HAE UK Winter 2018 Newsletter

HAE UK is an Association of HAE Patients, working together to improve the situation for all HAE Patients in the UK

Welcome to our Winter 2018 newsletter

As we get towards the Autumn and the end of 2018, we get tied up with the two Patient Days, one in Scotland and one for England, but some other events made headlines too, read on to find out more...

We are also excited to tell you that the HAEi Global Conference in 2020 will be held in Frankfurt, Germany. More details will be available from HAEi during 2019.

C1 INH SUPPLY

Since the extreme shortages of C1-INH throughout the past few months, supplies are slowly starting to build up, but CSL Behring stress that in order to ensure everyone gets supplied, they are sticking to their policy of delivering smaller amounts fortnightly, instead of larger amounts less frequently.

Shire can supply existing patients with Cinryze but have no extra supply.

CSL Behring hope that a couple more months using this strict supply control will be sufficient to replenish their supplies and deliveries can then start returning to normal.

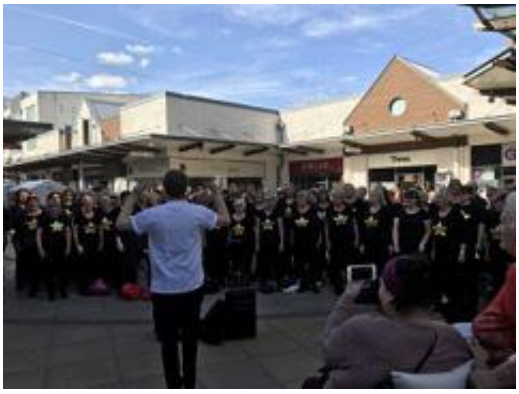
If you are having particular difficulties please contact HAE UK in person via e-mail or phone for advice and/ or support. We are here to help you, anytime, but can only respond to individuals contacting us in person.

SHIRE HAE PATIENT SURVEY

There is a survey running about your experiences of living with HAE. It is being run by Shire (manufacturers of Cinryze, Icatibant and Lanadelumab) to see where they can help your Immunology team to improve your care. Go to our website www.haeuk.org and click the link on the news ticker.....

ROCK CHOIR EVENT

One of these was organised by June Cole, who is absolutely passionate about raising awareness of HAE and educating both the public and clinicians.



June has successfully presented to both her A&E department and her local general practice doctors about Hereditary Angioedema, treatment and living with the condition. Her latest brainchild was to combine her love of music with awareness raising; June sings with her local Rock Choir and organised a 'Flash Mob' at the huge Westwood Cross Shopping Centre in Broadstairs. A 'Flash Mob' starts with one or two people casually starting to sing then all the choir (who are pretending just to be shopping) join in to end up with a fantastic event.

Try this link for more photos https://m.facebook.com/photo.php?fbid=305541840239351&id=296895801103955&set=a.305848060208729&refid=13&__tn__=%2B%3E

IMMUNOLOGY CENTRE PATIENT EVENTS

Several of the Immunology Centres have run their own Patient Days this year; Leeds, Birmingham, Salford and Addenbrookes These have all been well attended and a variety of interesting topics covered during the days. It is good to know that the consultants and nurses are really involved with treating HAE and are trying to make as much progress as possible with treating all patients as individuals rather than a 'one size fits all' approach.

TREATMENTS IN SCOTLAND AND WALES

We have also had success over the Summer with the devolved governments; Scotland accepting an application for Ruconest to be available to Scottish patients and Wales allowing Icatibant and Ruconest to be available. This means they are automatically funded by NHS Scotland/NHS Wales and are freely available for prescription.

SCOTTISH PATIENT DAY

We spent a wonderful weekend in Edinburgh for the third Scottish Patient Day with 46 attendees, mostly patients but some of the specialist nurses also attended.

We are particularly grateful to Dr Moira Thomas and Dr Charu Chopra for giving up their Saturday to attend and to help make it such a successful day.

Scotland is so lucky to have such expert and sympathetic clinicians looking after our HAE patients. Of course, there are always problems, one of which is getting a level provision of home delivery to patients but, with people like these looking after our patients, we can sleep more peacefully.

Paediatric specialist nurse Charlotte Vost gave us a presentation on dealing with younger patients and Rachel Annals told us her patient story.

The day finished with a lively question-and-answer session.

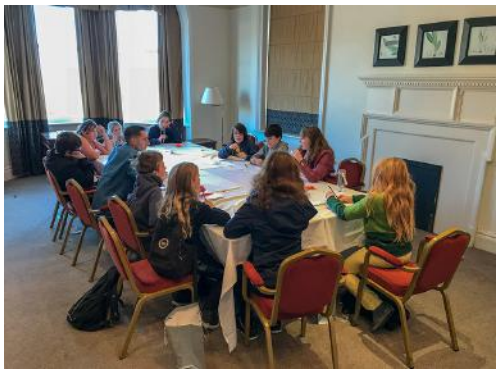
ANNUAL PATIENT DAY - FARNHAM

This year our annual patient day was held it in Farnham, an historic town, site of a battle in the 9th Century where the Anglo Saxons had a decisive victory over invading Danes. We were very fortunate to hold the meeting in the Mercure Hotel right in the centre of Farnham (it's not a very big town!) and not a Viking in sight. Our only sadness was that Furkhanda Haxton who has been at every HAE Patient Day since they started and is our 'front of house' when people arrive, could not be with us due to having injured her neck. I am glad to say she has recovered and is back to her usual self!

As in previous years, we held a social event the evening before the patient day, this was an opportunity for patients and family members to come together, meet new friends and get to know one another. It was a well attended event.

The patient day itself was led by Dr Patrick Yong, who is Consultant Immunologist at Frimley Park, nearby. Patrick is also the lead on the HAE Consultant Network, which is now functioning as a division of the UKPIN, the UK association of immunology doctors and nurses. The idea of the network is to have an HAE expert in each region who can be called upon to give advice where needed, also for the network to develop research projects, surveys and so on.

Our meeting had a truly international flavour as Michal Rutkowski, Vice President of HAEi and leader of the Polish affiliate, and Katie O'Sullivan who heads up the Eire affiliate, were both able to join us, as well as three other patients from Ireland.

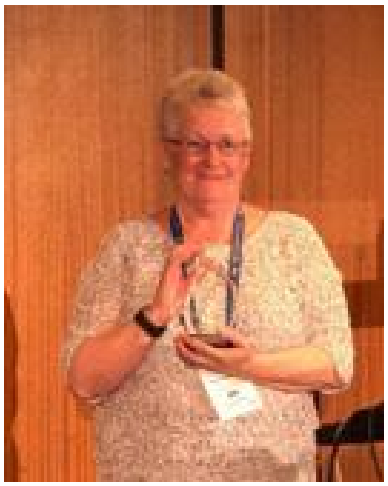


Our first presentation was by Dr Scott Hackett, one of our medical advisory panel, who gave us an overview of treatment for children. This was followed by a presentation from Alex Graham and Jack Cope, our Youth Ambassadors, two of the most inspirational young people anyone would care to meet and both of whom are going to Atlanta in 2019 to join in a HAEi Youth Advocacy Workshop….they gave us a taste of what young people can achieve. Alex and Jack then went off to lead the Youngsters track upstairs, with 14 delegates! We were so thrilled to have such a good response from younger people. We then didn't see much of the youngsters for the rest of the day, but they all seemed to enjoy the activities run by Alex and Jack and we have had some great ideas come out of this group.

Next up for the adults was Dr Sorena Kiani, giving a taste of the recent products undergoing clinical trials. He was lead investigator for the new product in the UK you have probably heard about, Lanadelumab, and he gave us a fascinating insight into how each product works and where and why one product may suit a patient more than another. Reinforcing the 'one size does not fit all' theme from our Scottish day. His session concluded with his clinical nurse specialist John Dempster giving some case histories of patients who have been involved in various clinical trials.

Ed Price, our Chair of Trustees, then gave us his patient experience and reinforced how vigilant one must be and not just dismiss an increase in attacks as 'oh, it's just my HAE' …….when so often there is an underlying cause that is nothing to

do with HAE. Just because you have HAE does not mean you cannot have other medical issues!



After a lovely lunch, the afternoon started with a double act of Christine Symons and Fran Ashworth, two of the most experienced HAE nurses one would ever meet. Indeed Fran had just come home from the European Congress on Immunodeficiencies having been awarded a Lifetime Achievement by the International Nursing Group for Immunodeficiencies! Very well done Fran!

Michal Rutkowski then gave us a World-Wide Perspective on HAE around the globe. Michal is one of our most popular speakers as people love hearing about what is going on with patients around the world and the various activities of HAE international.

The rest of our afternoon was filled by John Bell, a motivational speaker who gave us all some ways of turning our lives around to make them more fulfilling. He also put great importance on the benefits of family….which is what we have! We have our HAE family…..an invaluable support.

We did have a film crew filming during the Patient Day, so when it is finalised we will share it with you all.

PLASMA FRACTIONATION

You all know that your C1-INH (Berinert or Cinryze) is 'plasma derived' but what does that really mean? And how is it processed? I've put together some facts and figures for you.

Plasma is the liquid part of the blood - it is what is left when all the cells (red cells, white cells, platelets) have been removed. It is a pale straw colour and is water containing hormones, proteins and nutrients. Amongst the proteins are coagulation factors, immunoglobulins and C1-INH. Plasma is either 'recovered' from whole blood donations by removing the cells, or it is donated by a process called plasmaphoresis when only the plasma is taken from the donor. The advantage of this is that people can donate more often than when they give whole blood

- There are over 1,000 collection centres worldwide; 700 in USA, Canada and Europe
- Centres are stringently inspected and run to International standards
- Donors are carefully screened and tested to ensure that they are disease free
- Average 500ml per donation which is frozen to -35°C within 2 hours of donation
- Each bag of plasma has a unique bar-code
- Held in inventory for a minimum 60 days. Plasma can only be used if the donor returns within that time to make another donation. If they do not, their plasma is destroyed.

The plasma is then released to enter the 'fractionation process'. This consists of ways of separating out the proteins in various stages by use of precipitation, centrifugation, temperature differences in order to produce therapeutic treatments

- Mini-pools of plasma (about 24 donations) created and tested for possible viral contamination - any found positive is destroyed.
- Fractional pool is assembled; typically, 10,000-50,000 donations per fractionation 'run' - tested for viral contamination
- First products separated are the coagulations factors – for patients with haemophilia
- Then immunoglobulins
- C1-INH is further down the process.

The extracts are then further treated with various viral

reduction/inactivation processes such as solvent detergent treatment, pasturisation, chromatography. At least two of these processes must be used for a product to be licensed for treatment.

Finally the products are packaged into their vials. Each vial has a unique bar code which can trace it back through the whole process to the individuals whose plasma was used. So there is complete traceability from donor to finished product and back again.

The whole process takes between 7 and 12 months from donation to finished product. Each and every time you use your C1-INH, this represents 3 to 7 donations.

Sadly, at the present time UK plasma is not used for fractionation because of the risk of contamination with variant CJD. Currently there is no reliable way of removing 'prions' which are the contaminating proteins, so plasma used for fractionation is sourced from countries where there has never been any cases of 'mad cow disease'. Despite all the testing and incredibly high standards maintained by the plasma industry, there are still occasions when problems occur and this will result in an entire fractionation run being destroyed. This can then create shortages which may be only local or can be global.

Recombinant

The difficulties of the plasma supply was one reason Pharming felt there was a need to create an alternative independent of human blood. This is 'recombinant' C1-INH Ruconest.

The word 'recombinant' means that the product is initially designed in a laboratory. Nowadays, more than 400 different 'recombinant' therapies are used to treat many diseases including diabetes and haemophilia. The first recombinant product was insulin, created almost 4 decades ago (1982). To create recombinant C1 ('rhC1-INH'), cell lines are engineered to include the gene for C1-INH in order to produce rhC1INH. The rhC1-INH has the same protein composition when compared to C1-INH derived from human blood.

Briefly, rhC1-INH is produced as follows:

- The gene for C1-INH is injected into a cell that has the capability to divide (oocytes=early embryo stage). These cells now have the capability to produce rhC1INH and are transferred to a female mammal (Pharming uses rabbits for this).
- Once these rabbits start to produce milk, rhC1INH is produced as well.
- rhC1INH is then purified from the milk using technologies similar to plasma derived therapies.
- The purified product is also subjected to stringent safety procedures.
- Each dose of rhC1INH reduces the requirement for approximately 5-8 individual human blood/plasma donations.
- The collection of milk to the development of final product is 30 months.

QUARTERLY QUESTION - Do I need to flush with saline after my normal injection?

Our Nurse Advisors (John Dempster, Christine Symons and Lisa Smith) reply:
We never recommend patients either prime or flush their IV C1. The 'kit' is complete as it comes and this would just add another process to the procedure which is then open to more risk: infection, airbolus etc. There will be minimal loss of product as an extra amount is factored in when the vials are manufactured.

With our very best wishes for a merry Christmas and a happy, healthy New Year from Laura (CEO) & Rachel (EO) and the HAE UK team