Issue 38 / **May 2019**

THE
BRITISH PORPHYRIA ASSOCIATION
NEWSLETTER

Registered Charity No. 1089609

IRISH PATIENT DAY: SUNDAY 23 JUNE 2019

We're delighted to be holding our very first patient day in Dublin, Ireland on 23 June 2019. The conference will take place at the Ashling Hotel, Parkgate Street, Dublin 8, Ireland between 12.00 and 17.00. The venue is just a 20-minute walk or a 5-minute tram ride from Dublin's city centre. Heuston Intercity Railway Station is also just a 2-minute walk away.

The meeting is being organised with the support of Dr Crowley, Ms Brazil and various other porphyria doctors who have volunteered to help from the UK and Ireland, including Dr Bob Sarkany and Dr Mike Badminton. We'll have patient talks, updates on research and current treatments for the acute and skin porphyrias, as well as plenty of opportunities to speak with other porphyria patients and their families.

Reserve your places:
The event is free to attend, but registration is essential as spaces are limited. If you would like to join us, please forward the names of anyone wishing to come to sue.burrell@porphyria.org.uk or call 0300 30 200 30.

Travel bursary:
The BPA have funds to help support our members to attend meetings. If you would like to apply for funding, please get in touch (funding available up to: £50.00 for travel and £50.00 for hotels – Euro equivalent also available).

20th Anniversary Festival

12 OCTOBER 2019: 09.30 – 16.30 / 17.00 – 19.00
SCIENCE AND INDUSTRY MUSEUM, MANCHESTER, M3 4FP

To celebrate 20 YEARS since the formation of the British Porphyria Association, we are holding an exciting and completely new type of event. We are holding the first ever PORPHYRIA FESTIVAL designed especially for you!

With three main stages hosting a variety of sessions and an exceptional line-up of speakers and inspirational people, you can design your personal agenda for the day!

Whether that's understanding strategies to help you gain control of your condition, listening to a wealth of personal stories and experiences or focusing on connecting with others, our 20th anniversary event is designed with you at the heart.

Street food, snacks and refreshments will be available during the day. Early evening drinks and music will provide a relaxed atmosphere for you to continue making connections.

Visit www.porphyria.org.uk to find out how to get your FREE tickets.

SET LIST AND STAGES

We've created a sample set list so you can visualise how the programme will run on the day. The slots and speakers are currently being finalised, but the concept will not change. The sessions shown provide an idea of how the day will evolve. The final set list will be released a couple of weeks before the event, so you can design your own personalised experience.

We have three main stages to choose from:

UNDERSTAND STAGE (blue):

This stage will focus on the science of porphyria, management of symptoms and latest updates in the field of research. These sessions provide a deeper understanding of porphyria or outline new advances in science and treatments. Suitable for patients and medical professionals.

CONNECT STAGE (red):

This stage is patient centred and will focus on patients and their personal stories, interviews and interactive discussions. Suitable for all, this stage will help you to realise that you're not alone and aims to help you create vital connections with others. Discover inspirational people and feel empowered.

TAKE CONTROL STAGE (yellow):

This stage will focus on mental health and well-being, as well as ways to help you or your family take control and get the best out of life. Whether that's finding new activities, looking at alternative therapies or learning how to relax, this is for you.
We've also got a RELAX zone and a CHILDCARE room, so that you can get involved without being distracted.
Visit www.porphyria.org.uk to find out how to get your FREE tickets.

	UNDERSTAND STAGE	CONNECT STAGE	TAKE CONTROL STAGE (1)	TAKE CONTROL STAGE (2)	RELAX ZONE	CRECHE
Welcome & Coffee (09.15 - 09.45)						
Session 1	Introduction and AGM					
	Key note: Porphyria - History & Future					
Session 2	Interactive acute session	Patient perspective	Get active - EPP	TBC		
	Acute services Scotland	Husband's perspective - AIP	Challenges at school - EPP	TBC		
Lunch						
Session 3	PCT focus session	Pain session	Kids yoga	Photoprotection		
	Engaging in research		Martial arts	Light therapy - patient perspective		
Session 4	Interactive EPP session	Fertility/pregnancy in acute porphyria	Mindfulness	Workshop: What patients want from the NAPS service		
	Scenesse update	Family story – acute porphyria	Complementary therapies	Pathophysiology of acute porphyrias		
Session 5	Givosiran update	Denial cycle in acute porphyria	Patient perspective TBC	EPP interview – being involved in a book production		
	Key international developments	Psychology / mental health session	Well-being session TBC	Mental well-being and growing up with EPP		
Visit the Museum						
Get social	Early evening drinks and entertainment (17.00 - 19.00)					

NEARBY IN MANCHESTER

Why not make a weekend of it? Manchester is a vibrant and diverse city which is bursting with character and unique places to stay and things to do. Whatever your interest ... shops, sports, heritage, arts, culture, music, bars or restaurants you'll be able to find it here.

- **Shops:** High street shops and department stores on Manchester's Market Street, and in the Arndale Centre and Trafford Centre offer unrivalled shopping options. For something more special, why not try the independent or designer shops in King Street and Spinningfields boutique district.
- **Alternative:** if creativity is more your thing, look no further than Manchester's Northern Quarter. Home to vintage stores, record shops, cafes, bars and restaurants.
- **Family:** for a general family fun day, why not try Play Factore (indoor family entertainment arena), Chill Factore for snow fun, LEGOLAND Discovery Centre or Sea Life Manchester.
    - **Older children?** Try Escape Reality Manchester, ImmotionVR, or the Crystal Maze live experience.
    - **Young children?** Try Play Factore or the CBBC tour at Manchester's MediaCityUK.
- **Salford Quays:** at this close-by unique waterfront destination you can find entertainment, leisure and culture, as well as MediaCityUK – the regional HQ for the BBC and home to ITV and the Coronation Street set.
- **Heritage, arts and culture:** Roman fort Mamucium is a stone's throw away from the Science and Industry Museum, and The Lowry, the Imperial War Museum North, Tatton Park, Heaton Park, The John Rylands Library and Manchester Cathedral are just a few examples of the spectacular places to visit when in the region.

CONNECT

As always, there'll be plenty of opportunities to connect with other people with porphyria. Patients of all ages suffer from denial, anxiety and isolation due to their porphyria, but especially:

- Young adults with porphyria: who face a unique set of challenges and anxieties in the worlds of study, work, socialising and travel.
- Children with porphyria and their families: who face problems with school, friends and family life.

Visit www.porphyria.org.uk to find out how to get your FREE tickets. Questions: please contact festival@porphyria.org.uk or 0300 30 200 30.

Spread the word: help to make this the best porphyria event ever!

Follow our Facebook, Twitter and Instagram accounts to keep up to date with all the latest news and information about our exciting event!

Filming/photography will take place throughout the day. This is so that we can share presentations with those unable to attend and so that we can continue to improve education and awareness of the porphyrias, even after the event. We'll also have a video booth, so that you can share your stories and experiences with others.

Apply for help with costs of travel and accommodation

A long-term health condition can severely affect your physical ability or financial means to travel. But, as we want this to be a fully inclusive event we've managed to secure some funding to help people come to the festival. If you'd like to know more, please contact festival@porphyria.org.uk.

ABOUT THE VENUE

Entry to the Science and Industry Museum is free and is open to visitors 10.00 to 17.00.

We've allowed a specific slot after the main part of the day and before the evening event starts to allow festival goers to visit the museum, but you can take the opportunity to visit earlier in the day, if you have time.

Permanent galleries include the Experiment Gallery, the Textiles Gallery, the 1830 Station, Connecting Manchester, the Air and Space Hall and the Revolution Manchester Gallery. Other exhibitions and demonstrations may be available that weekend. Find out more at: www.scienceandindustrymuseum.org.uk

THANK YOU

As always, a huge thank you to all who are raising awareness of porphyria and raising funds to help us continue the work we do. WE SIMPLY COULDN'T DO IT WITHOUT YOU! Whether or not you've been mentioned below, we are enormously grateful to you and your supporters.

80TH BIRTHDAY CELEBRATIONS

Thank you to Dee Davies, friend of Kenneth Staines. Dee celebrated her 80th birthday in November and instead of presents at her party, she requested donations to the BPA in support of Kenneth who has struggled with the very rare porphyria – congenital erythropoietic porphyria (CEP). Dee raised a wonderful sum of £305. Kenneth's wife, Pauline states: My husband and I have only known Dee for a relatively short time but she has shown such concern for Ken's problematic state of health and we are deeply touched by her kind gesture. She has certainly raised awareness of porphyria since everybody has been asking questions about it. … it has meant a great deal to him that people have shown such kindness and generosity. THANK YOU Dee for such a thoughtful gesture.

30TH BIRTHDAY

Justine Schofield asked for donations to the BPA rather than people buying her gifts for her 30th birthday as she wanted to support the work we have done to support her aunt, sister and niece who are all affected by acute porphyria. Justine raised a wonderful £100.00 – THANK YOU to all who helped to raise funds and awareness.

BINGO BONANZA

Alyx Byrne has been very busy on the Isle of Man organising lots of events to raise awareness of porphyria, in support of her niece and nephew who have EPP. By selling sweets and holding a bingo night, Alyx has managed to raise a wonderful £1,300. However, that's not all! In June, Alyx is walking 32.5 miles of the Manx Parish Walk in full photo-protective clothing to raise awareness and funds. Go Alyx! You're doing a fabulous job. THANK YOU and keep up the good work!

KENWORTHY FAMILY

A huge THANK YOU also to the Kenworthy family for their substantial donation of £1,000 to the BPA.

GIVOSIRAN UPDATE

Recent months have seen exciting new developments in the world of acute porphyria. You might have seen lots of talk about **'gene silencing'** in the media and on news channels. Gene silencing is the name given to a group of treatments that modify certain cells to effectively 'silence' those that cause problems. Gene silencing does not alter the DNA sequence.

Alnylam's siRNA (small interfering RNA) trial drug, Givosiran, that we've introduced you to in the past, is a type of gene silencing treatment.

Last year, the Alnylam Phase III ENVISION trial enrolled 94 patients for a six-month period, where half of the patients were given the drug and half were given a placebo. In April, Givosiran was announced to have met its primary effectiveness endpoint with a 74% mean reduction (90% median reduction) in annual attack rate in AIP patients compared to the placebo.

Patients on Givosiran also saw a reduction in the use of haemarginate or hemin compared to the placebo group. Furthermore, levels of the neurotoxic liver components ALA and PBG, which are thought to cause attacks and other acute porphyria symptoms, were consistently lowered to a near normal level.

To learn more about the full details visit: www.alnylam.com/2019/04/12/givo-easl-2019/ The results are highly encouraging and we'll continue to watch developments eagerly.

So what does this mean to UK patients?

This does not have a direct impact on patients at this moment as the medicine development process is very long. Once a medicine has been created to treat a condition it is first checked for safety and effectiveness – which is what the trial study has been aiming to do.

The next step, is for the results of the study and other data to be presented to the European Medicines Agency (EMA), who will consider the medication's risks and benefits and decide whether or not to approve it for marketing.

If the EMA approve the drug as eligible to be marketed in Europe, it will then need to be assessed by NICE (the National Institute for Care and Excellence), the independent body that makes decisions on how the NHS spend money on health care. If you have been following the progress of Scenesse through NICE, you will already be aware that this process alone is very lengthy.

But we are watching the process carefully and will be invited to take part to put the patient perspective forward in due course.

Find out more about the EMA process: www.ema.europa.eu/en/about-us/what-we-do

Find out more about NICE: www.nice.org.uk/about/what-we-do

INTERNATIONAL PORPHYRIA AWARENESS WEEK (IPAW):

6 APRIL – 13 APRIL

Well, IPAW certainly saw some successes this year. As usual, we joined together with the other international porphyria patient groups to raise awareness of porphyria this April.

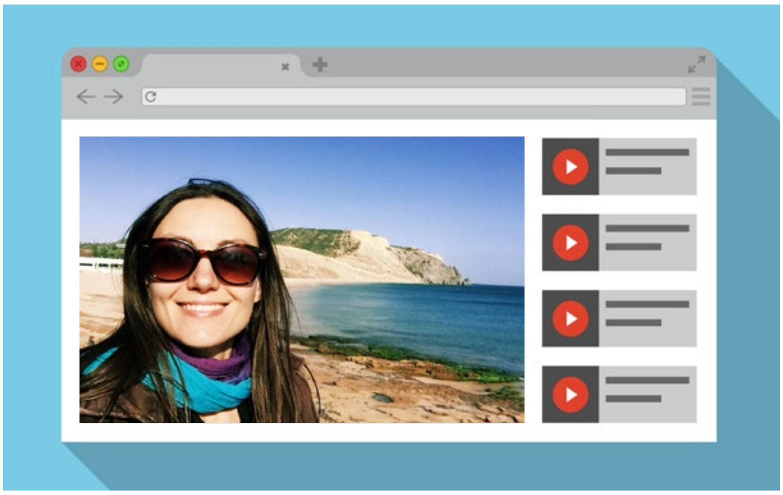
Diane Krypner and Alyx Byrne on the Isle of Man both held events to improve knowledge around EPP, and raised some fantastic funds in the process.

Our very own Events Coordinator and BPA Administrator, Sue Burrell, was interviewed in a lucky-timed series of articles with BBC News and The Times. IPAW coincided with the announcement of Alnylam's positive clinical trial results at an international liver conference and Sue was able to talk about her experiences of living with an acute porphyria in a live TV appearance, as well as being pre-recorded and being featured on BBC News online: www.youtube.com/watch?v=s5hi8ucm2qY and www.bbc.co.uk/news/health-47907971.

Well done everyone involved in IPAW this year!

SCENESSE®

Since our last newsletter, there have been few developments in relation to Scenesse. In response to last year's appeal hearing and further submissions from all stakeholders, another committee meeting was held at the NICE offices in Manchester. A collaborative and highly involved team of EPP patients, BPA committee members, representatives from the International Porphyria Patient Network (IPPN), leading photodermatologists from the British Association of Dermatologists, as well as an international EPP specialist from Switzerland all took part in the meeting. We are still awaiting the outcome of this session and will keep members posted as soon as we know more.



AIP PATIENT STORY

If you suffer from one of the acute porphyrias, or simply want to know more about them, then take a look at YouTube: https://www.youtube.com/watch?v=DpGg2seVO_g for a moving video in which Ania shares her personal story about living with acute intermittent porphyria. Many of you will relate to her experiences and her pain – it's well worth a view.

BLUE BADGE CHANGES

Blue badges can be extremely useful for those with porphyria, whether that be due to acute attacks that have caused paralysis and an inability to walk very well, or due to a skin porphyria and trying to avoid the light.

Importantly, blue badge parking permits are to be made available for people in England with "hidden disabilities". The Department for Transport said that changes will ensure that people with hidden disabilities will be able to enjoy the freedoms that many of us take for granted. Currently the rules do not explicitly exclude hidden disabilities, but councils' interpretations can vary. The changes which came into effect in 2019 may help to alleviate some of the stress involved in trying to obtain a blue badge. Similar changes have come into effect in Scotland and Wales.

When the changes to the blue badge scheme are introduced, they will extend eligibility to:

- people who cannot make a journey without "a risk of serious harm to their health and safety" or that of others, such as young children with autism
- people who cannot undertake a journey without "very considerable psychological distress"
- and those with considerable difficulty walking, meaning "both the physical act and experience of walking".

For example, for those with EPP, if a car has window films and is considered a 'safe' environment, a blue badge can help a patient get into buildings with a reduced risk of pain from exposure.

The scheme costs £10. Amongst other benefits, it allows badge holders to park for free in certain pay-and-display spaces across the UK, as well as to use disabled bays that tend to be closer to shops and businesses. The concessions do not apply to private car parks and cannot be used in central London, where residents need to apply for a special permit, although they do provide an exemption from the congestion charge.

Find out more at:
www.gov.uk/apply-blue-badge.

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.

Chairman: John Chamberlayne
Vice-chairman: Liz Gill
Treasurer: Anne Newton
Secretary: Richard Bennett
Administrators: Sue Burrell and Liz Gill
Patrons: Prof George Elder, Dr Mike Badminton, Prof Felicity Stewart, Dr Penny Stein, Prof David Rees, Dr Bob Sarkany

Embrace your creative side!!!

ART COMPETITION

Help us to design the front cover of the next BPSU & CAPSS annual report...and win a £50 Amazon e-Gift voucher!

THEME	RARE DISEASE & ME
PRIZE	£50 AMAZON E-GIFT VOUCHER & YOUR ART WORK DISPLAYED ON EITHER THE FRONT COVER OF THE BPSU OR CAPSS ANNUAL REPORT
WHO	ANY CHILD OR YOUNG PERSON LIVING WITH, OR HAS A FAMILY MEMBER AFFECTED BY, A RARE DISEASE
CLOSING DATE	MONDAY, 17 JUNE 2019
DON'T FORGET	DOWNLOAD THE ENTRY PACK AND SUBMIT THE ENTRY FORM WITH YOUR ARTWORK
MORE INFORMATION	VISIT HTTPS://WWW.RCPCH.AC.UK/BPSU/ARTCOMPETITION

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

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 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