

**PSC Forum 3 / Liver Forum 10**  
Thursday September 19 - Friday September 20, 2019  
Bethesda, Maryland

**DAY 1: Thursday September 19**

<b>8:00 AM</b>	<b>Registration &amp; Light Breakfast</b>	
<b>8:30 AM</b>	<b>Session I: PSC Forum Updates</b>	
	<b>Moderator:</b> <i>Veronica Miller, Forum for Collaborative Research</i>	
8:30 AM	<b>PSC Forum Project Updates</b>	<i>Jessica Weber, Forum for Collaborative Research</i>
	<b>Introductory Remarks</b> PSC Forum Co-Chairs	<i>Veronica Miller, Forum for Collaborative Research</i> <i>Rob Myers, Gilead Sciences</i> <i>Kris Kowdley, Swedish Medical Center</i>
8:45 AM	<b>PSC Updates</b>	
	<ul style="list-style-type: none"> <li>• IPSCSG</li> <li>• Meta-Analysis Project</li> </ul>	<i>Gideon Hirschfield, University of Toronto</i>
	<ul style="list-style-type: none"> <li>• PSC Endpoints Working Group</li> </ul>	<i>David Assis, Yale University</i>
	<ul style="list-style-type: none"> <li>• IQ DILI Position Paper</li> </ul>	<i>Melissa Palmer, Takeda</i>
	<ul style="list-style-type: none"> <li>• PSC Partners Seeking a Cure</li> </ul>	<i>Ricky Safer, PSC Partners Seeking a Cure</i>
9:30 AM	<b>Discussion</b>	<i>All</i>
<b>10:10 AM</b>	<b>Break</b>	
<b>10:30 AM</b>	<b>Session II: PSC Regulatory &amp; Clinical Trials Update</b>	
	<b>Moderator:</b> <i>Kris Kowdley, Swedish Medical Center</i>	
10:30 AM	<b>PSC Regulatory Updates</b>	<i>Elmer Schabel, BfArM/ European Medicines Agency</i> <i>Ruby Mehta, U.S. Food and Drug Administration</i>
10:55 AM	<b>Current Status of PSC Trials</b>	<i>Keith Lindor, Arizona State University</i>
11:10 AM	<b>Phase 3 Trial Endpoints</b>	<i>Gideon Hirschfield, University of Toronto</i>
		<b>Panelists:</b> <i>Elmer Schabel, BfArM/ European Medicines Agency</i> <i>Ruby Mehta, U.S. Food and Drug Administration</i> <i>Keith Lindor, Arizona State University</i> <i>Gideon Hirschfield, University of Toronto</i> <i>Stephen Rossi, CymaBay Therapeutics</i> <i>Rob Myers, Gilead Sciences</i> <i>Rachel Gomel, PSC Partners Seeking a Cure Canada</i> <i>Ricky Safer, PSC Partners Seeking a Cure</i>
11:25 AM	<b>Panel Discussion</b> PSC Trials & Execution	

**12:40 PM Session III: PSC Forum Wrap Up**

12:40 PM **Future Directions & Next Steps** *Veronica Miller, Forum for Collaborative Research*

12:55 PM **PSC Forum Adjourn**

**1:00 PM Lunch**

**2:00 PM Session IV: Liver Forum Updates**

2:00 PM **Liver Forum Project Updates** *Katherine Barradas, Forum for Collaborative Research*

**Introductory Remarks** *Veronica Miller, Forum for Collaborative Research*  
Liver Forum Co-Chairs *Arun Sanyal, Virginia Commonwealth University*

**2:15 PM Session V: Biomarker Development**

**Moderators:** *John Sninsky, Independent & Veronica Miller, Forum for Collaborative Research*

2:15 PM **Opportunities in Biomarker Development** *Christopher Leptak, U.S. Food and Drug Administration*

2:50 PM **NASH Consortia Update**

- LITMUS *Quentin Anstee, Newcastle University*
- NIMBLE *Arun Sanyal, Virginia Commonwealth University*

**Panelists:**

3:35 PM **Panel Discussion** *Quentin Anstee, Newcastle University*  
Challenges and Opportunities *Julia Brosnan, LITMUS & Pfizer*  
*Lara Dimick-Santos, U.S. Food and Drug Administration*  
*Elisabeth Erhardtsen, Nordic Bioscience*  
*Céline Fournier, Echosens*  
*Eric Huang, Glympse Bio*  
*Christopher Leptak, U.S. Food and Drug Administration*  
*Arun Sanyal, Virginia Commonwealth University*  
*Sudha Shankar, NIMBLE & AstraZeneca*

5:00 PM **Liver Forum Adjourn**

**5:00 PM PSC Forum and Liver Forum Evening Reception**

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**Day 2: Friday September 20**

<b>8:00 AM</b>	<b>Registration &amp; Light Breakfast</b>	
<b>8:30 AM</b>	<b>Session I: NASH Combination Therapy</b>	
	<b>Moderator:</b> <i>Sophie Jeannin, Summit Clinical Research</i>	
	<b>Combination Approaches for NASH Patient Populations</b>	
8:30 AM	<ul style="list-style-type: none"> <li>Pre-Cirrhotic NASH</li> </ul>	<i>Brent Tetri, Saint Louis University</i>
8:45 AM	<ul style="list-style-type: none"> <li>Cirrhotic NASH</li> </ul>	<i>Julia Wattacheril, Columbia University</i>
		<b>Panelists:</b> <i>Carol Addy, Genfit</i> <i>Clifford Brass, Novartis</i> <i>Rob Myers, Gilead Sciences</i> <i>Star Seyedkazemi, Allergan</i> <i>Brent Tetri, Saint Louis University</i> <i>Mette Thomsen, Novo Nordisk</i> <i>Julia Wattacheril, Columbia University</i> <i>Carla Yunis, Pfizer</i>
9:00 AM	<b>Panel Discussion</b> Current Combination Strategies	
<b>10:15 AM</b>	<b>Break</b>	
<b>10:45 AM</b>	<b>Session II: NASH Regulatory Considerations</b>	
	<b>Moderator:</b> <i>David Shapiro, Intercept Pharmaceuticals</i>	
10:45 AM	<b>NASH Regulatory Update</b>	<i>Elmer Schabel, BfArM/ European Medicines Agency</i> <i>Yao-Yao Zhu, U.S. Food and Drug Administration</i>
11:15 AM	<b>Innovations in Trial Designs in NASH Clinical Research</b>	<i>Arun Sanyal, Virginia Commonwealth University</i>
		<b>Panelists:</b> <i>Naga Chalasani, Indiana University</i> <i>Lara Dimick-Santos, U.S. Food and Drug Administration</i> <i>Arun Sanyal, Virginia Commonwealth University</i> <i>Elmer Schabel, BfArM/ European Medicines Agency</i> <i>Miriam Vos, Emory University</i> <i>Yao-Yao Zhu, U.S. Food and Drug Administration</i>
11:35 AM	<b>Panel Discussion</b> Challenges & Potential Approaches	
<b>1:00 PM</b>	<b>Lunch</b>	

<b>2:00 PM</b>	<b>Session III: Parallel Breakout Sessions</b>	
2:00 PM	<b>NASH Cirrhosis Working Group</b>	
	<ul style="list-style-type: none"> <li>Compensated Risk Stratification <i>Mazen Nouredin, Cedars-Sinai Medical Center</i></li> <li>Decompensated Risk Stratification <i>Jasmohan Bajaj, Virginia Commonwealth University</i></li> <li>Discussion <i>All</i></li> </ul>	
2:00 PM	<b>Pediatric Issues in NASH Working Group</b>	
	<ul style="list-style-type: none"> <li>Defining Response in Surrogate Markers <i>Miriam Vos, Emory University</i></li> <li>Considering Type 2 Diabetes as a Clinical Endpoint <i>Stavra Xanthakos, Cincinnati Children's Hospital</i></li> <li>Discussion: Pediatric Endpoints <i>All</i></li> <li>Discussion: Pediatric Phenotypes and Inclusion Criteria <i>All</i></li> </ul>	
2:00 PM	<b>Biomarker Development: Diagnostics</b>	
	<ul style="list-style-type: none"> <li>Frequent Missteps in Biomarker Studies</li> <li>Principles of Analytical Performance, Study Design, Biostatistics, and Levels of Evidence</li> <li>Understanding How to Critique Biomarker Data</li> </ul>	<i>John Sninsky, Independent</i>
<b>3:30 PM</b>	<b>Break</b>	
<b>4:00 PM</b>	<b>Session IV: Reports and Wrap-Up</b>	
	<b>Moderator:</b> <i>Veronica Miller, Forum for Collaborative Research</i>	
4:00 PM	<b>Reports from Breakout Sessions</b>	
	<ul style="list-style-type: none"> <li>NASH Cirrhosis Working Group <i>Mazen Nouredin, Cedars-Sinai Medical Center</i></li> <li>Pediatric Issues in NASH Working Group <i>Miriam Vos, Emory University</i></li> <li>Biomarker Development: Diagnostics <i>John Sninsky, Independent</i></li> </ul>	
4:45 PM	<b>Future Directions &amp; Next Steps</b> <i>All</i>	
<b>5:30 PM</b>	<b>Meeting Close</b>	