

PATIENT REGISTRY IMPACT REPORT

2025

PSC Partners 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. This report describes the impact of the Patient Registry since its launch in 2014.

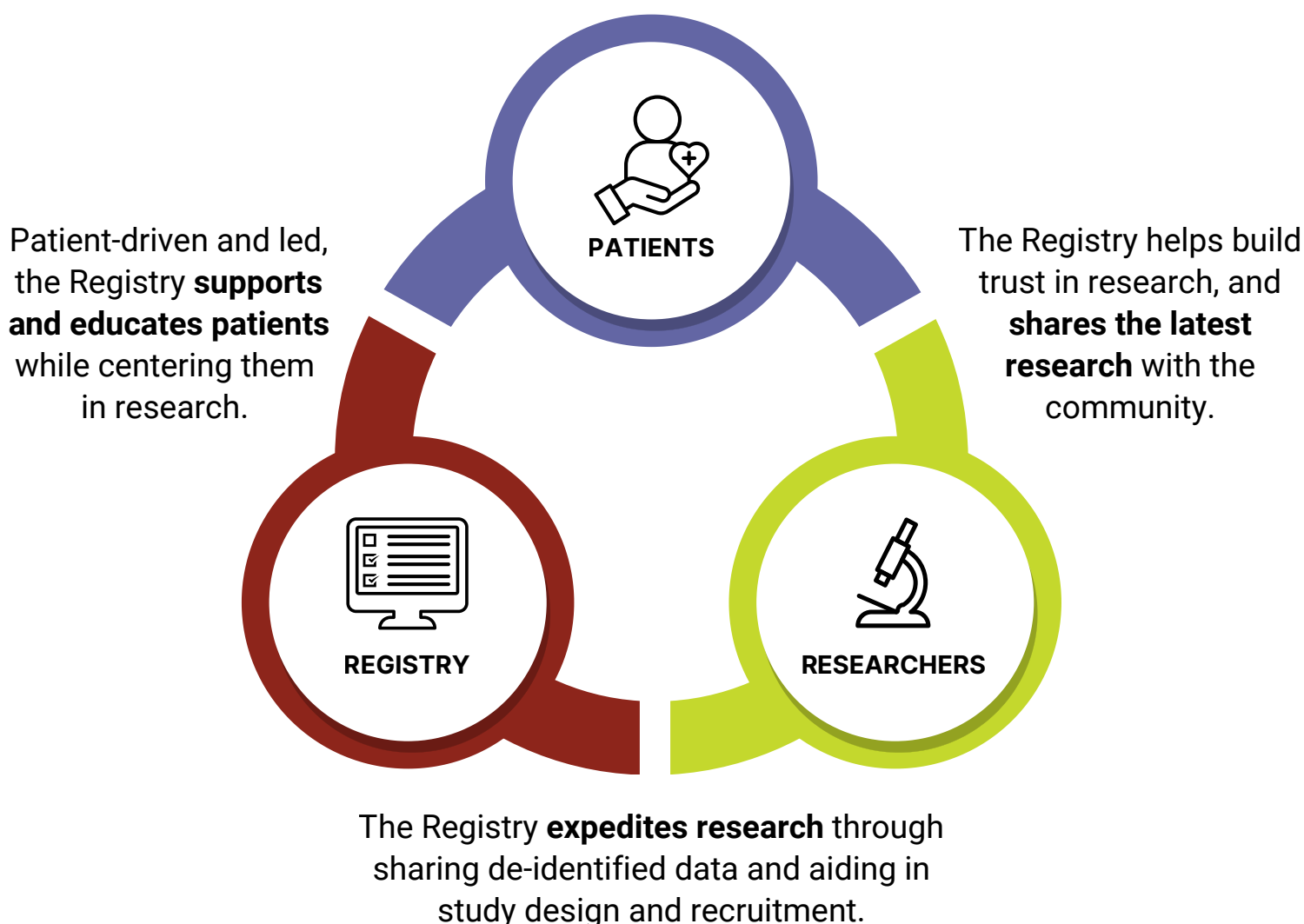
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The **PSC Partners Patient Registry** (Registry) is a digital platform where basic data on people living with primary sclerosing cholangitis (PSC) is collected and stored. Data is primarily collected through online surveys completed by the participant 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:
pscpartnersregistry.org

Powered By:
 **Matrix**

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).



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

L.N., Post-transplant Patient

2025 has been a banner year for the Registry.

Between new projects, expanded outreach and engagement, and the efforts of the dedicated Registry Team, by all metrics, the Registry is more active and engaged than ever before.

All this is only possible by active Registry participants, caregivers, and community members sharing their unique experiences to amplify the patient voice and move PSC research forward. **You have made the Registry a thriving hub for research,** and you have our deepest gratitude.



Another major milestone this year is the passing of the torch from Rachel Gomel, the Registry's founder and long-time director, to myself as the new director. While her presence in the day-to-day operations has ended, Rachel's vision for the Registry is the north star guiding us forward. She continues to serve as Senior Advisor for the Registry, offering invaluable wisdom and advice.

As a PSC patient, the Registry's mission could not be more important to me. In the six years since my diagnosis, I have seen major strides in PSC research. There have also been setbacks along the way: like most diseases, not every trial for a new treatment for PSC is a success. The most promising steps, however, have been made when patients are equal partners at the table. **Research is faster, more scientifically robust, and just plain better when patients are engaged throughout.** Your participation in the Registry has greatly amplified the patient voice in all research, and it is our mission to ensure your voice is heard loud and clear.

From the bottom of my heart, thank you to all patients, caregivers, clinicians, and researchers who have supported the Registry's mission of advancing patient-centered PSC research. Together, we will find a cure for PSC.

With hope,
Brian Thorsen
Registry Director, diagnosed in 2020



Registry Founder, Rachel Gomel (left), helping new participants sign up

The Registry was created with the guidance of the NIH Office of Rare Disease Research (ORDR). In 2012, PSC Partners was selected to participate in ORDR's Global Rare Disease (Patient) Registry and Data Repository pilot program. The Registry officially launched in April 2014 through Rachel Gomel's leadership and dedication.

The first publication for the Registry captured data from 715 participants as of September 2015.¹ This dataset showed that-- with the exception of fewer males-- the Registry's participants are similar to those of typical PSC studies. This was a key step in establishing the Registry's scientific validity.

Since then, the Registry has worked on dozens of studies with dozens of leading researchers. The Registry has helped shape the current state of PSC research by centering the patient voice. Through surveys like the "Our Voices" survey, PSC Partners has launched major research programs with patient and community input from day one. This includes the first-of-its-kind WIND-PSC study and the PSC Symptom Assessment Project.

The Registry has helped make it more viable to develop new treatments for PSC. Through education and outreach, the Registry has helped enroll many patients in clinical trials at all stages. Thanks in part to the Registry, PSC Partners has developed relationships with dozens of pharmaceutical and biotech companies. Companies are coming to PSC Partners earlier and earlier in the process of planning their clinical trials.

In a rare and highly variable disease like PSC, every patient's voice is crucial to put together the puzzle. Each new participant, each survey completed, provides new insights and brings us ever closer to a cure. Whether you have been with us since our humble beginnings or have only just learned of the Registry, we are in this fight together. Together, we are moving mountains.

ACTIVE SURVEYS

Clinical Survey: The core Registry survey collects information on a wide range of topics, such as PSC diagnosis, symptoms and complications, other diseases, family history, and more. A new Clinical Survey is available each year to track changes over time.

Health Equity Survey: This survey seeks to build an understanding of the impact of PSC and barriers to receiving high quality care.

PSC Flare or Cholangitis Attack Patient Survey:

Acute cholangitis is a significant complication in PSC, often with a high symptom burden. This survey captures participants' history of cholangitis and symptoms and care received during their most recent attack.

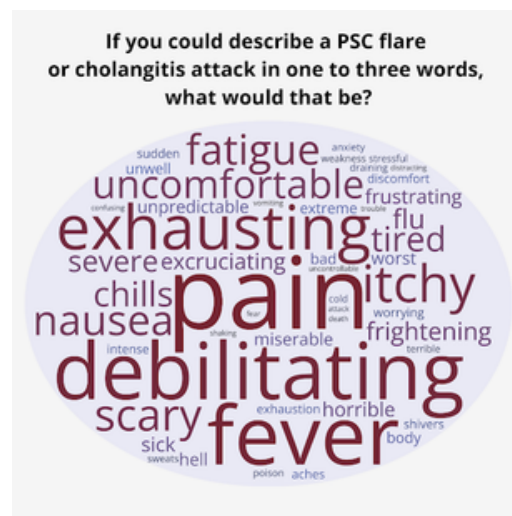
Healthcare Provider Submission Form: Registry participants can help others living with PSC to find a doctor who specializes in PSC care. Providers submitted via this form are listed on pscpartnersregistry.org.

PSC and Cholangiocarcinoma (CCA) Patient Survey: Conducted in collaboration with PSC Support (U.K.), this survey aims to understand the patient experience with CCA education and monitoring from their care team. and the community's priorities in advancing PSC-related CCA research.

Symptom Assessment Survey: As part of PSC Partners' Symptom Assessment Project, this survey collects responses to new patient-reported outcome measures for fatigue, brain fog, and liver pain to support validation of these 3 new measures.

For the Bereaved: If you have a loved one who had PSC and has since passed away, you can complete this survey on their behalf. Everyone with PSC is an important piece of the PSC puzzle, and their history is important to capture.

Past Surveys: Our Voices (for the Patient Focused Drug Development Forum) (2020-2023); **SF-36 Quality of Life** (2020-2023); **COVID-19 Questionnaire** (2020-2024); **Vancomycin and Insurance** (2023); **Timing for PROs in WIND** (2023)



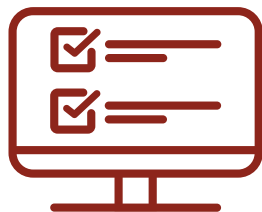
From the PSC Flare or Cholangitis Attack Patient Survey

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**.*

YOUR DATA IN ACTION

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.

EXTERNAL STUDY & TRIAL SUPPORT

Listed chronologically

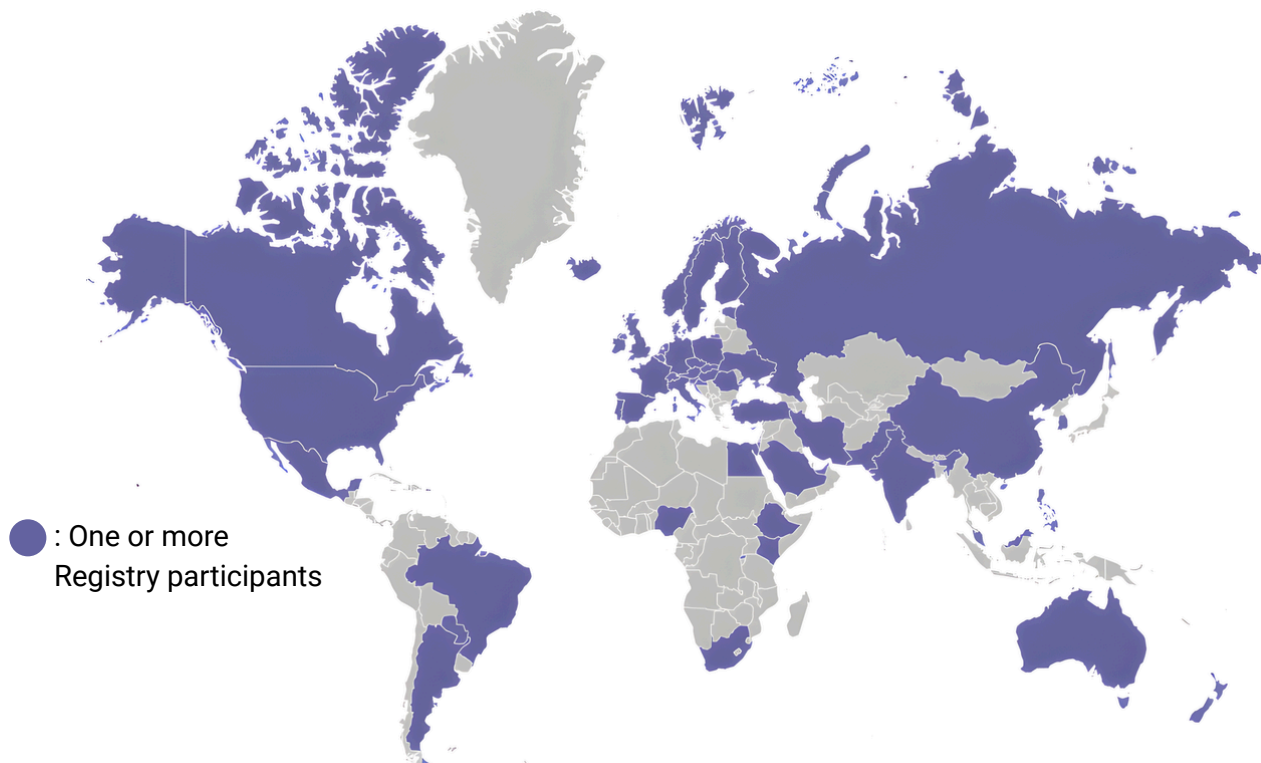
Potential Treatments for PSC: SHIP - Sulfasalazine in PSC (Brigham and Women's Hospital); DolPHin - Hydroxocobalamin in PSC (Brigham and Women's Hospital); VISTAS - Volixibat for treatment of PSC-related itch (Mirum Pharmaceuticals)

Other Studies: PSC and diet (European Reference Network); Quality of care in PSC (University of Miami); Patient perspectives on ctDNA in CCA surveillance (The Ottawa Hospital); Burden of illness in PSC (Initiate Consultancy, University of Sheffield, Mirum Pharmaceuticals); Patient-reported outcome validation (Lumanity); Shaping study design for the EMPOWER online wellness platform (University of Alberta, Duke University)

All study recruitment is confidential. The Registry team sends information to participants, and it is up to the participants to contact the study investigators to enroll. All external investigators agree to protect participant confidentiality.

GLOBAL REPRESENTATION

55 countries (and counting!)



PATIENT ENGAGEMENT

STUDY DEVELOPMENT

When new surveys are developed for the Registry, patient and caregiver feedback is sought throughout the process.

Survey testers help develop new questions, simplify where needed, and ensure that a wide variety of experiences can be accurately captured.

EDUCATION & EMPOWERMENT

All research made possible through the Registry is shared with the community in easy-to-read summaries and open-access texts. Plain language summaries of new trial recruitments also help engage all in the research process.

RESEARCH PARTNERSHIPS

DATA SHARING

Aggregate or de-identified individual-level Registry data can be provided for approved research. Additional questionnaires can be distributed to participants if needed.

STUDY RECRUITMENT

The Registry emails info on clinical trials and other studies to patients matching the criteria. For confidentiality, it is up to the patient to contact the investigators.

CLINICIANS HELP THE REGISTRY GROW!

Patients often learn of the Registry through their doctor. It is an easy and secure way for all with PSC to advance PSC research, whether pre- or post-transplant. Caregivers can join on behalf of children, or manage accounts on behalf of adults with permission.

We encourage you to discuss the Registry with your patients. Informational materials including brochures, flyers, and posters are available upon request.

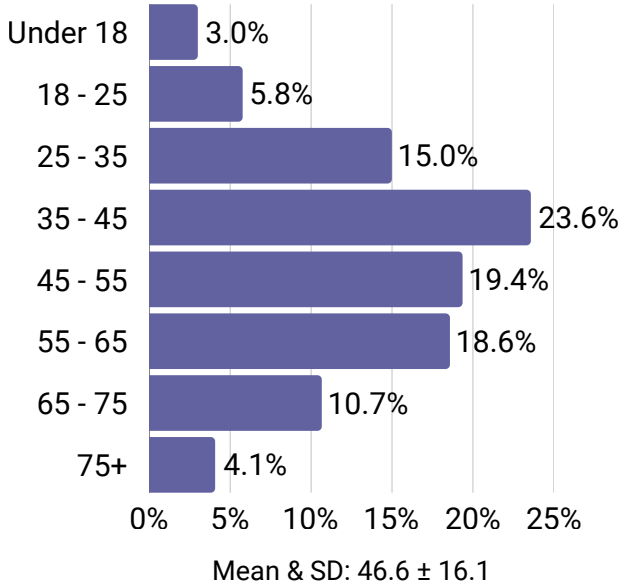


CONTACT US: registryresearch@pscpartners.org

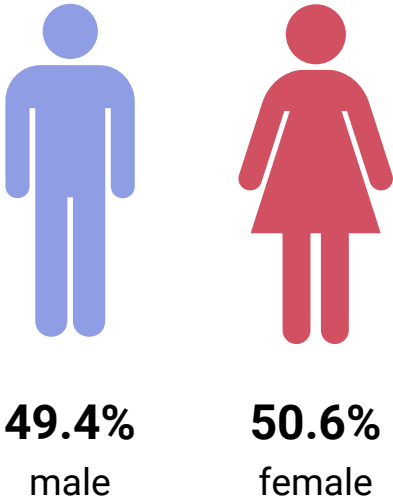
DEMOGRAPHICS

Collected during participant registration (n=2776)

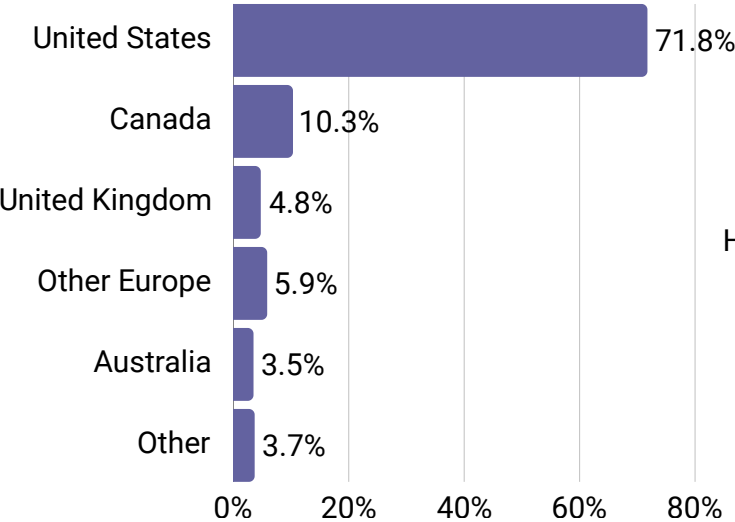
Age (years)



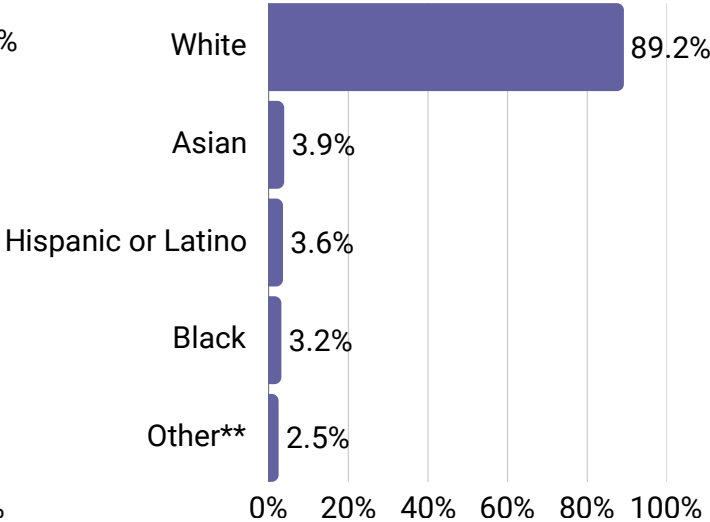
Sex



Country of Residence



Race / Ethnicity*



* Sum > 100% due to overlap

** Other races/ethnicities (n) where n ≥ 3: American Indian or Alaska Native (33); Middle Eastern (8); South Asian (8); Native Hawaiian, Māori, or Other Pacific Islander (6)

CLINICAL CHARACTERISTICS & HISTORY

From most recent valid Clinical Survey data per participant (n=2278)

Category	Value	Percent or Mean (SD)
Presentation of PSC	Age of Diagnosis	34.5 (15.9)
	Age of Symptoms Onset	31.8 (15.7)
	Years since Diagnosis	7.8 (8.8)
Type of PSC & Concomitant Liver Diseases	Small duct PSC (ever diagnosed)	14.9%
	Autoimmune hepatitis	11.9%
	Primary biliary cholangitis (PBC)	3.3%
	IgG4-related cholangitis	1.9%
Inflammatory Bowel Disease (IBD)	Any type	73.7%
	Ulcerative colitis	47.4%
	Crohn's disease	15.6%
	Indeterminate IBD, or unsure of type	5.4%
	Not sure if participant has IBD	5.3%
Transplant History	Received transplant	13.0%
	Years from diagnosis to transplant	9.7 (7.2)
	Multiple transplants (if post-transplant)	13.1%
	Recurrent PSC after transplant (ever)	20.3%
Surgical History	Colectomy	11.6%
	Cholecystectomy	30.9%
Cancer History	Cholangiocarcinoma (CCA)	1.9%
	Hepatocellular cancer (HCC)	0.6%
	Gallbladder cancer	0.6%
	Colorectal cancer	2.2%
	Lymphoma	0.9%
	Other cancers	8.4%

CLINICAL CHARACTERISTICS, CONTINUED

Category	Value	Percent or Mean (SD)
Symptoms, Ever	Fatigue, severe	72.1%
	Pruritus	60.6%
	Sleep disturbances	59.0%
	Liver pain	52.3%
	Pain over the belly	50.8%
	Weight loss, involuntary	47.3%
	Night sweats	46.8%
	Jaundice	41.6%
	Sadness or depression	41.0%
	Fever, unrelated to a cold/virus	40.0%
	Brain fog	27.4%
	Nausea	27.2%
	Vomiting	27.2%
Complications, Ever	Acute cholangitis	17.5%
	Ascites	15.2%
	Varices, with or without bleeding	14.2%
	Hepatic encephalopathy	9.1%
Current Medications	Ursodeoxycholic acid (UDCA)	42.8%
	Oral vancomycin	7.6%



15% of participants report ever participating in a clinical trial for PSC.



Brian Thorsen, Registry Director

Brian began as a volunteer for PSC Partners before joining the Registry team in 2023. He received a B.A. in Statistics from UC Berkeley, where he also worked post-grad as a math and statistics learning specialist. After his PSC diagnosis in 2020, he connected with PSC Partners to leverage his technical skills and teaching background towards PSC research, education, and advocacy.



Fernanda Quevedo, Assistant Registry Director

As Assistant Director, Fernanda is focused on advancing high-quality research and expanding collaborative partnerships. Prior to joining PSC Partners, she led research operations as Program Manager for the Resnek Family Center for PSC Research at Brigham and Women's Hospital, where she helped launch multiple PSC and IBD clinical trials and managed key external collaborations. Fernanda was drawn back to support the PSC community and individuals navigating the challenges of chronic health conditions like IBD.



Sharon Nanz, Registry Coordinator

Sharon joined our staff in 2018 after several years as a volunteer with PSC Partners. She holds a B.A. in English from Roanoke College, where she also worked as a staff writer and assistant in the marketing and communications office. Sharon volunteers with the Starzl Network for Excellence in Pediatric Transplantation and is an active member and volunteer at her church. She and her husband have three sons, a daughter whose PSC journey inspired Sharon's involvement in the PSC community.



Rachel Gomel, Registry Senior Advisor

Rachel has served as a fulltime volunteer at PSC Partners during the past twenty years. She is the founder of the PSC Partners Patient Registry and led the Registry as Director until recently. She currently serves as Senior Advisor to the Registry team and continues to be involved in many facets of PSC Partners. Rachel serves on the Board of Directors of PSC Partners and PSC Partners Canada.

1. Gomel R, Safer R, King J, Geraghty E, Everson G, Lindor K, Bowlus C. Characterization of A Patient Driven International Registry for Primary Sclerosing Cholangitis. In: AASLD Hepatology Posters (Abstracts 289-2348) Poster Sessions. Hepatology 62():p 93A-207A, October 2015. DOI: 10.1002/hep.28162
2. Thorsen B, Walmsley M, O'Dell S, Gomel R, Chatterley M, Rossi S. Identifying gaps in PSC patient education and understanding of cholangiocarcinoma screening and surveillance practices: results of a multinational patient survey. In: The Liver Meeting: 2025 Abstracts. Hepatology 82(S1):p S1-S2308, October 2025. DOI: 10.1097/HEP.0000000000001493
3. Evon D, Merkler K, Anderson C, Hatchett J, Safer S, Gomel R, et al. Symptom burden profiles identified among symptomatic adults with primary sclerosing cholangitis (PSC). In: The Liver Meeting: 2025 Abstracts. Hepatology 82(S1):p S1-S2308, October 2025. DOI: 10.1097/HEP.0000000000001493
4. Evon D, Wright L, Mkumba L, Lucas N, Merkler K, Safer S, et al. *"It's more like a dull, achy pain that's just constant"*: Liver pain experiences in adults with PSC to inform measure development. In: The Liver Meeting: 2025 Abstracts. Hepatology 82(S1):p S1-S2308, October 2025. DOI: 10.1097/HEP.0000000000001493
5. Evon D, Merkler K, Mkumba L, Wright L, Lucas N, Safer S, et al. *"This is not normal"*: Understanding cognitive impairment through qualitative interviews with adults with PSC to inform measure development. In: The Liver Meeting: 2025 Abstracts. Hepatology 82(S1):p S1-S2308, October 2025. DOI: 10.1097/HEP.0000000000001493
6. Yimam K, Kode V, Gomel R, Vyas M, Hatchett J, Thorsen B, et al. Diversity and socioeconomic status of patients with primary sclerosing cholangitis (PSC) and its impact on healthcare received, a survey of PSC Partners Patient Registry. In: Gastroenterology. Vol 169, Issue 1, S-1743. [gastrojournal.org/article/S0016-5085\(25\)04873-5/abstract](https://gastrojournal.org/article/S0016-5085(25)04873-5/abstract)