

Primary Immunodeficiency UK update for March 2016

Dear PID UK Member,

Spring has officially sprung and we hope you are enjoying the lighter evenings as much as are. Here's our latest news for you.



PID UK'S Patient Experience Survey

Thank you for the wonderful response to our survey. If you still haven't completed it please do consider taking part. Your answers will be anonymous and treated in confidence. We would especially like to hear from parents/carers of children with PID. You can access the survey [here](#). Thank you!

World PI Week is coming soon

World PI Week (WPIW) is a global campaign to raise awareness and improve diagnosis and treatment of PID and this year runs from the 22nd - 29th April 2016. In a nutshell it's a wonderful chance to get primary immunodeficiency noticed and to make your voice heard! Find out how you can get involved [here](#). This year PID UK will be working in partnership with IPOPI to launch the 'Burden of Treatment' study and we will be contacting members about this during WPIW. We will also be launching three new patient leaflets, which we hope will help empower people about their condition.

PIDs in the news

Gene therapy hit the headlines when the European Medicines Agency endorsed the use for children with ADA-SCID who do not have a bone marrow match. Read more [here](#). This landmark event was covered in the METRO newspaper and the magazine New Scientist and included a mention of PID UK.

GENE THERAPY LANDMARK FOR 'BUBBLE BABIES'

DNA treatment for severe immune deficiency is approved

W E NEARLY lost him twice,' says Kelly Gillon of her son Zeus, who was diagnosed with the potentially fatal condition ADA-SCID in the first weeks of his life.

Children born with severe combined immunodeficiency (SCID) lack a working immune system and the need to keep them isolated from routine infections led to the phrase 'bubble babies'. Sufferers of ADA-SCID have defective copies of the gene making the enzyme adenosine deaminase. Without it, toxic debris builds up in white blood cells and kills them.

Zeus was lucky enough to have a bone marrow transplant but many babies die before a donor is found. In a triumph for gene therapy, a new treatment, Strimvelis, was rubber-stamped for approval by the European Medicines Agency last week.

Strimvelis is the first gene therapy treatment to alter DNA in stem cells permanently and was developed by the San Raffaele Telethon Institute for Gene Therapy in Milan, Italy, and pharmaceutical giant GlaxoSmithKline. The procedure involves extracting the bone marrow stem cells that regenerate the immune system and infecting them with a harmless virus that uploads a correct copy of the gene for ADA. The altered cells are injected back into the patient to generate a healthy immune system. Data on 22 toddlers in Italy showed a 100 per cent survival rate.

Sven Kili, of GlaxoSmithKline, said: 'The first patient is now 13 years post-treatment. The gene we insert is assimilated completely into the genome. I'm hesitant to call it a cure. But the stem cells we've altered go on to replicate so we have no reason to suspect it won't be indefinite.'

Susan Walsh, director of PID UK, a charity for families affected by the condition, said: 'It's an absolute landmark for gene therapy.' Bobby Gasper of Great Ormond Street Hospital, London, said: 'Approval of a licensed therapy is very positive and shows gene therapy can become standardised medicine.'

Also in New Scientist this week: ■ The evolutionary roots of today's migration crisis ■ Hélène Hu world's shyest parrot find love ■ How your brain really sees the world

Health news

Prescription charges have gone up yet again. PID UK thinks that vital drugs for long term conditions like PID should be free and people shouldn't be punished financially for being sick. That is why PID UK is a member of the [Prescription Charges Coalition](#). Join the campaign for free prescriptions for those with long-term conditions by writing to your MP. You can download a template email [here](#).



The cost of prescription prepayment certificates (PPC) has been frozen at £29.10 for 3 months and the cost of the annual PPC will stay at £104. If you can afford to pay for a PPC then it may save you money - a 3-month PPC saves money if you need four or more items in 3 months and a 12-month certificate does so, if 14 or more items are needed over a year. Find out if you qualify for free prescriptions and how to apply for a PPC [here](#).

Genes, genetics, genomics!

Are you bamboozled by these words and what they mean? Then check out the short videos produced by Genomics England.

The aim of these videos is to get people talking and improve understanding. Genomics England now want to know how successful they have been by asking the public what they think after watching the films so that the topics of DNA, genes and genomics become something that most people feel they can relate to. It should take 10 minutes to watch the videos and answer questions on them. Click [here](#) to get involved.

PID UK activities

Benefits and Work Guides you can trust

PID UK became a professional member of [‘Benefits and Work - Guides you can trust’](#) so we can now offer better support on benefit queries.

PID UK responded to the NHS consultation on proposed changes to NHS England Clinical Reference Groups and took part in a National Institute for Health and Care Excellence (NICE) scoping workshop on gene therapy for ADA-SCID.

Our patient representatives Fiona Watt and Rae McNairney took part in several meetings in Scotland. Fiona attended an NHS Scotland National Plasma Product Expert Advisory Group meeting and Rae was at the Cross Party Group Meeting on Rare Diseases at the Scottish parliament and the ‘National Conversation programme - Creating a Healthier Scotland’ event. You can read Rae’s write-ups [here](#).

Questions and Answers

Here are some of the queries PID UK has received recently. Click on the links to find our answers.

Q. My child is at university and in the last few months have been too ill to complete exams and coursework. [What can we do?](#)

Q. What can I do to maximise my chance for appeal for [Employment Support Allowance?](#)

Q. I'm having trouble getting personal independence payment. [Can you help?](#)

Fundraising for PID UK

There's so much you can do to help us through fundraising and we have lots of ideas on our website for you to look at and we will support you throughout your fundraising journey. To speak to us about fundraising get in touch at events@piduk.org.

Here's what our sporty fundraisers are doing:

On the 9th April, Cheryl Gibbs ran the Milton Country Park 5k for PID UK and has so far raised over £850. Her gorgeous son, Lewis, has XLA. Huge thanks Cheryl! You can still sponsor her at <https://www.justgiving.com/Cheryl-Gibbs1>.

In September, Sandeep Longia is running the New Forest marathon because of the support PID UK has given his best friend. Show your support and sponsor him [here](#).

Facebook and forums

Get the latest news on our activities with PID UK's [twitter](#) and PID UK [Facebook page](#). Please give us a 'like'.

UK PID Patient forum

The UK PID Patient facebook forum is run by two PID UK patient representatives and gives patients and carers a chance to vent, talk and support each other. Check it out [here](#).

Thank you all for your support and generous donations.

With best wishes from,

The PID UK Team