DEC 2017

the platelet

JOURNAL OF THE ITP SUPPORT ASSOCIATION

itp
Immune
Thrombocytopenia

ITP SURVEY & RESEARCH NEWS, EVENTS, INFORMATION, & PLENTY MORE INSIDE

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Charity Registration No:1064480

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In the issue . . .

Following our plea for volunteers in the September Platelet we were delighted that Hayley Howie came forward to become our Social Platforms Manager and has already thrown herself into the task with enthusiasm. Unfortunately we have not yet found an Assistant Treasurer to work with Michael Levy, although at the Hammersmith Patient Day a lady whose name, sadly, I didn't get, mentioned that her husband was interested. Do please get in touch! The ITP accounts use Paxton charity software (which only runs on PC, not Mac). This post would suit an experienced treasurer or accountant. Although much work can be done from home an occasional visit to HQ would be desirable.

In this issue we are pleased to also welcome Dr Provan's replacement as ITP clinical lead at the Royal London Hospital, Dr Vickie McDonald (page 22). The survey of ITP Clinical Centres is still open until the end of December, so if you attend one of these Centres do fill in the form on page 20 if you haven't done so already (the on-line version is now closed). Details of another survey asking you to share your ITP experiences is advertised on page 5.

As always, we are grateful to the clinicians who have authored the interesting and educational medical items. Finally, although our charity remit is to provide information and support to those in the UK with ITP, we do participate in international ITP events and Derek Elston keeps us up to date with global news on page 24.

Shirley Watson

Front cover: Maddie Knight, Ross Olney, Trilly Whitby and Ben Wakeling (see pg 14)

Contents

The ITP Support Association is a registered charity which promotes and supports the general welfare of patients, and the families of patients, with Immune Thrombocytopenia. The Association aims to assist in funding approved ITP research projects, advancing the understanding and treatment of ITP in co-operation with the medical profession.

The ITP Support Association is primarily run by volunteers, with just one part-time paid worker. It is non profit-making and relies upon subscriptions, donations, bequests and fundraising by friends of the Associaton to enable its operation and to fund vital research into ITP. All donations are gratefully received and acknowledged.

News from the ITP office

by Mervyn Morgan, CEO

By the time you read this the festive season will be in full swing and we will all be looking forward to the New Year and (hopefully) warmer weather in the months to follow.

In the run-up to the festive season the office has been busy answering telephone and emails, with many enquiries from members who have questions about problematic ITP. Please remember signed up members also have access to the 'members' area of the website that contains lots of useful ITP related information.

This has also been the busiest time of the year for orders from the online shop, a big thank you to everyone who purchased items from us, every little helps.

Also many thanks to everyone who purchased (adopted) our Aware Bears from our online shop, it has been very sad to see them leave but we know they are all going to good homes. One is even travelling to Australia for Christmas in the sun.

I would like to say a big thank you to everyone for supporting the ITP Support Association over the past twelve months, without your time and commitment none of this would be possible.

Members continue to join the Association, but it is important that existing members remember to renew. With the new website it has never been so easy to renew, just go to www.itpsupport.org.uk, click on the 'Renew' Tab, login and follow the instructions. If you are a member and do not have your login details then please email me (mervyn@itpsupport.org.uk) and I will forward them to you. Likewise, if you cannot remember when your membership renewal is due then email me with your details and we will send you the information. We will also be emailing out to members (towards the end of the year) renewal reminders.

In the New Year, we will be running a campaign aimed at attracting more members, as membership of the ITP Support Association is also open to the friends and family of ITP sufferers. The larger our membership the more we can do in helping those in need. Don't forget to check out our website and social media pages (Facebook, Twitter, Instagram) for updates.

Finally, our social media platforms are now being managed by volunteer Hayley Howie who has been passed the baton from longtime friend of the charity, Anthony Heard. We thank Anthony for his hard work and dedication over many years and wish Hayley every success for the future.

The I-WISh Survey

Do you have
immune thrombocytopenia/ idiopathic thrombocytopenia (ITP)?

Would you like to share your experience of this disease?

What is the survey about?

The survey aims to understand how ITP impacts your life and how you cope with it on a daily basis.

Who can take part?

We are looking for patients who have a confirmed diagnosis of ITP and are over the age of 18, to complete a short online questionnaire.

Why is the survey being conducted?

The survey will be used to understand patients' perceptions towards their ITP, and how ITP can be managed more effectively. The survey results may also be published in scientific literature. All information will be anonymised.

How can I take part?

The survey will be ready for participation in December 2017 – January 2018. If you would like to take part in this industry-sponsored survey please contact **info@itpsupport.org.uk**.

ONC17-C105 / November 2017

American Perspective

Dr Cindy Neunert MD
Columbia University Medical Center


Dr Cindy Neunert

Splenectomy for Children with ITP - Similarities and Differences with Adults

We are fortunate that most children will have resolution of their ITP by 6 months and very few children go on to have ITP that lasts longer than 1 year. This makes children with ITP different from adults who are more likely to have the disease for a long time and fail many treatments. This realization that children are not simply "little adults" becomes very important when we think about how to treat ITP.

A big area of difference is with splenectomy, however some aspects of splenectomy are similar between adults and children. In children, just like in adults, splenectomy remains very good treatment with about 75-90% children having a sustained normal platelet count following splenectomy. For a long time it was also the only treatment we had for children with ITP who were not responding to first-line treatments like steroids and IVIg. Now we have additional treatments like rituximab and the thrombopoietin- receptor agonists (TPO-RAs), but often times, splenectomy remains the best option. The major side effect of splenectomy, increased risk for infections, is also no different between adults and children. The spleen is an important infection fighter and so splenectomy removes that defense and places the child at risk for infection. Just like adults, children who undergo splenectomy will need to be on life-long antibiotics, receive special vaccinations and seek medical attention each time they have a fever. Perhaps, children may also have less side effects with splenectomy because their bodies are healthier overall when they have the surgery. Lastly, just like in adults, splenectomy should always be urgently considered with life-threatening bleeding. Splenectomy provides the quickest and most reliable response in platelet count and therefore is always considered when bleeding needs to be stopped immediately.

So how are children and adults different? First, the chance that most children are likely to have their ITP go away even months to years after the diagnosis makes doctors want to wait longer before having a child undergo an irreversible procedure such as splenectomy. Secondly, when

we discuss side effects of splenectomy to a parent of a two-year old, talking about things that will occur life-long can seem very overwhelming. I have also found that sometimes parents find it very difficult to make a decision about surgery, which may be considered elective, on behalf of their child. Lastly, we now have additional medications that can be used in order to delay or avoid splenectomy, such as rituximab or the thrombopoietin receptor agonists. These have given us options and many parents wish to try these before their child has surgery.

So when should splenectomy be considered in children with ITP? The 2011 American Society of Hematology (ASH) guidelines recommended splenectomy for children with ITP of at least 3 months duration with significant bleeding, lack of responsiveness to other therapies and/or need for improved quality of life. It was, however, suggested that given the high likelihood of remission in children that splenectomy be delayed for 12 months unless the child needs more urgent treatment because of bleeding. When talking to a child or parent of a child who has failed to respond to first-line therapy the following factors should be determined: 1) How much bleeding is the child having 2) What is the impact of ITP on the child and family's quality of life 3) what other treatments has the child had and how did they with them 4) what is the family's biggest concern 5) what are the overall goals of treatment. This allows the physician to then discuss each treatment option, including splenectomy, with respect to each of these factors. This allows for determination of a treatment option that might best align with the needs and goals of the family. If the discussion favors splenectomy, then more time should be spent evaluating if this is truly the best option for the individual child. Physicians should consider the age of the child, how long they have had ITP, the family's experience of living with the condition, and the goals of treatment to make sure that splenectomy is still the most reasonable treatment option.

Splenectomy remains very good treatment with ITP, especially in the setting of significant bleeding, parent/child who desire "curative" treatment with long lasting results, and children with long-standing disease. There is no one right treatment for children who have ongoing ITP, but understanding the options and the goals of the family can assist with ensuring the best option is chosen. Sometimes this is splenectomy!

I have seen many (approximately 50 children) with ITP during my last 3 years at Columbia University Hospital. Fortunately most have recovered very quickly from their ITP. For those that have not responded a handful have needed additional treatment. Two such children underwent splenectomy (one for severe bleeding and one for desired remission). All responded with normal platelet counts, which have remained normal. So splenectomy for children with ITP is rarely done, but it is a very effective treatment when it's needed.

Dr John Grainger adds a UK perspective on splenectomy in childhood ITP overleaf . . .

.... and a UK Perspective

In the UK we would consider splenectomy in children a third line option, i.e. only to be considered when other medical treatments have been exhausted. We would recommend deferring splenectomy until after at least 12 months disease and preferable until the child was at least five years of age. Splenectomy involves invasive surgery, with no guarantee of success, and a risk of bleeding and other surgical complications. Long term splenectomy is associated with risks of overwhelming infection and blood clots even in children. These risks persist lifelong following splenectomy. We would remain hopeful that the ITP will remit spontaneously without splenectomy and in children with chronic ITP and frequent and/or severe bleeding I would use TPO-RA such as Romiplostim or Eltrombopag to support the platelet count to a safe level whilst awaiting remission of the ITP.

I see approximately 50 new patients per year and have performed 2 splenectomy in the last 5 years. Both have been successful and were performed in children with prior life threatening bleeding, and the children were unresponsive or intolerant of TPO-RA. So in conclusion it can be an effective treatment if other options fail.

Dr John Grainger
Consultant Paediatric Haematologist
Manchester Children's Hospital

Is your membership subscription due?

In the past our annual subscription reminders were sent out with the December Platelet as membership lasted a calendar year for administrative ease. However, now the majority of members pay by direct debit or standing order we will no longer be including a membership renewal form with the December issue. All subscriptions will now cover a 12 month period from whenever each individual's membership began, and those who pay by cheque will receive a reminder from the office when it becomes due. To save postage reminders will be sent by email where possible.

We greatly value your membership,

and hope we can rely on your continued support.

Sepsis Awareness

In September the Secretary of State for Health, Jeremy Hunt, announced that new measures were being taken to tackle sepsis in the UK as there are about 123,000 cases of sepsis per year in England alone. Back in December 2016 a special campaign was launched to alert parents on how to spot sepsis in children but a special effort is now being made to raise awareness with pharmacists and care home staff about the condition.

The NHS Choices website states that people most at risk of sepsis include those:

- with a medical condition or receiving medical treatment that weakens their immune system
- who are already in hospital with a serious illness
- who are very young or very old
- who have just had surgery or who have wounds or injuries as a result of an accident

The Patients' Association recently reported that sepsis currently kills an estimated 37,000 people in England each year and is a huge cost on the NHS. The most effective way to tackle sepsis is to recognise the symptoms quickly and treat it as soon as possible. Without quick treatment, sepsis can lead to organ failure and death.

Sepsis, also known as blood poisoning, is usually caused by bacterial infection, although viral or fungal infections can also be a cause. For ITP patients it is important to be aware about the risk of sepsis if you have no spleen or are taking immunosuppressants. Sepsis is treatable if it is identified and treated quickly, and in most cases leads to a full recovery with no lasting problems.

Early symptoms of sepsis may include:

- a high temperature (fever) or low body temperature
- chills and shivering
- a fast heartbeat
- fast breathing

As sepsis progresses symptoms may include

- extreme pain or general discomfort
- pale or discoloured skin
- sleepiness or confusion
- feeling very ill

If you are in an 'at risk' category, or have had a recent infection or injury, and suspect you have early signs of sepsis contact NHS 111 or your GP urgently. If you think you or someone in your care has more advanced sepsis, go straight to A&E or call 999. To find out more about sepsis symptoms, tests and treatment go to www.nhs.uk/conditions/sepsis/

The FLIGHT trial

by Dr Charlotte Bradbury

ITP is an autoimmune condition that can cause bleeding or bruising due to a low platelet count. It is also increasingly recognised that patients with ITP may suffer other problems such as severe tiredness (fatigue), other physical, psychological and socioeconomic impacts which combine to reduce quality of life. For patients who need treatment, the commonest first treatment is high dose corticosteroids (such as prednisolone or dexamethasone) which improves the platelet count in the majority of patients (approximately 80%) and works quite quickly. The response to corticosteroid is useful to confirm the diagnosis of ITP and reduce bleeding and bruising within a short timeframe. However, for some patients the corticosteroids don't work and in the majority of other patients, the illness comes back when the corticosteroid dose is reduced or stopped with only 20% of patients staying well long term with this approach. In addition, nearly all patients suffer one or more side effect with corticosteroids such as mood swings, weight gain, difficulty sleeping, high blood pressure, stomach irritation, bone thinning and diabetes. Therefore, this standard approach is unsatisfactory but has been unchallenged for decades due to a lack of research to test new approaches. A group of ITP patients from Bristol reported reducing the overall amount of corticosteroid and avoiding the trial and error approach to treatment (less relapses) were important priorities for research from their perspective.

Mycophenolate (MMF) is often used as the next treatment for ITP and works well in the majority of patients (effective in 50-80% of patients) with minimal side effects. Unfortunately, it can take about 2 months to work and during this time patients often need more corticosteroid (with more side effects) to keep their platelet count at a safe level. Patients who have "failed" first line corticosteroid (the majority) continue to be at risk of bleeding, bruising, fatigue, need frequent blood tests and hospital appointments and are often unable to continue their normal activities. It seems logical to try starting the MMF earlier (at the beginning with corticosteroid) so that it is working when the corticosteroids stop, aiming to prevent ITP relapse and need for further treatment including corticosteroid.

The FLIGHT trial is a clinical trial for patients in the UK with ITP who need to start their first treatment. The trial is independently funded by the National Institute for Health Research (NIHR, the research part of the NHS) under its Research for patient Benefit (RfPB) programme. The trial compares standard first line treatment with corticosteroid only to a new approach of corticosteroid combined with MMF.

The trial is supported by the UK ITP forum and aims to recruit 120 patients. Over 50 hospitals across the UK have asked to take part in this trial and 9 centres are already open for recruitment (the trial

opened 25th October 2017). In addition to measuring standard outcomes of platelet count response, relapse, bleeding and need for further treatments, the trial will also collect patient oriented outcome data (questionnaires to capture fatigue, quality of life, time off work and financial cost etc).

Within the trial, there will be an option for patients to have 2 additional blood tests. These blood samples will be taken at the same time as other bloods are done and sent to Bristol University where they will be used for multiple ITP science research projects. These projects include improving the understanding of the cellular and molecular basis of ITP and the development of blood tests to predict patient outcomes such as treatment response, duration of ITP, relapse, bleeding and fatigue.

We are very grateful to the members of the UK ITP forum, ITP centres and trial management team for supporting and delivering this trial. We would also like to thank the group of patients who have offered to continue to meet with myself throughout the trial to advise us on patient aspects. We look forward to updating you with results in the future!

date for

ITP Patient Day

your diary

on Friday 27th April

The ITP Support Association and ITP expert teams from hospitals in Bristol and Wales invite ITP patients and parents of ITP children to attend a

Bristol and Wales ITP Patient Day

This informal free event (at a venue yet to be arranged) will provide an excellent opportunity to share experiences and develop friendships

The event will include a discussion session in which you can put your questions to the expert clinicians:

Dr Charlotte Bradbury (Bristol University Hospital)
Dr Rachel Rayment (University Hospital of Wales)
Dr Emma Phillips (Bristol Children's Hospital)
Dr Philip Connor (Children's Hospital of Wales)

Tickets will be available from March 1st 2018
or you can register your interest now by contacting ITP HQ (contact details on pg 2)

Hello Hayley!

Those of you who check out our social media, in particular our instagram page, will know that Hayley Howie has been posting there on our behalf. When Anthony Heard stepped down as our social networks manager Hayley very kindly agreed to take on this volunteer post and is now looking after all the ITP Support Associations social media platforms. We asked Hayley to introduce herself to Platelet readers.

Being diagnosed with ITP in 2008, I was lucky enough to have a consultant who had years of experience behind her, and after a few years of treatment, I am now in remission.

Working in public relations, I have provided campaigns for Speedo & FIFA for the World Cup, in the Middle East. I have been running the ITP Instagram page for some time now, but after having my baby last year I decided it was time to take a step back from work and focus on my family. This has also given me the opportunity to put more time into the ITP Support Association. I am now running all our social media platforms, and love hearing about all your fundraising and other stories through your photographs online.

Please don't hesitate to contact us through social media, with any photographs, ideas or stories.

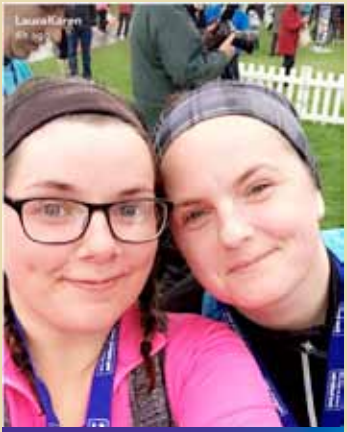
Hayley Howie

Can you hold an event to raise funds for ITP? Big or small, all contributions are very much appreciated and help us to fund much needed research.

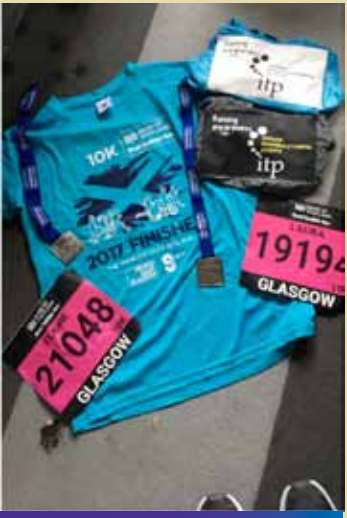
Fantastic Fundraisers!

We start with our hearty congratulations to **Laura Thomson** who has raised a huge £1,300 for the ITP Support Association! Laura's father, Neil Thomson (a father of five girls) has been suffering from ITP since 2011.

On Sunday 1st October Laura ran in the Great Scottish Run in Glasgow with her mother-in-law **Elaine Gibson** to help raise funds for ITP. Unfortunately one week prior to the run Laura took a tumble and dislocated her right shoulder, but did that stop her taking part? No! Like a true star she took part in the run and both Laura and Elaine completed the run with a time of 1 hour 16 minutes. The 10k course crosses the River Clyde four times and passes The Hydro, The Finnieston Crane, Pacific Quay and McLellan Arch, and ends at Glasgow Green. To keep the runners spirits up along the way they are entertained by a choir, bagpipers (of course!) a band of drummers and charity cheering points. Laura told us, "We crossed the finishing line with the biggest smile, the worst jelly legs and that overwhelming feeling of pride and achievement".



Laura Thomson and Elaine Gibson



Laura & Elaine's running gear

Prior to the Great Scottish Run (in September), Laura held a Quiz Night and a Bingo Night, these alone helped to raise an amazing £900.

Also in Scotland, **The Learig Orchestra** in Aberdeen dedicated their November concert to the memory of **Richard Vote**, an ITP sufferer and stalwart of the orchestra, who sadly passed away last year. They most generously donated the £500 from ticket sales to our charity and sent a further £80 raised from donations and selling ITP Christmas cards. The concert was held in Midstocket Church on the 18th November and the programme included Beethoven's Overture to "Consecration of the House", Delibe's suite "Sylvia", Schumann's third symphony, The "Rheinische", and there was a rousing performance of "76 Trombones" which was specially arranged by their conductor Ronnie Gibson as a tribute to Dick Vote.

Maddie, Trilly, Ross and Ben

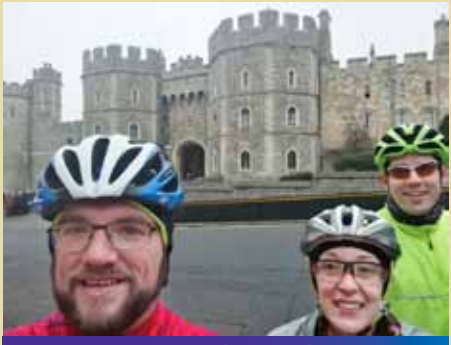
Ross Olney raised £676 from a 100km cycle ride with the help of Lea Barn Stables in Wexham, followed by a cake and coffee sale. Ross told us that this was the first time he had organised such an event, and the ITP Support Association was his chosen charity as we had helped his parents through a tough time in 2016 when his father was diagnosed with ITP. Sadly, due to further complications, his Dad passed away at the end of November last year.

Starting out

Ross thoughtfully sent us a summary of the adventure, along with the photos (in which you can see what a miserable day it was by the rain on their glasses!) Ross explained "The three of us Ross Olney (in red), Ben Wakeling (in yellow), and Maddie Knight, are all cyclists generally, so were keen to test our distance capabilities. Saying that I have done this distance only a couple of times before, Ben has completed an Iron Man race so is no stranger to time in the saddle, but for Maddie it was the furthest she had ever ridden so we are all proud to have accompanied her on breaking the 100km barrier. The ride went well, the weather on the other hand was not so well. . .

Eton to Windsor Bridge

On 18th November we set off from Lea Barn stables, in Wexham, at 09:40ish on a cold sunny day, this quickly changed and by 11am it was raining and stayed wet for the rest of the day. The weather aside, it was a very pleasant ride in great company.



Passing Windsor Castle

We fulfilled our aim to take a photo at Windsor castle 20mile/30km into the ride, and at the George III Copper horse statue which is roughly at the half way mark. We lunched at Ascot and then took a route

Half way at the Bronze Horse

via reservoirs near to Heathrow airport up to Ivor Heath and back to the stables without a stop, due to weather and temperature. In all we covered 64.1 miles in 4hrs 40min.

Made it!

Dandy looks out to say well done

Whilst we were hard at work Trilly Whitby, the owner of Lea Barn Stables, ran a cake and coffee day, with help from all the ladies at the stables, to help raise funds through the riders at the stables".

We congratulate Ross, Ben and Maddie on completing this gruelling ride, and send our heartfelt thanks to them, and to Trilly and her helpers for their efforts in raising funds and awareness.

Sue Swinchin's 2½ year old granddaughter was diagnosed with ITP and Non-Hodgkins lymphoma in March of this year. Sue explained "We would like to raise money for several charities in appreciation of the help and support Isobel and her parents received." Sue and a group of friends took part in a 7 day sponsored walk of 127 miles (wow!) along the Leeds to Liverpool Canal. This is the longest canal in Northern England and it passes through 91 locks with the summit level at 487 feet. The towpath crosses The Pennines, an area of beautiful scenery, stretching from the banks of the Mersey in Liverpool to the city centre of Leeds. Sue held a fundraising evening on top and raised a tremendous £509 for ITP.

Yes, definitely 127 miles!

We are very pleased to record our sincere gratitude to the following five supporters who have involved colleagues and businesses in their fundraising efforts:

A fantastic £728 was raised during recent fundraising events at the Howden Group. Ms N. Irvine informed us "Your charity was nominated by our colleagues and we take great pleasure in donating this sum to you." We appreciate the efforts of everyone who was involved, and particularly the unnamed person who nominated The ITP Support Association.

Sue Swinchin and family

Antony Lamberton, whose son Henry has ITP, held a charity day at Car Care Plan's head office which raised an excellent £230 for our cause.

Rita Hobson, Shirley Watson and Patti Pitt

Atlas Trading Ltd of Ashton under Lyne raised a further £102 from their ITP collection tin, who kindly support our charity as Jimmy, son of the Managing Director Simon Gledhill has ITP.

Karen Heath sent £56 kindly raised from staff of the Co-op Office in Stafford who held a dress down day.

Anthony Budding told us of his daughter Skyla Budding, aged 5, who has ITP and as a result suffered spontaneous intracranial haemorrhage. He said "She has lost her left field vision but has recovered relatively well although her platelet levels remain low and fairly unresponsive to treatment. My colleagues at SSE Business Energy held a Halloween quiz and decided to donate to ITP in Skyla's name." We send our thanks to Anthony and his colleagues for the £50 raised, and our very best wishes go to Skyla.

An additional donation of £350 has been received from local neighbour Patti Pitt whose Heartsease dance group had nominated ITP as their charity of the year. Shirley attended Heartsease's AGM in November and after giving a short talk about ITP and the work of the Association she was presented with a cheque by the dance group's treasurer, Rita Hobson and chairman, Patti Pitt, both of whom have been Shirley's friends for over 40 years.

Shirley ran a coffee morning in the Steven Sims room at ITP HQ in early September which raised £182. Our office services clerk Natalie Baylis and her daughter Poppy did a sterling job serving tea, coffee and home made cakes to everyone, many of whom had second and even third helpings.

We thank Stuart Godfrey for his £20 donation following a talk at the Hope Baptist Church Sisterhood. We are grateful to the congregation for their continued support.

Finally, we send our deepest sympathy to the family and friends of Esme Miller, with thanks for the £20 received in her memory.

The ITP coffee morning

Letterbox

Dear Shirley,

I am 69 and have had ITP since 2010. I was treated with Prednisolone which worked for a while and then Azathioprine to which I had a poor response. Then followed Dexamethasone, but I was unable to tolerate this and so had intravenous Rituximab.

By November 2013 my platelets were still very low with bruising and bleeding which is when I began Romiplostim.

It was a pleasure to meet you at the Convention in London in May, the first one I have attended. I was very interested in all of the presentations but particularly the project looking into a possible link between gut bacteria and auto immune disease. Following a chat with the professor in the interval I decided to take Yakult daily to see if it had an impact.

My treatment at this stage was 500 micro grams of Romiplostim every 5 days and this maintained a count of 20-40 and I had no symptoms at this level of platelets.

At my next appointment in July my count had risen to 78, so the haematologist changed my Romiplostim to 500micrograms every 7 days which has made my life a lot easier and obviously less side effects to manage.

My count has continued to improve and at my appointment today it was 122. I am continuing with the same dose for the time being and still take the Yakult every day.

At some stage when my haematologist and myself are confident that this is a permanent trend and as he put it ,we are both feeling brave, we need to experiment with a reduced dose to see if I can be weaned off Romiplostim and still maintain a "safe" platelet count.

I thought you would like to know as you are always interested in individual member's cases and taking the probiotic was the only thing I changed. So maybe there is a connection between a better gut flora and auto immune diseases

Kind regards

Linda Willis

Thank you for sharing this with us, Linda. Professor Newland reported in a previous issue of The Platelet that about a quarter of patients on TPO drugs are going into remission, so that may be another possibility for your improvement. However, whether or not your increased platelet count is due to Yakult or Romiplostim it is excellent news and will offer encouragement to others.

Do you have an interesting experience to report or case history you would like to share with other readers?

If so please send it for the attention of The Platelet Editor to info@itpsupport.org.uk

Bleeding Disorders

SURVEY UPDATE

by Derek Elston

Firstly a big THANK YOU to our members who contributed to the Bleeding Disorders Survey which closed in September. In all some 500 replies were received from patients, carers and medical personnel alike. The majority of the replies related to those concerned with ITP or haemophilia. Sadly, relative to all conditions, many submitted questions relating to their respective situation and treatment, but did not submit ideas or questions that could be related to research projects. Surprisingly, many respondents were unaware of research that had already been undertaken, the results and findings of which are already in the public domain. This indicates to me, there is still much to be achieved in making patients, carers and even medics, aware of what has and is happening in research and treatments for bleeding disorders.

Notwithstanding this, there are many items proposed for research which are common across the two main conditions of ITP and haemophilia, apart from specific condition related questions and ideas. These are now subject to review by the steering group and the top ten questions considered by them, will be referred for consideration by the NIHR (National Institute for Health Research). Those not in the top 10, will be made available to pharmaceutical companies and other research establishments for consideration.

The steering group is composed of eminent haematologists with varying medical interests, patient group representatives, relatives and carers of patients, and physiotherapists. The broad-spectrum of expertise was considered necessary from the outset and has proved extremely beneficial.

Below you will find an analytical abstract of the result of the survey prepared by HCD Economics based on the first initial submissions. This will be followed in due course by a final analysis of the survey results. You will appreciate, the review of the submissions will take time to consider. We are however hopeful that this will be completed by the summer of 2018.

Abstract

RESEARCH PRIORITIES IN BLEEDING DISORDERS

A James Lind Alliance Priority Setting Partnership

Composition of the steering group

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Introduction and objective

The James Lind Alliance (JLA) is a non-profit making initiative established in 2004. It brings patients, carers, and clinicians together in Priority Setting Partnerships (PSPs) to identify and prioritise the top 10 'uncertainties', or unanswered questions, in identified diseases/conditions. The JLA Bleeding Disorder PSP is an independent and evidence-based project funded by the United Kingdom Haemophilia Centre Doctors' Organisation (UKHCDO) with the purpose of identifying uncertainties around diagnosis, treatment, and supportive care relating to inherited and acquired bleeding disorders.

Materials and methods

As per the JLA priority setting process, current and previous patients with bleeding disorders, relatives, carers, and health professionals were surveyed to elicit unanswered questions regarding diagnosis, treatment and care. Respondents were targeted via social media, mailing lists, medical conferences, and the websites and forums of patient organisations, and responses gathered between April and September 2017. The elicited questions are currently undergoing review for relevance in line with the agreed scope of the PSP, to be followed by categorisation into topic-based subgroups.

Results

Two hundred and thirteen initial surveys were returned. Approximately one-third of respondents (n=68; 32%) identified themselves as a healthcare professional; the same number (n=68; 32%) described themselves as someone who had experienced excessive bleeding in the past, or who was currently diagnosed with a bleeding disorder. The remaining respondents (n=77; 36%) were relatives or carers of someone with a bleeding disorder, representatives of organisations supporting patients with bleeding disorders, or a combination of the above.

Conclusions

Preliminary review of survey responses suggests that submitted questions cover a wide portfolio of research (including causes and genetics of bleeding disorders, diagnostics and symptoms, choice of medication, and future treatment options.) The next stage will see the generation of structured questions that research should address based on the survey responses, following checks to ensure that the questions posed are currently unanswered. A shortlist of research questions, chosen by the steering group, will then be reviewed at a final consensus meeting. The final list of questions should prove to be an invaluable resource to inform the prioritisation and funding of future research into bleeding disorders.

Seen at an ITP Clinical Centre?

HAVE YOU COMPLETED OUR FEEDBACK SURVEY?

Last quarter we invited patients seen at an ITP Clinical Centre (listed below) to complete a survey to help us assess how successfully the Centres are meeting patient needs. ITP specialists at these Centres have encouraged their patients to take part and we have received 125 responses to date. Early results show how highly the vast majority of you rate these Centres, especially at this time when the NHS is under so much pressure.

We have closed our on-line survey now, but you can still participate up to the end of December by completing the paper survey on the opposite page. ITP can be a difficult disease and as we recognise that it is more likely to be patients with problematic ITP who ask for a referral, we are interested in knowing about your overall experience of attending your ITP Centre rather than how successful they has been in raising your platelet count.

Please send completed surveys to the ITP office (by post to The Platelet Mission, Kimbolton Road, Bolnhurst, Beds, MK44 2EW or email a pdf scan to info@itpsupport.org.uk). Your answers will be kept confidential – only the result summaries will be shared with ITP Clinical Centres to help them continue to strive towards the highest of standards.

ITP CLINICAL CENTRES

The Royal London Hospital
Manchester Royal Infirmary
QE Hospital, Birmingham
Glasgow Royal Infirmary
East Kent Hospital Trust
University Hospital of Wales, Cardiff
Belfast City Hospital Trust
University Hospital of Coventry & Warwick
University Hospital of Leicester
University Hospitals Southampton
Aberdeen Royal Infirmary
Bristol Royal Infirmary
St George's Hospital, Tooting
Guys and St Thomas Hospital
Norfolk & Norwich Hospital
St James University Hospital, Leeds
Oxford University Hospital
Newcastle upon Tyne Trust
UCLH (University College London Hospital)
Addenbrookes Hospital, Cambridge
Hammersmith Hospital
Derriford Hospital
Royal Manchester Children's Hospital
Evelina London Children's Hospital
Birmingham Children's Hospital
Edinburgh Children's Hospital
Children's Hospital for Wales
Sheffield Children's Hospital
Leeds Children's Hospital
Belfast Children's Hospital
Royal Aberdeen Children's Hospital
Great Ormond Street Hospital
Bristol Royal Hospital for Children

ITP CLINICAL CENTRE FEEDBACK SURVEY

Please complete this survey if you have attended an ITP Clinical Centre (listed opposite) any time since September 2011.

If you've been seen at more than one ITP Clinical Centre please use a separate form for each.

1. *Centre Name* _____

2. Did you specifically ask to be referred to this Centre or the doctor running the Centre? *Yes / No*

3. When did you last visit the Centre? *(month/year)* _____

4. Are you completing this survey as an ITP patient or the parent of a child with ITP? *Patient / Parent of child*

5. What is the name of the doctor in charge of your care? _____

6. Does your doctor have a treatment plan for you? *Yes / No / Don't know*

7. Have you ever had conflicting advice from different doctors in the team? *Often / Occasionally / Never*

8. Is there an ITP specialist nurse in the clinic? *Yes / No / Don't know*

9. Are the staff friendly, polite and attentive? *Yes / No*

10. Are you given time to ask questions or express concerns? *Yes / No*

11. Do the team attempt to answer your questions (bearing in mind much is unknown in ITP) *Yes / No*

12. Are you happy with your doctor's efforts to manage your ITP? *Yes / No*

13. Have you ever felt pushed into having an ITP treatment you didn't want? *Yes / No*

14. Have you ever been refused an ITP treatment you did want? *Yes / No*

15. Have you been given clear instructions about any medications and know that you need to take them as prescribed. *Yes / No*

16. Were you told about any possible side effects from your medication? *(Yes / No)*

17. Have you been offered the opportunity to take part in any clinical trials or studies? *Yes / No*

18. Have you been given a number to ring in case of emergencies or urgent enquiries? *Yes / No*

19. Has the overall quality of care met your expectations? *Yes / No*

20. Were you offered information about the ITP Support Association during your first visit? *Yes / No*

21. If you were referred to the Centre from your local hospital please list up to 3 reasons why you prefer the ITP Centre:-

1. _____

2. _____

3. _____

22. Do you have any improvement suggestions for your ITP Centre?

_____

_____

23. On a scale of 1 to 10 (1 = very poor, 10 = first class) please rate your ITP Clinical Centre? _____

Any comments?

News & Views

New clinical lead at the Royal London

We extend a very warm welcome to Dr Vickie McDonald having taken up her new role at the Royal London, and we invited her to introduce herself to Platelet readers.

"I am delighted to have joined the team at the Royal London Hospital this year as their clinical lead for ITP. I feel very lucky to have been given this opportunity. Following on from Dr Provan after his retirement I have big shoes to fill but I am supported by a great team, not least by Louise Taylor (ITP clinical specialist nurse), and am proud to be joining them.

I am equally pleased to announce that Dr Provan will be joining us for some clinical sessions as we reconfigure the ITP services and develop the service not only for the local population but also for those travelling from further afield to see us. It's great to see several important trials open for ITP patients at the Royal London and look forward to being able to understand responses and outcomes with novel approaches to treatment. In addition, the Registry continues to recruit from over 89 centres in the UK and we are pleased to see increasing numbers of patients joining us. We look forward to sharing the results of several of our studies during the course of 2018.

So a final thanks to the team at the Royal London, the UK ITP Forum and the ITP Support Association for being so welcoming and I look forward to working closely with you all."

NPlate for children

After five studies evaluating the safety and efficacy of Nplate in children with ITP Amgen's Nplate has been recommended for EU approval for the treatment of chronic ITP in symptomatic children. Many children with ITP are managed by a 'watch and wait' strategy until their ITP goes into remission, but for those with a chronic severe condition this will be a useful addition to the small armoury of available treatments.

Patient day in Manchester & Leeds

We are planning to hold a patient day with the adult and paediatric ITP teams from Leeds and Manchester ITP Clinical Centres in the Spring 2018. Details are yet to be arranged but will be advertised on our website and in the March issue of the Platelet. Please email info@itpsupport.org.uk if you would live in the area and would to be informed about this free event.

Two new ITP booklets published

The long awaited Fatigue in ITP booklet has now been published and can be ordered free of charge by members. A book written especially for children with chronic ITP is also newly available, and follows on from those written for newly diagnosed children. It is beautifully illustrated by 15 year old Harriet Ellis (who has had ITP for 3 years) and will contribute towards her achieving the Duke of Edinburgh's Silver Award.

ITP Research Study

Research Study for adults with ITP

A number of UK ITP Centres are conducting a clinical research study of an investigational drug (PRTX-100) in the treatment of chronic or persistent Immune Thrombocytopenia (ITP). This study is being done to see if PRTX-100 will interfere with the immune response related to ITP and prevent your body from destroying your platelets and to evaluate its safety in individuals with ITP. If you received treatment for ITP and still have platelet counts below 50, or are on no treatment and have a platelet count less than 30, you may be eligible for the trial.

To be eligible for the study you must also:

- Be 18 years of age or older
- Have previously received treatment with one other standard ITP treatment

The following ITP Centres are open for the study:
Hammersmith Hospital (Dr Nichola Cooper – National Co-ordinator for the study)
Royal London Hospital (Prof. Adrian Newland)
Derriford Hospital (Dr Tim Nokes)
University College London Hospital (Dr Marie Scully)
St George's Hospital (Dr Steve Austin)
University Hospital, Southampton (Dr Rashid Kazmi)
Guy's and St Thomas's Hospital (Dr Susan Robinson)

How to register on the study

If you believe you meet the eligibility criteria stated above and are at a participating hospital you should speak to the doctor you usually see. If you are not at a participating hospital you will need to ask your GP or consultant to refer you to the Centre of your choice. In the first instance you may wish to find out what the trial entails by contacting Camilia Vladescu (Clinical Trials Co-ordinator at Hammersmith Hospital) by phone or email and she will talk you through the trial. If you then decide that you would like to take part, you can asked to be referred.

For further information, please contact Camilia Vladescu
tel: 020 3313 4306 or email Camelia.Vladescu@nhs.net

TEXT GIVING

Don't forget that you can make a donation through **Justtextgiving** at your mobile operator's standard rate by texting the message ITPA22 and the amount (£1 – £5 or £10) to 70070.

The Association will receive 100% of your donation which can be increased by adding Gift Aid.

ITP International update

by Derek Elston

I am sure everybody is well aware of our involvement overseas. This is an update of what has been happening over recent months and a look ahead.

As you may remember from previous reports, the ITP International Alliance was set up two years ago following a period of collaboration between various groups in other countries. The prime organisers in the Alliance are ourselves and the PDSA in America. We now have a total affiliated membership of 29 ITP groups around the world, from China, to Europe; from India to North and South America – truly international. We currently have interest in Australia from a haematologist who specialises in ITP. I met this gentleman in Portugal at an EHA scientific conference on ITP where I was making a presentation on patient involvement to many of the leading specialists in ITP from Europe and the USA and beyond. Dr Wan Chee (an avid Liverpool supporter) is wanting to follow our examples and start a group which would be based in Adelaide. We communicate on a regular basis and it is hoped he will be visited next year by a board member from PDSA who will be there to give practical advice and guidance. Why not look at the International Alliance web site at www.globalitp.org.

Also on the web site is a report and photographs on the Global ITP Awareness week in September. The website received nearly 4000 hits during this international event. Visit http://www.globalitp.org/index.php/global-awareness-week-photos

Much of the promotion and communication is handled by Nancy Potthast who is based in New York. She is the PDSA communication and marketing director. We work closely with Nancy and hold regular skype calls with her and Caroline Kruse, the PDSA director.

As you can imagine, international communication is not straight forward and great reliance is placed on emails. We have however tried one international teleconference. On that occasion, we had several groups live on line, including representatives from China. The language used was English but even so, conversational English is different to technical and medical English. Thankfully, everybody participating, had patience and helped wherever necessary. The agenda on that occasion was not complicated, more of a general nature, but nonetheless informative and I hope, helpful for the new members to this group. Another meeting will undoubtedly be arranged in the coming months.

The last meeting of the Alliance was held in Melksham, near Bristol and attended by many other groups from Europe, one representative from India and two from the USA. A real international gathering which afforded us

all time to meet, talk and learn about the problems other countries have, especially the developing nations. We should have had a representative from Argentina but that did not materialise. The next meeting was planned to be hosted by the Dutch group but sadly, due to problems in Holland, the meeting was cancelled. However, there is a meeting planned for next year here again in the UK, in all probability in the autumn. As with the last meeting in Melksham, it is planned to precede our UK convention which will allow our visitors the opportunity to attend both. This will be beneficial both in terms of education and hopefully help with jet lag after long flights. Preliminary discussions have been held with pharmaceutical companies and funding is in the course of being sought. Although this international meeting may be months away, we are even now trying to establish who will be able to attend and provide an interesting and informative programme for them when they are here. We anticipate at least one full day for the conference.

Patient Mentors

a listening ear . . . . . .

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Shirley would tell you, the ITP Support Association was the first ITP patient group to be formed worldwide AND the first group to become involved internationally, particularly in Europe. Since those early days, this involvement has grown and, as with many different patient organisations for many different conditions, patient advocacy is becoming more involved, demanding and is recognised internationally from the vast majority of medical practitioners. The Alliance recognises this fact and between the ITPSA and PDSA, we are represented and attend, the two major international haematology associations' conferences for medics, on both sides of the Atlantic. That is the American Society of Haematologists (ASH) in America and the European Haematology Association (EHA) in Europe. Both these organisations hold annual conferences and are attended by haematologists from the world over. EHA's usual attendance figure is circa 10,000 delegates and ASH, 25,000. These are not insignificant numbers and affords the opportunity for medics, pharmaceutical companies and, importantly from our viewpoint, patient advocates, the opportunity to network and meet to discuss, learn and equally important, acquaint medics with patient views. It is very evident that the medical profession has over recent years become very conscious of patient's knowledge and the value of their contribution both during consultation and also collectively from a patient group perspective. It is

certainly the intention of the EHA patient workgroup to continue this development.

EHA has over the last five years, increasingly involved the patient advocates in their program. This year in Madrid, the patient advocates were given more opportunity to represent patients and their views than ever before. There were sessions specifically organised by various patient groups to which any delegate could attend. This has already been reported earlier this year in the Platelet. However, since that conference, the patient advocates workgroup or board, have been engaged with the EHA board of management to develop the patient's advocacy further. This will involve a properly constituted board for the advocates with stronger and closer connections to the main board. This will no doubt require more time to be spent on EHA matters and the likelihood of attending meetings in The Hague. They have also appointed a new director for this purpose, Robin Doeswijk. At the last EHA meeting in Madrid, Robin spent a considerable amount of time talking to all the patient representatives gathering information on each group. This has resulted in firm proposals being presented to the EHA management board for the further development of the patients' involvement. Inevitably, due to the sheer number of haematological conditions, malignant and non-malignant, it will not be possible for all to be represented within the workgroup. We are very fortunate to hold a seat on the patient advocates workgroup or board and represent ITP throughout Europe.

After the last conference, EHA formulated a European declaration requiring specialist haematologists to undergo a minimum of 5 years education before being eligible for consultancy status. This would bring many of the EU countries in line with the UK and other developed medical training requirements in some other EU countries. In this connection, all the patient advocacies involved with EHA have been asked to put their names in support of this declaration. This emphasises the progress of the advocates within the medical community of EHA.

All this is relative to the UK, regardless of Brexit. In my opinion, the UK will remain one of the leading lights in medicine in the world, regardless of whether we are in the EU or outside. Our connections with Europe may not be political, but will remain for the benefit of all here in the UK especially.

The world is shrinking and much more emphasis is placed on international relationships. Many of our medical advisers travel overseas to educate haematologists specifically on ITP and the developments of modern medicines and knowledge. I am sure they also promote the benefits of a patient group for the countries they visit. One of our regular medical advisers from overseas, who himself visits and speaks in many countries on a regular basis, has already asked to be kept abreast of our international involvement. I will not be surprised if we, or the ITP Alliance, are asked to also speak and assist in setting up a patient group abroad.

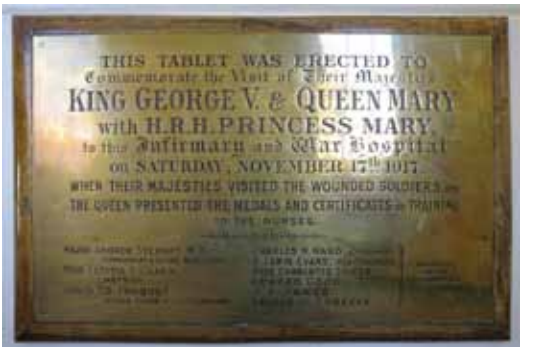
Whipps Cross Centenary

by Rhonda Anderson

As a member of Whipps Cross Hospital Patients' Panel I was invited to join a commemoration of the 100 year anniversary of the naming of Whipps Cross Hospital on Tuesday 14 November 2017. It was formerly called the West Ham Infirmary.

The date for this special event and afternoon tea was chosen as it was exactly 100 years since the hospital was formerly named Whipps Cross by King George V and Queen Mary with HRH Princess Mary. In November 1917 Whipps Cross was a war hospital and medals were distributed to military patients and certificates given to graduating nurses. The special event was part of a series of activities during the Centenary week to celebrate this wonderful milestone and show appreciation of the valuable contribution staff make to delivering safe and compassionate care for patients.

In addition, a mystery guest, who turned out to be the Sarah, Duchess of York, joined us to unveil a centenary plaque. The past was commemorated with an exhibition of archive photographs of the hospital, and a short film was shown where staff and volunteers reflected on what Whipps Cross Hospital means to them. A delicious afternoon tea was served whilst the Duchess of York circulated amongst the guests and posed with them for photographs. She had previously been to Acorn, the children's' ward, and spoken to a number of the patients there with the Chief Executive of Barts Health, Alwyn Williams. Sarah has a special interest in teenage patients with cancer and spoke movingly of her involvement and commitment to turn up and be counted with those facing these challenges. Whipps Cross continues to care for patients and we hope this will be so for at least the next 100 years.



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Send this form to:– The ITP Support Association,
The Platelet Mission, Kimbolton Rd, Bolnhurst, Beds, MK44 2EL