



Welcome to the Winter newsletter.

The shortest day is looming as well as the holiday season. We can then start to look forward to the days getting longer.

This is always a busy time of year for everyone and in the WSUK household it is no different with four family birthdays, a son and his partner moving into their first home, Christmas and our first grandchild due from our other son and his partner. Things certainly won't be dull!

Whatever you are doing and however you celebrate the festive season, I hope that you remain safe and well. With the start of a New Year we can all look forward to the results of the TREATWolfram Trial. We will report on these as soon as we hear anything.

As always there is lots of information in this newsletter.

Remember, if you have anything that you would like to see included in future newsletters, then please send it to the office, details at the bottom of this newsletter. Likewise, if you

have any questions or concerns you can contact [Tracy](#), [Phillippa](#), [Georgina](#) or [Olivia](#) about these.

Take care

Tracy



Wolfram Syndrome UK values your involvement with the newsletters, so please remember to share anything that you would like to see included in future newsletters as well as telling us what you have been doing. These can be sent to us at admin@wolframsyndrome.co.uk.

An audio version of this newsletter is posted onto our website and Social Media pages or if you would prefer the audio version or a large print printed version mailed to you, then please let us know.

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News and Updates

WS Conference



Thank you to everyone that attended this year's conference as well as all our presenters. Dr Jamie Timmons from Amylyx Pharmaceuticals, USA attended giving us a further update on the Helios trial and plans for the future. Three of our WS community, Abby, Naomi and Georgina gave a brilliant talk about living with WS and answering questions. It was great to see some new community members attending for the first time. I hope you got a chance to talk to others and learn more about what is happening with research and the help out there for day to day life.

The presentations are now available on the [WSUK website](#) for those of you who were unable to attend or who would like to re-watch a presentation.

Next year's conference is **Saturday 20th September 2025**, at the same venue in Daventry. Updates will be added to the website and future newsletters as they are confirmed. I'm afraid, there is a very slight increase next year for single and double room prices; but we continue to subsidise the cost of the rooms, pay the conference day costs and the dinner on Saturday night.

Wolfram Syndrome Global Awareness Day

This is our 4th year promoting the WS Global Awareness Day.

This year we created a Top 10 of things to know about WS that was compiled from suggestions sent in from the worldwide community. These were shared in a countdown format from 22nd September finishing with the last one and all 10 being re-shared on 1st October, Global Awareness Day. It was great to see members from the community sharing these social media posts and helping to raise awareness.

The two Spanish support groups got involved by lighting up public buildings in their home towns in green and purple. One group held a table sale and another had a stand at a European Researcher's Night to raise awareness of the syndrome.

Let's see what ideas we can come up with for 2025?



Wolfram Syndrome International Researcher's Symposium

24th and 25th October saw thirty WS researchers and doctors from around the world come together in Windsor to give updates to everyone on their work. We had some new attendees from Slovakia, Brazil and Italy which was fantastic!

Notes from the presentations have been shared with all the WS researchers as well as the global community and can also be found via the link in the article below, on the [WSUK website](#) and the [WSRA website](#).



Global Clinical Trial and Research Updates

Instead of separate updates from the UK and the USA we are sharing the link to the notes from the presentations given by the researcher's at October's international researcher's symposium. These notes can also be found on the WSUK, Snow Foundation and the WSRA websites.

Use this [link](#) to read the presentation notes.



WSUK Research Funding

A funding call was sent out to international researchers for a small WS research grant back in June. The closing date has

now passed and our Scientific Advisory Board will be meeting to discuss and review the applications. We hope to be able to announce the recipient of the grant early in the New Year.

WSUK Funded Research Projects

WSUK has now paid the final instalment of funds to Dr Sarkar and his team at University of Birmingham towards their project which is **related to the development of autophagy and NAD enhancers for the treatment of Wolfram syndrome.**

We have also contributed to a research project at KU Leuven in Belgium looking at the **Therapeutic efficacy of base editing (BE) in a novel Wolfram syndrome (WS) mouse model.** Any updates will be shared in future newsletters as well as on the WSUK website.



Orion Project - UK

Be a part of a research study!

The research team at Cambridge University Hospitals are needing some new participants for their ORION study after a few people dropped out. They have asked us to put out a call for anyone that may be interested in taking part. Please read

the details below.

We are looking for participants to take part in our observational study of Wolfram Syndrome. The **Outcomes Research in Inherited Optic Neuropathy (ORION)** study is taking place in Cambridge led by the team of Professor Patrick Yu Wai Man. The aim of ORION is to better understand how the disease progresses in Wolfram Syndrome, in particular, how the eye is affected over time.

As a part of this study, we will measure various aspects of your vision, including how well you can read letters on a chart (visual acuity), colour vision, visual fields and contrast. We will also take some high-resolution scans of the back of your eye (retina and optic nerve) to measure its thickness (optical coherence tomography).

We are one of the few centres in the world to have access to an advanced camera known as the Ocumet Beacon, which uses a bright blue light to take a picture of the back of the eye. This will tell us how well the mitochondria in the eye are working. Mitochondria are the tiny batteries within a cell that produce energy needed for it to work properly. There is good evidence that mitochondria are affected in Wolfram syndrome.

As part of this study, we will also record the movement of your eyes using a camera to give us a better understanding of how some brain areas might be affected in Wolfram Syndrome.

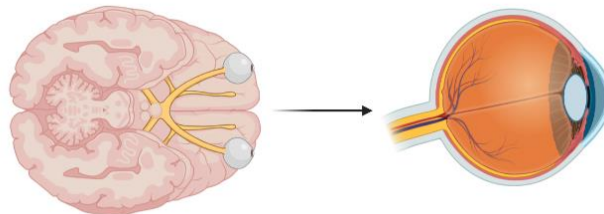
We are keen to recruit individuals in the United Kingdom with:

**a genetic diagnosis of Wolfram syndrome
aged 16 years or older**

As a participant in this study, your **travel expenses will be reimbursed** and the visit can be arranged at a time that is convenient for you. If you are interested in getting involved in this study or have any questions, please contact us using the details below:

Email: riddhima.gautam@nhs.net

Phone Number: 01223 586716 (Monday to Friday, 9am-5pm)



If you are interested please get in touch with them directly.
Thank you.

Trustee Treasurer

WSUK are recruiting for a new Trustee Treasurer. Would you like to be a part of the WSUK Trustee Board or do you know someone that might? Full details of requirements for this post can be found on the home page of our [website](#). If you or anyone is interested then please send a CV to admin@wolframsyndrome.co.uk by Friday 14th February 2025.

News You Can Use

International Patient Registry

Have you wondered how to get involved in Wolfram Syndrome research but did not know how? Here's your chance...check out the Wolfram Syndrome Global Patient Registry at

wsglobalregistry.iamrare.org.

Wolfram Syndrome Global
Patient Registry

Welcome to the Wolfram Syndrome Global Registry

[Learn more »](#)



It is also possible for parents and siblings who are carriers to enter their details to the registry and to answer questions relevant to them. All this information will be helpful to researchers in the future. Thank you for contributing.

Aira - Visual interpreting app - Support whilst travelling

Aira is designed to provide visually impaired people with on demand access to visual information and increase their confidence travelling on the rail network independently.

This app was trialled earlier this year at a few rail stations. It is designed to assist blind and partially sighted passengers, it helps users navigate, read signs, and find assistance when needed.

The results proved very positive and it has now been rolled out to 236 stations across the Southern, Thameslink and Great Northern networks.

The app is free to download from Google Play or the App

Store. As an Aira user, you can access the service **free of charge** at all GTR-managed stations – no time limits! **Just note, while Aira calls are free, data charges may apply depending on your plan.**

Key benefits and features of Aira:

- ✓ **Seamless navigation:** Receive step-by-step audio guidance to help you navigate stations effortlessly.
- ✓ **Real-time updates:** Get live info from an interpreter on train schedules and station details.
- ✓ **User-friendly interface:** Simple to use, no matter your age or tech know-how.

To find out more about Aira, go to the Aira website [here](#)

You can find more information about Aira and other support whilst you are travelling by rail from the [Southern website](#). If you use other rail networks please check their websites for information about support they offer whilst you are travelling.

A step forward in making insulin 'smarter'!

A new type of 'smart' insulin has shown promise in early stage research. In animals, the [insulin](#) was found to only turn on when blood sugar levels are high and stay off when levels are low.

Millions of people with all types of diabetes in the UK use insulin to manage their blood sugar levels. This requires a careful balancing act and it's difficult to get right. So, scientists are trying to develop cleverer insulins that can sense blood sugar levels and respond in the right way. This

would make life for people with diabetes dramatically simpler.

In new research [published in Nature](#), an international research team tested a new type of 'smart' insulin they've developed, called NNC2215. It has a special molecular switch.

When blood sugars are low, a molecule called glucoside locks NNC2215 in the 'off' position so it won't lower blood sugars further. When blood sugar levels rise, glucose (sugar) in the blood replaces the glucoside, switching NNC2215 'on' so it will bring blood sugars down.

Read more on the [Diabetes UK website](#).

Benefit changes announced in Autumn Budget

In the November budget, the Chancellor [set out a number of changes to the benefits system](#).

The biggest news story for carers was the announcement that the Carer's Allowance earnings limit will increase to £196 per week from April 2025. This is equivalent to working 16 hours at National Living Wage.

Alongside this, the budget also included a number of announcements about Universal Credit and other benefits.

[Read Contact's benefit expert Derek's breakdown of all the changes you need to know about.](#)

[You can also read how Contact responded to the budget.](#)

Planning for the Future with Wills and Trusts, Decision Making for Vulnerable People and Welfare Benefits

Renaissance Legal who have presented at several of our conferences about planning for the future have a series of free webinars running through December and into 2025. It isn't a topic that anyone likes to think about, but if you have a child under 18, now is the time to start looking to the future with regards to the possibility of a need for Lasting Power of Attorney as an example.

To see what events they have planned go to their [website](#). To find out more about the services they offer then click [here](#).

From Contact - Reform of mainstream school for children with SEND

Bridget Philipson, Secretary of State for Education, [announced "bold reform" to improve inclusivity and expertise in mainstream education](#). Read the full article by clicking the link above.

Lifestyle Magazine by Motability.

Some of our community who receive the higher rate for mobility in their PIP or DLA and are part of Motability may receive the quarterly 'Lifestyle' magazine from them.

This quarter's newsletter has a few articles that I felt were of interest to you, especially parents of young children. On page 32 there is an article covering 6 pages on inclusive toys for children with all types of disabilities, there is also a small article on the new Barbie dolls being launched, which includes a VI Barbie, page 7.

On page 10 there is a small article about the trial with 'smart' insulin which is also mentioned further up this column.

If you would like to have a look at the articles, you can find the magazine [here](#).

Support Co-ordinator's Updates

Family Support Co-ordinators

Hello everyone!

As we end 2024, what an incredible year we have had! I am pleased to say I have really enjoyed getting to know you all better and meeting new families during the year. We have delivered training to schools, had orientation visits to the adult rare disease centre at the QEH in Birmingham and of course had a new member of staff start with us! I am happy to say Olivia has settled in nicely to our team and is already making contact and supporting some of you.

I am currently completing the IPSEA SEND Law training, in my own time. Did you know, parents and carers can join workshops and webinars from as little as £10! Click the link to find out more:

[SEND law training for parents and carers](#)

Some of you may remember that I asked you to complete an online survey on my work in the community and how you feel I have supported your family in my role. Please keep a look out in your inbox for the link and if you can, I would be grateful if you could complete it. Please remember to mark our emails as "safe/trusted sender".

Alternatively, you can contact my manager directly if you wish to speak with her rather than completing the survey - ginaisherwood@wolframsyndrome.co.uk

To those who celebrate, Merry Christmas and Happy Holidays.
See you in 2025!

Georgina

Email: georginaking@wolframsyndrome.co.uk

Mobile: 07592 629813



A Warm Welcome

I want to take a moment to express my heartfelt gratitude for the warm welcome I've received since joining Wolfram Syndrome UK. It has been a joy to meet and speak with so many families, and I truly appreciate the openness with which you've shared your experiences and insights. I want to extend my heartfelt thanks to the families who have welcomed me into their homes. Your warmth and hospitality have made my visits truly enjoyable. I've loved the opportunity to support you with applications and educational resources, and I'm grateful for the trust you've placed in me. Working together, we can navigate these challenges and create positive outcomes for your children.

My first clinic visit was particularly inspiring; witnessing the dedication and hard work from both families, healthcare professionals and Georgina reinforced the importance of our mission. It was a privilege to see firsthand the care and

support provided to individuals living with Wolfram Syndrome.

I'm excited about the journey ahead and look forward to continuing to collaborate with all of you as we work to improve awareness and support for this community. If you would like me to visit you at home then please drop me a text or an email. Thank you once again for your kindness and support!

Best wishes

Olivia

Email: oliviaedwards@wolframsyndrome.co.uk

Mobile: 07756 778454



Family Support Co-ordinator Page -

<https://wolframsyndrome.co.uk/family-support-coordinator/>



QR code for FSC web page

Adult Support Co-ordinator

Here we are again - I am writing this from a chilly and slightly snowy Liverpool, during one of my adult support visits. Everywhere is very Christmassy but I'm not sure I'm quite ready for any of that yet. See photos!

I have been very busy recently out and about visiting some of you. Sometimes the reason is to put names to faces and offer to help support the family, while some involve assisting with specific issues whether they are medical, practical or related to accessing services of varying kinds. I tend to travel by train, and I like to travel efficiently so please do ask if you would like an in-person visit, at your home or somewhere local to you, as I can then look to see who else in your area might need a visit too.

I am off to the last clinic of the year tomorrow (Friday 22nd Nov). Clinics have been very busy this year, and of course as our community increases these clinics will become busier, not least because some of you took part in the clinical trial and now need to be followed up in the adult clinic. The clinic team are doing their best to get everyone seen.

Going back to Christmas, I am trying to organise a social get together online for those that can manage the technology. I have been racking my brains to come up with something that can be as inclusive as possible. I will possibly run an informal get together with a simple Christmas Quiz, and chat. Perhaps you could share your favourite carol/song, family recipe or tradition that you enjoy at this time of year in whatever way you celebrate. Watch this space for a date and time.

Going into 2025, the 28th February is rare disease day, where all rare diseases share stories, and try to bring more awareness to these diseases. As a trustee at Genetic Alliance UK, I have been helping to develop ideas for this event. We are currently collating an anthology of creative things which tell the stories of people's journeys with their rare disease. These can be in the form of poetry, music, pictures, etc. We have received one entry from a young

person with WS already; but if you have something you would like to share as well, please either let me know and I can submit it on your behalf, or alternatively go to: [More than you can imagine: an anthology of rare experiences - Genetic Alliance.](#)

So, whatever way you celebrate over the festive season, have a lovely time. I will see you in 2025.

Best wishes
Phillippa

Tel: 07752193635

Email: phillippafarrant@wolframsyndrome.co.uk



WS Clinics

WS clinics are usually face-2-face but, if requested, can be done virtually if there are reasons why you can't travel to Birmingham.

Adult's Clinic



Adult clinics are run from the Centre for Rare Diseases in the Heritage Building at The Queen Elizabeth Hospital, Birmingham.

If you are no longer able to attend the date you are invited to, **PLEASE** let the clinic team, [Tracy](#) or [Phillippa](#), our Adult Support Co-ordinator, know so that someone else can be invited in your place. There is always someone ready to take a slot if you can't.

Due to hospital policy, a clinic appointment can only be changed once for a patient as there is now a larger WS community than previously. **Failure to attend will mean you will be discharged from the service.**

Please ensure contact details are kept up to date with both the hospital, whichever one you attend, and the Wolfram Syndrome UK office!

Clinic dates 2025

24th January
29th March
23rd May
25th July (Transition Clinic - TBC)
26th September
28th November

Children's Clinic



The Children's Clinic is run from Waterfall House, the Rare Disease Centre at Birmingham Children's Hospital.

If you are unable to attend **PLEASE** let [Georgina](#) or [Olivia](#), Family Support Co-ordinators, [Tracy](#) or the hospital know asap, so that the place can be offered to someone else, even if you are having a virtual clinic appointment. There will always be someone ready to take up that slot if you can't.

Places for both clinics whether virtual or face to face are by invitation only.

Please **DO NOT** book or make any travel arrangements until you have received confirmation of your clinic invite and have heard from the hospital as to whether you need to attend in person or will be having a virtual clinic appointment.

Clinic Dates 2025

13th-14th January

10th- 11th March

16th - 17th June

25th July (Transition Clinic at QEH, TBC)

6th - 7th October

Please ensure contact details are kept up to date with both the hospital, whichever one you attend, and the Wolfram Syndrome UK office!

There is a '[Who's who at the WS Clinic](#)' page on the website, as well as a [Family Support Co-ordinator's](#) page with information from Georgina and Olivia.

A map of the [Queen Elizabeth Hospital](#) site and a floorplan of [Waterfall House](#) for new visitors, can be found on the clinics page of the WSUK [website](#).

Fundraising News

Upcoming events can be found listed on the events page of the Wolfram Syndrome website www.wolframsyndrome.co.uk

Remember to keep checking the website for upcoming events, any recent news articles & links.



Debra's Big Half in Memory of Amy

On 1st September 2024 myself and my husband ran the Big Half in memory of Amy Hawkins. The Big Half is a half marathon held in London starting at Tower hill and finishing in Greenwich.

Del (my husband) and I were proud to pound the streets of London wearing the Wolfram Syndrome UK running vests to raise awareness and funds in support for the children and families relying on WS UK.

The challenge was tough, but the thought of achieving our goal and a picture of Amy kept us going. A lovely visit along the route from Paul and Sam, Amy's mum and dad, gave us the much needed extra push with their cheering!

Thankfully our friends, colleagues and family have been very generous and to date we have raised £1,477.50 and with my company generously supporting WS UK, they have pledged £500.00 making a grand total (so far) of £1, 977.50.

Update - This total has since risen and is currently around £2257.80 including the pledge by Debra's work.



Continued Support!

Thank you to all those members of the WS community and their friends/families who donate regularly, through monthly standing orders/direct debits, to support the work that we do.

Would you consider making a monthly donation to us? If so, then please contact Tracy in the [office](#) for bank account details. By donating this way, we get the full amount

of your donation without losing any funds from "processing charges" from the online giving platforms.

Why not set yourself a task of asking **3 friends or family members** if they would like to do the same? Can they spare £2.00 a month? Less than the cost of a fancy coffee shop coffee.

Don't forget to let the office know if we can Gift Aid your/their donation. We just need a name and address to do this (forms for [single](#) or [multiple](#) donations are on the website, these can be printed off and sent back to us). If you can Gift Aid this increases your donation to us.

Why not get involved with fundraising for WSUK?

All the fundraising sites we are registered with can be found on the WSUK [website](#).

If you have any event that you would like to share or possibly promote, or you take part in an event to raise funds please let us know in the WSUK office and we can share it on our social media pages and the newsletter.

Ways to Donate to WSUK



Facebook Donations

We continue to be thought of by WS members and their families when it comes to their birthdays or they are doing a fundraising event through creating [Facebook fundraiser pages](#).

A big thank you to everyone who supports us in this way.



PayPal Giving Fund

PayPal Giving Fund is a way of donating to Wolfram Syndrome **without** any fees being **deducted**, unlike many of the other fundraising platforms, including normal PayPal. The link for this page is paypal.com/gb/fundraiser/charity/76200.

PayPal Giving Fund



This QR code can also be used to make a donation to WSUK. Please feel free to share with friends and family.

Other Ways to Support WSUK

PayPal - Give at Checkout



Want to support WSUK when you shop online? Set us as your favourite charity, then click to donate £1 when you checkout with @PayPal for your purchases.

There are 2 ways to do this.

1. By using the link below, select us as your favourite charity and then log in to your account to complete the process.

paypal.com/gb/fundraiser/charity/76200

2. Log in to PayPal. Click " Set your favourite charity", type in our name Wolfram Syndrome UK. Confirm this is your favourite charity and we will appear on your homepage.

Make your online shopping count!



Want to help us raise more just by shopping online? Well now you can! It's very easy to do.

We are registered with online shopping portals [Give as You Live](#) and [easyfundraising](#).

Shop at your favourite stores and many more as usual, using one of these portals, and when you check out a donation from that store will be paid to WSUK at no extra cost to you. You can also raise funds when shopping for things like

holidays and insurance.

Links to register can be found [here](#); or you can click on the relevant image above to be taken straight through to the registration page. Install the reminder link so you will be asked if you want to raise a donation when you go straight to the retailer's website, so you don't need to worry about forgetting. Simple!

Sometimes it may be a few pence, other times it can be up to £40 for a retailer donation. Help us by signing up today!

WSUK Shop Page

As well as our WSUK branded items, which now also include a 330ml travel cup with lid, you will find some other items now available to purchase; all raising funds for WSUK. You can access the shop page [here](#).

Charity Christmas cards

There is still time to order your WSUK charity Christmas cards!

We have 4 new designs remaining which have been added to the 5 remaining designs from last year. Stock levels on a lot of the cards is now very limited!!

Each pack has 10 cards of the same design, with our logo inside and on the back. Each pack costs £3.00 with P&P starting from £2.95.



Unique Art Work

Unique pieces of art by Jennifer Lynch can be found on our shop page for you to purchase. If you would like to purchase a piece of art then please contact the office for P&P to be calculated and to make payment. Jennifer has donated the cost of materials so 100% of the sale price goes to WSUK.

Blank Greetings Cards

We have also added some blank greetings cards to the shop page. There are 5 designs, copies of Jennifer's paintings. Cards are available individually for £1.50 with P&P starting at £1.00 (dependent on weight or order). Please contact the office if you would like to purchase any.



You can access the shop page [here](#) for any of the items and more mentioned above.

WS clothing and accessories website

WSUK has created a new website for WS branded clothing and some accessories such as tea towels, bags and mugs. There is a link to this site from our shop page.

The products are made from organic cotton and printed in a renewable energy powered factory. When they are no longer wearable or wanted the items have instructions on how to send the product back to be re-milled and remade.

Delivery can be made to almost every country, just check the bottom of the website for delivery details and answers to any questions you may have. (At the end of each month they usually offer free UK P&P as well.)

WSUK will receive funds from the sale of each product. Currently we have a minimal range of products available. There are three logos to choose from - Wolfram Syndrome UK; WS Global Awareness Day for anyone that would like to help raise awareness every October 1st and the Wolfram Syndrome UK and Snow foundation joining together logo. The two organisations work closely together putting on the virtual global events and for the last two years the International Researchers Symposium.

Please send us any images of you wearing or using any of these products if you make a purchase.

Go to the website [here](#) where you can also read a bit about the company we are using.



Information about ways to raise funds can be found on our website [Funding Inspiration](#) page.

If you are arty or crafty and have something that we might be able to sell to raise funds then please let us know in the office. Or you could look to see about selling them at a craft fair as a way of raising awareness and funds. Let us know and send pictures so we can share this in future newsletters.

Your News

Please send in anything you would like to share with the WS community. The deadline for sending to be included in the Spring newsletter is **25th February 2025**.

Working whilst living with WS

Hi my name is Nat Costigan. I'm a young adult diagnosed with WS in 2018, and I believe it was discovered after genetic testing was done on a blood sample.

I had lots of support at school when growing up, with the use of an iPad and reasonable adjustments, and I always had my mind set (and was fully supported by those around me) on

going to university to study accounting and finance and then going to work in a job related to this.

I am currently on a commercial graduate scheme with SSE, I have a six months placement moving around departments in the group experiencing different businesses and roles. I am currently in my third placement in corporate finance within the enterprise business. My first placement was in court doing cost assessments of the Dogger Bank Windvane project, which is within the renewable business, and my second placement was within the energy markets business looking at using AI to develop validation tours. The company are being really supportive and I'm really enjoying it. My guide dog even has his own space in the office and the staff look after both of us.

In my spare time I enjoy participating in sport. In the para-triathlon this year, I came second in the British triathlon super series (behind the Paralympic champion). I competed in the swim England north-west regional summer championships and British summer championships (making finals at both), I am competing in the winter regionals in a couple of weeks and I'm hoping to qualify for the winter nationals.



Birthdays (UK and worldwide)



Happy birthday to everyone that has a birthday to celebrate during December, January and February.

We hope that you all have a great day!



Happy birthday to you all!!



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