

The Future of Porphyria...

RESEARCH ROUND UP

PORPHYRIAS CONSORTIUM and UPA SCIENTIFIC ADVISORY BOARD

The [United Porphyrias Association](#) is proud to be the designated patient advocacy organization of the NIH-sponsored [Porphyrias Consortium \(PC\)](#).

The expert physicians of the PC provide expertise and experience in the diagnosis and management of patients with all the porphyrias. Each physician also serves on the [UPA Scientific Advisory Board](#).

The PC shares that “Together with United Porphyrias Association, our only designated patient advocacy group, the Porphyrias Consortium enables a large-scale collaborative effort to develop new strategies and methods for diagnosis, treatment, and prevention of illness and disability resulting from these rare disorders.”

This team of dedicated porphyria specialists include 6 Porphyria Centers and 7 Satellite Porphyria Centers across the United States. UPA engages with each center daily – referring patients, requesting responses to patient questions and concerns, and connecting for peer-to-peer physician consults, and recruiting for current research surveys and studies!

Porphyria patients are at the center of all porphyria research! There are multiple studies going on NOW that need your patient expertise. Check out Latest News in Research projects and recruitment and how to participate.

SEEKING INTERVIEW
PARTICIPANTS
IN THE

UNITED STATES



The United Porphyrias Association (UPA) is collaborating with Aynlam Pharmaceuticals and RTI Health Solutions (RTI-HS) to identify individuals with acute hepatic porphyria (AHP) who are interested in participating in an interview study conducted by RTI-HS (on behalf of Aynlam Pharmaceuticals).

Do You Qualify?

You qualify for this one-time 60-minute telephone interview study if

you...

- **Participated and completed an Aynlam Pharmaceuticals clinical trial for givosiran (the Phase 1/Phase 2 Open Label Extension or "ENVISION" trial),**
- **Continued to take, and currently take givosiran (GIVLAARI®)**

The objective of these interviews is to capture, in the patients' own words, the symptoms and impacts of AHP and their experiences over the time they have received givosiran.

Interview participants will each receive \$100 in appreciation of their time.

Next Steps...

If you qualify and are interested in participating in an interview or just want to learn more about this study, please contact Kristen Wheeden of the UPA by email or telephone:

Email: Kristen@porphyria.org

Telephone: 800-868-1292

MEDICATION STUDY TO INFORM DRUG SAFETY

The NIH-Supported [Porphyrias Consortium](#) has launched an online observational study to collect information on acute porphyria attacks that may have been caused by a medication.

The "Evidence-Based Assessment of Medication Sensitivity in Acute Hepatic Porphyria" requests patient and caregiver reports on suspected medication/drug-induced acute porphyria attacks, as well as safe use of drugs previously labeled "unsafe" or with unknown risk.

The goal of this project is to create an updated list of medications that are risky or safe for people with one of the acute porphyrias.

After consenting, participants 18+ will be provided a unique web link to

NEW STUDY IN AHP

YOUR PATIENT EXPERIENCE IS NEEDED!

STUDY NOW OPEN FOR
ACUTE HEPATIC PORPHYRIA (AHP)



complete the initial survey. Participants will be encouraged to update their surveys when they have new information to report.

To participate, please contact [United Porphyrias Association](https://www.unitedporphyrias.org) on info@porphyria.org or call 800-868-1292.

You are Needed!

STUDY NOW OPEN FOR
ACUTE HEPATIC PORPHYRIA (AHP)
and **Erythropoietic Protoporphyrin (EPP)**



 UNITED PORPHYRIAS ASSOCIATION  THE PORPHYRIAS CONSORTIUM

AHP and EPP

Patients with Acute Intermittent Porphyria (AIP) and Erythropoietic Protoporphyrin (EPP) anecdotally report using **Complementary and Alternative Medicines (CAMs) and cannabis** to alleviate their symptoms, but this has never been formally assessed.

Click the links below to participate in a de-identified survey so we can learn more!


Anyone with [EPP](#) or [AIP](#) who is 18 years or older can participate. If you have never used CAMs or cannabis, you can still participate!

Click Below!

[AHP](#)

[EPP](#)

EPP INTERVIEW RESEARCH STUDY



EPP/XLP INTERVIEW RESEARCH STUDY

Do you have Erythropoietic Protoporphyrin (EPP) or X-linked protoporphyria (XLP)?

Interested in participating in Zoom interview?

We are seeking ~ **20 adults and adolescents with Erythropoietic Protoporphyrin (EPP) or X-linked protoporphyria (XLP)** in the US and Canada who are at least 12 years of age and speak English.

If you qualify for the study, you will be asked to participate in one Zoom interview that will last **no more than one hour**.

Each interview will be scheduled at a time that is convenient for you, including during the day, during the evening, or on weekends and will be conducted by a member of a project team.

After you complete the interview, you will receive a **\$150 Amazon gift card** for your time.

Email info@porphyria.org or call 800-868-1292 to participate!

UPCOMING

Stay tuned for several studies related to:

- CEP
- EPP pediatric/caregiver interviews
- EPP clinical trials, and
- AHP Patient-Reported Outcomes

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