

PSC PARTNERS RESEARCH PROGRAMS

IMPACT REPORT

2025

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Dear PSC Partners Community,

At PSC Partners Seeking a Cure, patient partnership is not an add-on to research — it is the foundation that drives it forward. Across every research initiative we support, the insights and lived experiences of those affected by PSC shape the questions we ask and the direction we pursue. This 2025 Research Programs Impact Report highlights the collective progress of all our research programs, each one rooted in patient voices in action.

By bringing patient perspectives directly into the research process, our community helps identify meaningful priorities and highlight unmet needs, ensuring that scientific efforts remain grounded in what truly matters. Every research program we champion reflects this partnership, guiding the development of research efforts that are relevant, focused, and responsive to real-world challenges.

This collaborative model is transforming the landscape of PSC research. When patients and researchers work side by side, discoveries become more aligned with community needs and more likely to lead to meaningful change for everyone living with PSC.

To the donors, researchers, medical and scientific advisors, and community members who make this work possible — thank you for your commitment, generosity, and belief in our shared mission.

With gratitude and hope,

Ricky Safer
Founder and CEO
PSC Partners Seeking a Cure



Stephen Rossi, PharmD
Chief Scientific Officer
PSC Partners Seeking a Cure



Audra Fleming
Executive Director
PSC Partners Seeking a Cure

About PSC Partners Seeking a Cure

- Founded in 2005, PSC Partners Seeking a Cure is a 501(c)(3) nonprofit organization with a Canadian affiliate, PSC Partners Canada.
- Our **mission is to drive research to identify treatments and a cure for primary sclerosing cholangitis (PSC)**, while providing education and support for those impacted by this rare disease.
- Our programs give support to patients, caregivers, families, and friends; educate patients and the medical community about PSC; and encourage ground-breaking research on PSC and closely associated diseases.



About PSC

- PSC is a rare liver disease that damages the bile ducts inside and outside the liver (Chapman 2010, Karlsen 2024). Bile ducts become inflamed, and the inflammation leads to scarring and narrowing of the affected ducts. Eventually, blockages may occur.
- As the scarring blocks more and more ducts, bile becomes trapped in the liver. The scarring damages the liver and can result in fibrosis and cirrhosis of the liver and liver failure. Patients may eventually require a liver transplant.
- PSC is frequently associated with inflammatory bowel disease (IBD), particularly ulcerative colitis (UC) (Barberio 2021). Unfortunately, recurrent PSC occurs in 14%-25% of transplanted livers (Leung 2021).
- Additionally, patients with PSC are at increased risk of developing cholangiocarcinoma (CCA), a rare bile duct cancer (Karlsen 2024).
- Although the causes of PSC are unknown, research is underway to better understand the disease, develop effective treatments, and find a cure.

Our Research Team coordinates several research programs and initiatives.



Stephen Rossi, PharmD
Chief Scientific Officer



Zuha Jeddy, MPH
Director of Clinical Research



Sarah Curup Callif, DrPH
Associate Director, Clinical Research



Priya Kannusamy, BSHS
Clinical Research Coordinator

The Patient Registry has its own team within our organization's research department.



Brian Thorsen, BA
Registry Director



Fernanda Quevedo, BSA
Assistant Registry Director



Rachel Gomel, MA
Registry Senior Advisor



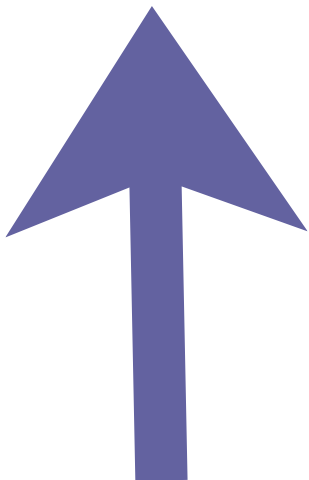
Sharon Nanz, BA
Registry Coordinator

Patient Community Leading the Way in PSC Research

At PSC Partners Seeking a Cure, we believe that the future of PSC research must be shaped **with** patients, not just **for** them. As a patient-founded and patient-led organization, patient voices have historically guided our research priorities and research programs.

What is Patient Partnership in Research?

Patients can bring expertise that improves scientific design, sharpens research questions, and ensures findings are more relevant and translatable to the community. We use a four-tier model to describe different levels of patient engagement, depicted below in order of *most to least* patient engagement:



Patient-Led:

Patients are leaders with 360-degree oversight of a study or research program.

Patient-Partnered: Patients are co-creators with participation in every step of development.

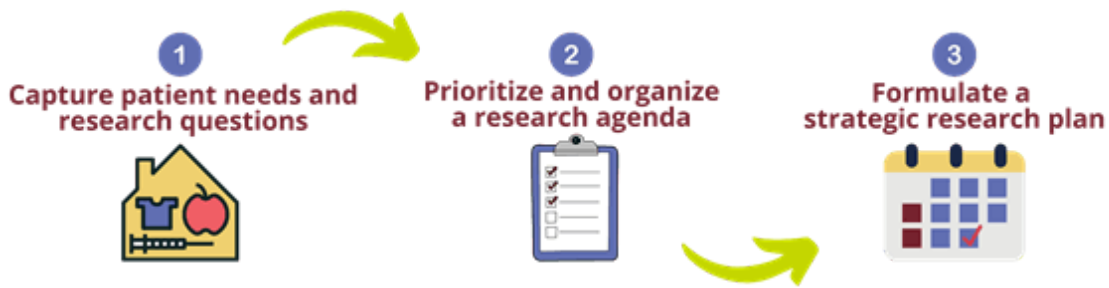
Patient-Centered: Patients are stakeholders (e.g. Community Advisory Boards or Steering Committees, input into protocol design and community)

Patient-Targeted: Patients are consumers (e.g. market research and DTC advertising, recruitment and participation in studies)

Are you a patient, relative, caregiver, or member of the PSC patient community?

Read on to learn how patient and community voices have shaped PSC Partners research programs, and then contact research@pscpartners.org to get started with sharing your voice!

How Have Patient Voices Helped Shape PSC Partners Research Strategy?



- The process incorporated the lived experiences of more than 500 patients, caregivers, and family members, using surveys, interviews, and structured focus groups to identify key priorities and challenges.
- Community members worked with clinical and scientific experts to develop the research agenda, using a structured scoring rubric, committee review, and a priority-ranking process to ensure transparency, equity, and alignment.
- This effort ultimately led to the creation of the PSC Partners Strategic Research Plan –a patient-driven roadmap that shapes how we fund research, build partnerships, and advocate for progress.
- Today, this plan continues to guide decision-making across programs, ensuring every research approach remains grounded in what matters most to those living with PSC. The priority areas are listed below:

1	Improve understanding of the mechanisms contributing to the development and progression of PSC
2	Further understanding of the gut-liver connection in PSC and PSC-IBD
3	Develop safe and effective treatments to slow progression and reduce symptom burden
4	Create early detection and treatment of PSC-related cholangiocarcinoma

PSC Partners has several patient-led research programs:

WIND-PSC

- *Program Summary:* Prospectively collects real-world data to support PSC drug development
- *Patient Partnership:* Entirely planned, financed, and overseen by PSC Partners and PSC Partners Canada; carefully designed to minimize burden for participants; over 400 patients currently enrolled across 14 sites in Canada, Europe, New Zealand, and the U.S.

INTERNATIONAL COLLABORATIVE RESEARCH NETWORK (ICRN)

- *Program Summary:* Brings together global experts and patients for scientific convenings and global projects
- *Patient Partnership:* Researchers and community members co-develop research proposals aligned with community priorities; PSC Partners first ICRN-funded project was selected with input from 235 community members

PSC SYMPTOM ASSESSMENT PROJECT (SAP)

- *Program Summary:* Develops the first PSC-specific symptom measurement tool, for use in clinical trials and everyday care
- *Patient Partnership:* Patients participate as advisors, interviewees, and co-creators; priority symptoms were identified by patients through ROADMAP and multiple focus groups

RESEARCH GRANTS PROGRAM

- *Program Summary:* Funds innovative research ideas for PSC
- *Patient Partnership:* Researchers are encouraged to incorporate patient partnership into their proposal; community voices are embedded into the application evaluation process

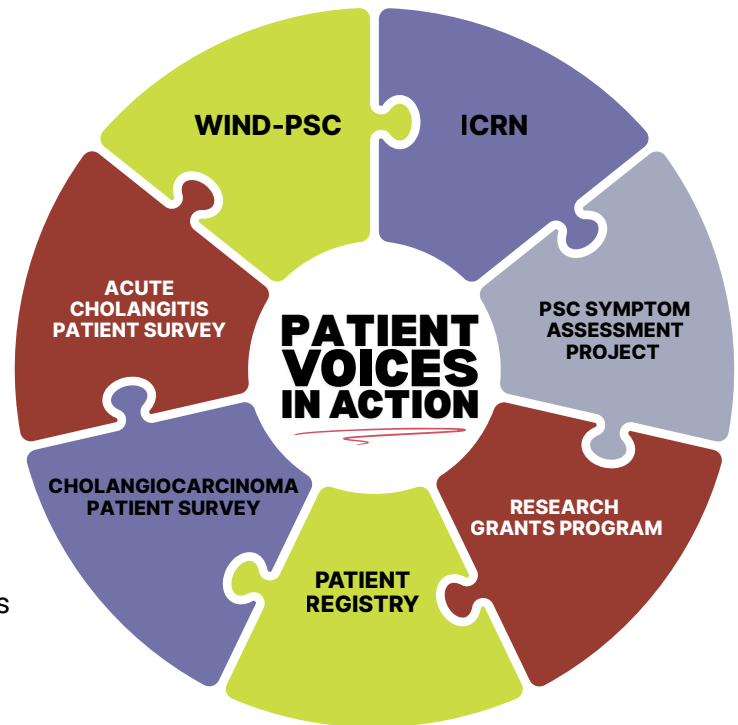
PATIENT REGISTRY

- *Program Summary:* Gathers PSC patient-reported data to support research on patient experiences, recruit for and facilitate clinical trials, and provide a platform for patient surveys
- *Patient Partnership:* Currently there are over 2,700 patients from 55 countries participating

PATIENT EXPERIENCE SURVEYS

Several surveys are done through PSC Partners and in collaboration with global PSC groups; patient voices on emergent topics are integral for research strategy and partnerships. Recent examples include:

- **Acute Cholangitis Patient Survey:** 726 responses captured experiences of urgent PSC-related complications; fatigue & anxiety emerged as under-recognized concerns, highlighting the need for further investigation
- **Cholangiocarcinoma (CCA) Patient Survey:** 623 responses revealed gaps in provider communication and patient education about risk & surveillance of cholangiocarcinoma, a rare bile duct cancer associated with PSC



A Global Multi-Center Prospective Observational Cohort to Support Drug Development in Adult Patients with Primary Sclerosing Cholangitis (WIND-PSC Study)



Program Summary: *WIND-PSC study is a prospective cohort study designed to collect regulatory quality real-world data to better understand the progression of PSC.*

Patient Partnership: *It is the first study of its kind launched and funded solely by the patient community (sponsored by PSC Partners and PSC Partners Canada), demonstrating the power of patient advocacy in driving research.*

History: In 2022, PSC Partners unveiled the WIND-PSC initiative inspired by the PSC community’s call to identify new treatment options for PSC and address the unmet medical needs of PSC patients. With no current FDA-approved therapies for PSC, WIND-PSC was developed to generate key data to advance research, remove barriers and potentially accelerate the development of effective treatments for PSC. This prospective observational cohort can overcome recruitment and ethical challenges that previous external controlled trials have faced during the FDA pathway for drug approval. The real-world data collected for WIND-PSC could serve as an external control arm to complement clinical trial design while addressing these issues and could help us answer important questions regarding the progression of the disease.

STUDY QUESTIONS

- How does PSC progress over time?
- Can we find tests that predict PSC progression or treatment response?
- How do PSC symptoms and daily life change over time?

STUDY OUTCOMES

- Improve our understanding of the disease and patient experience
- Establish a global dataset to serve as invaluable resource to research community
- Support faster PSC drug development

Overview: The WIND-PSC study is a groundbreaking global, patient-led research initiative that collects regulatory quality data from up to 2000 patients over five years. Designed to layer on top of standard of care, patients complete a baseline and subsequent annual in-person study visits which include their regular PSC care, fibroscan, blood work, and an online symptom survey. Additionally, patients are contacted remotely every quarter to review their medical history and complete an online symptom survey.



CLINICAL DATA



IMAGING RESULTS



BIOMARKER DATA



PATIENT REPORTED OUTCOME MEASURES

How Patient Input Shaped WIND-PSC

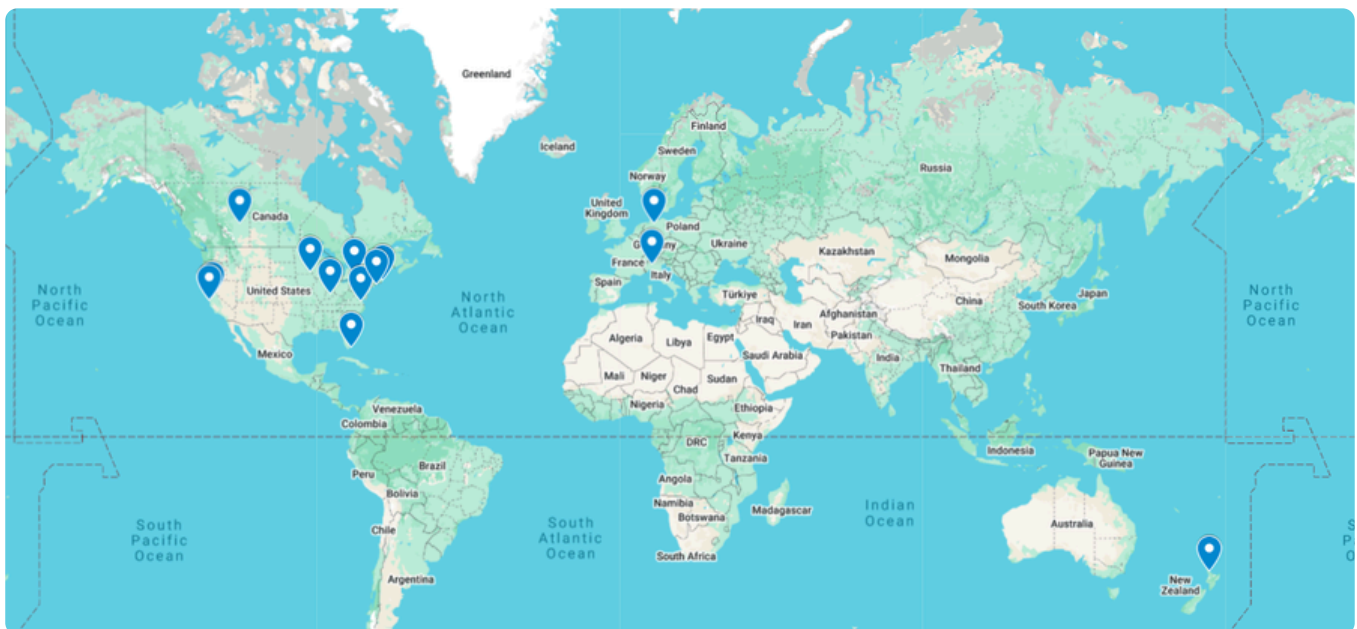
- Patients set the research direction, with the 2020 Our Voices survey revealing that understanding PSC progression—not just curing PSC—was a top priority, directly driving PSC Partners' decision to launch the WIND-PSC study. More than 70% of PSC patients said that the most important area of focus is the development of treatments to slow the progression of their PSC.
- Patients identified a critical unmet need, prompting PSC Partners to design a study aimed at supporting development of treatments.
- Patients influenced the study’s leadership and structure, inspiring PSC Partners to build a global, patient-driven research initiative fully planned, funded, and overseen by the community’s own advocacy organization.
- Patients shaped the study’s design principles, contributing feedback—alongside researchers and regulators—that ensured the WIND-PSC study is truly patient-centric, reduces burden, and focuses on outcomes that align with what patients value most.

FROM INCEPTION TO IMPACT: 2025 STUDY METRICS

14
Enrolling Sites Globally

475
Baseline Study Visits Completed

63
Annual Follow up Visits Completed

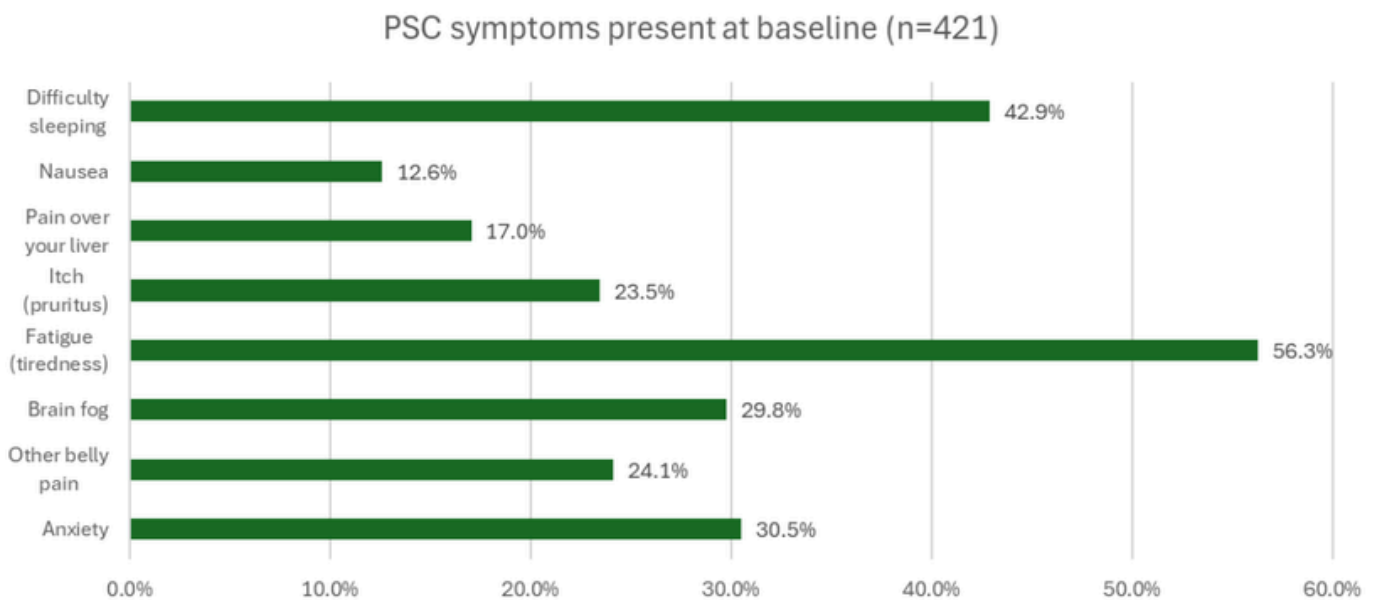


Global Impact

Initial data from the patients enrolled to date were presented in multiple forums at major international medical meetings, garnering global attention and reinforcing the project’s leadership in defining PSC natural history as well as establishing a roadmap for other rare diseases.

- American Association for the Study of Liver Diseases (AASLD)
- European International Liver Congress (EASL ILC)
- PSC Partners Annual Conference
- PBC Consensus Meeting
- The Liver Forum
- International PSC Study Group

DATA SPOTLIGHT: SYMPTOMS AT BASELINE (N=429)



Key Takeaways

- WIND cohort represents similar characteristics as general PSC community
- Fatigue is the highest reported symptom at baseline

Age (years) at PSC diagnosis	
18 to 25	72 (16.86%)
26 to 40	145 (33.95%)
41 to 55	85 (19.91%)
Under 18	74 (17.33%)
IBD Status	
Crohn’s Disease	74 (17.33%)
Ulcerative Colitis	243 (56.91%)
Indeterminate Colitis	20 (4.68%)
None	74 (17.33%)
Liver Stiffness	
<7.4 kPa	212 (49.53%)
>7.4 to <14.4 kPa	115 (26.87%)
>14.4 kPa	47 (10.98%)

The WIND-PSC Steering Committee is comprised of internationally recognized PSC experts who provide high level strategic oversight to ensure the study stays scientifically sound, ethically conducted, and operationally on track. The Steering Committee meets regularly to review study progress, monitor data quality, and help resolve major issues that may affect timelines of integrity. The committee played a critical role in the development of the WIND-PSC protocol and actively advises on any necessary protocol amendments and ensures the study continues to align with its scientific objectives and regulatory requirements.

In addition, the steering committee reviews analytic proposals to ensure planned analyses are appropriate, scientifically justified, and consistent with the protocol and statistical analysis plan. It also evaluates completed analyses to confirm their validity, interpretability, and relevance to the study's objectives before findings are disseminated or used for decision-making.



Bettina Hansen, PhD
*Professor of Clinical
Biostatistics*
Erasmus University Medical
Center



Christoph Schramm, MD
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Hamburg-Eppendorf,
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Christopher Bowlus, M.D.
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Cynthia Levy, MD
Professor of Medicine
University of Miami,
Department of Medicine



Gideon Hirschfield, FRCP, MD
University of Toronto - UHN,
Autoimmune and Rare Liver
Disease Programme



Mary Pressley Vyas
Patient Partner and Former
President, PSC Partners
Seeking A Cure - Canada

2025 WIND-PSC STUDY SITES	
SITE	PRINCIPAL INVESTIGATOR
Auckland Hospitals Research Center	Hannah Giles, MBChB, FRACP
California Pacific Medical Center	Kidist Yimam, MD
University Medical Center Hamburg- Eppendorf	Christoph Schramm, MD
Indiana University	Craig Lammert, MD
Massachusetts General Hospital/Harvard	Daniel Pratt, MD
Mayo Clinic Rochester	John Eaton, MD
University of California Davis	Christopher Bowlus, MD
University Health Network	Gideon Hirschfield, FRCP, MD
University of Alberta	Aldo Montano-Loza, MD, MSc, PhD
University of Miami	Cynthia Levy, MD
University of Milan	Laura Cristoferi, MD, PhD and Marco Carbone MD, PhD
University of California San Francisco	Michael Li, MD, MPH
Virginia Commonwealth University	Sayed Aseem, MD
Yale University	Marina Silveira, MD

LOOKING FORWARD: Next Steps and Priorities

- Complete study recruitment and enrollment
- Achieve high quality annual data along with alignment with standard of care
- Establish central biorepository to store blood samples and allow for analysis of study blood samples.
- Deep dive analyses of data to better understand progression of PSC
- **To learn more about the study, visit: [ClinicalTrials.gov](https://clinicaltrials.gov/ct2/show/study/NCT06297993) study record NCT06297993**



Program Summary: *Brings together global experts and patients for scientific convenings and global projects.*

Patient Partnership: *Researchers and community members co-develop research proposals aligned with community priorities; projects are selected for funding via patient community input.*

History: PSC Partners launched the International Collaborative Research Network (ICRN) in 2022 to unite patients, caregivers, and international experts around one table to accelerate research on PSC and closely associated diseases. The model blends community members' lived experience with scientific rigor to drive research on what matters most to the community.

How Patient Input Shaped ICRN

- **Identified Patient Needs:** PSC Partners collected patient perspectives on unmet/unaddressed research and treatment needs from those living with PSC through the Patient-Focused Drug Development Forum (PFDD), *Our Voices* Survey, and the ROADMAP (research education) webinar series.
- **Community Engagement:** PSC Partners collaborated with the PSC community to establish research priorities at the 2022 PSC Partners Annual Conference
- **Scientific Engagement:** PSC Partners analyzed scientific literature and consulted with international experts via interviews and the 2022 virtual ICRN thinktanks
- **Draft Research Agenda:** PSC Partners formulated a research agenda with priority areas based on patient community and research experts' questions

2024 Scientific Convening

At our second ICRN scientific convening, participants collaborated across six co-led Working Groups (community + researchers) to brainstorm research questions and develop proposal concepts aimed at high-impact progress for PSC. Sessions were designed to: **collaborate** across disciplines, **create** actionable proposals, and **strategize** the pathway to studies and tools that can speed therapies and improve care. The event was co-chaired by Yury Popov, MD, PhD and Bettina Hansen, PhD.



FROM INCEPTION TO IMPACT: ENGAGEMENT METRICS

61

Attendees at the convening

235

Community members engaged in proposal prioritization

\$200,000

Awarded to the first ICRN-funded, patient-partnered project

About the ICRN Scientific Convening

The second ICRN gathering took place in Phoenix, AZ, on October 17-18, 2024. This event brought together diverse voices and expertise from around the world. Over two days, participants engaged in collaborative discussions, refined research questions, and laid the groundwork for patient-centered initiatives aimed at improving care and accelerating the development of treatments.



Working Group Topics, Co-Chairs, and Project Proposals

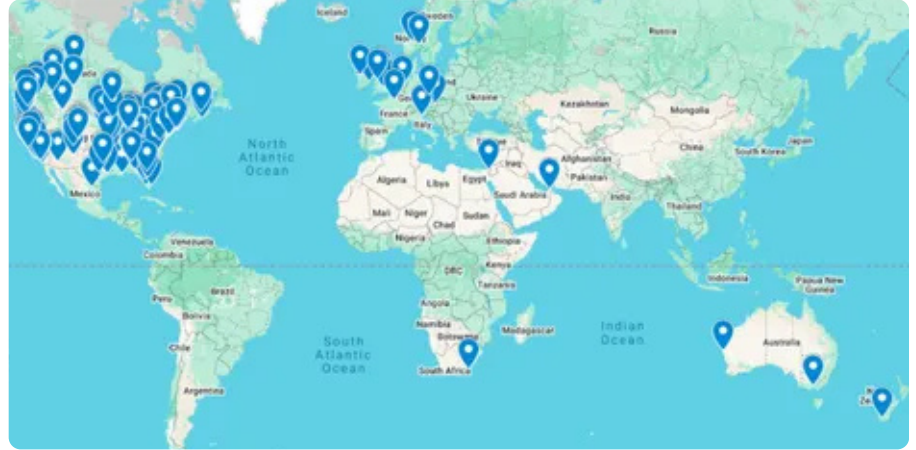
Six Working Groups, each with co-chairs representing the patient community and clinical or academic research, developed innovative research project proposals:

- *Pathophysiology of PSC: Inflammation and Fibrosis Working Group*
 - Co-chairs: Jesse Kirkpatrick, PhD and Yury Popov, MD, PhD
 - Proposal: Genetic Drivers of Inflammation and Fibrosis
- *Pathophysiology of PSC: Gut Microbiome Working Group*
 - Co-chairs: Mahesh Krishna, MD and Michael Trauner, MD
 - Proposal: Dietary Impact on Quality of Life, Clinical Outcomes, and Gut Microbiome
- *Diagnosis, Prognosis, and Clinical Management Working Group*
 - Co-chairs: Marco Carbone, MD, PhD and Christine Lam, PhD
 - Proposal: Radiologic Assessment of Disease Progression
- *Clinical Trials and Endpoints Working Group*
 - Co-chairs: Bettina Hansen, PhD and Stephen Rossi, PharmD
 - Project: Patient-Reported Outcome Use in Clinical Trials for PSC
- *Managing Transplants and Recurrent PSC Working Group*
 - Co-chairs: Lisa Forman, MD and Fred Sabernick, MSEE
 - Project: Post-Transplant PSC - Characterizing Practice Variations, Studying Clinical Outcomes, and Investigating Biomarkers of PSC Recurrence
- *CCA Screening, Surveillance, and Treatment Working Group*
 - Co-chairs: Jesús Bañales, PhD and Matt McMurtry, MD
 - Project: Biomarkers for Detecting Cholangiocarcinoma (CCA)

Award Details: PSC Partners committed \$200,000 over two years to launch a research proposal resulting from the 2024 ICRN Scientific Convening. The project selection process continued ICRN’s model of co-engaging patient community members and expert researchers.

1) Community Priority Survey:

235 community members from 17 countries provided feedback & ranked proposals. Although <2% of patient respondents reported a cholangiocarcinoma diagnosis, the CCA biomarker project received the highest community priority score.



Community participant types:

64% patients, 24% parents, 9% other relatives; the remainder caregivers and friends.

2) Independent Scientific Review:

External experts (not Working Group participants) assessed feasibility, innovation, and potential for near-term impact. The CCA biomarker project was ranked as the top scientific priority, citing strong preliminary data, impressive study design with international networks in place, and immense clinical relevance.

Project Selected for the Inaugural ICRN Award

- The project selected for the inaugural ICRN Award is called “BIOMAP-PSC: Prospective validation of candidate blood **biomarkers** for PSC-CCA and **roadmap** for clinical implementation.”
- BIOMAP-PSC is coordinated by **Pedro Rodrigues, PhD** and **Jesus Banales, PhD** at Biodonostia Health Research Institute in Spain, and in continued partnership with PSC Partners and the associated ICRN CCA Working Group.
- It aims to validate a relatively **non-invasive blood test** that could enable earlier, more accurate detection of CCA in people with PSC. It harmonizes protocols across an international network, utilizing diverse, multi-center PSC samples (pediatric and adult).

“ **Patients are not merely participants, they are essential partners in translating discoveries from the laboratory to real-world clinical solutions. None of these advancements would be possible without the generosity and active participation of patients and their families.** - Jesus Banales, PhD ”

Jesús Bañales, PhD

Full Professor of Molecular Biology and Genetics

Head of Liver Diseases Group

Biogipuzkoa Health Research Institute - Donostia University Hospital

San Sebastian, Spain

**Research Focus:**

Dr. Bañales' research centers on PSC, while also exploring related liver conditions such as fibrosis, cirrhosis, hepatocellular carcinoma (HCC), and cholangiocarcinoma (CCA), looking for new therapeutic strategies. Through innovative strategies grounded in patient input and engagement, he is accelerating the path from scientific discovery to clinical impact, making research more impactful for the PSC community.

PSC Partners Research Awards**Douglas Carlson Research Award for Exceptional Patient Partnering**

- Dr. Bañales is a recipient of the 2024 PSC Partners Standard Seed Grant Award for his project, "International Validation of Serum Protein Biomarkers for Predicting and Early Diagnosing Cholangiocarcinoma (CCA) in Individuals with primary sclerosing cholangitis (PSC)".
- His project incorporated patient perspectives throughout its development, design, and dissemination; prioritized diverse patient representation across ethnic and geographic backgrounds; and showed strong potential to translate findings into clinical practice.
- In recognition of this exemplary patient engagement, PSC Partners honored him with the *Douglas Carlson Research Award for Exceptional Patient Partnering* at our 2024 Annual Conference.

ICRN Award

- Dr. Jesús Bañales, along with Dr. Pedro Rodrigues, was also awarded funding through PSC Partners International Collaborative Research Network (ICRN) to lead the BIOMAP-PSC study. Building on their established preliminary work, the researchers aim to validate identified biomarkers and develop a clinical roadmap for early, non-invasive detection of PSC-associated cholangiocarcinoma (PSC-CCA), identified by patients as one of the most urgent unmet needs in PSC care.
- The study will utilize PSC patient samples from around the world, including both children and adults, and bring together data from several PSC registries and biobanks. Early biomarker detection could identify high-risk CCA patients sooner, enabling timely intervention. This can pave the way for personalized screening strategies based on individual CCA risk.

Program Summary

The Symptom Assessment Project (SAP) is a patient-partnered initiative led by PSC Partners to develop PSC-specific Patient-Reported Outcome (PRO) measures for potential use as validated clinical trial endpoints. Dr. Donna Evon (UNC) and Dr. Bryce Reeve (Duke) have created PRO tools to capture patients' real-world experiences with key PSC symptoms and to evaluate how these symptoms change over time and in response to treatment.

From Patient Voices to Validated Measures

- **Patient Partnership:** Patients contributed as advisors, interviewees, and co-creators. Priority symptoms—fatigue, brain fog, and liver pain—were identified through [ROADMAP](#) and focus groups.
- **Symptom Discovery:** Through in-depth interviews with 126 symptomatic patients, researchers defined symptoms from patients' own words.
- **Customized Measures:** Fatigue and brain fog were adapted from validated tools; liver pain was developed from scratch based on patient descriptions.
- **Cognitive Testing:** Draft measures were tested with patients to ensure clarity, relevance, and accuracy.
- **Registry Validation:** Now validated through the PSC Partners Patient Registry, these measures can serve as reliable benchmarks for clinical trials and effective tools for patient advocacy.

Practical Tools for Patients, Clinicians, and Researchers

- Allow patients to bring their symptom experiences directly to appointments.
- Ensure these experiences are formally documented in medical records, supporting informed clinical care.
- Improve clinical trial readiness and support treatment evaluation.

Key Metrics for 2025:

- **126** patient interviews
- **3** symptom measures
- **280** Patient Registry survey responses
- Ready for clinical use

We presented 3 posters at the American Association for the Study of Liver Diseases (AASLD)'s 2025 "The Liver Meeting"

- [View the brain fog poster](#)
- [View the fatigue poster](#)
- [View the liver pain poster](#)

“*If a new medication improves your lab values or markers of disease, that's fantastic... but what if the medication also reduced your fatigue, brain fog, and discomfort around your liver? **We cannot 'discover' these added benefits if we don't use the right tools to capture potential changes.***”

- Donna Evon, PhD

**Donna Evon, PhD**

Professor of Medicine

Division of Gastroenterology and Hepatology

UNC School of Medicine at Chapel Hill

North Carolina, USA

About the Researcher

Dr. Donna Evon is a clinical health psychologist and behavioral researcher with over 20 years of clinical and research experience in chronic liver diseases. Her research focuses on understanding psychosocial and behavioral factors affecting the health and well-being of individuals living with chronic liver disease. She develops and evaluates psychosocial interventions to improve the health, well-being and symptoms of those with chronic liver disease. Dr. Evon uses patient-reported outcome measures and qualitative methods as part of her research with patients with viral hepatitis, PSC, and cirrhosis. She works closely with patient partners and organizations like PSC Partners to ensure the patients' voice is central to research aims, outcomes and measures.

Why is it so important for patients to use these specific PSC tools instead of the generic surveys often used in the past?

The three symptom measures we designed are grounded in PSC patients' lived experiences, not in the general population or other liver diseases. They were developed through in-depth interviews with adults with PSC, and patients confirmed the measures are clear, understandable, and accurately capture their experiences.

How can patients use these new symptom surveys to make sure they are "heard and seen" by doctors during short clinic visits?

Though developed for clinical trials, patients can complete and share these surveys with their providers as communication tools. Upload them to your electronic medical record or bring them to appointments. Reviewing responses on symptom frequency, severity, and impact helps providers discuss medical or lifestyle interventions.

How will the data collected through the SAP and other PRO tools shape future PSC clinical trials and, ultimately, help move new treatments toward approval?

Adding symptom-based measures to clinical trials will allow researchers to evaluate the multiple benefits of new treatments on the disease as well as how patients are feeling. Treatments may be approved for PSC based on meaningful improvement in symptoms, not just disease markers.

OVERVIEW

- The PSC Partners Research Grants Program was launched in 2007, aimed at funding seed grants for novel research projects to improve our understanding of disease progression and finding a cure for PSC.
- Today, PSC Partners and affiliate PSC Partners Canada offer Standard Seed Grants and Young Investigator Awards annually to conduct research that addresses an important and novel, basic or clinical research question related to PSC and closely associated diseases (such as inflammatory bowel diseases (IBD) and cholangiocarcinoma (CCA)).
- Our Research Grants Program seeks to encourage investigators to conduct research in promising new areas, with the goal that data generated will lead to federal (NIH) or external international funding.

2025 HIGHLIGHTS

Application Cycle

- Received **43** Letters of Intent (LOIs) and **38 applications** (26 preclinical, 12 clinical; **17** Standard Seed Grant applicants, **21** Young Investigator applicants).
- Deep alignment with 1 or more of our research priorities: **25** proposals focused on mechanisms of PSC development/progression, **16** explored the gut-liver axis in PSC and PSC-IBD, **17** aimed to develop safe/effective treatments to slow progression, **9** addressed early detection/treatment of PSC-related cholangiocarcinoma.

Awards

- Awarded **five grants** in total: two Standard Seed Grants, two Young Investigator Awards (US), and one Standard Grant (Canada).
- The award amount is \$100,000 (for 2 years) for Young Investigator Awards and \$75,000 (for 2 years) for Standard Seed Grants.
 - Applicants are eligible for a Standard Seed Grant if they have an advanced terminal degree, expertise in preclinical and/or clinical research, and are affiliated with an academic medical institution, research institution, or nonprofit/community-based organization.
 - Applicants are eligible for a Young Investigator Award if they have a mentor, are in training or within five years post-training or degree, have completed an advanced terminal degree, and are affiliated with an academic medical institution, research institution, or nonprofit/community-based organization.

The grants program has an annual cycle, starting with letters of intent (LOIs) and progressing to applications, reviews, funding decisions, and reporting:

- Applicants move through a competitive, multi-step review process beginning with a Letter of Intent screening, followed by full application review by both the Scientific/Medical Advisory Committee (SMAC) and the Community Review Committee.
- PSC Partners Canada participates in the scientific review and then makes independent funding decisions in accordance with Canadian charity law.

Scientific/Medical Advisory Committee (SMAC) Chair



Dr. Christopher Bowlus is the Lena Valente Professor of Medicine and the Chief of the Division of Gastroenterology and Hepatology at the University of California Davis School of Medicine. His extensive research is in autoimmune liver diseases, including the immunopathogenesis of PSC. He also chairs the Consortium for Autoimmune Liver Disease (CALiD) and is an active member of the International PSC Study Group (IPSCSG) and PSC Partners ICRN.

Additional SMAC Reviewers in 2025:

- David Assis, MD
- Dennis Black, MD
- John Eaton, MD
- Richard Green, MD
- Bettina Hansen, PhD
- Gideon Hirschfield, FRCP, PhD
- Johannes R. Hov, MD
- Joshua Korzenik, MD
- Cynthia Levy, MD
- Cara Mack, MD
- Sonya MacParland, PhD
- Alexander Miethke, MD
- Yury Popov, MD, PhD
- Kidist Yimam, MD

Ad Hoc SMAC Reviewers in 2025:

- Jana Al Hashash, MD, MS
- Vik Meadows, PhD
- Juan Valle, MB ChB, MSc, FRCP



- The grants review process elevates the patient voice by giving the Community Review Committee—made up of patients, caregivers, and key stakeholders—50% of the total scoring weight.



- While the Scientific/Medical Advisory Committee (SMAC) ensures each application meets scientific rigor and credibility using NIH-style criteria, the community reviewers utilize our customized PSC Partners Community Rubric to assess components such as mission and program alignment, overall reach, and impact.



- Once scientific feasibility is confirmed, the community perspective becomes the deciding factor in final rankings and prioritization.

Community Reviewers

- Jesse Kirkpatrick, MD, PhD
- Joanne Hatchett, RN, FNP, ACHPN
- Mahesh Krishna, MD
- Rachel Gomel, MA
- Nathan Baggett, MD
- Jean-Philippe Lambert, PhD
- Jaimie Pheneger, MPH
- Christine Lam, PhD
- James McMurtry, MD
- Stephen Rossi, PharmD
- Brian Thorsen, BA
- Priya Kannusamy, BSHS
- Kelly Butler, BA
- Matt McMurtry, MD



Jesse Kirkpatrick, MD, PhD | Internal Medicine Resident, Brigham and Women's Hospital| Board Member, PSC Partners Seeking a Cure



"The patient voice is critical to advocating for research that is laser focused on what matters most to patients: finding treatments and ultimately a cure for PSC." - **Jesse Kirkpatrick, MD, PhD**

BRIDGING SCIENCE AND LIVED EXPERIENCE

- Jesse Kirkpatrick, PSC Partners Board Member, has been deeply committed to the PSC mission since 2010, after a family member's diagnosis.
- He is a bioengineer, a PSC researcher, and a hepatologist-in-training, whose research centers on engineering nanoparticles to enable early detection of bile duct cancer and "bile ducts in a dish" to shed light on the underlying cause of PSC.
- He was the co-chair of the Pathophysiology of PSC - Inflammation and Fibrosis working group at the 2024 PSC Partners International Collaborative Research Network (ICRN) convening as a representative of the PSC patient community.

COMMUNITY VOICE: Q & A

- ***How do you balance your scientific expertise with the community lens expected in this reviewer role?*** As someone with deep personal ties to PSC, my central motivation is to identify projects with the greatest potential to improve or extend the lives of patients. My scientific training allows me to rigorously evaluate proposals while keeping this patient-centered lens front and center.
- ***What factors help you determine whether a research proposal will make a meaningful and practical impact on the PSC community?*** I'm looking for demonstrated understanding of the greatest unmet needs of PSC patients and a concrete, PSC-specific solution to one or more of those needs. The best proposals clearly link back to the PSC patient, either through direct patient involvement or through preclinical models that are meaningfully connected to human disease.
- ***What motivates you to participate in the review process, and what are your hopes for the future direction of PSC research?*** There has been an explosion of progress across biomedicine, and, at the same time, growing excitement about PSC, which remains "the last black box of hepatology." I want to see the grants program capitalize on this momentum by attracting outstanding minds from across disciplines, including emerging fields like generative AI, to tackle PSC in new and transformative ways.

In 2025, PSC Partners awarded two Standard Seed Grants & PSC Partners Canada awarded one

Characterizing the Cellular Microenvironment of the PSC Extrahepatic Bile Duct



Sonya MacParland, Ph.D. | University Health Network | Canada

This study Investigates large extrahepatic bile ducts (outside the liver) in PSC vs. PBC vs. healthy donors using single-cell and spatial transcriptomics, paired with matched liver and blood samples, to reveal unique cellular/immune features in PSC.

Dissecting Unique Immunopathogenic Mechanisms in PSC (UnikPATH)



Xiaojun Jiang, Ph.D. | Oslo University Hospital | Norway

This project focuses on a putative genetic mutation (CD100 K849T) in a family with inherited PSC, exploring tissue-immune interactions in bile duct disease to identify targeted therapeutic pathways.

Elucidating Fatigue-Associated Alterations of Brain Structure and Function in PSC



Mark Swain, MD. | University of Calgary | Canada

This study, funded by PSC Partners Canada, addresses a major patient-relevant symptom (fatigue) in PSC by using advanced MRI to assess brain structure/function changes and linking these to fatigue severity and immune activation, aiming to identify treatment targets.

PSC Partners awarded these Young Investigator Awards in 2025

Modulation of Hepatic Macrophage Differentiation and Efferocytosis by Propionate to Ameliorate Fibrosis



Olumuyiwa Awoniyi, MD, PhD | Cleveland Clinic Foundation | USA

This Young Investigator researches how the short-chain fatty acid propionate influences liver macrophages in PSC models, promoting clearance of dying cells (efferocytosis) and reducing liver fibrosis.

Exploring Mechanisms of PSC Progression with an In Vitro Bile Duct on a Chip



Henry Hoyle, PhD | University of Oslo | Norway

This Young Investigator aims to build a human-relevant bile duct model (using patient-derived cells and microenvironment elements) to test drug candidates and accelerate preclinical PSC therapy development.

**Sonya MacParland, PhD**

Associate Professor, Department of Laboratory Medicine Pathobiology and Immunology

University of Toronto

Dr. Sonya MacParland, in collaboration with **PSC Partners and PSC Partners Canada**, is actively conducting a PSCP-funded project focused on the Transcriptomic Characterization of the Extrahepatic Bile Duct niche in PSC, Primary Biliary Cholangitis (PBC), and healthy tissue samples. This study takes a patient-oriented approach by involving patients, caregivers, and families as essential partners to ensure the research addresses patient-identified priorities and advances meaningful outcomes.

THE PILLARS OF PATIENT-PARTNERED RESEARCH

Collaborative Model

- Patients, caregivers, and families serve as active partners shaping study decisions and priorities.
- Research is guided by what the PSC community identifies as most meaningful for improving outcomes.

Guiding Principles

- **Mutual Respect:** Scientific expertise and lived experience are valued equally.
- **Co-Building:** Patients and researchers work together from the start to identify gaps and co-create solutions.

Patient Contributions

- Patient partners provide ongoing input through monthly research meetings.
- Recruitment is supported through the PSC Partners Patient Registry.
- Patient partners help translate complex science into accessible language for the community.

Impact

- Research remains grounded in real patient experiences.
- Clear communication and shared knowledge strengthen meaningful, real-world outcomes.

Since 2007, PSC Partners has awarded over \$5 million through several types of research grants:

- Standard Seed Grants funded between \$60,000 - \$75,000 for 2-year studies
- Young Investigator Awards funded between \$80,000 - \$100,000 for 2-year studies
- Other types of grants support collaborations with AASLD, EASL, CASL, and various patient partners
- Explore previously funded PSC Partners and PSC Partners Canada grants.


121
Awards from
PSC Partners
(USA)

13
Awards from
PSC Partners
Canada

9
Young
Investigators
funded

HOW TO GET INVOLVED

There are many ways to participate in the Research Grants program:

 **Apply Now** Submit an LOI during our next review cycle Fall 2026



Support grant applicants as a patient-partner



Become an Ad Hoc reviewer (community or scientific)



Donate to the program to support future research proposals

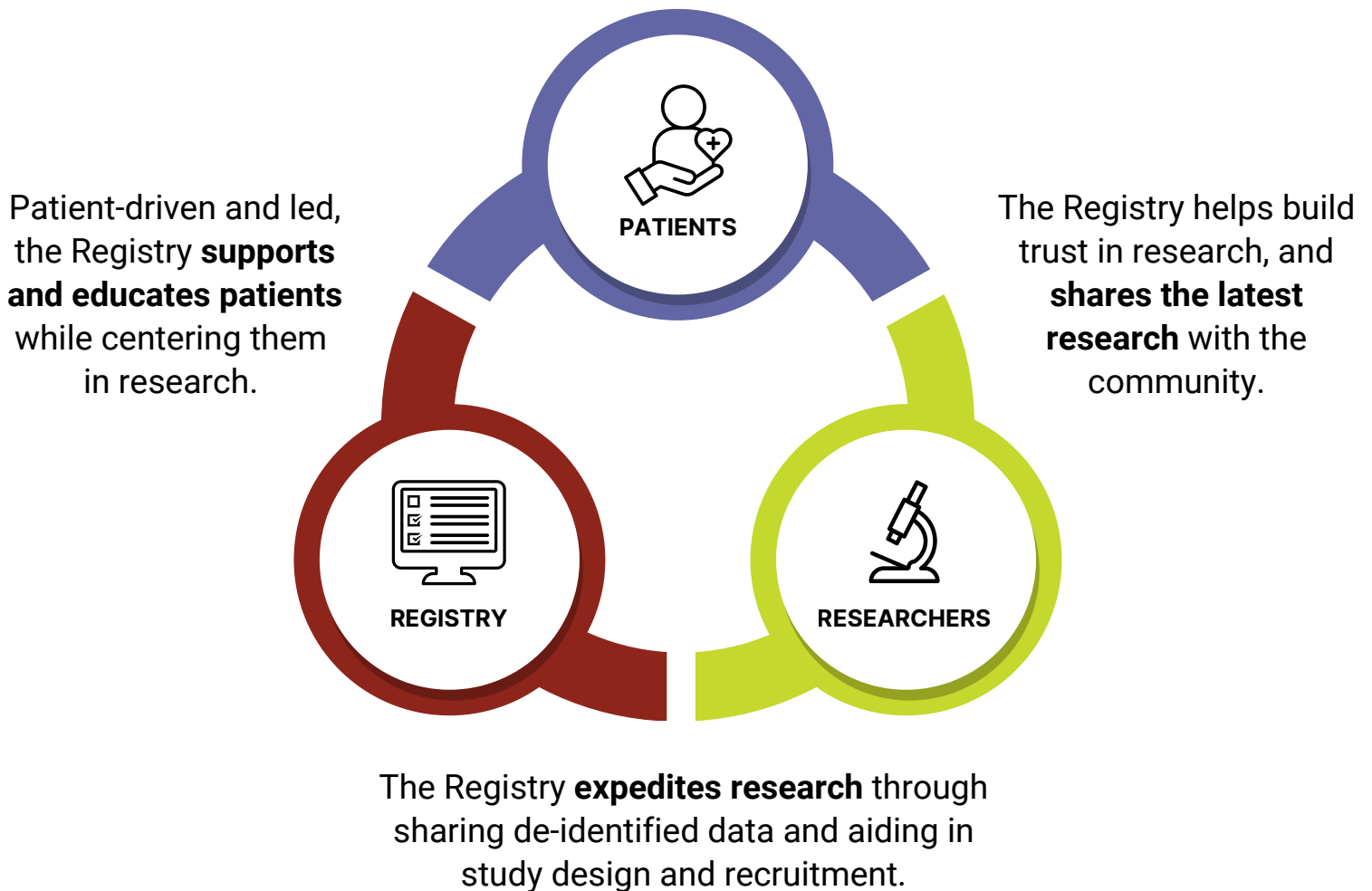
Thank you to all the applicants, grantees, mentors, reviewers, and other supporters who've helped grow the grants program!

Contact grants@pscpartners.org to get involved or learn more.

The **PSC Partners Patient Registry** (Registry) is a digital platform where basic data on people living with primary sclerosing cholangitis (PSC) is collected and stored. Data is primarily collected through online surveys completed by the participant living with PSC, or a qualified caregiver. Confidentiality and privacy measures are built into the Registry to ensure participant privacy.

Our mission is to **facilitate, expedite, and advance patient-centered research** towards treatments and a cure for PSC, by educating, supporting, and engaging all PSC patients to participate in patient-reported and clinical research.

The Registry is **open to everyone with PSC**, wherever you are in the world. Parents and caregivers can join on behalf of their child or loved one. Over 2,700 people living with PSC from 55 countries are currently participating in the Registry.



Learn more at pscpartnersregistry.org

PATIENT-DRIVEN RESEARCH

Registry data is used to accelerate PSC research and advocate for the unmet needs of PSC patients. Researchers request de-identified patient data which is analyzed to gain new insights on PSC. The Registry also supports recruitment for clinical trials and external studies through confidential messages sent by Registry staff on behalf of investigators.



The Registry is patient-led and engages patient participants regularly to incorporate their feedback on new surveys and other projects. Results from publications enabled by Registry participants– 23 to date– are shared in easy-to-digest newsletters.

CONFIDENTIAL AND SECURE

No identifying data ever leaves the Registry.

The Registry’s operating software, Matrix, operates in accordance with applicable US privacy protection provisions of HIPAA, the European GDPR, and FDA regulations. To protect the best interests of Registry participants, the Registry is annually reviewed by an Institutional Review Board (IRB).



“
*I participate in the Registry because I want **my unique PSC experience** to be accurately reflected in the research.*

L.N., Post-transplant Patient

PARTICIPANT ACTIVITY & ENGAGEMENT



2,776

Participants
(223 new)



2,012

Survey responses



5

Publications using
Registry data



9

External study/trial
recruitment campaigns

... all in 2025 alone!

Key Achievements

- Conducted the PSC and Cholangiocarcinoma Patient Survey in collaboration with PSC Support UK, receiving 623 responses in 7 weeks, and presented results at AASLD's The Liver Meeting
- Conducted the Symptom Assessment Survey to support validation of new fatigue, brain fog, and liver pain measures as part of the Symptom Assessment Project
- Record-high response rates (n=662) to annual Clinical Survey
- Translated all Registry surveys and core website content into Spanish and French, increasing access to research
- Presented on patient leadership, innovation, and collaboration in research at AASLD's The Liver Meeting
- Significant ongoing work in survey curation to ensure data accuracy and validity across historic and recent surveys



*I am hopeful that every detail I provide, every survey I fill out will spark a little something that gets us **closer to a cure**.*

- Registry Participant

Cholangiocarcinoma (CCA) Survey Results: CCA (bile duct cancer) is a significant risk in PSC, with up to a 20% lifetime risk. Annual imaging surveillance can help with early detection and expanded options for treatment. Proactive discussion between patients and clinicians of CCA patients is associated with better knowledge regarding CCA and greater adherence to annual surveillance. Patients report high anxiety and fear regarding CCA and a need for better educational resources.

Demographics of PSC Patients and Quality of Healthcare: In PSC patients, 38% of patients report a delay in PSC diagnosis, more commonly in women. Only 10.8% of respondents have participated in a clinical trial, and 66.9% have a good understanding of PSC. 55.4% of people living with PSC work full time. There is a need to better understand experiences of PSC in minority groups.

PSC Symptom Profiles: In adults with PSC symptoms, the most common symptom was fatigue (83%) followed by daytime drowsiness (73%) and liver pain, cognitive impairment, anxiety, fatigue, and itch (all 62-63%). Participants were evaluated for the overall impact of their symptoms. A history of cholangitis attacks was linked to higher symptom burden. Higher symptom burdens were associated with worse overall health.

Liver Pain Experiences: Liver pain is an under-researched topic in PSC and often under-reported by the PSC community, both in terms of its frequency and severity. In a small representative sample of 17 PSC patients, researchers cataloged location, frequency, impact (life burden & interference) and severity of liver pain. These findings will inform the development of PSC-specific pain measures which can be useful in future research.

Brain Fog Experiences: Understanding brain fog (cognitive impairment) experiences for adults living with PSC is essential for the development of PSC-specific measures that can be used to aid in future research. This study captured severity, frequency, life interference and distress associated with cognitive impairment. These findings were used to customize an established measure of cognitive function originally developed by PROMIS, further strengthening and enhancing its relevance for future clinical research and treatment evaluation in PSC.

You can help drive research to identify treatments and a cure for PSC.

CONTACT US

Contact research@pscpartners.org to explore opportunities for patients, relatives, caregivers, clinicians, researchers, and other partners.

SUBSCRIBE

Sign up to receive the information that matters most to you—from research updates to our e-newsletters:

- **The Beacon** - Scientific research updates
- **The Duct** - Latest PSC news, updates and events
- **The Viaduct** - Patient Registry news

CONTACT US

- Visit pscpartners.org to learn more about PSC Partners
- Visit pscpartners.ca to learn more about PSC Partners Canada

REFERENCES

Barberio, B., Massimi, D., Cazzagon, N., et. al (2021). Prevalence of primary sclerosing cholangitis in patients with inflammatory bowel disease: A systematic review and meta-analysis. *Gastroenterology*, 161(6), 1865-1877. <https://doi.org/10.1053/j.gastro.2021.08.032>

Chapman, R., Fevery, J., Kalloo, A., et. al (2010). Diagnosis and management of primary sclerosing cholangitis. *Hepatology (Baltimore, Md.)*, 51(2), 660–678. <https://doi.org/10.1002/hep.23294>

Karlsen, T. H., Kaasen Jørgensen, K., & Bergquist, A. (2024). Medical treatment of primary sclerosing cholangitis: What have we learned and where are we going? *Hepatology*. <https://doi.org/10.1097/hep.0000000000001172>

Leung, K., Deeb, M., Fischer, S., et. al (2021). Recurrent primary sclerosing cholangitis: current understanding, management, and future directions. *Seminars in Liver Disease*, 41(3), 409-420. <https://doi.org/10.1055/s-0041-1730950>