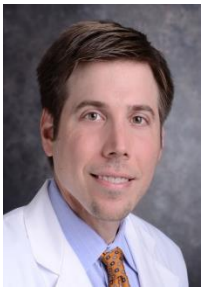
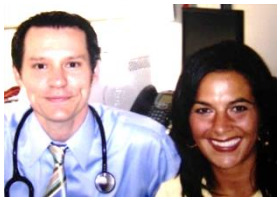
AMERICAN PORPHYRIA FOUNDATION

3nd Quarter, 2013



Dr. Paul Schmeltzer Our newest *Protect the Future* (PTF) trainee, Dr. Paul Schmeltzer, joined porphyria expert, Dr. Herbert Bonkovsky, and the hepatology team at the Carolinas Medical Center in Charlotte, North Carolina in 2011. He works as a Clinical Assistant Professor of Medicine at Wake Forest University and University of North Carolina School of Medicine in transplant hepatology. A native of Northern Virginia, Dr. Schmeltzer earned his undergraduate work at Cornell University with distinction and his medical degree from Georgetown University. He completed his internal medicine training at the University of Pittsburgh Medical Center. Dr. Schmeltzer went on to complete a gastroenterology fellowship at the University of Cincinnati and a transplant hepatology fellowship at the Mayo Clinic in Rochester, Minnesota. His clinical interests include transplant hepatology, hepatobiliary malignancies, drug-induced liver injury, viral hepatitis, and porphyria. Recently, Dr. Schmeltzer received a research grant from the American College of Gastroenterology to study iron homeostasis in Erythropoietic Protoporphyria. This pilot study currently is in progress at CHS.

We are proud to have Dr. Schmeltzer as one of our PTF doctors. It is comforting to know that our future health is in the hands of such an esteemed young physician. For those of you who are not familiar with the PTF program, it was initiated several years ago to train young doctors as the next generation of porphyria experts. Each trainee studies with one of the porphyria experts for a lengthy period of time, as well as attends educational meetings like the recent international porphyria conference. Your donations support this important program. Please consider your participation. If you would like to support this program, please mark your donations "For PTF." Thank you!



First AIP Liver Transplant APF member, Jennifer Long, is a very special young woman. She is the first person in the United States to have a liver transplant to cure her Acute Intermittent Porphyria. After the transplant, she now no longer has AIP. Jennifer's case was so severe that she spent most of the last ten years in the hospital with all the difficult symptoms of AIP: pain, nausea, muscle aches, vomiting, swelling and all the other problems an AIP person endures. Her very normal life took a terrible turn when she was 26 years old. Until that point, she had not had AIP attacks. She was on a great career path and had even just purchased her own condominium. Then she had a menstrual related attack that was quite severe. Six months later she was finally diagnosed but the attacks continued frequently. Regardless of the treatments, nothing would stop the attacks. One attack was so severe she was totally paralyzed for seven months without even the ability to move her fingers. Her recovery took over two years to finally be able to walk again. Still the attacks continued with no end in sight. She tried Lupron to no avail and fortunately was able to have Panhematin, which controlled the attacks but could not stop them. However, she did have three great blessings. First her devoted parents kept watch with her and cared for her every need. Next, she found a devoted doctor, **Dr. David Martin** at Bayview Johns Hopkins Hospital. Although he did not know about porphyria when she first became his patient, he tried to learn everything he could about the disease, including information from the APF and publications from porphyria experts. When it was time for the transplant, she had porphyria expert, **Dr. Lawrence Liu**, as her doctor at Mount Sinai hospital in New York. We are proud of Dr. Liu as he was one of the first PTF trainees, our program to train future porphyria experts. Although it was rough for Jennifer after the transplant, she is now in good health and good spirits.

Dr. Martin was not able to arrange a liver transplant at Johns Hopkins, so he sought out the Porphyria Center at Mount Sinai under the direction of Dr. Robert Desnick. There they began a process with Dr. Liu to have Jennifer enter the hospital and the transplant program. Once her insurance approved the transplant, she became a patient in the hospital on January 20, 2013 and began the two month wait for a liver. When I asked her if she was afraid, Jennifer said, "No!" she was so grateful to have a chance to be well again that she was filled with optimism. On April 25, 2013, Jennifer became the first AIP patient to have a liver transplant to cure her AIP. At that point, things became very rough for her. According to Jennifer, the liver transplant and the aftermath was very difficult, so much so that she wondered for a while if she had done the wrong thing. Like all transplants, there is a period of adjustment to the many medications and the surgery itself. But now that she has had time to heal and no longer has AIP attacks, she says it was all worth the difficulties she endured. Jennifer is a courageous young woman.
attacks, she says it was all worth the difficulties she endured. Jennifer is a courageous young woman.

RareConnect The APF has five active FB groups with over 1000 members for each type of porphyria. Interestingly, *https://www.**rareconnect**.org/* is a new internet support group that promotes global conversation and collaboration to improve the lives of rare disease patients and assist the organizations that serve them. You can meet and interact with others from around the world who share your condition in a supportive and friendly environment by joining Rare Connect.

Andrew Turell…. Diana Ijames…..Mike Kenworthy..Florence Rollwagen..Pierre Mouledoux..Mike Ferry…..Steve Ferry…….. Jonathan Turell…… Kristen Wheeden

SHADOW JUMPING AND THE FDA *What does it mean to "shadow jump?" The FDA representatives had ten people with EPP explain why jumping from one shadow to another to stay out of the sun's harmful rays is a way of life for them.* August 2, 2013 was a memorable day for these APF members, who met at the FDA to explain EPP and ask that the FDA approve Afamelanotide/SCENESSE for EPP. Most of the attendees had participated in the Phase II and Phase III clinical trials and were eager to elaborate on their positive experience on the drug. We sincerely thank Florence Rollwagen, Diana Ijames, Mike Kenworthy, Steve Ferry, Pierre Mouledoux, Mike Ferry, and Andrew Turell, who spoke eloquently about EPP and its effect on their lives. Each person presented common threads and unique aspects of dealing with EPP, as well as their experience with the treatment. It was poignant to hear how a daily life of pain, isolation and anxiety was changed when they were given the implant. Johnathan Turell, and Kristen Wheeden spoke about being parents of a loved one with EPP and the family dynamics that occurs with the disease. Each of their fantastic presentations to the FDA representatives were outstanding.

Although they all had much to say, some of the points they made were unique. Florence was the first person with EPP to receive a liver transplant in the country. Her EPP is so severe that she must work in a closet and live in her windowless bedroom. Diana Ijames did not have to say much for everyone to know the suffering she was enduring. Her hands were already swollen and burned, she could not endure the dimmed lights in the meeting room, and her story of beatings as a child and her present sufferings brought tears to the attendees. Diana also explained that her young son was suffering from Vitamin D deficiency because he did not want to go in the sun when his mother could not join him. Mike Kenworthy explained why visible light affects the skin in EPP and how he dealt with pain so severe that major pain medications were not effective. Twins, Mike and Steve Ferry, told the FDA representatives that although their cases were not as severe as some, they still had great pain associated with EPP and had to guide their day by the light. Their experience on the treatment was like a gift from heaven enabling them to do all the things in life others could do without fear of pain and angst. Mike said the experience was "AWESOME."

Andrew Turell is a young college student who enlightened the group on the difficulties and isolation that comes from always having to answer "No" to the requests of his friends to join them in almost every activity and how he was able to say, "Yes" to every request while on the Afamelanotide treatment. It was heartrending to hear Andrew share how even a campus walk from class was a difficult experience and how EPP psychologically and physiologically affected his college life. Pierre Mouledoux brought yet another slant to the EPP picture, namely how EPP affects his work and earning power. He told the FDA about his concern for the future of his yet unborn child.

Andrew's father, Jonathan Turell, gave a great overview of the EPP family when one member had EPP. The dynamics included how their lives were geared by EPP and how later in life, the EPP sufferer could not participate in many of the family activities, which added guilt to both the EPP sufferer and the family. Kristen Wheeden is the mother of son, Brady, who has EPP. She imparted the sadness, isolation and teasing that Brady endures and how the family compensates in a thousand ways. She also mentioned how her life as a mother is spent protecting him from light. Like all the other EPP people, she, too, begins each morning assessing the light, the weather, the heat, etc. to determine Brady's day and how to make it better for him and the whole family.

Desiree Lyon Howe, Executive Director of the APF, also attended the gathering and shared several of the hundreds of letters that EPP people and their loved ones had sent to the FDA to elaborate on EPP and the great need for a treatment, as well as their phenomenal life change on the Afamelanotide/SCENESSE treatment. Dr. Karl Anderson and Dr. Robert Desnick elaborated on the medical explanation of EPP and what they had learned and seen from the patients on the Phase II and Phase III clinical trials. It is thrilling to see people who were changed by a new drug.

All of the FDA representatives were moved by the presentations and thanked everyone for their participation, as well as all the letters and photos from EPP members, like Shawn Willis. This photo of Shawn on vacation in Wyoming was worth a thousand words.

As a result of the meeting, the FDA has asked Kristen Wheeden to become a member of the FDA Patient Representative Program. Kristen, who is the mother of a child with EPP, is a very impressive, articulate woman who lives in the area and can be available to share her input. She will make an outstanding representative for the FDA and also for the APF and all people with porphyria. Thanks !!

New Safe/Unsafe Drug List Mobile APP APF member, **Nicholas Frias**, has created a fantastic new mobile web APP for the Safe/Unsafe Drug List. Like all the lists for safe and unsafe drugs for the acute porphyrias, the list is meant to be a guide for health care professionals and patients. The prescription of drugs to a patient with acute porphyria is entirely at the risk of the physicians in charge, but they and their patients may find this new APP very helpful. Give the site a try by going to porphyriadrugs.com.

We sincerely thank Nicholas for his interest, time and hard work to create this list and his willingness to share it with everyone. Nicholas and his family are strong supporters of the APF and our mission to help people with all types of porphyria. He has Acute Intermittent Porphyria as do two of his three children. Sadly, he lost a sister to AIP when she was given an unsafe medication and died of the ensuing complications. Since then, Nicholas carried a list of safe and unsafe drugs with him at all times, but now in the era of ubiquitous internet and smartphones, Nicholas decided to create this site and the mobile web app that goes with it. He dedicated the site to people with porphyria. Thank you, Nicholas for devoting your expertise to meet a great need for porphyria.

Since the APF belongs to all of us, we encourage all of you to devote your talents to help others just as Nicholas has done. To participate, please contact Desiree Lyon Howe at the APF lyonapf@aol.com or 866.APF.3635.

Megan Parrish Davenport The events that led to Megan Parrish Davenport's diagnosis were typical of others in many ways but with an added twist. Since she had a myriad of symptoms with no medical reason, she was sent from doctor to doctor. At puberty, she began to be very ill with terrible stomach problems, tachycardia, nausea, anxiety and other symptoms, including the loss of the use of her legs for three days and having to use a catheter. At 16, she passed out driving her car and was thought to have had oxygen deprivation. There was still no medical answer for her endless sickness. Doctors decided she should have antidepressants and see a psychiatrist because there was no medical reason for her many symptoms.

Her mother instinctively took her off all medicine and got an appointment for Megan with her own doctor who told them he would not stop until he discovered what was wrong with Megan. After exhausting all tests and finding nothing, he said, "This is a shot in the dark but maybe porphyria is the answer." He ordered the tests and shortly thereafter discovered that AIP was the correct diagnosis. Megan was finally diagnosed in 2006 after her senior year of high school. She had become extremely ill with an undiagnosed illness. She was in the hospital extremely ill for 53 days. Not only were her parents by her side, but her new month long beau remained at her side, too. That young boyfriend was as scared of losing her as the family and even slept in the chair beside her hospital bed. That same young man is now Megan's husband, Derrick.

Until that time, Megan had endured only minor attacks of Acute Intermittent Porphyria (AIP) but when the critical attack occurred, she found out that the physicians were not prepared for the onslaught of critical AIP symptoms, much less terrible fevers, cellulitis and C.diff, which is a bacterium that can cause symptoms ranging from diarrhea to life-threatening inflammation of the colon. Her attack was so agonizing and devastating that she was sure she was dying. Megan and her mother spoke with Desiree at the APF office who directed them to porphyria expert, Dr. Karl Anderson. He helped them and their physicians administer a regimen to help her with her attacks. The doctors prescribed Panhematin but did not administer it correctly, which set off another series of terrible events. Megan suffered phlebitis for weeks on end and had severe problems with her veins. A hematology team at the Arkansas School of Medicine began to take care of her and installed a PICC line, so they could better administer the Panhematin. The attack began to abate and she began to feel like a normal person again after taking the Panhematin. Eventually, her doctors installed a port, which gave her an even better infusion treatment experience. Megan was also happier with the freedom she was able to have with the port, like swimming and sports. She was also very happy with her new team that included the head hematologist, gastroenterologist, palliative care physician and others who made her life so much better. The attacks still come every few months and at times require hospitalization. However, most often as soon as the symptoms of an attack begin, she heads to the infusion center and has Panhematin infused into her port to halt the attack quickly and goes home to sleep in her own bed that same night. Megan knows now what will bring on an attack, namely being over tired and over stressed, not eating properly and her menses. At 25 years old, Megan says her life with ongoing attacks does restrict how she wants to live, but she is determined to live a happy life with a positive attitude.

RESEARCH-THE KEY TO YOUR CURE ***The Research Consortium has a very good chance at winning a $4.5 million research grant but YOU are needed***. Unless we can locate enough research patients there can be no research trials. These projects are different than others. All that is required of YOU is to donate some of YOUR blood, swab YOUR cheeks for DNA and answer a questionnaire. There are seven studies in which you can choose to participate. Contact Hetanshi Niak at ***hetanshi.naik@mssm.edu*** or the APF.

To be eligible for the Longitudinal Study of the Porphyrias:

- ✓ Must have a confirmed diagnosis of one of the porphyrias
- ✓ It is preferable if patients are able to come to a participating center to be clinically evaluated (through insurance if possible)
- ✓ If patients cannot come to a center they can be seen by a local physician and have their records sent to a participating center along with the necessary samples (DNA, porphyria labs, etc)

To participate in the following studies the patient *MUST* already be in the Longitudinal Study:

- ✓ Erythropoietic Protoporphyrias: Studies of the Natural History, Genotype-Phenotype Correlations and Psychosocial Impact
- ✓ Mitoferrin-1 Expression in Patients with Erythropoietic Protoporphyria
- ✓ Quantification of the Effects of Isoniazid Treatment in Erythrocyte and Plasma Protoporphyrin IX Concentration and Plasma Aminolevulinic Acid in Patients with Erythropoietic Protoporphyria

To participate in Clinical Diagnosis of Acute Porphyrias, there are two parts:

- ✓ Part 1 is looking for 1st degree relatives of patients with confirmed DNA diagnoses of the acute porphyrias (Patients who are subsequently diagnosed with an acute porphyria through this study are then eligible to participate in the Longitudinal Study) We are only recruiting for this part right now.
- ✓ Part 2 is looking for patients with mild elevations in urine porphyrins and symptoms consistent with acute porphyria but who *do not* have a confirmed diagnosis yet (Patients who are subsequently diagnosed with an acute porphyria through this study are then eligible to participate in the Longitudinal Study.)
- ✓ For this study patients do not need to visit a participating center but it is preferred that they do.

To participate in Hydroxychloroquine vs. phlebotomy for porphyria cutanea tarda:

- ✓ Patients must have a biochemically confirmed diagnosis of PCT, these patients are also eligible to be in the Longitudinal Study however it is not required that they be in the Longitudinal Study to be in this study.
- ✓ They must also be over 18 years of age and willing to take precautions to prevent pregnancy.
- ✓ For this study, the patients need to visit a participating center.

THE WARRIOR DASH From the beginning of her diagnosis to now, Merideth McGinley, was not going to sit around and let porphyria attack her without attacking AIP right back. She had a plan that included doing her best to identify what triggers her attacks and also doing her best to enhance awareness and help the APF with its mission to educate doctors and patients, train future experts and support research. Her most recent challenge was the *Warrior Dash*, a 3½ mile obstacle race that she completed in 45 minutes with her boyfriend, Mark Reggiero, by her side. As you can see by the photo, this was no easy race and involved lots of MUD!!!

Merideth was diagnosed with AIP at age 19 by her pediatrician, Dr. Helio Pedro, who she says was determined to find out what was causing her severe abdominal pain, nausea, muscle weakness, etc. He diagnosed her with AIP within only a few days and gave her impeccable care to get her through attacks. When she entered the ER with her next attack, she said it opened her eyes to what many patients endure from some physicians and nurses, namely disbelief, thinking patients are drug seeking, lack of understanding of porphyria, etc. She immediately wanted to participate in changing that scenario by running races and finding other ways to spread the word about porphyria. Now Merideth has discovered that overdoing and menses causes attacks, so she avoids all triggers, like unsafe drugs and alcohol and immediately gets plenty of rest, Panhematin and other techniques to halt the attack. Merideth is blessed to have wonderful parents, Karen and Tom, who helped her recover and stay well and Mark to stand by her side in the hard times and the fun races. Since Merideth lives in New Jersey near the Mount Sinai Medical School, she is volunteering to participate in their research project. What a warrior she is!!!! The APF will be in good hands in the future as we are training future experts and have young people like Merideth, who have taken on the charge of physician awareness and education. THANKS Merideth!!!

UPDATED APF WEBSITE GOES LIVE If you have not visited the APF website in a while, take a look! There are lots of new activities and educational opportunities and lots to come so watch the site carefully. One of the enjoyable sections is on the home page where there are photos of our members with quotes about the APF. You will also enjoy the videos by Tracy Yelen and Amy Chapman about their experience with Panhematin, and sections from the APF video, *Porphyria Live,* "starring" several of the porphyria experts and patients with every type of porphyria. If you are interested in Diet and Nutrition for the Porphyrias, the diet section is HUGE and written by nutritionists and porphyria experts. The section even includes menus and meal plans. With more exciting new sections to come, check the site often. Watch for research news and traveling clinics with experts coming to you soon.

APF T-SHIRTS The APF now has T-shirts. The shirts are top quality and are emblazoned with the APF version of the heme molecule logo as shown on the photo. If you would like to purchase a T-shirt, please contact Amy Chapman. Amy Chapman who is already known for writing the APF Purple Light blog and is serving as a Facebook administrator, is now heading our APF T-Shirt project. You can order a T-shirt by contacting the APF at porphyrus@aol.com or calling 866.APF.3635, and your request will be sent to Amy who will contact you directly. The cost of the shirts are $19.00 with sizes available: S, M, L, XL, XXL, XXXL. Get your order in early because the first round of T-shirts is already out the door. Thank you AMY, we look forward to wearing our T-shirts. You can also order the *Porphyria Live* DVD "starring" experts and patients for only $10. When you order a DVD, your doctor receives one free!

In Memory At the APF we make many friends over the years, so we are very saddened to know of their passing. Often family members and friends make a donation to the APF in their memory. The following people have made a gift to the APF in memory of their loved ones: Barbara Smith for *Kenneth Smith*, Desiree Lyon Howe and Ralph Gray for *Gary Eyster,* Annie Depukat for *Patricia Depukat*.

In Honor Others have chosen to honor their friends through a gift to the APF:
The following people honored Cason and Caul Cook with gifts to the APF for their wonderful Hat Day event for EPP; Cable-Burdette Mechanical, Inc., Bevo's Drive-In, Lisa Herber, Judy Tatum, Mildred Speer, Pao Enterprises, Sally Curtis, Patricia Kidd, Claudette M Owen, William E Pinchak, Linda Shivers, Pam Surber, Donna Hager, Central School Activity Fund, Vernon Middle School Student Activity Fund, Shive Elementary School Student Activity Fund, Coy's Discount Foods No. 2 Inc., Herring Bank, Pepperberries, Inc, Sharon Lawrence, Kathy McClellan, Jean C Clark, McCord Elementary School Student Activity Fund, Lee Ann Cook, Vernon H.S. Student Activity Fund for *Cason and Caul Cook.* Also, Teresa L Witter for *Dee Bruno and Jeanie Lindsay;* Sharon I Koch for *Jagger and Jake Liguori;* Reston Masters Swim Team for *Mike Kenworthy*.

Georgina Davis, who is a member of the APF EPP Facebook Group, lives north of London in Hemel Hempstead Hertfordshire. She is a very creative woman, who is quite adept at drawing, painting portraits, most crafts and writing poetry, which she began 25 years ago, after a bad bout of her other disease, Multiple Sclerosis. Georgina writes poetry to express her feelings and help others understand her EPP. She says that the Facebook groups were a Godsend for her and has thanked the APF for its efforts to promote understanding of the porphyrias to doctors and the general public and most of all for bringing porphyria people together to reduce the loneliness.

Look at the lucky people having fun.
They can go out in the sun,
Scantily clad in the heat of the day.
If we did that, oh how we'd pay.
But, we are rare, not many like us,
Who when the sun comes out,
We 'make a fuss.'
We have a genetic condition you see,
And it goes by the name of,
Erythropoietic Protporphyria aka EPP.
Isolation is its game,
While others play and have fun,
We hide indoors away from the sun.

If we dare to fit in,
My oh my, watch out for the pain to begin.
The slightest exposure can cause such pain
That we don't want to go out again.
Feeling as though our bloods on the boil,
And, lack of understanding for all our toil.
Swelling and itching all part of the course,
When into the sun we are forced.
With large floppy hats, long sleeves and gloves,
Covered all up from our heads to our toes.
What a funny sight to see,
People like us who suffer from EPP.

If you have a talent to use for porphyria, please contact the APF.

THE TEAM Recently a reporter asked why the APF has such great success stories and why other organizations adopted many of their strategies and programs, like the *Protect the Future* program to train future experts, the Emergency Room Guidelines and Assistance, the Global Partners program to assist patients and doctors around the world and our Porphyria Research Consortium, not to mention the eight support networks we have in place. The answer is simple!!! We have the most esteemed team of experts anywhere in the world, and they are willing to help us on a daily basis. To have such a prestigious Scientific Advisory Board (**SAB**) is important to physicians who rely on the APF information and educational programs and services. Because most doctors do not have any experience with rare diseases, like the porphyrias, they want to know that renowned experts have written the materials and conducted the research on the disease they are reading to learn to treat your disease. Our team is not only respected as the best in our country in the field of porphyria, they are also renowned worldwide. It is not unusual for doctors around the world to contact the APF and ask how they can speak with any member of the **SAB**.

As I mentioned previously, at least one member of our **SAB** team works with the APF every day. Since their time is so valuable, I am in awe that they are so devoted to the porphyria community. They are all well aware of the problem that most doctors have no experience with porphyria, and as a group, they try to help physicians who reach out to them. They also assist the APF in developing educational materials for patients as well as physicians. One member of our **SAB**, Dr. Peter Tishler, oversees the Safe and Unsafe Drug list. Plus, he worked with our webmaster to update the list on the APF website and Nicholas Frias, who developed the Mobile Web APP. Experts like, Dr. Steven Shedlofsky, kindly help the APF when doctors and patients in his area need assistance. In fact, all of the experts do this as a service to doctors. Some of the members of this team are shown above with their trainees in the *Protect the Future* program. ***Front Row*** *LtoR Makiko Yasuda, Hetanshi Naik, Desiree Lyon Howe, Dr. Manisha Balwani, Dr. Ryan Caballes, Dr. Guilherme Perini, Dr. Sahil Mittal.* ***Row 2*** *LtoR Dr. Richard Howe, Dr. Cemal Yazici, Dr. Manish Thapar, Dr. Angelika Irwin, Dr. Eric Gou, Dr. Ashwani Singal, Dr. Karl Anderson* ***Row 3*** *LtoR Jason Marcero, Dr. Jeremy Latimer, Dr. Herbert Bonkovsky, Dr. Joseph Bloomer. The only one we cannot see in the very back row is Dr. Herbert Bonkovsky, sorry Dr. Bonkovsky.*

NEW FACEBOOK ADMINISTRATORS Amy Rose Burke and Pierre Mouledoux

Amy Rose Burke is the new APF FACEBOOK Administrator for the PCT–Porphyria Cutanea Tarda. She and her husband, Ronn, whom she says is a loving and compassionate spouse, live in Grand Rapids, MI and are parents of two children and also grandparents. Aside from PCT, Amy also suffers from Multiple Sclerosis. Yet despite her two major illnesses, she owns and directs Rugrats Hideout Daycare, a childcare center which has been in business for 13 years and has 10 employees and 52 children enrolled. Her hobbies are photography and gardening and spoiling her granddaughter. Amy's PCT journey began with odd symptoms, including strange urine color and bad blistering and rash. The typical misdiagnosis odyssey continued until she finally received a correct diagnosis of PCT. Her entire story can be read on the APF website in the Member Stories section. Amy said she became involved with the APF because she is hopeful that more people will understand porphyria and more research can be performed to find a cure. She thanks the APF for helping people connect.

Pierre Mouledoux is the new APF EPP FACEBOOK Administrator. Pierre was diagnosed with EPP at the age of 7 by an intern at Charity Hospital in New Orleans. Since that time, like most EPP people, Pierre has struggled to rearrange his life to accommodate his sensitivity to light. Not one to shy away from the difficulties of the disease, Pierre is a licensed real estate agent and the project manager for Sperry Van Ness/Gilmore Auction & Realty Co. in New Orleans where he lives. He recently passed the Louisiana State Notary Exam and will be receiving his commission soon. Pierre also serves as State Publicity Chairman for Louisiana Ducks Unlimited State Committee, member of the Jefferson Parish Ducks Unlimited and New Orleans Ducks Unlimited Committee and is an Eagle Scout. He and his wife Alyse are expecting their first child in January 2014, which is the major reason Pierre wants to help facilitate getting a treatment and a cure for EPP. In fact, he participated in the Phase III Clinical Trials of Clinuvel's Scenesse/Afamelanotide and recently attended the FDA meeting in Washington DC to push for the approval of the drug. Pierre shared how EPP affects his daily life and what it was like as a youngster with EPP. It was a powerful presentation. He has a great deal to offer all the EPP people on FACEBOOK. Thanks, Pierre!

AN APF FIRST This story is a FIRST !!! Trial volunteers have had to get to their clinical trials appointments many different ways. Some have flown, driven themselves, traveled by train and bus but none rode the entire way to the visit and back in a Yellow Cab. This was a first and we have the photos to prove it. Michelle Jarrett found herself with a problem when there was no way to get from her rural home for her visit with Dr. Herbert Bonkovsky for her EPP trial appointment at Carolinas Medical Center. Since Michelle could not drive there, Elizabeth tried to arrange the travel, but she, too, was unable to find any public transportation. Finally, she found a taxi and a great driver. We thank Michelle for not giving up when she could not arrive at her appointment any other way. We thank her driver, Mike, for taking such good care of his treasured traveler and for understanding Michelle's photosensitive issues and waiting patiently for her to complete the research part of the day and return her home safely!

GARY EYSTER Famed artist, Gary Eyster, passed away recently and graciously left the APF as a benefactor of his estate. Gary had been a longtime supporter of the APF and even in his passing, Gary bequeathed the APF half of the sales of his paintings for the next five years. His friend, APF member Ralph Gray, facilitated this wonderful gift to the APF. Ralph is a renowned artist himself, as well as an art dealer and owner of Webster Gallery in Luling, Texas. Ralph's artistic relationship with Gary Eyster began in the early 1960's in Richmond, Virginia where they painted together and displayed their paintings. Ralph continued to represent Gary's paintings and interested him in the work of the APF. Gary began his illustrious career as an artist after studies at the Philadelphia Museum College of Art, the Ecole de l'Art et Publicité á Corvisart in Paris, the Central School of Arts and Crafts in London, the Lecia School in Germany, the Richmond Professional Institute of the Colleges of William and Mary and the Virginia Commonwealth University. Some of his awards, one man shows and paintings are listed on his website: GaryEyster.com. We sincerely appreciate Gary's gift, and we also thank Ralph for the part he played in this amazing donation.

INSPIRATION FROM ABROAD Greetings again, everyone! My name is Jason Marcero, and I am writing in follow-up to my 1st Quarter APF newsletter article, "Joining the Fight: My Mission to Make a Difference". Since that article, I have continued to conduct porphyria-related research as part of my Ph.D. training at the University of Georgia, including work focused on my own disease, XLPP. Thanks to generous funding provided by the APF, I had the privilege of traveling to the 2013 Porphyrias Congress in late May, set against the Alps in the picturesque city of Lucerne, Switzerland. The remainder of this article will provide a synopsis of my social, educational and motivational experiences at this conference. Without question, the most enjoyable part of the conference was networking with – and learning from – doctors, patients and other trainees from around the world. Eighteen countries in all were represented, with especially large contingents from the APF and the European Porphyria Network (EPNET). Of special note were members of the APF's *Protect the Future* training program, all of whom are clearly committed to continuing and expanding the work of senior physicians and laboratory scientists. In addition, I had the pleasure of meeting two European researchers with EPP who, like myself, are currently investigating their disease. As with traditional science meetings, lectures and posters were the primary means of communicating the frontiers of porphyria research in Lucerne. These formal presentations detailed the latest in everything from biochemical hypotheses for heme biosynthesis and porphyria pathology to clinical case studies and treatment initiatives. The former were based on data collected from animal models, such as mice and worms, as well as human patients. The latter most notably included Afamelanotide/SCENESSE (Clinuvel Pharmaceuticals) for EPP and XLPP and RNAi gene-silencing strategies for AIP (Alnylam Pharmaceuticals), both previously described in this year's 2nd Quarter Newsletter. Opportunities for informal discussions with other participants supplemented growing optimism for future treatments. This was especially true on the final day of the conference, Patient Day, when representatives from organizations like the APF and EPNET from around the world gathered with patients in an effort to strengthen the fight against porphyria. Of particular interest to me was a South African woman with XLPP I met that day. Both she and her doctor explained a unique treatment regimen that has allowed her to sunbathe for the first time in her life.

Personally, the 2013 Porphyrias Congress was much more than a platform for social networking and educating others on the latest advances in porphyria research; it was a motivational experience, as well. Given the entirety of information disclosed in Lucerne, one thing is very clear to me: it is simply a matter of when, not if, effective treatments for all forms of porphyria will be developed. It is my hope that – with the help of organizations like the APF – I can do my part to make "when" happen sooner rather than later.