Welcome to the March Immunodeficiency UK newsletter!

Hello Immunodeficiency UK member,

Welcome to the Immunodeficiency UK newsletter. This month we update you on the arrival of UK plasma products, share some good news about a specialised treatment for a rare primary immunodeficiency, changes in NHS England, and provide details about our upcoming dealing with diagnosis webinar.

Read on for our monthly news round up and, don't forget to visit us on Facebook to get updates throughout the month.

Grifol has provided a grant for the creation of the newsletter with no input into content.

NICE recommends the treatment Leniolisib for APDS

The National Institute for Health and Care Excellence (NICE) has recommended Leniolisib, for treating the rare primary immunodeficiency, activated phosphoinositide 3-kinase delta syndrome (APDS). Leniolisib is made by Pharming, and it is the first-ever treatment for activated phosphoinositide 3-kinase delta syndrome (APDS) licensed for NHS use in England and indeed in Europe. Currently, the recommendation is for Leniolisib to be available for people aged 12 years and over.

Sophie, whose little boy, Jasper, is affected by APDS, said “ ***Gives families like ours hope.***” ***This is amazing.***

Susan, our CEO, said ‘*This is a hugely welcomed decision that will make a massive difference to people with APDS by allowing them to have a better quality of life. I’m delighted that NICE and Pharming were able to reach a mutual agreement on cost-effectiveness and a suitable price point. I would like to thank Sophie, our patient expert at the NICE committee meetings, and all the people who took part in our APDS impact survey and shared their personal stories of living with APDS with us. Your help was invaluable in supporting our extensive advocacy work with NICE over the last year.*’

First licensed treatment for ultra-rare immune disorder recommended | NICE

Rare Disease report

Rare Disease Day 2025, on the 28th of February, was an important milestone for the government strategy on rare conditions, the UK Rare Diseases Framework as it marked the fifth and final year of the existing Framework.

Genetic Alliance UK's Rare Disease Day policy report 'More than you can imagine: opportunities for improving the lives of people with rare conditions' reflects on the impact of the UK Rare Disease Framework and highlights the significant unmet need that continues to affect people with rare disorders.

The report calls on the governments of the UK to renew their commitment to rare conditions and ensure that the successor to the existing UK Framework is supported by:

- Ring-fenced funding for each nation
- Well-resourced delivery teams for effective implementation
- Ongoing involvement of the rare condition community
- Enhanced collaboration across the four nations, including joint initiatives
- Development of clear metrics to monitor progress and evaluate outcomes
- Regular reporting of progress to facilitate comparison and collaboration

At the Westminster Rare Disease Day reception, the England Rare Disease Action Plan was launched. You can find a summary of what's new here.

Immunodeficiency UK is a member of Genetic Alliance UK, and we thank them for their work for people affected by rare diseases.

Making history - UK plasma products

After more than 25 years, immunoglobulin (IG) therapy made from plasma from UK donors is being used to treat people with primary and secondary immunodeficiency. Over 8,000 people with immunodeficiency rely on IG therapy and this is a huge step forward for people who rely on this treatment to keep them well.

Immunodeficiency UK supports the NHS in establishing this new medicine supply chain as UK-sourced plasma provides resilience against global shortages and ensures that UK immunodeficiency patients receive the medicines when they need them.

However, the need for plasma-derived treatments is growing every year. We know there are only three plasma donation centres at present, in Birmingham, Reading, and Twickenham and we need more, but asking family and friends to donate blood is a good alternative. Roughly 55% of blood is plasma. The 'recovered' plasma from blood donations can make a difference to plasma supplies.

This video takes you through the journey—setting up new plasma donor centres, collecting and processing plasma, and sending frozen batches to the company Octapharma for manufacture. Watch to see how donors across the UK are helping to save and transform lives.

Please become a blood or plasma donor today:
https://www.blood.co.uk/plasma/

Sign up for our dealing with diagnosis webinar

raremnds
Mental Health for the Rare Disease Community

IMMUNO DEFICIENCY UK
Supporting families affected by primary and secondary immunodeficiency

Dealing with Diagnosis:
making time for you and your feelings

An online workshop for those diagnosed within the last 3 years

Date: Tuesday 22nd April 7pm - 9pm
Location: Zoom

When you first receive a diagnosis of an immunodeficiency, the focus can often be on the practical and physical implications. There may not always be the time to work through the emotional impact of receiving a diagnosis.

Part presentation, part interactive workshop, this small supportive group of up to 20 participants is facilitated by Rareminds Counsellor, Rebecca. It offers a friendly, informal opportunity to discuss together how you felt (and feel) about the impact of an immunodeficiency on your life and relationships with others who will share similar experiences.

"Being able to share our story and hear we are not alone in this journey was really helpful." (Dealing with Diagnosis workshop participant)

If you would like to know more about this workshop or make contact with the Facilitator in advance, please contact Rebecca (Rareminds Specialist Counsellor) on rebecca@rareminds.org

Rebecca Hargreaves MBACP (Accred.)
is an Adult and Young Peoples Counsellor with a particular interest in the psychological impact of health conditions on the family as a whole. She originally worked as a Nurse for over 20 years including in hospices, and is the former Lead for a large schools counselling service. She works as a counsellor with several rare disease patient organisations.

To reserve your place please email Immunodeficiency UK at hello@immunodeficiencyuk.org

We look forward to seeing you there!

Receiving a diagnosis of an immunodeficiency can be a challenging experience, impacting not only your physical well-being but also your emotional and mental health. To support you through this journey, Immunodeficiency UK is pleased to invite you to our upcoming workshop with Rare Minds, which will take place during World PI Week.

Dealing with Diagnosis: Making Time for You and Your Feelings

This is an online workshop for those who have been diagnosed within the last 3 years.

Date: Tuesday, 22nd April
Time: 7:00 pm – 9:00 pm
Location: Zoom

This webinar workshop, facilitated by Rareminds counsellor Rebecca Hargreaves, offers a blend of presentation and interactive discussion. It provides a safe and supportive space for up to 15 participants to explore and share their feelings about the impact of immunodeficiency on their lives and relationships.

This workshop is targeted at people who have been diagnosed with a primary or secondary immunodeficiency within the last three years.

Testimonial from a Previous Participant

"***Great session, really enjoyed listening to others experiences of living with a chronic condition and how it has impacted them personally and professionally.***

To reserve your place for this valuable workshop, please email us at hello@immunodeficiencyuk.org with a short paragraph on why you think this workshop would benefit you.

Each place on this workshop costs our charity £60, so we kindly ask that you only sign up if you fully intend to attend. If circumstances change and you can no longer make it, please let us know as soon as possible so we can offer the space to someone else who would benefit.

We encourage you to secure your spot as soon as possible, as places are limited. We look forward to seeing you at the workshop.

Abolition of NHS England

As you will have heard, the Prime Minister has announced that NHS England (NHSE) will be abolished, and its functions brought back into the Department of Health and Social Care. This is being done to bring the management of the NHS "back into democratic control".

The plans will cut the NHSE workforce by 50%. Legislation is expected to be announced in the King's Speech later this year to formally abolish NHSE, though steps to effectively merge ways of working between DHSC and NHS England are expected in the short-term "transformation period".

The whole organisational reform and merger process will take two years, to reduce duplication in functions, leading to a "much leaner top of the NHS" and redirecting hundreds of millions of pounds of funding to the front line.

Integrated care boards (ICBs) also face staff cuts as they have been asked to reduce costs by 50%.

Taken together, the announcements create a chaotic backdrop for the delegation of specialised services to ICBs, such as immunology and allergy services, which are planned for April. Given that there were already questions about the capacity to take on these services within ICBs, there is a risk any disruption may have a negative impact.

We will be watching developments and await further news about the Government's 10-year plan for the NHS.

Budget constraints impact the consideration of medicines for rare conditions

Prior to the news above, NHS England had announced that the annual 24/25 prioritisation process, which considers specialised treatments for routine commissioning within the NHS, could not go forward due to budget constraints.

Each financial year NHS England runs a process for making discretionary investment decisions about which new specialised treatments can be routinely commissioned (funded). This process is supported by an independently chaired

Clinical Priorities Advisory Group (CPAG) and is known as an annual prioritisation process. It offers an alternative route to make treatments not suitable for assessment by NICE processes, available to patients.

As part of the 2024/25 CPAG annual prioritisation round, 11 treatments were under consideration, including abatacept for autoimmune complications of primary immunodeficiencies caused by CTLA-4 or LRBA genetic mutation.

The prioritisation funding is discretionary, and levels of investment are determined annually based on spending elsewhere. We have been informed that any treatments not prioritised will be eligible for consideration at subsequent rounds, but we understand the 25/26 round will also be delayed until funding can be identified. This is a concerning development that does not align with the UK's Rare Disease Strategy of providing access to targeted therapies for rare conditions.

Thank you, Katy

For Katy Rogers, running a marathon wasn't just about personal achievement—it was about honouring her sister Emma and raising awareness for those living with immunodeficiency.

Emma was diagnosed with Common Variable Immune Deficiency (CVID) at 16 after years of recurrent infections and serious illness. Katy has witnessed firsthand the impact of CVID on her sister's life, from the struggles of early diagnosis to the resilience she shows every day.

She decided to take on the Brighton Marathon to support our much-needed work to help people just like her sister.

Katy pushed through 26.2 miles in under two hours and raised over £2,000 to support our work.

From all of us at Immunodeficiency UK, thank you, Katy, for your dedication and for highlighting this vital cause.