14 May 2025 MW

PSC Support Research Strategy 2025-2030

Our vision is to see a world without PSC.

PSC Support is the only patient organisation in the UK dedicated to helping people affected by PSC. We do this through funding and supporting vital research, sharing trusted information, providing support, and working to improve healthcare for everyone with PSC.

Our research goal is to better understand PSC and turn discoveries into real tools and treatments faster - helping people with PSC to live well and for longer.

Our Strategic Research Priorities 2025-2030

As a small charity with ambitious goals yet limited resources, our efforts, people and funds are best focused on **breaking down barriers to research** and **speeding up research** to develop effective treatments, and making sure everyone with PSC gets **access to high quality healthcare** that's **built on strong evidence**.

Our research priorities are to invest in research that will:

Unlock understanding

Generate fundamental scientific knowledge about PSC

This means supporting research that **uncover the processes that lead to PSC** and to **establish biomarkers, tools and simple, noninvasive tests** that can

a) be used as **endpoints** in clinical trials
b) **diagnose** PSC (including recurrent PSC after transplant) quickly and with certainty
c) **monitor** disease progression effectively
d) **detect cancers** early and with certainty
e) reliably **predict** disease progression and complications

Accelerate treatment development

Fast-track the development of effective treatments

This means championing innovative trial design and supporting clinical trials to test **new and existing drugs** (those already in use for other diseases) in people with PSC to

a) slow or stop disease progression
b) manage symptoms and complications
c) reduce cancer risk
d) improve quality of life

Promote personalised care

Make healthcare work for people with PSC

This means supporting

a) **the development of evidence** underpinning recommendations in clinical practice guidelines and expert consensus statements
b) the development of tools to **assess symptom burden and quality of life**
c) healthcare professionals to share **timely and compassionate delivery and discussion of diagnosis, symptoms, disease risk and test results**.
d) **access to psychological services** for people with PSC

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Our Research Goals 2025-2030

Over the next five years, we will:

- invest in the highest quality research delivered by the best research teams

Why	With limited funds, we must focus our resources on research that has the promise to yield **the greatest benefits in the shortest time in the most robust way**.
How	We will offer an annual research funding programme. PSC Support is a member of the Association of Medical Research Charities, an indicator of **excellence in research governance and standards**. Research we fund is reviewed by well-respected medical professionals and patients to ensure precious funds are allocated to the highest quality research that will make a positive difference to the lives of people with PSC.

- strengthen relationships with industry, academic institutions, regulators and patient organisations, and stimulate innovative thinking

Why	No single researcher or team has all the tools and knowledge needed to develop effective treatments for anyone with PSC. To achieve this, stakeholders from across the board (beyond the world of PSC) must bring their **collective skills, insights and experience together**.
How	We will work with **stakeholders from across the scientific and patient community** to stimulate partnerships, bridge knowledge gaps, advocate for the needs and priorities of people with PSC, and encourage multidisciplinary research.

- champion the voice of people with PSC to influence and shape PSC research

Why	We have a track record of exemplary patient involvement in research through our Scientific Review Committee and Patient Panels**. Research with strong patient involvement leads to better quality studies, improved health outcomes, and increased relevance of research findings**.
How	We will provide even more opportunities to a **diverse group of people with PSC** to support researchers and make sure their work really **reflects what matters most** to those living with the condition.

- support recruitment to clinical trials and studies

Why	**New medicines for PSC can only be developed through clinical trials**—and these depend on people with PSC getting involved.
How	Everyone should be considered for a clinical trial as soon as they are diagnosed with PSC, and taking part should be as easy and stress-free as possible. We'll keep **sharing clear, reliable information about clinical trials** - what they are, where they're happening, and what taking part involves. We'll also continue to support researchers by **giving feedback on study design, trial plans, and how to reach the right people.**

Disease areas: Primary sclerosing cholangitis (PSC), recurrent PSC (rPSC), cholangiocarcinoma (CCA) with PSC, inflammatory bowel disease with PSC (PSC-IBD).

Why we focus on research

“Primary sclerosing cholangitis represents the greatest unmet need in liver medicine.” Dr Palak Trivedi, February 2022.

PSC was first described nearly 160 years ago in 1867, and despite extensive research, there is so much more we need to understand. This is because PSC research is HARD. Why?

We don’t yet fully understand how or why PSC develops

- There is likely to be because there are multiple processes at work. We may need multiple treatment drugs to address these different processes. **We need to keep an open mind about the path to a cure.**

Finding enough of the right people for trials is a challenge

- Many people with PSC are keen to take part in research, but **not everyone is eligible**. Researchers need to compare individuals with similar characteristics of the disease, which is difficult because **PSC varies so much** and individuals often have multiple health conditions. Some people also have inflammatory bowel disease or other autoimmune diseases, some don’t. Different parts of the bile ducts can be affected, and while some people experience severe liver damage, others don’t—and we still don’t know why.
- Because **PSC is rare**, there aren’t large numbers of people ready to join clinical trials. This means it can take a long time to find enough volunteers to test new treatments.

PSC can take years to progress

- That means proving a new drug works to slow it down could also take years. To speed things up, **researchers want to use tests or biological markers that are reliable indicators of future progression.** The challenge is, we don’t yet have trusted tests like this for PSC. Without them, clinical trials become difficult—because we can’t confidently assess whether a treatment is working without running long and expensive studies that last decades.

A diagnosis of PSC can take time and may not be definite

- Diagnosing PSC depends on how individual healthcare professionals interpret scan and other test results. This can lead to delays, with **some people waiting years for a diagnosis or being left uncertain,** told they 'might have PSC' without a clear answer.

Tests to screen for cancer are not good enough

- Inadequate screening leads to **late cancer diagnoses and delays to vital treatment**.

There is no reliable way to monitor quality of life

- Even biological tests don’t reflect how someone **is really feeling day to day**.

Funders need to see evidence that research ideas have potential

- It is almost **impossible to secure funding for large research studies** without early evidence that the study is likely to lead to positive results.

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Are we making progress?

Yes we are!

New technologies

Researchers are taking advantage of **new technologies** to better understand how PSC develops and what biological changes are involved. These breakthroughs are opening the door to new treatment possibilities.

Promising biomarkers

There are **promising new biomarkers** on the horizon - tools that could help diagnose PSC earlier, detect bile duct cancer sooner and even predict who might experience PSC returning after transplant (rPSC)

Better clinical trial design

Thanks to **smarter trial designs** for rare diseases, fewer people are given placebos (inactive drugs), and more receive the actual treatment. Some trials now measure treatment success (‘efficacy’) based on real-life outcomes - like fewer hospital visits or complications—rather than just lab results.

Faster trials

Researchers are testing existing drugs (used to treat other diseases) to see if they help in PSC. **This is called drug repurposing.** These have huge potential to accelerate research. Developing a brand-new treatment can take 10 to 15 years, but we’re finally seeing some **PSC drugs reach the final stages of testing**.

There’s real hope on the horizon—though there’s still work to do.

Mission 2030:

help us find a breakthrough
in PSC research

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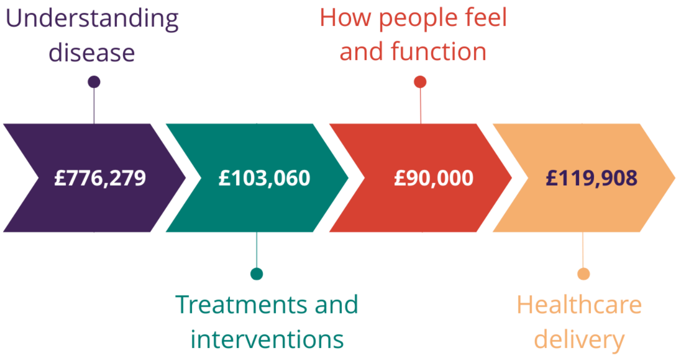
How we fund research

As at April 2025, PSC Support has committed £1.1million to PSC research. This funding has supported 19 research projects to better understand PSC and its complications, improve care, quality of life and find a cure, and we're not about to stop.



One of our biggest priorities is funding high-quality research to improve the lives of people with PSC. We're proud to be a member of the Association of Medical Research Charities (AMRC), which means we meet high standards for how we manage and support research.

PSC Support provides research grants that address important and novel, basic, clinical or social research questions relating to PSC, recurrent PSC, PSC-IBD and bile duct cancer. The questions must be aligned with our Research Strategy. We offer:

- **Seed funding awards** - to generate early evidence that ideas are promising. This evidence allows researchers to apply for grants for much larger studies.
- **Project awards** - larger 2-3 year studies.
- **Research Training Fellowships** (3 year PhD clinical fellowships in PSC) to help develop the careers of future PSC specialists and to fill the critical gaps in our knowledge about PSC.
- Bid preparation awards - to support researchers to apply for funds for larger studies and clinical trials.

The funding applications include details of the research they want to do and how much funding they need. **Every application is carefully reviewed by several experts** - at least two scientific and one with lived experience of PSC. They look at things like the quality of the science, how original and relevant the idea is, and whether it offers good value for money.

Next, our **Scientific Review Committee**—made up of researchers, clinicians and patients—reviews the feedback and makes recommendations to our **Board of Trustees,** who then decide which projects to fund.

Experts send detailed reviews on applications to the Scientific Review Committee (SRC)

The SRC reads all applications and reviews and sends a report to the Trustees

The Trustees make the final decision

It's a thorough process, making sure **every penny we invest supports research that truly matters to people with PSC**.

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How we support research

At PSC Support, **we do more than fund research**.

We have a track record of exemplary patient involvement in research, making sure it's shaped by the real experiences of people affected by PSC. This is called PPIE (patient and public involvement and engagement), and it's something we're proud to lead on.

Our **Patient Panel** is made up of people living with PSC and family members who generously share their time and insight. They aren't scientists, but they are experts in what it's like to live with PSC—and that perspective is essential.
Here's how we support researchers through PPIE:

- review academic and commercial clinical trial protocols to make sure they're practical, inclusive and as easy as possible for people with PSC to join and stay in.
- advising on other elements of trial design such as endpoint choice and which Patient Reported Outcome Measures are most suitable for the study.
- writing or editing plain english versions of published research publications, sections of funding application forms and patient facing trial documents.
- co-authoring manuscripts, abstracts and posters where we have been involved in the research.
- helping researchers to develop applications to request research funding from other funding bodies - in 2024, we helped secure £14 million in this way!
- governance on trial steering committees, trial management groups and national and international PSC and rare disease research groups.

Why does this matter?

Because research that's shaped by patients leads to **better quality studies,** increased relevance of research findings, an increased likelihood of securing large scale funding and ultimately, improved health outcomes for people with PSC. By keeping the patient voice at the centre of all our research activities, we make sure PSC research truly reflects what matters most to the people living with it.

We need your help

We cannot do any of this important work without our amazing volunteers and fundraisers. Please help us to find the breakthrough people with PSC so desperately need.

Get involved	**Donate**
Join the PSC Support Patient Panel *www.pscsupport.org.uk/get-involved/*	We need your support more now than ever *www.pscsupport.org.uk/donate/*