AMERICAN PORPHYRIA FOUNDATION

AMERICAN PORPHYRIA FOUNDATION

Educate. Support. Advocate. ***Together.***

FALL 2026 **NEWSLETTER**

IN THIS ISSUE

Announcements	2-3
Member Stories	4-5, 7
Treatment Explained	6
Advocacy	8-9
News & Resources	10
In Memory & Honor	11

NICOLE IS BACK — AND BACK ON NATIONAL TV

Lifetime Network's The Balancing Act features the APF's return to the national stage



On her very first day back at the American Porphyria Foundation, former Executive Director Nicole Castellano was back in action—traveling to Boca Raton to appear on the nationally recognized Lifetime Network *television program, The Balancing Act.*

This marks Nicole's second appearance on The Balancing Act alongside Montel Williams. This time, the focus was on Erythropoietic Protoporphyria (EPP) and the progress being made for the EPP community. Nicole shared treatment information and highlighted the APF's newest initiative for children with EPP—Shade Squad—a fun, educational program for students in kindergarten through sixth grade, featuring cartoons, activities, and materials that help children and families understand and manage life with EPP. Additional topics included the APF's EPP School Guide, which helps schools support students with photosensitivity, and the Foundation's growing network of monthly patient meetings held across the country.

The production team at The Balancing Act was amazed to learn about Nicole's remarkable health journey since her last appearance. Over the past year, she underwent major open-heart surgery to replace her aortic valve and repair an aortic aneurysm, followed by a long and challenging recovery while also battling Fluoroquinolone-Associated Disability (FQAD), a debilitating condition associated with a commonly prescribed class of antibiotics.

Throughout her recovery, Nicole remained determined to return to the work she loves. With the encouragement and support of the APF staff, Board of Trustees, volunteers, and members, she never gave up on her goal of returning to the APF and continuing to serve the porphyria community. Everyone is thrilled to welcome Nicole back. Her first day could not have been a more fitting beginning—sharing hope, education, and the latest advances in porphyria care with a national television audience.

The Balancing Act AIRING ON Lifetime

1 **113,000+** WEBSITE VISITORS

2 **190** COUNTRIES REACHED

3 **200,000** NEXT GOAL

THE APF REACHES ANOTHER INCREDIBLE MILESTONE

Over the past several years, the APF has welcomed more than 110,000 new visitors to our website annually. And now, we've done it again! More than 113,000 new visitors from 190 countries have come seeking information, education, support, and hope.

For a group of rare diseases like the porphyrias, this is extraordinary. You're going to love our new APF website—watch for the announcement soon. Our next goal: 200,000 visitors! Let's keep making porphyria impossible to overlook.

SCENESSE® APPROVED IN CANADA

CLINUVEL's SCENESSE® (afamelanotide) has been approved by Health Canada to help prevent phototoxicity in adults with erythropoietic protoporphyria (EPP), following authorizations in Europe, the U.S., and Australia. EPP causes painful phototoxic reactions to light and affects an estimated 5,000-10,000 people worldwide; over 21,000 doses of SCENESSE have been given globally.

Visit SCENESSE.COM to learn more or contact the APF to connect with an EPP patient being treated with SCENESSE. Thank you to Clinuvel, and to Ian McCool and Kelly Burns from the Canadian Association for Porphyria.



IVY SNOW AND HER APF "ATTACK DOG"



Ivy is a fun-loving 11-year-old with HCP, along with her mom Amanda and grandmom Jaime – a close family and wonderful porphyria advocates. Ivy has her own APF "attack dog" for comfort, named Max after Dr. Max Dobrschansky, who described the first case of acute porphyria. The plush dogs wear their own APF T-shirts, available at the APF store: **porphyriafoundation.org/apf-store.**

PATIENT SUPPORT MEETINGS



Would you like a meeting in your area? The APF has in-person Patient Educational and Support Meetings across the country, giving patients and families a rare chance to meet others affected by porphyria while receiving medical and emotional guidance.

Watch our website, eNews, Facebook, and the *RARELY DISCUSSED* podcast for meeting announcements. Want to help bring a meeting to your area? Become a Host Leader – Contact the APF at **866-APF-3635** or **general@porphyriafoundation.org**.

A COMMUNITY THAT SHOWS UP



From national TV to every hat worn, every story shared, every interaction in our online support groups, and in-person patient meetings, APF EMPOWERS every person and family impacted by porphyria.

Other patients' journeys are one of the things that help new patients feel less alone in fighting this rare disease. If you'd like to share your story, we guarantee there's someone who needs to hear it. Please email **general@porphyriafoundation.org** or call 866-APF-3635 for more information on participating in our community.



GONDOLABIO AND PORT-77

GondolaBio announced positive Phase 2a results for PORT-77, an oral therapy for Erythropoietic Protoporphyria (EPP) and X-Linked Protoporphyria (XLP), showing a 79% reduction in plasma protoporphyrin IX (PPIX). PORT-77 works by helping the body clear excess PPIX, acting as a disease-modifying treatment designed to reduce sun sensitivity over time.

Two U.S. sites are now open for PATHWAY, GondolaBio's clinical research study evaluating PORT-77 in people living with EPP or XLP. If you're interested, please contact a site directly using the information below—their study team can answer questions, discuss what participation involves, and determine eligibility.

CALIFORNIA **Marvel Clinical Research**

PI: Dr. Manucher Darani · Huntington Beach, CA

Kresalyn Docuyanan, CRC

kdocuyanan@marvelclinical.com · 714-375-5970

OHIO **Remington Davis, Inc.**

PI: Michelle Chambers · 1335 Dublin Road, Suite 106A, Columbus, OH

RDI634Portal@remdavis.com · 614-487-2560

PATHWAY is open to individuals ages 12 and older with a confirmed diagnosis of EPP or XLP who have symptoms consistent with the condition and meet the study's other eligibility criteria. Participants are randomly assigned to receive PORT-77 or placebo during the blinded portion of the study; those who complete it may enter an open-label extension in which all participants receive PORT-77 for up to 12 months.

QUESTIONS ABOUT PATHWAY, OR NOT SURE IF A SITE IS CLOSE TO YOU? THE APF IS HAPPY TO HELP—REACH OUT AT GENERAL@PORPHYRIAFOUNDATION.ORG OR 866-APF-3635.

JESSICA MELTON'S JOURNEY



My symptoms started at puberty. After seven hospital visits searching for answers, a pediatric neurologist finally said the word “porphyria.” At 19, a hematologist diagnosed acute intermittent porphyria (AIP) and started me on Panhematin. Since starting GIVLAARI in 2020 alongside Panhematin, I've been able to teach crochet, volunteer, and enjoy everyday life again. The APF has supported my journey since 2001, helping secure treatment access and insurance coverage every step of the way. Jessica leads the APF's Purple Crafters Club—ask the APF for details!

PIERRE'S NEW LIFE IN THE SUN



Pierre Mouledoux was born with Erythropoietic Protoporphyria (EPP), causing extreme sensitivity to sunlight—as a child he covered up in scarves and wide-brimmed hats until a geneticist confirmed EPP. Pierre joined the Bitopertin clinical trial and was so confident in its effect that he bought a boat; after three trials it succeeded, and he can now enjoy the sun with his wife Alyse and their four sons, especially fishing together near their home outside New Orleans.

Please email general@porphyriafoundation.org OR Call 866-APF-3635

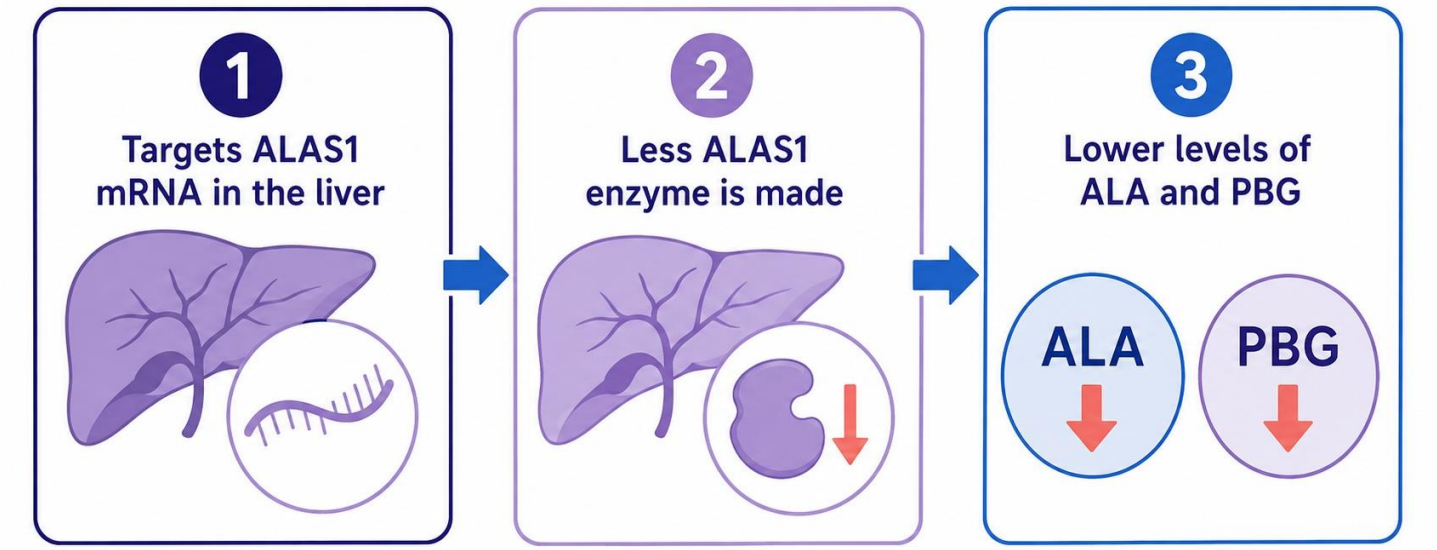
FOR MORE INFORMATION ON PARTICIPATING IN OUR COMMUNITY.

HOW GIVLAARI® WORKS — IN SIMPLE TERMS

We've had many questions about how GIVLAARI treatment works — here's a plain-language explainer

GIVLAARI is a medicine used to help people with Acute Hepatic Porphyria (AHP), namely AIP, HCP, VP and ADP. It works by using a natural process in the body called RNA interference, or RNAi. Think of it this way: your DNA is like an instruction book for your body—it contains instructions for making proteins. Messenger RNA (mRNA) is like a copy of one of those instructions. It carries the message from the DNA to the part of the cell that makes the protein.

Sometimes, the body makes too much of a protein that contributes to disease. This is where GIVLAARI enters the picture. GIVLAARI uses a small piece of RNA called siRNA—think of it as a "stop message." It finds a specific mRNA message and prevents that message from being used to make the unwanted protein. In Acute Hepatic Porphyria, an enzyme called ALAS1 plays an important role in a process that can lead to a buildup of substances associated with acute porphyria attacks. GIVLAARI reduces the message that tells liver cells to make ALAS1. With less ALAS1 being made, the liver produces lower amounts of the substances that can build up and cause problems in AHP.



ALAS1 is an enzyme. ALA and PBG are substances that can build up in AHP and cause attacks.

RNAi medicines work differently from many traditional medicines, which act on a protein after the body has already made it. RNAi medicines act on the message used to make the protein, helping reduce production before the protein is made. RNA interference is a naturally occurring process in our cells; the discovery of how RNAi works was so important that it was recognized with the 2006 Nobel Prize in Physiology or Medicine. In the simplest terms: GIVLAARI turns down one of the liver's instructions involved in the chain of events that can lead to acute porphyria attacks.

This information is for general education and is not a substitute for medical advice. Patients should discuss treatment choices, benefits, and risks with their healthcare provider.

INVITATION TO THE NEW CEP INTERNATIONAL GROUP



Fide Mirón, President of the Spanish Porphyria Association, leads the new CEP group.

Dear CEP Friends, We hope this message finds you very well. We are reaching out from the Global Porphyria Advocacy Coalition (GPAC) with a very special purpose. We know that living with Congenital Erythropoietic Porphyria (CEP) presents unique daily challenges, and that this journey can sometimes feel lonely and difficult to navigate.

We are thrilled to invite you to become part of our new CEP Patient Group, associated with GPAC and led by Fide Miron. This group provides the opportunity to meet CEP friends around the world, built around four clear objectives:



Together, we can raise our voices, make ourselves visible, and open doors that would be difficult to reach on our own. If you want to join or receive more information, please contact the APF at **general@porphyriafoundation.org or call 866-APF-3635.**

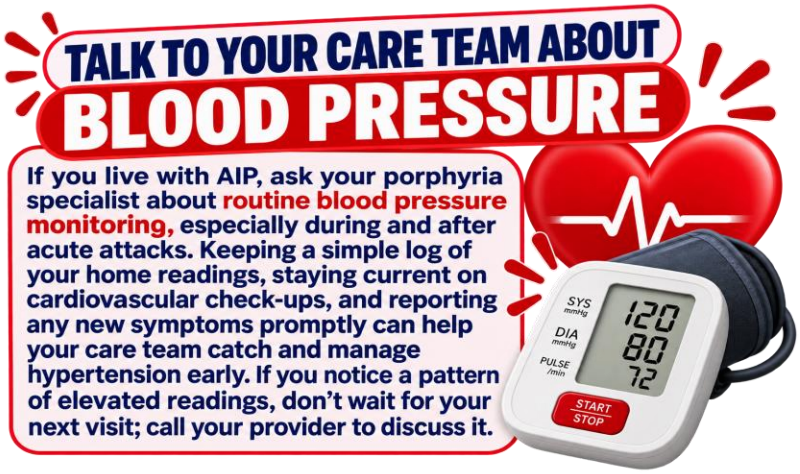
AIP AND HYPERTENSION



According to a recent study, Acute Intermittent Porphyria (AIP) is associated with a higher prevalence of high blood pressure and heart structural changes.

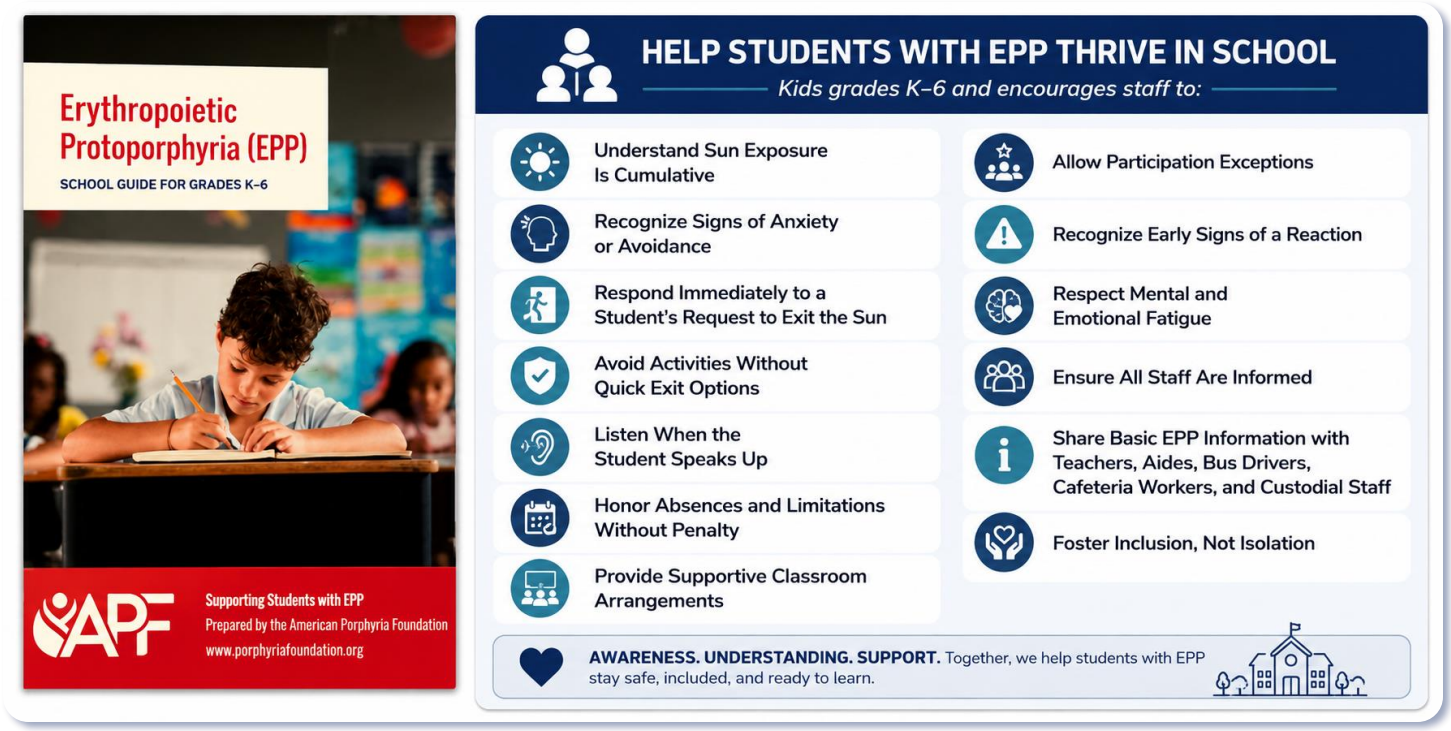
The results suggest that age, body mass index (BMI), and a history of nerve-related muscle weakness or paralysis were associated with higher odds of hypertension. During AIP attacks, blood pressure also rose—possibly due to an increase in stress-response hormones. The study showed that multidisciplinary evaluation of AIP patients impacted early diagnosis and proper management of hypertension in both phases of the disease.

TO READ MORE, SEE PORPHYRIANEWS.COM/NEWS/AIP-RAISE-RISK-HYPERTENSION-HEART-STRUCTURAL-CHANGES/



APF EPP SCHOOL GUIDE

The APF EPP School Guide is an innovative instruction manual for teachers, school administrators, and everyone who interacts with EPP kids to help provide them with a safe environment. The guide focuses on the needs of EPP kids grades K-6 and encourages staff to:

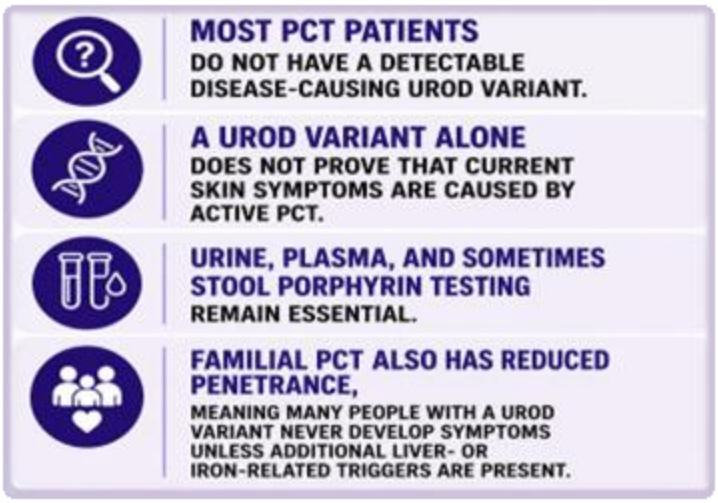


The APF guide also helps educators understand the physical effects of sun exposure for a child with EPP, the emotional toll of constantly avoiding light, and the acute anxiety that often builds with each moment of unwanted sun exposure, especially when the student feels powerless to escape. Thank you to Clinuvel Pharma, who not only supported the Guide, but also accomplished the awesome design and printed the booklet for us. Thank you to Disc Medicine for supporting the project and assisting us in providing access to the EPP School Guide worldwide.



PORPHYRIA CUTANEA TARDA

PCT patients now are asking for DNA testing. It is important to know that genetic testing is useful, but it does not replace biochemical testing. Remember that PCT can also be acquired, which would not show on a genetic/DNA test. UROD genetic testing can identify familial PCT and may help determine whether relatives should be offered testing. However:





THE PANHEMATIN® LOCATOR

Helping patients find treatment when they need it



For patients with Acute Hepatic Porphyria, knowing where to go for treatment during an acute attack can be extremely important. Because porphyria is rare, not every hospital or treatment facility routinely stocks PANHEMATIN®. The PANHEMATIN Locator was created to make that search easier.

The online tool allows patients, caregivers, and healthcare professionals to enter a ZIP code and locate nearby hospitals or facilities that may currently have PANHEMATIN in stock or may be able to access it quickly. It's as easy as searching: **panhematin.com/find-panhematin.**

When someone is experiencing an acute porphyria attack, delays in locating appropriate treatment can add another challenge to an already frightening situation. The American Porphyria Foundation emphasizes the importance of appropriate and timely treatment of acute attacks. PANHEMATIN is a hemin therapy used in the treatment of acute porphyria. Physicians experienced in treating porphyria recommend early use when hemin therapy is indicated. The locator can be a valuable resource before an emergency happens.

If you cannot find a location or have questions about availability, the PANHEMATIN Locator Concierge is available at 866-738-0800, from 8:00 AM to 11:00 PM ET. The concierge can assist with locating PANHEMATIN but does not provide medical advice or discuss whether PANHEMATIN is appropriate treatment for an individual patient. Consider keeping the locator bookmarked on your phone so it is readily available if ever needed.



DONATION IN MEMORY AND IN HONOR

APF friends often give donations in memory or honor of their dear loved ones. We are grateful for each of them.

IN MEMORY OF

David Anderson in Memory of **William Hepp**

Lori Hanson in Memory of **Ralph Gray**

Desiree Lyon and Nicole Castellano in Memory of **Ralph Gray**

Kim Le in Memory of **Ralph Medlin Gray**

Nicole Castellano in Memory of **Paolo Castellano and Laszlo Castellano**

Stephanie Urquhart in Memory of **Pamela A. Dunn**

Dianne Fletcher in Memory of **Darlene and Melvin Bishop**

George Rusnak in Memory of **Carol Rusnak**

IN HONOR OF

Paul Stickler, Warren Hudson, Brian Allen, Desiree Lyon, and Nicole Castellano in Honor of **Tom Pletkovich**

Mike and Rosemary Sieben in Honor of **Nicole Castellano**

Shirley Schrag in Honor of **Desiree Lyon**

Cheyenne Muddiman in Honor of **Cheyenne Muddiman**

Amy Chapman in Honor of **Amy Chapman**

Address Service Requested

AMERICAN PORPHYRIA FOUNDATION
6605 33rd St E, Suite C
Sarasota, Florida 34243

Non-Profit
U.S. Postage
PAID
Sarasota, FL
Permit No. 264