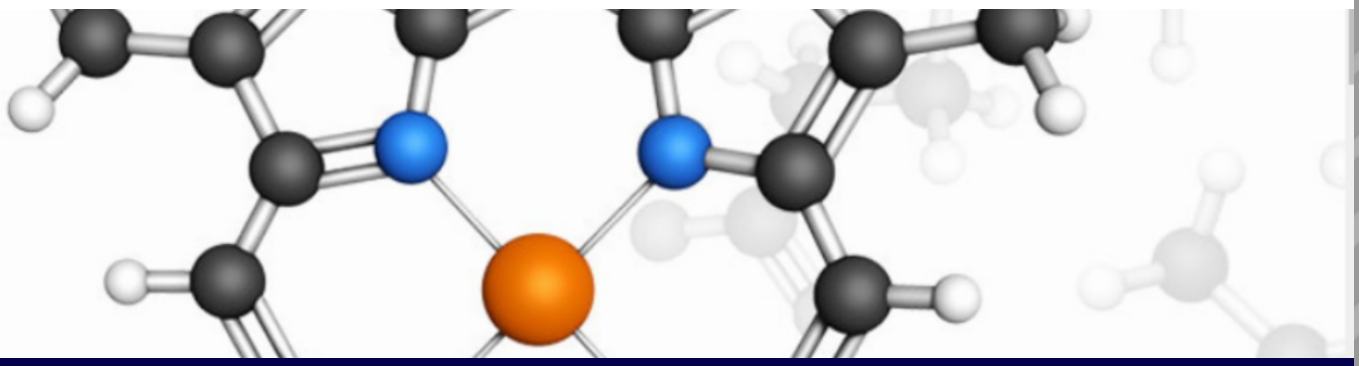
UNITED PORPHYRIAS ASSOCIATION
Advancing Awareness, Research & Therapies

Get in Touch



Advancing Awareness, Research and Therapies

Hello Member,

EPP/XLP CIMETIDINE RESEARCH STUDY

Announcement: Recruiting volunteers for new EPP and XLP research study

Study Name: Effect of Oral Cimetidine in the Protoporphyrias

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.

Email Now

DISC MEDICINE PHASE 2

CLINICAL TRIAL

Disc Medicine has announced a Phase 2 clinical trial for participants with Erythropoietic Protoporphyria (EPP).

A Randomized, Double-blind, Placebo-Controlled Study of Bitopertin to Evaluate the Safety, Tolerability, Efficacy, and Protoporphyrin IX (PPIX) Concentrations in Participants With **Erythropoietic Protoporphyria** (EPP).

- Ten study sites are anticipated
- 62 participants (age 18+)
- Potential enrollment for adolescents ages 12-17
- 24-week trial
- Oral medication (Bitopertin)
- Inclusion/Exclusion criteria will apply
- Must reside in the US

For detailed information visit **www.clinicaltrials.gov** or **click here.**

Disc Medicine is a Massachusetts-based pharmaceutical company focused on Hematology. To read the press release, **click here.**

To learn more, contact United Porphyrias Association on 800-868-1292 or email kristen@porphyria.org.
UPA will connect you with a study site when recruitment begins.

Email Now

UPA LAUNCHES NEW RESOURCE HUB!

United Porphyrias newly launched Resource Hub includes information on rare diseases support, caregiver support, aging resources, and more.
Click below to explore a range of resources available to you.

Read More

EPP CONNECT

United Porphyrias Association is proud to share an invitation for EPP patients, families, and caregivers! In coordination with **Global Porphyria Advocacy Coalition (GPAC),** the umbrella organization for porphyria patient advocacy groups across the globe, we are delighted to invite you to a virtual meeting.

EPP Connect is a first ever event to bring EPP patients together from across the world.

The gathering will be on Zoom and will last 1 ½ hours. There will be breakout rooms in different languages and for different age groups too – all are welcome!
We are very excited and look forward to meeting all of you who are affected by EPP, whether a patient, parent or caregiver – we can't wait to talk with you all!
Once registered, an email confirmation will be sent and Zoom details for the gathering will be emailed directly to you in the week of the meeting!

All attendees must register!

REGISTER HERE

NORD

Last week, Kristen Wheeden (President, UPA) participated in a panel discussion at a National Organization for Rare Disorders (NORD) Industry Council meeting in Washington, DC.

The panel included two industry leaders in patient advocacy (Sanofi Genzyme and Pfizer) and two NORD member organization leaders to share best practices and to exchange ideas about strategies and challenges in industry/organization partnerships.

The response of the in-person and virtual audience of 60+ pharmaceutical companies was terrific as they sought to understand perspectives on effective engagement, and to learn insights on patient organizations' desired involvement in drug development, generating real world evidence, and helping industry understand unmet patient needs.

(Pictured (l-r) Janice Frey-Angel, CEO & Executive Director, Aplastic Anemia and MDS International Foundation; Kristen Wheeden, President, United Porphyrias Association; Debbie Drell, Director of Membership, NORD)

LEARN MORE

United Porphyrias Association (UPA) was proud to be one of the 85 advocacy organizations who came together and to urge the Senate to include S.4185, the RARE Act, in the FDA Safety and Landmark Advancements Act (FDASALA). "The RARE Act would clarify the original intent of the Orphan Drug Act (ODA) and codify the Food and Drug Administration's (FDA) longstanding interpretation of that landmark law."

The ODA provides a set of incentives to support research and development into drugs for rare diseases. One of the key incentives is a seven-year term of "exclusivity" for the orphan drug once approved and marketed. The ODA established a two-part process for obtaining orphan drug exclusivity. First, at an early stage in development, a company can request that FDA "designate" the drug as an orphan drug to prevent, diagnose or treat a rare disease or condition. Once a company receives this designation from the FDA, the company can access other ODA incentives, including tax credits for research and clinical testing of the drug. Second, after completing the necessary clinical studies and obtaining FDA approval, the drug is then awarded exclusivity that protects from competition the specific use of the drug that is approved. In most cases, the orphan designation is intentionally broader than the use ultimately approved.

The letter included information including that the implications could leave some rare disease patients, including children orthose with less common variations of a rare disease, without access to an FDA approved treatment that has been proven to be safe and effective for their specific circumstances and/or condition."

Of note, Panhematin was the first orphan drug approved after the Orphan Drug Act of 1983. Givosiran (Givlaari), afamelanotide (Scenesse), and dersimelagon (MT-7117) all have Orphan Drug approval.

To read the letter that was sent on June 3rd, click below.

Read More

RARE Act UPDATE

Senator Baldwin's amendment to add the RARE Act (S. 4185), as introduced, was adopted into the text of FDASALA during today's Senate markup!
The vote was 13-9, with all HELP Committee Democrats plus Republican Senators Paul and Braun voting in support of Senator Baldwin's amendment.
Next, the bill will move to the full Senate for consideration, but it is not clear when the Senate floor vote will happen.

STAY TUNED FOR UPdates!

Rise UP. Speak UP.

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