

# THE BRITISH PORPHYRIA ASSOCIATION NEWSLETTER

Registered Charity No. 1089609



## SCENESSE® TO BECOME AVAILABLE TO SCOTTISH EPP PATIENTS WHILE MORE DATA IS GATHERED

The Scottish Medicines Consortium (the body that assesses clinical- and cost-effectiveness for all new medicines for NHS Scotland) released exciting information in February 2021 about SCENESSE® for patients with erythropoietic protoporphyria (EPP). [www.scottishmedicines.org.uk/medicines-advice/afamelanotide-scenesse-full-125117](http://www.scottishmedicines.org.uk/medicines-advice/afamelanotide-scenesse-full-125117)

Despite being designated as 'not recommended', the new Ultra-Orphan Medicines Pathway allows this medication to be made available to patients through

the NHS in Scotland for up to three years while further data is collected on the medication. It will then be reassessed and a further decision will be made on whether it can be recommended as a routine treatment in Scotland.

NHS Scotland, the Scottish porphyria doctors and Clinuvel (the manufacturer of SCENESSE) are currently agreeing what data needs to be collected and how. They are also finalising how and where it will become available, as well as the logistics of getting the medication to patients

– a process which may be hindered by Covid-19 restrictions. EPP patients in Scotland wanting to discuss this treatment must be referred to their local dermatology department, who can refer them to the Scottish Cutaneous Porphyria Service (SCPS) for further discussion.

This is a really exciting development for patients which we are following closely. The availability of SCENESSE in Scotland could have an immensely positive impact for EPP patients. We are very keen to see how this develops in the future.

Dr Deepak Ravindran  
LEADING NHS PAIN CONSULTANT



### The Pain Free Mindset

7 steps to taking control and overcoming chronic pain

Some of you may remember hearing from Dr Deepak Ravindran at prior BPA events. His presentations have been very well received by our members. Videos of his talks from the Festival 2019 and the Connect 2020 event are available on our YouTube channel: <https://www.youtube.com/channel/UCP4eYSS1l01Xc1LxZ3l9eGw/videos>

Dr Ravindran is a pain consultant working within the NHS. Over many years, he has honed the themes of these presentations into a book which has recently been released: 'The Pain Free Mindset'. This book focuses on helping patients to 'take back control' by helping them to understand:

1. What happens to your brain and body when you experience pain
2. How you can change the way you perceive and respond to pain
3. How to find the best pain management plan for you and your lifestyle

Available now from book stockists.

Awareness is a hugely important area of work for the BPA. It helps to create conversation and interest around what porphyria is and how people are affected by it. Ultimately, awareness can help generate real change and speed with diagnosis, treatment and condition management. It also helps with understanding for those affected by porphyria or for those supporting them.

**Rare Disease Day – 28 February 2021**

As part of the BPA's commitment to raising awareness of porphyria, Liz Gill (BPA) took part in a webinar with a panel of leaders from rare disease organisations. The audience was an international group of over 350 Alnylam Pharmaceuticals' staff and health professionals.

**Porphyria Awareness Week (PAW) – 10-17 April 2021**

The global Porphyria Awareness Campaign for 2021, #LetsTalkPorphyria, was a great success! The BPA had a large programme of activities that people could get engaged with, including 'purple' themed food, pyjama and wear purple days, alongside a whole series of interactive Zoom sessions with the aim to get people talking!

Thank you to everyone who got involved in the interactive Zoom sessions – it was heartening to hear your



**I SUPPORT  
 RARE DISEASE DAY  
 28 FEBRUARY 2021  
 #RAREDISEASEDAY RAREDISEASEDAY.ORG**



stories and we are so grateful for the openness and respectful manner that you all came to the sessions with. All of the sessions ran over – demonstrating just how interactive the conversations were, the advice that you all offered each other was amazing and inspiring too. THANK YOU AGAIN to all involved!

We made a note of all those involved in the week's activities, then entered all names into a competition to win one of four prizes. We utilised the 'wheels of names' platform to select winners at random and are delighted to announce the following winners for their involvement in PAW.

Additionally, we shared various 'conversations' of porphyria, including one with Alicia (AIP patient) and Victoria (EPP patient); one

with Liz and Sue from the BPA (AIP patients) and another one with Antony (EPP patient) and Ian (AIP husband). Antony and Ian talked openly about porphyria whilst cycling on an indoor Zwift/Zoom session – you can view these via our website now: <http://porphyria.org.uk/porphyria-awareness-week-2021/>. Thank you to all who took part in generating conversations and awareness.

The BPA, as part of the Global Porphyria Advocacy Coalition (GPAC), were also involved in an international awareness video campaign. Why not check out the BPA's video, along with our international friends' videos, on GPAC's website: [www.gpac-porphyrria.org/porphyria-awareness-week](http://www.gpac-porphyrria.org/porphyria-awareness-week).

Sophie Wyllie from the Eastern Daily Press

interviewed Sue Burrell from the BPA during Porphyria Awareness Week to help generate local awareness of the porphyrias and the work the BPA do. The article, entitled 'Don't lose hope - Mother uses experience of illness to spread support' was in the printed EDP on Saturday 24 April and can also be viewed online: [www.edp24.co.uk/news/sue-burrell-helping-others-through-british-porphyrria-association-7897740](http://www.edp24.co.uk/news/sue-burrell-helping-others-through-british-porphyrria-association-7897740)

**SAVE THE DATE: Porphyria Awareness Week 2022: 3-10 April 2022**

If you have been inspired by any of these conversations/pieces, why not start your own awareness campaign. More details of how you can get involved in 2022 will be circulated when available. But, in the meantime, if we can help you with any local awareness campaigns, or you would like further information or guidance, please just get in touch.

<b>1st Prize</b>	Porphyria Awareness Hoodie	Karen (who partook in various Zoom activities)
<b>2nd Prize</b>	Porphyria Awareness Snood	Isaac (various Purple activities & kids Zoom)
<b>3rd Prize</b>	Porphyria Awareness Facemask	Donna (Wear Purple Day)
<b>4th Prize</b>	Dr Ravindran – Pain Free Mindset Book	Graham (EPP interactive Zoom)

# Porphyria AWARENESS

[www.porphyrria.org.uk](http://www.porphyrria.org.uk)



## Porphyria Awareness Merchandise

We have some great porphyria awareness merchandise available, including facemasks (delivery in 7 days), suprafleece snoods, and hoodies for adults and kids (delivery in around 21 days). The items are great quality and provide a wonderful opportunity to get people talking about porphyria.

Keep an eye on the website for new porphyria awareness products: [www.porphyrria.org.uk/bpa-merchandise/](http://www.porphyrria.org.uk/bpa-merchandise/) All profits go to the BPA. Why not help to raise awareness of porphyria and get your merchandise now:

- Facemasks (one size): [www.virginmoneygiving.com/fund/porphyria-awareness-facemasks](http://www.virginmoneygiving.com/fund/porphyria-awareness-facemasks)
- Hoodies (sizes: adults and kids): [www.virginmoneygiving.com/fund/porphyria-awareness.hoodies](http://www.virginmoneygiving.com/fund/porphyria-awareness.hoodies)
- Suprafleece snoods (one size): [www.virginmoneygiving.com/fund/porphyria-awareness.snoods](http://www.virginmoneygiving.com/fund/porphyria-awareness.snoods)

Large quantity orders can also be made directly from the BPA. Or if online payment/ordering isn't possible, please contact [merchandise@porphyrria.org.uk](mailto:merchandise@porphyrria.org.uk) or 0300 30 200 30.

## A NEW Podcast Series

Alnylam Pharmaceuticals have been working on a new project, the 'Voices of AHP Podcast Series' to help raise awareness of the acute hepatic porphyrias (AHP). Featuring Alicia and Sue from the BPA, the first episode focuses on 'Initial symptoms and the journey to diagnosis'. Alicia talks openly about her experience of living with acute intermittent porphyria and her passion for raising awareness and meeting others with porphyria. The podcast is just 15 minutes long – why not have a listen: <https://www.livingwithporphyria.eu/voices-of-ahp#voices-of-ahp-podcast-series>



## BPA CHANGES

As Natassja Chadwick has now taken over the role of Treasurer, we wanted to provide a significant note of thanks to Anne Newton for the countless years managing the charity's finances. Although stepping back as Treasurer, Anne will remain a Trustee and will stay involved in the BPA. We really hope this gives her a little more time to spend with her grandchildren and family.

John Chamberlayne, the BPA's long-standing Chairman of the BPA also plans, in due course, to step back a little from some of his duties with the charity. John is a very well-recognised figure within the UK and international porphyria community and his continued service and dedication to the BPA is gratefully received. We're very pleased to report that despite stepping back as Chairman of the charity, John will remain a Trustee in his official capacity as the Chair of the Board of Trustees.

Alongside this, Liz Gill will move from the role of Vice Chairman into the role of Strategic Lead of Operations and Advocacy, while Sue Burrell will become the Strategic Lead of Engagement and Advocacy – alongside the rest of the dedicated committee, they will continue their day-to-day work for the BPA, reporting to the Board of Trustees.

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



## SAVE THE DATE

SATURDAY 9 OCTOBER 2021

We're delighted to provide a few details about our Connect 2021 online event. The event will follow a similar structure to the very well received Connect 2020. We're planning an engaging and interactive virtual event with an expert panel of porphyria doctors, patients and key speakers to really bring the event to life. We'll communicate more information once available, but please add the date to your diary!

We look forward to connecting with you all in October!



## INTERNATIONAL CONGRESS ON PORPHYRINS AND PORPHYRIA (ICPP)

POSTPONED TO 2022

Due to the ongoing Covid-19 pandemic, the international medical porphyria conference, which is normally held every two years, will be postponed a year. It has been confirmed that the location of Sofia, Bulgaria will remain the same with a new date now set for 4-7 September 2022. It will be preceded by an international porphyria patient day. The BPA plan to attend and will share further details in due course.



## 2021 WEBINAR SERIES: THE PORPHYRIAS

Epnet – the European Porphyria Network – have been running a series of webinars. These are presented by porphyria experts and aimed at medical professionals and laboratory specialists who are not experts in porphyria. The webinars are around an hour long and are available on Epnet's website. Even though they are not targeted specifically at patients you may enjoy watching them, or sharing with medical professionals who care for you. To view the webinars, or to sign up for the final session, please see: <https://porphyria.eu/en/content/2021-webinar-series-porphyrrias>.

## CONGRATULATIONS

Professor Felicity Stewart has been a senior porphyria specialist at Salford, Manchester. Many of you will know her, from being her patient, or from BPA meetings where she has kindly given up much time to speak at our many events over the years. She has recently retired from medical practice. The BPA sent her a "best wishes" card and wish her the very best for her retirement. We

are very pleased to hear that she hopes to continue her links with the BPA in the future as a Patron of the BPA.

Dr Denise Darby and Dr Leizel Griffin will continue to provide care for all of the porphyria patients at Salford Royal Hospital and we look forward to inviting them to meet you all at future BPA events.



## FUNDRAISING RUNS IN 2021

Could you join Team BPA for the Great North Run 2021? The 40th anniversary year is set to be an amazing event and places are in high demand due to no public ballot this year. Get in touch to reserve your place.

Would you like to run the Virtual London Marathon on Sunday 3 October 2021? Could you run, walk or hike 26.2 miles within the designated 24 hours from 00:00 to 23:59 on Sunday 3 October, from anywhere in the world... All whilst fundraising for the BPA?

If you're interested in any of these places, please contact [helpline@porphyria.org.uk](mailto:helpline@porphyria.org.uk) or 0300 30 200 30 – places will go on a first come, first served, basis.

# GIVLAARI® (GIVOSIRAN) UPDATE

After a long time in trial phases and after a delay due to Covid-19, GIVLAARI® is soon due to be assessed in England by NICE (the National Institute for Health and Care Excellence).

GIVLAARI® received European Medicines Agency (EMA) marketing authorisation in January 2020. But this was only the first regulatory step in getting this medication to patients. Before it becomes available on the NHS in the UK, it has to be assessed by NICE in England and the relevant regulatory bodies in Scotland, Wales and Northern Ireland (although Wales and NI generally follow NICE's lead, Scotland has its own process).

GIVLAARI is being assessed by NICE's Highly Specialised Technology (HST) process which considers whether a drug or technology (for a very rare condition) is likely to benefit patients and whether it is value for money, i.e. cost effective. Once NICE issues its guidance, the NHS must find the money to make a drug or treatment available.

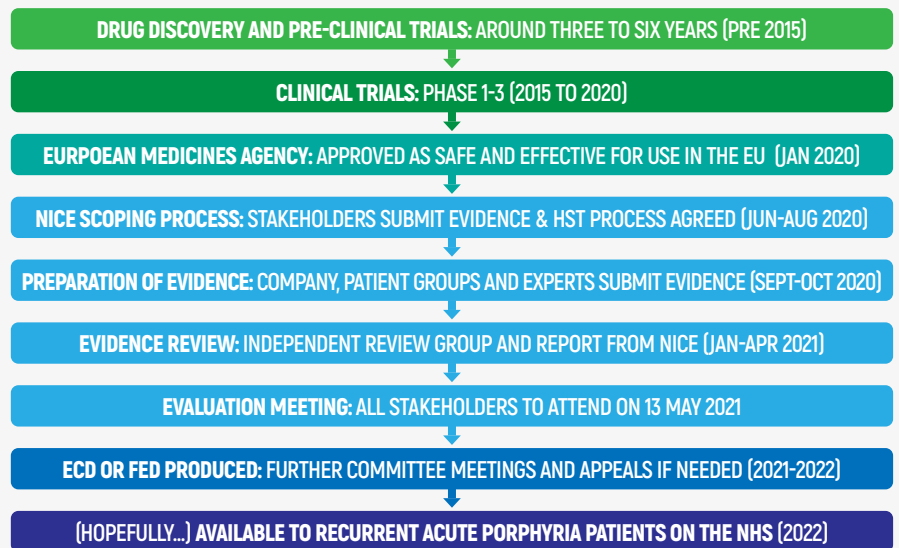
The NICE process has already been underway for some time, where UK porphyria specialists, the BPA, and acute porphyria patients have been involved in various stages of submitting evidence for the evaluation. We were able to nominate two patient experts to

attend the (virtual) evaluation meeting on Thursday 13 May 2021. After this, an Evaluation Consultation Document (ECD) or Final Evaluation Determination (FED) will be produced, depending on whether there are still uncertainties after the assessment. If successful, a FED will recommend how the medication should be used on the NHS in England. If there are no appeals, or an appeal is not upheld,

the final recommendations would be then issued as NICE guidance, after which NHS would be able to provide GIVLAARI® via expert centres around England with the remaining nations hopefully following soon after.

The process is lengthy and time-consuming, but we will keep you up-to-date on developments as soon as we know more.

## Getting GIVLAARI® from test tube to the patient



# Thank you

FOR YOUR SUPPORT

**THANK YOU** to Darren Oliver for cycling over 100 miles in April in his Dazza Proxy Pedal using his wife's bike, raising over £180 for the BPA – great loan Kari!

Great work to Caitlin for nominating the BPA to receive a donation. Staff at TSG Interactive (Isle of Man) voted for the BPA to receive £500 – **THANK YOU** Caitlin and all involved!

Natassja Chadwick and all your friends and family managed some amazing fundraising efforts in 2020, despite Covid-19 restrictions. **WELL DONE** and **THANK YOU** for raising around £2,000 from various fundraising activities, including Zumba classes, discos, dress-down days,

a car boot sale, a Christmas collection from the Ova Church, an interview with a marketing company, Christmas bauble sales and various donations. **WELL DONE** to all involved!

A **HUGE THANK YOU** to all who have purchased awareness merchandise, raising more than £250, so far. We'd like to give a special **THANK YOU** to Alnylam Pharmaceuticals for supporting **Porphyria Awareness Week** by supplying all of their UK/EU staff with our facemasks – great awareness and fundraising in one go! **THANK YOU** to Kate and Alicia for your bake sales and sales of Scenzztastic Melts during PAW.

The BPA's Christmas Bauble Fundraiser was a great success and helped raise over £400 – **THANK YOU** to all who supported this campaign.

PayPal Giving Fund generated around £500 in the last few months. **THANK YOU** and a belated **HAPPY BIRTHDAY** to Catherine, Reese, Samantha, Chris, Carys and Gillian who asked people to donate

to the BPA using Facebook Birthday Fundraising. **THANK YOU** also to all your supporters who donated!

**THANK YOU** to those who've set the BPA as your favourite charity through Amazon Smile, raising around £70 in the last few months. Don't forget you can help raise funds for the BPA through [www.easyfundraising.org.uk](http://www.easyfundraising.org.uk) and <https://smile.amazon.co.uk> – with absolutely no cost to you!

The BPA would like to give a belated **THANKS** to Busy Bees Nursery in Bishop Stortford for sending through a donation of over £390 from fundraising/donations from back in 2016.

If we haven't named you individually here, it isn't because we don't care, we simply have limited space. Please be assured that all of your efforts and generous contributions, including regular donations, Just Giving gifts and all the anonymous donations, no matter how small or large, are massively valued and enable us to continue doing the work that we do. **THANK YOU TO YOU ALL.**

# 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](http://www.porphyria.org.uk)  
email: [helpline@porphyria.org.uk](mailto:helpline@porphyria.org.uk)  
address: BPA, 136 Devonshire Rd  
Durham City, DH1 2BL

The BPA Newsletter is published by the BPA twice yearly.

**Chairman:** John Chamberlayne

**Treasurer:** Natassja Chadwick

**Secretary:** Richard Bennett

**Strategic Lead of Operations and Advocacy:** Liz Gill

**Strategic Lead of Engagement and Advocacy:** Sue Burrell

**Patrons:** Prof George Elder, Dr Mike Badminton, Prof Felicity Stewart, Dr Penny Stein, Prof David Rees, Dr Bob Sarkany

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.

## Are you a UK lawyer or solicitor?

We have an amazing opportunity for someone with legal expertise to utilise their skills and experiences to help out the BPA on an ad-hoc and infrequent basis. As we have grown and developed as a charity, we have a couple of instances a year where we'd like to seek the advice of a legal professional.

In particular, we'd like someone to be able to review any contracts and be an initial point of contact for general legal queries. We don't expect this to be anything onerous and all volunteers have complete flexibility to help only when they feel able to do so.

Please contact [liz.gill@porphyria.org.uk](mailto:liz.gill@porphyria.org.uk) or 0300 30 200 30 if you (or someone you know) would be interested in joining our friendly and energetic team by providing basic legal support to the BPA.



## 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@porphyria.org.uk](mailto: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  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/britishporphyriaassoc](http://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 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