



BUILDING
COMMUNITY
& SEEKING
A **CURE,**
PIECE BY PIECE



PSC PARTNERS
SEEKING A CURE

PSC: THE BASICS

Primary Sclerosing Cholangitis (PSC) is a rare immune-mediated liver disease that damages the bile ducts inside and outside the liver. As the disease progresses to cirrhosis, some PSC patients require a liver transplant. Even after transplant, PSC can recur. Currently, there is no cure.

PSC strikes people of all ages, backgrounds, and lifestyles, but affects about twice as many males as females. Many PSC patients are asymptomatic at the time of diagnosis. It is estimated that there are more than 30,000 PSC patients in the United States.

Approximately 75 percent of PSC patients also have inflammatory bowel disease (IBD), most often ulcerative colitis (UC) and sometimes Crohn's disease. PSC also is associated with other autoimmune diseases. PSC patients are at increased risk for bile duct cancer (cholangiocarcinoma), colon cancer (for those patients with IBD), and other potentially life-threatening issues.

Although the causes of PSC are still unknown, research is underway to better understand the disease, develop more effective treatments, and, eventually, find a cure.



OUR MISSION

Founded in 2005, PSC Partners Seeking a Cure is a 501(c)(3) nonprofit organization. The mission is to provide education and support to PSC patients, families, and caregivers, and to raise funds to research causes, treatments, and cures for PSC.

OUR ORIGIN

As a person who had been a health and fitness fanatic her whole life, PSC Partners Founder and CEO Ricky Safer found her life thrown into a tailspin after her 2003 diagnosis with PSC. Between anxiety, fear of the unknown, anger, worry about her family, and a host of other emotions, Ricky searched for accurate answers and support, but quickly realized there were few places to turn.

She decided to be proactive, and, in 2005, with the support of her family, she formed PSC Partners Seeking a Cure. From the start, the nonprofit's mission was to provide support, fund groundbreaking research, create working partnerships between PSC patients, caregivers, physicians, nurses, nutritionists, pharmacists, researchers, and more. Now, 14 years later, the organization is going strong, and remarkable progress is being made on improving the lives of those affected by PSC by offering education, support, and hope in the search for improved treatments and a cure for PSC.

“There’s a certain comfort that comes with being surrounded by other people who just get it.”



MESSAGE FROM FOUNDER AND CEO RICKY SAFER

When I was diagnosed with primary sclerosing cholangitis (PSC) in 2003, I thought, primary sclero...what? I couldn't pronounce it, much less understand where it came from or what to do about it. Like my fellow PSC patients, this diagnosis took me by surprise and changed my world. The start of my journey with PSC was so lonely and confusing, despite the love and support of my family and friends.

This void inspired me to create PSC Partners Seeking a Cure, and, in 2005, we held our first conference to provide education and support to the PSC community. More than 84 enthusiastic supporters attended that first conference, and I knew we were headed in the right direction. The annual conference now attracts more than 300 patients and caregivers from around the world. Together, we form a unique community that acts as a family for PSC patients and caregivers and offers hope to all of us. I'm proud to say we've invested more than \$3 million in research, and we're still going strong... together.

In 2018, a significant accomplishment was our collaboration with the PSC community to secure the designation of a unique disease code (known as an ICD-10 code) for PSC - K83.01. The code will help move PSC research forward by allowing

doctors and scientists to finally identify PSC patients specifically, rather than utilizing a single code for all diseases involving cholangitis. Other accomplishments in 2018 included:

- Expanding the patient registry
- Supporting additional research grants
- Starting a Young Investigator Grant Program
- Presenting the patient voice at international hepatology conferences
- Participating in international collaborations

These accomplishments - combined with our accelerating momentum and positive breakthroughs in research - have led us to a place of real hope and optimism. Thanks to strong international partnerships formed after more than a decade of effort, we have a great group of dedicated people working together in innovative ways toward the same goal of improving the lives of PSC patients, and, eventually finding a cure. We are dedicated to ensuring that our organization continues to move forward in an honest and transparent way, and we want to say thank you to the PSC patients, families, caregivers, medical providers, researchers, volunteers, and donors. Thank you for your trust in us and your steadfast support. We truly are together in this fight, whatever it takes.

Sincerely,
Ricky Safer



**We are together in the fight,
whatever it takes.**

SEEKING A CURE: PROGRAM SERVICES

EDUCATION AND SUPPORT

PSC Partners Seeking a Cure holds a conference every year in conjunction with a major medical center with an expertise in PSC. The conference draws patients and caregivers (spouses, partners, parents, siblings, and friends) to learn about the latest research on treatments, medications, procedures, and the disease outlook. During the weekend, there are discussion and support sessions with physicians and other attendees. Conference speakers are national and international leaders in PSC. PSC Partners also educates and supports our community via our website, monthly newsletter, podcasts, social media, collateral materials, conference booths, mentor program, and more.

THE GRANTS PROGRAM

Every year, on the recommendation of the Scientific/Medical Advisory Committee (SMAC) and a final vote of the board of directors, PSC Partners Seeking a Cure supports the most promising international PSC research. Grants are awarded to projects addressing an important and novel, basic, or clinical question related to PSC. For PSC Partners, this is a crucial endeavor bringing the PSC community closer to new treatments and an eventual cure. Through 2018, 76 grants have been awarded internationally for a total of \$3,191,666. Additionally, 2018 was the first year PSC Partners offered Young Investigator Awards to promising researchers who are at the early stages of their research career and are interested in PSC.



2018 Grants:

Gianfranco Alpini, Indiana University School of Medicine
TREATMENT OF PRIMARY SCLEROSING CHOLANGITIS USING EXTRACELLULAR VESICLES

David N. Assis, Yale University School of Medicine
AN INVESTIGATION OF BILIARY-IMMUNE INTERACTIONS AND CANDIDATE DRUG-SCREENING USING NOVEL BILE-DERIVED ORGANOID TECHNOLOGY

Annika Bergquist, Karolinska Institutet, Stockholm, Sweden
PSCATOR – ATORVASTATIN FOR THE TREATMENT OF PRIMARY SCLEROSING CHOLANGITIS (PSC) – A RANDOMIZED CONTROLLED STUDY

Michael Choi, Harvard Medical School
NOVEL USE OF MICRORNAS TO ATTENUATE PSC INDUCED LIVER FIBROSIS

Aliya Gulamhusein, Toronto Centre for Liver Disease, Toronto, Ontario, Canada (Young Investigator Award, granted in partnership with PSC Partners Canada)
CHARACTERIZATION OF CLINICAL AND IMMUNOLOGIC PHENOTYPES OF PATIENTS WITH RECURRENT PSC AFTER LIVER TRANSPLANTATION: AN INTERNATIONAL NETWORK STUDY

Chantal Housset, Saint-Antoine Research Center, Paris, France
VITAMIN D, NUCLEAR RECEPTOR-INDEPENDENT ACTIONS IN PRIMARY SCLEROSING CHOLANGITIS: POTENTIAL ROLE OF PROTEIN DISULFIDE ISOMERASE FAMILY A MEMBER 3 (PDIA3) AS A THERAPEUTIC TARGET

Wei-Yu Lu, University of Birmingham, Birmingham, UK (Young Investigator Award)
DISSECTING THE ROLE OF T REGULATORY CELL POPULATIONS IN PRIMARY SCLEROSING CHOLANGITIS (PSC) WITH A MOUSE PSC MODEL

Mario Strazzabosco, Yale University School of Medicine
UPREGULATION OF BILIARY EPITHELIA INNATE RESPONSES IN PSC

Veronica Miller, UC Berkeley, School of Public Health The Forum For Collaborative Research/PSC Forum
ESTABLISHING PSC ENDPOINTS FOR CLINICAL TRIALS

American Association for the Study of Liver Diseases (AASLD) Foundation Award
Dr. Angela Cheung, BSc, MD, FRCP, Mayo Clinic, Rochester, MN
HIGH-RESOLUTION METABOLOMICS AND EXPOSOMICS IN PRIMARY SCLEROSING CHOLANGITIS AND PRIMARY BILIARY CHOLANGITIS UNCOVERS NOVEL, DISEASE-SPECIFIC ASSOCIATIONS IN BILE ACID AND AMINO ACID METABOLISM AND ENVIRONMENTAL TOXICANT EXPOSURES

The European Association for the Study of the Liver (EASL) Award
Dr. Knut Stokkeland, Karolinska Institutet, Stockholm, Sweden
STATINS ARE ASSOCIATED WITH REDUCED MORTALITY AND MORBIDITY IN PRIMARY SCLEROSING CHOLANGITIS

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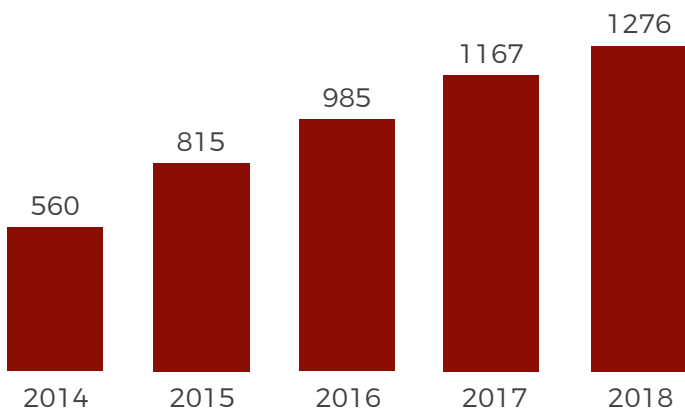
EXPANDING PARTNERSHIPS

Through a new emphasis on data analytics, PSC Partners is committed to expanding our reach and moving our mission forward by identifying research partners and clinicians knowledgeable about PSC. Together, we are determining the most impactful ways to collaborate, educate, and improve life for those impacted by PSC.

THE PATIENT REGISTRY

The PSC Partners Patient Registry is a vital research component that adds critical patient perspectives to clinical trial protocols to make research participation easier. Through the registry, PSC Partners helps recruit patients for studies and trials, and provides de-identified registry data for PSC research. Since it began in 2014, the registry totals have grown by 128%, for a year-end total of 1,276 participants.

Patient Registry Totals



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2018: THE HIGHLIGHTS

Thanks to the generous support of our donors and the invaluable engagement of everyone in the PSC community, PSC Partners Seeking a Cure had a productive and impactful year!

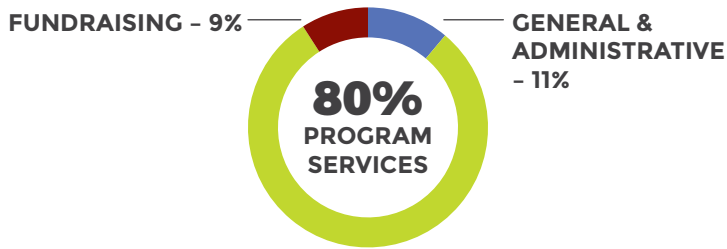
PSC Partners Seeking a Cure:

- Awarded **two Young Investigator Awards** for the first time.
- Funded annual awards to support PSC research at AASLD and EASL.
- Continued its ongoing, valuable collaboration with PSC Partners Canada.
- Collaborated with research teams conducting clinical trials and studies.
- Hosted a successful conference for patients & caregivers, June 23, 2018, in Sacramento.
- Collaborated with Mayo Clinic to plan for the 2019 Conference, **June 21-23, 2019**, in Rochester, MN.
- Launched the **Living With PSC podcast series**, which explores the latest research and knowledge about PSC, including patient perspectives, research updates, and the latest news from the PSC world.
- Added **three PSC thought-leaders** to the Scientific/Medical Advisory Committee.
- Welcomed **three new community members** to the PSC Partners' board of directors.
- Awarded **nine new research grants** in the 2018 cycle, bringing the grand total to **more than \$3 million invested in the most promising international PSC research**.
- Increased PSC patient registry participants to **1,276** – and growing!
- Joined the ChiLDreN's Network after PSC was added to their list of diseases to be included in pediatric research.
- Represented the voice of the PSC community at international medical conferences: AASLD, EASL, CALD, and the PSC Forum for Collaborative Research.
- Spread the word about the many unmet needs of PSC patients through **PSC Awareness Day on October 29, 2018**.
- Secured an **ICD-10-CM code specific to PSC, K83.01**. Having a code specifically for PSC is an incredibly exciting milestone, because proper coding enables clinicians and researchers to effectively track PSC statistics, which improves vital research into treatments and cures.

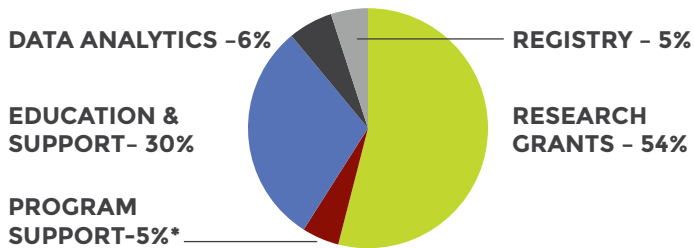
FINANCIAL HIGHLIGHTS

For the years ending Dec. 31, 2018 and 2017

ANNUAL OPERATING EXPENSES



PROGRAM SERVICES



*Continually focusing on the mission while giving our dedicated staff the support needed to meet these goals.

ASSETS, LIABILITIES & NET ASSETS

	2018	2017
ASSETS	\$1,671,506	\$1,730,436
LIABILITIES	\$3,917	\$5,311
NET ASSETS	\$1,667,589	\$1,725,125

PSC Partners will continue to invest in high-quality grants and other program services to further our mission of finding treatments and a cure for PSC.

SUMMARY OF REVENUES & EXPENSES

	2018
CONTRIBUTIONS	\$799,674
CONFERENCE REVENUE	\$227,996
INVESTMENT REVENUE	\$3,526
TOTAL REVENUE	\$1,031,196
EXPENSES	\$1,088,732
CHANGE IN NET ASSETS	(\$57,536)

BOARD MEMBERS

PSC Partners is fortunate to have a talented and motivated group of volunteers serving on its board of directors. Members come from a variety of backgrounds and experiences and are united by a common cause to find a cure for PSC. All board members are either PSC patients or caregivers.

Ricky Safer - CEO
Fred Sabernick - Chair
Joanne Grieme - Vice Chair
Britt Moore - Secretary
Jennifer Sims - Treasurer
Rachel Gomel - Registry Coordinator
Stuart Barnett
Kathy Halloran
Joanne Hatchett
Wes Hendrix
Willie McKinney
Matt McMurtry
Ken Shepherd
Travis Stoltzfus
Bill Wise

SCIENTIFIC/MEDICAL ADVISORS

The Scientific/Medical Advisory Committee (SMAC) shares their time and expertise to direct the path of the PSC Partners research program in order to find new treatments and a cure for PSC. All research grant proposals submitted to PSC Partners are reviewed annually by the SMAC.

Dr. Christopher L. Bowlus - Co-Chair
Dr. Richard Green - Co-Chair
Dr. David Assis
Dr. Dennis Black
Dr. John Eaton
Dr. Bertus Eksteen
Dr. Denise M. Harnois
Dr. Gideon Hirschfield
Dr. Johannes R. Hov
Dr. Keith Lindor
Dr. Stephen Miller
Dr. Cyriel Ponsioen
Dr. James Tabibian





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