



## CILIAREN – New National Approach to PKD Patient Care

We're delighted to be the patient charity partner of CILIAREN, a major new national clinical and research network funded by the Medical Research Council (MRC).

CILIAREN is a collaborative effort between clinicians, scientists, and patient groups to improve the diagnosis and care of patients with 'renal ciliopathies', including polycystic kidney disease (PKD), and accelerate the development of new treatments.

Renal ciliopathies are a group of rare inherited kidney diseases, accounting for around 1 in 10 of all patients with kidney failure. Both autosomal dominant PKD (ADPKD) and autosomal recessive PKD (ARPKD) are in this group, together with other conditions such as Nephronophthisis (NPHP) and Bardet-Biedl Syndrome (BBS). Recent advances in genetics and cell biology have provided important insights into these diseases, but there is a significant unmet need for treatments that can prevent or cure them.

CILIAREN aims to improve patient care nationwide by harmonising clinical, imaging, and molecular genetic work-up, improving interpretation of genetic variants, and developing personalised therapies and care plans based on individual requirements and genetic diagnosis. Partnerships with industry will be established and a national system of support for patients and families will be created.

## New Research in Polycystic Liver Disease (PLD)

**Scientists have discovered cell signals that could possibly be intercepted to stop liver cysts growing in polycystic liver disease.**

Dr Scott Waddell, one of the scientists from The University of Edinburgh said: "Research into liver cysts has been lacking and little is known as to what drives liver cyst growth. Our new work tries to plug this gap and understand if there are ways to stop liver cysts growing. We have identified two exciting routes to stop the growth of liver cysts and hope this has the potential to impact the lives of ADPKD patients."

The main genes involved in PLD make proteins that interfere with hair-like structures called cilia on cells lining tubes in the kidney and liver. Without functioning cilia, the cells switch to a new way of signalling, using a protein called TGF $\beta$ . This causes them to make extracellular matrix, which acts as scaffolding for new cells being constructed.

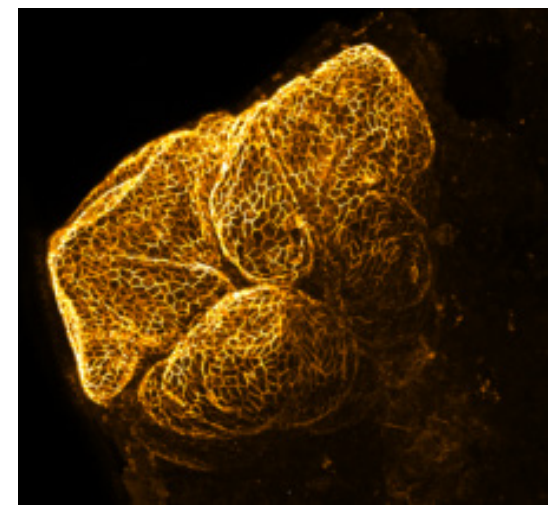
The research team used genetic engineering to stop the cilia working in the livers of mice, mirroring what happens in ADPKD. They blocked the TGF $\beta$  signals using an inhibitor, the matrix was not made, and the cysts stopped growing! They discovered that the same TGF $\beta$  signals occur in liver cells from people with PLD. When they

inhibited TGF $\beta$  signalling, the cyst cells stopped growing in the lab.

The team also found that cyst cells make a protein called integrin which helps them to detect the extracellular matrix. When they blocked integrin with an inhibitor, this also stopped cyst growth!

So, the researchers think that TGF $\beta$  inhibitors and integrin inhibitors could be potential treatments for PLD.

TGF $\beta$  inhibitors and integrin inhibitors are already being used, or tested, for some other conditions. They'll need to be researched further and carefully tested in clinical trials before they could become available as a treatment for PLD.



Microscopy reveals the structure of polycystic liver disease cysts  
(©Dr Scott Waddell)

# Information and support

## Living well with PKD

### The Lowdown on PLD

More and more people are finding out they have liver as well as kidney cysts. Here are some answers to questions that we often get asked at the charity.

#### Does everyone with ADPKD get liver cysts?

By age 30, about 9 in every 10 people with ADPKD get cysts in their liver, known as polycystic liver disease, or PLD. Some never get PLD though. Learn more about PLD [www.bit.ly/adpkd-pld](http://www.bit.ly/adpkd-pld)

#### Do liver cysts cause liver failure?

Liver cysts rarely cause the liver to fail. This is because they form in the tubes of the liver, not the liver tissue where the liver does most of its work.

#### Does PLD cause any other liver changes?

Yes. PLD can make the bile duct larger. This is the tube that carries bile (a digestive juice) from the liver to the gut.



#### If I get PLD, will it make me unwell?

Most people with PLD don't have symptoms. If you get many cysts, your liver may get much larger. This can make you feel bloated. It can be uncomfortable, cause pain, breathlessness and affect body image. About 5% of people with PLD get complications that need treatment.

#### How will I know I have PLD?

Most people find out they have PLD after a scan is done as part of their ADPKD follow up or to check for tolvaptan eligibility.

#### Do liver problems ever need surgery?

It is uncommon to need surgery due to PLD. If you have a problem liver cyst, you might need a procedure to drain it. Less often, people with PLD have a section of their liver removed or a liver transplant.

#### Do people with ARPKD get PLD too?

ARPKD also affects the liver, but in more ways than ADPKD does. Sometimes, these changes affect other areas of the digestive system too. Learn more about ARPKD and the liver [www.bit.ly/arpkd\\_liver](http://www.bit.ly/arpkd_liver)

### Matching for Transplant Q&A

We recently welcomed Dr. Judith Worthington, Principal Clinical Scientist at Manchester Royal Infirmary to a live online session on matching for transplant. Judith's presentation offered a special opportunity to learn more about tissue typing and antibodies, and their crucial role in the organ donation matching process.

To view this recording and talks from other events, please visit: [www.bit.ly/PKDevtalk](http://www.bit.ly/PKDevtalk)

Other recorded presentations include the topic of Transplant and Living Kidney Donation.

**Matching for Transplant**  
A guide to Compatibility and Blood Tests!

**Recording now available!**

Dr Judith Worthington  
Principal Clinical Scientist

*"Extremely well presented, factual & gave insight into the various factors involved in process."*

*"Information was easily understandable and not overly technical."*

*"Clear explanations."*



### Coming soon!

#### PLD Research Q&A

Thursday 30 November 2023

#### Kidney Beam Live Session

Wednesday 6 December 2023

#### ARPKD Family Day

London - Saturday 27 January 2024

#### ADPKD Information and Support days

- Newcastle upon Tyne – Saturday 10 February 2024
- Wales – Spring 2024, date to be confirmed

For more information, please visit [www.bit.ly/pkdc-events](http://www.bit.ly/pkdc-events)

# Information and support

## Living well with PKD

Our volunteer led PKD Connect Groups give you and your loved ones the chance to connect with others, chat, and discuss issues over a cuppa in a relaxed, informal setting.

There's no pressure, of course. Whether you're outgoing or more reserved, simply being among people who understand what you're going through can bring great comfort.

Most groups meet on Saturdays or Sundays and last for two hours, but you can just pop in for a quick chat or stay until the end. Currently, we have in-person groups in Bedfordshire, London, Newcastle, and Norfolk. Online groups are available for people in Wales and for those at the 'low clearance' stage of their PKD journey. We hope to establish groups soon in Sheffield and Manchester with the help of volunteers.

If you are interested in joining a group visit [www.bit.ly/pkdc-events](http://www.bit.ly/pkdc-events) and register. If you want to know more or are interested in starting a group in your local area, email Susan at [susan.muirhead@pkdcharity.org.uk](mailto:susan.muirhead@pkdcharity.org.uk) or call, text, WhatsApp her on 07739 632836.



"I joined the low clearance group around a year ago. Sharing experiences about PKD specific issues is so helpful. You are with people who truly understand our day to day lives. Nothing is off the table, from transplant, dialysis, itching, holidays and even advice on clothes for a wedding. A truly supportive and encouraging group." **Sue, West Sussex**

"It's a great way to compare notes with others that are going through a similar journey as I don't have this elsewhere. Everyone genuinely cares and shows interest in the rest of the group, it would be great if the group can continue growing." **Rob, Cambridgeshire**



Who	Eligibility	Autumn Booster	When & How
Adults aged 65 years and over	All	✓	From 3 months after last Covid vaccination, starting in mid-September 2023 and completing by early December 2023
Adults/children aged 16-64 years	<ul style="list-style-type: none"> <li>Chronic kidney disease (CKD) stages 3, 4 or 5, chronic kidney failure or nephrotic syndrome</li> <li>Dialysis patients</li> <li>Transplant patients</li> <li>Immunosuppressed individuals</li> <li>Household contacts (aged 12 and over) of an immunosuppressed person</li> <li>Carers (receiving carer's allowance or primary carer of an elderly or disabled clinically vulnerable person)</li> <li>Living in a care home for older adults</li> </ul>	✓	<p><b>England</b> – The NHS will contact you if your NHS record suggests you may be eligible. Those eligible can use online booking, the NHS App or call 119 from 18 Sept</p> <p><b>Wales</b> – wait to be invited by your Local Health Board</p> <p><b>Scotland</b> – wait to be invited by email, text or letter. If not registered with a GP, call 0800 030 8013 to register for a vaccine and receive an invitation</p>
Children aged 6 months-15 years	<ul style="list-style-type: none"> <li>With a chronic kidney condition</li> <li>Immunosuppressed individuals</li> <li>Household contacts (aged 12 and over) of an immunosuppressed person</li> </ul>	✓ (Age-appropriate dose)	<p><b>Northern Ireland</b> – make yourself aware of your own GP surgery's or community pharmacist's vaccination arrangements and work with them to be vaccinated</p>



### KIDNEY PATIENTS COVID-19 AUTUMN BOOSTER GUIDE.

## Get Winter Ready!



As summer becomes a distant memory and colder days approach, we urge eligible individuals to get their booster vaccine.

Winter is typically the time of greatest threat from respiratory infections, and vaccination remains a key defence against severe Covid-19 illness.

You might qualify for both flu and Covid-19 vaccines, and it's safe to receive them simultaneously.

For the complete Kidney Patients' Covid-19 2023 autumn booster guide from The Kidney Charities Together Group, visit: [www.bit.ly/Aut23Boost](http://www.bit.ly/Aut23Boost).

# Raising awareness

## PKD Voices



## Jonathan Scores Big for PKD Awareness!

**Meet 11-year-old Jonathan, a passionate sports enthusiast, determined to shoot for the stars while raising awareness for PKD.**

Hailing from Oxfordshire, he plays in the under-12s Junior Premier League for Oxfordshire Skills Academy and is the driving force behind adding the PKD Charity logo to his team's kits

Jonathan's life took an unexpected turn when diagnosed with PKD (ADPKD) during an unrelated medical test, which was a big shock to his parents, as neither had PKD. ADPKD is typically inherited from an affected parent, with a 50% likelihood of being passed on to each child. Yet, in rare instances, a "spontaneous" mutation causes ADPKD in children born to healthy parents, occurring in about 1 in 10 cases.

Undeterred, he refuses to let PKD hinder his love of sport, which includes motocross, cricket, and skiing, as well as the 'beautiful game'. To protect his kidneys, he wears a kidney belt, following advice from his kidney consultant, Dr. Manish Sinha of Evelina Children's Hospital.

Unsurprisingly, Jonathan is the apple of his parents' eyes. As his mum, Zoe, says, "We're immensely proud of how Jonathan has bravely shared his condition at such a young age, working toward a positive impact that may one day lead to a PKD cure."

We're certain Jonathan's star will continue to rise, on and off the field.

## A Classic Tribute to a Beloved PKD Community Member

**In August, we participated in Classics Over Sands, a cherished tradition in the South Lakes since 1995. Located in the charming Edwardian town of Grange-over-Sands, the event features 300 vintage vehicles and family-friendly attractions overlooking Morecambe Bay.**

While the event draws car enthusiasts and fosters community spirit, its true essence shines through in supporting worthy causes.



This year held special meaning as we were chosen as one of the selected charities by GP Dr. Katharina Frey, in memory of her late husband, Dr. Simon Fisher, with whom she ran Coniston Medical Practice. Notably, Katharina drove their beloved 1950s Messerschmitt micro car to and from the event. This quirky vehicle,

a symbol of their shared enthusiasm, added a unique charm to the occasion.

Beyond the joy of the day and the opportunity to connect with the PKD community, the event also raised a remarkable £3,000 in support of our cause.



Derek Seward, Chairman of the Classics Over Sands car show, presenting the cheque for £3,000 to Katharina.

# Raising awareness

## PKD Voices

# Transplant Games Triumph in the Heart of England



Coventry played host to the 46th British Transplant Games this year, an extraordinary annual event that brings together athletes of all ages, ranging from 2 to 80+. Since its inception in 1978, the Games have embodied resilience, unity, and the life-altering impact of organ transplantation - a testament to the generosity and bravery of donors and their families.

Among the many participants were our very own Tess and Jane, who enthusiastically engaged in the Donor Run, setting their own brisk walking pace. Liz from Manchester, eagerly awaiting a transplant, achieved an impressive speed in her first Donor Run, marking an extraordinary personal milestone.

Meanwhile, Games veteran Andy Taylor, representing Great Britain and Northern Ireland at this year's World Transplant Games in Australia, added to his impressive medal collection by securing gold in badminton and table tennis.

Despite a downpour on the second day, spirits remained undeterred. Donors, donor families, and recipients pressed on, determined to make their mark. The Coventry 2023 British Transplant Games once again highlighted the transformative power of transplantation and the unwavering determination of those embracing their second chance at life.



# Fundraising

## Get involved

### Shop Now for your Christmas Cards

Our 2023 range of Christmas cards are now available to order. We have 10 lovely new designs on offer as well as some old favourites, and this year we are also offering a range of other Christmas essentials including gift wrap, labels, crackers and MORE!

#### Order yours now

- By post using the enclosed order form
- You can place an order over the phone by calling 01227 811669
- Or visit [www.charitycardshop.com/pkd](http://www.charitycardshop.com/pkd) to order

Every purchase from our Christmas shop helps fund groundbreaking research and support for people affected by PKD.



# Farewill

## Claim your free Will with Farewill

PKD Charity has teamed up with Farewill, one of the UK's top Will writing specialists, to offer you free support with writing or updating your Will.

You can write your free Will in as little as 15 minutes with their professional help. Although PKD Charity is covering the cost, you don't have to leave us a gift to use the service.

By including a gift in your Will, you can help our scientists to build on today's discoveries and develop tomorrow's.

Write your Will for free here:  
[www.farewill.com/pkd-newsletter](http://www.farewill.com/pkd-newsletter)



# Fundraising

## Get involved

### Team PKD Raise Over £14,000 at the Great North Run

A big thank you to our wonderful team of runners who took part in the Great North Run in September. Alex, Marc, Paul, Chris, Rob, Katherine, James, Jack, Daniel, Craig, Joan, John, Patrick and Grace.

It was a tough day on the course with very hot running conditions before the torrential rain arrived, but our team did it and they have now raised over £14,000 to help support people affected by polycystic kidney disease and fund life-changing research into finding treatments and a cure for PKD.

If you want to be part of the action next year, email [fundraise@pkdcharity.org.uk](mailto:fundraise@pkdcharity.org.uk) to register your interest in a charity place with us in the Great North Run 2024.

57,000 determined and dedicated runners make the Great North Run the world's biggest and best half marathon – famous for its warm North East welcome and unbeatable atmosphere.



# Our support services

## Helping you cope with PKD

Whether you're looking for PKD information, practical advice or just someone to talk to, we're here to help you.



### Ring our Helpline

If you need a friendly person to talk to about your worries or are just looking for information, ring **0300 111 1234**, our confidential helpline. Available from 9:30am to 5:00pm Mon – Fri.

"You'll never know how much I valued speaking to you."



### Find a PKD Support Group

For understanding and companionship, join one of our PKD Support Groups. Hosted by trained volunteers with lived experience of PKD. Everyone is welcome – patients, family members and friends.

"It's great to meet others in a similar situation."



### Join a Facebook Group

For a friendly and private place to connect and share with others like you, join one of our three UK-only private Facebook groups. There's a group for everyone affected by PKD.

For ADPKD (autosomal dominant polycystic kidney disease)

- PKD Support (PKD Charity UK)
- Parents of children with PKD (PKD Charity UK)

For ARPKD (autosomal recessive polycystic kidney disease)

- ARPKD Support (PKD Charity UK)

"I found the group on Facebook and was immediately immersed into a very friendly and welcoming environment."



### Take part in a workshop

Share your experiences and discover new coping strategies in a confidential online space with others

who understand the difficulties of life with PKD. Run by a renal psychologist and psychiatrist.

"To be able to share experiences helped me realise I am not the only one out there feeling the way I do."



### Learn more about PKD at an educational event

Want to find out more about PKD, worried for your future, or baffled by the medical jargon? Ask the experts at one of our free educational events.

"Fantastic sessions...a very informative and interesting day."

### Visit our website for more information

How to get support, visit [www.bit.ly/pkd-support](http://www.bit.ly/pkd-support)

Upcoming events, visit [www.bit.ly/pkdc-events](http://www.bit.ly/pkdc-events)

## PKD app

Download our **FREE App**, created to inform and educate people who are newly diagnosed or at early stages of PKD.

Scan here



## PKD Health Information

If you need reliable information about all aspects of PKD, visit our website for a range of accredited factsheets written by medical experts.

Key factsheets include:

### ADPKD Basics

[www.bit.ly/adpkd-basics](http://www.bit.ly/adpkd-basics)

### Polycystic Liver Disease

[www.bit.ly/adpkd-pld](http://www.bit.ly/adpkd-pld)

### Diet and Lifestyle in ADPKD

[www.bit.ly/adpkd-diet-lifestyle](http://www.bit.ly/adpkd-diet-lifestyle)

### ARPKD Basics

[www.bit.ly/arpkd-basics](http://www.bit.ly/arpkd-basics)

If you don't use the internet, we can send you printed copies. Just ring our helpline: **0300 111 1234**.

## Links to other sources of help

We recommend:

UK Government Benefits Info  
[www.gov.uk/browse/benefits](http://www.gov.uk/browse/benefits)

Kidney Care UK - individual grants  
[www.kidneycareuk.org](http://www.kidneycareuk.org)

Turn2us – broad range of advice on welfare benefits and grants  
[www.turn2us.org.uk](http://www.turn2us.org.uk)

Family Fund – grants for seriously ill or disabled children  
[www.familyfund.org.uk](http://www.familyfund.org.uk)

Contact - for families with disabled children  
[www.contact.org.uk](http://www.contact.org.uk)