

# Primary Biliary Cholangitis (PBC)

## From the Patient's Perspective

### Webisode 3: PBC Treatment: A Shared Decision



## CMEO Podcast Transcript

**Carolyn Legaspi, NP:**

What kind of treatment did they offer to you when you were diagnosed with primary biliary cholangitis (PBC)?

**Tiffany Brown:**

The gastroenterologist (GI) immediately told me that I would probably be on urso for the rest of my life, as long as I was a responder. And so, we knew that coming out of the gate. So, that was my first line of medication, and it still is. I'm still on urso today.

**Carolyn Legaspi, NP:**

Okay. So, you were taking ursodiol, and then did they tell you how long you would be on the medication before you started to see an improvement, or maybe when they would recheck your labs to see if you were a responder?

**Tiffany Brown:**

Because of my Fibroscan results and my blood work, we chose to do blood work. I think I did it every 2 months out the gate. I saw him, ... I did Fibroscan in December, maybe, I saw him in late November and I did Fibroscan in December of 2018. And then January we were doing blood work again, and I am, it was pretty consecutive every 2 to 3 months.

**Carolyn Legaspi, NP:**

And did you feel like, did your numbers improve on the ursodiol?

**Tiffany Brown:**

Yes, I did.

**Carolyn Legaspi, NP:**

Okay. All right. I got you. Now, so they treated the PBC with ursodiol. Symptomatically, it sounded like you're having some pruritus. Did they address the pruritus with any medications, or how did you manage that?

**Tiffany Brown:**

I don't think we've ever talked of addressing the itch with any medications. The GI specialist felt that was something that would come and go, and he felt like the more we got my enzymes down, and I also changed my diet. I did, now I am, I hate using the term, but vegan. I don't eat meat. I watch what breads I eat. So, I began to make changes on my own to try to help some of that too.

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**Carolyn Legaspi, NP:**

Sure. So, you made some lifestyle modifications to help see if that route would help with your symptoms.

**Tiffany Brown:**

Yes.

**Carolyn Legaspi, NP:**

And it sounds like you responded to the ursodiol. Do you know if your liver enzymes normalize just on the ursodiol?

**Tiffany Brown:**

No. Well, they came very close, and then they started to elevate again. And the new hepatologist that I had at Piedmont, one of the partners in the group, he said, "Anything over 200, and we're going to look at different medications." So, one summer he was doing blood work on me pretty frequently and they got over 200. Actually, I think they came near 300 or maybe a little over 300. And he said, "We're going to have to take another defense in this. I want you to stay on urso." But I did fenofibrate.

**Carolyn Legaspi, NP:**

Okay. So, you were saying ...?

**Tiffany Brown:**

Did I respond to it?

**Carolyn Legaspi, NP:**

No. Sorry, you started up fenofibrate, and did that help? Yeah, I guess did that help your liver enzymes?

**Tiffany Brown:**

It did help, it brought them... I will tell you the truth, it brought them normal, and maybe a little bit lower than normal. But I had what was considered hypoglycemia after I started fenofibrate, and they had to look at my kidney function all the time. And so, once they got normal or a little lower, the hepatologist and I discussed it and he said, "Okay, we can end that."

**Carolyn Legaspi, NP:**

Okay. All right. And then after stopping the fenofibrate, did your enzymes stay stable or near normal?

**Tiffany Brown:**

They stayed near normal. We, at my last appointment with him, which was in July, should have another one coming up very soon, we discussed the new medication, elafibranor.

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**Carolyn Legaspi, NP:**

Elafibranor.

**Tiffany Brown:**

And that's one of the things that we probably will add after the first of the year, probably should maybe have thought of adding it before now, but ...

**Carolyn Legaspi, NP:**

Sure. Yeah, I think it's kind of exciting at this time, well, exciting for us providers because we have more options to treat PBC with. Just this year we have two new U.S. Food and Drug Administration (FDA)-authorized medications to treat PBC. Classically we only had ursodiol that we could rely on, and some patients are non-responders. You're lucky in that you responded, that's actually quite miraculous for your alkaline and phosphatase to be a thousand, and then near normal just on ursodiol itself.

**Tiffany Brown:**

Yes.

**Carolyn Legaspi, NP:**

And I'm just thinking about your case, and when you have a patient who has, who like yourself has advanced scar tissue, you really want to be quite aggressive in normalizing that alkaline phosphatase because we know that that is what prevents you from having cirrhosis or developing scar tissue.

**Tiffany Brown:**

Yes.

**Carolyn Legaspi, NP:**

And that's the goal of treatment always is to normalize that alkaline phosphatase. So, we had ursodiol, and then we had obeticholic acid (Ocaliva), which is another medication. But unfortunately some patients who suffer from pruritus, that can exacerbate the pruritus. So, if they have a lot of itching, we are a little wary of starting that medication on patients. Fenofibrate is another option, but it's actually not FDA-authorized for PBC, right?

**Tiffany Brown:**

Yes.

**Carolyn Legaspi, NP:**

So, we use it because we know it works, we don't have options, and some people do respond to that. But it's a nice a year where we have two new FDA-authorized medications, elafibranor is one that your doctor discussed with you. I think it was the one that was first FDA-authorized this year, and then seladelpar also.

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**Tiffany Brown:**

Yes. Yeah.

**Carolyn Legaspi, NP:**

And it's nice because those medications may help patients who suffer from pruritus, along with helping the PBC alkaline phosphatase normalize. So, I think even if your doctors are correct, if your alkaline phosphatase is beyond 200, they have tools now in the shed that they can provide to you to help really keep that normalized, because they want to keep your liver intact for many decades to come.

**Tiffany Brown:**

As do I.

**Carolyn Legaspi, NP:**

Yeah. The other thing that we wanted to touch on, the shared decision making, I think that's with any care that you provide to your patients. All of the things that your doctors are telling you are recommendations, right?

**Tiffany Brown:**

Yes.

**Carolyn Legaspi, NP:**

Because these are recommendations based off of guidelines, based off of their own experience dealing with a disease process and their patients' experiences. But everybody's life is different. Everybody has different needs, different occupations, different family circumstances, different financial circumstances.

So, I think that it's always a shared decision, if my patient is very adamant about not proceeding with a medication, an intervention, a recommendation, we talk about the risk and benefit, and my role is to provide that information. And if they don't want to move forward, then we don't move forward, and we hash out another plan of care.

So, I think that that is how I'd go about it. I never have any hurt feelings if a patient doesn't want to do something that I recommend because they're the ones that are doing it.

**Tiffany Brown:**

Yes.

**Carolyn Legaspi, NP:**

They're the ones that have to live that. Right? Tiffany, you're the one that has to execute that.

**Tiffany Brown:**

Absolutely.

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**Carolyn Legaspi, NP:**

You're the one that has to go in for that magnetic resonance imaging (MRI). You're the one that has to go in and take that medication and deal with potential side effects. But my role is really to educate about the potential benefits and the potential risks with ...

**Tiffany Brown:**

Yes.

**Carolyn Legaspi, NP:**

... everything we recommend. And to make it clear and educate. The one thing I love about being a nurse practitioner is I am able to educate, educate, educate patients about disease processes, about medications. And then I would not feel comfortable pressuring somebody. If they say, "I'm uncomfortable, I want to research this on my own." Please do. Please do, so that we're all going into this eyes wide, right? So, I don't know, Tiffany, how do you feel about shared decision making with your providers? I think we touched on it a little bit.

**Tiffany Brown:**

I highly agree with it. I certainly do. The doctors that I have right now, I say I have some of the best doctors that I can have right now, and we talk things over, whether it's through a telehealth appointment or in an office appointment, we talk things over. And there've been some things that I was just, I don't want to do that.

**Carolyn Legaspi, NP:**

Right!

**Tiffany Brown:**

There've been some things that I was apprehensive about doing that eventually I did. My primary care provider (PC) suggested antidepressants and I did not want to take antidepressants.

And I bucked her for a long time, and finally it was just one of those things that I gave into. But she was patient with me like you're talking about being with your patients. She didn't pressure me on it. She didn't ... when the time came that I was ready to sit down and talk to her about it, she was open. She didn't say, "I told you so," or anything like that. And so, I think understanding and empathy on behalf of both patient and doctor. We have a relationship, and I think relationships, they grow as we seek to understand one another.

**Carolyn Legaspi, NP:**

Absolutely. You have to ... sometimes it's difficult in that first visit to say, "This is what you need to do, this is what you need to do, this is what you need to do?" Because there's no trust there yet.

**Tiffany Brown:**

Right.

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#### **Carolyn Legaspi, NP:**

Like you said, it is a relationship. So, it may take several visits, it may take several interactions, it may never happen.

#### **Tiffany Brown:**

That's right.

#### **Carolyn Legaspi, NP:**

And that's when you seek other care if that trust is broken down. So, I think it takes some time to build that trust. But shared decision-making is so important, and it's something that actually we don't think about, but is so important when it comes to providing care for patients.

So, I do encourage my patients to participate in clinical trials. A lot of the PBC medications that came through FDA authorization this year were all enrolled and possible because patients who have PBC participated in clinical trials.

And a lot of the times I have patients that actually seek clinical trials. They're coming to Cedars-Sinai because traditional medications or the first-line and second-line treatment haven't actually normalized their alkaline phosphatase, and they're seeking that next level of potential, what's in the pipeline care.

So, I think it's important actually that patients participate in clinical trials. We run a lot of clinical trials and academic centers. Obviously you want to go through your trusted doctor, and then if your doctor says, "This is a good trial for you," then I would listen to that. So ... and I think that a lot of clinical trials go through big processes to get approved in order to move forward. So, the risks are really put forth for you and the benefits are put forth for you, and you have to weigh those things as a participant. But I always encourage my patients to participate in clinical trials.

#### **Aliya Gulamhusein, MD, MPH:**

So... so, you were treated pretty quickly after being diagnosed, and take us through a little bit about the peritransplant period, when you recognized and you and your physician team recognized that you were manifesting these symptoms of advanced liver disease, the ascites you talk about, the varices, the encephalopathy. When did the decision about transplant come, and what did that look like in your journey?

#### **Maria Morais, RN:**

So, the decision for transplant. So, I pretty much spent maybe a year and a few months dealing with severe symptoms, as I mentioned. And having paracentesis was the thing that kept me connected with the healthcare system on a weekly basis. So, I was very well-connected with my hepatologist that during my hospital visits every Friday morning for 3, 4 hours, I had a quick visit during procedure, I had a consultation with my hepatologist who would administer the procedure and drained, or an insertion of a needle to have those hard conversations of the need for transplant, or, "Let's see how you're doing for next week." Making sure that my diet was well under control in terms of low to no sodium diet, to control the ascites, to make myself feel better and not as fluid-filled every week.

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But it didn't matter what I did. So, my Model for End-Stage Liver Disease (MELD) score started to be a factor in calculating my risk of end-stage liver and cirrhosis. So, that was well monitored. So, I had lots of blood work regularly, almost every other week, certainly during paracentesis, there would be additional testing done just to really monitor where I was at and how that was relating to the symptoms that were progressing. So, I'd say that that care was really intense, and conversations were more supportive of, "I think we need to investigate, at least go for evaluation for pre-transplant."

So, that was December of 2020, which was not ideal during COVID, of course, with not being supported by a family member or a friend to be able to go to hospital, if we all recall the long lineups for screening, even to get to an appointment. So, I was fatigued, I had a big, big belly. I was ... I had to drive myself there and back. It was just, it started to be routine for me, and I actually look forward to going because I felt such a relief after being drained of 10 liters every week. And I had a lot more to be taken off, but of course there are limitations so that my body wouldn't go into some sort of shock or just not healthy for me to take more off. Yeah.

#### **Aliya Gulamhusein, MD, MPH:**

It's incredible. And then what happened? How did you feel after transplant, when that day came where you were called in for a liver transplant, or you had a living donor you mentioned.

#### **Maria Morais, RN:**

Yes. Yeah.

#### **Aliya Gulamhusein, MD, MPH:**

Can you talk a little bit about that, so what's sort of the patient level understanding about organ availability and allocation, and living donors, deceased donors, waiting lists, all those sorts of things?

#### **Maria Morais, RN:**

Yeah, it's a world in and of itself, right? The pre-transplant, there are a lot of tests. They're not difficult tests to complete. They're like echocardiograms, ultrasound, lots of blood work, and discussions with a psychologist, sociology, anesthesiologist. The transplant team at University Health Network (UHN) was very, very helpful and all very supportive, and all had the same message for me. And I did get approved. So, I was referred December of 2020 to the program. I had my referral within 2 weeks acknowledged, and by March of 2021 I was approved to be on the transplant list, the deceased list.

And so, I continued. Then I got transferred for sort of pre-transplant care at UHN, while still seeing my hepatologist locally in Brampton, Ontario, where I would still receive my paracentesis and PBC care, let's call it. But then I received an honest call from a hepatologist at UHN to say, although I was sick, I probably would not be receiving a deceased donor for a while, to think about living donor.

And I had never really considered that. I was still very much in shock that a transplant was in my future, imminent future, that I needed one, that I was this ill. And then to think about who would give me a liver, right? How do you ask somebody for a liver? And then I started learning about that a little bit more connected with the Center for Living Organ Donation Program at UHN, who now I sit on their advisory committee as a patient

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advocate, which is a wonderful program, and learned about that and learned how to ask and put out a social media campaign, although I'm pretty private.

And I sort of for weeks said, "I can't do this. I can't ask people." I knew that my immediate family was not a match, so I had to go outside of my family. I was inspired by a young gentleman who's 23 looking for a kidney, and he did an Instagram campaign and I thought, "Well, with my public health experience on social campaigns, I think I can do this. I think I have to do this." So, I pivoted. It became more of a survival thing than a pride thing for me. And I put out a Liver for Life campaign for Maria, and was amazed, was amazed at the response, that humanity is still very much alive in our society. It's so wonderful to feel and see.

I had six people register to be donors. I had strangers, acquaintances and friends, co-workers, and of course the first person was a good friend of mine. In my immediate mom's group, were a group of 10 moms that have seen our children grow up. So, we all have 15 or 16-year-olds right now. And just that support, that immediate support. But she didn't tell me at first because she didn't want to disappoint me if she wasn't evaluated to be my donor. So, good news came, and that same year that I was on the list, I got transplanted, and we're both, she's wonderful and recovered. No issue. Of course, her liver regenerated, which is a fact that a lot of people don't know, and she's fine. And mine is doing wonderfully as well. Very grateful.

#### **Aliya Gulamhusein, MD, MPH:**

Yeah, it's a true gift. I think about this all the time in the clinic. You think that this is a real second chance at life, and it's a really incredible intervention, and unique for the patient with PBC compared to maybe other causes of liver disease, insofar as that MELD score that you talked about is a good score, not a perfect score. It doesn't serve some etiologies of liver disease like PBC maybe as well as it could. And patients with these manifestations of liver dysfunction and portal hypertension like you talked about ascites, varices, don't often get MELD points because those aren't accounted for on the MELD score.

#### **Maria Morais, RN:**

Right. Yeah.

#### **Aliya Gulamhusein, MD, MPH:**

So, living donation is something that a fair proportion of our patients have to think about and confront. But all these other issues about how do you ask and who do you ask, and what does that even look like? And the variables that are involved, being a part of a center who does a lot of living donation, there are patients that are part of regions where living donation doesn't happen as frequently.

So, while you are navigating all these complexities of liver disease in your lived experience as a healthcare practitioner, in a way a lot of things lined up, in a sort of beautiful, interesting way. So, thank you for sharing that. Now I want to just ask, physically and emotionally or mentally, how did you feel after the transplant dust had settled? I mean, not day one or day two, but after the dust had settled, how did you feel symptomatically? Psychologically?



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#### **Maria Morais, RN:**

Yeah, I think it's ... I was very nervous about the surgery, but then maybe a week before I just sort of calmed and this felt really right, so ... and I wanted to live. I wanted to live well. I wanted to have a second chance. I wanted to have a new life that is sort of a new a hundred percent, and that's what I'm living now and trying to do my best, live well, appreciate others in the small things. And it's only, you hear it all the time, that second chance, that do-over.

Immediately after transplant, I can recall my husband saying that my skin, my color was really good. I was no longer yellow. It's immediate. The fatigue, I think it was a different fatigue, a drugged fatigue, like medications, obviously. I just felt different. I felt like that PBC fog was just gone. The ascites took its time to go, but that was deemed normal. Psychologically, I just had this very grateful feeling of making it.

It was a very significant surgery. It's abdominal surgery. Even on your average abs surgery, there's a big recovery involved. And so, I knew I was going in for something like that. But then it was layers deep, it was like a new liver taking on what psychologically that meant. And I just focused mentally on growing that liver. And I called her Lola, Lola the liver. And just trying to be a little bit more spiritual and focusing on growing Lola, and making her adhere to all my vessels, and just energy moving, really.

And just trying to keep very positive, despite some hiccups. And that can happen in any post-surgery. And just being well-supported. But psychologically just being very grateful, having this sense of giving back. And that is one of the major forces that inspire me to be a peer PBC advocate and participate in the Canadian PBC Society. Post transplant, my year one was really physical healing. I also needed to be mentally strong.

And then towards the end of year two, into year three, which I just celebrated, it's much more about going outward into community, and the PBC community, and working with researchers, clinicians, attending major conferences and industry and trying to just tell my story, share my story so that it can impact on finding that cure, keeping research alive, inspiring my story so that the next PBC patient doesn't have to go through everything I did, and that we can live well with PBC.

#### **Aliya Gulamhusein, MD, MPH:**

Wonderful. You keep sort of segueing into things that I really want to ask anyway. So, you mentioned the place you're at right now is in a place of outreach almost, education and outreach and learning at meetings and such. And you'll know that the world of PBC has changed a ton in the last 5 years, 10 years, just with approaches to understanding disease risk and treatments available for patients. So, tell us, what's the pulse of patients on the treatment landscape for PBC, within the Canadian PBC Society and other sort of patient organizations that you've interacted with, what's people's understanding of medical management of PBC in 2024, because that's so different to just urso. Right? As you ...

#### **Maria Morais, RN:**

Yeah. I think for many PBC patients that were diagnosed many years ago, because the Canadian PBC Society has celebrated their 20th anniversary a few years ago, 2 years ago perhaps. And so, there are patients that were placed on urso, and that there's so many other options since then. And from what I understand, and hear part of these conferences that I've attended is that there's so many other options being studied, and treatments that

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just need to go through their phases, and then approval. So, it's hopeful for patients, and many PBC patients are on peer groups like Facebook, where information is shared, and internationally.

So, we're in Canada, and in other countries there may be treatments that have been approved. So, there's an excitement about when are other treatments coming to Canada. So, knowing that worldwide there are many efforts and many pharmaceutical companies look invested in PBC, is very inspiring for PBC patients. Now knowing that is one thing, but having immediate access right now is another thing. So, I think that gap we have to work hard and support and tell our unmet needs in the areas that we live in so that we can have that access to treatments or treatment options, right?

#### **Aliya Gulamhusein, MD, MPH:**

Yeah, absolutely. I mean, there's a lot of work that has been done. There's still a ton to do. And maybe I'll sort of start to conclude by asking two things. Number one, what do you still feel like are the unmet needs in PBC for a patient? And number two, what advice do you have for patients living with PBC today?

#### **Maria Morais, RN:**

Yeah. So, the unmet needs still are very much dealing with the fatigue, because the fatigue just sort of cycles, other symptoms cycle with that. So, if we're not rested, if we're not dealing with our fatigue, then we can't be effective in our daily living. So, fatigue. The itching, there are some medications available, but they're not wholeheartedly effective for all. So, there's still a gap in that. And so, PBC patients that suffer from itch, it impacts their fatigue. It's a vicious cycle.

Advice for patients would be to know yourself, know when you are experiencing a new symptom, what kind of symptom management that you've tried, and really bringing it forward to your physician, to hopefully you have access to a hepatologist, gastroenterologist, someone that understands PBC, someone that understands and is knowledgeable about the treatment options that are currently available.

And inspiring hope for other treatments that might be coming down the pipe, and just to take that time to go beyond the blood values and the blood work and the alkaline phosphatase (ALP), and treat that person holistically. So, I would say that as a nurse, but I would also say that as a patient, wanting that, to ask about how I'm doing, not, "How's your blood work?" Because the blood work may not be reflective of how I'm actually doing or how a PBC patient is doing.

And I think just to take that time to listen, to validate, and there may be nothing that the healthcare provider can do in that moment to solve that itch, to solve the fatigue, but I think it makes patients feel better when they're able to have the time to be heard, to have their questions, that they ideally would prepare ahead of time into that appointment.

I know physicians are very, very busy with the healthcare system that we're experiencing, but perhaps there are other supports in a clinic, maybe a nurse practitioner, maybe other folks that are supportive, maybe a fellow that can take a little bit more time, if that's the kind of clinic, a specialized clinic that patients attend to. If it's a community-based hepatologist, they're few and far between as well. So, they have big patient lists. But for the patient just to push through and be your own advocate.