



Making Our Voices Heard: Externally-Led Patient-Focused Drug Development (PFDD) Forum October 23, 2020, 10 AM – 4 PM ET

AGENDA

10:00 AM Welcome and Introductions – (0:00)

Veronica Miller, PhD, *Executive Director, Forum for Collaborative Research and Professor (Adjunct), UC Berkeley*

Mary Vyas, *President, PSC Partners Seeking a Cure, Canada*

Ricky Safer, *Founder and CEO, PSC Partners Seeking a Cure*

10:12 AM Overview and Objectives of the Patient-Focused Drug Development Program (14:20)

Rub Mehta, MD, *Medical Officer, FDA-CDER-OND-OII-Division of Hepatology and Nutrition*

10:20 AM Natural History, Clinical Management and Treatment of PSC (22:00)

Christopher L. Bowlus, MD, *Lena Valente Professor and Chief, Division of Gastroenterology and Hepatology, University of California at Davis*

10:35 AM HOW DO PSC CLINICAL SYMPTOMS AFFECT MY QUALITY OF LIFE? (33:21)

- **Patient Perspective on Key Clinical Symptoms – Patient Testimonials:**

Pain: Afsana (35:19)

Pruritus: Tim (39:46)

Fatigue: Jessica (44:03)

Impaired cognitive function: Elizabeth (48:09)

Living with co-morbidities: Dan (52:11)

- **Overview and Relevant Results of PFDD Survey and Polling Instructions/Questions**

Joanne Hatchett, RN, MS, FNP, ACHPN, *Medical Science Liaison, PSC Partners*(56:05)

- **Open Forum Q & A – Zoom Panelists (1:08:42)**

Elizabeth, Jerome, Tawny, Joe

11:50 AM WHAT ARE THE CONSEQUENCES OF THE LACK OF EFFECTIVE TREATMENTS FOR MY PSC? (1:53:25)

- **Patient Perspective on Advanced Disease – Patient Testimonials:**

Cholangiocarcinoma: Kevin (1:53:32)

Cirrhosis and pre-transplant: Kristian (1:57:35)

Post-transplant and recurrent PSC: Todd (2:01:08)



Making Our Voices Heard: Externally-Led Patient-Focused Drug Development (PFDD) Forum

October 23, 2020 10:00 AM-4 PM ET

- **Relevant Results of PFDD Survey and Polling Questions (2:04:58)**
Joanne Hatchett, RN, MS, FNP, ACHPN, *Medical Science Liaison, PSC Partners*
- **Open Forum Q & A – Zoom Panelists (2:14:58)**
Alison, Niall, Monika, Nicola

12:45 PM LUNCH and view community images and artwork (2:47:03)

1:15 PM WHAT ARE THE UNIQUE ISSUES FACING PEDIATRIC PSC PATIENTS AND THEIR CAREGIVERS? (3:17:12)

Natural History, Clinical Management and Treatment of PSC for Pediatric and Adolescent Patients (3:18:13)

Jorge Bezerra, MD, FAASLD, *President, American Association for the Study of Liver Diseases; Professor of Pediatrics, Cincinnati Children's Medical Center*

- **Pediatric and Adolescent Patient Perspective - Patient and Caregiver Testimonials:**
Living with PSC: Bek (3:32:26)
Parent concerns: Alex (3:35:10)
Living donor pediatric transplant: Richard (3:39:06)
Pediatric transplant journey: Jennifer (3:43:16)
- **Relevant Results of PFDD Survey and Polling Questions (3:47:36)**
Meegan Carey, MBA, *Executive Director, PSC Partners Seeking a Cure*
- **Open Forum Q & A – Zoom Panelists (3:57:05)**
Chelsea, Afsana, Willie, Richard

2:20 PM HOW CAN THE CURRENT TREATMENTS OF PSC BE IMPROVED AND MADE MORE TOLERABLE FOR ME? (4:25:43)

- **Patient Perspective on PSC Treatment and Related Conditions – Patient Testimonials:**
Treatment of pruritus: Kristina (4:26:15)
Management of other key symptoms: Lindsey (4:30:02)
Diagnosis and treatment of acute cholangitis: Laura (4:34:23)
Management and treatment of IBD in PSC patients: John (4:38:30)
Use of off-label therapies: Jay (4:42:48)
- **Relevant Results of PFDD Survey and Polling Questions (4:47:05)**
James M. McMurtry, MD, FACS, *Board Member, PSC Partners Seeking a Cure*
- **Open Forum Q & A – Zoom Panelists (4:54:26)**
Kerrie, Lalit, Fred, Rachel



Making Our Voices Heard: Externally-Led Patient-Focused Drug Development (PFDD) Forum

October 23, 2020 10:00 AM-4 PM ET

3:20 PM HOW CAN THE PATIENT PERSPECTIVE BE INCORPORATED INTO THE CLINICAL DEVELOPMENT OF PSC TREATMENTS? (5:21:01)

Patient Testimonials

- **Special Considerations for Clinical Trials in Pediatric and Adolescent Patients**
Lisa Pedicone, *PhD, PSC Parent* (5:21:20)
- **Understanding the Patient Experience and Clinical Trials – Lessons Learned**
Nathan Baggett, *MD, PSC Post-transplant Patient* (5:26:05)
- **Relevant Results of PFDD Survey and Polling Questions** (5:32:00)
James M. McMurtry, *MD, FACS, Board Member, PSC Partners Seeking a Cure*
- **Strategies to Incorporate the Patient Voice into Clinical Drug Development**
Ricky Safer, *Founder and CEO, PSC Partners Seeking a Cure* (5:41:47)

3:54 PM Summary and Wrap-Up (5:56:39) Veronica Miller, PhD, *Executive Director, Forum for Collaborative Research and Professor (Adjunct), UC Berkeley*

Making Our Voices Heard: PFDD COMMUNITY SPONSORS

	<p><u>Platinum</u> James F. Collins Trust The Kenneth S. Hollander Charitable Foundation David and Rosalind Parry Family</p>	
	<p><u>Gold</u> Matt and Cindy McMurtry Family</p> <p><u>Silver</u> Anonymous John and Chris Browner Family Todd and Erin Litton Family Fred Sabernick Family Don and Ricky Safer Family</p>	
	<p><u>Bronze</u> Anonymous Steve and Joanne Hatchett Family Ken and Patty Shepherd Family</p> <p><u>Copper</u> Stuart and Debra Barnett Family</p> <p><u>Titanium</u> Jeff and Reggie Belmont Family Willie and Raquel McKinney Family</p>	

Thanks to these Sponsors, the PSC Partners Community is
Making Our Voices Heard