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