

# The Duct

Bringing you news on PSC



research, education, and support

PSC Partners Seeking a Cure Foundation Summer 2011 - I



## We Have Lost a Treasure: Pat Bandy

*We have lost a treasure. As our supremely dedicated newsletter editor and co-designer of our website, Pat worked behind the scenes to help us reach a new level of professionalism. Due to her extreme intelligence, sense of perfectionism, and ability to see the whole picture as well as every minor detail, Pat was a constant and valuable mentor to me. While I am planning future projects or just writing a post, I still hear her determined voice in my head, giving me helpful reminders.*

*Pat was also a dear friend who never forgot any tidbit of information that I had shared with her. She was thoughtful, loyal and always concerned about others. I miss her greatly.*

*Without Pat, PSC Partners is left with a void that will be difficult to fill. She has left us all a beautiful legacy.*

*Ricky Safer*

**\* It is with the utmost respect that we dedicate this post-conference newsletter issue to Pat. \***

## A Tribute to Pat by Sandi Pearlman

When I first met Pat, I was newly diagnosed and unsure of what PSC would bring. I had barely met another PSCer and I didn't know anyone who had had a transplant. Then, I met Pat and her husband Bill. Not only did Pat and I have journalism and a love of grammar and the written word in common, we both also understood the shorthand of editors and how the benefits of train travel are far superior to that of plane. Oh, yeah, and we both had this rare disease called PSC, new for me, post-transplant for Pat.

Pat and I were very different people. She was elegant and reserved and loved rules and order. I'm not elegant and I'm anything but reserved and...well, I'd much prefer free-form living to structured. And although it sometimes caused us to shake our heads at each other, it also made for a well-balanced

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team...my outrageousness combined with her tried and true.

At the core was our desire to help; to help each other and to help PSCers out there feel less alone, more informed and anything but helpless. Pat relentlessly and tirelessly slaved over every single issue of The Duct long past when I thought everything looked fantastic. Together, we'd work on formatting rules, something our grammar-based brains actually found fun, and layout principles and even editing of articles where we could discuss whether to use "sic" or just change text. It may sound mundane or silly or like the littlest of trivialities to some, but for Pat and me it was the basis of a friendship, one that included health updates and check-ins, check-ups and venting, laughter, love and respect. I didn't get to know Pat as well as I wish I would have. I didn't get to have years to debate the merits of train travel versus plane or how skiing is far superior to sitting indoors under a cozy blanket with hot cocoa and Scrabble (my argument). I didn't get months and years to discuss all these things and more and it makes me sad and brings tears to my eyes. But I did get to know Pat and to be a part of her life, and she mine. And for that, I'll be forever grateful.

So, Pat, wherever you may be, I hope there are lots of fluffy slopes to ski on, a parlor car on a train reminiscent of the one in North by Northwest and a good book to pass the time until we can pick up our last chat where we left off. From me, from all of us, thanks for helping us to feel more connected, more informed and for letting us be a part of your life. You are a special part of ours. We'll miss you.

## That Glow of Hope

Conference in Sacramento, April 29–May 1  
Co-sponsored by the University of California Davis Health System



Every year during the week before the conference, after I've finished months of work on conference details, the same question keeps gnawing at me. Will the "glow" come back this year again? I

knew that the hand picked speakers would enlighten us as always with updated medical information and that our chosen venue in sunny California would be a comforting place to meet. The conference attendees would arrive from near and far, eager to learn and to share their experiences with PSC. But, once this was all in place, would that special aura magically emerge? This glow encompasses a sense of no longer feeling alone, of being surrounded by a positive energy and power, and a can-do attitude that come from being all together ... Would that glow of hope

that rejuvenates all the conference attendees and that carries us through the rest of the



year  
wondrously  
reappear?

Thanks to  
the amazing  
attendees  
who joined  
our seventh  
annual

conference in Sacramento, the magical glow was evident throughout the weekend. This year, we had 223 attendees from 31 different states. Because this was the first time we were holding a conference on the west coast, 34 percent of the attendees were from California. Eight foreign countries were represented: Canada (25 attendees), England, Wales, Germany, Italy, Norway, Israel and Australia. The growing diversity of our group makes us stronger and spreads our mission to the far corners of the earth. We greatly appreciate all the attendees who



travel long  
distances to join us  
at the conference,  
and especially our  
international  
guests.

A special thank you  
goes to William  
and Catherine

Williams who came from Wales to represent the PSC-Support group from the UK.

I can't even begin to thank two people, without whose help the conference would never have materialized. It was pure pleasure to work with Dr. Chris Bowlus of the University of California in the

## TABLE OF CONTENTS

### Part I

Tribute to Pat Bandy - Ricky Safer, Sandi Pearlman	p. 1
That Glow of Hope - Ricky Safer	p. 2
The Annual Conference Experience	p. 7
Fact-O-Rama	p. 16
<b>Conference Session Summaries- Arne Myrabo</b>	
Introduction to PSC - Dr. C. Bowlus	p. 17
Urso Pro vs Con - Dr. G. Hirschfield, Dr. M. Peters	p. 19
Managing PSC Symptoms - Dr. N. Török	p. 20
PSC Partners Research Overview, Dr. D. Rhodes & Dr. S. Miller	p. 23
PSC Immunology: Lessons to be Learned from PBC - Dr. E. Gershwin	p. 25
New PSC Genes - Dr. E. Melum	p. 26

### Part II

Tribute to Ivor Sweigler - Ricky Safer, Rachel Gomel	p. 1
<b>Saturday Breakout Session Summaries</b>	
IBD Management in PSC - Dr. S. Tejaswi	p. 3
Transplantation & MELD Score - Dr. M. Bonacini	p. 6
Interpreting your Blood Tests - Dr. C. Bowlus	p. 8
The Role of ERCP in PSC - Dr. J. Ostroff	p. 8
Natural Cuisine - Chef Russell Michel	p. 10
Liver Transplantation for PSC - Dr. J. McVicar	p. 11
Coping for PSCers - Dr. P. Burke	p. 15
Pediatric PSC - Dr. P. Rosenthal	p. 17
Screening & Treatments for CCA - Dr. C. Selmi	p. 19
Practical Considerations Before Transplant	p. 22
Caregivers: Helping Us Help Others	p. 27
Medical Therapies on the Horizon - Drs. Bowlus, Selmi, Hirschfield, Melum and Zern	p. 29
Effects of Diet on Inflammation - Dr. C. Stephensen	p. 31
Financial & Insurance Considerations for Transplant - C. Allman	p. 32
<b>Healthy Living Perspectives: Nutrition, Exercise &amp; Alternative Therapies</b>	
Integrating Chinese Medicine & PSC - Dr. M. Cohen	p. 34
<b>Home Health</b> - Cindy Campbell, RN, BSN	p. 36
<b>An Overview of Disability Law</b> - Becky Long, Esq	p. 37

planning of speakers and topics for the weekend. Every step of the way, Dr. Bowlus went out of his way to ensure that this would be our best conference ever! Joanne Hatchett, our conference co-hostess, was the efficient and creative energizer bunny that we all know and love. With her superb organizational skills, she helped make the conference work seamlessly.



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Thanks to our conference sponsors, each year we are able to keep our conference

registration fees as reasonable as possible. The conference sponsors listed below covered more than 50 percent of our costs for putting on the meeting!

**Premier Platinum Sponsors:** ITW (Illinois Tool Works), Dike and Rilee Ajiri, Anonymous Donor

**Gold Sponsors:** Don and Ricky Safer

**Silver Sponsors:** Stephen and Joanne Hatchett, Ken and Patty Shepherd, Jeff and Reggie Belmont, Jason and Jennifer Drasner, Division of Gastroenterology and Hepatology at the University of California Davis, Global Treatment Options

**Bronze Sponsors:** Jennifer Soloway and Mark Stivers, Genentech, Shire, Peter Holmgren Family

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Attendees enjoyed our PSC merchandise booth, and many of us are now sporting PSC Partners hoodies, tees, sweats and hats. Dr. Chris Bowlus held a blood draw for our attendees to participate in University of

California Davis PSC research studies. Also for the first time this year, we had a teenaged group of attendees. The five teenaged participants formed strong bonds and hope to return next year. We plan to prepare a special program for them. I'd like to thank Tawny Holtz, who was a fantastic mentor to our teens.

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For those who couldn't attend our conference, here is a quick rundown of the weekend activities. On Friday, pre-conference activities included a casual brunch at the beautiful California Capitol Building, followed by our afternoon session, *Healthy Living Perspectives: Nutrition, Exercise and Alternative Therapies*.



Attendees enjoyed a mini Farmers Market with California products, followed by our informative keynote address by Dr. Misha Ruth Cohen who spoke on *Integrated Chinese Medicine and PSC: Accessing the Best of East and West*. Afterwards, attendees could choose two breakout sessions from the following topics: *Kinetics for PSCers*; *Connecting Food, Health and the Environment*; *The Glories of Eating Seasonally and Locally*; *Gardening - Good for the Gut, Good for the Soul*; *Yoga: Feeling is Believing*; *A Gluten Free Update*; *Coping with a Gluten Free Diet in Real Life*; and *An Integrated Chinese Medicine Case Study*

in PSC.

In the late afternoon on Friday, we offered two new and much appreciated sessions for first time attendees: *Newcomer Orientation* and the *Mentor/Mentee Meet and Greet*. For our Friday evening Welcome Reception and dinner, attendees were seated by regions to meet other PSCers and caregivers from their area.

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Saturday was an inspiring educational day for all of us. We avidly listened to the incredible presentations given by all the excellent speakers chosen by Dr. Chris Bowlus.



The speakers covered a wide variety of PSC and PSC research related topics, and welcomed questions from the audience. They brought us up to date on new information and research, and gave us hope for the future. All the speakers were very engaging and compassionate. This year, we also included some outstanding breakout sessions, some of which were led by our own members: Philip Burke, Aubrey Goldstein, Becky Long, Joanne Grieme and Mike Pearlman.

If you'd like to view the speaker PowerPoint slides and Karen Pearlman's photos that perfectly capture the spirit of the weekend, please

click on <http://www.pscpartners.org/PSCConf11/index.htm>.

For the summaries of each presentation written by one of our volunteers, please read through the two parts of this newsletter edition.

Saturday's presentations were the heart of our conference, and they were phenomenal! I'd like to


thank all our speakers again for generously giving us their time on a

weekend and for sharing their expertise and advice with all of us eager listeners.

Our Saturday evening banquet was a celebration of hope and included Hal Smith's presentation of a \$10,000 check for PSC research from their organization, *Hoops4Healing*, our *Inspiring Stories Panel*, our volunteer awards presentation and our exciting announcement about having raised \$173,000 for PSC research through our Road to Sacramento fundraiser! Philip Burke's operatic rendition of *The Impossible Dream* was an unforgettable experience.

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Congratulations to the following volunteers who received our 2011 special awards:

We thank our official conference photographers, Karen Pearlman, of K Pearlman Photography and Arne Myrabo for covering the conference with cheer and with great talent. Karen is generously donating receipts received for photos you purchase to PSC Partners. Her website is: <http://kpearlmanphotography.com>. 

**Male Partner of the Year:** Dr. Chris Bowlus

**Female Partner of the Year:** Rachel Gomel

**Corporate Citizen Award:** Google Inc.

**Appreciation Awards:** Dike and Rilee Ajiri, Hoops 4 Healing, Anonymous Donor

**Fundraiser Awards:** Libbie Bailey, Steve and Joanne Hatchett, Daniel Schachter, Ken and Patty Shepherd, Craig and Ali Wiele, Sheldon and Robin Wohl

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Sunday morning was our chance to break into our various PSCer and caregiver groups and spend the morning talking about all our concerns with others who understand. It was a wonderful time to process experiences, questions, suggestions, and just to laugh and cry together. When the conference ended midday, it was very difficult to say goodbye to the new and old members of our PSC family. There is such a strong feeling of support and hope when we are all together!

Thanks to all our volunteers, the entire weekend ran smoothly. Meegan Carey did a phenomenal job keeping every detail under control, always with a smile on her face! A heartfelt thank you to Arne Myrabo for writing summaries for all of Saturday morning's general session presentations and for creating our 2011 conference website ([www.pscpartners.org/PSCConf11/index.htm](http://www.pscpartners.org/PSCConf11/index.htm)), to Joanne Grieme for producing our name tags and the attendee booklet, to

Sandi Pearlman for running our Mentor/Mentee program and the Newcomer Orientation, to Ken and Mary Ann Long for helping us create our new Team Leader project, and to Karen Pearlman for her beautiful photographs that chronicle the entire weekend. I'd also like to thank our volunteers who were in charge of manning the PSC Partners booths all weekend: Joanne and Steve Hatchett, Joanne and Steve Grieme, Beth Merrill, and Phyllis Zisman; our Team Leaders: Lee Bria, Rachel Gomel, Joanne and Steve Grieme, Joanne and Steve Hatchett, Allison Long, Ken and Mary Ann Long, Sandi Pearlman, Nick and Theresa Valenti, Julianne Vasichek, and Tim Wholey. A huge thank you to our notetakers who have shared their breakout session summaries with us in this newsletter edition and to all the other unnamed volunteers who made this weekend such a huge success!

As I look back on our 2011 conference, I continue to be energized by the unique spirit of our PSC community. I love reconnecting with my old friends, and I especially welcome the new attendees who added a new, passionate dimension to the group. You are all a remarkable network of inspiring people, and I thank you for your support in helping us in our goal of eventually finding a cure for PSC.

PSC Partners  
welcomes Ken  
Shepherd on the  
Board of  
Directors

*Next year at the MAYO CLINIC, ROCHESTER*

*You're invited to the 8th Annual Patient Conference held by PSC  
Partners Seeking a Cure at the Mayo Clinic, Rochester  
May 4 to May 6, 2012*

*Check our website which will be updated periodically*

<http://www.pscpartners.org/nextannual>

That expected glow of hope was in evidence all weekend. I'll end with some short quotes from some of our 2011 attendees, who have described their experience brilliantly.

*"Thanks for introducing me to a world where people understand."*

*"I feel hope and encouragement and support and that is priceless." "Because of your efforts, we face this disease armed with information and support rather than the tears and fear we had a few years ago."*

*"Your organization has been a life raft for us from the dark devastating days after our daughter's diagnosis to this wonderful weekend filled with inspiration and hope."*

*"Being normal for a few days each year raises my spirits in a way I really can't describe, but I always leave the conference feeling so uplifted and positive and looking forward to the next one."*

I'm already planning for our 2012 conference at the Mayo Clinic in Rochester for the weekend of May 4-6, so save the date, and try to join us there!

*Ricky Safer*  
*President*

**Addendum:** *I can't begin to thank Rachel Gomel enough for stepping in to become the editor for this all-important post-conference newsletter edition.*

### **The Annual Conference Experience**

*We asked seven participants at the conference to share their experiences.  
Most were first-time attendees.*

## **We Didn't Know What to Expect**

*By Ali & Craig Wiele (parents of a pediatric PSCer)*

We didn't know what to expect heading to our first PSC conference, but there was never a question whether or not we would attend. Once we found the PSC Partners website after our 4 (then 3 ½) year old daughter was diagnosed with PSC last August, and heard how amazing the conferences are from the PSCMOMS group, we booked our tickets to Sacramento.

From the first night, we knew it was going to be a special weekend...the people we met were just incredible. Our mentor immediately took us under her wing, welcoming us into the fold and making us feel comfortable right from the start. Despite being tired from our trip, we stayed up well into the night, talking and laughing with our new friends as if we'd known them for years.

The brave and inspiring stories we heard over the next two days were so heartfelt and moving, we were glad we had packed the tissues! Even through our tears, the strength and resilience of all the attendees battling PSC helped us to feel stronger after having met them. We are grateful to Ricky and Don for connecting us all together, for their wonderful leadership and for the support and encouragement that surrounded us all weekend.

We pray as all caregivers do, that our little girl's journey with this disease is a smooth one, and that she remains asymptomatic until an effective treatment is available. But it is comforting to know that the new friends we made in Sacramento will be there for us wherever this road may lead. We are committed, even more so since the conference, to raising awareness and funds for PSC Partners, and to doing what we can to help advance research as quickly as possible... "Together in the fight, whatever it takes."

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## Impossible to Put into Words

*By Tawny Holtz (a 20-30's PSCer)*

My experience at the PSC Partners conference was in one word AMAZING. It was wonderful to see my friends. I am always surprised to see how much I can learn about PSC even after fourteen years of living with the disease. Yet there is so much more to the conference than learning new information. It's impossible to put into words all that I gain from attending these conferences.

I was really lucky to have my husband, mom, dad and sister with me this year. It felt good to see them realize what it means to live with PSC. And after we were back home, it was equally great to see their excitement and desire to take part in helping to find the cure! Their excitement was so contagious that my two sisters and my niece would like to attend the conference with us next year. I would like to thank everyone who worked so hard to make this conference possible. It was FANTASTIC!

\*\*\*

## Was a Blessing to Attend

*By Hal Smith of Hoops4Healing, a foundation created by four PSCer basketball coaches whose beautiful story can be read on their site <http://www.hoops4healing.org/about.html>. Hal has had two liver transplants and a kidney transplant.*

I had the opportunity to attend and be part of the PSC Partners Conference in Sacramento. It was an outstanding experience. I really did not know what to expect, but had an inkling from Chuck Cooper, fellow founding board member of Hoops 4 Healing, who had attended last year. I wish the group existed back in the mid-nineties when I was first told I had PSC.



Some random thoughts: 1. Was amazed at the passion of those involved with the leadership of PSC Partners 2. Came in contact with PSCers of many ages . . . did not realize so many young men and boys, women and young girls were suffering with the disease 3. Learned an immense amount during the various sessions led by many professionals and was deeply touched by the amount of support afforded to, and given by, those in attendance with PSC 4. So much hope exists for the future in regards to current research to find a cure, reasons why we get it, and finding better ways to cope with it 5. Many caregivers were there even if their loved ones with PSC were not, and that really affected my wife and me 6. Was personally blessed with being able to meet the chair of the Scientific/Medical Advisory Committee and to be able to present him with a check from our foundation, Hoops 4 Healing 7. Was greatly encouraged personally by those in attendance even though I have already had two liver transplants (and a kidney) as a result of having PSC. I hope and pray that I was able to have been an encouragement to them, including to the other post-transplant people in attendance. It is our desire as a foundation to have more of us in attendance next year at the Mayo Clinic. The work of this group is to be admired and copied. Was a blessing to have been able to attend. See you next year!

\*\*\*

## Fourteen Year-Old Me

*By Chelsea Yaeger*

Many things ran across my mind as I boarded the plane with my mother. I was excited to meet people "like me." I was scared that the things I heard would be disheartening. I was worried because I knew I'd be the youngest. I wondered what people would think about me, and I wondered what I'd think about other people.

As we landed, I still didn't know what to expect. Walking into the hotel room on Thursday night, I couldn't sleep, waiting for Friday to roll around. It did, and brought with it a weekend of unforgettable memories that I absolutely did not expect. It exceeded my great expectations.

For me, it was the laughing and the talking, the crying and the hugging that made it worthwhile. And meeting new friends - no, meeting new FAMILY. The people I met are my true family, each and every one of them. The PSCers, and the caregivers.

And yeah, I heard some disheartening things. I was the youngest there. And people can think what they want... But I had an amazing time. I think about all of the people I met there - each one touched my life forever. I am staying in contact with most of them, and I miss them all dearly.

If I could change one thing about the weekend I spent with them, I just wouldn't change a thing. Because it was perfect in each and every way.

*Fourteen year-old me*

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## A Newcomer's Perspective

*By Sandra Burke, RN, BSN, mother of a PSCer*

Though my son Philip was diagnosed with PSC many years ago, this year's PSC Annual Conference in Sacramento, was a "first" for me. The impetus for my attendance was primarily because Philip was a presenter at one of the breakout sessions, and the event provided an opportunity for a "mother/son" vacation.

Planning for every detail of the conference was evident:

- 1) Attendees were made to feel part of a supportive community, from the moment of arrival and throughout the conference
- 2) Sessions provided general information for the entire group, as well as break-out sessions for special interest groups
- 3) Topics were diverse enough to meet a variety of educational and experiential backgrounds
- 4) Small group sessions focused on building relationships as well as providing helpful information
- 5) Resources were made available for further study and networking

I was particularly impressed with PSC Partners' growth, both financially and in reaching out to those needing support. I am looking forward to continued participation in PSC Partners Seeking a Cure and am hoping to be able to attend next year's annual conference.

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## All in the Same Boat...

*By Emily Spannagel (a 20-30's PSCer)*

When I registered for the Sacramento PSC conference, I honestly expected to be overwhelmed with medical jargon and questions that didn't have the answers I wanted. I was certain that I would learn new information and meet some new people who shared my rare disease. But, I was not prepared for all of the smiles, hugs, and laughter that were a constant each and every day of the conference. I looked around at our small group and was amazed at the positive glow that seemed to be present despite the seriousness of our condition. The weekend quickly turned from a medical conference to a family reunion.

It was amazing to see past attendees catch up with each other and share what the past year had brought to their lives. As a first time attendee, I was welcomed into this group with open arms. Not once did I feel out of place. How reassuring to be in a group of people who would know exactly what I was talking about! Yes, they had ulcerative colitis, too. Yes, they were fatigued and itchy as well. No judgments. No excuses. We were all in the same boat.

Sharing stories, wacky remedies, and advice was incredibly helpful. We laughed and cried together those three days, and in the end, I left with a group of friends that I felt I had known for years. I love it that I can connect with my 20s/30s gals anytime I need to, and I am honored that I can be there for them. Even now, weeks after the conference, I know that I have a support system that spans the country and even the world. I am so incredibly grateful for all that I have gained from going to the conference, and I am ecstatic to see everyone next year in Minnesota!

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## There's a Song in my Heart \*

*Sandi Pearlman*

Okay. I admit it. I'm a musical junkie. *Singing in the Rain*, *Thoroughly Modern Millie*, *Mama Mia*, *Rent*, I could go on and on and...well, okay, on. But before you write me off as some musical loving goonie, bear with me a bit because here's the thing: in a musical, there's escape from the world in a way that just isn't generally offered in real life. In musicals, when something terrible happens, as it often does, there are still songs to sing, hugs to share or catchy anthems to occupy us as the tears roll down our collective cheeks. Musicals aren't just movie experiences, they're visceral ones. Even decades after watching a film, hearing the first few bars of the score transports us back. It's kind of like our very own time machines traveling *across the universe* granting instant access to memories, emotions and experiences. Honestly, even you nonmusical enthusiasts must admit to occasionally finding yourself humming along to a theme song or two--right? I mean, who among us hasn't been whisked away to *Bali Hai* or wondered exactly how to spell *Supercalifragilisticexpialidocious* at least once or twice a year? And although the conferences you see in movies (Rosie's sexy dance in *Bye, Bye, Birdie* anyone?) vary quite a bit from anything you'll find at a PSC Partners' conference--although this year we did include bull riding, so never say never--still, they put a song in my heart like I'm *singing in the rain*. So, going with that, ahem, theme, here are a few of *my favorite things* about PSC Partners conferences and the memories and realities they make:

*You'll Never Walk Alone.*

Diagnosis of a rare disease which most people--heck, most doctors--have never heard of is downright terrifying. Like *Thoroughly Modern Millie's* Mrs. Meers is forever saying, "Sad to be all alone in the world." And it is...only, we're not. Let me emphasize that. We are NOT all alone in the world and there's no place in the universe that is more proof positive of that than a PSC Partners conference. This past conference was our largest yet and a whopping 62 percent were first-time attendees. That's 62 percent of attendees who took a leap of faith that a conference covering a rare, currently incurable disease was going to be worth their time, effort and energy.

So many of us walking through the doors of our first conference feel as kicked about and alone as Little Orphan Annie. We've discovered we are PSCers in a non-PSC world and feel it's practically a given that it's going to be *a hard-knock life*. For many, information on their disease has been scarce or nonexistent and, for an alarming number, just downright wrong. So, if you entered your first conference feeling much more *I shall scream* than *I think I'm gonna like it here* or those feelings have kept you from attending, rest assured, you're not alone. But like any good musical, there's a silver lining to be found and attending the conference guarantees...

*The sun 'll come up tomorrow.*

You can bet your bottom dollar on that, no wishing on a star needed. No matter what life throws at us, and we all know far too well that life can be a big, old bully at times, shining a light on each other and on ourselves really does make all the difference. We don't have to live in the shadows, vampire-like victims of a dreaded disease. PSC is hard. Conferences can be hard. This one was hard at times and the next one will likely be hard at times, too. We don't have a disease with simple answers or very many solutions. The temptation to drown in ourselves and our situations can be downright enticing and to say a conference saved me is at once true and far too pat. But I can tell you that the decision to attend, for me and for so many other PSCers I speak with, helps us *[get] out of bed on the right side*. And because of that, even when we're feeling lousy, it's still a pretty wonderful day.

I heard from several PSCers that initially they were none-too-happy about attending their first conference and terrified to say no to whomever told them about it all at the same time. In my case, like in most of yours, before my first conference I'd never met another PSCer. Honestly, I didn't even particularly want to meet another PSCer. I was symptomatic, a bit angry and unwilling to take a leap into *a whole new world*. I was pretty darn certain that if the sun was going to come up tomorrow, I'd probably be burned to a crisp instead of basking in its warm vitamin D bestowing glow. I'd never been a pessimist before, but I was well on my way. So, when I say that the conference is life-changing and the sun is shining, I know from where I speak. I'm not some talking head where the words have no meaning and I know that when others tell me how the conferences changed them, they're not just whistling Dixie either.

Before each conference, I end up with an inbox full of e-mails from worried PSCers. People are depressed and they're scared and they're not sure they're ready to face a room full of the very sickness that plagues them like monsters in a child's closet. I offer reassurance, but it takes attending a conference to get it. I can tell you until I'm blue in the face about the benefits, but until you experience them, it's just words. As many of you know, I wasn't feeling well at this year's conference and aside from the yellow that I couldn't hide, I tried to play it off as best I could. But I was busted. A new attendee called me out. She's the wife of a PSCer, a symptomatic PSCer at that, and they were scared. They are scared. And she took time out of her day to sit with me, bring me a cup of tea and ordered me to sit still. We didn't talk about the events of the day. We didn't even utter the words "biopsy" or "liver." We sat and sipped tea, content in each other's company, an understanding between us that even when things are rough, as it was for me, as it was for her and her spouse, that there's nothing so tough that we can't attack it together...even if our attack plan of the day is no more than *tea for two*.

The conference has been over for weeks now and yet, the connections are not. Some of our college-aged PSCers have already hooked up for movies or pizza, skyping late into the night bemoaning how much they need sleep and miss each other. Others have made meet-up plans or written to me to see how they can be more actively involved in the next conference or with PSC Partners. Despite the official conference weekend's end, we're still *together wherever we go...*

### *Somewhere Out There.*

Because that's the thing, we are together no matter the distance. We don't need Doris Day singing *Que Sera Sera* or the confines of a certain city to maintain our newly formed and newly reinvigorated bonds because they're more than a fleeting fancy. They're not just yearbook promises to be BFFs 4-ever. In big things and small, fun and not so fun, we've got each other's backs, fronts, livers and lives and we're well aware of *what a beautiful morning* it is. A first-time attendee mentioned he was having his first procedure a week after the conference. You know what happened? He got cards and flowers and visits from PSCers who just two weeks beforehand he hadn't even met. There were names on some of his cards that he didn't even recognize without turning to the conference photos for some help. We're not just *people who need people*, we're PSCers who need each other and when we say *I'd do anything*, we mean it.

When I was stuck in the hospital over Thanksgiving, my favorite holiday, hundreds of my conference pals spent their time making apple turkeys and sending me pics by Facebook just to give me a smile. There are scores of stories, volumes and tomes that even *Marian the librarian* would have trouble keeping up with and cataloging. And here's one more: There's a little girl in Australia, a true *super trouper*. Every year she holds fundraisers and every day she uses her tiny little body and all of her far-too-precious energy to send words of love out over the internet and out into the universe. She's waiting for a transplant now and do you know what's waiting with her? Aside from all of our love and thoughts and prayers, she's got some flowers...and not just any flowers, but plush flowers that have seen quite a few of us through transplants and long hospital stays, flowers that have travelled the globe spanning *seasons of love* and proving, once again, that even geography can't keep us from being *one of us*. See, our Australian friend has never been to a conference, but her flowers have and we know someday soon she will have, too. Until then, we'll just hum the *Sisters* song and remind ourselves that sister, brother, mother, father, husband, wife or any other delineation, we're no longer searching for answers in the dark, hearing our own worst fears echoing back at us. There are answers. There are voices and experts and people who have been where you are and are going where you're headed. There's no *I can do anything you can do better*. There's no egos. Not in that room and not in our group. We have medical gods (for lack of a better term) and they cry with us and hold our hands and rededicate themselves to the fight, knowing *we need a hero* and they are, we all are, the proverbial girls who just *can't say no*. We're a team and there's strength in numbers. When we're together in that room, in each other's lives, we're not the other. We're not even the sick. We're the kids at the popular table who have the whole world shining upon us so bright that we practically need to wear shades. We can do anything. We're *free to be you and me* (see, the musical references just keep coming!). Normal rules don't apply. When we're

together, we *come together* instead of sleeping the day away or even wanting to. We're announcing we get by...

*With a Little Help From [our] Friends.*

We stay up until 3 and 4 in the morning, so hungry for each other's company, warmth and presence that sleep, which is at once our best friend and worst enemy, ceases to matter. Are we itching? Sure. Are we scratching? Sure. Taking throw-up breaks in the bathroom and occasionally forgetting what we're saying as the words come out of our mouth? You got it. Do we care? Not even a bit. We've got gum, empathy and a quick capacity for laughter that drowns out even the toughest of nausea or deeply entrenched RUQ pain. High on pop (you girls know who you are!) or high on each other, we've formed bonds that outlast a few simple days. Whether connecting on Facebook or phone, in person or at a hospital, we refuse to simply say *Goodnight, Sweetheart* when a conference must finally come to an all-too-soon end. It doesn't matter if we're discussing *So You Think You Can Dance* or CCA odds, transplant or our love for tiramisu, I don't have a day go by that I don't hear from a member of my PSC family. And that makes me an incredibly blessed girl. And it's not just me that's staying connected. So, I'm asking...

*Take a Chance on Me.*

I beg you. Not just me, but on you as well. *We are family.* So, *let's get together*, yeah? I know I'm asking a lot of some of you to put your faith in me and in PSC Partners. I'm asking you to *follow the yellow brick road*--and no, that's not a jaundice joke--because at the end is something even better than Oz; it's a place where you not only find that you possess a heart, a brain and more courage than a den full of lions. It's a place where in one weekend, a few short hours, you can change your life. It's so much more than a *spoonful of sugar* designed to mask the bitter and the tough. It's a place where you know you *must've done something good*. So whether you can get behind pulling down curtains to somehow garner enough fabric for 7 children's playclothes or simply can't fathom the thought that anybody would name their child *Truly Scrumptious*, just remember that for so many, for me, when our annual PSC Partners conference ends each year, it says so much more than *so long, farewell*. We might have tears in our eyes. We might have lumps in our throats. We might be more than a bit weepy about the thought of waking up tomorrow and not seeing the faces of those who love us, understand us and have become as much a part of our systems as our own breath over the course of the weekend. But we also are saying *Good Morning* to a whole new life, one in which there's definitely something to sing about.

So from me to you, thank you for being *the wind beneath my wings* and for teaching me and helping me to teach others that *[we] can fly*. Until next conference, there's a song in my heart and a bluebird on my shoulder...and if you don't know what that means, well, you've clearly got some musicals to watch....

\* Wondering what musicals and movies were quoted in this article? Sounds like a perfect reason to Goodsearch ([goodsearch.com](http://goodsearch.com)) for PSC Partners! Oh, and if you're feeling a bit sing-songy yourself at the moment, give the song below a try...after all, we say, "*if you want to sing out, sing out.*" And

if you're thinking singing out is where it's at and want to send us a videotape of you singing the blues away, you'd make our day! Send videos (for PSC Partners use only) to [Sandi@pscpartners.org](mailto:Sandi@pscpartners.org) :

*A Few of My PSC Things* (to the tune of *A Few of My Favorite Things* - rewritten by Sandi Pearlman):

Itching and scratching in inconvenient places. Being too tired to tie our shoelaces. Being driven mad by what a stray hair can bring. These are a few of my PSC things.

Yellowing skin, ERCPS and constant dehydration. Mercedes signs without the car payments. A medical vocabulary without the degree. This is some of what PSC brings.

Swollen legs and middles and daily medications. Problems with both diarrhea and constipation. Forgetting what words we're saying even as we sing. Always wondering what tomorrow will bring.

When the liver strikes.  
When the bills sting.  
When I'm feeling sad.  
I simply remember my PSC Pals  
and then I don't feel so bad.

PScers of all shapes and all sizes. Having people here from so many different places. Knowing that together a cure we will bring. These are a few of my favorite things.

Laughter and sharing and tons of random hugging.  
Nobody caring what body part you're scratching. Falling asleep anytime is a okay with me. Loving what PSC Partners can bring.

Research and proposals and always fundraising. Together awareness of PSC we are raising. Power, Strength, Courage is what we will sing. Today's efforts we hope a cure will soon bring.

When the liver strikes.  
When the bills sting.  
When I'm feeling sad.  
I simply remember my PSC Pals and then I don't feel so bad.

# Fact-O-Rama 2011 PSC Partners Conference Statistics

Number of conference attendees in 2011:	223	(In 2010: 208; in 2009: 185)
Number from outside the United States:	33	(In 2010: 29; in 2009: 18)
First time attendees:	122	(In 2010: 110; in 2009: 116)
Attended pre-conference activities:	140	(In 2010: 98; in 2009: 90)
PSCers:	99	(In 2010: 79)
Transplanted PSCers:	10	(In 2010: 10; in 2009: 10)
Number of teens:	5	(First-time group in 2011)
Spouses:	41	(In 2010: 31; in 2009: 37)
Other caregivers:	21	(In 2010: 17, and 7 were siblings; in 2009: 12)
Parents total:	60	(In 2010: 69; in 2009: 53)
Parents of pediatric PSC patients:	20	(In 2010: 9; in 2009: 13)
Parents of adult PSC children:	40	(In 2010: 60; in 2009: 40)
20's/30's PSCer group:	35	(In 2010: 37; in 2009: 30)

**Would you like to propose your home town as the site for the 2013 PSC Partners  
Conference?**

**Deadline is January 15th, 2012, and here's the site to get you started:**

**<http://pscpartners.org/conferencelocations>**



### **Note to Readers:**

Articles in this newsletter have been written by persons without formal medical training. Therefore, the information in this newsletter is not intended nor implied to be a substitute for professional medical advice.

Please consult with your doctor before using any information presented here for treatment. Nothing contained in this newsletter is intended to be for medical diagnosis or treatment. The views and opinions expressed in the newsletter are not intended to endorse any product or procedure.

## Conference Session Summaries

Reported by Arne Myrabo

### Introduction to PSC

Dr. Christopher Bowlus, Associate Professor,  
Division of Gastroenterology and Hepatology, University of California Davis

*Dr. Bowlus' PowerPoint presentation is available at*  
<http://pscpartners.org/PSCConf11/PDFs/Bowlus-Intro%20to%20PSC.pdf>

Dr. Bowlus covered the major features of PSC and addressed typical questions patients ask about the disease. He started his presentation by defining the term primary sclerosing cholangitis. He explained that the word *primary* means that PSC is not secondary to another cause, although some think that it is secondary to inflammatory bowel disease (IBD). He noted that other conditions that can cause similar symptoms do exist and that the cause for PSC remains unknown. The term *sclerosing* refers to the hardening/thickening/narrowing of the bile ducts, and *cholangitis* means inflammation of the bile ducts.

A classic PSC-like lesion exhibits radial scarring around the bile duct. Dr. Bowlus presented numerous examples of normal versus abnormal cholangiograms of PSC patients.

Men are affected more than women are, and most patients have IBD. It was thought that 90 percent of PSCers had IBD, but currently this figure has been revised to 60-70 percent. The IBD of people with PSC is often mild and asymptomatic. Most have ulcerative colitis (UC), and a small number have Crohn's (CD). Some have small duct PSC which presents with a normal cholangiogram and with a biopsy that looks like PSC. Small duct PSC displays a different disease progression.

The course of PSC is unpredictable and extremely variable and is therefore very difficult to predict. The average time from diagnosis to liver transplant is 12-15 years (though some recent studies have raised the average to 18 years).

Typical symptoms of PSC are ascending cholangitis, right upper quadrant (RUQ) pain, fever, jaundice (yellowing of skin and eyes), fatigue, itching, and stress of the unknown. Cirrhosis is the end stage of chronic liver disease.

Most PSCers do NOT get cancer. Whenever there is chronic biliary inflammation, there is a risk of cancer (*cholangiocarcinoma*). For PSCers there is also the additional risk of colon cancer. An annual colonoscopy is therefore recommended and necessary.

What can the PSC patient do about PSC? Unfortunately, there is currently no proven therapy. Urso (bile acid supplementation) may improve symptoms, as may ERCPs. Liver transplantation is used when needed.

Why does one get PSC? There aren't any great answers. As is the case with many diseases, the combination of genes & environment brings about disease. We are starting to understand the genetic aspects of PSC. We see that the main risk is having an IBD. (Please see the summary on the presentation of Dr. Melum from the PSC Center in Oslo.) Males are at greater risk, as are African-Americans. Smoking is protective for UC. It is strongly recommended that you NOT start smoking.

Will there ever be a cure for PSC? We are more interested in a treatment at this time, much like treatments for cardiovascular disease, diabetes, etc. There will be another panel discussion on future treatments.

Dr. Bowlus ended his presentation with a positive statement: "The skies are clearer now (with some clouds); more research is being implemented; there **is** hope for new, effective therapies and treatments.

*Arne Myrabo*



## *Join Team PSC!*

*We PSCers have many talents and skills--but mostly we have big hearts. If you have a desire to contribute to the organization as a volunteer, let us know.*

*Check our website at: <http://www.pscpartners.org/opportunities> for ideas on where your talents and skills might fit in. Contact Dike Ajiri, our volunteer coordinator at: [volunteers@pscpartners.org](mailto:volunteers@pscpartners.org).*

## URSO – PRO vs CON

Dr. Gideon Hirschfield, Assistant Professor of Medicine, University of Toronto, Hepatologist, Toronto Western Hospital

Dr. Marion Peters, Professor of Medicine, Chief of Hepatology Research, University of California San Francisco

### The argument pro Urso

Dr. Hirschfield's presentation is available at <http://pscpartners.org/PSCConf11/PDFs/UDCA-Pro-Hirschfield.pdf>.

Dr. Hirschfield started his argument by suggesting that we think of PSC as a family of diseases. First, he drew a parallel between PSC and primary biliary cirrhosis (PBC); the latter is a disease of the small bile ducts, where the immune system attacks the small bile ducts within the liver. In PBC, bile duct scarring eventually leads to cirrhosis. Urso (a soothing bile acid) is clearly shown to be beneficial for PBC.

Secondly, autoimmune hepatitis (AIH) sits in the middle of everything, and can occur both with PBC and PSC. With the exception of Japan, no researchers think Urso has a role as a treatment option for this condition. Thirdly, we have PSC, and this brings up the real question. Can the soothing effects of Urso slow down the disease? Keep in mind that PSC is a very slow progressing disease – 20+ years sometimes. Clinical trials that last a year or so cannot be useful, and longer studies are just too expensive to sustain.

Also, we are much better at diagnosing PSC prior to the occurrence of symptoms, predominantly because we are better informed, but mostly because of the improvements in magnetic resonance cholangiopancreatography (MRCP). More than half the time, it is picked up through liver function tests (LFT) when the patient has no symptoms. This early detection means that there is more time to intervene.

There may be many diseases hiding under the term *primary*. We just don't know, but based on the extreme variability of PSC, the disease probably comprises many different versions. Some day, we may have PSC-1 through PSC-10, for example. Therefore, different patients may need different treatments.

The only animal model for PSC is not a very good one. But from this model, we do know that bile toxicity can contribute to bile duct scarring.



We thank **Arne Myrabo** for setting up our 2011 conference website where everyone can access information from our 2011 conference presentations. <http://pscpartners.org/PSCConf11/index.htm>

Approximately 4 percent of bile is made up of naturally occurring ursodeoxycholic acid (Urso). By adding Urso, the *soothing* bile acid, our aim is to enrich the bile. Normally produced in the bowel, Urso is a byproduct of bile acids. Urso stimulates secretion, makes bile flow, stops liver cells from dying, and has a soothing effect on bile ducts.

Ever since the early 90's, LFT improvement was noted when PSCers took Urso. The first high dose study took place in Europe. Dr. Hirschfield noted that there were more PSCers in the audience at this conference than there were in that first study! That early study showed that for the majority, biochemistry improved while no changes in the disease symptoms were noted. These results have been consistent with all subsequent studies up until Dr. Keith Lindor's recent high-dose study.

There are some questions about Dr. Lindor's study-design. Moreover, the selection of the 150 study patients may have pre-selected the outcome. High dose may not be beneficial, but that does not mean that low dose is not.

Due to the variability of PSC, one size does not fit all. "That is the *art* of medicine, not the *science* of medicine," he said. Some other studies have shown improvement in biopsies. Also, there is some data to suggest that Urso *may* have a preventive effect against colon cancer.

In summary, Urso in normal doses has been shown to be safe and to reduce liver biochemical inflammation. We can't expect cures, but in a PSC subgroup, Urso may slow down the disease.

## The argument against Urso

Following Dr. Hirschfield's presentation, Dr. Marion Peters presented the argument against Urso in PSC. Her presentation is available at <http://pscpartners.org/PSCConf11/PDFs/UDCA-Con-Peters.pdf>.

Dr. Peters started her argument by stating that survival studies are truly the ultimate studies in testing any medication. What we most care about is whether a treatment will prolong a patient's life or not.

It is important to remember the reason for this debate, she said. Had another therapy existed for PSC, we would not be using Urso. Forty years ago, we saw that Urso was marginally useful in dissolving gallstones (too slow, though). Urso does change bile from an abnormally toxic bile to a more soothing, water-loving bile. However, liver damage occurs when bile remains stagnant in the liver.

We know that Urso can be protective (possibly, also against colon cancer) under these conditions, but the data shows that Urso "is not that good. Nor is it bad." If we consider the nine largest PSC studies, two showed improved symptoms while seven showed no improvement in the symptoms.

Dr. Lindor's high-dose Urso study went on for five years, with a selection process similar to that of the rest of the studies. The data safety monitoring board terminated the study because the outcome was worse for those on high-dose Urso (note that only 25 percent actually reached the 5-year mark). Although LFTs improved, the risk of achieving the *hard* endpoints (transplantation, varices, death) was higher for those on Urso. It is very clear that long-term high-dose Urso is associated with LFT improvement, but it is also clear that symptoms and survival do not improve.

In conclusion, it is agreed that a one-size fits all approach does not work. High-dose Urso does not improve endpoints, and we need to think about whether low-dose Urso is an appropriate treatment. We need to find the correct dose for all PSC types and stages – early stage, late stage, small duct, large duct, AIH overlap, IgG4, etc.

*Reported by Arne Myrabo*

## Managing PSC Symptoms

Dr. Natalie Török, Associate Clinical Professor, Gastroenterology and Hepatology  
University of California Davis

*Dr. Török's PowerPoint presentation is available at <http://pscpartners.org/PSCConf11/PDFs/Torok-PSC.pdf>.*

Dr. Török began by noting that 15-40 percent of newly diagnosed PSC patients are asymptomatic, their diagnosis made following discovery of elevated liver enzymes.

Fatigue is one of the major initial complaints PSC patients report. She noted the importance of ruling out other causes and said that there were no reliable medications to relieve fatigue in PSC.

The second major complaint of PSC patients is pruritus which is typical with any form of cholestasis. The causes are thought to be bile acids, endogenous opioids, perhaps histamine, and autotaxin-LPA.

The severity of pruritus does not correlate with any of the first above-mentioned three suspected causes. Autotaxin-LPA is the only one that correlates well with pruritus in cholestatic patients and merits to be researched. The techniques followed to manage mild to moderate pruritus are the following:

- Ruling out biliary obstruction (progressive bile duct dilatation may relieve pruritus)
- Using cooler water with shower or bath, using moisturizing soap, avoiding deodorant soaps
- Applying Eucerin cream to all itchy areas after taking a shower
- Wearing fewer and lighter clothes and using light blankets (stay cooler)
- Using Cholestyramine powder which binds with bile salts in the intestine to decrease the absorption of bile salts (*Note: All other medications should be taken one hour before and four hours after taking Questran*)
- Taking Doxepin (a prescription medication 10-20 mg) at bedtime due to drowsiness this medication may cause
- Urso (20 mg/kg/d)
- Rifampin (10 percent liver toxicity)

For difficult pruritus cases,

- Naloxone (intravenously given in hospital)
- Dranabinol
- Gabapentin
- Molecular Adsorbent Recycling System dialysis (MARS). This treatment is used for encephalopathy and intractable pruritus
- Zoloft was noted to improve pruritus in patients taking Zoloft for depression
- Liver transplant

Other Symptoms include:

- Upper right quadrant (URQ) pain which may occur in the chest, upper back or right shoulder, nausea and vomiting, belching, fever, chills, jaundice, low blood pressure, lethargy, decreased level of alertness, itching, pale stools, dark urine. Most patients respond to therapeutic drainage of the obstruction plus antibiotics. Some patients with recurrent bacterial cholangitis may benefit from long-term antibiotics. Recurrent, severe cholangitis may become the primary indication for liver transplant.
- Portal hypertension could result in esophageal varices, a type of varicose vein in the esophagus that may result in bleeding. They are evaluated and treated by endoscopy.
- Osteopenia and osteoporosis - Screening for metabolic bone disease is recommended at diagnosis, then every two to three years. Treatment involves vitamin supplementation (calcium, vitamin D) with bisphosphonates if needed.
- Fat soluble vitamin deficiency (Vitamins A,D,E,K) is treated with vitamin supplementation.
- Although pruritus and abdominal pain may occur with PSC during pregnancy, the outcome has been satisfactory for both patients and children. In some cases, pruritus may be intense enough to warrant early delivery. No serious deterioration of liver function before, during, or after pregnancy has been reported.

*Reported by Arne Myrabo*

Before attending a conference, I only had one reason for wanting to find a cure for PSC and now I have over 200!

The connection I made with other attendees is more of a treatment than cholestyramine is for itching!

I think I was in denial before I got here. Now my eyes are wide open, tearing, but wide open. Thank you.

What YOU Said About the Conference . . .

The above sessions were followed by a Q&A session which is available on audio.

## Questions and Answers Panel

(Please click for audio)

Panel: Drs. Selmi, Bowlus, Hirschfield, Peters, and Török

## PSC Partners Research Grants Overview

David Rhodes, PhD, Purdue University, Chair, PSC Partners Scientific/Medical Advisory Committee

Stephen Miller, MD, PSC Partners Scientific/Medical Advisory Committee

*Dr. Rhodes and Dr. Miller's PowerPoint presentation is available at [RhodesandMiller-2011](#).*



The Scientific/Medical Advisory Committee of PSC Partners plays a key role in research, education, awareness and advocacy. Above all, it implements and facilitates practical research and development towards finding an effective treatment for PSC.

The committee is responsible for the PSC Partners Seeking a Cure research grant program. In this program, researchers submit grant applications in promising and significant PSC research areas. Seed grants are currently limited to \$40,000 over two years. Proposals are reviewed and ranked by

the committee, which then makes recommendations to the board. To date, the committee has helped guide PSC Partners Seeking a Cure towards investing over \$500,000 in PSC research.

Seed funds were provided to STOPSC in 2007 and 2008. Since 2009, 13 research proposals have been funded. We are just starting to see results in terms of publications, acknowledgments to PSC Partners, useful research data, and leverage of federal funds. The seed funds to STOPSC resulted in a \$600,000 grant from the FDA to research the use of urso in pediatric patients with PSC. The seed funds that enabled Dr. Lazaridis to study the environmental and genetic risk factors associated with PSC led to a \$3.5 million grant from NIH.

The tasks for the committee over the next two years are to review the successes and limitations of the grant program, identify new areas for urgent study, and consider giving larger grants (up to \$100,000) in the high priority areas.



Immediate and near-term goals (1-3 years, assuming adequate resources) are to reduce the damaging impact of cholestasis both on symptoms and consequences. This will be accomplished by optimizing the utility (singly and in combination) of currently available drugs and supplements.

Intermediate term goals (3-6 years, assuming adequate resources) are to reduce inflammatory damage by identifying and disrupting key disease-related pathways, many of which are increasingly being more accurately identified. The main goals are to reduce the incidence of liver failure and to convert PSC to a more benign, chronic illness.

Long-term goals (6-10 years, assuming adequate resources) are to silence the disease mechanism via identification and definition of the structure and function of the source of PSC (likely a genetic complex that disturbs normal immunity). The key goals are to arrest disease progression and enable a normal lifespan with a good associated quality of life.

The research funded to date has been distributed approximately equally among these three goals. This distribution will be increasingly dynamic, according to new data that is growing in quantity and quality.

Another objective is to address inconsistencies in clinical practice by: fostering better understanding and heeding of published guidelines for PSC; achieving better symptom evaluation and control; clarifying the role of medications such as Urso; better navigation of transplantation issues; encouraging increased interdisciplinary and integrative care.

Finally, it is essential to better empower patients and caregivers. Increased knowledge = increased control and better care. Be your own best advocate!

*Reported by Arne Myrabo*

This amazing organization has the ability to raise research funds, collaborate with the best PSC doctors and provide us the most current information AND does this so humbly and in such a friendly way! I will never miss a conference unless I physically can't get to it!

*Better  
and  
better  
every year!*

*I'm willing  
to go to any length,  
including going into  
debt to go to the next  
conference. It was well  
worth it!*

**What YOU Said About the  
Conference . . .**

*From a teenager:  
PSC isn't who I am.  
No, it's just a little  
part of me. And I  
know it may sound  
weird, but I don't  
see it as a disability  
anymore. I see it as  
a capability.*

*This  
organization has  
saved my sanity!*



# PSC Immunology: Lessons to be Learned from PBC

Dr. Eric Gershwin, Distinguished Professor of Medicine, The Jack Donald Chia Professor of Medicine, Division of Rheumatology, Allergy and Clinical Immunology, University of California Davis

*Dr. Gershwin's PowerPoint presentation is available at [Gershwin-PBC](#).*

Though primary biliary cirrhosis (PBC) may seem to be similar to PSC, it is in reality very different, about as different as a respiratory disease would be from PSC. Despite the differences, there are some aspects of PBC research that are applicable to PSC.

PBC is caused by bad genes and bad luck, as well as environmental factors such as chemicals, bacteria, and such. This also probably pertains to PSC. Through a combination of fortuitous circumstances and brilliant insight, a good predictor for developing PBC was found (anti-mitochondrial antibodies). This was the big breakthrough for PBC. If a person has this marker, in ten to fifteen years, s/he *will* get PBC.

Some diseases take a long time between onset and clinical presentation, which makes identification and etiology very difficult. Suppose PSC is a disease that is similarly triggered by an event occurring ten years before the development of any symptoms. How would one ever know if disease is present prior to symptoms?

We still don't know why PBC has a high female to male ratio. Getting pharmaceutical companies to fund research in PBC had been very difficult. It will be even worse for PSC due to the relatively smaller numbers of patients.

PBC is a heritable disease and tends to cluster with other diseases including Sjogren's, rheumatoid arthritis, celiac sprue, and others. PBC differs from PSC in that PSC clusters with IBD. PBC is an immune disease, but it does not respond to immunosuppressants.

PBC *does* have a genetic influence. Studies of twins show that, in the case of identical twins, it is very likely that if one twin has PBC, the other does, too. The same is not true for fraternal twins. Epidemiology studies show "clustering" in some locations and a correlation with chemical exposure to products such as nail polish and cigarette smoke, and also with urinary tract infections.

A chemical that was found to mimic PBC in a mouse provided another leap in the ability to study PBC. Dr. Gershwin said that the development of an animal model for PSC must be one of the highest research priorities. Virtually every advance made in the treatment of lupus, rheumatoid arthritis and other diseases has resulted from animal models.

Currently, more than 20,000 people in the US are waiting to have a liver transplant. Only 6,000 liver transplants were performed last year, and 2,000 will die waiting for a transplant. We really cannot rely on liver transplants to solve PSC.

Dr. Gershwin related an exciting experiment that was implemented to push PBC research forward. A group of people who previously had never heard of PBC and who were experts in various unrelated fields were invited. A PBC specialist gave a presentation on PBC and left this think tank to ruminate on PBC. Their outside-the-box recommendations resulted in important PBC studies.

Dr. Gershwin concluded his talk by saying that "... the lynchpin, the critical discovery in PSC will not be made by anybody who studies PSC. It will be made by somebody who happens to want to apply a new technology to a disease, and somehow they will stumble upon PSC as a model..."

*Reported by Arne Myrabo*

## New PSC Genes – What Should We Do With Them

Dr. Espen Melum, Postdoctoral Researcher, Norwegian PSC Research Center  
Clinic for Specialized Medicine and Surgery  
Oslo University Hospital Rikshospitalet

*Dr. Melum's PowerPoint presentation is available at [Melum-PSC\\_partners\\_PSC\\_genes](#)*

Humans are 99.5 percent genetically identical. It is the remaining variation that defines human traits, including disease risk. The tool to determine disease risk is simply to look for genetic variation. We compare the genetic code in PSC patients with "normal" controls and see what is different. We need to identify pathways that affect disease risk, and then find a drug that disrupts that path.

The HLA human leukocyte antigen (HLA) association is the most important genetic risk factor for PSC, and has been known since 1982. However, from a genetics standpoint there is no large overlap with ulcerative colitis.

The first gene found in the overlap with other diseases is MST1, a gene involved in the regulation of macrophages (a type of immune cell). It is associated with inflammatory bowel disease and represents an overlapping genetic risk factor.

The second gene found to overlap with other diseases is involved in the regulation of the immune response (IL2RA). There are some differences, however, which show that there is not a genetic homogeneity in the patients – not every PSC patient has the same genetic variants.

Another gene found to overlap is not reported in any autoimmune disease (BCL2L11), but it plays a role in eliminating immune cells that are over-reactive. Testing in a mouse model missing this gene showed increased infiltration of immune cells in the liver, thus validating this genetic finding.

So far, over 70 genes have been found to be associated with IBD. It is likely that PSC will prove to have a similar number of variant genes. By hitchhiking on other disease research, a matrix of disease overlap was developed. With the exception of BCL2L11, there is a great deal of overlap among diseases such as PBC, UC, Crohn's, celiac disease, diabetes, rheumatoid arthritis, and multiple sclerosis.

So how do you apply this information? Although the gene function can be predicted, we are missing the knowledge of the mechanism by which a gene affects the organ. With the current data, the vast majority of the people carrying the variant do not have the disease. Testing at this point would not be useful.

Bile duct diseases should be defined on the basis of their genetic variants (molecular phenotypes) such as PSC, small-duct PSC, PBC, and other bile duct disorders. This may allow tailored treatment based on the molecular phenotype.

In summary:

- Genome-wide associations studies are effective for uncovering genetic variation.
- Several new PSC genes have been discovered, but a vast number remains to be found.
- Findings will help us understand the biology and pathogenesis of PSC.
- The small impact of each of the associated variants means that the genetic variants do not have any current use in clinical medicine.

*Reported by Arne Myrabo*

## Questions and Answers Panel

(Please click for audio)

Panel: Drs. Rhodes, Miller, and Melum

# Saturday Night in Sacramento



And Saturday night arrived... Suddenly there was a buzz in the hotel lobby... Some of us in our group were to ride a mechanical bull to fundraise for PSC Partners! We all went to watch our brave PSCers holding on to dear life as they rode the bull in a Sacramento bar among laughter and fun. The merriment peaked when it was unanimously decided that Ricky Safer should sit on the bull. The bar lost money that night in the absence of the usual consumption, but no one was left out of the fun . . .



## Note to Readers:

Articles in this newsletter have been written by persons without formal medical training. Therefore, the information in this newsletter is not intended nor implied to be a substitute for professional medical advice.

Please consult with your doctor before using any information presented here for treatment. Nothing contained in this newsletter is intended to be for medical diagnosis or treatment. The views and opinions expressed in the newsletter are not intended to endorse any product or procedure.

For summaries of  
the Breakout  
Sessions and of  
Healthy Living  
Perspectives,  
please continue  
with Part II of the  
2011 Summer issue  
of  
The Duct



**PSC Partners Seeking a Cure** is a 501(c)3 nonprofit foundation that endeavors to find a cure for Primary Sclerosing Cholangitis.

The three-fold purpose of the **PSC Partners Seeking a Cure** foundation is to: raise funds for research on the causes and cures of PSC, provide education and support to PSC patients and their families, and promote PSC and organ donation awareness.

Ricky Safer is the principal contact person for the PSC Partners Seeking a Cure Foundation. Reach her at: [contactus@pscpartners.org](mailto:contactus@pscpartners.org)

To make a tax-deductible donation, please click on [www.pscpartners.org/waystodonate](http://www.pscpartners.org/waystodonate).

### Website

[www.pscpartners.org](http://www.pscpartners.org)

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### The Duct Newsletter

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