



## **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**

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**

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**

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?**

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

Pain: Afsana

Pruritus: Tim

Fatigue: Jessica

Impaired cognitive function: Elizabeth

Living with co-morbidities: Dan

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

Joanne Hatchett, RN, MS, FNP, ACHPN, *Medical Science Liaison, PSC Partners*

- **Open Forum Q & A – Zoom Panelists**

Elizabeth, Jerome, Tawny, Joe

#### **11:50 AM WHAT ARE THE CONSEQUENCES OF THE LACK OF EFFECTIVE TREATMENTS FOR MY PSC?**

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

Cholangiocarcinoma: Kevin

Cirrhosis and pre-transplant: Kristian

Post-transplant and recurrent PSC: Todd



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

**12:45 PM LUNCH and view community images and artwork**

**1:15 PM WHAT ARE THE UNIQUE ISSUES FACING PEDIATRIC PSC PATIENTS AND THEIR CAREGIVERS?**

## **Natural History, Clinical Management and Treatment of PSC for Pediatric and Adolescent Patients**

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  
Parent concerns: Alex  
Living donor pediatric transplant: Richard  
Pediatric transplant journey: Jennifer
- **Relevant Results of PFDD Survey and Polling Questions**  
Meegan Carey, MBA, *Executive Director, PSC Partners Seeking a Cure*
- **Open Forum Q & A – Zoom Panelists**  
Chelsea, Afsana, Willie, Richard

**2:20 PM HOW CAN THE CURRENT TREATMENTS OF PSC BE IMPROVED AND MADE MORE TOLERABLE FOR ME?**

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



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## 3:20 PM HOW CAN THE PATIENT PERSPECTIVE BE INCORPORATED INTO THE CLINICAL DEVELOPMENT OF PSC TREATMENTS?

### Patient Testimonials

- **Special Considerations for Clinical Trials in Pediatric and Adolescent Patients**  
Lisa Pedicone, *PhD, PSC Parent*
- **Understanding the Patient Experience and Clinical Trials – Lessons Learned**  
Nathan Baggett, *MD, PSC Post-transplant Patient*
- **Relevant Results of PFDD Survey and Polling Questions**  
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*

3:54 PM

### Summary and Wrap-Up

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

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