PSC Forum 3

Jessica Weber, MSc, BSN

Forum for Collaborative Research
Agenda

8:00 AM  Registration & Light Breakfast

8:30 AM  Session I: PSC Forum Updates

Moderator: Veronica Miller, Forum for Collaborative Research

8:30 AM  PSC Forum Project Updates
Jessica Weber, Forum for Collaborative Research

Introductory Remarks
PSC Forum Co-Chairs

Veronica Miller, Forum for Collaborative Research
Rob Myers, Gilead Sciences
Kris Kowdley, Swedish Medical Center

8:45 AM  PSC Updates

- IPSCSG
Gideon Hirschfield, University of Toronto

- Meta-Analysis Project

- PSC Endpoints Working Group
David Assis, Yale University

- IQ DILI Position Paper
Melissa Palmer, Takeda

- PSC Partners Seeking a Cure
Ricky Safer, PSC Partners Seeking a Cure

9:30 AM  Discussion
All

10:10 AM  Break
## Agenda

**10:30 AM  Session II: PSC Regulatory & Clinical Trials Update**  
**Moderator:** Kris Kowdley, Swedish Medical Center

<table>
<thead>
<tr>
<th>Time</th>
<th>Session</th>
<th>Speakers</th>
</tr>
</thead>
<tbody>
<tr>
<td>10:30 AM</td>
<td>PSC Regulatory Updates</td>
<td>Elmer Schabel, BfArM/ European Medicines Agency Ruby Mehta, U.S. Food and Drug Administration</td>
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<tr>
<td>10:55 AM</td>
<td>Current Status of PSC Trials</td>
<td>Keith Lindor, Arizona State University</td>
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<tr>
<td>11:10 AM</td>
<td>Phase 3 Trial Endpoints</td>
<td>Gideon Hirschfield, University of Toronto</td>
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<tr>
<td>11:25 AM</td>
<td>Panel Discussion</td>
<td>Panelists: Elmer Schabel, BfArM/ European Medicines Agency Ruby Mehta, U.S. Food and Drug Administration Keith Lindor, Arizona State University Gideon Hirschfield, University of Toronto Stephen Rossi, CymaBay Therapeutics Rob Myers, Gilead Sciences Rachel Gomel, PSC Partners Seeking a Cure Canada Ricky Safer, PSC Partners Seeking a Cure</td>
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**12:40 PM  Session III: PSC Forum Wrap Up**

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<tr>
<th>Time</th>
<th>Session</th>
<th>Speaker</th>
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<tr>
<td>12:40 PM</td>
<td>Future Directions &amp; Next Steps</td>
<td>Veronica Miller, Forum for Collaborative Research</td>
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<tr>
<td>12:55 PM</td>
<td>PSC Forum Adjourn</td>
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<tr>
<td>1:00 PM</td>
<td>Lunch</td>
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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
Working Groups:

- PSC Endpoints Working Group

New Work Streams:

- Pediatric Cholestatic Disease

Closed Working Groups:

- Inclusion/Exclusion Criteria Working Group
- PSC PRO Working Group
- PSC Patient Databases Working Group
Reminders

- Membership:
  - We restrict participation to experts with the necessary scientific knowledge from organizations or entities with a clear commitment to advancing the therapeutic field related to PSC.
  - We recruit project members meeting the scientific expertise criteria from the various stakeholder groups.
  - We expect all meeting participants to behave professionally, engage in discussion, and discourage the presence of passive observers.
Updates Since PSC Forum 2

- The Forum is now operating as a membership organization, in relation to industry stakeholders.
- The PSC Forum is now part of the Liver Forum.
PSC Forum 2

- PSC Forum 2, Paris, France
  - 61 attendees: 57 in person, 4 remote

**Attendance by Organization Type**
- Industry: 45%
- Academic/ Clinical Research: 16%
- Government: 13%
- Other: 16%
- Patient/ Foundation: 13%

**Attendance by Position**
- CEO: 6%
- CMO: 19%
- President/ Founder: 13%
- Director/ Project Lead: 13%
- Researcher: 13%
- Regulatory Specialist: 10%
- Other: 10%
PSC Forum 2: Evaluation Results

Impact and Collaboration

<table>
<thead>
<tr>
<th>Statement</th>
<th>Strongly Agree</th>
<th>Agree</th>
<th>Neutral</th>
<th>Disagree</th>
<th>Strongly Disagree</th>
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<tbody>
<tr>
<td>I am interested in attending the next PSC Forum event</td>
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<td>I would recommend joining the PSC Forum to a peer/colleague</td>
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<td>The presentations and discussions at PSC Forum 2 will help guide the work of my organization now or in the future</td>
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<td>Participating in the PSC Forum is valuable to my work</td>
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<tr>
<td>PSC Forum 2 extended existing collaborations or interactions with individuals and/ or organizations</td>
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<tr>
<td>PSC Forum 2 facilitated new collaborations or interactions with individuals and/ or organizations</td>
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19 September 2019

UK-PSC
# UK-PSC QoL Measure

## Steering Committee

- Elena Marcus
- Professor Douglas Thorburn
- Professor David Assis
- Dr Bella Vivat
- Martine Walmsley, *PSC Support*
- Ricky Safer, *PSC Partners*
- Audrey Cornelius, *British Liver Trust*

## PhD Supervision for Elena Marcus

- Professor Paddy Stone
- Dr Bella Vivat
- Professor Douglas Thorburn
## UK-PSC QoL Measure

<table>
<thead>
<tr>
<th>Phase</th>
<th>Status</th>
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<tbody>
<tr>
<td>1 Identification of QoL issues</td>
<td>Review of the literature and discussions (PSC patients + clinicians)</td>
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<td>2 Provisional questionnaire construction</td>
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<tr>
<td>3 Pilot testing in UK participants</td>
<td>Inc debriefing interviews (acceptability, clarity, relevance, and redundancy of the items and measure) and analysis</td>
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<tr>
<td><strong>International development</strong>&lt;br&gt;- 3 Centres (USA, Europe)</td>
<td><strong>Administrative</strong>&lt;br&gt;- Identify centres&lt;br&gt;- Local costs&lt;br&gt;- Local research approvals</td>
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<td></td>
<td><strong>Phase 1</strong>&lt;br&gt;- Local interviews/ transcript translations</td>
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<td></td>
<td><strong>Phase 3</strong>&lt;br&gt;- Local provisional measure test and debrief interviews</td>
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<tr>
<td>4 Large scale questionnaire validation</td>
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Thank you. Any questions? (or email martine@pscsupport.org.uk)
Options for Discussion

- **Microphones**
  - 1 mic per table
  - Signal moderator
  - Introduce self

- **Submit Questions**
  - https://www.sli.do/
  - Event Code: LF10

Instructions:
1. Open browser
2. Go to slido.com
3. Enter event code
How to use Sli.do

1. Go to www.slido.com
2. Enter event LF10

Type questions & submit

“Like” questions
Diamond Sponsors

- Novartis
- Novo Nordisk
Sponsors

The following sponsors contribute at their assigned membership levels:

- altimmune
- ALIGOS
- Allergan
- AMRA
- Arrowhead Pharmaceuticals
- BLADE
- Boehringer Ingelheim
- Bristol-Myers Squibb
- ChemomAb
- CIRIUS
- Coherus Biosciences
- Conatus Pharmaceuticals
- Covance
- CymaBay
- Durect
- Echosens
- Enanta Pharmaceuticals
- Enyo Pharma
- Escopics
- EA Pharma Co., Ltd.
Sponsors

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- NGM Bio
- Nitto BioPharma, Inc.
- NORDIC BIOSCIENCE
- northsea THERAPEUTICS
- Pfizer
- PLANT
- prosciento
- RESOUNDANT
- SAGIMET BIOSCIENCES
- Syneos Health
- Takeda
- TARGET PharmaSolutions
- WuXiNextCODE
- Zafgen
THANK YOU!

- The Forum for Collaborative Research
  - Executive Director: Veronica Miller
    - Katherine Barradas
    - Terry Daniels
    - Luis Javier Hernandez
    - Vin Keane
    - Brenda Rodriguez
    - Jessica Weber

- Prism Event Management
  - Paula Blay
  - Mairead O’Reilly
  - Sarah Matthews
Thank you!

Contact Information:
Jessica Weber
jweber@forumresearch.org