## 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)
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
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