The Duct

Bringing you news on PSC

research, education, and Support

PSC Partners Seeking a Cure Foundation Summer 2013

Our PSC Community Spirit Rocks!

by Ricky Safer

Now that it's summertime and our post-conference work is nearing completion, I've been sitting down and trying to pinpoint what it is that creates the magical energy that appears every year when our group gets together for a weekend of learning and sharing. Here, at PSC Partners headquarters, we spend a year ironing out logistic details, topics and speakers for the Saturday presentations, peer group sessions, healthy meals, and fun activities to break up the seriousness of the academic sessions. We create the framework, but it's truly all of you, our unique community of PSCers and caregivers, who create the special ambiance of the weekend.

Our attendees come from different regions and countries and represent different backgrounds, experiences and age groups, but the unifying factor PSC, a disease that none of us would choose, creates an instant bond. This year, we were thrilled to have 232 attendees join us in Pittsburgh. They came from throughout the United States and Canada, and also from the UK, Sweden, The Netherlands, Australia and South Africa. Of these attendees, 98 were first-timers who were quickly absorbed into our PSC family.

I had never thought about this before, but one newcomer mentioned to me, "It was refreshing that I met so many new people this weekend, yet no one ever asked me what I do for a living." It's true that there seems to be none of the normal small talk all weekend. We come to the conference to learn as much as we can about the medical aspects of PSC from the medical professionals and about living positively with PSC from our fellow attendees. Our group is so caring and compassionate about helping others that enduring friendships form almost instantly. It's comforting to know that everyone in the group understands us: our symptoms, worries, fears, and hopes. If we fall asleep during presentations, scratch ourselves in public, or suddenly break out into tears, rather than being stared at, our behavior is understood and accepted. As opposed to the rest of our lives, here we have no explaining to do. There is a feeling of warmth, encouragement and positivity when we are all together. Thank you to all this year's attendees for helping us to create the compassionate group spirit!

I cannot give enough thanks to Joanne Grieme, my conference co-chair, and Dr. Kapil Chopra of the University of Pittsburgh Medical Center, our conference co-host, for their central roles in planning and carrying out our outstanding weekend! It was such a pleasure working with both of them.

Our boat cruise on Friday morning was the perfect opportunity for attendees to see the charm of Pittsburgh and to start to get to know each other. After get-acquainted activities on Thursday night and Friday, we spent all day Saturday soaking up the phenomenal presentations from the medical professionals at the University of Pittsburgh Medical Center, as well as guest speakers from New York, Connecticut and Stockholm, Sweden. We all reviewed past information, learned valuable new information on all aspects of PSC and research on PSC, and broadened our perspectives. This year's speakers made a point of mentioning hopeful progress in the field of PSC, and they spent extra time answering individual questions. Thank you to our volunteer speakers for their participation! Please click on www.pscpartners.org/prevannual to view our 2013 conference website which includes the PowerPoint presentations, our weekend agenda, and photographs of the weekend.

Without the wonderful volunteer nature of our group, this conference would never have happened, so I'd like to thank just a few of the people who helped make the weekend such a success. Thank you so much to our generous sponsors, who covered 50% of the conference costs for each attendee!

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Our conference volunteers are too numerous to thank everyone personally, but I'd like to thank some people who played a central role in the conference: Joanne and Steve Grieme, Joanne and Steve Hatchett, The Pearlman Family, Rachel Gomel, Roz and David Parry, Ken Shepherd, Arne Myrabo, Tawny Holtz, Tiffany Rotondo, Jeremy Burke and all our volunteer team leaders. Our terrific staff members, Meegan Carey and Rachel Mittelstadt, oversaw every detail of the weekend, and kept things running smoothly. Their dedication and understanding of our group are so deep that I often forget that they are not PSCers!

For those of you who weren't able to join us in Pittsburgh, please consider attending our 2014 patient/caregiver conference. Our tenth anniversary conference will take place where our foundation started … in Denver, Colorado. The conference will take place the weekend of April 25-27, 2014 in conjunction with the physicians at the University of Colorado Health Sciences Center, under the leadership of Dr. Greg Everson. The conference will be held at the Hyatt Regency Denver Tech

Center. To reserve a room at the hotel at our group discounted rate of $109 per room per night, click here.

If you are interested in becoming our 2015 conference co-chair, please fill out the form at www.pscpartners.org/conferencelocations and return it to us before November 15, 2013.

To everyone in our PSC Partners community, I'd like to extend a heartfelt thank you for your willingness to volunteer your talents, time, and the support needed to help us continue our mission. One of our attendees referred to us as "a tiny mighty army." Together, we WILL find new treatments and an eventual cure for PSC!

Wishing you all a healthy and peaceful year until we reunite in Denver. I was so sad to say goodbye to everyone in Pittsburgh, but I returned home, feeling refreshed, rejuvenated and full of promise. I hope that our attendees left the conference with added knowledge, new lifelong friends, and a feeling of hope. Stay in touch!

Please watch for the launch of our patient registry, an exciting project that will hasten the pace of PSC research.

Together in the fight, whatever it takes!

Ricky Safer
CEO

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Do not miss next year in Denver!
10th Anniversary Celebration!

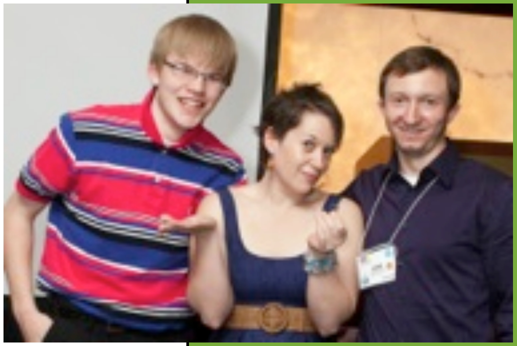
You're invited to the 10th Annual Patient Conference held by
PSC Partners Seeking a Cure
and organized in association with the
University of Colorado Health Sciences Center, Denver
April 25-27, 2014
Check our website and frequent updates
http://www.pscpartners.org/nextannual

Contents

From Our Members

Saturday Morning Session Summaries by Arne Myrabo

Friday Afternoon Session



We thank our official conference photographer, Karen Pearlman, of K Pearlman Photography for covering the conference with such great talent and heart. Karen is generously donating to PSC Partners receipts received for photos you purchase. Her website is: http://kpearlmanphotography.com. Her conference slide show and her conference pictures are on the 2013 conference page!

A Group Like None Other

by Joanne Grieme, Board Chair, Conference Co-Chair

Hello PSC Family,

I want to begin with a sincere and heartfelt "THANK YOU" for attending this year's conference in Pittsburgh. This year I was given the honor to co-chair with Ricky and showcase my hometown and its wonderful doctors and professionals at UPMC.

When I think back to the earlier conferences, I am struck by how much the conferences have grown in size and how far they have come, but one thing that has remained constant each year is the love and support of this amazing group that we call our PSC family. No matter how large the group becomes, we are instantly connected whether we are a red, blue, yellow, green or orange dot. We are a group that can laugh (and boy were there some good laughs this year) and cry together. And we are a group that is like none other.

When the last attendee left Pittsburgh, I felt a sense of relief that the myriad details that went into organizing the conference had seamlessly fallen into place. I also felt the success of the conference through the huge feeling of emptiness that was left in me. I greatly missed having all of my old and new friends around me.

Thank you again to each and every one of you for attending and giving me the honor of being your host.

'Til we meet again, in Denver!

Joanne

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PSC Partners Conference 2013

by Kapil Chopra, MD, UPMC, Conference Co-Host

As a first time attendee to this conference, what impressed me was the number of individuals – patients/caregivers who attended from different parts of the United States as well as international countries – Canada, UK, Europe, Australia, and South Africa – a true international gathering.

The questions asked during the interactive portions of the conference underscore the depth and sophistication of knowledge about PSC among the attendees; awareness of liver transplantation and its nuances; quest to learn more about diet; lifestyle and alternate approaches and an "open mind" to educating themselves about PSC/medical advances in this field.

It was refreshing to see the camaraderie among the attendees with attendees always "looking out" for each other.

From an operational standpoint, the flow of the conference was superb with volunteers available at every step to ensure that time management was maintained.

I have never before come across such a motivated, enthusiastic and caring group – clearly who will achieve their goal – i.e. find a cure for PSC. It truly was a pleasure and honor to be associated with the PSC Partners organization and helping organize this conference in Pittsburgh.

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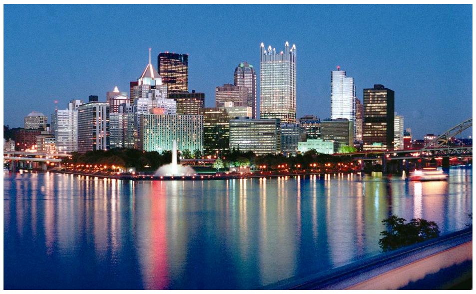

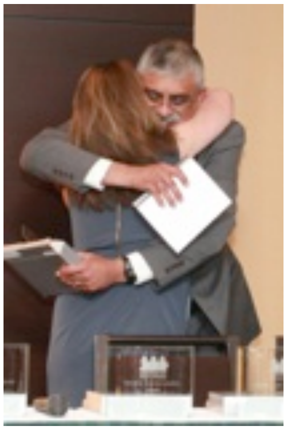

To Conference or Not to Conference?

by Sandi Pearlman, Community Relations Chair

I wasn't going to go. If you know me, you know that's no small thing. I half expect when the vampires take my blood one of these days that I'll get a call saying, "Um, you know, we're wondering something. You see, our techs are finding all these little *PSC Partners* floating in your bloodstream like some kind of madcap chicken soup...." So, when I say I wasn't going to go, even if you don't know me, maybe you can appreciate the gravity of those words and feelings.

Why? Well, I wasn't sure I had it in me, physically, emotionally or otherwise. I was just plain, old spent. I'd need to negotiate endless doctors arguments against leaving Clinic grounds/territory--I'm listed and rather ill--and I'd have to negotiate the terms of leaving and then I'd probably end up in the hospital upon return. Plus, how was I going to enjoy myself, run my sessions, do all I needed to do when I'm so far past empty that the red line gave up on the gauge and called out for pizza instead? But, pal, boy am I glad I went and here's why: You all rock. We rock. PSCers are amazingly extraordinary people and stunningly wonderful in so many ways.

Whether eating pizza, going up to The Incline or just hanging around in the lobby, our group is restorative, loving, loud (really loud) and so connected even strangers know upon seeing us that we're family. We share stories and secrets. Fears come out to see the light of day and we learn they're not quite so scary after all. We hold the hands of people whom two days ago we didn't even know but are now forever a part of our lives, inexplicably intertwined through this madness called PSC which only those touched by it can truly understand. We say we're PSCers and caregivers, but we're all caregivers. Not once did I look around at the conference and see somebody alone. Not once was somebody looking for a warm embrace unable to find it. And the camaraderie is evident, we miss each other, need each other, crave this world that restores us all in our sameness and our abilities to lose our inhibitions and just be. We scratch; we cry; we laugh; we vomit; we dance; we go through box after box of Kleenex and we love. Most of all, we love.

At the conference, it doesn't matter if you're 13 or 33, 40 or 75, we're all the same, even in our differences because we choose, on a level not even our subconscious recognizes as decision making, to recognize each other. During sessions, we devour information and look to the experts to fill gaps in our knowledge or teach us something new. Out of the sessions, we sing (boy, do we ever) and we shop and

we savor each moment because we know how fleeting they are. Those of us who normally sleep 18 hours a day or can't get up off the couch are suddenly going out to get sandwiches at 2:00 a.m. because we can't bear the thought of missing a moment. We literally exhaust ourselves for that one more second to spend together.

And goodbye is never easy. We know it's not forever, that another conference will come around, but still it hurts. Tears flow and hearts break a little knowing that the next morning we won't wake up and see those we've come to love and rely upon. But we know, too, that the invisible tether will hold strong. That we'll connect via technology (shameless plug for PSC Partners Facebook), via telephone calls and visits and, of course, the next conference.

So, I thank you. Thank all of you. Because, you see, you've restored me. You've given me strength to take with me and memories to keep me laughing (anyone who missed Karaoke, please, don't make that mistake again!). When I go into my next appointment, I'm not walking in alone. You're there with me, holding my hand and whispering words of encouragement. And when the news isn't good and I want to cry, your shoulders are ready for me to lean on. We're never in this thing all alone because we have each other. My cells may say PSC Partners, but they also bear each of your names and I hold them close. They shelter me, comfort me and remind me that no matter what the war against PSC brings, that we're all together in this fight, whatever it takes. We're stronger than PSC. We're stronger than anything thrown at us and together we'll find a cure. You'll save me and I'll save you because, quite simply, that's what family does.

YOUR EXPERTISE NEEDED!

Do you have online newsletter experience?

We need your expert advice!

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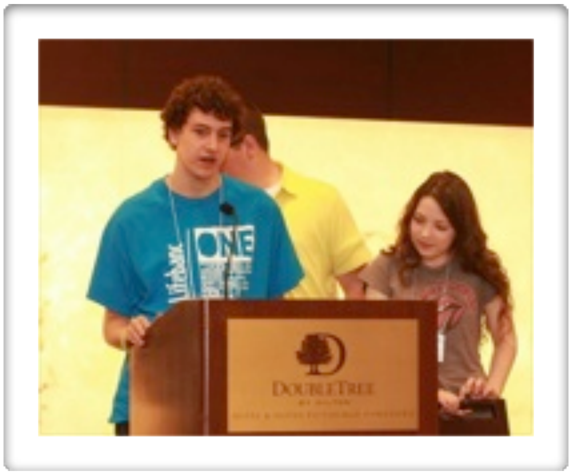

My Conference Experience

by Erik Loy

My conference experience started the day before we left for Pittsburgh. That Thursday night, before we had planned to leave, we got a call from Cleveland Clinic saying that they had a potential donor for me. However, I wasn't told until later that I was the backup recipient. So as we left for the hospital, all the emotions seemed to hit me at once. When we got there, they put in an IV and started fluids, so we assumed the primary recipient hadn't panned out, and I was going to get the liver. We waited until 2:00 AM before they told us the primary recipient had accepted the organ. So we went home, got a few hours of sleep, and left for the conference.

My mind was all over the place on the way down, and even for most of the first day. The conference was like a vacation from the real world though. It was so nice that I could be tired and that no one would say anything if I went to my room and took a quick nap. Before Pittsburgh, I had never met another teen with PSC. I knew several older men with the disease and a few women, but being able to talk to other teens was so much fun. There were three of us and we never left each other's sides because we just connected so well. I made so many friendships I know will last forever. I definitely found a second family at the conference and I'm so happy I did because I know I can turn to them whenever I need support. The conference was one of the best experiences of my life, and I plan to go to many more.

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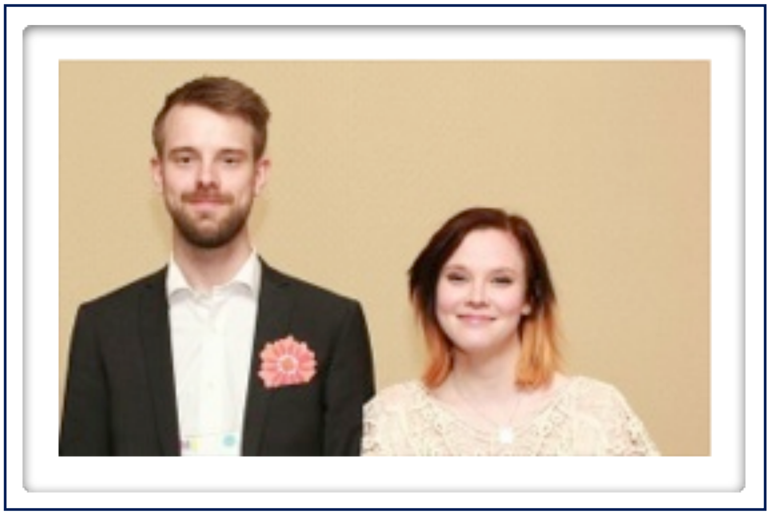

A Growing Sense of Direction

by Nicklas Holmgren (Sweden)

When I was heading back home from the first PSC Partners conference I attended, I felt a significant sense of direction and comfort. More than I had felt in years.

It was the first time I had met a group of PSC patients and their caregivers; people that were in the same situation as myself, people that I could instantly relate with. We shared some of the same problems and situations, and we were able to talk about a lot of things I hadn't been able to talk about with others previously. It was an amazing experience.

That is why I have returned almost every year since, when I have been able to. The conference in Pittsburgh was my sixth so far. I was sad when it came to an end, but afterwards I was left with the same great feelings as after each of the conferences before it.

I am happy that PSC Partners hosts this amazing conference every year; happy to have met a lot of dear friends, both old and new; happy to have learned so much more about our common disease from the great presentations; happy to have enjoyed the great community and the kindness of everyone I met; happy that my sister joined me for the trip once again.

As I am writing these words, I am still filled with the same sense of direction and comfort that have lasted ever since that first conference, but that have only grown stronger every year since.

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A Breath of Fresh Air

by Suzi Lengyel

The conference in Pittsburgh was my first conference. I am from Pittsburgh so it was perfect that it was in my city! I am a little angry at myself for not attending a conference sooner. I had an amazing time. I was a little apprehensive at first, as I'm sure everyone is. I soon came to realize that this was a place where I was loved and understood more than I've ever been.

I think my favorite part of the conference was meeting and getting to know the young adults with PSC and the 20's and 30's women, or as we like to call ourselves "The Pretty Sick Chicks". Before the conference, I didn't know anyone with PSC. So it really has been a breath of fresh air to have these people in my life. I finally have someone who I can call on a bad day and not have to explain every single thing that's going on. All I have to say is I'm itchy and they understand. I am so happy that I experienced the conference this year and I will definitely be there next year in Denver!

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back to Contents

It's Not Just About Having Something in Common

by Suzanna Donahue

It was with mixed emotions that I registered my husband and myself for the annual PSC Conference. It wasn't too long after registering that I questioned this use of our limited funds. I (as true for most PSCers) had done an awful lot of research and figured there was little more to learn, despite others saying that they learn something new every year at this conference. However, I remained hopeful that even if the presentations proved to be a repetition of what I already knew, I could leave there with some small foundation of a social network that I knew I would need in the near future. The conference was in my backyard. Surely, I would meet others in the area who are also dealing with this disease.

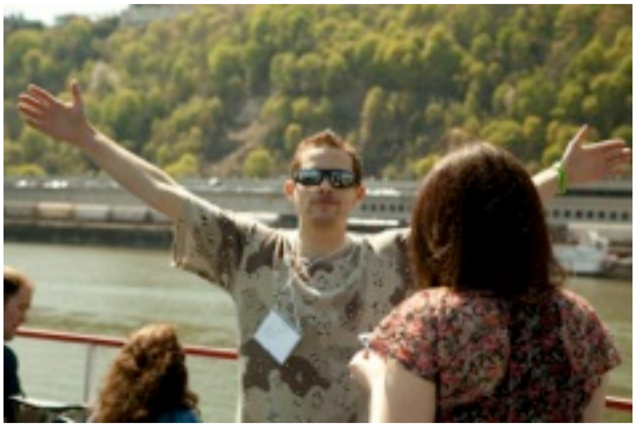
Newcomers to the conference are often overwhelmed, and we were certainly not immune to that feeling. Thankfully, "mentors" were assigned to the newcomers and we left feeling like we had at least one person we knew and felt comfortable with. Next was the Welcome Dinner. An enormous ballroom was filled with tables and people – people who are dealing with PSC either as the patient, family member, caregiver, or friend. I sat there staring at all these people and thought in my best imitation of a three-year-old not getting her way, "I do NOT want to be a part of this group." I didn't want anything to do with this. I wanted to get out of Dodge.

I sat at my table filled with mostly 20-somethings (I'm 42), and listened in awe to their stories (everyone there has one) and how they are dealing with it with so much grace and humor. I didn't feel much better about my lot in life that evening, but a seed of something had been planted.

The next day was filled with presentations, some of which were given to all attendees at breakfast, and some breakout sessions. We also had breakout groups of people with similar "positions," if you will: caretakers, teen PSCers, post-transplant PSCers, etc. I could not easily sum up everything I learned at the presentations, but trust me when I say, you really do learn something new at these conferences despite all the research you may have done on your own.

I met so many people with so many different stories that held one common theme: We are all affected, in some way, by PSC. I worried about how this conference would affect our outlook. I was concerned about my husband being overwhelmed by the stories of other caregivers and had suggested he avoid the group (thankfully, we were talked out of that idea). I was concerned that I would see what my future held and would come out of there more depressed.

Instead, we both left there with something we never expected: a family we never knew we had. It's not just about the stories. It's not just about having something in common (although that is a big part of it). This disease can be very isolating, and it is comforting to know we are not alone in this battle. The fact is, these people are truly amazing and wonderful human beings. I came in feeling overwhelmed and alone and left there feeling connected and inspired. And, honestly, I cannot imagine going to battle without these incredible people by my side.



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Back to Contents

Rex Schechter's Song

How could we ever forget Rex's song to the tune of "Piano Man"? You can hear him ***right here*****.**

PSC is a Liver Disease
It's nine o'clock on a Saturday
And Ricky's turn at the mike
Don stands to support her cause
They're in this fight together with life

Philip's been talking gobbledegook
Bout treatments and lab tests and such
It's alphabet soup, but we all need to learn it
To beat this thing that we hate so much.

La la la, did a da
La la didid a da dum

Chorus: PSC is a liver disease
That tries to take over your life
It gives yellow skin, takes all our spoons
And sometimes it cuts like a knife.

Most of our doctors aren't up to speed
We have to educate them.
But they're quick with prescriptions and another lab test
Or to question the color of phlegm.

Sandi's lived with this a very long time
Her transplant is long overdue
She still inspires, comforts and organizes
Knowledge for me and for you.

Chorus

Karen is conference photographer
She'll document all that we do
We talk about bile ducts, strictures and stents
And yes, I had one. Have you?

Ken is a fund-raising dynamo
And keeps us on the right track
We're collecting donations and more information
It's one way that we can fight back.

Chorus

Some doctors talking bout bodily secretions
And genetics gets called into play
They don't know the cause, but we're still fightin back
Cause we don't know a-nother way.

Our leaders have set up the conference
Attendance has certainly grown
We gather and share our experiences
So we know that we're not alone.

La la la, did a da
La la didid a da dum

Chorus

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My Brother's Two Liver Transplants: How I became His PSC Partner

by Kathryn Kallmes

Kate could not make it to the conference in Pittsburgh, but she asked us how she could participate in PSC Partners. We asked her to write about her experience as a sibling of a PSCer. This is what she wrote for The Duct.

My brother Andy was diagnosed with PSC when he was seventeen. He is now forty-four and healthy, but he has faced more medical challenges in his life than anyone I know. People familiar with PSC are aware of the symptoms, the tests, the medications, and the uncertainty. I'm writing because I want to share how Andy inspired me to turn the negative into positive, motivated me to take action, and demonstrated how to live as though PSC didn't exist. It's easy to be sad or to feel pity, easy to dwell on the daily struggles of living with PSC (which I know I cannot imagine), easy to believe there is nothing an ordinary person can do. But Andy, in one miraculous week, changed all that for me.

I was in college when the doctors told my parents their youngest son had a rare disease, one that would perhaps involve liver failure, possibly cancer, and maybe a shortened life expectancy. My father reacted by denying anything was wrong, and my mother was just overwhelmed. In a sense, he was abandoned. Once I reached adulthood, I read the literature and was aware of the seriousness of Andy's disease. I knew about it intellectually, and had seen Andy itching, getting jaundiced, losing weight. I knew he had the best care (Tufts Medical Center and Mayo Clinic, Rochester). I knew my mother and his wife were there to help. I knew and I didn't do enough. I now know that just being aware is not advocating, lobbying, researching or helping.

I had worried about his health all those years, but now I was completely floored. Even though the Mayo team told him repeatedly that they would be able to do the second transplant, I couldn't quite believe them. I saw Andy on New Year's Eve, twenty days before his transplant, and knew he was struggling. That didn't deter him. We laughed at his funny stories at family dinners, touched on serious subjects, and played with the kids in the snow. He joked about his jaundiced skin and about being stared at by kids and adults alike. He slept a lot and we gave him pain medication. It was all we could do. All I could think of was his wife and young children, his thriving business, and his optimism gone forever. Of course, being Andy, he just said, "I need a liver, that's all. I'll get one, don't worry." Oh my. The drive back to Ohio was endless and I felt the unbearable pain of thinking I wouldn't see him again.

Over the week, we were together the whole time. We spent a lot of time at the Clinic, but we had our share of fun. We went to Target together and laughed that I was pushing the cart too fast and that I wouldn't let him pick up a gallon of milk, but we did fine. I was overjoyed to be doing regular, everyday things with him. It was not difficult to be there for him, and I found I couldn't do enough for him. I helped him to clean the room he'd occupied for several months leading up to the transplant, organized his medications, helped with the new procedures (e.g., checking insulin levels), worked on the endless insurance claims from the past year, went out to lunch and gleefully watched him enjoy food for the first time in months. He gained weight. His color improved. We compared the skin on our arms and recorded the daily change in his color. By the end of the week, not even two weeks post-transplant, his skin-tone almost matched mine. I repeated to myself, "The new liver is really working, it's really working." He sent an email to family and friends with the subject line, “Remember to change your liver every ten thousand miles or forty four years, whichever comes first.”

But more than anything, I stilled myself and I listened. I learned that it had been discouraging waiting and hoping for new cures, that his numerous admissions to the hospital had stolen precious time from his life, that he and his wife had spent $70,000 out-of-pocket on the first transplant and would likely spend more on this one. I learned that his oldest friends stayed with him through all the trying times, and that one friend, though unhealthy and overweight, had wanted to donate part of his liver.

Our family came together in a way it hadn't since my father's death. Two of my brothers underwent testing to be partial liver donors. One was a perfect match, we found out the day after the transplant. One brother accompanied Andy to his endless pre-transplant appointments. His wife made amazing food for Andy. My mother, for the first time in many, many months, allowed herself to hope.

Six months on, I'm now a Donate Life Ambassador, an official role in which I advocate for organ and tissue transplantation. I have met donor family members, transplant recipients, and those waiting for a transplant. In addition to educating others about organ and tissue donation, I wear my green "Donate Life" bracelets every day and respond with a smile when people ask what the bands are for. I help out at health fairs and fundraisers and encourage people to register as donors. I see this as a second job. I tell my kids it‘s for Uncle Andy and they get it. I invite people to learn more about PSC and UNOS (United Network for Organ Donation) by clicking on a link in my email. I read and forward articles about liver research. I learned about legendary Thomas Starzl, MD, PhD, the Pittsburgh physician who performed the first human liver transplant. I found PSC Partners Seeking a Cure. I sent Andy a care package of his favorite foods when he returned

home. Thinking back on the hours I spent in the family lounge, I collected and sent puzzles to replenish the Eisenberg 10 stock. Little things, all. But I think they're starting to add up.

On Andy's last day at the Clinic, I met a man in the elevator. He was as jaundiced as Andy had been at the beginning of the year. My heart sank. He told me he was in the right place and that he believed he would get a liver soon. I told him I would think of him and hope. I knew that I was fighting for him, too.

Though I still can't wrap my mind around two liver transplants, I believe I can make a difference in Andy's life and in the lives of everyone affected by PSC. I don't have the disease, but PSC is part of who I am. We are, indeed, Partners Seeking a Cure.

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Back to Contents

Conference Session Summaries

Reported by Arne Myrabo

Natural History of PSC

Kapil Chopra, MD, Director of Hepatology, Associate Professor of Medicine, UPMC

Click for presentation slides
Reported by Arne Myrabo

Dr. Chopra started his presentation with *one* example of a liver clinic patient to show the variability of PSC.

This is the case of a twenty-six year old female with elevated liver function tests and Crohn's disease. Endoscopic retrograde cholangiopancreatography (*ERCP*) resulted in PSC diagnosis after which she was started on Urso.

On her return visit, she was engaged to be married and had the following questions: "Should I get married? Should I have children? Will I be alive to see my children grow up? How fast will PSC progress? When will I need a liver transplant? Can Urso be used during pregnancy?" Her visit lasted two hours!

Eleven years later, she is married, has had two successful pregnancies, has near normal liver function tests, and has no PSC symptoms. Her MRI shows only mild PSC.

Natural History

PSC is a progressive, chronic cholestatic (limited bile flow) disease of unknown cause, commonly associated with chronic colitis. It often leads to advanced liver disease and failure that require transplantation. There is currently no effective medical therapy.

PSC is thought to result from a combination of genetic predisposition and immune-mediated events leading to bile duct damage. Relative to the general population, the risk of PSC increases among siblings of PSC patients. PSC often occurs along with other autoimmune diseases.

Clinical History

A few population-based studies exist, but they are mostly limited to North America and Europe. At any given time, there are up to 16 PSC patients per 100,000 population. PSC predominantly affects men (2:1), at a median age of 40 years, and is strongly associated with inflammatory bowel disease (usually ulcerative colitis - 70%).

Studies show that 2 to 7.5% of UC patients develop PSC (1.3-3.4% of Crohn's disease patients). In general, IBD is diagnosed several years earlier than PSC, but it can be diagnosed at any time during the course of PSC and vice versa. PSC may also develop years after colon removal for UC, and IBD can develop many years after liver transplantation for PSC.

PSC can vary significantly in individuals, but it usually follows a progressive course. Mean survival time from diagnosis to death (without transplant) ranges from 7 to 18 years. Those who have no symptoms at diagnosis may have better survival compared to those with symptoms.

In blind trials, there is no significant difference in long-term survival between patients with PSC given Urso. However, patients who have reduced or normal levels of alkaline phosphatase (ALP) have longer survival times regardless if they receive Urso or a placebo. Improvement in serum alkaline phosphatase (SAP) to below 1.5 times upper limit is associated with better outcome and reduced risk of cholangiocarcinoma (CCA).

Disease complications

The usual course of PSC is a continual worsening of cholestasis (blocked bile flow) and eventual development of jaundice and end-stage liver disease. The most common symptoms of PSC are fatigue and itching. The most common initial symptoms are abdominal pain, itching, diarrhea, jaundice, fatigue and fever. Some other possible complications are metabolic bone disease, gall bladder stones and polyps, peristomal varices, dominant stricture, bacterial cholangitis, and malignancy.

Variant forms of PSC

Small duct PSC: Both biochemistry (blood tests) and biopsy are indicative of PSC, but the cholangiogram is normal. This type of PSC has a milder disease course, longer survival and is less likely to develop cholangiocarcinoma (CCA). Twenty-five percent progress to classic PSC.

Overlap with autoimmune hepatitis: This type of PSC may respond to steroid therapy.

IgG4-related sclerosing cholangitis: In about ten percent of PSC patients, IgG4 levels are elevated. These patients have a more severe disease course but may respond to steroid therapy.

PSC and Pregnancy

Little is known about pregnancy in patients with PSC, but liver disease activity is not significantly worsened during this period. Fertility does not seem to be reduced in PSC since there is no difference between the number of children in PSC patients and healthy controls. Urso has no negative effects on pregnancy outcome; however, close monitoring is required.

Transplantation

Excellent survival rates are observed (90-97% at one year and 83-88% at five years). Recurrence of PSC in the new liver is estimated at 10-20%, and may be more likely for biologically-related living donors.

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Back to Contents

Current Concepts and Management of the Pruritus of Cholestasis

Nora Bergasa, MD, Chief, Department of Medicine, Metropolitan Hospital Center, NY, Professor of Medicine, NY Medical College, Valhalla, NY

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Reported by Arne Myrabo

Definitions

Cholestasis is defined as the impaired secretion of bile, by whatever means that may be. It is a consequence of most liver diseases and results in an accumulation in tissues of substances excreted in bile.

Pruritus (itching) is a complication of cholestasis (impaired bile flow). The cause is unknown. Treatments are not always satisfactory, and by itself, pruritis can be an indication for liver transplantation. It can be intermittent, localized or generalized, and is worse in the premenstrual period for one fourth of female patients.

The pruritogen (the substance causing itching) is made in the liver, excreted in bile and accumulates in tissues due to restricted bile flow.

Substances/Neurotransmission Systems of Interest

Bile acids accumulate as a result of cholestasis. Concentrations of serum bile acids are unrelated to pruritus symptoms. Levels of bilirubin and other liver function test results do not correlate with pruritus symptoms.

Serum histamine concentration increases in cholestasis and pruritus, but the skin of patients does not show any histamine-related signs such as redness or swelling. Those who get relief from pruritus using histamines may be experiencing a sedation effect.

Serotonin is involved in mediation of pain response. Some patients report relief with Zofran (type 3 serotonin receptor antagonist).

Substance P is an excitatory substance that is involved in pain, inflammation and possibly pruritus. Concentrations are significantly elevated in patients with liver disease and pruritus, suggesting a possible path of treatment.

Autotaxin is the enzyme that activates lipophosphatidic acid (LPA). It is reported to be high in patients with liver disease and pruritus.

Endogenous Opioids – Opiate antagonists prevent the effect of natural opioids and opiate drugs, and should relieve this type of pruritus. Low dose naloxone has been shown to be effective in some patients, with acceptable safety.

Pruritus Management

The goal is to remove the pruritogens. Currently used are nonabsorbable resins (e.g., cholestyramine), extracorporeal albumin dialysis (MARS), and plasmapheresis. Albumin dialysis treatment is quite popular in Europe.

Antibiotics such as Metronidazole and Rifampicin are prescribed for pruritus. These have possible hepatotoxicity.

Neuromodulators including Setraline, Dronabinol, Gabapentin may be prescribed.

Challenges

Opiate antagonists: Some patients don't respond and some develop tolerance. It is not desirable to be in an antiopiate state.

Kappa agonists: Nalfurafine is approved in Japan for pruritus that arises from kidney disease. It is being studied for pruritus in cholestasis.

Previously Approved Drugs

Medications used for other conditions are being studied as treatments for pruritus.

Neuropathy: Lyrica
Substance P antagonists: Aprepitant (anti-emetic)

Dr. Bergasa stated that there has been a tremendous international interest in research on pruritus, and that she is very optimistic we will soon see progress in pruritus research.

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MELD Issues - The Unknowns About How UNOS Works

Christopher Hughes, MD, Associate Professor of Surgery, Surgical Director,
Liver Transplantation Starzl Transplant Institute, UPMC

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Reported by Arne Myrabo

Transplant surgery may be one of the most complicated medical fields. It includes religious, government, medical, and technically challenging surgical considerations.

The United Network for Organ Sharing (UNOS) is a federal agency created under the National Organ Transplant Act (NOTA) of 1984. UNOS has established the Organ Procurement and Transplantation Network (OPTN) and authorized the formation of Organ Procurement Organizations (OPO).

OPTN consists of 250+ transplant centers, about 60 OPOs, 150+ histocompatability labs and 26 medical professional scientific organizations. It falls under the Executive branch, through the Department of Health and Human Services, Health Resources and Services Administration, Healthcare Systems Bureau, and finally, the Division of Transplantation. UNOS is a private, not-for-profit organization contracted to run the OPTN with a contract renewal every 4 years.

UNOS was formed to increase the efficiency of organ sharing and equity in the national system of organ allocation and to increase the supply of donated organs for transplant. There are 11 UNOS regions. Organ Procurement Organizations are charged with evaluating and managing a potential donor in a specific geographic area. This area is called a Donor Service Area (DSA), of which there are 58. UNOS then allocates the organ(s).

When an organ becomes available, its allocation depends on the following:

Status 1A means that a patient is considered first in the region. The organ is allocated to those who have had immediate organ failure following a transplant or to those with fulminant hepatic failure.

If there are no Region 1A patients, the organ is offered to those with MELD scores higher than 35 located in the region.

If there are no patients in the above categories, the local donor service considers those with a MELD score of 15-34.

MELD Score

MELD score is calculated using creatinine, bilirubin and INR levels. INR measures blood coagulation and is more heavily weighted in calculating the MELD score. The MELD score is a measure of the probability of living for an additional three months, and cannot exceed a value of 40. PSC patients are somewhat disadvantaged due to recurring infections that can result in multiple organ failure.

A review of MELD scores at transplant for each DSA shows that the OPTN rules do not really result in an equitable distribution. For example, the Minnesota DSA MELD score at transplant averages 32, while Missouri's is about 22.

A revised MELD calculation that includes serum sodium has recently been proposed. There is much more work to be done at many levels to improve this situation.

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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.

Our big thanks to Arne Myrabo for setting up the 2013 conference website and for being our webmaster.You can access the presentation slides and other information on the 2013 conference webpage.

Back to Contents

Advances in the Endoscopic Evaluation of PSC

Adam Slivka, MD, PhD, Professor of Medicine,
Co-Director of the Liver Pancreas Institute, Associate Chief of the Division of Gastroenterology Hepatology and Nutrition, UPMC

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Reported by Arne Myrabo

Endoscopic retrograde cholangiography (ERC) is used to diagnose PSC and manage complications of PSC (bile duct stones, acute cholangitis, dominant strictures), and to diagnose cholangiocarcinoma (CCA). Dr. Slivka's focus was on the techniques used for the diagnosis of cholangiocarcinoma.

Cholangiocarcinoma may develop in 15-25% of PSC patients, with the highest risk occurring in the first years after diagnosis. The nature of this tumor (lots of scarring and collagen) and the presence of multiple, non-cancerous strictures make diagnosis extremely challenging. The tumors "shield" themselves with scar tissue. In scraping for cells, it is possible to get scar tissue rather than the cells in question. It is important to remember that, "highly suspicious for cancer" does not equal cancer in PSC. Inflammatory processes may create the semblance of CCA. These diagnostic difficulties notwithstanding, there are some differences between CCA and non-CCA PSC patients: CCA patients are more recently diagnosed, less likely to have IBD and more likely to be male.

Cytology and tumor markers

Tissue sampling (bile duct brushings, needle, forceps). All of these are specific for CCA (if positive, CCA is present), but have low sensitivity (may not detect CCA). Multiple brushings improve the sensitivity of this test.

Tumor markers CEA and CA19-9. The cutpoint for CCA using CA-19-9 is around 180-200 U/mL. The cutpoint for CEA is 5 ng/m.

Combinations of these tests can yield better accuracy.

Some advanced cytology tests include *flow cytometry* which detects DNA content changes (with cancer) and *FISH (fluorescent in situ hybridization),* an automated version of flow cytometry. Accuracy is improved, but there are still false positives.

Similarly, *KRAS, LOH, Ocongenes tests* do not give the optimum results.

Novel imaging

Positron Emission Tomography - Cholangiocarcinoma is a very slow growing cancer and PET requires a fast metabolic process for effective results.

Cholangioscopy (imaging the interior of the bile duct). This test does look promising, but the bile duct has to be large enough for the scope, and the area of interest may not be accessible.

Adaptive optics is the process some telescopes use to account for atmospheric distortions from the image using vibrating mirrors. A similar process can be used in biomedical applications.

Probe-based Confocal Laser Endomicroscopy (pCLE) was developed by astrophysicists and is showing good promise for cholangiocarcinoma detection. It yields improved sensitivity, earlier detection, uses a cholangioscope or catheter, but more work is needed to reduce false positives.

Controversies

Does screening tumor markers make sense?

Will molecular markers allow for premalignant diagnoses?

What are the performance characteristics of direct cholangioscopy and pCLE of dominant strictures for diagnosing CCA?

Diagnosing cholangiocarcinoma in PSC is usually a death sentence. How hard do we push?

Should PSC patients be transplanted for prophylaxis against CCA?

Should transplant be used as an oncologic procedure?

What is the role of living related donor transplants in PSC with possible CCA?

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Metabolic Bone Disease in PSC Patients

Mara Horwitz, MD, Associate Professor of Medicine, Division of Endocrinology, University of Pittsburgh School of Medicine

Reported by Arne Myrabo

Bone Physiology

Bone is an active organ that is continually broken down and reformed, both for growth in the young and to maintain strength. Peak bone density is achieved at 25-30 years. Even after your linear growth stops, you are still building bone. The growth process is controlled by numerous hormones as well as immune factors. You have two components: the scaffold/bone structure and the bone marrow. There is a very complex interaction between the two. Calcium levels are kept at a very tight level around 9.5, and controlled by the parathyroid gland. 1,25–dihydroxy vitamin D is the active form of vitamin D, and allows calcium absorption from the intestine. Dietary vitamin D (and that produced by sunlight) is activated to 25 dihydroxy vitamin D in the liver and then to 1,25 hydroxy vitamin D in the kidneys. So when the liver is not functioning properly (as in PSC) or your intestines are not absorbing properly (as in IBD), vitamin D deficiency may affect your calcium balance.

Most of your bone structure is quiescent, just maintaining structure. Bone remodeling is accomplished starting with osteoclasts which break down old bone. Osteoblasts then lay down osteoid, which is bone material before calcium and phosphate minerals are added. The osteoid fills up the cavity to the level it had been before. As the osteoid mineralizes, the bone returns to the quiescent state. (For better visualization of the bone remodeling process, Arne Myrabo included this short YouTube video by AMGEN http://www.youtube.com/watch?v=78RBpWSOl08 .)

In some circumstances, as in post-menopausal women, those taking steroids, or those having an overactive immune system, the cavity may not get completely filled in, reducing bone strength.

Men's bone density values do not drop as precipitously as women's at menopause, but they do go down. Much of bone mass behavior is determined by genetics.

If you get very ill (as in IBD, PSC, and many other liver diseases), you may have a rapid fall in bone density and hit the fracture threshold at a much earlier time.

Osteoporosis

Osteoporosis is a skeletal disorder where decreased bone strength predisposes to increased fracture risk. Bone is a lattice structure, much like a scaffold. In osteoporosis, the "scaffold" thins, making it much more likely to fracture. Risk factors of low bone density include having low body mass index (being very thin), older age, being post-menopausal, muscle-wasting, family history of osteoporosis, malnutrition and glucocorticoids. Bone density is measured using a Dexa-scan which is a dual energy x-ray used to assess bone density. The normal, representative areas measured are the lower spine and hip. Bone density is a very good predictor of fracture risk. For every 1-2 decrease in T-score, you approximately double your risk of fracture. A T-score of -2.5 or lower defines osteoporosis. -1 to -2.5 is osteopenia (pre-osteoporosis). -1 and higher is normal bone density.

Glucocorticoids, liver or kidney disease and several other conditions can increase the risk of osteoporosis. This includes people with PSC, IBD and celiac disease. Glucocorticoids affect your ability to make bone, retain calcium in urine and absorb calcium in the gut. For those diagnosed with PSC in childhood, it may be difficult to achieve peak bone density, and linear growth may be affected. All age groups are at risk, depending on dose and length of treatment. The risk of fracture is increased when using glucocorticoids, since both bone quality and quantity are decreased.

Malnutrition is a huge contribution to bone loss. Vitamin D and calcium deficiency are seen in many liver diseases, IBD (especially with bowel resections in Crohn's Disease), celiac disease and those with extremely low body mass index. Many girls with IBD have late onset menstruation, and patients that are acutely ill may lose their periods. In men, there may be a decline in sexual function.

Cytokines are made by inflammatory cells in PSC and IBD, and cytokine-related bone loss is common. Renal failure, thyroid disease and liver dysfunction itself are also contributing factors to bone loss. High bilirubin and low vitamin K can be "toxic" to bone.

In a study of PSC/PBC transplant patients (end-stage liver disease), 40% had osteoporosis, 35% had mild bone loss and only 25% had normal bone density pre-transplant. 20% had already had fractures.

The real question is to find out your fracture risk. In a study of patients with IBD, the overall fracture risk was 40 times that of the general population. Risk of spinal compression fracture was 72% higher, and hip fracture 50% higher. Only 13% were being treated for low bone density.

Treatment

There *are* treatments, and they should be started *before* the onset of osteoporosis. Reducing inflammatory processes, minimizing both dosage and duration of steroid treatment and maintaining adequate nutrition are very important. PSC patients should be getting about 1500 mg Calcium/day and 800 units of vitamin D (dietary + supplement). Vitamin D levels should be measured. It is not uncommon to have to use very high levels of supplementation with severe disease. Reducing risk factors and adopting fall prevention techniques are important. Numerous medications are available to treat osteoporosis, but determining the appropriate one depends on the individual circumstances.

Summary

Patients with liver disease are at increased risk of bone loss and fracture. There are numerous causes: age, sex, glucocorticoid use, disease severity, low body mass index and malnutrition. This disorder is often underdiagnosed and undertreated.

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Back to Contents

Treatment with Ursodeoxycholic Acid in PSC: What Do We Learn From the Scandinavean Urso Study?

Annika Bergquist, MD, PhD, Associate Professor, Karolinska University Hospital
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Reported by Arne Myrabo

Background

Numerous studies with Urso have been performed with 3-5 years follow-up, at various dosages and with results varying from improved biochemistry to no change, to increased adverse outcomes. If these studies are lumped together and results are analyzed, Urso does not appear to be of use in PSC.

Biochemical response and PSC

Use of Urso in Primary Biliary Cirrhosis (PBC) has shown to be beneficial (not so for PSC). Recent research shows that improvement in alkaline phosphatase (ALP), by whatever means, is associated with better outcome.

Scandinavian Trial (17-23 mg/kg)

The survival distribution of this trial shows a slight trend for improvement with Urso. What would happen if we followed the study longer?

The study ended eight years ago, but most of the trial participants were tracked down, and further evaluated. However, the newly extended survival graph still shows no or little difference with Urso.

Those responding to Urso (defined as improvement of ALP) clearly did better than non-responders. If you then compared all the patients with improved ALP regardless of treatment, the difference was even more pronounced. The responders to placebo did the best of all.

Results

Five years of treatment with Urso at 17-23 mg/kg/day does not affect long-term outcome.

ALP reduction, regardless of Urso treatment, is associated with better long-term prognosis (this is consistent with other studies).

If Urso leads to a reduced or normalized ALP level, treatment may be beneficial.

ALP is a suitable marker for disease progression and should be considered for further evaluation as a PSC trial endpoint.

* * *

For Q & A sessions
following the presentations

http://pscpartners.org/PSCConf13/session_i_qa.htm

http://pscpartners.org/PSCConf13/session_ii_qa.htm

Affordable Care Act and Its Impact on Persons With Serious Chronic Diseases

Victoria Veltri, JD, LLM, State Healthcare Advocate
Office of the Healthcare Advocate, Hartford, CT

Click here for presentation slides

Following the Friday morning brunch on the river cruise, a large and cheerful group of conference participants walked into the conference hall to hear Victoria Veltri speak about a topic that is on the minds of most Americans, the Affordable Care Act and, with this audience, its impact on PSCers.

Those of us who had been present in previous conferences, had heard Jennifer Jaff, the compassionate chronic-disease advocate, speak on the rights of chronic disease patients. She had held an important place among PSCers who sought her generous help and guidance. We had felt a great sense of loss when she passed away last year. Hearing Victoria Veltri reminisce about her colleague instantly made us feel that she, too, would become an equally great resource for PSCers.

Victoria Veltri started by describing her experience locally, at the Connecticut Office of the Healthcare Advocate (in existence since 2001) and explained how similar offices have been recently set up in some states. With the Affordable Care Act, each state will be setting up this kind of federally recognized independent office to advocate for patients facing issues with health insurance coverage, billing disputes, and difficulty of access to quality healthcare. The role of these independent state offices is to serve as watchdog for the whole healthcare industry, to represent the consumers collectively, and to reduce waste in health services.

They help with the filing of complaints and appeals and help educate consumers on their rights and responsibilities with group health plans and health insurance coverage. Their mission and *duty* are to provide access to quality healthcare to everyone. In Connecticut, the office helps about 6000 people every year. Eighteen states have refused to join, so to receive information about the state of residence, you can refer to, www.healthcare.gov.

The need for such a regulatory body is clear when we look at healthcare in the US: 16% of 48.6 million people are uninsured (those insured are mostly insured by their employers); of those 7.6 million, that is, 10%, are children; 20% of the total US population lives in poverty. And in recalcitrant (noncooperative) states that refuse to comply with the Affordable Care Act (18 states), the federal government will step in.

What has been troubling for PSC patients is that no insurance company will give an insurance policy to anyone with pre-existing conditions. Today, anyone with PSC will not get an insurance policy, nor will someone with high cholesterol. *More than 40% of the US has one or more chronic condition.* And that is not an insignificant number of people. The likelihood of having a chronic disease increases with age. And half of working-age people in the US have *at least* one chronic condition! Chronically ill people are poorly served and neglected. The Affordable Care Act has the mission to make changes to this situation.

Studies have clearly shown that health coverage is directly correlated to health, and that the uninsured are seven times less likely to get care for a medical emergency, cannot afford tests and preventive medicine, cannot have access to care to manage chronic disease, and tragically, 25% are more likely to die prematurely. Obviously, those who have access to medical care are healthier.

The Affordable Care Act

Through this act, the uninsured and self-employed are now able to buy insurance through state-based exchanges [which are governmental or quasi-governmental entities that exist to help insurers comply with consumer protections and to facilitate the expansion of insurance coverage to more people. No "cherry-picking" of customers will be possible in the state-run Exchanges mandated by the Affordable Care Act]. The government has provided funding to the states until January 1, 2015, to establish these health insurance Exchanges.

The health insurance Exchanges create a marketplace that is guided by a "no wrong-door approach." No one is refused or told to knock on another door. And every insurance package people get has to have all the basic benefits. If people cannot afford insurance, they are assisted and given advance tax credit.

If your employer is self-insured, the employer pays for your health bills. Your coverage will naturally be decreased so that the employer can cover cost. Or, if you are a small business and cannot afford to pay for all your employees, you will pay an insurance company a fixed amount to cover your employees. Under the Affordable Care Act, if a state doesn't create an Exchange, then the federal government will either partner with the state or will do it alone. The Affordable Care Act guarantees money to set up a marketplace for people to buy insurance. People who come through the door online, on the phone, or in person will be evaluated for Medicare/Medicaid or private insurance eligibility.

The law says that essential benefits must be provided to everyone. If you don't qualify for Medicaid or do not have a high enough income, the exchange will help you with your insurance and give you an advanced premium tax plan which is going to be invisible to you. Unfortunately, the form you will have to fill will be long, as the federal government will have to check your income against the IRS, your immigration status (undocumented immigrants will not be eligible) and other information.

The Exchange system is based on the fact that some people will pay more to keep their premiums down. Some will have higher deductibles. Others will want more complete coverage. There are four levels of coverage:

Bronze – 60% (most current individual and small business plans are under 60% – and most are considered as grandfathered plans. In this category, people face problems of affordability)

Silver – 70%

Gold - 80%

Platinum – 90%

(The actuarial value is the percentage of expenditures paid by the plan, and the remainder is the consumer's responsibility. If an individual's and family income is $29,327, purchasing on the Exchange will be possible. If people are covered by their employer, or once they use the Exchange, they cannot be eligible for Medicare or Medicaid. On the Exchange, they can receive subsidies to purchase health insurance in the form of premium credits with a cap on how much they will have to contribute to the premiums on a sliding scale.)

The Affordable Care Act has a mandate requiring every individual to purchase health insurance or face a $695 annual fine. Some exceptions are made for low-income people. Employers having over fifty employees must provide health insurance. For smaller businesses, tax credits are available for purchase in the Exchange. Undocumented immigrants will not be allowed to buy insurance in the Exchanges.

Essential Health Benefits (EHB)

These are core area benefits that are offered to insurance plans in the Exchanges to individuals and small employers. These broad categories of health services must be present in every state:

Ambulatory patient services, emergency services, hospitalization, maternity and newborn care, mental health services, prescription drugs, rehabilitative services and devices, preventive and wellness services, chronic disease management, pediatric services and essential wellness clinics.

EHB cannot discriminate against age, expected length of life, present or predicted disability, degree of medical dependency, or quality of life. Though these core area benefits are required in every state, each state has different items under each category. The insurance laws of every individual state still govern the extent of coverage. In Connecticut, for example, diagnoses and all specific services are covered under behavioral health. Two states over, only two kinds of treatments are available. Many current plans do not include chronic disease management and often exclude chronic care, so the patient needs to be vigilant about what is included in each category of EHB.

Caveat of Essential Health Benefits

Know your plan. You must remember that if you are in a self-funded plan, EHB are not applicable. Make sure to ask your employer if you have an employer based plan.

Remember that not all states are establishing state based Exchanges.

The presence of Exchanges most often means that current state insurance laws are covered in addition to the new requirements. This is advantageous if you are in a progressive state. Some states don't have certain kinds of emergency care. Most states don't cover chronic diseases. Make sure to check what your state covers in chronic disease management. For example if your state covers ten visits for acute cancer treatment, this would be a problem.

If your employer is self-funded and is not under state law, check your coverage with your employer. Find out if your employer is self-funded (not state-regulated) or fully-funded (state-regulated).

The Affordable Care Act allows a state to add Exchanges above essential health benefits. Some states are including some specific chronic diseases and not others. Your state could add provisions to protect people with chronic diseases. For example, if there are ten doctors in your state for your specific condition, all patients suffering from this disease could participate in the Exchange or be allowed to be cared outside your state. Exchanges will have the power to make the carriers do more for the patients.

Pre-Existing Conditions (PEC) and ACA Regulations

No child will be denied based on pre-existing conditions. Currently, too many are denied, and this exclusion has serious repercussions.

Starting in 2014, no one with PSC will be denied coverage, and no condition will be excluded from a policy.

There can be no medical underwriting. In other words, your medical risk will not play a role in making a decision about your insurance coverage.

Insurers MUST cover individuals with chronic conditions such as PSC, high blood pressure, diabetes, cancer, or other conditions. If you need to have frequent visits to your doctor, your premium will not be higher.

Similarly, gender rating is prohibited.

Insurers must spend 80-85% of premium on medical expenses.

Dependents under 26 years of age can stay on their parents' policy.

Lifetime limits are lifted. In 2014, annual limits will be phased out. For example, in Connecticut, $1000 is the amount allotted for ostomy supplies for a year. This amount will not get you very far. These limits are being removed.

You will have the right to make external appeals outside your insurance company.

Preventive Services will be available to you without co-pay or deductible (this excludes grandfathered plans and certain contraceptive coverage). These preventive services include birth control, mammograms, cervical cancer screenings, pelvic exams, well women exams, osteoperosis and colon cancer screenings, FDA approved contraceptive methods, breast feeding support, screenings related to interpersonal violence, children's screenings and vaccinations, medicare coverage for screenings and annual wellness exams. For further information, go to http://cciio.cms.gov/resources/files/prev-services-guidance-08152012.pdf.

Chronic Disease Management, a major focus of the ACA, is offered by using the following "techniques":

Establishing a treatment plan that involves regular monitoring; coordination of care between multiple providers and/or settings (this item includes housing and unemployment, and these are currently the subject of heated discussion); medication management by ensuring that the patient receives the right prescriptions and that someone is overseeing the patient's medical treatment); evidence-based care; measuring care quality and outcomes (we don't have this service, but it is under discussion); and support for the self-management of the patient through education or tools.

Provisions in the Affordable Care Act

At this point, there is no clear definition of chronic disease management but a requirement of reimbursement, forcing the insurance company to get involved. The Essential Health Benefits described above will of course be there for chronic disease patients as well.

The federal government is pushing hard for powerful reporting requirements to make sure that chronic disease management is properly implemented.

The Center for Medicare and Medicaid Innovation (Innovation Center) has a growing portfolio for testing various payment and service "delivery models" that aim to achieve better care for patients, better health for our communities, and lower costs through improvement of our health care system. The goal is to create these Innovation Centers and test innovation models.

The ACA requires the establishment of a grant program for medication management services to help pharmacists in implementing medication management services for the treatment of chronic diseases.

Another delivery model is called the *Extended Patient Navigator Program* that is subsidized by a grant and that will provide patient navigator services to assist patients in coordinating health care services for the diagnosis and treatment of chronic diseases. Victoria Veltri repeated over and over that patients must show extra vigilance at every level, and that patients will have the power to report and to advocate. Patients will need to determine and report how successful the Patient Navigator Program is.

Community-based prevention and wellness programs and self-management of chronic diseases will be evaluated. A plan has been developed for promoting healthy lifestyles and chronic disease self-management for Medicare beneficiaries. The federal government is pushing each state to think big and to move away from sickness-based medicine.

There will be new *educational funding for health care workers* who practice in the field of chronic care management: The ACA will create a grant program to provide new training opportunities for direct care workers in long-term care settings.

We're aging rapidly. Workers must agree to work in the field of geriatrics, disability services, long-term services and supports, or chronic care management for at least two years. The ACA also promotes the creation of a work force to provide direct assistance in communities, including management of chronic diseases.

Accountable Care Organizations provide coordinated care and chronic disease management while lowering costs; have the goal of avoiding unnecessary duplication of services and preventing medical errors; bring together providers to account for coordination; make payment contingent on caring for a panel of patients; can work across healthcare settings to improve outcomes; can work across payers; share risks with other providers to become accountable; and work towards the goal of providing a healthy population.

The Patient-Centered Medical Home (PCMH) is a model for care. Its mission is to strengthen the physician-patient relationship by replacing episodic care. The physician-led care team is responsible for coordinating all of the individual's health care needs and arranges for appropriate care with other qualified physicians and support services. The individual decides who is on the team, and the primary care physician makes sure that team members work together to meet the individual's needs in an integrated fashion. Patients are thus incentivized.

This model encompasses all the medical, behavioral health, and social supports and services needed by a beneficiary with chronic conditions. The problem is that the definition of chronic conditions is limited, and only fifteen of the most common chronic diseases such as asthma and diabetes are included. The goal is to expand the list and to understand how many people have these chronic conditions. Whether the number is going up or down, the size of the expenses incurred by a chronic disease. This will be a tool for transparency and will force insurance companies to be more accountable.

A state-by state list giving the incidence of a series of chronic conditions for Medicare recipients and developed to monitor the impact of the ACA on certain conditions is available at http://www.ccwdata.org/web/guest/interactive-data/chronic-conditions-dashboard. Unfortunately PSC is not one of the included conditions. As a patient you will be able and encouraged to influence the dashboard.

Healthcare Reform Issues can be broken into the following categories for discussion:

- Method of financing (federal, state, employer, self-pay)
- Method of insurance reimbursement (employer mandate, individual mandate, single-payer, universal healthcare)
- Method for delivering services (doctor, specialist, auxiliary and allied health, hospital, e.g., coordinated, integrated: Kaiser Permanente, Veterans Administration)
- Comprehensiveness of health insurance
- Cost and cost containment (competition, cost-sharing)
- Degree of patient choice
- Administrative costs

The anticipated reforms clearly require transformation beyond the Exchange.

When the patient comes in, the no wrong door policy will be at work. If a person works but has to stop work on and off, what will happen to those going in and out of coverage? What will happen for those who are between income brackets and cannot be covered? Though the federal government has a plan for these “in-between” people, it is ultimately up to the state to implement coverage. Will community providers have specialists? For accountability, providers, too, must be part of insurance plans. That is why activism will be very important. And how do we sustain these reforms? How do we deliver these services? Are we going to make primary care the major focus? How comprehensive will a patient’s care be? How will equitability be ensured? If there are five insurance companies, won’t it be those with high income that will have the bigger choices? How much profit can the insurers make? How do we make sure that no one is denied insurance? Many issues remain unanswered.

Chronic disease patients are in an altogether different position than those without a chronic condition. Our model is based on a “sick system.” In other words, we pay for care when people need it. We need to have chronic disease at the heart of the system. We need to address disparities between conditions, disparities based on race, gender, ethnicity, or age, in accessing health care. A registry will be an important tool for understanding issues. And we need to get away from the “sick system.” That means we need to get healthy people, those who never buy insurance, to be part of the pool, so that cost can go down. A major theme of the ACA is to include healthy people in the pool.

Q & A

Q What happens when a young person in his 20’s works part time and sporadically, and does not qualify for Medicaid?

A Coverage depends on the state and on the minimum hours the state considers to be full-time. If you work part time, you can work on and off. If your part time work places you under the Medicaid income level, you can either go on Medicaid or on your employer plan if your employer allows it. If you work six hours one week and ten on another, the average is taken. If you are eligible, you will be under Medicaid for at least thirty days unless your income changes. You can cycle up and down as necessary. If you are on Medicaid for the first two months, but then start to work longer hours, you can cycle up. Make sure to check what your state considers full-time.

Q If I live in Minnesota, can I be treated in Connecticut?

A If you are on Exchange, and if you are going on a trip, you will be covered. Emergency has to be covered, even when you travel out of the country. If it is a visit to your family, then it will be covered. If there is no provider in your state for your condition, then you will be covered because your state does not have coverage. It is important that you ask for authorization before. Otherwise you will be facing problems.

Q Do you have suggestions for surviving until 2014?

A The federal government cut off enrollment of pre-existing conditions very quietly. Hurry up and enroll before it goes away. If pre-existing condition plan has been removed, negotiate with your provider and be very aggressive. Collect bills, show your income, and show them you cannot survive this way. Call my office, and I will help you with information on “spending-down.”

Q Can an international non-resident student be covered?

A As long as you are documented and have legal status, you can buy a policy right now. Some carriers do not like to give it.

Q Will the ACA have Cobra coverage in 2014?

A Yes, Cobra coverage will be given when you lose your job or a dependent becomes independent. If you have a life status change as in turning 26 and becoming independent, Cobra kicks in. It is often for 18 months, though in Connecticut, it is for 30 months.

Q Can there still be life time limits?

A Yes, because self-funded plans have time limits. This is troubling because states are raising deductibles so that the huge increases can be grandfathered in 2014. If your plan is the same, then it continues. It will not be grandfathered if your condition changes. And if not grandfathered, lifetime rules will apply.

Q Michigan is raising deductibles now before 2014 arrives. Is it so the new rates become grandfathered?

A Yes! Many insurance companies are incurring huge increases so that they can be grandfathered. Large employers are less likely to have these limits. We run into problems with small business insurance plans but less with large business plans that are more likely to be stable. There are exceptions.

Q My employer is incorporated in Delaware. Company headquarters are in Massachussets, and I live in Georgia. Which state regulates the insurance?

A It depends. If it is a large employer plan and is regulated by the federal government, nothing changes. If you are in small business (100 employees), then it is where the insurance policy has been written. It is best to call the company to find out where it is located, and what laws govern the insurance company and the state.

Q Who determines the penalty if insurance is not purchased?

A The federal government imposes the requirement to get an insurance policy. If you don't, the penalty will be in your taxes. When you enroll, there will be a checkmark with your name sent to the US Treasury showing that you have enrolled. If you haven't, then you are assessed a penalty. Employers do not face such a requirement. Small businesses may not buy insurance and may leave employees to work with the Exchange.

Q How does ACA affect PSC?

A There aren't any specific provisions for PSC. Lifting pre-existing conditions, lifting limits, extension of Medicare and some of the grant programs discussed above will help PSCers. There will be many incentives for hospitals and doctors not to cut off care. For example, currently, people who are suicidal are asked to leave the hospital after given a treatment for their condition. The provisions from the ACA will be kicking in, and the patient will be kept in the hospital in such a case. Reporting will be very important for patient protection. Denials will be reported.

Community Alternatives Programs (CAP) help people who need long term care stay in their own homes. These help access care and offer great advantages. Encourage people to participate in these programs. These programs are run by federal grants. CAP will serve as brokers and advocate for you.

Q Will high-risk pools be wiped out?

A It depends on the state. In Connecticut, we got rid of high-risk pools. It is likely that most will go away and that Exchanges will replace them. On Exchange, you will be able to get better plans.

Q What agency will help me understand which policy will work best for me?

A It depends on what Exchange you have in your state. If you are in a state like Connecticut, we have a personal assistance program, or you can use a broker. The federal government has grants for an extensive community outreach program that goes beyond TV commercials. They will go to barbershops, where people are, to find trusted people in communities. These selected people will have background checks and will be trained in a certification program. They will have to become familiar with Medicaid and ACA Exchange. To decide what plan to choose, you can also go to the Navigator Program or use a broker. Again, it depends on the state. Connecticut has an interactive hotline.

Q Here, we get Medicare and sometimes Medicare and Medicaid when we lose our jobs. What will happen after 2014?

A If you are only on Medicare, you cannot go on Exchange. And Medicare rules are staying the same. The only changes are around the edges: They will be adding additional access to medical visits. If you are on Medicare disability, you can stay on it. Medicare will be adding more eligibility as well.

Q Is there a residency requirement when moving from an Exchange state to another state?

A You will need to re-enroll. If you are in a federally controlled Exchange, then you don't need to re-enroll. If you are on Medicaid, you will have gap coverage. Medicaid will give you at least thirty days until you meet their residency requirements.

Q I am a small employer watching insurance rates going up. I want to give good coverage to my employees. Is there an agency that can help me?

A In Massachussetts, call Health Law Advocates. Your concern is the individual employees. The Exchange for small businesses will lower the cost of insurance to employees.

Q You mentioned that there eighteen states that will not participate. What if we are in one of these states?

A The Exchange segment will protect you. The Medicaid segment is what scares me because Medicaid is a state-federal partnership, and it is up to each of these states to make decisions. Medicaid is optional, so states may get rid of the program. Medicare will continue. Subsidies will be offered to individuals. You must work with CAP in your state.

Q How will we know what effect the ACA will have on life expectancy?

A There is a streamlined application capturing race, ethnicity, gender and language. The Exchange and carriers will have the data. This information is part of the eligibility data that every carriers holds. These demographics will be reported to the federal government. This data will be studied against the Health Equity Index to understand who is involved, how many are enrolled, what experience they are having, and if the health of the community is improving.

Victora Veltri very generously offered to answer questions by contacting her directly at
Office of the Healthcare Advocate
860-331-2441; Victoria.veltri@ct.gov

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See you in Denver next year!

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.

PSC PARTNERS
SEEKING A CURE

PSC Partners Seeking a Cure is a 501(c)3 nonprofit foundation that is working towards finding a cure for primary sclerosing cholangitis (PSC).

The mission of PSC Partners is to provide education and support to PSC patients, families and caregivers and to raise funds to research causes, treatments and potential cures for primary sclerosing cholangitis.

Ricky Safer, CEO and founder, is the principal contact person for the PSC Partners Seeking a Cure Foundation. Reach her at: contactus@pscpartners.org

To make a tax-deductible donation, please click on www.pscpartners.org/waystodonate.

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[Back to Contents](#)