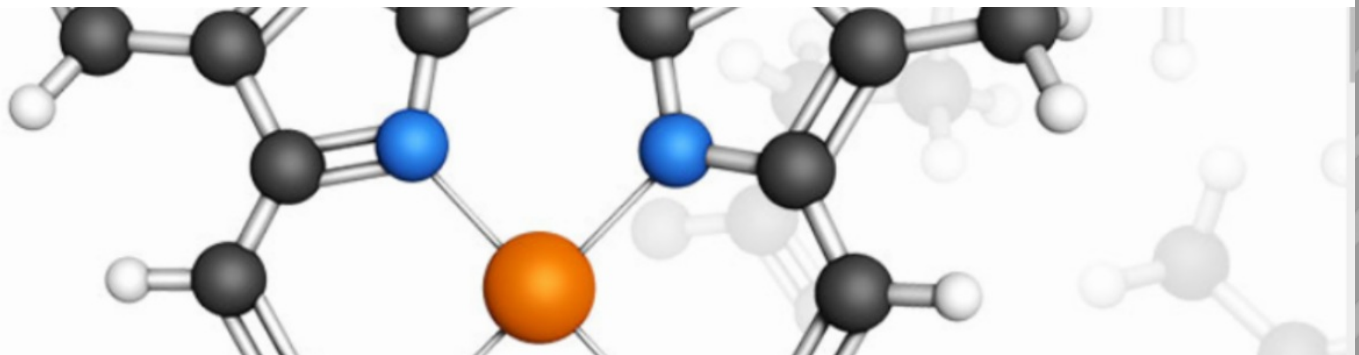




UNITED PORPHYRIAS
ASSOCIATION
Advancing Awareness, Research & Therapies

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Advancing Awareness, Research and Therapies

Hello Member,



Thank You



United Porphyrias Association celebrated its six-month anniversary since our launch in January 2022! We are growing in leaps and bounds and remain hard at work every day serving the porphyria patient and healthcare professional community alongside the porphyria experts.

Our mission is in our name - **uniting** patients, expert and treating physicians, pharmaceutical partners, and advocates in

our vision of a world without the pain and challenges of porphyria. We send deep and sincere gratitude for the tremendous support that UPA has received.

We will continue our UNITED effort!



PATIENT MEETINGS



Dr. Karl Anderson (Porphyria Expert, University of Texas Medical Branch and Chair, UPA Scientific Advisory Board) led an informative virtual Patient Education Link UP on July 28th. After introductions and a few words from Dr. Anderson about the work of the **Porphyrias Consortium**, participants asked a wide array of questions including those about diagnosis, acute porphyria treatments, and

late-onset erythropoietic protoporphyria (EPP).

Dr. Anderson answered with care and compassion. After joining the session, several participants were motivated to participate in current research!



Current Research

IN-PERSON PATIENT EDUCATION MEETING – WE'RE COMING TO BOSTON!

Save the date! **Sunday, September 25, 4-6pm.** Join United Porphyrias and the Massachusetts General Hospital (MGH) Porhyria Center leaders Dr. Amy Dickey and Dr. Rebecca Karp-Leaf for an educational and networking event. Bring your questions and meet others who are impacted by porphyria. Contact UPA for registration and address information.

Email UPA



EPP/XLP CIMETIDINE RESEARCH STUDY

Recruiting volunteers for new EPP and XLP research study

Study Name: Effect of Oral Cimetidine in the Protoporphyrins

Dr. A. Dickey at Massachusetts General Hospital, **Dr. K. Anderson** at the University of Texas Medical Branch, and **Dr. H. Bonkovsky** at Atrium Wake Forest Baptist Health are currently recruiting patients with EPP who are 15 years of age or older for a study investigating whether taking oral cimetidine pills can affect your EPP or XLP symptoms. The study will test whether **Cimetidine** (a medication normally used to treat gastrointestinal issues such as ulcers or acid reflux) can reduce the level of protoporphyrin in your blood, and if it can affect your symptoms.

The entire study will last 9 months. For 4 months of the study, participants will be asked to wear a wearable light sensor and respond to brief surveys send by text message. In some cases, the entire study may be able to be completed with telemedicine visits.

Please contact the United Porphyrias Association (UPA) on 1-800-868-1292 or email info@porphyria.org for more information.

Note: Participants can be on Scenesse (afamelanotide) for this study if symptoms still

occur within approximately 30 minutes of bright light exposure.

Email UPA



PUBLICATIONS



Mount Sinai Science and Medicine

Manisha Balwani, MD, MS, was recently featured in the **Mount Sinai Science & Medicine** magazine (Summer 2022) in an article titled *The Gene Therapy Revolution*.



The article discusses the capacity of RNA and DNA therapies to transform medicine. Dr. Balwani led a study site for the phase 3 trial of the RNA-based treatment givosiran for adult patients with acute hepatic porphyria. "The results showed that patients who received givosiran experienced 70 percent fewer episodes of intense abdominal pain compared to those who took placebo." Givosiran was FDA-approved as Givlaari in November 2019.

"I've conducted clinical trials for more than 10 years now," Dr. Balwani says. "It's been an incredibly rewarding process when these therapies are successful, and you can see how patients' lives are transformed."

We hope you enjoy reading the article! To connect with Dr. Balwani's clinic or to learn more about Givlaari, please contact United Porphyrias at 1-800-868-1292.

Read it Here

Acute Hepatic Porphyria- Patients with Sporadic Attacks

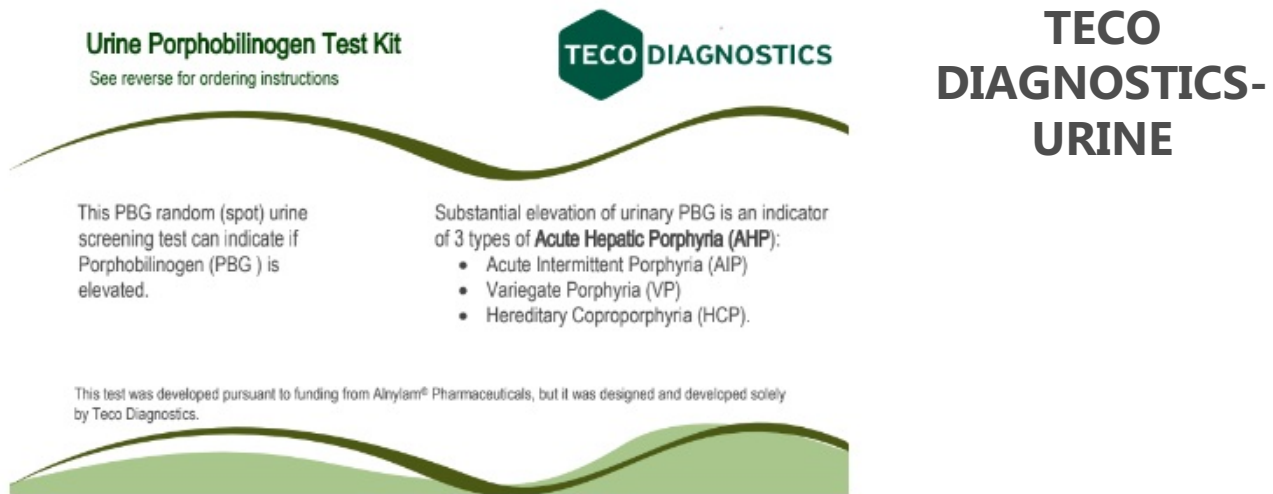
**AHP- PATIENTS WITH SPORADIC
ATTACKS**



A new publication, **Patient Perspective on Acute Hepatic Porphyria with Sporadic Attacks: A Chronic Disease with Substantial Health-Related Quality of Life Impacts**, was released this week. The publication describes the experience of a small set of patients with acute hepatic porphyria who experience sporadic attacks. It concludes that these patients face significant chronic symptoms that frequently require significant pharmacological and clinical treatment.

"While patients with frequent or recurrent attacks (three or more attacks annually) are known to have reduced health-related quality of life...limited data exists in patients with sporadic attacks. This research aims to identify porphyria-related symptoms between attacks, characterize the frequency, severity, and bothersomeness of these symptoms, and more generally understand the burden of this disease in patients who experience attacks sporadically." Kristen Wheeden, UPA, is an author on this publication. Read the article below!

[Read it Here](#)



Urine Porphobilinogen Test Kit
See reverse for ordering instructions

TECO DIAGNOSTICS

TECO DIAGNOSTICS-URINE

This PBG random (spot) urine screening test can indicate if Porphobilinogen (PBG) is elevated.

Substantial elevation of urinary PBG is an indicator of 3 types of **Acute Hepatic Porphyria (AHP)**:

- Acute Intermittent Porphyria (AIP)
- Variegate Porphyria (VP)
- Hereditary Coproporphyria (HCP).

This test was developed pursuant to funding from Alnylam® Pharmaceuticals, but it was designed and developed solely by Teco Diagnostics.

PORPHOBILINOGEN (PBG) TEST KIT

Teco Diagnostics, an industry-leading manufacturer of in-vitro diagnostics and medical devices, recently announced that it has developed a urine rapid test to aid in the primary screening for acute hepatic porphyria (AHP). AHP is a rare and potentially life-threatening genetic disease that often goes misdiagnosed. Because the disease is so rare, physicians will often attempt to rule out other conditions before testing for AHP.

The Teco Diagnostics test is approved as a primary screening tool for AHP. It assists the user by determining the approximate concentrations of porphobilinogen (PBG) in urine within approximately 30 minutes. As this is a primary screening method, every positive result should be confirmed by quantitative estimation.

The rapid turnaround time could potentially help improve patient management and reduce the number of patients lost to follow-up. "It can take an average of 15 years to get a diagnosis for this disease, which from a patient's perspective, can equate to a lot of unnecessary suffering," said Teco leader Aquil Merchant. "Teco realized that there is an unmet medical need for such a test to be in the healthcare sector after speaking with key opinion leaders and experts in porphyrias. This test has been requested for by the FDA as well"—Aquil. The test is currently available For Professional Use Only and can be purchased directly from Teco Diagnostics.

United Porphyrias held an educational training session for the experts of the **Porphyrias Consortium**. Your porphyria specialists are now trained in the ordering and use of this new tool.

Please contact UPA for information on this new screening tool and other diagnostic testing. We will connect you or your physician with a knowledgeable doctor.

Thank you to Alnylam Pharmaceuticals for funding Teco Diagnostics to develop this product!

To watch a demo of the rapid urine screen, click below.

[Watch the Demo Here!](#)

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RECOMMENDED BY CANCER RESEARCH AND COOLIBAR

Background image: A family of four (mother, father, and two children) running on a beach with a large green kite flying in the sky.

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Reach Out to UPA!



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