

PWS NEWS



All the latest developments
from the world of PWS

Issue no. 163



www.pwsa.co.uk

Follow us for instant
updates:



@PWSAUK



Prader-Willi Syndrome
Association UK



@PWSAUK



@PWSAUK

What's inside

03 Welcome

04 Research News

06 PWS in Parliament

08 Support Team Updates

09 PWS Awareness Month

11 Fundraising

13 PWS Superheroes!

14 Events



PWSA UK
c/o Metcalf Commercial Decorators
3 Deer Park Road, Moulton Park,
Northampton
NN3 6RX



01332 365676



www.pwsa.co.uk



admin@pwsa.co.uk
supportteam@pwsa.co.uk

Royal Patron: HRH Princess Michael of Kent
Patron: Prof AJ Holland
Hon Chair: Danny Leslie
Hon Treasurer: Matthew Christensen

Meet The Team



Jackie Lodge

Chief Executive
jlodge@pwsa.co.uk



Karen Wilkinson

Fundraising Manager
kwilkinson@pwsa.co.uk



Vicki Cooke

Finance & Operations
Manager
vcooke@pwsa.co.uk



Patsy Lecont

PWS Specialist Team
Manager
plecont@pwsa.co.uk



Ruth Consterdine

PWS Specialist Team
Deputy Manager
rconsterdine@pwsa.co.uk



Sheila Inwards

PWS Specialist Advisor
sinwards@pwsa.co.uk



Sarah Brindle

PWS Specialist Advisor
sbrindle@pwsa.co.uk



Tracey O'Donnell

PWS Specialist Advisor
todonnell@pwsa.co.uk



Lizzie Pratt

Operations & Communications
Officer
lpratt@pwsa.co.uk



Myles Kelly

Project Manager
mkelly@pwsa.co.uk



Catherine Carey

Engagement Manager
ccarey@pwsa.co.uk

WELCOME

It's been a busy few months here at PWSA UK.

There has been exciting news from the US where the first drug for hyperphagia was approved by the FDA. This is a huge step for the PWS community but we still need to wait to see if this will be approved and funded in the UK.

You can read more about the next steps for this and all the other exciting developments in research on the following page.

We've enjoyed two community weekends - one in Scotland and one in Wales. There are more events happening over the summer and we hope that you will be able to join us.

These are wonderful opportunities to meet with other PWS families and build connections.

I've been visiting PWS care homes to hear from providers and the people with PWS who live there. We need to work together to ensure that care and support is tailored to the needs of adults with PWS.

We've gone orange and moved throughout May.

We've also taken some strong messages to Parliament. Thanks to so many families and members for writing to their MPs about this event.

This is the first step for us, not only raising awareness of PWS but working to improve services and support for everyone in this amazing community.

We worked with FPWR UK again this year on PWS Awareness month. FPWR UK focuses on advancing and funding PWS research, and we continue to work on care, information and support.

Our shared mission brings us together to ensure a better quality of life for everyone affected by PWS, both now and in the future.



As ever, none of this is possible without the fantastic fundraising efforts of our community, so a huge thank you to all of you.

We've had climbers conquering Snowdon overnight and runners completing the 26.2 miles in the London Marathon, and many, many more finding new ways to support our work.

We recently celebrated Volunteers Week and all the many ways in which PWSA UK is enriched through our fantastic volunteer team, so a big thank you to all our volunteers, too!

Please do get in touch if you want any more information about any of the articles in this edition or if you have any feedback. We always want to hear from you!

Jackie Lodge

First ever treatment for Hyperphagia in Prader-Willi Syndrome approved by the FDA



What happens next?

In March, the FDA approved VYKAT XR (diazoxide choline), previously referred to as DCCR, for the treatment of hyperphagia in adults and children 4 years of age and older with Prader-Willi syndrome in the US.

This marks a significant milestone for people with Prader-Willi Syndrome as it is the first approved medication option for the incessant hunger that affects people with PWS.

As yet, VYKAT XR has only been approved for use in the US, however, its creators, Soleno Therapeutics, have already started the process of seeking regulatory approval and funding to make the drug available in the UK.

Vykat has already been trialled in the UK and we worked with Soleno to ensure that our community were able to participate in this.

On May 22, Soleno reached another milestone in this process when the European Medicines Agency (EMA) validated their Marketing Authorisation Application for VYKAT™ XR.

This means the EMA will now review Soleno's application to make VYKAT XR available in the EU. Due to the UK's departure from the EU this currently does not include the UK, however this is still a very encouraging step towards making the treatment available to PWS patients beyond the USA.

What else is happening?

There are four other clinical trials of potential treatments currently happening in the UK:

Aardvark Therapeutics: ARD-101

Aardvark Therapeutics is conducting clinical trials to evaluate ARD-101, an investigational oral medication, as a potential treatment for PWS. ARD-101 is an orally administered small molecule designed to activate specific receptors in the gut, promoting fullness and reducing excessive hunger. By stimulating the release of hormones like CCK and GLP-1, ARD-101 aims to address the persistent hunger experienced by people with PWS.

Harmony Biosciences: Pitolisant

Harmony Biosciences is testing a drug called Pitolisant to help people with PWS who struggle with excessive daytime sleepiness (EDS). They are running clinical trials to see if this drug can help people stay more awake and alert during the day.

Consynance: CSTI-500

Consynance Therapeutics is developing CSTI-500, an oral medication aimed at treating PWS. CSTI-500 works by increasing levels of three brain chemicals – serotonin, dopamine, and norepinephrine – to help manage anxiety and hyperphagia in PWS.

ACADIA: ACP-101 (Compass Study)

The purpose of the COMPASS PWS study is to learn more about a study drug in participants with Prader-Willi syndrome (PWS) who have hyperphagia (excessive appetite).

The study drug being tested in this study is called carbetocin nasal spray (or ACP-101).

Carbetocin binds to oxytocin receptors with greater sensitivity than oxytocin, a naturally occurring hormone.

In the study, carbetocin nasal spray or placebo is inhaled through the nose three times each day.



For updates on all of the latest clinical trials and to find out which are currently recruiting participants please go to:

<https://www.pwsstrongertogether.co.uk/trials>

Filling gaps in our understanding of PWS:

By Professor Tony Holland

Help needed from adults with PWS

Research in PWS is much like completing a jigsaw puzzle - as pieces begin to come together and the last few pieces are put in place the final picture emerges.

In the case of PWS we are not there yet but I believe we are getting closer. Led by Alison Sleight at the University of Cambridge we are about to embark on a new project that may help complete the picture and ultimately inform future treatment development.

The big gap we seek to fill is to understand how the genetics of PWS affects the way the brain develops from when the baby with PWS is in the womb and then after birth. Our hypothesis is that in PWS there is a dysfunction of the mitochondria - these are the energy source ('batteries') present in every cell of the body. Mitochondrial dysfunction, if found to be present, would account for many of the unexplained symptoms experienced by people with PWS and it would offer a potential new approach to treatment.

For this study we need the help of people with PWS 18 years or older who would be willing to do some leg exercises whilst lying in an MRI scanner. The pictures taken would then show if the chemical signature that measures how the mitochondria work, is normal or not. The scanning is non-invasive and has no side effects. It would require a day trip to Cambridge - we would cover all the costs.



You could volunteer for both parts of the study or just the first part. It would be for the person with PWS to decide.

For those who are willing, a second part of the study is to take a very small sample of muscle for examination under the microscope. While MRS is powerful enough to provide substantial critical information, direct observation of mitochondrial structure and numbers in muscle cells using a high-powered electron microscope can offer additional insights that MRS alone cannot achieve.

If you have PWS and are over 18 years of age and might be interested in taking part or you think that someone with PWS you support might like to hear more about the study please contact the researcher on the project, who is known by the initials, LZ, using the study email (cuh.mitopws@nhs.net).

BETTER UNDERSTANDING = BETTER LIVES

Our campaign to improve awareness of PWS among professionals and policy makers.

With the right care and support, people with PWS can now live much longer and healthier lives. However, in too many cases, PWS is still being mismanaged - often with devastating consequences.

On Wednesday 14th May, we hosted an event for policymakers at the House of Lords to raise awareness of the challenges faced by people with PWS.

We know what good care and support looks like for PWS, but we need policymakers and professionals to understand this too, and to commit to implementing best practice.

At this event, we met with MPs, Peers and key stakeholders from across the health and care sectors to educate them about PWS, and engage their focus around three key challenges:

- Hyperphagia - the **persistent insatiable hunger** people with PWS feel and the implications this has for support needs and mental capacity.
- The absence of **warning signs** in a medical emergency - how serious injury and illness can be missed in people with PWS.
- The need for **specialist support** for people with PWS across all areas of life.

We summarised this for them in a simple awareness message to pass on and remember:

Prader-Willi Syndrome?

THINK!

Persistent hunger

Warning signs absent

Specialist support needed



P

Persistent hunger

Sadly, our support team receive far too many calls where the implications of hyperphagia for people with PWS have not been properly understood by professionals. This leads to inappropriate decisions about the care and support they need, leaving people in danger, with inadequate care.

We need all professionals to understand that without strict control of food intake, people with PWS are at serious risk of harm.

We also need them to recognise that people with PWS should never be deemed to have mental capacity around food.

This is being ignored in too many cases, with devastating consequences.



Charlie Gooding died in 2022, weighing over 200kg, after he was deemed to have the mental capacity to make his own decisions about food and money. Charlie had previously been living in a specialist PWS setting in Northampton, where his weight was well controlled, but he was unhappy living so far away from his friends and family in Manchester.

He moved back home and was discharged into the care of his GP, who deemed Charlie to have the mental capacity to make his own decisions about food and money. Charlie's weight escalated at an alarming rate but without a power of attorney in place, Charlie's family were not allowed to input into decisions about his care, and were powerless to intervene as his health deteriorated.

Charlie's mum, Annabel says *"Charlie strove for autonomy and independence, and wanted to be in charge of his life. He was very articulate, but in terms of understanding himself and what he needed to do to control his behaviour and appetite, he couldn't do those things. Nobody understood the seriousness of Prader-Willi Syndrome."*

W Warning signs absent

People with PWS have a unique set of medical vulnerabilities, meaning they may not present with typical **warning signs** in a medical emergency.

- Individuals may **lack a vomit reflex**, increasing the risk of poisoning and gastrointestinal rupture.
- A **high pain threshold** means injuries like fractures can go unnoticed.
- Impaired temperature regulation means **people may not develop a fever** when they have an infection, leading to missed diagnoses.
- People with PWS will **normally still eat and drink**, even when seriously unwell.

These atypical symptoms mean that **standard medical protocols often fail individuals with PWS**, sometimes with devastating outcomes.



Kate, (pictured above) was left permanently disabled and unable to walk after a double fracture and infection in her hip joint was missed by clinicians.

Despite complaining of pain in her leg, due to Kate's high pain threshold, she was dismissed without it being properly investigated. Her carers were told to try to make her walk on it and that it was probably psychological. Subsequently a large infection developed around the fracture site, which was also missed, as Kate wasn't presenting with a temperature. By the time the fractures and infection were discovered, it was so severe that surgeons had to carry out a girdle stone procedure, removing Kate's whole hip joint, and grafting her leg to her pelvis.

Kate also put on a large amount of weight in hospital after her surgery, despite her sister, Vicki trying to inform nursing staff about her PWS.

Kate's sister, Vicki says, *'Kate was a happy, loving, sociable person and she lived a full life. She loved walking the dogs, shopping, and socialising with friends and family. Now, all that's changed. All she can do is sit in her bed or her chair in her room.'*

If the medical professionals had fully understood the characteristics of PWS, my sister could still be doing all the things she loved. I feel so sad for her, I don't want this to happen to anyone else.'

We are now in discussions with NHS England to try and add red flags to medical notes to ensure that every clinician who sees someone with PWS is aware of these specific vulnerabilities.



S

Specialist support needed

Caring for someone with PWS needs specialist knowledge and understanding of the syndrome.

There is guidance for medical, educational and social care for young people with PWS. Unfortunately, in many cases this guidance is not adhered to. All too often this is because of a lack of understanding of PWS.

The cost of implementing best practice doesn't have to be more expensive - it should ultimately save costs as well as prevent further suffering for people with PWS as well as their families.

What are we asking for?

1: 24 hour support

We need professionals to acknowledge that everyone with PWS requires provision for 24hr support to keep them safe and enable them to thrive.

For children this includes access to respite and specialist wraparound care to relieve pressure on carers, as well as social care and support in education and employment for adults.

2: PWS care to follow best practice guidelines

Peer reviewed guidance exists and must be followed by professionals.

The consensus guidelines recommend that everyone with PWS is seen in a specialist multidisciplinary clinic, however, many people are not able to access these.

3: All adults with PWS to be able to access specialist residential settings

Evidence shows dramatically better outcomes for BMI and behaviours of concern in specialist PWS settings*. Unfortunately there aren't enough of these to enable access for most adults.

**Outcomes-for-persons-with-PWS-in-full-time-services Brian-Hughes*

4: Red flags added to medical notes

To ensure medical emergencies are spotted and fatalities avoided.

5: New medication needs to be approved and funded quickly

Several clinical trials of new treatments are happening - approval of new treatments will save money and lives.

The work of our specialist PWS support team

So far this year our support team have been busier than ever, working on nearly 500 cases.

The support they provide includes everything from advocacy work, helping people understand their rights and access the right care, to just providing a supportive listening ear.

This can involve signposting to relevant services, writing letters of support, helping to complete forms, attending meetings and tribunals, delivering training and delivering community events, and more.

Increasingly we are being asked to intervene in ever more complex cases involving mental capacity. These cases require significantly more time and skill to support.

Overview Jan-May



People Supported
494



Actions Taken
1030



Training Sessions
12



People Trained
234



P2P Zooms
4



P2P Attendees
24

We supported people with issues around:



Family Support



Medical



PWS Adult Support



Residential Care Issues



Benefits



General Information



Behaviour



Dietary



LPA & Deputyship



Education



Care funding



Many more...

PWS AWARENESS MONTH



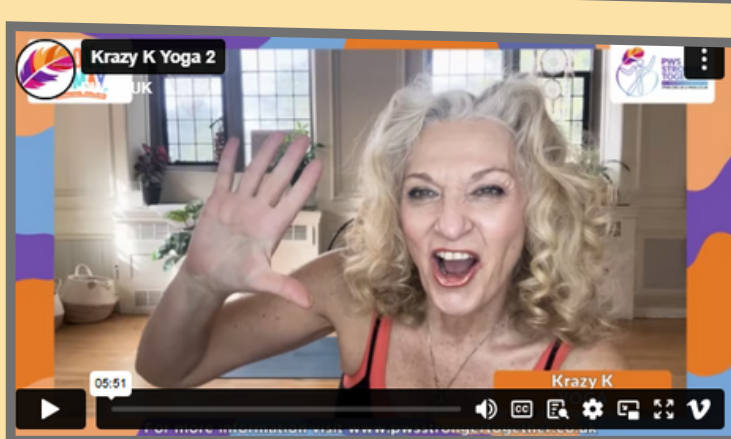
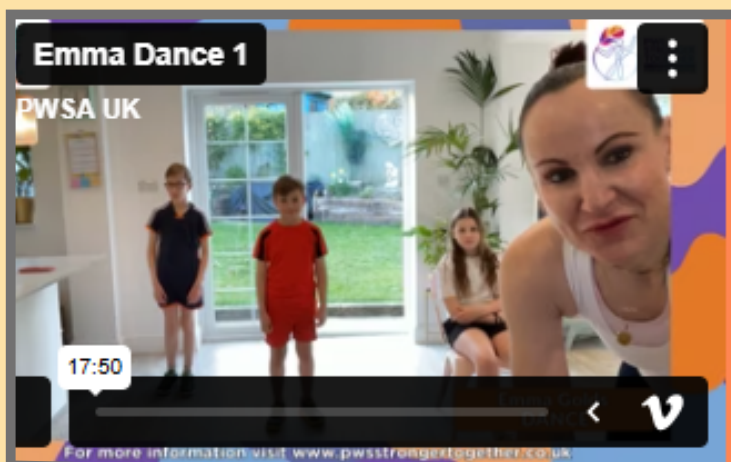
In May we celebrated PWS Awareness Month and this year, it was bigger and better than ever!

Lots of amazing families, schools, workplaces and people with PWS took part in our Move it May challenge, committing to getting active for at least 15 minutes a day.

All the funds raised will go towards vital PWS research, as part of PWS Stronger Together, our partnership with FPWR UK.

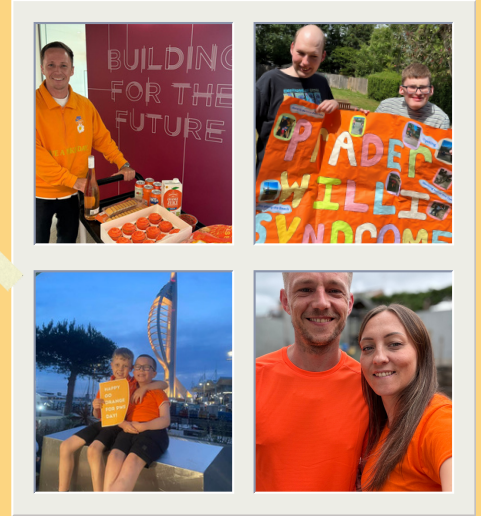


Fitness guru Lucy Wyndam-Reid, pilates master, Joakim, yoga teacher 'Krazy K' and PE teacher, Emma all curated special PWS friendly workouts for Move it May.



GO ORANGE FOR PWS

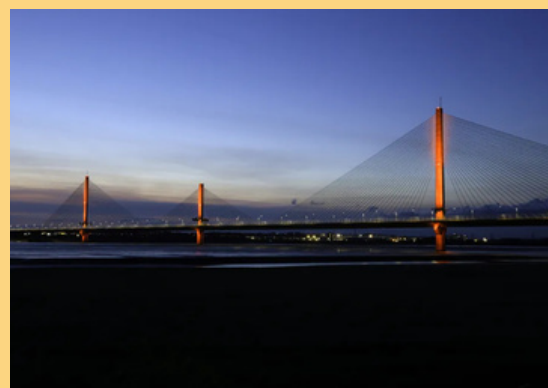
On 30th May we joined the global community in 'going orange', to raise awareness of PWS. We loved seeing all your pictures!



Some of the UK's most iconic landmarks even went orange, lighting up specially to raise awareness of PWS.



London's Tower 42, Green's Windmill in Nottingham, Cardiff Castle and The Mersey Gateway Bridge were among the buildings 'glowing orange' for PWS



This story was covered in the national press, reaching an estimated 157,000 people!



Fundraising

It's been an incredible few months for our fundraisers...

As always, we are in awe of the creativity and grit you put into raising much needed funds to support our vital work, thank you!



A heartfelt thank you to Boro Rangers Football Club for dedicating a special day to raising awareness for Prader-Willi Syndrome (PWS).

Josh and his family did an incredible job, raising an astounding £5,600 for PWSA UK! With over 500 attendees, a specially designed kit, and even coverage from local news, the event was a huge success in bringing attention to PWS!



Thank you to Natalie, who hosted a coffee morning in aid of PWSA UK, raising £531.20, and spreading much-needed awareness of PWS at the same time.

Proud PWS dad Jake took his fight with PWS to the boxing ring in Trilogy nightclub in Colchester. Thank you Jake, for raising £320 for PWSA UK. We hope it didn't hurt too much!



Kate and friends held a 'Fizz Friday' raising £500 ahead of her big 50km walk in July, serving fizz and food donated by the Crate campsite and café near Helston.

Snowdon at Sunrise

A team of 21 intrepid fundraisers took on the Snowdon at Sunrise challenge in April, setting off at 2:30 AM to summit Snowdon in time for sunrise.

The conditions were tough - cold and windy, but our team reached the summit just after 6 AM and were rewarded with a breathtaking view, watching the sun rise right over the horizon.

A huge thank you to everyone who took part to fundraise for PWSA UK, and to all who donated. Your support means the world.



London Marathon

8 amazing runners took part in a very hot and sunny London Marathon for PWSA UK this year. So far, their combined efforts have raised over £26k! Thank you to all of you for your incredible efforts - you are heroes!

Thanks to everyone who also came to support at mile 20, it's a great event and well worth going to cheer people on.



Dan raised over £2000 holding a quiz to aid his fundraising.



Well done Darren for completing the Manchester marathon which was the same day as London so rather warm. Darren raised over double his target reaching £1025!



Aiden ran Blackpool half marathon and raised £542 and also achieved his target time well done!



Siblings Anna and Aaron ran together and raised £246

Easyfundraising

Support PWSA UK Every Time You Shop – It's Easy!

Did you know you can help raise money for PWSA UK simply by shopping online? By using easyfundraising, every purchase you make with over 8,000 retailers – including your favourite brands – can generate a free donation for us, at no extra cost to you.

It's easy to get started: just sign up and activate the easyfundraising browser extension. With one simple click, you'll be supporting our work every time you shop.

Thanks to our amazing community, we've raised £10,081.99 so far, with 422 supporters. In the last quarter alone, you helped us raise £286.85 – just by shopping as normal!

If you haven't signed up yet, now is the perfect time to join and make a difference with every purchase.



How to Sign Up to easyfundraising

Step 1: Visit our page

Go to our dedicated easyfundraising page:
<https://www.easyfundraising.org.uk/causes/pwsa>

Step 2: Click “Support this cause”

You'll see a button that says “support this cause” – click it to begin the sign up process.

Step 3: Create your account

You can sign up with an email address or through Facebook or Apple. It takes just a minute.

Step 4: Install the Donation Reminder (optional but recommended!)

When prompted, install the Donation Reminder browser extension. This clever tool pops up whenever you're on a site that supports easyfundraising – so you never miss a chance to raise money!

Step 5: Start Shopping!

From big brands like John Lewis, M&S, eBay, and Booking.com to everyday favourites like Tesco and Argos – just shop as normal. Each time, a small percentage will be donated to PWSA UK – at no extra cost to you.

Step 6: Track Your Impact

You can log into your easyfundraising account anytime to see how much you've raised.

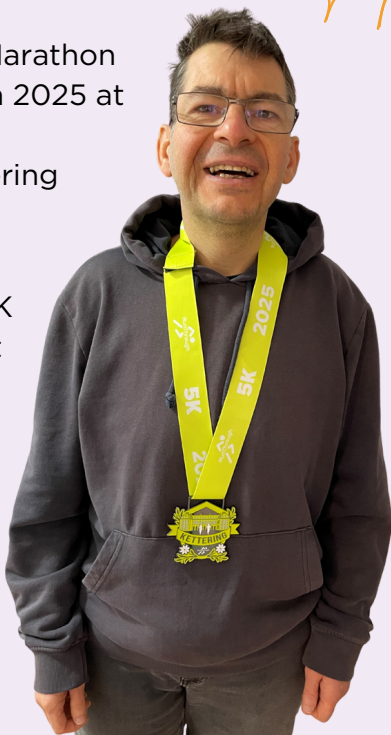
PWS Superheroes! Amazing people doing amazing things

Jameel's 5k

The first-ever Kettering Half Marathon took place on Sunday 9 March 2025 at Wicksteed Park, organised by RunThrough Events and Kettering Town Council.

Among the participants was Jameel, who completed the 5K run after deciding to take part just ten days before. With a few practice runs—first with staff and then independently—he rose to the challenge.

Supported on the day by a staff member, Jameel finished the run proudly and was thrilled with his medal. A fantastic achievement!



Congratulations!

Congratulations to Jodie and Jake who took the plunge and got engaged recently following a very romantic proposal!



Disco Fever

For the past ten years, Bonnie Dunion has been the driving force behind monthly discos for adults with learning disabilities in Northamptonshire. What began as a one-off event has grown into a regular highlight in the local calendar, with attendees eagerly counting down the days.



Inspired by her mum and stepdad (our very own Myles), who have both supported people with Prader-Willi syndrome throughout their careers, Bonnie wanted to create a fun, inclusive space where everyone could feel welcome.

Thanks to her dedication, these events have become more than just discos - they're a place for connection, confidence, and community.

From all of us at PWSA UK: thank you, Bonnie, for ten amazing years of joy.

Tommy's Rugby

Well done to Tommy Cunis who recently took part in a mixed ability rugby tournament and loved it - Go Tommy!



Community Events



It's only June, but we've enjoyed two wonderful community weekends already this year!

Seton Sands

Back in April we got together at a new location in Scotland, Seton Sands. We hit the beach and the beautiful nature trail by day, then partied all night in the entertainment lounge.



Ty Mawr

In May, over 100 of you joined us at Ty Mawr in Wales for a fun-filled few days of crabbing, beach adventures, miniature railway rides, arcade games, and evening entertainment - and we loved every minute of it!



Join us at a community event

Our community events are a great way to meet and spend time with other families in a supportive, PWS friendly environment.



Community Day: Northern Ireland

Join us for a fun-filled family day at the Stormont Hotel, Upper Newtownards Road, Belfast.

Get creative with a fun Arts & Crafts session hosted by Flourish, enjoy a chance to connect with other families and take a gentle nature walk or enjoy free time in beautiful Stormont Park.



Community Day at MOD Lyneham

Spend time with others with PWS and make new connections at our community fun day.

Join us for a fun-packed day of activities for all ages and abilities, including a PWS friendly obstacle course!



**FOR MORE
INFORMATION
OR TO SIGN UP
FOR AN EVENT,
HEAD TO OUR
[EVENTS PAGE](#)**



Camber Sands Weekend and Community Day

Come and join us for a beach-side weekend at Camber Sands Holiday Park.

Our fun-packed programme includes crafts and creative activities, gentle nature walks and plenty of time to unwind and connect with others.

If you can't come for the whole weekend but would still like to meet up, we're having a Saturday afternoon get together at Camber Memorial Hall.



Yorkshire and Humber get together

Come and meet up with other families for an afternoon at Cawthorne Village Hall.

We've arranged a fun and inclusive afternoon of activities including Zumba, arts and crafts and an introduction to BSL!

