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Welcome to the June Immunodeficiency UK newsletter!



Hello [\$FNAME|Customer|Guest]\$, we hope you are well and enjoying the sun that has (for many of us) finally arrived!

You may have seen several posts on our social media about plasma donation and how people can help. There is a global shortage of plasma, the vital ingredient in immunoglobulin therapy, and we are doing all we can to encourage donations.

Read on for further details about this, and everything else we have been working on this month...

Support Immunodeficiency UK while you shop!

[\$FNAME|Customer|Guest]\$, did you know you can donate to Immunodeficiency UK while you shop and it **won't cost you a penny?**

We know it sounds too good to be true, but many big retailers offer to donate a percentage to your chosen charity every time you make an eligible purchase.

Amazon Smile

Just click the link below to select Immunodeficiency UK, and every time you shop on Amazon you'll help to support our work!



Support us with Amazon Smile

fasyfundraising



We've also registered with easyfundraising, which means over 4,000 shops and sites will now donate to us for FREE every time you use easyfundraising to shop with them.

These donations will help SO MUCH, so please sign up to support us – it's completely FREE and doesn't take long.

Support us with easyfundraising

Gene Therapy Update



The news on the development of gene therapy as a treatment option for children affected by a primary immunodeficiency is looking bright.

A gene therapy approach uses a person's own blood stem cells, into which a working copy of the missing or faulty gene is inserted using a virus to carry the gene into the cells. By giving these gene-corrected stem cells to the patient, the aim is to permanently correct genetic disorders with a single treatment. Here we summarise recent developments:

Gene Therapy Trials for ADA-SCID

The results from 50 patients with ADA-SCID (30 in the United States and 20 in the United Kingdom) treated with gene therapy (GT) have been published in the New England Journal of Medicine. The results are compelling for the use of GT to treat this rare condition. Overall survival was 100% through the end of follow-up (two years for U.S. study patients and three years for UK study patients). At one year, event-free survival was 97% in U.S. study patients and 100% in UK study patients. The results also showed sustained ADA gene expression, metabolic correction of the disorder, and functional reconstitution of the immune system in 48 out of the 50 patients. [Autologous Ex Vivo Lentiviral Gene Therapy for Adenosine Deaminase Deficiency | NEJM](#)

Susan, CEO of Immunodeficiency UK says, 'The impact of having a child affected by ADA-SCID is massive for families. Parents live in constant fear and anxiety of their child getting a life-threatening infection, with all aspects of life, for both child and family, affected in a negative way, so we welcome the extremely positive data from this ADA-SCID gene therapy trial. We know, first-hand, from families that having gene therapy can be life-saving and life-changing and these results will be hugely reassuring and informative for newly diagnosed families who are considering this treatment option. The results are timely we accelerate towards to the start of England's SCID new born screening programme in September.'

Gene Therapy for X-SCID

A collaborative trial led by Boston Children's Hospital and Great Ormond Street Hospital treating children with X-SCID has been expanded to include several other US centres. The trial is still actively recruiting with promising early data from the first patients treated.

Gene Therapy for Leukocyte Adhesion Deficiency (LAD-1)

LAD-I is a rare genetic disorder affecting the immune system. Those affected can develop life-threatening infections because their white blood cells are unable to leave the bloodstream to fight them. Without a successful bone marrow transplant, severe LAD-I can often be fatal during the first 2 years of life.

The company Rocket Pharma is developing a gene therapy treatment known as RP-L201 to treat LAD-1 with a Phase 2 clinical trial currently underway. This part of the trial is designed to assess the therapeutic safety and efficacy in paediatric patients with severe LAD-I. The trial will enrol nine patients across three sites including Great Ormond Street Hospital, in the UK, and two sites in the USA. Read more at [Leukocyte Adhesion Deficiency-I | Rocket Pharmaceuticals](#). Promising early results of the first 7 patients treated have been presented at International meetings over the past few months.

Gene therapy trials for autosomal recessive CGD (p47-CGD)

Following on from early phase clinical trials to treat X-linked chronic granulomatous disease (X-CGD), a clinical trial is due to open in the UK at Great Ormond Street Hospital and in the US at NIH to investigate the safety and efficacy of the same approach to treat an autosomal recessive form of CGD

(p47-CGD). Up to 10 patients will be recruited across sites and the trial is due to open later this year.

Gene therapy for XLA, WAS, ADA-SCID and X-CGD

The company CSL Behring and Seattle Children's Research Institute have formed a partnership to help advance gene therapy for PID. The alliance will initially work on therapies for Wiskott-Aldrich Syndrome (WAS) and X-linked Agammaglobulinemia (XLA). You can read their announcement [here](#).

The company Orchard Therapeutics Focus – [Orchard Therapeutics \(orchard-tx.com\)](#) is also developing gene therapy for WAS, ADA-SCID and X-linked Chronic Granulomatous Disease (X-CGD).

Opticom Patient Recruitment

Opticom - an independent market research agency acting on behalf of a pharmaceutical company active in the development of treatments for rare diseases – is currently looking into devices used for subcutaneous infusions.

They are interested in hearing patients' views and opinions as users of infusion pumps to better understand the current needs and future expectations of patients who are / will use infusion pump. To be eligible for their study you need to be:

- Between 18 and 80 years old
- Receiving immunoglobulin treatment regularly to treat your primary immunodeficiency
- Using a mechanical pump for your subcutaneous infusions of immunoglobulin.
- Carrying out infusions yourself at home and for at least the last year.

If you fit these criteria, Opticom would like to invite you to take part in an in-depth interview – via Teams – which will take about 30 minutes depending on your answers.

To thank you for your time and engagement in the study, you will receive a remuneration of £30. Immunodeficiency UK will receive a donation too.

For any questions about the study, please contact Cécilia Nyholm, Project Manager at Opticom: cecilia@opticom.se

Welcome to Dr Lisa Devlin - a new member of our Medical Advisory Panel



Lisa graduated from Queens University Belfast (QUB), and completed her general professional training in Northern Ireland and Perth, Western Australia. After completing specialist training at the Regional Immunology Service, located in Belfast, she was appointed as a Consultant Immunologist in the service in 2011.

She is clinical lead for the clinical and the laboratory service and has been integral in progressing the home therapy program, developing nurse led specialist clinics, and achieving and maintaining QPIDS accreditation status of the service. Lisa has worked as a UKAS assessor for immunology laboratories, is a member of the UKPIN committee, and the QPIDS/IQAS steering groups. She is involved in the preparation and delivery of the immunology curriculum to QUB undergraduates, and the RCPATH professional examinations for specialist trainees in immunology.

[Meet our Medical Panel](#)

Are you or someone you know living with secondary immunodeficiency?

We're looking for individuals and families to share their experiences and help us create greater understanding about secondary immunodeficiency. These stories will be added to our new website and help us improve the resources available to those with secondary immunodeficiency. We'll also share them on our social media to help raise awareness about diagnosis, treatment and what it's like to live with a secondary immunodeficiency.

If you've been affected by a secondary immunodeficiency and would like to get involved, please email Jen at jen.rush@immunodeficiencyuk.org.

Immunoglobulin Shortage

The impact of the immunoglobulin shortage is now starting to be felt by the primary and secondary immunodeficiency community and we are continuing to press the NHS for transparency on the specific guidance they are giving to clinicians managing patients reliant on this therapy. Despite many requests, Immunodeficiency UK has not received any official communication concerning the scale of the problem, how the shortage will be managed, how it will affect patients and for how long. Our community deserves to be kept informed.

There is however some good news we can share on UK immunoglobulin fractionation. The DHSC, NHS & Treasury have now at last approved the Transfusion Services' business plan for saving and exploiting recovered plasma and source plasma for fractionation. The necessary funding has been allocated for the current year and will form a routine part of the services' public expenditure allocation(s) in the years ahead. This ends the uncertainties that had persisted around this topic despite the Government's announcement on February 25th of the partial lifting of the ban on UK donors' plasma. Immunodeficiency UK welcomes this good news although it will be some time before we start to see the UK becoming more self-sufficient regarding immunoglobulin supplies.

The NHS needs plasma to make life-saving medications

Plasma saves the lives of thousands of people with rare diseases, including those with immunodeficiency. We encourage you to highlight the need for plasma donations through sharing with friends and family this leaflet from NHS Blood Transfusion Service.

There is a growing need for plasma for medicines and the NHS need people to donate.

Call 0300 123 23 23 to find out more and book an appointment.

[Find out more about plasma donation](#)

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