Pediatric Cholestatic Disease Working Group- Introductory Call

Friday, March 19, 2021
Welcome & Introduction

Jessica Weber, MSc, BSN
Forum for Collaborative Research
## Agenda

<table>
<thead>
<tr>
<th>Time</th>
<th>Event</th>
<th>Participants</th>
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<tbody>
<tr>
<td>12:00 PM</td>
<td>Begin</td>
<td>Veronica Miller, Forum for Collaborative Research</td>
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<td>Jessica Weber, Forum for Collaborative Research</td>
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<td></td>
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<td>Saul Karpen, Emory University School of Medicine/ Children’s Healthcare of Atlanta</td>
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<td>Henkjan Verkade, University of Groningen</td>
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<tr>
<td>12:00 PM</td>
<td>Welcome and Meeting Goals</td>
<td>Pam Vig, Mirum Pharma</td>
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<td>12:20 PM</td>
<td>Stakeholder Perspectives on</td>
<td>Roberta Smith, Alagille Syndrome Alliance</td>
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<td>Demonstrating Efficacy and Safety in</td>
<td>Ruby Mehta, U.S. Food and Drug Administration</td>
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<td>Clinical Trials</td>
<td>Chrissi Pallidis, European Medicines Agency</td>
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<td>Pam Vig, Mirum Pharma</td>
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<td>1:00 PM</td>
<td>Next Steps</td>
<td>Jessica Weber, Forum for Collaborative Research</td>
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<td></td>
<td>All</td>
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<tr>
<td>1:10 PM</td>
<td>Discussion</td>
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<td>1:30 PM ET</td>
<td>Adjourn</td>
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How to Participate

- **Q&A**
  - Submit questions for one or more panelists to answer
  - Allows panelists to respond directly, or Moderator to pose the question
  - Attendees may also reply to, or “like” questions

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How to Participate

- “Raise Hand”
  - To be unmuted, use this function which will allow the Host to unmute your line to make a comment or ask a question.
Rules of the [Zoom] Room

- Presentations, discussions, comments, and questions are not for attribution
  - Participants speak as individuals and express views that may not represent those of their organizations

- Participation Reminder (Forum Operating Principles)
  - We restrict participation to experts with the necessary scientific knowledge from organizations or entities with a clear commitment to advancing the diagnostic and therapeutic field of the disease area of the project they participate in, whether they be from government, academia, industry or community. Only in this way can we achieve the effectiveness and productivity needed to add value to the field.
  - The Forum is not a venue for marketing and/or investment experts.
Forum for Collaborative Research

- The Forum for Collaborative Research (The Forum)- part of the University of California Berkeley School of Public Health- is a public/private partnership that addresses cutting edge science and policy issues through a process of stakeholder engagement.

- The hallmark of the Forum is inclusion of all stakeholder groups, including patient and advocacy organizations, academia, federal agencies, industry, professional societies and other relevant entities.
Forum Perspective

▪ “Once new drug candidates and therapeutic strategies are identified, their efficient, safe development is in the best interest of all stakeholders, most of all, the patients”
Operating Principles

- Independent and neutral venue
  - Ongoing multi-stakeholder dialogue
  - Each stakeholder group has an equal voice

- Facilitate best science-based decisions, in real time
  - Break down inefficiencies and redundancy
  - Increase clarity, collaboration and innovation
  - Benefit to whole field, especially patients
PSC Forum Mission

- To facilitate evolving consensus, based on real time scientific development in the areas of appropriate methodologies for novel therapeutic approaches, clinical trial design, and harmonization across the PSC community.

Goals:

- To identify and address gaps and barriers in regulatory science to advance the development of safe and effective treatment of PSC
- To provide an independent and neutral environment for ongoing multi-stakeholder dialogue
- To provide scientific guidance to facilitate discussion between industry, regulatory bodies, the academic and patients’ communities in the areas of PSC drug development
PSC Forum Steering Committee

Co-Chairs
- Kris Kowdley
  Swedish Medical Center
- Rob Myers
  Gilead
- Veronica Miller
  Forum for Collaborative Research

Regulatory
- Lara Dimick
  FDA/CDER/DGIEP
- Ricky Safer
  PSC Partners Seeking a Cure
- Rachel Gomel
  PSC Partners Seeking a Cure Canada
- Martine Walmsley
  PSC Support

Academia
- Christopher Bowlus
  UC Davis Medical Center
- Gideon Hirschfield
  University of Toronto
- Tamir Miloh
  University of Miami
- Arun Sanyal
  Virginia Commonwealth University

Patient Advocates
- Ricky Safer
  PSC Partners Seeking a Cure

Industry
- Stephen Rossi
  Industry Expert/ Consultant
- David Shapiro
  Industry Expert/ Consultant
- Stanislav Stoyanov
  Intercept Pharmaceuticals
Working Groups:

- Pediatric Cholestatic Disease
- PSC Biomarkers

Closed Working Groups:

- Inclusion/Exclusion Criteria Working Group
- PSC PRO Working Group
- PSC Patient Databases Working Group
Thank You to our Sponsors

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Thank you!

Contact Information:
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