simple carbohydrates", (I) meaning their molecules are not that big and are quickly broken down in the body. They



are quickly absorbed into our bodies as an energy source. These sugars may be found naturally in foods, such as fruit, fruit juice, some vegetables, and milk and milk products. Sugars are also found in higher levels in foods like honey, table sugar, candy, syrups and regular soda pop. Note that this last group of foods, although high in sugar, lack vitamins, minerals and fiber. Starches and sugars eventually break down into a substance called glucose, which is used as fuel by the body. When carbohydrates are broken down into glucose in the body, it may help minimize the over-production of porphyrins in the liver. Because this

seems to be such a simple treatment, it is not adhered to by some who don't understand that this simple treatment involves a very complex mechanism. The guidelines suggest that when a person is having symptoms of porphyria, the carbohydrate intake be increased. In fact, some people help prevent attacks by maintaining a high carbohydrate intake daily.



RESEARCH IS THE KEY TO YOUR CURE. Lyon's Share The phone rang very late, so I almost let the voice mail answer but decided I had best answer in case it was an emergency. Indeed it was. On the other end was a desperate mother whose daughter, Beth, had just been diagnosed with porphyria and was in the hospital in Intensive Care. The frantic mother told me that Beth had been ill with terrible abdominal pain that had gotten progressively worse since her teenage years. After many appointments with a succession of doctors who could find nothing wrong with her, Beth had no diagnosis. The doctor suggested

she visit a psychiatrist, but Beth refused because she knew that the source of her symptoms were physical, not mental. Every few months, however, she would call her mother to take her to the Emergency Room, where she would be given pain medicines to relieve the horrendous pain and be sent on her way with no diagnosis in sight. Over time, she and her mother became expected visitors at the ER, and sadly Beth was deemed a hypochondriac and drug seeker. Despite Beth's obvious overwhelming pain and illness and her mother's protestations, the ER staff remained unconvinced that she was very ill. Her mother became more proactive in the search for her diagnosis and finally found a physician who would take Beth's illness seriously. At the onset of Beth's next attack of abdominal pain and weakness, the doctor placed her in the hospital and ordered multiple tests. To the doctor's surprise, Beth's tests were normal, except for a urinary infection which he promptly treated with a sulfa drug. In no time, Beth was in agonizing pain, agitated, and unable to speak coherently except to wail in pain. None of the medical staff in the hospital could determine why.

Fortunately, Beth and her mother had viewed a national television program that the APF had secured featuring a patient with similar circumstances, including the strange dark urine they had seen in Beth's bedpan. On a hunch, she found the APF website and called immediately and reached me. The overwrought mother asked if porphyria could be Beth's illness. I explained that porphyria cannot be diagnosed via symptoms but that the APF could send Beth's physician a wealth of material on diagnosis and treatment and an emergency number to procure Panhematin® if needed. I also assured her that an expert would consult with her doctor if needed. With the information, the physician was able to diagnose Beth and began treating her with Panhematin®. Beth began to recover quickly. **YOU made it possible for the APF to help save Beth's life!**

The next morning, I began writing newsletter articles. The first was on the *Protect the Future* program to train future porphyria experts. We now have twenty young doctors in training as clinicians and researchers. They are vital to our future health and the health of our children. Many of the present experts are approaching retirement. Their expertise will be lost without this training program. Please consider a donation specifically to this program. **YOU made this program possible, too.**

During the rest of the day, I spoke with several of the experts about their research projects and the number of patient volunteers they needed. Next, I contacted Medicare representatives about disability requests. The following task was a call to the FDA to inquire about the EPP trials and the Office of Rare Diseases to ask their help to gain funding for the multiple research projects underway for the acute and photosensitive porphyrias. My last call of the day was to a person who suffered from PCT caused by Agent Orange. YOUR donations enabled the APF to be the major force in securing government funding for veterans who acquired PCT after exposure to Agent Orange. These are all APF services and programs that **YOU made possible.**

For 30 years, the APF has been the only US foundation with a board of experts who assist porphyria patients. YOUR support has made possible great advances in research, physician and patient education and patient support. The APF is YOUR foundation and exists to serve **YOU and YOUR families.** Now the APF needs your help to continue all these valuable programs and services. We need YOUR donations to help us continue the PTF program and expand our physician and patient education programs, as well as our research efforts. Every one of YOUR donations are tax deductible. One dollar is as important as one thousand, because it comes from YOU!!! Thank You.

UPDATING BROCHURES The APF will be updating our *EPP* and *PCT* brochures, as well as our *Diet and Nutrition* and *Safe/Unsafe Drug* brochures. We have already updated our *Panhematin* and *Management of Acute Porphyrias*. Each of these brochures is written and reviewed by porphyria experts as is the medical information on the APF website. This is crucial to provide you with the most reliable information, particularly since misdiagnosis and misinformation on the porphyrias is widespread.


If you have suggestions on what you would like to see in these brochures that is not already included, please let us know. Your input is essential, because you are the ones sitting in the Emergency Rooms and doctors' offices.

Watch the APF website for news on the brochures. We mailed an updated *Panhematin* brochure to all the people on the APF database, who have an acute porphyria. If you did not receive this brochure, please contact the APF and we will remail the brochure to you. If your doctor did not receive a packet with this new brochure, please give your doctor's names and we will send one to your doctor, too.


AN ADVOCATE UNTIL THE END Dear friend and longtime APF member, Florence Rollwagen, Ph.D. recently passed away from liver complications due to EPP. We owe a great deal to Florence as she was the first person to have a liver transplant for EPP. Although the transplant lasted over twenty years, her new liver was severely damaged from the EPP. A patient of porphyria expert Dr. Joseph Bloomer, she participated as a patient volunteer for both Phase II and Phase III clinical trials with Afamelanotide. Unfortunately, she received the placebo both times. Florence traveled to the FDA with the group of EPP patients and gave a powerful testimony about her severe photosensitivity and how despite the placebos, she continued the trials to help other patients.



Florence received her Ph.D. at Cornell University and worked in pathology at the National Institute of Health in Bethesda, MD. Because of her medical experience, she diagnosed herself with EPP, and the diagnosis was quickly confirmed after a skin biopsy by her dermatologist. Her EPP worsened over time until a liver transplant became necessary. Concerning her EPP journey, Florence said, "It seems as if my whole life was spent overcoming obstacles, career, health, education. I'm definitely a stronger person for it, and although it's been hard, I feel prepared for anything that can come my way." Her story can be found in the Member Stories section of APF website. The following is a poem she wrote after her transplant years ago. *Hello again, Death, Back again for another try? Oh, you'll win, eventually, But not today!*

 **IN MEMORY** We have so many friends at the APF, like Florence. It is very hard to lose any of our friends and colleagues. Many of the following APF friends have passed away recently. Many of them have been friends of our APF staff. We are saddened to make this list, but feel that you would want to know too. The following loved ones have chosen to honor the memory of their loved one with a donation to the APF. Please join us in thanking them for their gift which will benefit all who have porphyria:

Scott Zumbrink, Mr. & Mrs. Whit Johnson III, Gerald L Parisi (ISupply Company), Brooke Pinkerton and Ches Johnson, Raymond and Wanda Jordan, Mary A Smith, Mr. & Mrs. Thomas M. Smith, Mary & James Prier, Dolores & Richard L Wiegand, Kay E Sandusky, Mary & Patrick Burch, Kelli Cember on behalf of Delta Gamma, Monica M Snow, Ellen P Havkahl and Ken Gold, Amy & Dave Breweer, Theodore & Betty Dodds, Deborah & Michael Kenton, Patricia L Jones, Linda S Jones, Selina McDine, Donna and James Penny, Jr., for *Linda D. Manos*; Goble and Associates, Inc., Karl and Patty Anderson for *Ellane Heflin*; Donald Johnson for *Peggy Lewis Johnson*; Nettie Carter, Charles Laidlaw for *Mary Jo Benham*; Thomas A Ford, Jr. for *Tommy and Randy Ford*; Mary Crown for *Dean Puccia and Mary B Crown*; Thomas W. Walsh for *Jane I Walsh*; Ralph Gray for *Gary Eyster and Fred Gray*; Norma and Ronald Brown for *Matthew Brown*; Kimberly B Watkoske for *Mary Ann Blanchette*; Davida Hansen for *James C Hansen*; Michael P and Carol L Farina for *Vincent K Farina*; Thomas A Ford, Jr. for *Tommy and Randy Ford*; Carole E Kuklewski for *Vince Kuklewski*; James and Lisa Ezell, Jr. for *Randall A Herbst, Sr.*; Kathleen Toelkes for *Donna Pagano*; Anne Williams for *Florence Rollwagen*; Beth Hosier for *Cindy L Gerley*.

 **IN HONOR** We also thank those who donated to the APF in honor of a friend or family members. It is such a wonderful way to show that you are interested in porphyria and helping find better treatments for this group of diseases: Mary Frances Donnelly, Myrna C Cartledge, Robert Hendrix, Theron and Jolynn P Foldesi, Allene C Martin, Kent Martin, Ian Gray, Arlene De La Mora, William A Gray, Paula Hendrix, Ruth Wilson, Lori Hansen, Grayfred Gray, Sarah and Lawrence Pritchard for *Ralph Gray*; Stephanie Adler for *Melinda Marcala*; Catherine M Gear for *Gloria Sheehan and Family*; Michael and Deborah Pagano for *Andrea Pagano Reyes*; Robert and Margaret Whittenburg for *Jocelyn and Jamie Whittenburg*; Richard J Drew for *Michael J Drew*; Lisa J Baria for *Alexis and Nicolas Baria*; Patri-

cia Hoover for *Cassie Hoover*; Michael Leppert, David Russell for *Craig and Nicole Leppert*; William D McCabe for *Kathie E McCabe*; Elaine E Sasso for *Elizabeth Britton*; Diana Parrish for *Megan Davenport*; Anne L Johnson for *Candace Johnson*; Kathleen Toelkes for *Valene Jensen and Linda Peterson*; Carole F Gaudette for *Tristen Gaudette*; Edward Geffner for *Mira Geffner*; Zila Reichman for *Danielle, Arielle, Lielle Ovadia, Tobey Reichman*; Jessica Melton and Family for *Desiree Lyon Howe*.

YOUR STORY IS NEEDED Over the years we hear a recurrent comment: "One of the best parts of the newsletter and APF website are the patient/member stories." People can identify with one another and the experience of having a rare disease is not so isolating. Please consider writing your story or telling one of the APF staff who will write it for you. If you will read the Members Story section of the website, you will see a variety of people with all types of porphyria who have contributed. We would like to read about you too.

EMA MEETING IN LONDON In February, Desiree visited the European Medicines Agency (EMA) headquarters in London to discuss the need for a treatment for EPP and the great success of the newest treatment, Afamelanotide/Scenesse. The EMA is the European Union agency for the evaluation of medicinal products. Roughly parallel to the U.S. Food and Drug Administration (FDA), but without FDA-style centralization. They did, however, coordinate their efforts with the individual members of the EU. The EU is currently the source of about one-third of the new drugs brought onto the world market each year.

Desiree's trip to meet with the EMA was very beneficial and was followed closely by a visit with a number of European patients, including EPP patients, Jasmin Barmin, who is an EPP researcher in Switzerland, a mother and son from the Netherlands and a German physician with EPP. We are very grateful to our friends in Europe who have given their time to travel to London to make a presentation to the EMA. It is our hope that the EMA and FDA will approve the treatment soon. Please watch the APF weekly E-News for information on the approval process.



PANHEMATIN FOR PREVENTING ACUTE ATTACKS

PANHEMATIN™ (hemin for injection) manufactured by Recordati Rare Diseases is a treatment for the amelioration of recurrent attacks of acute porphyria. In the past, Panhematin was infused to stop attacks. Now it is being widely used to prevent attacks. Many APF members with acute porphyrias are receiving Panhematin to prevent attacks with great success. If you would like to speak with another patient who is receiving Panhematin™ for prevention, the APF can arrange the opportunity to communicate. If your physician would like to speak with an expert about this process, this can also be arranged. In addition, Dr. Karl Anderson is heading a research

project involving the use of Panhematin™. The protocol for the research includes patients who have frequent, predictable attacks. For example, some women have attacks congruent with their menstrual cycle and can, therefore, almost predict the day the attack will begin. If you would like to volunteer for this research study, please call Natalia at the APF at 866.APF.3635. Natalia will put you in direct contact with the research team at the Porphyria Center at the University of Texas Medical Branch in Galveston, Texas.



HEALTHWELL
FOUNDATION®

HEALTHWELL FOUNDATION provides financial assistance to eligible individuals to cover coinsurance, copayments, health care premiums and deductibles for certain medications and therapies for acute porphyria treatment (like Panhematin™). To determine eligibility and apply online, visit: <http://www.healthwellfoundation.org/eligibility>



MORE PANHEMATIN NEWS

Recordati Rare Diseases has instituted a new Patient Assistance program. You may be eligible for the program. If you are interested, please contact the Patient Assistance Program at 1-866-209-7604 (M-F 9AM-5PM ET). Remember, you can always contact the APF about any of these programs. Medical and Safety information is also important. Please give the following numbers to your doctor. Don't forget the CME courses are available online for your doctor's review.

Medical information

For healthcare professionals only
1-888-575-8344 (M-F 8AM-6PM CT)
medinfo@recordatirarediseases.com

Safety information Adverse events/side effect reporting and product complaints

1-888-575-8344 (M-F 8AM-6PM CT)
safety@recordatirarediseases.com