



THANKS FOR 30 YEARS OF APF BIRTHDAY MEMORIES!

Row 1, L to R: Wendy vor Woert Walk, 1992; Porphyria Experts and Protect the Future Experts, 2009; Rebecca Peek giving Panhematin, 1999; Rupa Gill, 2010; Alamo the Turtle, APF Mascot, 1990; Florence Rollwagen first EPP liver transplant. Row 2, L to R: Support Group, Wisconsin, 1995; Lelia stuffing APF envelopes, 1985; producing APF video, 2000; Allison Linnert, 2008; Yvette and Ovation Leaders, 2006; Dr. Chul Lee in Porphyria Lab at UTMB, Galveston, Texas, 2007; Nicholas Ashby, CEP, 2001. Row 3, L to R: Allie Campbell, member since 1989; Marlene Breezee, member since 2001; Dr. Herbert Bonkovsky, Expert, SAB since 1982; Dr. Gagen Sood, first Protect the Future trainee, 2008; Yvette Strange, APF Office Administrator since 1994; Cook Family, 2011; Finnegan Family, 2007. Row 4, L to R: Dr. Montgomery Bissell, SAB since 1982; Mira Geffner member since 1999; Grace Warfield mans Convention booth, member since 2001; Dr. Joseph Bloomer, Expert, wins Liver Disease Highest Honor, 2010, SAB since 1982; Chelsea Kanscar and mother, Lisa (not shown), have been members for 20 years; Karen Eubanks and daughter, Haley, members for 15 years; Desiree and Dr. Karl Anderson film the first porphyria video with Roland Woerner; Dr. Michael Moore, Scotland, 1984. Row 5, L to R: Dr. Karl Anderson, 2009; Okinawa Honeymoon Story, First Lt. Dano and Andrea Reyes, 2010; Dr. Kenneth Astrin, Desiree, Dr. Robert Desnick, Mount Sinai, 2008; Desiree and Jeff Aronin, Ovation President, receiving Corporate Award, 2007; Dr. Richard Howe fun in cowboy hat at porphyria meeting, 2002; King George play, 2011.

All of the stories are memorable, but here are a few memories, like the time Desiree was a guest in a hot air balloon at her first porphyria meeting and landed in an insane asylum by mistake or the sad time four young girls died from critical attacks within one week or when the FDA told Abbott labs that they had to stop making Panhematin® because their manufacturing plant was not up to par and there were only 100 vials left when Ovation (now Lundbeck) saved the day by purchasing Panhematin®. Or when we began the Protect the Future program to train future experts or the time two porphyria patients met through our IN TOUCH program and discovered that they lived one block from each other.

EPP PHASE III TRIALS MOVE AHEAD THANKS TO APF MEMBERS



Before you read Mike Kenworthy's marvelous, funny description of his and Mike and Steve Ferry's visit with the FDA, I will briefly overview the circumstances we faced concerning the EPP clinical trials. Clinuvel had just been informed by the FDA that they would meet about the Phase III trials in May, which precluded any trials in 2012. I was in despair for a few minutes but bowed my head in prayer and then picked up the phone and asked Elizabeth Pettit at the APF office to locate three EPP people who were articulate and could attend the FDA Advocacy Day. Immediately, she had three volunteers, Mike and Steve Ferry and Mike Kenworthy. Having spoken with all three men, I had no doubt that when they talked, people at the FDA would listen and the result would be Phase III trials. Mike Kenworthy, PhD describes the rest.

I had just gotten back from a sailing trip to the Dry Tortugas (no shortage of sun), and there was a voice mail from Elizabeth, something about an FDA Rare Disease Patient Advocacy Day. This was complete news to me but sounded like something I should attend. I called her back and agreed to attend. She registered me but was told the next day that I was wait listed at number 4,032 for a meeting with a limit of 200 people. Who would have thought that there were so many people with rare diseases right there ready to go? Turns out that there weren't so many with rare diseases -- more on that later. The picture didn't look good. So, we talked to Desiree. She said she would "speak" to the FDA. Short story short, the next day I was in! Must have been something Desiree said. In addition, she had Steve and Mike Ferry moved from somewhere on the waiting list in the three thousands into the meeting as well. It seems that Desiree is a superb diplomat who knows how to persuade with thinly veiled threats. I figure we had them surrounded now -- more on that later. The day arrived, March 1st. I got in my car shortly after 6:00am to make an 8:30am meeting just 25 miles away. For those of you who don't live in the Washington DC area this might not make sense, but I didn't get there until nearly 9:00am. Fortunately, registration went smoothly, and I was seated in time to hear the presentation on the history of the FDA. It began with the cooling of the Earth and progressed slowly through the late nineteenth and early twentieth centuries followed by detailed presentations on the organization, procedures and regulations of each of the dozens (or so it seemed) departments of Center of Drug Evaluation and Research (CDER) of the FDA.

The coffee had begun to wear off. I was beginning to wonder why I was here. Finally, after nearly 3 hours of riveting presentations, the floor was opened to questions. I thought, 'At last. Now we will hear about what is going on with rare diseases.' Several people lined up behind two microphones to begin the Q&A session. I was on the edge of my seat. The first lady was from the National Institute of Health. She gave a 5 minute speech on how impressed she was with the work that the FDA was doing and thanked them for their work. I forget if there was even a question. The next gentleman also praised the FDA and wanted to know about some arcane administrative procedure. This went on for the next several speakers, so I decided that maybe I should ask a question. I went to the microphone and asked, "Was I in the right meeting?" Of course that was rhetorical, so I went on to explain that I was an EPP "patient" and had been part of the Phase II clinical trial for a drug, Afamelanotide, that had proven to be the next thing to a miracle for someone with EPP. I went on to explain that the results had shown a significant improvement in the quality of life (an understatement) with no recorded negative side effects. The trial had been conducted in 2010, and, in 2011, Matt Johnson and I had come and given taped testimony to the FDA (see a last year edition of the APF newsletter). We had been told that the tape would be made available to the FDA and that things would 'move' along, which is 'government speak' for 'until there is nothing else to do.' So my question was, "To whom must I speak and what did I have to do to get things moving?" There was an awkward silence. Then the moderator calmly said that the director of the Patient Liaison Program would address that later in the afternoon, probably hoping I would have given up by then. I thanked him and returned to my seat wondering if my question would even be remembered. After all, I had left out the most important part: What an outstanding job the FDA had done.

Before lunch I met Steve and Mike, only the second two EPP "patients" outside of two cousins that I have ever met (Matt was the first). It was as if we had been friends since childhood. I suppose that in effect we were. In fact, it turned out that we all had been in one of the earliest studies conducted in the US back in the early 60's by Dr. Redeker. As we were talking, a member of the Q&A panel came to us and introduced himself as Richard Klein, Director of the aforementioned Patient Liaison Program. We talked, and he inquired as to which office the reports of the Phase II trial had been filed, hematology or dermatology? We told him that we had no idea but felt that there must be some central database for all trials. Apparently, not so, after all this is the government. We continued our talk and sat down to lunch together. And we did surround them! Steve took the left flank; Mike flanked right and I held the center. Steve pinned down a representative from the CDER while I worked on Richard. Mike was doing the same from my right side. For nearly an hour we bombarded them with everything about EPP and Afamelanotide. Fortunately for them, the lunch ended and the seemingly endless presentations began anew, but the message had been delivered and, more importantly, heard. Richard and others had apparently gotten the message and would do something.

*The following Monday, Desiree called to tell me that things had begun to move - finally, after two years! The FDA was holding a conference call that day with Clinuvel, the company that manufactures the drug for EPP. There could be only one reason to move so quickly, but no guarantees. Sure enough, the next thing we heard was the Phase III trial had been approved. What a team effort! Elizabeth handled the logistics, Desiree the "diplomacy," and Mike, Steve and I the persuasion. Hopefully, this will eventually (in government speak that means many more years) lead to the approval of Afamelanotide for the mitigation, not treatment, of EPP. **Thank you!....Mike Kenworthy, Steve and Mike Ferry, Matt Johnson and all the APF members who wrote the FDA about EPP.***



Wonderful Awareness Week Activities

All of our APF members who participated in the APF **National Porphyrria Awareness Week** are to be commended, but there are a few who promoted porphyria awareness in some unique ways. Wanting to honor her Aunt Becky, who has porphyria, Kate Ruby, decided to bless her in a special way by enhancing porphyria awareness. Kate decided to purchase Porphyrria Wrist Bands and place them in the guest baskets at her wedding. This is certainly one of the most unique means of letting people know about porphyria and one of the dearest gestures of love for her aunt, Becky Thompson. Thanks Kate and Congratulations on your marriage! We wish you much happiness and good health.



Hat Day in Wichita

The Cook Family take their APF membership seriously. Since they became members, they have been very active in our **National Porphyrria Awareness Week** (NPAW) programs to enhance porphyria awareness in their community. The reason is that LeAnn and Chris have two sons, Cason and Caul, and both boys have Erythropoietic Protoporphyrria (EPP). Because of the EPP, the young boys are very photosensitive and must take a number of measures to protect themselves from the sun's harmful rays. Last year, during NPAW, the local newspaper featured the family in an outstanding full page article and family photo about their experience with EPP. This year they initiated a unique and memorable event that was hugely successful and engaged four schools, a local business and the local television stations.

The schools allowed the students to wear a hat for the day if they donated \$1.00 to the APF. The Pepperberries Gift Shop honored **Hat Day**, too, by giving a customer who wore a hat into the store a discount, which they matched for the APF. In turn, the boys explained EPP to the students in four schools. Within a week, the whole town was so enthralled that the television stations produced wonderful news features on Cason and Caul and their Porphyrria Awareness activity. You can view these interviews at www.kfdx.com and www.kauz.com. The story was aired on April 20, 2012. Prior to their porphyria presentation, the boys found a Bible verse that they said God gave to them giving them hope. Psalm 121:5-6 *The LORD watches over you— the LORD is your shade at your right hand; the sun will not harm you by day, nor the moon by night.*

Robert Dawson heightens awareness making presentations for the Dermatology students in his local teaching hospital. Robert says, *"Every time I go to a dermatologist, I offer to let him use me as a learning tool. When they bring all the medical students into the room, the doctor and I describe my symptoms, and then ask the students to name my condition. I believe that over the last year, I have had the honor of helping these young physicians learn about porphyria. I will I keep doing it, because I have the perfect opportunity to spread the word about porphyria."* Each of you can do the same.



Joanna Floyd's (L) used her amazing talent as a photographer to advance porphyria awareness and the work of the APF. Joanna was joined by her fellow porphyria supporter, **Ariel Lager** (R) in her "Promote Porphyrria" photo. Joanna offered her services and sent the funds from her photography to the APF. Joanna is a caring member of the APF FACEBOOK group. See her fantastic photos at <http://www.2jphotography.blogspot.com/>. You, too, can use your talents to spread the word or help the APF enhance our physician education programs.



Others participated in clinical trials, like **Arthur Shull**. Their participation is of ultimate importance for all of us to have a new treatment to abate our symptoms. If you have never participated in clinical trials, you cannot understand the energy it takes to devote yourself to such a project, the result for yourself and others is worth every moment. We thank everyone who participated in all of the porphyria clinical trials, like Rose Jeans, Mira Geffner, Terri Whitter, Herta Woerner, Karen Eubanks, for acute porphyrias and for EPP, Victor Mejias, Sandra Bolding, and Ann Warnke and a hundred others. *Thank you, too, for your long hours!*



Georgina Davies writes poetry to cope with her EPP. EPP can cause severe pain and swelling when the person is exposed to sunlight, certain wave lengths of light, and reflected light from water, sand, concrete, snow, etc. Her simple poem tells the whole story.

Look at the lucky people having fun. They can go out in the sun.



Amy Chapman and Carrie Hunter Raise Funds for Physician Education

Amy Chapman, who is a long time member of the APF and very active porphyria advocate, deserves our thanks for her efforts to raise funds for the APF physician education program by selling Porphyria Wristbands. The bands, which can be purchased in either blue or purple are still available. Amy was able to donate \$1000 to the APF for her efforts. Amy set up her own page on the APF FirstGiving website and used that page to sell the wristbands. Amy, who has AIP, has also been active by writing the APF blog, *Purple Light*, and acting as one of the administrators on the APF Facebook group. She also makes presentations to groups of doctors by asking them to attend and facilitating the meeting. She shares her personal journey with Acute Intermittent Porphyria and shows them the APF DVD, *Porphyria Live*, or the PowerPoint Presentation, which can be downloaded on the APF website. Amy has done this in her local area and even when she went on vacation to Bermuda. *Amy, thank you!!!*



Carrie Hunter is new to the APF but is already an active member. Like Amy, she found the APF FirstGiving website and created her own APF FirstGiving page so that her family and friends could donate to the *APF Physician Education* program. Unfortunately, it takes funds to facilitate so many educational materials, set up exhibit booths at medical conventions, print medical journal articles, etc. Carrie will be collaborating with the APF on other activities in Denver, where she calls home. Please contact Carrie if you live in the Denver area and want to help promote a Fun Run. Such awareness projects can transpire all year long, not just during the April Week. *Thank you, Carrie!*



Kelly Mask Joins APF Staff

My name is Kelly Mask, and I'm very honored and excited to join the team at the American Porphyria Foundation as the Patient & Physician Education Director. A little bit about me – I was born in Kentucky, but have lived in Houston since my early teenage years. I hold a degree from the University of Houston in Arts/Humanities as well as a post-baccalaureate Certificate in Paralegal Studies. My background is in the legal field as a paralegal, a stay-at-home mom for 15 years, then as a book editor/research assistant, and now as a staff member at APF. I've been married 25 years this year, and have two teenagers in high school (and am also hosting an exchange student from France this year), plus two mutts. I love to read, especially biographies and World War II history. I also love to travel, practice my French and am an avid runner, combining all a few years ago by running the Paris Marathon. I've always loved to learn, and learning about Porphyria has been challenging and is proving endlessly interesting. I am humbled and grateful for the opportunity to give help and support to porphyria patients and their families, as well as doctors seeking information to improve their patient care. Editor's note: *Kelly's work will entail enhancing physician and patient education programs. One of the most important activities is to help your physician provide you with better health care.*



Lyons' Share: PAIN and More PAINNNNN !!!!

I don't know about you, but I have experienced porphyria PAIN too many times and now I am scared of it. I never, not ever want to experience it again. I know many of you feel the same. That is why at the APF we will be launching a program to educate medical professionals about the intensity of porphyria PAIN so that patients do not suffer the misunderstandings and indignity of being falsely accused as a "drug seeker." PAIN is the one symptom that courses through all of the porphyrias and PAIN is the most socially isolating of all symptoms. Ideally, doctors and all healthcare workers could understand the kind of PAIN porphyria patients suffer but unless a person experiences this flaming PAIN, it defies description. One of the major programs of the APF is to help physicians and other healthcare workers, families and friends understand the PAIN issue and the devastation it takes on the person with porphyria.

First, we will send our database of 2000 doctors this important *PAIN* article followed by a focus on Emergency Rooms. Many patients are received in ERs with less than the attention they deserve often because the staff is having trouble identifying with the extraordinary PAIN porphyria patients endure. Next, since porphyria PAIN is so widely misunderstood, we hope to focus on the major disciplines, like hematology, hepatology, etc, and enlighten the doctors within those fields. Just recently, we were helping a patient who had just been diagnosed in a hospital in Tennessee. She was suffering with an acute attack and the doctor told her since they had finally diagnosed her with porphyria, she should not be in the kind of PAIN she was exhibiting. How could it be possible for any doctor to come to that conclusion, but he did indeed! It was not until he received the *PAIN* article and packet from the APF that he gave the patient adequate pain medication. We know that this scenario occurs far too often, so we at the APF want to help this from not happening again. *Watch for more news on our progress!*