

INTERNATIONAL PORPHYRIAS SYMPOSIUM 2023

ADVANCING THE SCIENCE & ART

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www.porphyrina.org/symposium



 Hyatt Regency Bethesda, MD

 October 26-29, 2023

IMPORTANT DATES

- Early Registration: Before September 1, 2023
- Abstract Deadline: September 1, 2023
- Registration Deadline: October 5, 2023
- Hotel Reservation Deadline: October 5, 2023
- Conference Begins: October 26, 2023

UP TO 25 CME CREDITS

Joint Accreditation Statement

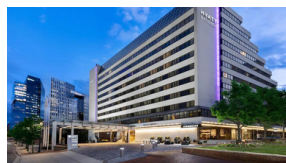
In support of improving patient care, this activity has been planned and implemented by the University of Arkansas for Medical Sciences and the United Porphyrias Association.

University of Arkansas for Medical Sciences is jointly accredited by the Accreditation Council for Continuing Medical Education (ACCME), the Accreditation Council for Pharmacy Education (ACPE), and the American Nurses Credentialing Center (ANCC) to provide continuing education for the healthcare team.



AMA Credit Designation Statement

The University of Arkansas for Medical Sciences designates this live activity for a maximum of 25.0 AMA PRA Category 1 Credits™. Physicians should claim only the credit commensurate with the extent of their participation in the activity.



VENUE

HYATT REGENCY BETHESDA
7400 Wisconsin Ave, Bethesda, MD

ROOMS: \$149 + tax (single or double)

Rooms are limited and the discounted rate will expire in advance of the meeting. All reservations should be made via the symposium website.

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SYMPOSIUM PROGRAM: AT-A-GLANCE

THURSDAY OCT 26

SESSION 1: PORPHYRIAS INTRODUCTORY COURSE (Included in Registration)

An introductory course designed for physicians, researchers, genetic counselors, diagnostic laboratories, research coordinators, residents, pre- and post-doctoral fellows & industry representatives.

1:00 pm Introduction to Heme Biosynthesis & Substrate Pathogenesis

1:30 pm Porphyria Genetics: Inheritance, Prevalence & Mouse Models

2:00 pm Acute Hepatic Porphyrias (AHPs): Pathogenesis, Manifestations & Diagnosis

2:30 pm Acute Hepatic Porphyrias (AHPs): Current Treatments

3:15 pm The Erythropoietic Protoporphyrins (EPP & XLP): Pathogenesis, Manifestations & Diagnosis

3:45 pm Congenital Erythropoietic Diagnosis, Manifestations & Current Treatment

4:15 pm Porphyria Cutanea Tarda (PCT): Pathogenesis, Manifestations & Treatment

4:45 pm Diagnosis & Treatment Issues: Case-Based Approach

5:15 pm Session 1: Q&A and Discussion

SESSION 3: RECENT ADVANCES IN ACUTE HEPATIC PORPHYRIAS (AHPs)

1:10 pm Keynote Speaker: Accelerating Treatments & Cures for Rare Diseases

1:30 pm AHPs Recent Insights into Pathogenesis & Comorbidities

2:00 pm AHPs: Prevalence, Modifying Genes & Diagnostic Algorithms

2:30 pm Therapy for AHPs

2:30 pm Experience with Hemin

2:45 pm Experience with Givosiran

3:30 pm Autonomic/Small Fiber Neuropathy in Acute Hepatic Porphyrias: Data from Objective Testing

4:00 pm AHP: Use of Complementary & Alternative Medicine

4:20 pm Acute Intermittent Porphyria (AIP)

4:40 pm Variegate Porphyria (VP)

5:00 pm AHP Rare Variants: Characterization & Management Issues

5:25 pm Open Discussion: Moderated by Session Co-Chairs

5:45 pm Selected Poster Presentations: Moderated by Session Co-Chairs

SATURDAY OCT 28

SESSION 4: RECENT RESEARCH IN HEME BIOSYNTHESIS & PORPHYRIAS

8:00 am Single-Cell RNA-Seq of Murine & Human Liver Cells

8:30 am ALAS1 & Hepatic Heme Regulation

9:00 am Update on Heme Transport

9:30 am Acute Attacks in AHPs: Mitochondrial Studies

10:00 am HCC in AIP: HMBS As A Tumor Suppressor Gene

10:30 am Emerging Treatments for AHPs

11:00 am Mechanism of ALA Accumulation in AHPs & Pharmacologic Chaperones for CEP

11:30 am Selected Late Breaking Abstract

SESSION 5: RECENT ADVANCES IN ERYTHROPOIETIC PROTOPORPHYRIAS (EPPs)

1:00 pm Keynote Speaker

1:20 pm Erythropoietic Protoporphyrins: Consensus Guidelines

1:45 pm EPP Patient Interview

2:05 pm EPP/XLP Case-Based Liver Complications Panel

2:25 pm Erythropoietic Protoporphyrins: Current & Emerging Treatments

2:25 pm Experience with Afamelanotide Experience with Cimetidine Trial

2:45 pm Bitopertin Clinical Trial Results

3:05 pm Emerging Therapy: ABCG2 Transporter Inhibition

3:25 pm Dersimelagon Clinical Trial Results

SESSION 6: RECENT ADVANCES IN CONGENITAL ERYTHROPOIETIC PORPHYRIA (CEP) & PORPHYRIA CUTANEA TARDA (PCT)

4:05 pm Congenital Erythropoietic Porphyria (CEP): Pathophysiology & Manifestations

4:25 pm Current & Emerging CEP Therapies: Panel Discussion

4:45 pm CEP Patient Interview

5:05 pm Porphyria Cutanea Tarda: Diagnosis, Manifestations & Treatment

5:30 pm PCT Patient Interview

5:50 pm Open Discussion: Moderated by Session Co-Chairs

6:05 pm Selected Poster Presentations

SUNDAY OCT 29

Session 7 Concurrent with Porphyria Patient Day

SESSION 7: CURRENT STATUS OF PATIENT CARE, RESEARCH, ADVOCACY & FUTURE PROJECTIONS

9:00 am Need for Harmonization of Porphyria Diagnostics & Reporting

9:30 am Award-Winning Selected Oral Poster Talks

10:30 am Porphyria Forward-Looking Predictions

11:00 am Closing Remarks

PORPHYRIA PATIENT DAY

(In-Person & Live Stream)

10:00 am Welcome & Introduction - United Porphyrias Association

10:10 am UPA Scientific Advisory Board & The Porphyrias Consortium

10:20 am Patient Spotlight - Featured Patient Story

10:30 am Porphyrias 101: The Basics

10:30 am Bringing the Heme Biosynthetic Pathway to Life

10:00 am HCC in AIP: HMBS as a Tumor Suppressor Gene

10:30 am Emerging Treatments for AHPs

11:15 am Present Therapeutics, Future Advances & Research: Where Are We Headed?

11:30 am Patient Point-of-View: Moderated Patient Discussion

12:00 pm PORPHYRIA PATIENT DAY LUNCH

Section 1: Acute Hepatic Porphyrias (AHPs)

1:00 pm Important Issues in AHPs: A Discussion

1:15 pm AHP Guidelines & Patient Resources

1:45 pm Pain & Neurology in AHP

2:15 pm AHP Power Study

2:30 pm Ask the Experts: Panel Discussion

3:00 pm Closing Remarks

Section 2: Cutaneous Porphyrias

1:00 pm EPP/XLP Consensus Guidelines

1:20 pm Photosensitivity & Light Dosimetry in Cutaneous Porphyrias

1:45 pm Current Studies & New Treatments in the Cutaneous Porphyrias

2:15 pm Shadow Jumpers: Sun Avoidance & Emotional Health

2:30 pm Ask the Experts: Panel Discussion

3:00 pm Closing Remarks

FRIDAY OCTOBER 27

10:00 am Welcome & Announcements

SESSION 2: WESTERN HEMISPHERE PORPHYRIA RESEARCH & ADVOCACY FORUM

Presentation of Diagnostic Capabilities, Available Therapies, Regional Expertise, Research Activities & Advocacy Groups

10:05 am Intro & Session Goals

10:15 am Argentina

10:30 am Brazil

10:45 am Canada

11:00 am Chile

11:15 am Mexico

11:30 am United States

11:45 am Panel Discussion: Establishment of Western Hemisphere Porphyria Working Group

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