

THE BRITISH PORPHYRIA ASSOCIATION NEWSLETTER

Registered Charity No. 1089609



DIFFICULTY WORKING DUE TO YOUR PORPHYRIA?

Maintaining a normal working life when experiencing health problems can be difficult for lots of people. Yet many people who are ill still want to maintain their independence and a sense of normality by continuing to work.

We recently learned about a new non-profit organisation called ASTRiiD. ASTRiiD aims to link businesses with professionals who have long-term health problems, but who still have lots of skills to offer to employers.

It takes the form of a website and online tool that matches individuals struggling to apply for full or part time jobs through the ordinary channels, with businesses that can use their skills for paid or unpaid work.

It's a very simple concept. The person seeking work registers their skills and experiences on the website, along with an

idea of how much time they might be able to give to a potential employer. This might be a number of days a week or even just a few hours a month.

Employers register and highlight the skills and experiences that they are looking for in order to complete some element of work.

ASTRiiD compares all of the registrations to see if there is match. If there is a good match, they will introduce one side to the other.

ASTRiiD calls the people who are affected by long-term/chronic health conditions the Invisible Talent Pool. "The role of ASTRiiD is to make the invisible visible and by doing so help those that wish to continue to work in some capacity to find an outlet for their skills."

To learn more about this brilliant concept visit: <https://astriid.force.com/s/>

REMINDER: LIGHT PROTECTION FUNDS AVAILABLE

We have funds available for light protection projects for members with photosensitivity. For example, window films on cars and homes can significantly improve the quality of life for those with EPP. If you would be interested in applying for a grant to help with the cost of installing a light protection measure, please do let us know on helpline@porphyria.org.uk or 0300 30 200 30.



GENERAL DATA PROTECTIONS REGULATIONS (GDPR): OPT IN NOW!

Changes to data protection laws will soon affect the way we can contact you. After May 2018, if you still wish to receive our newsletters, leaflets and information on our events, we will need your explicit consent.

We support these important legal changes and want to make sure that you only receive information from us that you want to receive, and in the ways that you would like.

Look out for our letter that asks you to choose how you would like to be contacted. Alternatively, go to www.porphyria.org.uk and select 'Opt In' to complete the form.

ACUTE PORPHYRIAS: SAFE DRUGS LIST

The SAFE drugs list produced by the UK Porphyria Medicines Information Service (UKPMIS) is updated each year. It is important that acute patients keep an up-to-date copy with them. Please contact 02920 743877 / 02920 742251 to be added to the UKPMIS database to receive your new copy each year. The 2018 version will come out in May and will be yellow.

To help comply with the new General Data Protections Regulations, UKPMIS need everyone to opt-in to receive communications from them, so that they can continue to send safe list updates. Patients can do this by emailing their consent to welshmedicines.information@wales.nhs.uk or calling 029 2074 4298.

WOULD YOU LIKE TO ATTEND A BPA EVENT IN 2018?

We are delighted to be able to invite you to two events this year.

OPEN DAY

Saturday 9 June 2018 in Glasgow, Scotland

Our open day will be held at the Queen Elizabeth University Hospital in Glasgow with the support of Dr Galloway and Dr Jane McNeilly.

AUTUMN CONFERENCE/AGM

Saturday 6 October 2018 in Reading, England

Our autumn conference will be held at the Holiday Inn Reading West with the support of Professor Rees from Kings College Hospital, London.

The programmes are yet to be finalised, but will include doctor and patient talks, updates on research and plenty of opportunities to speak with other patients and members.

We very much hope to see you at one of these events. They are free to attend, but registration is essential in order to reserve your place and for catering arrangements to be made. If you would be interested in attending, please forward your name, and the names of anyone wishing to come with you, to sue.burrell@porphyria.org.uk or call 0300 30 200 30 to confirm your attendance. We will send further details out a short while before the event.

Travel bursary: We have funds available 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).

NEW MEDICINE DEVELOPMENT

Following the promising results of the Phase 1/2 trial, Givosiran, a potential treatment for recurrent attacks of acute porphyria, is now being investigated through a Phase 3 trial. It is taking place in the UK and at various sites around the world. Alnylam have named this phase, ENVISION. They aim to enrol around 74 patients worldwide over the next few months, to obtain data on the effectiveness of Givosiran at reducing acute attacks. Find out

more on how the trials are progressing at www.alnylam.com. At some point over the next few months, Alnylam also aims to start a patient journey project. The goal of the project is to look at quality of life factors, such as the socio-economic and physical burden of acute porphyria, in greater detail than has been done before. Would you like to be involved? Get in touch on helpline@porphyria.org.uk or 0300 30 200 30 and we will pass on your details to the relevant team.

SCENESSE® UPDATE

NICE is presently reviewing submissions made in response to their initial recommendation not to make Scenesse available in England via the NHS. Released in December 2017, in the format of an Evaluation Consultation Document, this initial recommendation was highly disappointing, but is not yet a final decision.

A meeting at NICE on 20th Feb 2018 saw the BPA, UK EPP patients, expert clinicians, and Clinuvel all put forward rationale for the initial recommendation to be reviewed and reversed. The BPA is grateful to all parties, along with the International Porphyria Patient Network and EPP patients throughout Europe and the rest of the world, who added highly valuable commentary to the debate. We send special thanks to the

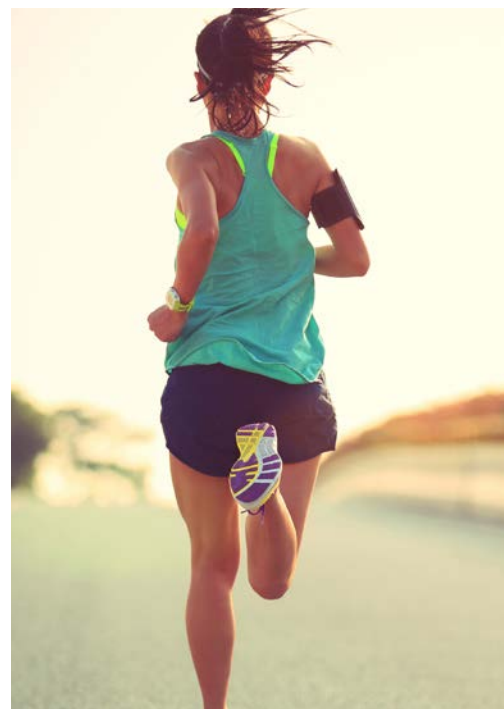
German and Swiss patient networks for both the strength and quality of responses delivered to NICE's initial Evaluation Consultation Document.

The current timeline for further recommendations and decisions by NICE is as follows:

23rd May 2018 - Final recommendation from NICE to be published.

Further details can be found on the NICE website at: NICE – Afamelanotide Appraisal ID927

A press release from Clinuvel following the initial recommendation, not the February committee meeting, is available at: Clinuvel – Investors Announcements (see article dated 21/12/2017).



FUNDRAISING AND AWARENESS

As can be seen from our regular thank you section on the newsletter, you, and your families and friends are amazing fundraisers. We are enormously grateful for all the time and effort put into raising funds and awareness of the porphyrias.

There are lots of events happening again this year. Some of you will have been involved in International Porphyria Awareness Week (IPAW – 21-28 April 2018). If

you did something to mark IPAW, we'd love to hear your stories and see your pictures.

ACTIVE EVENTS

Would you like to take part in an active challenge? We currently have the following official places available:

- Ride London-Surrey 100 (Sunday 29 July 2018 – fundraising target £600): 2 places

- Great North Run (Sun 9 September 2018 – fundraising target £300): 5 places
- Great South Run (Sun 21 October 2018 – fundraising target £300): 1 place

Places are allocated on a first come, first served basis, so please let us know ASAP if you or someone you know is interested on liz.gill@porphyria.org.uk and we'll send

you more information.

If that sounds a bit challenging and you'd like to do something different, please get in touch or check the website for our fundraising pack for lots of ideas.

Alternatively, most of our fundraisers have Just Giving pages if you would like to support them.

Please go to www.justgiving.com/britishporphyriaassoc.

JEANS FOR GENES DAY

Friday 21 September 2018

1 in 25 children has a genetic disorder that makes their life very difficult. That's roughly one in every class in most UK schools. Simply by wearing jeans and donating, Jeans for Genes hopes to change the lives of these people.

Lots of rare disease charities are joining together to help share the Jeans for Genes brand and raise awareness together in the hope that increased numbers can make everyone more effective.

A Jeans for Genes Day is fun and simple to organise, and is a great way to bring a whole school or workplace together, while raising money for Genetic Disorders UK and the BPA at the same time. All you need to do is ask everyone to wear their jeans and make a £1 donation.

Go to www.jeansforgenesday.org to sign up for a free fundraising pack. Don't forget to choose the British Porphyria



Association as your affiliated genetic disorders charity, so that we get 50% of any income raised. Remember it can be great awareness for porphyria as well as your school or workplace. Why not get the local paper involved and tell your story at the same time?



THANK YOU

Each year, Alnylam employees run a silent auction at their end of year holiday party. They typically divide the funds raised between different patient organisations working in rare diseases. This year, they split some of their fundraising between the BPA and three other porphyria patient groups in the EU including the Spanish, Swiss and French groups. Each group received USD \$7827, which was converted into £5428 for the BPA. We would like to express a massive THANK YOU to the employees who raised an exceptional amount of funds for patients in the UK and abroad.

Another significant group of fundraisers to which we owe enormous gratitude are those involved with the Helen Gibbs Trust Fund. Helen died in 2006, aged just 26, due to complications from acute porphyria. Family and friends set up a trust in the period after Helen's death to raise funds for research into

the acute porphyrias. Over the years, enthusiastic and generous fundraisers raised more than £28,000 through a variety of events, such as fun runs, marathons, football matches and their annual 'Hint of Pink' May Ball!

This year, the trustees closed the fund and transferred the remaining balance of £4645 to the BPA. We would like to express a huge and heartfelt THANK YOU to all of those involved.

IN MEMORY

The BPA were sorry to hear of the sad loss of Terry Maguire, one of our loyal members and supporters. In his memory, Terry's family and friends kindly sent donations to the BPA raising more than £900. Thank you.

Thank you also to the family of Kathleen Watton. We received a donation of £60 in January in memory of Kathleen.

TEDx TALK

Sue Burrell, from our committee, was kindly invited by TEDx University of East Anglia to speak at their first ever TEDx event in February this year. Sue talks about living with AIP and its complexities, as well as about rare diseases and the work she does with the BPA.

Her talk, entitled: 'Try, try, try: my porphyria fight' is now available to watch via the TEDx Talks YouTube Channel: <https://www.youtube.com/watch?v=8uHdArMPpVs>



MY LOST SUMMERS

My name is Hazel. I had my 70th Birthday this year. I am an EPP sufferer.

I've always wanted to write my story about living with EPP, if only to help some parent with a child who is suffering. I never thought anyone would be interested until I attended the BPA Conference in October. Hearing the very emotional story of an EPP sufferer at the conference, and the heartfelt experience of the husband of an AIP sufferer and the pain they both suffer directly, or because of a loved one, I now feel it is the right time to speak of my own experiences.

Back in the 50s when I was a child, I don't think EPP was even a known condition. It was not until the age of 26/27 years that I was diagnosed. But, from the age of six or seven years I knew I was different. I don't remember much about the pain before then, so maybe that's when it started. All I remember was dreading the summer.

Spring was my favourite time of year, when the earth woke up, pushing the daffodils and tulips out of the cold ground. The trees just started to pop with green shoots reaching out of the bare branches. Longer days with respite from the cold, when I felt excited at the few days of mild warmth I could experience before my freedom would once again be curtailed.

I had just moved with my Mum, Dad, older sister and younger brother to Ilkley. I loved living in Ilkley, it was the most wonderful place. The river Wharfe running almost at the bottom of our street, and the Cow and Calf rocks up on the moors just a mile away. Lots of green lush places around us. It was a child's paradise.

I had not been at the Primary School very long before I was assessed, my parents being told I needed to be taught by a remedial teacher for under achievers. I was, in their opinion, "not bright enough to be taught in the mainstream classes". Even at seven years old, this was quite a blow, especially as my sister was clever enough to attend the local Ilkley Grammar school. I felt like a dunce.

Every day I would walk to school and back, four journeys as I went home for lunch. School was probably three quarters of a mile away, this was fine through autumn and winter and

wonderful at the start of spring, but then the inevitable sun would appear and with the sun something bad started to happen. When I look back, I wonder how no one connected the dots. I knew even from a very early age it was the sun that caused my pain...why could no one else see it.

My mother took me to the doctors several times throughout the summer. I don't remember going but I do remember my mother telling my dad that the doctors said it was all in my imagination. Another time, when my hands had broken open in sores, she thought it was due to a cat scratch, only I knew the truth as I prayed for rain.

How I ever learned anything at school is a mystery to me. Throughout the long hot summers of the 50s, I spent my time with my hands inside my cardigan or behind my back, which never worked. From April until October, when the sun came out I suffered.

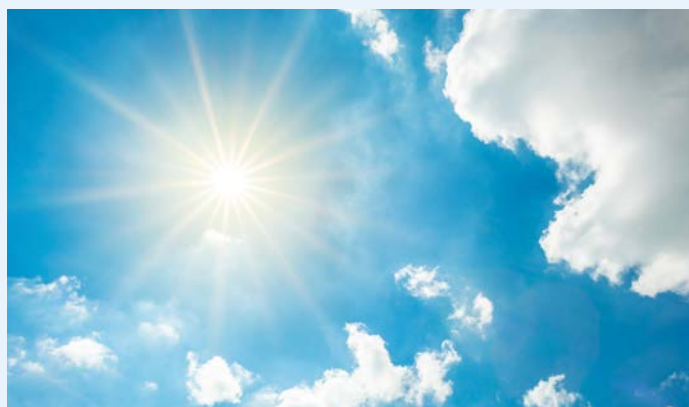
When I arrived at school I would put the backs of my hands on the cold metal under the school desks to try find some relief. When that did not work, I would perpetually ask to use the toilet. My teacher must have thought I had a bladder problem! I would rush to the sink and run my burning hands under the cold tap which made them itch and tingle, so I would flap them about like a ducks wings or even press them with my other hand to get relief... I could not bear anyone to touch my hands, something as simple as a scratch felt like knives were ripping my skin. I never told my school friends, I thought they would think I was making it up. Even though at times the backs of my hands would break open and bleed.

Playtimes were spent in the shadows, and at the end of the day I would run home and spend the rest of the day and night wetting flannels and putting them over my hands as a way of easing the pain, just so I could get a few hours respite and some restless sleep. I was exhausted, and the hot days seemed never ending like the pain. I would pray for rain.

I had many friends in Ilkley and just wanted to be like everyone else. I wanted to play out, go down to the river, cross the stepping stones and run in the fields. I would always seek the shade of the trees, I loved the trees, but it was not enough, and if I wanted to play out I had to suffer, and so it went on year after year after year.

We moved again to Kirkburton, near Huddersfield, just before my 11 plus. Needless to say, I failed that! I had by that time realised that I was not an absolute dunce as I quite enjoyed English and Maths and seemed to be quite good at it. Nonetheless I was assigned to class 1B. I am relieved to say that despite my problems I attained top of the class when I reached 2B and was sent up to 2A. I spent the rest of my school life in the A stream, even though I was near the bottom I never went back down.

I'm not saying that was the end of it. It would take a further 15 painful and depressing years before I had my diagnosis. But that is another story.



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. Timothy Cox

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CALLING ALL ARTISTS

The 2018 Rare Artist Contest is an international competition hosted by the Every Life Foundation for Rare Diseases, United States. It celebrates the unique talents of the rare disease community and has an online gallery dedicated to artists affected by a rare disease.

All drawings, paintings, mixed media, sculpture, photos and digitally-created images are welcome. Submissions can be made via Facebook by uploading a high-resolution image of the artwork. All entries must note the inspiration for the piece, the artist's age, and the name of the rare disease affecting them or a loved one.

Entry categories are: Children (4-11), Teens (12-18), Adults (19+), Adults (19+) – Photography/Digital Art.

Pieces selected as award recipients or for special artistic merit awards will be displayed at a Rare Artist Reception on Capitol Hill, and all award recipients will be invited to view their displayed work. A travel stipend will be provided to enable this, but this is solely for US applicants.

The full 2018 contest rules can be found at: <http://www.rareartist.org/2018-art-contest/>. Questions or concerns can be directed to the Contest Curator, Grant Kerber: gkerber@everylifefoundation.org.

Entry deadline: 19 October 2018

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 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).
- 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