Welcome to our first newsletter from PID UK’s Director, Dr Susan Walsh

‘Hello everyone! PID UK has been up and running for just 6 months but we have so much to report. Of course there’s so much more to do but I hope this newsletter gives you a real flavour of PID UK and what we are doing for people affected by PIDs. None of what we have achieved so far would have been possible without the amazing support from our patient representative panel and volunteers, our medical advisory panel, the clinicians, nurses and patients I have met and you, our valuable members. Thank you so much. Over the festive period I will be raising a glass (or maybe more) of wine to a successful 2014 for PID UK, full of hard but rewarding work in our ambition to be a major source of support for PID patients and their families.

Wishing you all a Merry Christmas and a Happy and Healthy New Year!’

Getting to know us

Our website

The PID UK website was launched in July 2013. It is packed with information, practical help, patient stories and news ranging from the basics about PIDs to in-depth, medically reviewed information on specific medical conditions including CVID, XLA, Hyper IgM and IgE Syndromes, Selective IgA deficiency and rare conditions, such as Wiskott Aldrich Syndrome. We will be expanding the list of the conditions covered over the next year and developing patient leaflets so watch this space.

You can find out lots about immunoglobulin therapy, including frequently asked questions and your rights as patients to this treatment. There’s also practical information on living with PIDs with advice on claiming benefits, making the most of your hospital appointment and looking after yourself if you are a carer.

We also signpost to other organisations to ensure you get as much support as you need. These include condition specific organisations to international support groups, professional organisations and Facebook forums.

We welcome your input, so don’t hesitate to get in touch with your comments and/or suggestions.
Our Patient Representative Panel

PID UK is really our Patient Representative Panel (PRP). Our members come from different regions of the UK, cover different age groups, conditions and experience of PIDs. They bring a wealth of experience to PID UK about what having a PID really means and help ensure we have a continual stream of new ideas of how to support patients.

Hannah and Sadie, members of our PRP, give their personal perspectives on why they are involved with PID UK:

Hannah: ‘Having been diagnosed with CVID as a baby, I have always wanted to be able to do my part and volunteer to help others suffering from PIDs. I believe having a central point providing a wealth of information for patients is important and that PID UK is doing a brilliant job in doing so. I think it is especially important to help children and young adults with PIDs as they are growing up and face challenges their friends do not. Hopefully with support from members, PID UK will be able to offer more and more help to patients so that no-one with a PID feels alone, and I look forward to being part of this’.

Sadie: ‘I am mum to 2 little boys, one of whom has had a complex immune disorder since birth. He has endured many severe infections and hospital stays and has recently undergone a bone marrow transplant. I have joined the PID UK patient representative panel to support other parents and children with primary immune deficiencies and to help spread the word about PID UK.’

Our Medical Advisory Panel and its Chairman

We are extremely fortunate to have the support of an incredible Medical Advisory Panel who are leading experts in caring for people with PIDs. The members are doctors and nurses with knowledge of adult and paediatric care and come from different UK centres including Scotland, Wales and Northern Ireland. They ensure the information PID UK provides to you on all things medical is accurate; evidence based and reflects current practice.

Dr Matthew Buckland (left), Consultant Immunologist at St Bartholomew’s Hospital London is the Chair of the Panel. He says:

‘It has been a pleasure to be part of PID UK during this inaugural year. In a very short time an extraordinary amount has been achieved. GD UK has been essential in providing the infrastructure for PID UK and in turn Dr Walsh and the team have taken PID UK from concept through to fully functioning patient organisation.

In that time the website has been setup, the medical and other patient literature sourced, reviewed and published, to provide a basic level of information for patients with a wide variety of disorders.

PID UK has supported individuals and whole groups of patients and worked with host clinical institutions on specific initiatives such as supporting patient days. PID UK has rapidly been recognised by government and non-government organisations as a suitable partner to discuss and progress key issues in primary immunodeficiency with a patient facing focus. For all these achievements and many more I am delighted at the success of this year and am sure that this will continue, since the ever increasing patient and family membership tells us that what we are doing really is worthwhile’.
Health issues and PIDs

Confused by the do’s and don’ts of immunisations?

Don’t be! If you want to know if you or your child with a PID should have a certain vaccination then take a look at our section on immunisation in PIDs.

Remember our Medical Advisory Panel’s two golden rules and you won’t go wrong:

- You should ALWAYS seek advice from your specialist immunologist before you have an immunisation.
- No person with a PID should receive ‘live’ vaccines unless particularly recommended in a special case.

Have you had your flu jab yet? Don’t delay in having it!

Having a flu jab is a good idea if you have a PID. Although it may not offer full protection it is a useful precaution against developing flu complications such as chest infections and may help shorten how long you are ill for. If you are in the UK and have or live with someone who has a weakened immune system, you are entitled to a FREE flu jab at your doctor’s surgery. Or you can choose to pay for the vaccine at shops, including Boots and Sainsbury’s.

Important immunisation updates

The children’s flu vaccine

This vaccine comes as a nasal spray and is called Fluenz. It is a live vaccine so it should not be given to children with a PID without getting individual specific advice from your Immunology centre. There are other killed (injectable) flu vaccines and most Immunology centres recommend both patients and their household contacts receive such vaccination.

A new vaccine against Meningitis B is available

Meningitis B remains the leading cause of invasive meningococcal disease in the UK. In December 2013 a new protective vaccine for Meningitis B was made available for the first time for children and adults with PID at high risk of getting this infection. This includes patients who are currently recommended to receive the ACWY vaccine - namely those do not have a spleen, or have a spleen that does not work properly, complement deficiency or where your immunologist or relevant specialist believes there is an increased risk. If you think you may benefit from this vaccine do talk to your GP or your immunologist.

Looking after your lungs

Unfortunately this is probably the peak season for people with PIDs to get respiratory infections such as bronchitis, sinusitis and even pneumonia. The long-term effects can be unpleasant feelings of breathlessness. As a result, it can be tempting to take the easy option and give up trying to lead an active life when you are well and free from infections. Please don’t!

Remember, the right sort of exercise does you no harm at all and can do you a great deal of good. Even when it causes breathlessness, exercising can:

- Increase the fitness of your muscles
- Reduce the fear of breathlessness
- Reduce the feeling of breathlessness
- Increase your ability to exercise
- Make you feel better and improve your self-confidence
- Break the vicious circle of inactivity

Just a few simple exercises performed for a couple of minutes a day can make all the difference to your physical and mental well-being. Ask your medical team about what they recommend for you. Remember though when you do have an acute infection it may not be wise to exercise. The PID UK website has lots of information about keeping your lungs healthy and helps answer your questions about this important health topic.
News from conferences

Update from the International Primary Immunodeficiency Congress (IPIC 2013)

‘Half-way house’ for the implementation of a UK national screening programme for X-SCID

Professor Bobby Gaspar, Professor of Paediatrics and Immunology at Great Ormond Street Hospital, updated the conference on the current position of the UK national screening programme for X-SCID.

SCID can be a fatal condition for children who are not diagnosed within the first 2-3 years of life. However, if detected early the condition can be managed effectively so as to avoid life-threatening infections and treatments such as stem cell transplants and gene therapy are available with good success rates. In 2012 proposals were put forward to UK National Screening Committee (UKNSC) to put in place a national screening programme to test newborns for SCID using blood spots taken from Guthrie cards. This would help ensure children do not die needlessly from this condition.

Review and consultation

Professor Gaspar explained ‘For the national screening to be implemented it had to meet 19 strict criteria set by the UKNSC and this was assessed by an independent review, followed by a public consultation. We involved a professional lobbying company to help and organise awareness and coordinate a response to the consultation process. We also had some political support with meetings between families affected by SCID and MPs. The committee received around 200 letters of support for the programme.’

However, in March 2013 the committee decided that they were not yet ready to implement national screening. This decision was based on the need for further evidence covering four key areas: knowledge of how many people in the UK are likely to be affected by SCID, known as epidemiology; the performance of the test for SCID, for example taking into consideration if the test picks up babies with other related conditions and how these children might be managed and the likelihood of good clinical outcomes, and finally the clinical cost effectiveness of having a test in place.

‘A very big step forward’

‘Although disappointing, the recommendations of the committee were actually a very big step forward,’ said Professor Gaspar. ‘We have been asked to do an evaluation study to address the committee’s areas of concern. These studies will lead ultimately to screening 350,000 newborn babies per year, representing about half the number of babies born each year in the UK. There will be a full policy review after 3 years. It’s been a long and tortuous process so far but we are making progress. However, even when an effective screening programme is in place some immunodeficiency conditions will not be picked up so awareness of these conditions still remains extremely important.’

The aim is to implement screening at a European level and this is a key objective for the International Patient Organisation for Primary Immunodeficiencies (IPOPI). ‘This will need an economic cost benefit analysis at a European level’, said Johan Prevot, IPOPI’s Chief Executive.

You can access the presentations from other speakers at this conference at www.ipic2013.com/speakers-presentations.php
Next Generation Sequencing and its application to PIDs

Next Generation Sequencing is a new and powerful method that can give a read out of a person’s DNA code very quickly and accurately. This means that finding ‘errors’ (mutations) in DNA, for example those genetic changes that cause PIDs, is much easier to do. Already it is being used to help the diagnosis of PIDs and helping further research, however it will be some time before this technique is used routinely in clinic. When it is, many ethical and societal considerations will need to be taken into account. These include potentially finding DNA mutations unrelated to the condition being looked for and how these findings would be handled, and how insurance companies could use the data generated.

Newborn screening: TREC and KREC

Screening for X-SCID in newborns uses a method that looks for the absence or low numbers of T-cells using what is known as TREC (T-cell receptor excision circles) analysis. Dr Hammarstrom, from the Karolinska Institute, Sweden described his research to pick up PID conditions where few or no B cells are made. This uses a similar technique to TREC but is called KREC (B-cell kappa chain excision circles) analysis. The number of KRECs in the newborn blood sample being directly related to the number of B cells present. This would lead to the widening of newborn screening programmes in some countries in Europe, to include combining both TREC and KREC analysis into one screening programme. This means that many different PIDs could be detected at the same time in the screening test, without the need for additional sampling.

Dr Hammarstrom showed evidence that this combined approach could be used to detect not only SCID but XLA, Nijmegen Breakage Syndrome and Ataxia telangiectasia, conditions where B cells are absent or reduced. The method is not perfect and does not detect all these conditions, so awareness in combination with other diagnostic methods remains important. In the future it may be possible to start to screen for complement deficiencies and disorders that affect how phagocytes work, such Chronic Granulomatous Disease (CGD).

The UKPID Registry

Dr Buckland updated the conference on the progress of the UK Primary Immunodeficiency Registry (UKPID Registry). It is the first on-line registry of primary immune deficiency in the UK and contains data on over 2890 patients. The registry is in its fifth year and is starting to give valuable information on how many patients are affected by particular PID conditions, the geographical spread of PID patients across the UK, their diagnoses, how old they when they are diagnosed, details of diagnostics delay and the effectiveness of different treatments for patients.

Clinical trial update

Gene therapy back on track with 5 children with ADA-SCID successfully treated

Professor Bobby Gaspar presented at the European Society of Gene and Cell Therapy conference in Madrid, Spain in October 2013 the preliminary results for the first two children, who had received 18 months ago, an improved SCID gene therapy treatment. The children have a form of severe combined immunodeficiency (SCID), known as ADA-SCID. ‘The children’s immune systems have continued to improve since receiving the treatment’, said Professor Gaspar of Great Ormond Street Hospital in London, who led the trial. Three more children have been treated since then, and they too are showing signs of a full recovery. Read the full story here and find out more about gene therapy on our website.
Working on your behalf

As part of our role PID UK is working to help improve healthcare for people affected by PIDs. This is just some of the work we have been involved in this year.

**NHS England: Consultation on Specialised Services**

Influencing how immunology services are delivered in NHS England is a key objective for PID UK. We responded to the consultation on specialised immunology services directly commissioned by NHS England that were put in place in April 2013. We also supported the new Clinical Commissioning policy for the treatment Hereditary Angioedema (HAE) ensuring HAE patients get the treatment they need.

**Postgraduate medical education and training across the UK**

PID UK joined forces with the Academy of Medical Sciences and 31 other organisations to support their position on reforms to the structure of postgraduate medical education and training across the UK.

This was to help ensure that people working in the NHS have the right training, support and opportunities to learn about research and are encouraged to conduct research and use the findings to improve the care and treatment they give to their patients.

**Supporting greater transparency of clinical trial results**

PID UK supported the campaign for greater transparency of clinical trial results, calling for all clinical trials to be registered and their methods and results reported.

Our statement of support reads: ‘PID UK strongly support the AllTrials campaign because it will help avoid duplication of effort, add to the knowledge base and better inform treatment options, so ensuring that the invaluable contribution made by people participating in clinical trials and the benefit gained, is shared fully with society.’

**Supporting the UK X-SCID screening programme**

PID UK submitted a full response to the UK National Screening Programme supporting setting up of a UK based newborn screening programme for SCID. Our submission was strengthened by a moving testimony from a mother whose child sadly died as a result of SCID.

**Supporting the ‘It’s Ok to ask campaign’**

This campaign encourages patients and carers to ask their consultant about clinical research and whether it might be right for them. Find out more about the campaign and give your support click here.
Immunology Home Therapy Patient Fun Day

PID UK was proud to sponsor and attend an immunology home therapy fun day at the London Transport Museum on Saturday 12th October. The event, organised by the Immunology team at Great Ormond Street Hospital (GOSH), brought together children aged 12 and under who are on home immunoglobulin replacement therapy. For many children and their parents this was the first time they had met other affected families. ‘The event was a great success’, said Clare Malcomson, Immunology CNS at GOSH, who looks after these families. ‘It was a chance for everyone to meet each other in a relaxed, fun and safe environment surrounded by nurses and doctors they know. Parents and children shared experiences, tips and ways of coping, as well building new friendships and supportive relationships.’

Royal Free Hospital opens the UCL Institute of Immunity and Transplantation

The Royal Free Hospital looks after the largest number of patients with a primary immunodeficiency in the UK. On the 10th June the first phase of a major global medical research centre for immunity and transplantation was officially opened by HRH the Duke of York Prince Andrew. You can read more here.

PID UK’s very own SuperHero

PID UK has collaborated with Medikidz and the pharmaceutical company BPL to produce a unique educational resource explaining PID to children and young people. Medikidz is the world’s first medical education initiative for children. Using comic books they explain medical conditions to children – in their language, at their level. Think Marvel comics meets medicine and you’ve got the concept!

PID UK used social media to find children to take part and Tom, aged 11 years old, was finally chosen by Medikidz, as the character to appear in the book.

The rest of the children nominated: Lily, Shannon, Elliot, Henry, Jamie, Marc, Ryan, Toby, Tom and Joseph are helping review the book and will have their names in print. A big thank you from PID UK for your amazing response!

‘Tom was delighted to be chosen as a cartoon character, he loves superhero comics and now he gets to be one himself! He is also of an age where he can understand how such publications can help children to gain a better understanding of their condition and he is so pleased to be a part of that’, said his mum, Kate.

Over to Tom: ‘I can’t believe I was chosen, I can’t wait to see my character!’
Here’s a project update from Dr Selena Audit from Medikidz: ‘The ‘Medikidz Explain PID’ project is well underway and our in-house doctors, with the guidance of the leading PID clinicians and nurse specialists, have written the basic medical information. We have a team of award-winning comic book writers and artists who are now bringing the story and artwork to life.

PID UK ran a fantastic campaign in helping us to find a child affected by PID to be the main character of our upcoming book! The response was phenomenal and we are very grateful to PID UK for their incredible support on this project. The final comic is expected to be in print in Summer 2014.’

Local patient groups and regional news

We would like to make this a regular feature in our newsletter with news from local patient groups. If you would like to contribute to this or any other section of the newsletter please get in touch with us.

Patient Meetings

West Midlands Immunology Patient Day 2013, 29th September 2013

Over 92 patients and family members attended the patient day hosted by the Immunology Team, led by Dr Aarn Huissoon and Dr Scott Hackett from Birmingham Heartlands Hospital. The event covered a range of topics including information on immunology services and how they are now funded, their research on CVID and involvement in new clinical trials.

Dr Naveen Sharma, Gastroenterologist at Birmingham Heartlands talked about gut problems and PIDs and was followed by Louise Dickie, a Senior Dietician, on seeking dietary advice to improve gut complications. Dr Terry Woolley, from CSL Behring, gave an overview of the processes involved in purifying immunoglobulin for patient use and the mechanisms that all immunoglobulin providers must have in place to ensure the safety of their products.

Dr Scott Hackett covered practical issues too with talks on travelling with PID ‘All trips are possible!’ followed by an inspirational talk from Tom, a PID patient, who told the audience about his fantastic six weeks in Borneo.

The meeting also launched the West Midlands Immunology Patient Group (WMIG) with Martin Parry and Margaret Bennett giving an update on its aims and progress.

You can read a full report of the day covering all these topics on the PID UK website.

Patients’ forum launched for the immunology services at Central Manchester University Hospital

The launch event in April, and a follow up event in October, brought together patients and members of the Immunology team to discuss how patients can help shape the future of local services. ‘Patients can give us valuable feedback on their experiences of our services that we can learn from. This all about working in partnership’, the Central Manchester Team said.

The North West Immunology departments are working to arrange another patient forum event. If you would like to be involved please contact the Central Manchester Immunology Nursing team on 0161 276646.
HAE UK Patient Day, 16th November 2013

At this well attended event Ann Price, Trustee and Founder of HAE UK, updated the audience on how much HAE UK had achieved since its launch in October 2010, the challenges some patients still face in getting best care and plans going forward to support patients with Hereditary Angioedema (HAE) and Acquired Angioedema.

Dr Mark Gompels discussed the need for clinical skill in the diagnosis of HAE because of the variable way it can present medically, the role of shared care and how empowered 'expert patients', in control of their own condition, can lead to better patient outcomes. Dr Hillary Longhurst introduced the new Clinical Commissioning Policy for the treatment of HAE and described it as 'a major step forward' in how HAE patients can access the medicines they need in England, with funding coming centrally rather than through GP budgets. This policy does not, as yet, cover Scotland or Wales, but these areas are likely to be highly influenced by its adoption in England. John Dempster and Christine Symons, Clinical Nurse Specialists who care for patients with HAE, discussed the benefits of home therapy and how everyone should be considered for this, alongside a personalised care plan.

Hull and East Riding Patient Group Event, 16th October 2013

Patients and their family members enjoyed an informative and relaxing evening with hospital staff from Castle Hill Hospital-East Yorkshire Adult Allergy and Immunology Centre. The evening covered how the services work and asked for patient opinion on the shape of services going forward.

The evening also marked the retirement and the long and prestigious career of Consultant Immunologist, Dr Philip Dore, who has worked in the Hull area from 1986. Dr Dore gave a humorous view of the many changes in the NHS he has experienced and described the evolution of the immunology services he was instrumental in developing in Hull. The meeting also welcomed Dr Pavel Gordins, as a new member of the immunology team, joining Consultant Dr Mohammed Abuzakouk and Dr Nakonechna and the nursing team headed by Jackie Moor.

Olympic gold medallist opens the Clinical Immunology and Allergy Unit at Northern General Hospital, Sheffield

Nicola Adams MBE (right), who won a boxing Olympic Gold medal at London 2012, officially opened the unit, led by Dr William Egner, on the 24th July. Nicola is coached by one of the centre’s immunology patients.

PID UK is getting connected!

Working to make your voice heard means joining forces with other organisations.

In addition to being a member of Genetic Alliance UK and Rare Diseases UK, PID UK became an associate member of the International Patient Organisation for Primary Immunodeficiencies (IPOPI) and the Specialised Healthcare Alliance, a coalition of patient-related groups and corporate members with a strong record of campaigning on behalf of people with rare and complex conditions in need of specialised care.
Fundraising news

We are hugely grateful to our first fundraisers for PID UK. Here we highlight just some of people who are helping us to help others through their donations. Thank you so much!

Bob, Emma, Lizzy and Caroline cycled a combined distance of 108 miles together on Sunday the 1st September in the Southend area. They raised over £450 for PID UK. Thank you!

Huge thanks to Henry (far-left on the photo) who is our youngest fundraiser at 8 years old! Henry raised £47 by hosting a raffle. Henry is a keen supporter of PID UK and his mum Deborah tells us he has lots planned for the new year to help support us and raise awareness. You are a star!

Henry also made the news this year in his local newspaper ‘The Retford Times’.

Please support!

Jo Herbert is thinking ahead and has planned a 250-mile cycle ride around the north of France for May 2014. Jo was inspired to fundraise for PID UK after attending a PID UK sponsored Great Ormond Street Hospital family event where her son, Toby, had the chance to meet other children with PIDs. Thank you Jo and we wish you every success.

If you would like to support Jo then visit her Just Giving page at www.justgiving.com/JoHerbert-PID-UK

Credit: With thanks to the Crawley Observer and Jon Rigby.

Inspired?

There are so many ways you can fundraise for PID UK. Here are just some of them.

Celebrate and give

If you're celebrating a special occasion, why not ask your friends and family to make a donation to PID UK instead of spending money on presents?

Get sporty

Want to get a bit fitter and raise money for a good cause? We’ve lots of activities that suit all abilities.

Set up a JustGiving fundraising page

This helps you personalise your fundraising. It’s really easy to set up so don’t feel daunted.

You can find out more about fundraising for PID UK at http://www.piduk.org/fundraising
Your donations

We are extremely privileged to have received a lot of individual donations this year. We are also grateful for two generous donations from the pharmaceutical company CSL Behring.

Sincere thanks to our recent donors:

- Solihull School
- The Sawyer Family
- Mr Beddows
- Mrs Ingleston
- Mr McGaughy
- Mrs Johnston
- In memoriam donations to celebrate the life of Mr Steven Andrews

And to all those who have contributed to our organisation throughout the year a BIG THANK YOU!

Social Media

Be sure to like our facebook and twitter pages to be kept up to date with our latest news.

Would you like to be a volunteer?

Do you think you can help PID UK in some way? This may be by helping us develop content for our website or providing us with information that you think would help other families. One of the biggest struggles that many people have faced is that of applying for benefits. If you have experience of the benefits system or have worked as a benefits advisor and would be able to volunteer just a few hours each week to offer advice to members then please contact us.

Would you like to donate to PID UK?

We hope you think that PID UK has achieved a great deal this year, and we assure you we will be working even harder next year to continue our work. If you would like to support us in our aim to help people with PIDs then please make a donation.

You can make a donation online or send a cheque in the post payable to ‘PID UK’ to the following address

PID UK
199A Victoria Street
London
SW1E 5NE

Contact Us

There are three ways to get in touch with us here at PID UK and we look forward to hearing from you whether you have a question, want to volunteer your services or just want to say hello.

Call us on 0800 987 8986
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Write to us at PID UK, 199A Victoria Street, London, SW1E 5NE