Issue 30 / May **2015**

THE
BRITISH PORPHYRIA ASSOCIATION
NEWSLETTER

Registered Charity No. 1089609

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SCENESSE®: COMMERCIAL DISTRIBUTION IN PREPARATION FOR EUROPEAN EPP PATIENTS

As many of you may have heard, at the end of 2014, Clinuvel Pharmaceuticals Ltd announced that SCENESSE® (afamelanotide 16mg) had been granted "marketing authorisation under exceptional circumstances" by the European Medicines Agency (EMA).

What does this mean for UK EPP patients?

Clinuvel is now allowed to market SCENESSE® for the reduction of light sensitivity in adult patients with EPP. They will first focus on rolling out SCENESSE® in the eight countries across Europe where EPP clinical trials were conducted, including the UK. As a condition of the approval, they have to record patient safety and their benefits from the treatment. This involves setting up a registry of patients, and their diseases.

The drug will not be available immediately, as Clinuvel must agree arrangements with individual government bodies (including their funding of the treatment), and then of course organise the distribution of the drug. In the UK, Clinuvel is currently discussing with NICE the payment for SCENESSE® by the NHS in England, Scotland and Wales. These approvals can take quite some time, but the BPA are assisting with this. Our EPP patients are providing evidence and explaining how severely affected those with EPP are, on a daily basis, as this input is important for any approval for use in the NHS. You can read a little more about the process with NICE, who are responsible for England, here: *www.nice.org.uk/about.*

As soon as the BPA hears more, we will let our members know.

SAM'S STORY (AGED 14)

I can remember being on a beach running from the sand into the sea. It was boiling hot and the sun was shining and I ran in and out of the sea over and over again – it was wonderful. Mum says I was five years old.

When I was seven I went on a school trip that changed my life. On the second day there, we went on a long walk, it was very hot and sunny and I remember walking in a T- shirt. Suddenly my skin was burning and I put my jumper on. The next three days were terrible because my skin hurt so much but the teachers kept saying there was nothing wrong.

When I got home and got off the coach, Mum knew – she knew I had EPP (because she has it too). It causes unimaginable pain and I wanted to give up – it has made my life very stressful and I find looking to the future very difficult.

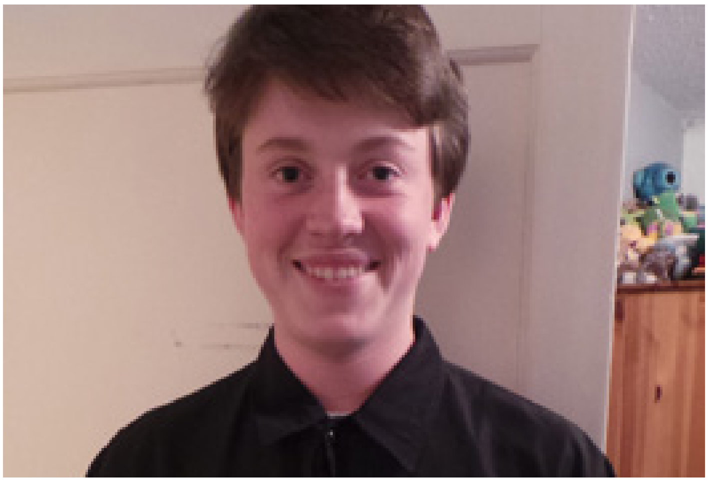
Everything has changed, school is difficult and I am always worried that if I have different teachers they won't know that I can't sit in the sun or under some of the class lights. If a teacher doesn't believe me I am allowed to leave the class and go to a safe room. I have given up with PE, it is difficult to explain that even if I am not in the sun my skin can still be painful and I don't want to be bashed about. The PE teachers were not able to understand this.

If I didn't have my friends, I wouldn't go to school, they are the best – they understand me, they don't think I am making it up and they look after me. Some of my friends have problems too so we look after each other – we are very close and we cope with stuff together.

Mum started taking me to BPA meetings soon after I started with symptoms. The first one I went to a man shook my hand and said I was the first man (I was 8!!) he had met with EPP. We have been to lots of meetings since then and I have met other children with EPP. I met a little person and I think their mum was pleased to see that you can grow into a big person like me with EPP and be OK. Recently, I went to a meeting where I met a young boy with EPP who was bored with the technical talks, so we went and played games on our phones, but we found a place where we could turn the light off (of course)!

I help mum do 'the patient experience talks' and I have discovered that I don't mind doing this. I am not nervous at all which I think is something that I may be able to use when I am looking for a job.

Being involved with the BPA has also meant that I have been asked to explain about being light sensitive to many different people and so I have: given three talks at BPA meetings, been on Oxford Radio, been on Central News and in the Daily Mail and Chat Magazine. (I got paid for Chat Magazine and it has gone towards my new laptop).



MUM STARTED TAKING ME TO BPA MEETINGS SOON AFTER I STARTED WITH SYMPTOMS. THE FIRST ONE I WENT TO A MAN SHOOK MY HAND AND SAID I WAS THE FIRST MAN (I WAS 8!!) HE HAD MET WITH EPP.

I have light treatment every year to try to make my skin tougher – this is my seventh year and I have just come home from my third light treatment (out of 20). Amersham hospital has new light boxes this year, they are incredible – it's like being in a Ferrari after a Fiesta. When I started light treatment my nurses Bianca and Claire were big people, but now, seven years later, they are tiny – I am six foot 2 inches – but they are still in charge of me and they are one of the rare breed of people who understand me. They are amazing!

In his article, Sam has demonstrated some of the great reasons why we run the Open Days and meetings. It isn't simply about our scientific talks or updates on the latest research. It is also about meeting people that you can relate to, being comfortable with your condition because others there understand; and also about feeling empathy with others who may be living in similar circumstances, or supporting someone who is. If you would like to attend any of our upcoming meetings, please call 0300 30 200 30 or email helpline@porphyria.org.uk to let us know and we will send you more details.

WHAT DO YOU WANT FROM YOUR BPA?

As mentioned elsewhere in this newsletter, the BPA have recently appointed their first paid member of staff. This gives us a great opportunity to review what things are most important for the BPA. We would therefore like to ask all members what they would like to see from their BPA:

- How can we help you most?
- What things are we doing that you like?
- Have you any comments or suggestions for things that we could do to improve or help you more?

If you have any thoughts, we would love to hear them, you can contact us via: *helpline@porphyria.org.uk* or 0300 30 200 30.

WITH THANKS

CHICAGO MARATHON 2014

Samantha Best ran the Chicago Marathon in October 2014 to raise funds in aid of Helen Gibbs, a close friend who sadly passed away from AIP. Samantha raised well over £4,500, which has been donated to the Helen Gibbs Fund, a restricted BPA fund set up solely to further research into the acute porphyrias. THANK YOU so much for your continued support, Samantha.

RAISING AWARENESS OF EPP - CLAIRE CREAG

Claire Creag is continuing to do some amazing work to raise funds and awareness of EPP for the BPA. A HUGE THANK YOU Claire for everything that you are continuing to do!

HELL RUNNER EVENTS

A massive THANK YOU goes to Seb Clark and his friends for running three brutal Hell Runner events in the North, Middle and South of the country! They managed to smash their target and raise over £750.00 for the BPA.

FUNERAL DONATIONS

The BPA would like to send a heartfelt THANK YOU to Valerie Woodhouse's family for £440 in donations that were taken at her funeral late last year as the BPA was a charity close to her heart.

Similarly, we'd like to also offer a sincere THANK YOU to Colin Potter's friends and family who also chose to collect donations for the BPA at his funeral, raising £200.

THANK YOU to Sheryl Carstairs, partner of the late Gordon Boyd, and his family and friends who raised £310 in donations in his memory.

THANK YOU LUCAS ROCHFORD

A huge THANK YOU to Lucas Rochford, aged five, who decided to raise funds for the BPA after learning about two friends with EPP and understanding that they can't play out as much as he can. By running the Mini Great North Run in October 2014, in a pink t-shirt, he managed to raise a fantastic £115 for the BPA. What a little star!

HELP FUNDRAISE FOR THE BPA

Could you sell merchandise to fundraise for the BPA?

We have various BPA labelled items available; if you would like to sell some to your friends, at coffee mornings, school fetes or anywhere else, please let us know.

We can supply presentation boxes for the items, money pots to collect the funds in and various merchandise items, including: red BPA wristbands, purple EPP wristbands, pens, propelling pencils, stylus pens, key ring torches, pin badges, canvas bags, cotton t-shirts, professional running vests and professional cycle jerseys. All have our name / web address on them, so they are good advertisements for the BPA.

If you would like more information on the merchandise that we have available, please contact our helpline via: *helpline@porphyria.org.uk* or 0300 30 200 30.

CUSTOM VAN SHOW: WEEKEND 12/13 SEPTEMBER 2015

For the second year running, Ray Lancaster and Jenny Coombs are organising the Revolution Custom & Classic Van Club Show in Skegness, to raise funds for the BPA. Van owners and enthusiasts will be coming from all over the country to show off their custom vans. There will be entertainment and competitions for adults and kids! Starts at 10am each day.

The event will be held at the Pine Trees Leisure Park, Croft Bank, Skegness Lincolnshire, PE24 4RE (Tel: 01754 762949). £1 entry at the gate. £5 per day for vans showing, but not camping. For an overnight stay at the camping/caravan site, bookings are £14 a pitch or £16 with electric hook-up. Bookings are to be made directly with the venue, quoting ref: BPA. *www.pinetreesholidays.co.uk.*

Anyone interested in helping with the event/a stall can contact Ray on 07799 392 771 or *magicoazulcc@yahoo.co.uk*. If any business or individual has a raffle prize that they would like to donate, then please let Ray know.

OUR NEW ADMINISTRATOR - SUE BURRELL

Last year, the BPA committee discussed appointing a part-time paid administrator. We had a list of things we needed help with. Our volunteer committee often don't have time to attend joint charities meetings (such as Genetic Alliance), and we can miss out on some useful information. We also needed help with social media (Facebook, etc., answering queries and comments properly), help with paperwork (writing letters, leaflets, helping with layouts), and someone to look at fundraising. Thanks to yearly subscriptions and fundraising by enthusiastic members, we have been able to provide start-up grants (of up to £5,000) for research into EPP, CEP and AIP. However, at this stage, we have too little to fully-fund any large research projects (one researcher costs at least £70,000 a year, including lab space, equipment, etc.).

We advertised on two charity jobs sites and our newsletter/website in November, asking for someone who could give six hours per week, self-employed. By the deadline of December 31st, we'd had almost 100 people contact us for the job specification and 36 people formally applied for the role.

Since our Events Co-ordinator, Sue Burrell, applied for the job, the selection committee who looked at the applications had to exclude any of her family to ensure impartiality. This committee went through the CVs, letters and application forms, and chose a shortlist of seven who were asked to provide references.

Once the references were in, we again went through them, picking the three best candidates who were invited to a Skype interview. Since we use Skype for meetings, it was important to know that they could use it.

Each time the candidates were ranked, Sue Burrell was at the top of the list, so the selection committee decided to appoint her on a six-month trial.

Sue has degrees in psychology and HR, plus very good computer skills, as well as plenty of enthusiasm. She surprised the selection committee by giving a PowerPoint presentation at the Skype interview using full screen for the slides. None of the selection committee knew you could do that!

As someone who is already self-employed, Sue's appointment gives her more time to spend on BPA affairs, in addition to what she will continue doing as a volunteer. As a paid employee, she cannot remain a Trustee, so has resigned. Claire Taylor (our EPP expert) is taking her place.

At the beginning of March, Sue attended a charities conference, which gave us additional information for our new leaflets on benefits. It was a good demonstration of how having someone available to attend these conferences can give the BPA really useful information.

NEW LEAFLETS

In addition to updating our old leaflets, the BPA have been working on some new patient information leaflets. Our latest leaflet relates to benefits and concessions available for people struggling with their porphyria. There are two versions, one for EPP patients and one for those with an acute porphyria.

We now have the following available:

- Introduction to the porphyrias
- AIP
- EPP
- Testing and inheritance
- Benefits and concessions: acute porphyrias
- Benefits and concessions: EPP
- Drugs and porphyria

These are available electronically (online) and at our meetings, but please let us know if you would like paper copies sending out to you (simply contact us on 0300 30 200 30 or *helpline@porphyria.org.uk* to tell us which ones you would like).

Finally, we recently obtained a copy of the Disability Rights Handbook, a very useful resource, especially for those needing to go through the application for Personal Independence Payment. This and other resources will be available for members to look at during our meetings/open days.

NAPS IN SCOTLAND

NHS National Services Scotland has been working with NHS England to ensure that Scottish patients have access to the National Acute Porphyria Service (NAPS) centres in Cardiff and London. Both centres have agreed to provide expert advice to Scottish clinicians and, when required, hospital admission for residents of Scotland. A supply of haem arginate is currently held in Glasgow, if needed by patients with acute porphyria. This ensures the medicine is urgently available if required on the advice of the experts.

The BPA have also recently learned that there is discussion underway regarding the setting up of an outreach clinic in Scotland, where one of the specialists from England/Wales would travel to one of the Scottish hospitals to run a clinic in association with one of the Scottish doctors. This process isn't finalised as yet, so no promises made, but we are very hopeful that this will happen soon.

EVENTS 2015:

The BPA are pleased to announce that we have two events scheduled this year.

If you would be interested in attending any of the following events, please contact: sue.burrell@porphyria.org.uk for more information and directions.

OPEN DAY – LEEDS – FRIDAY 29 MAY 2015

On **Friday 29 May** we will be holding our **Open Day in Leeds**. The meeting is free to attend and will be held at Devonshire Hall which is one of the University of Leeds' city centre campuses. The programme will start with registration and refreshments at 10am, for a 10.30am start, and will finish with refreshments at around 4pm. Lunch will also be provided.

The programme is coming together nicely and we have already confirmed the following speakers: Dr Stein (one of the lead acute specialists who deals with some of the most severe acute patients); a consultant Clinical Biochemist from the Specialist Laboratories in Leeds; a representative from Clinuvel Pharmaceuticals Ltd; a representative from Alnylam Pharmaceuticals; a window film company; and two patient experience talks. In addition to these talks, we are planning on having a breakout area for any younger people who attend, with some games and a couple of films to watch. The day will conclude with a question and answer session, and time for informal chats with other porphyria patients and their families.

Would you like to pose any questions to the experts?
In an attempt to really meet our members' needs, all of the speakers have agreed that we can pose questions before the meeting, and they will do their best to answer these questions (time permitting) throughout the day. Please send any questions that you have, in advance, to *sue.burrell@porphyria.org.uk*.

Live Twitter Feed for the Open Day in Leeds
We have been looking into ways to make our meetings more interactive and accessible, after a suggestion on Facebook for the meetings to be recorded and then either streamed live or uploaded online later. This is something we would love to look into for future events, but feel that we would need a little more time to do this properly.

In an attempt to increase accessibility and interaction we are pleased to announce that Jo Taylor has volunteered to hold a 'Live Twitter Feed', for questions and comments that can be posed to the speakers on the day. We will endeavour to answer as many questions as possible, but as this is the first time that we'll be trialling this technology, please be patient if for any reason we have any teething problems on the day!

The BPA is more than happy to provide funds towards your travel to meetings (up to £50 for travel and £50 for hotels), if you would like more details please do not hesitate to contact us on 0300 30 200 30 or *helpline@porphyria.org.uk*

AUTUMN CONFERENCE AND AGM – LONDON – SATURDAY 24 OCTOBER 2015

Our **Autumn Conference and AGM** will be hosted by Dr Sarkany. It will be at Guys and St Thomas' York Road Education Centre in central London (near Waterloo Station) on **Saturday 24 October**. The meeting will start with refreshments and registration at 10.30am for an 11am start, a light lunch will be provided in the middle of the programme and refreshments will be served to finish at 3pm.

We are still planning this event with the help of Dr Sarkany (a dermatologist who is a lead specialist in EPP and other skin porphyrias), who will also be a speaker. The BPA will hold a brief AGM and the following speakers will be in attendance as well as Dr Sarkany: a representative from Clinuvel Pharmaceuticals Ltd, a representative from Alnylam Pharmaceuticals, a window film company and two patient experience talks.

Again, we will look into options that will help to create access to all of our members, whether in the form of a Live Twitter Feed or through videoing the speakers.

PORPHYRINS AND PORPHYRIAS – INTERNATIONAL CONFERENCE – DÜSSELDORF, GERMANY – 13-16 SEPTEMBER 2015

The **4th International Patient Day** will be held on **Sunday 13 September** at the City Hostel (*www.jugendherberge.de/en/youth-hostels/duesseldorf442/Portrait*) in Düsseldorf, Germany. The main theme of the day will be new therapies for the cutaneous and acute porphyrias.

The main **International Porphyrins and Porphyrias Conference** will be held on the following three days from **Monday 14 to Wednesday 16 September**. Although the conference is aimed at medical personnel, the BPA plan to have a representative at the Patient Day and three-day Scientific Conference, so as to be able to keep informed and then report back on any new developments or areas that may help the porphyria community in the future.

Further details of all of these events will follow, once available. We will promote these on Facebook and on our website.

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: Jacqueline Binns
Administrator: Sue Burrell
Patrons: Prof. George Elder, Dr. Mike Badminton, Prof. Felicity Stewart Prof. Timothy Cox

We have lots of people helping to raise funds for the BPA this year, we would like to wish the following people (and anyone else doing events that we are unaware of) all the best of luck.

Lorraine Valentine, we salute you! It will be a massive achievement to run the London Marathon when you have EPP – what an inspiration to the EPP world. As this newsletter goes to print, you will be running for us! In our next edition, we are hoping to have an article written by Lorraine about the trials and tribulations of training for and running such an event.

Wishing the best of luck to our Great North Run fundraisers, Laura Welch, Emily Todd, Debra Baker and Craig Bell! We still have one place available, please let us know if you know anyone who may be interested in running in this event.

As for our Ride London-Surrey 100 riders, let's hope you get better weather than last year!

THE **BRITISH PORPHYRIA ASSOCIATION** Registered Charity No. 1089609

MEMBERSHIP / DONATION FORM & STANDING ORDER

Title Name

Address

Postcode

Email

Telephone

Type of Porphyria

Date

WAYS TO PAY

☐ I would like to pay my annual membership fee of £15
☐ I would like to make a donation of:
  ☐ £10 ☐ £15 ☐ £20 ☐ £25 ☐ £50 other £
☐ I would like to set up a standing order (please fill in the form opposite)
☐ I have set up a standing order using my internet banking*
☐ I enclose a cheque made payable to the 'British Porphyria Association' for £
☐ I have made a payment using *www.justgiving.com*
☐ I would like a receipt

**please use your name and postcode as reference for an online payment*

By making a donation to the British Porphyria Association you will know that you are making a vital contribution to our work. Simply making a small donation will help us raise awareness, man our helplines or support research.

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.

Standing Order British Porphyria Association
Please send this form to: The Treasurer, British Porphyria Association, Rothlea House, 7-8 Quarry Lane, Butterknowle, Bishop Auckland, County Durham, DL13 5LL
A monthly standing order or any donation you can give will make a difference.

Bank / Building Society name

Branch address

Postcode

Please pay the British Porphyria Association the sum of £

each month / quarter / year (delete as appropriate) from my account until further notice.

Account name(s)

Sort code Account No.

Starting on* (Date)

*This date must be more than one month after today's date

Signed Today's date

This cancels all existing standing orders to the British Porphyria Association
(please tick) ☐ yes ☐ no ☐ not applicable

Please pay to British Porphyria Association bank account:
Sort code: 20-43-63 Account No:7099 6904

Gift aid declaration
I am a UK tax payer 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, until I notify you otherwise. 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