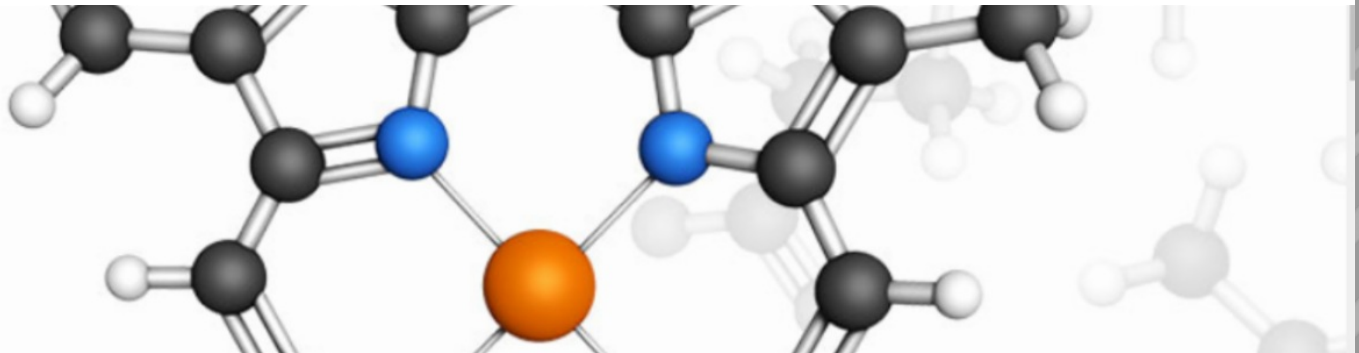




UNITED PORPHYRIAS
ASSOCIATION
Advancing Awareness, Research & Therapies

Sign Up



Advancing Awareness, Research and Therapies

Hello Member,

PHASE TWO CLINICAL TRIAL IN EPP NOW UNDERWAY!

ATTENTION EPP FRIENDS! ANNOUNCING A PHASE 2 CLINICAL TRIAL



disc)medicine

Participant recruitment is *now underway* for a Phase 2 clinical trial on Bitopertin, an investigational treatment for Erythropoietic Protoporphyrria.

This is a Randomized, Double-blind, Placebo-controlled Study of Bitopertin to Evaluate the Safety, Tolerability, Efficacy, and Protoporphyrin IX (PPIX) Concentrations in Participants With Erythropoietic Protoporphyrria (EPP).

Fast Facts

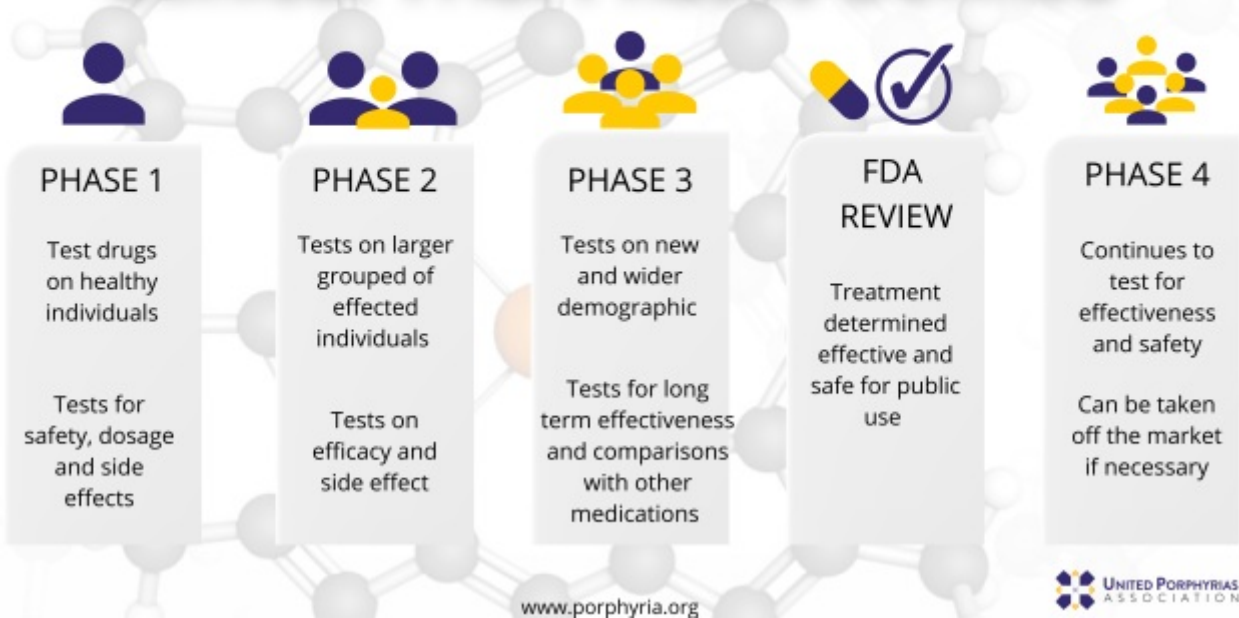
- 10 study sites across the US
- 75 participants needed
- Patients ages 18+ are eligible
- Inclusion/Exclusion criteria will apply
- The study drug is an oral medication
- Study includes an optional open-label extension

This trial is sponsored by Disc Medicine.

Contact info@porphyria.org or **1-800-868-1292** for further information and to be connected to a participating study site.

WHAT IS A PHASE 2 CLINICAL TRIAL?

Clinical Trial Phases Defined



In Phase 2 clinical trials, a new drug or treatment is given to a group of people to determine its effectiveness and to further study its safety. Here is a resource offered by the National Institutes of Health (NIH) to learn more: [Click learn more.](#)

[LEARN MORE](#)



EPP/XLP CIMETIDINE RESEARCH STUDY

Patients with Erythropoietic Protoporphyrria (EPP) or X-Linked Porphyrria (XLP) are needed for an FDA-sponsored research study on cimetidine. The study will assess whether cimetidine (a medication normally used to treat gastrointestinal issues such as ulcers or acid reflux) can reduce protoporphyrin IX (PPIX) levels which may impact photosensitivity.

Participating Study Sites:

- Dr. Amy Dickey, Massachusetts General Hospital,
- Dr. Karl Anderson, University of Texas Medical Branch
- Dr. Herbert Bonkovsky, Atrium Wake Forest Baptist Health

Fast Facts:

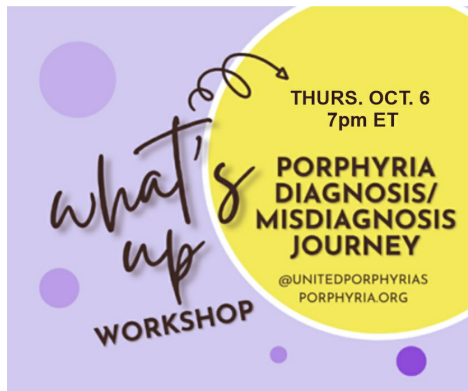
- 3 study sites in the US

- 20 total participants needed
- Patients ages 15+ are eligible
- Participants must experience symptoms within ~30 minutes of sun exposure
- Patients currently on Scenesse (afamelanotide) are eligible

Of interest, a recent study in Denmark indicated the potential for cimetidine to lower protoporphyrin IX (PPIX) in patients with erythropoietic protoporphyria. **Click here to read about it.**

To learn more about study details, **Click here.** Contact UPA on 1-800-868-1292 or **info@porphyria.org** to participate

WHAT'S UP WORKSHOP!



EMAIL NOW

Sign up now for the first **WHAT'S UP WORKSHOP** – happening on October 6th, at 7pm Eastern!

Join other porphyria patients and caregivers to discuss your porphyria diagnosis...or misdiagnosis...journey. This workshop will help us create new resources that will be most useful for the patient community.

Email **info@porphyria.org** to request Zoom login information.

CONNECTING THE TUBE TO THE PATIENT WITH MAYO CLINIC

MAYO CLINIC



The Biochemical Genetics Laboratory at Mayo Clinic and United Porphyrins Association recently held two webinars titled “Connecting the Tube to the Patient.” The goal was to educate biochemical lab staff on the porphyria patient journey and lived experiences behind the lab sample.

The lab was re-educated on testing in the porphyrias, then patients humanized the diseases through a moderated discussion. The cutaneous porphyrias were featured in an

August event followed by the Acute Hepatic Porphyrins this week. The lab staff was reinvigorated with the reminder that every tube is connected to a patient.

UPA thanks Colleen and Morgan McKillop, Jeff Gesoff, Monica Fleegel, Claire Richmond, Donna Bunch, and Mike Boone for sharing their important patient stories. Feedback from the event was tremendous!



“Hearing about conditions from the patient/parent perspective is always enlightening and meaningful”



“Just wanted to thank you for the BGL all meeting today, it was really fantastic. It’s so valuable to hear the patient’s perspective.”



“It’s nice to humanize the disease to the tests being performed in BGL.”



“I think we sometimes forget the impact that these diseases have on patients and their families, and it was so great to be able to hear the patient’s stories.”

PORPHYRIA NEWS



Check out the most recent porphyria news column from Kristen Wheeden after a recent visit to Capitol Hill on behalf of the American Academy of Dermatology and the Coalition of Skin Diseases.

[CLICK HERE](#)

Add United Porphyrias **INSTA, FACEBOOK, TWITTER** to your social media feeds.



UNITED PORPHYRIAS
ASSOCIATION
Advancing Awareness, Research & Therapies

6701 Democracy Boulevard,
Suite 300, Bethesda, MD 20817
(800) 868-1292

[Donate](#)



United Porphyrias Association | 6701 Democracy Boulevard, Suite 300, Bethesda, MD 20817
800-868-1292

[Unsubscribe kristen@porphyria.org](mailto:kristen@porphyria.org)

[Update Profile](#) | [Constant Contact Data Notice](#)

Sent by info@porphyria.org in collaboration
with



Try email marketing for free today!