

THE BRITISH PORPHYRIA ASSOCIATION NEWSLETTER

Registered Charity No. 1089609



CONNECT 2021: ONLINE... LIVE... TOGETHER

Our second virtual event took place on 9 October 2021. We were delighted to see all those who attended and very grateful to all who took part in the day. Whether presenters or attendees, your engagement and enthusiasm was fantastic.

The webinar started with an AGM, then provided an opportunity to meet porphyria specialists from across the UK with representatives from centres in Cardiff, Salford, Edinburgh and Dundee.

The next part focused on the new drug development process and the importance of research trials with Prof Jean-Charles Deybach and Dr Mark Farrar. Prof Deybach provided some very encouraging updates about research into

the porphyrias, while Dr Farrar explained how patients can get involved in research.

Finally, we heard from a counsellor (Susie Fearn) and a yoga teacher/wellbeing educator (Sarah Hodgson) about holistic approaches to managing life, pain and porphyria – as well as tips on how best to care for your body and mind.

In case you missed the event, or would like to view again, the recordings are now available on our YouTube channel: <https://www.youtube.com/channel/UCP4eYSSJl01Xc1LxZ3l9eGw/videos>

It was fantastic to meet so many of you in the interactive sessions in the afternoon. Thank you to all those who took part. Thanks especially

to our expert patients, Salman Fatmi and Daniel Powell, who took the time to start off our afternoon with accounts of their patient journey.

Finally, we'd like to offer a huge thank you to our sponsors, Alnylam Pharmaceuticals, Clinuvel, Mitsubishi Tanabe and Recordati Rare Diseases. Their continued assistance enables us to continue helping and supporting porphyria patients and their families.

Feedback

We're delighted that the day was a wonderful success. Survey respondents overwhelmingly reported that the event met their expectations, giving it an average of 4.9 star rating out of 5.

Looking forward to 2022, the Connect 21 attendees noted a big desire to meet face to face – we look forward to being able to do this with you all while also continuing to offer virtual elements through the year.

The feedback continued to highlight the importance of having formal scientific presentations about porphyria (the New Treatments session was rated as excellent by attendees) and that the programme should be balanced with patient testimonials (pivotal to the discussions) and interactive breakout opportunities where patients can share their own experiences. Thank you for completing the feedback, it really helps with the planning of our future events.

Save the date: Sunday 5 June 2022

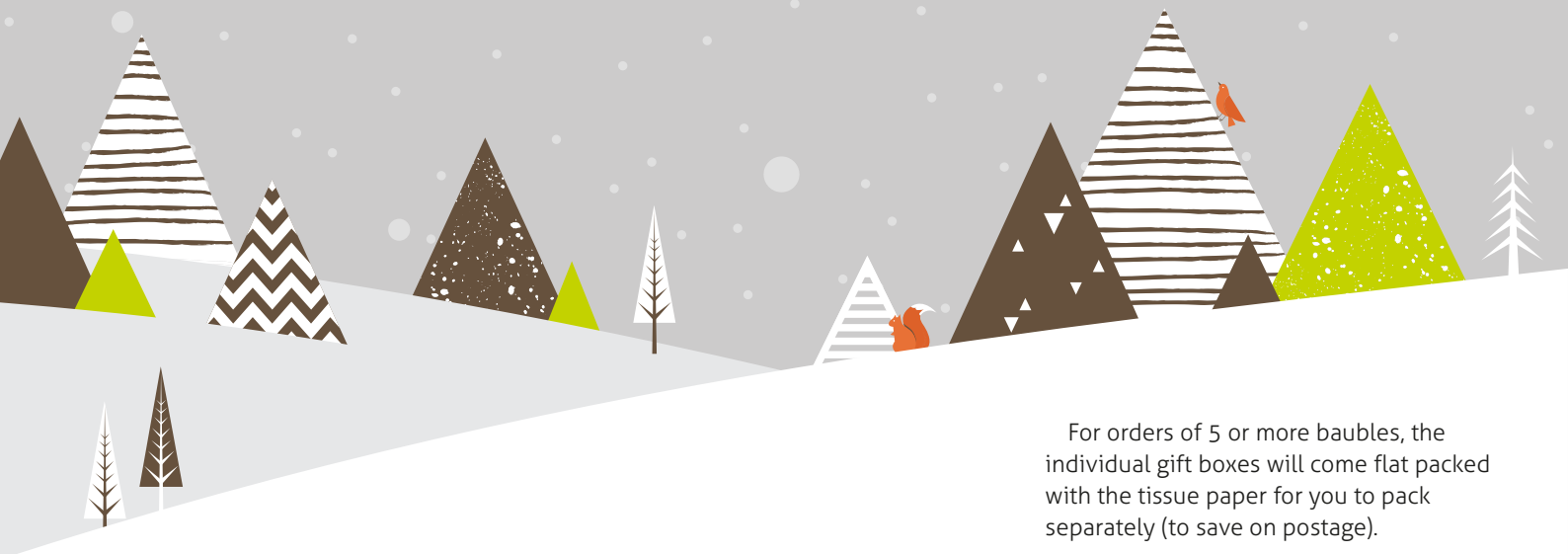
With the support of the team in Dundee, we are excited to be starting to plan a fantastic patient day on Sunday 5 June 2022. There will be opportunities to learn more about porphyria and meet specialists as well as other porphyria patients and their families.

As always, the event will be free to attend, but booking will be required. Keep a look out for more details as they are announced. We're very much looking forward to seeing you all again next year.



Photo credit: orlando alberghi / Shutterstock.com

Christmas Fundraisers



Christmas card fundraiser

This year, the BPA are selling packs of Christmas cards. 10 cards per pack with two designs in each (five cards of each design). The wording inside each card reads 'Wishing you a Merry Christmas and a Happy New Year'.

Three packs (30 cards) for £10.00 (plus £2.95 p&p). Choose from the following options:

- Pack 1: Robin/Hot Chocolate (3 packs = 30 cards)
- Pack 2: Fox/Snowman (3 packs = 30 cards)
- Pack 3: Santa/Bus (3 packs = 30 cards)
- Pack 4: Multipack (1 pack of each: Pack 1, 2 and 3 = total of 30 cards)

100% of all profits go to the BPA, so you can enjoy sharing these lovely festive cards with your friends and family, whilst also supporting the important work that the BPA do.

Place your order now: <http://porphyria.org.uk/christmas-fundraiser/>

When ordering, please make sure you indicate:

- Which three pack option you would like (Pack 1, 2 or 3, or 4).
- Your name, postal and email address in the information section, so that we can send you your order.
- You can also leave a contact telephone number.

Christmas bauble fundraiser

The BPA are also selling beautiful glass Christmas baubles. These baubles will make the most wonderful Christmas gifts for friends and family, secret Santa presents, teacher thank you gifts or even table decorations! They come in individual gift boxes.

100% of all profits go to the BPA, so you can enjoy these beautiful baubles whilst also supporting the BPA. Price: £6 per bauble (plus £3 p&p per order of up to 6 baubles)

Choose from:

- Christmas Snowflake Bauble £6
- Christmas Deer/Tree Bauble £6
- Christmas Robin Bauble £6

For orders of 5 or more baubles, the individual gift boxes will come flat packed with the tissue paper for you to pack separately (to save on postage).

Place your order now: <http://porphyria.org.uk/christmas-fundraiser/>

When ordering, please make sure you indicate:

- Which design you would like (and how many of each).
- Your name, postal and email address in the information section, so that we can send you your order.
- You can also leave a contact telephone number, in case we need to contact you about your order.

If you place an order (for cards or baubles) and haven't had an email from the BPA to confirm the order within 72 hours, please contact us on merch@porphyria.org.uk or 0300 30 200 30.

Please note: the last date for orders guaranteed for delivery in time for Christmas is Wednesday 8th December 2021.

We hope you like the BPA's Christmas fundraiser ideas! We'd also like to take this opportunity (we know it is a little early) to wish you and your loved ones a very Merry Christmas, from all at the BPA.

THANK YOU

We are delighted that we've had some amazingly dedicated fundraisers going the extra mile to support the BPA. THANK YOU to all of you, whether mentioned below or not. We really appreciate your efforts. We would also like to say a huge thank you to fundraisers' families and friends for supporting them through their fundraising challenges.

Active events

THANK YOU to all those choosing to take part in sporting events to raise awareness and funds. A massive WELL DONE and THANK YOU to our London Marathon runner, Luke Banks and two Virtual London Marathon runners, Alan Nolan and Vicky McGuire, who collectively brought in nearly £3,000 in October this year. Each runner made the challenge of 26.2 miles look an absolute breeze. Absolutely amazing – thank you so much for all your training, dedication and hard work.

In September this year, three runners took on the

13.1 mile Great North Run in its 40th year. Sarah Hodgson, Kimberley Gill and Adele Norris together raised more than £855 for the BPA – a fantastic amount, thank you. Meanwhile, Ben Meade who was unable to run the Great North Run due to injury donated a superbly generous £870 in lieu of running. A HUGE thank you to you all.

Another energetic challenge, Glow in the Park, was completed by Alicia and Kate Cawthorne, and Alicia's friends, raising a wonderful £310. Even more notable, Alicia managed to complete the 2k course while on crutches following a series of recent attacks. Thank you!

Meanwhile, Sarah and Jo completed the Isle of Man Parish Walk in June raising a fabulous £622. THANK YOU!

Other donations

Lots of people have been choosing to donate to the BPA in lieu of birthday presents this year, such as Hannah and Julie who used Facebook to bring in fantastic donations of more than £300. Thank you to

all those who are choosing this idea, donating anonymously or using Easyfundraising or AmazonSmile to donate too.

Other thanks go to a porphyria specialist who donated an honorarium to the BPA after speaking about porphyria, and a lady who donated Santander Shares and Dividends to the BPA. Thank you so much for these alternative ways of donating to support porphyria patients.

Finally, the BPA were successful in obtaining a grant of £1,000 from the Albert Hunt Trust, and sponsorship of £3,000 from Recordati Rare Disease. These funds will be used to help support a Wellbeing Project. We are also very grateful to our sponsors of the Connect 2021 event (Alnylam Pharmaceuticals, Clinuvel, Mitsubishi Tanabe Pharma Company and Recordati Rare Diseases). These funds not only help us to create and run an event for patients, they allow us to create valuable online resources that patients and medical professionals can access whenever needed.



VACANCY: VOLUNTEER TREASURER/TRUSTEE

We are looking for an enthusiastic and organised candidate to take on the role of Treasurer, following an extended handover period. It is likely to take 3-6 hours a week.

A competent treasurer or part-qualified book keeper or accountant would be ideal. Experience in the charity field is desirable but not essential. Our accounts are not complex, but we do have multiple income streams to consider. The successful candidate would need to maintain financial control

and ensure we comply with our legal responsibilities as a Registered Charity, which would be explained during the handover.

Find the full details at www.porphyria.org.uk/vacancies

Applications must be made via John Chamberlayne, the BPA Chairman chair@porphyria.org.uk. Please provide your CV, along with a covering letter stating why you wish to work for the organisation, how your skills would add value to the board and any other relevant information.



EPP ACTIVITY EVENT: WE NEED YOUR INPUT!

Supporting young patients is a priority for the BPA due to the impact EPP has on childhood and teenage years. The missed school trips, missed holidays and social experiences that young EPP patients suffer, often have a significant impact on self-confidence, building peer relationships and making life choices.

The BPA is constantly striving to find ways to enable EPP patients in understanding and taking as much control of their condition as possible. To assist with this, we are actively pursuing the idea of an activity day or residential camp to take place in Autumn 2022. Would you like to take part in an adventure break in a safe environment, organised by people who are fully aware of the problems of EPP?

Time is short, so to support the planning and decision making that will allow this event to take place, we urgently need to know how many people would be interested in taking part. Please contact events@porphyria.org.uk to tell us you're interested.



EXPERT PORPHYRIA CARE

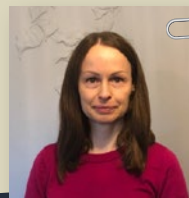
There are various expert porphyria centres across the UK. If you are not regularly seeing a porphyria specialist and would like help accessing care, please get in touch with the BPA. Salford are keen to develop their service further for the porphyrias affecting the skin, see the note from Dr Liezel Griffin regarding the centre in Salford.

I would like to introduce myself to those porphyria patients whom I haven't already met. I'm a Consultant Dermatologist at Salford Royal Hospital in Greater Manchester with expertise in porphyrias involving the skin (cutaneous porphyrias). Since the retirement of Professor Felicity Stewart earlier this year, I now run the porphyria clinic alongside my colleague, Dr Denise Darby, Consultant in Clinical Biochemistry & Metabolic Medicine, who has expertise in porphyrias affecting the liver (hepatic porphyrias). Dr Darby is involved in the National Acute Porphyria Service (NAPS), holding clinics twice each year with colleagues from the Cardiff centre. Salford also has one of the few porphyria reference laboratories in the UK.

In addition to the clinical service for porphyria patients, I perform clinical research and trials of new treatments for cutaneous porphyria patients, through my work with Professor Lesley Rhodes in the Photobiology Unit at Salford Royal Hospital. The Unit is the UK's National Coordinating Centre for a phase 3 clinical trial assessing a new treatment for patients with Erythropoietic Protoporphyrin (EPP). This trial is fully recruited, and we recently had the good news that the trial has been extended for a further 2 years for those patients already participating. It is hoped this trial will enable the new treatment to become licensed for EPP patients in the UK. My other clinical research will include examining the impact of cutaneous porphyria on patients and their families, and further evaluation of treatments.

I am very happy to see EPP patients from across Northern England, the Midlands, North Wales and beyond, at Salford Royal Hospital, as clinic patients and/or volunteers for clinical research. Referral letters from your family doctor or dermatologist can be sent to me at the Photobiology Unit, Dermatology Centre, Salford Royal Hospital, Stott Lane, Salford, M6 8HD or emailed to my secretary (Rachel.Tang2@nca.nhs.uk). Together we can better understand EPP and its management, and help improve the lives of patients and families living with the condition.

Dr Liezel Griffin
North-West England Porphyria Service



MAKE YOUR DONATIONS GO FURTHER WITH GIFT AID

Every donation you make to the BPA could be worth more at no extra cost to you by choosing to Gift Aid it. 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. Saying 'yes' to Gift Aid means that every £1 you give is worth an extra 25p for patients with porphyria.

Make sure you update your Gift Aid status with the BPA. Just complete and sign our Gift Aid form on <https://tinyurl.com/bu7drcsd>. Thank you.

RESEARCH AND PATIENT INVOLVEMENT

The BPA are regularly involved in supporting research opportunities for the porphyrias, by helping with the design of trials, and asking for volunteers to take part. Patient involvement in research and the design of research is hugely important. If the BPA can help support you to feel confident and valued in a research project, please do get in touch.

EPP research opportunity

The Photobiology Unit at Ninewells Hospital (NHS Tayside, Dundee, UK) routinely diagnoses light sensitivity, including but not limited to EPP. In addition to their clinical duties, they also perform research activities which aim to improve the diagnosis, treatment or management of light sensitivity.

They are currently at the early stages of designing an app that would assist people with the management of EPP. It is hoped that the app would be able to help track daylight exposure and warn of potentially high light levels. The first step in this project is to collect responses from a survey to help decide the scope and aims of the project, and find out what matters most to those who are likely to use the app.

If you would like to be involved in the survey from Ninewells Hospital, you can access it here: <https://forms.office.com/r/Q99dkXM1L4>



ACCURATE INFORMATION STATEMENT

The British Porphyria Association are in full support and endorse the 'Accurate Information Statement', produced by the Global Porphyria Advocacy Coalition (GPAC) and with the support of patient advocacy organisations from around the world (including the BPA). The statement has approval from international clinical and research networks, including the British and Irish Porphyria Network (BIPNET), the European Porphyria Network (Epnet), the American Porphyrias Expert Collaborative (APEX) and Porphyria South Africa.

The statement has been produced as the global porphyria community has become aware of potentially dangerous information that is being circulated across social media platforms and on various websites regarding the diagnosis, management, and treatment of the porphyrias. Unfortunately, some of the information is inaccurate, misquoted or taken out of context. Such information could be medically dangerous if taken as advice or guidance without input from a porphyria expert or qualified healthcare professional.

We wanted to take the opportunity to reiterate that if you have concerns about your porphyria management, please connect with a recognised porphyria specialist/expert centre or get in touch with the BPA.

A full copy of the statement can also be found on our website: <http://porphyria.org.uk/accurate-information-statement/>

GIVLAARI[®] RECOMMENDED FOR USE ON NHS

For the last 18 months, GIVLAARI (Givosiran) has been going through the NICE (National Institute for Care and Excellence) Highly Specialised Technologies (HST) process to try to obtain approval for use on the NHS in England. Porphyria expert clinicians and BPA members were involved in this process.

Initially the medication was 'not recommended', but we are delighted by the recent announcement (21 October 2021) that NICE has issued draft guidance which recommends the use of GIVLAARI on the NHS in England for the treatment of severe recurrent attacks of acute porphyria (www.nice.org.uk).

What kind of medication is GIVLAARI?

You may have seen articles about 'gene silencing' in the media and on news channels. Gene silencing is the name given to a group of treatments that modify the effect of genes on certain cells to effectively block the protein production that causes problems. Gene silencing does not alter the DNA sequence. GIVLAARI, Alnylam's siRNA (small interfering RNA) medication is a type of gene silencing treatment.

Who will be able to be treated with GIVLAARI?

This treatment will be applicable for a small set of acute porphyria patients who experience recurrent attacks (those experiencing more than 4 attacks in a 12-month period). There will be strict criteria on adherence to the protocols for treating and it will, initially at least, have to be given by an expert centre due to the risk of anaphylaxis, and this travel and commitment may be difficult for some patients. All decisions on who will be prescribed GIVLAARI will be made via a multi-disciplinary team that will take these considerations and much more into account.

When will it become available to patients?

Following the announcement, there will be a month-long appeal period. After that, as long as there are no appeals, the NHS in England will have three months/90 days to be able to organise getting the treatment to patients. So, by late February, eligible patients will hopefully be able to have access to the treatment. In the meantime, please direct any questions you may have about your porphyria care and/or whether this treatment may be appropriate for you to your porphyria specialist.

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.porphyrria.org.uk
email: helpline@porphyrria.org.uk
address: BPA, 136 Devonshire Rd
Durham City, DH1 2BL

The BPA Newsletter is published by the BPA twice yearly.

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Strategic Lead of Engagement and Advocacy: Sue Burrell

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Viewpoints and opinions contained in this newsletter are reproduced in good faith and do not necessarily reflect the judgement of the British Porphyria Association or its patrons.

INTERNATIONAL LEARNING

Over the last few weeks, leaders from the international rare disease community have been attending the virtual Global Rare Disease Patient Advocacy Collaborative Summit (hosted and funded by Alnylam Pharmaceuticals) with some amazing and interactive sessions from international experts. The sessions have been both educational and inspiring.

Sue Burrell represented the BPA and the Global Porphyria Advocacy Coalition (GPAC) alongside Kika Shabot from the Mexican Society for Porphyria (SMP), Kim Hollander from the American Oxalosis and Hyperoxaluria Foundation (OHF) and Jaime Christmas from the New Zealand Amyloidosis Patients Association (NZAPA). It was a pleasure talking and hearing about these organisations and the amazing work that is happening across the international rare disease community.



THE BRITISH PORPHYRIA ASSOCIATION Registered Charity No. 1089609

MEMBERSHIP / DONATION FORM



MEMBER DETAILS

Please complete (*required field) and return to:
BPA Treasurer, Rothlea House, 7-8 Quarry Lane, Butterknowle, Bishop Auckland, DL13 5LL or email it to treasurer@porphyrria.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 Newsletters
 Patient events/information 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/britishporphyrriaassoc).
 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 am a UK taxpayer and would like the British Porphyria Association (Registered Charity No.1089609) to treat all donations that I have made in the last six years and all future donations that I make from the date of this declaration as Gift Aid donations. I understand I must pay an amount of income tax and/or capital gains tax equal to the tax reclaimed on my donations. (I will advise the BPA if my tax status, name or address changes.)

DATE

SIGNATURE