Issue 49 / **June 2025**

THE BRITISH PORPHYRIA ASSOCIATION NEWSLETTER

Registered Charity No. 1089609

CARDIFF MEETING 2025

Our 2024 Patient Conference took place on Saturday 29 March 2025 at the beautiful National Museum of Wales in Cardiff – and what a fantastic day it was! We were thrilled to welcome patients, families and friends, alongside healthcare professionals from Cardiff and across the UK, speakers with lived experience – who travelled all the way from Ireland, international scientists, and our industry partners.

Together, this inspiring group helped create an engaging, informative and supportive day filled with shared stories, learning and new connections. It was especially meaningful to meet newly diagnosed patients and families we hadn't connected with before – some of whom we've since been able to offer further support. That alone made the event feel incredibly worthwhile.

The day before the conference, a few members of the BPA team were proud to present and take part in the BIPNET Annual Conference. Our collaboration with this clinician-led network continues to grow, and it's a brilliant way to keep sharing knowledge and improving care for people living with porphyria.

SAFE DRUGS LIST FOR THE ACUTE PORPHYRIAS:
NEW TELEPHONE NUMBER ALERT!

From 23rd June 2025, the UK Porphyria Medicines Information Service (UKPMIS) can be contacted on **029 2003 2011.**

Our email address remains unchanged *WelshMedicines.Information@wales.nhs.uk*
The safe drug list can still be accessed via their website at: *www.wmic.wales.nhs.uk/porphyria*

NEW NUMBER, SAME EXPERT SUPPORT ON THE SAFE USE OF MEDICINES IN PORPHYRIA!

BRITISH PORPHYRIA ASSOCIATION AND BETTERHELP ARE BRINGING YOU FREE THERAPY

BetterHelp removes the traditional barriers to therapy to give everyone access to affordable and convenient mental health care – 100% online and led by you.

The British Porphyria Association has partnered with BetterHelp and Teen Counselling to offer 6 months of free therapy to support you and give you a head start on your mental wellness journey. The offer is available for adults and young people aged 13-17 who live with porphyria or are a family member of someone living with porphyria. It is a completely confidential journey between you and BetterHelp.

- **It's convenient.** Wherever you are - via video, phone or text.
- **It's tailored.** BetterHelp matches you with a licensed therapist based on your needs, preferences, and location.

Get started today:

1. Scan the QR code or go to *betterhelp.com/voucher* and type in: bpa
2. You'll then be prompted to answer a few questions about your therapy needs and preferences. This helps BetterHelp match you to the right therapist.
3. Then you can activate your BetterHelp account via email.
4. Most new members are matched with their therapist in as little as 48 hours.

What to expect:
Starting therapy for the first time is something to be proud of, but it can also feel daunting. Here are a few reminders about the process:

- Your therapist isn't here to judge you. Be honest and be authentically you.
- It's ok to start therapy even if you feel like you don't have lots to talk about. Figuring out your goals is part of the process.
- Your therapist cares about who you are and how you're doing. The relationship between you is where a lot of the most important work happens.
- Switching therapists is totally ok. You can switch anytime and as many times as you need to find the right fit.

We are hopeful that the services offered by BetterHelp may work well for those affected by porphyria, due to the convenient and accessible style of therapy offered.

If you have already used the service and would be open to talking to us about your experience with BetterHelp – whether positive or negative – we would love to hear from you. All feedback would remain 100% confidential and would just be used to help us assess the effectiveness of the partnership.

Visit *www.porphyria.org.uk/betterhelp* to find out more. You can also access resources and information on managing your mental health with a rare condition at *www.rareminds.org/wellbeing-hub*.

LIVE VIDEO LIVE CHAT LIVE CALLS MESSAGING

THANK YOU TO CLINUVEL FOR A FURTHER DONATION OF CYACÊLLE!

The BPA are excited to announce that Clinuvel Pharmaceutics LTD have donated a further 'huge' batch of CYACÊLLE suncream for the BPA to share with those affected by any of the skin porphyrias, across the UK and Ireland. CYACÊLLE is a large particle (polychromatic photoprotection) sunscreen product that is described as "A broad spectrum SPF 50+ with photoprotection against UVB, UVA and HEV light, CYACÊLLE is water-resistant, dermatologist approved and formulated with 100% mineral UV filters." for more info see *clinuvel.com/photocosmetics/cyacelle/#section-next*

We are currently awaiting a shipment into the UK... If you'd like one sending out (once they arrive), please complete the order form: *www.peoplesfundraising.com/fundraising/bpa-cyacelle-2025*

We are asking for a minimum donation of £5.00 to cover the BPA's administration costs, including P&P, for sending the creams out to our members. If you would like more than one cream sending, please select the appropriate box(es) on the form. If you are facing hardship and need the creams to be sent out at no cost to you, please email us *helpline@porphyria.org.uk*

We'd love to be able to report back on the experience of this product for porphyria patients who experience phototoxic/photosensitive reactions. With this in mind, we hope that you will be happy to complete a survey - using the link/QR code that you will find on your letter within the box.

NEW VIDEOS RELEASED FOR GLOBAL PORPHYRIA DAY

WATCH, LEARN AND SHARE!

To mark Global Porphyria Day (GPD), we shared a brand-new collection of powerful and inspiring videos that highlight the strength of our community, raise awareness and celebrate the work being done – both here in the UK and around the world.

Two of these videos were recorded during our fantastic **Connect Alfresco** event last October. One video '*Empowering people with porphyria*' captures the atmosphere and impact of the day, offering a glimpse into the vital work the BPA is doing and the importance of bringing people together. The second video, '*We're not alone*' shares a deeply personal story of living with EPP, told through the eyes of Tiernan and Niamh, a family navigating life with this rare condition.

We partnered with the Global Porphyria Advocacy Coalition (GPAC) to share powerful stories:

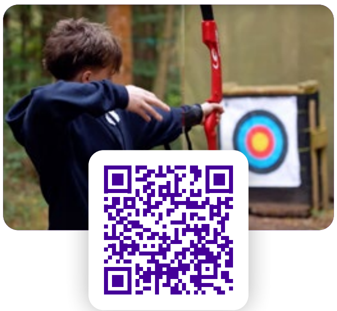
- **Science Slam –** A creative, animated explanation of porphyria, drawn and presented by Dr Jasmin Barman-Aksözen, scientist and EPP patient.
- **Global Porphyria Advocacy –** Inspiring voices from global leaders on their mission to empower others.
- **CEP Awareness –** The final #MyPorphyria video, spotlighting three amazing CEP patients..

Whether you're newly diagnosed, a family member, or a long-time advocate, there's something here to inform, inspire and connect.

We've made it easy to view and share – simply follow the YouTube links or scan the QR codes below.

Empowering people with porphyria

We're not alone: it's a porphyria family



Science Slam with Dr Jasmin Barman-Aksözen

Porphyria advocacy around the world

Check out: YouTube: @GlobalPorphyria

MAKE SHOPPING MATTER WITH EASYFUNDRAISING AND GIVE AS YOU LIVE

There are so many ways to get involved with fundraising for the BPA (see our full host of fundraising tips at *porphyria.org.uk/fundraising*), but one of the easiest and low-pressure options is simply to sign up with one (or both!) of the **two** online shopping platforms **Easyfundraising** and **Give As You Live.**

These websites let you support your favourite charity at no extra cost to you, by donating a percentage of whatever you spend on their platforms to the BPA. With over 7,000 retailers to choose from, whether it's your weekly groceries, fashion or gadgets, your everyday online purchases can help make a big difference. Every penny raised goes towards our work supporting families affected by porphyria. They both also offer a free **Donation Reminder plugin** you can add to your internet browser toolbar so you don't even have to remember to shop via their websites – after you download the plugin, just browse the internet as you would normally and they'll tell you when there are free donations available via a handy pop-up!

To start raising funds with every click, sign up to Easyfundraising at *www.easyfundraising.org.uk* **and Give As You Live Online at** *www.giveasyoulive.com***.**

Support us through easyfundraising

Give as you Live

MEMBER UPDATES FORM

We have just been made aware that our website Member Updates Form has not been working for some time. While we work on sorting this out, please send any address changes or new member requests to *helpline@porphyria.org.uk*. If you completed the form during March, April or May, we suggest that you re-send details to us, so that we can make sure we have your details on file.

Please tell us your full name, address, email, phone number, type of porphyria and confirm that you are happy for us to contact you with updates. If you have a preference for email or postal newsletters, please tell us that too. The default position will add you to our email distribution list rather than postal distribution list.

Thank you for your understanding and patience while we sort this issue.

RESEARCH UPDATES FOR EPP AND XLP

We're in an exciting chapter for those living with Erythropoietic Protoporphyria (EPP) and X-linked Protoporphyria (XLP). After years of limited treatment options, lots of research is taking place and this will hopefully lead to promising treatments in the future. Here's a reminder of the current field of research.

Scenesse® (Afamelanotide)

Currently, Scenesse® (afamelanotide) remains the only approved treatment available for EPP/XLP. It is manufactured by CLINUVEL. By creating a tan, it helps reduce the severity of phototoxic reactions, allowing more freedom and comfort in sunlight.

In the UK, access to Scenesse is currently limited to Scotland, where it's available through the Ultra-Orphan Medicines Pathway (until later in 2025). This means patients in Scotland can receive the treatment through the NHS while more data is collected on its impact. Once this data is reviewed, a decision will be made on whether Scenesse can be recommended as a routine treatment across Scotland.

Dersimelagon (MT-7117)

Developed by Mitsubishi Tanabe Pharma, dersimelagon is an oral medication designed to increase sunlight tolerance. It works by increasing eumelanin (the pigment that gives us a natural tan), creating a protective barrier in the skin against visible light.

Dersimelagon is currently in Phase 3 clinical trials around the world, including the UK, through a study called INSPIRE. This double-blind, placebo-controlled study includes 150 patients aged 12 to 75, some of whom are based here in the UK.

Several UK participants remain in the long-term extension phase of the first part of the Phase 3 trial, which began in 2020. This continued data collection will help evaluate how effective, safe, and tolerable the treatment is over time.

Bitopertin

Another promising candidate is bitopertin, an oral therapy being trialled by Disc Medicine. Unlike other treatments, bitopertin works by reducing protoporphyrin IX (PPIX) levels in the blood - the chemical that causes phototoxicity in EPP and XLP.

Early results are encouraging, showing up to a 40% reduction in PPIX and a noticeable increase in light tolerance. Plans are now underway for a Phase 3 trial to launch by mid-2025, with 150 patients to be enrolled across the US, Canada, Europe, and Australia. UK trial sites will be included.

PORT 77

Portal Therapeutics is a clinical stage biotechnology company based in California that is developing a novel investigational therapy for people living with EPP and XLP. Portal recently started its Phase 2A clinical trial, GATEWAY, which is currently enrolling adults with EPP* (NCT06971900). The following words from Portal Therapeutics explain more below:

"Portal has developed PORT-77, which takes a new approach to helping people with EPP and XLP. In EPP or XLP, a molecule that is a part of heme biosynthesis, called protoporphyrin IX (PPIX), builds up in the red blood cells. A protein in red blood cells (called ABCG2) acts like an open gate, moving this PPIX out of red blood cells and into the bloodstream. When PPIX travels to the skin from the bloodstream, light, especially sunlight, can cause painful reactions. PPIX movement and build-up in the liver and biliary tract can also sometimes create liver complications. PORT-77 is designed to "close" the ABCG2 gate. By blocking the ABCG2 protein, more PPIX stays inside the red blood cells, instead of moving into the bloodstream where it can cause subsequent problems in the skin and liver."

Looking ahead

We'll keep you updated as these treatments/potential therapies evolve. In the meantime, if you might want to take part in a clinical trial, please do make sure you are connected to a porphyria specialist. Contact us if you need help to find the closest centre to you.

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*If you are interested in learning more about the GATEWAY study, please contact the BPA on *helpline@porphyria.org.uk* or 0300 30 200 30.

SAVE THE DATE

We're delighted to invite you to the **Autumn Patient Event** — an afternoon of connection, learning, and fun.

Date: Saturday 8 November 2025

Location: Holiday Inn Express, Stratford, London, E15 2NE

Time: 13:30 – 18:00

The event will feature **updates and Q&A sessions** with porphyria specialists and patients, as well as a **short Annual General Meeting (AGM)**. It's a fantastic opportunity to gain insights into the latest developments and ask questions in a supportive environment.

We're also hosting a **lively escape game activity!** Whether you're a puzzle pro or a first timer, we're hoping for a fun and memorable experience for all ages.

After the structured sessions, there will be time to **chat informally with others living with porphyria,** share stories, and build connections over light refreshments. This is a great chance to meet others who understand your journey, in a relaxed and friendly setting.

Spaces are limited to 60 attendees, so please register ASAP to secure your spot: *https://buytickets.at/thebritishporphyriaassociation/1748560*

We're asking for **a nominal fee of £5 per person** to confirm bookings — but please don't let cost be a barrier. Fee waivers are available for you and your family if needed. We're committed to ensuring that our events are fully accessible to all within the porphyria community, and we have a small budget to help to cover travel costs for anyone in need.

If you would like to discuss any specific access, financial or other needs, please reach out to us *helpline@porphyria.org.uk* or call 0300 30 200 30.

We look forward to seeing you in Stratford in November!

THANK YOU

We're thrilled to celebrate our amazing community members who have gone above and beyond to make a difference recently through their incredible fundraising efforts. And it's certainly been a busy first half of the year, with Global Porphyria Day on 18 May and other individual fundraisers bringing in a combined total of over **£7,450** for the charity!

Every ticket, every mile, and every big birthday has helped support our mission, and we couldn't be more grateful. Here's a huge shout-out to some recent fundraisers.

First and foremost, thank you to **each and everyone of you** (and your family and friends!) who bought tickets for our Global Porphyria Day raffle in May! With your help, we managed to raise a whopping **£1902**, and every ticket helped shine a light on porphyria and bring hope to those affected. An extra special shoutout to volunteer **Kathryn Wilson** who worked tirelessly to source all the fantastic prizes, and to our members who drew on their networks to contribute goodies to the draw – like **Paul Fisher** whose connections at Tottenham Hotspur FC secured us an exclusive signed pennant from the players. We truly could not do it without you.

And congratulations to all the lucky winners – we hope you're enjoying your Nintendo Switch, iPad, spa day, and sun protective clothing!

Also in May, **Lynne Garbutt** celebrated a recent big birthday by taking the time to set up a JustGiving page which raised a total of **£320** for the cause – thank you for thinking of us on your special day, Lynne! Asking your friends and family to donate to the BPA in honour of your birthday, wedding, retirement party, or other important milestone is an easy, low-pressure way to raise funds, and you can visit our website to find out how to do it: *porphyria.org.uk/celebrations-and-special-occasions*.

Now to our superstar athletes, whose discipline and dedication never ceases to amaze us!

The partners and relatives of our members living with porphyria have continued to go the extra mile (or twenty-six point two!) to support their loved ones – in April, **Rob McAuley** and **Reece Boughton** ran the Brighton Marathon for Charlotte (Rob's sister and Reece's partner) who has EPP, and **Nick Lock** travelled all the way from the USA to complete the London Marathon in honour of his cousin in the UK, Will, who has EPP.

Then we had three official #TeamBPA members in the Edinburgh Marathon Festival at the end of May: **Cam Prokas**, who took on the marathon in support of his partner Robyn (see pictured their adorable pooch modelling the BPA running vest!), and newlyweds **Gráinne Kennedy** and **Nick Winton** who ran the half marathon and marathon, respectively, because Nick lives with EPP.

Together our incredible fundraisers racked up **£5,235** over **117.9** combined miles – the immense physical challenge of a marathon is no small feat so you should be very proud! Your endurance, dedication, and community spirit truly inspire us all. Every penny raised will go directly towards supporting our projects and services.

Thank you to all of our incredible fundraisers for their time, energy, and generosity. Because of you, we're able to make a difference in the lives of those we support. We're proud to have such passionate and dedicated people in our community.

If you've been inspired by the fundraisers in this article, visit *porphyria.org.uk/fundraising* **to find out how you can get involved.**

A huge "THANK YOU" also goes out to our industry supporters who engaged in Global Porphyria Day efforts by funds for us to purchase some of our amazing raffle prizes. Many also engaged in #PurpleForPorphyria events to raise awareness too!

If you have your own business, you could get involved in a number of ways too! You could become a regular donor to the BPA, donate skills/time, organise a local/national fundraiser or even sponsor the next batch of BPA running vests, or you might have other amazing ideas that we'd be keen to listen to!

CHANGES WITHIN THE BPA

The BPA would like to take the opportunity to reflect on a few changes from the last 6-18 months.

Firstly, we'd like to offer sincere thanks to John Chamberlayne who, after more than 20 years, stepped down from the BPA as the Chair of Trustees. We'd also like to welcome Dr Vicky McGuire into this role as she works with Liz and Sue to steer the BPA forward.

Richard Bennett officially stepped back as the BPA's Secretary, we'd like to thank him for his dedication to the BPA for many years.

Over the last year, some of our volunteers became Trustees and have taken on more responsibilities, including Victoria Harrold. We have also welcomed volunteers Kathryn Wilson, Pete Carter and Jenny Kirk who've already been contributing significantly with specific skills/expertise.

Georgia Newman has had a change of title to Fundraising & Communications Officer which better reflects her role and the contribution she has on a weekly basis to the BPA.

Join our Trustee Board – Make a real difference with the BPA!

Are you passionate about making a meaningful impact? Do you have professional skills and a few hours a month to spare? We are looking for dedicated volunteers to join our Trustee Board as we embark on an exciting new chapter of growth and development.

As a trustee, you'll play a vital role in shaping our future direction, supporting our mission, and ensuring our work continues to benefit those affected by porphyria. The time commitment is expected to be just 4–6 hours per month, and your contribution could be life-changing for the people we support.

What skills do you have? Could you donate some of your time and experience to help us grow?

We're keen to hear from individuals with all types of expertise, but we're particularly keen to hear from individuals with expertise in:

- Lived or family experience of porphyria
- Fundraising and development
- Legal/contractual advice
- UK benefits system
- Human resources
- Social media

We'd love to hear from you. To express your interest or find out more, please get in touch: *helpline@porphyria.org.uk*

Together, we can make a brighter future for those living with porphyria.

HELPLINE
0300 30 200 30

Would members please note that our helpline is only manned at specific times, due to work and family commitments.

When it is not manned an answer machine will be in operation.

Please leave your name, phone number, day and time of message and someone will call you back as soon as possible.

web: *www.porphyria.org.uk*
email: *helpline@porphyria.org.uk*
address: BPA, 136 Devonshire Rd
Durham City, DH1 2BL

The BPA Newsletter is published by the BPA twice yearly.

Chair: Dr Vicky McGuire
Treasurer: Claire Jarvis
Secretary: Victoria Harrold
Co-CEO: Liz Gill
Co-CEO: Sue Burrell
Fundraising & Communications Officer: Georgia Newman
Patrons: Prof George Elder, Prof Mike Badminton, Prof Felicity Stewart, Dr Penny Stein, Prof David Rees, Dr Bob Sarkany, Dr Vicky McGuire

PATIENT PASSPORT

If you're living with a rare condition, you probably know how tough it can be to explain your health needs – especially in emergencies or when meeting a new doctor. It can feel overwhelming trying to get people to understand something so complex and, well, rare.

That's why we're excited about the '**This is Me**' Rare Patient Passport. Created by Cambridge Rare Disease Network (CamRARE) alongside families and medical professionals, it's a powerful little tool that helps tell your story clearly and quickly. It gives essential, personalised info in a format that's easy for healthcare teams to understand—so you can focus less on explaining and more on getting the care you need.

The BPA are delighted to join the growing global network of patient groups partnering with CamRARE to bring the Patient Passport to the porphyria community!

Find out more and sign up for yours at *www.camraredisease.org/patient-passport*

....have worked with families & clinicians to create a fillable, personalised **health & care passport** to help communicate your key medical & care information in an emergency, away from home or when meeting new people.

THE BRITISH PORPHYRIA ASSOCIATION Registered Charity No. 1089609
MEMBERSHIP / DONATION FORM

MEMBER DETAILS

Please complete (*required field) and return to:
BPA Treasurer, 69 Cannell Road, Loddon, Norwich, Norfolk, NR14 6TP
or email it to *treasurer@porphyria.org.uk*

Title * Full name * ____________________

Address * ____________________

____

Postcode * ____________________

Landline Mobile ____________________

Email ____________________

Type of porphyria ____________________

Are you a (please tick):

☐ Patient ☐ Relative ☐ Medical professional ☐ Supporter/donor ☐ Other

What information are you happy to receive? Tick all boxes that apply:

☐ All BPA communications
☐ Patient events/information
☐ Newsletters
☐ Fundraising/awareness events

How would you like us to keep in touch with you? Tick all boxes that apply:

☐ Post ☐ Email ☐ Telephone ☐ Text message

DONATIONS

By making a donation to the British Porphyria Association you will know that you are making a vital contribution to our work. A suggested donation of £15 per year will help us raise awareness, man our helplines or support research.

I would like to make a donation of:

☐ £10 ☐ £15 ☐ £20 ☐ £25 ☐ £50 ☐ other £..............................

WAYS TO DONATE:

☐ Cheque: made payable to the British Porphyria Association.

☐ Online banking (one-off payment or regular standing order):
BPA bank account: Sort code: 20-43-63, Account no. 7099 6904
Please use your name and postcode as the payment reference.

☐ Credit card: via Just Giving (*www.justgiving.com/britishporphyriaassoc*).

☐ If you would prefer to complete a standing order form, please call us on 0300 30 200 30 and we will arrange to send one to you.

☐ I would like a receipt

DO YOU PAY UK TAX?

If you pay UK tax, the BPA can reclaim 25p of tax on every £1 you give. This does not cost you anything and does not affect your personal tax position. Simply sign and date the Gift Aid declaration.

GIFT AID DECLARATION

I want to Gift Aid my donation and any donations I make in the future or have made in the past 4 years to the British Porphyria Association (Registered Charity No. 1089609). I am a UK taxpayer and understand that if I pay less Income Tax and/or Capital Gains Tax than the amount of Gift Aid claimed on all my donations in that tax year it is my responsibility to pay any difference. (I will advise the BPA if my tax status, name or address changes.)

DATE SIGNATURE ____________________