



THE FORUM
For Collaborative ResearchSM

Pediatric Cholestatic Disease Working Group- Introductory Call

Friday, March 19, 2021



THE FORUM
For Collaborative ResearchSM

Welcome & Introduction

Jessica Weber, MSc, BSN

Forum for Collaborative Research

Berkeley Public
Health

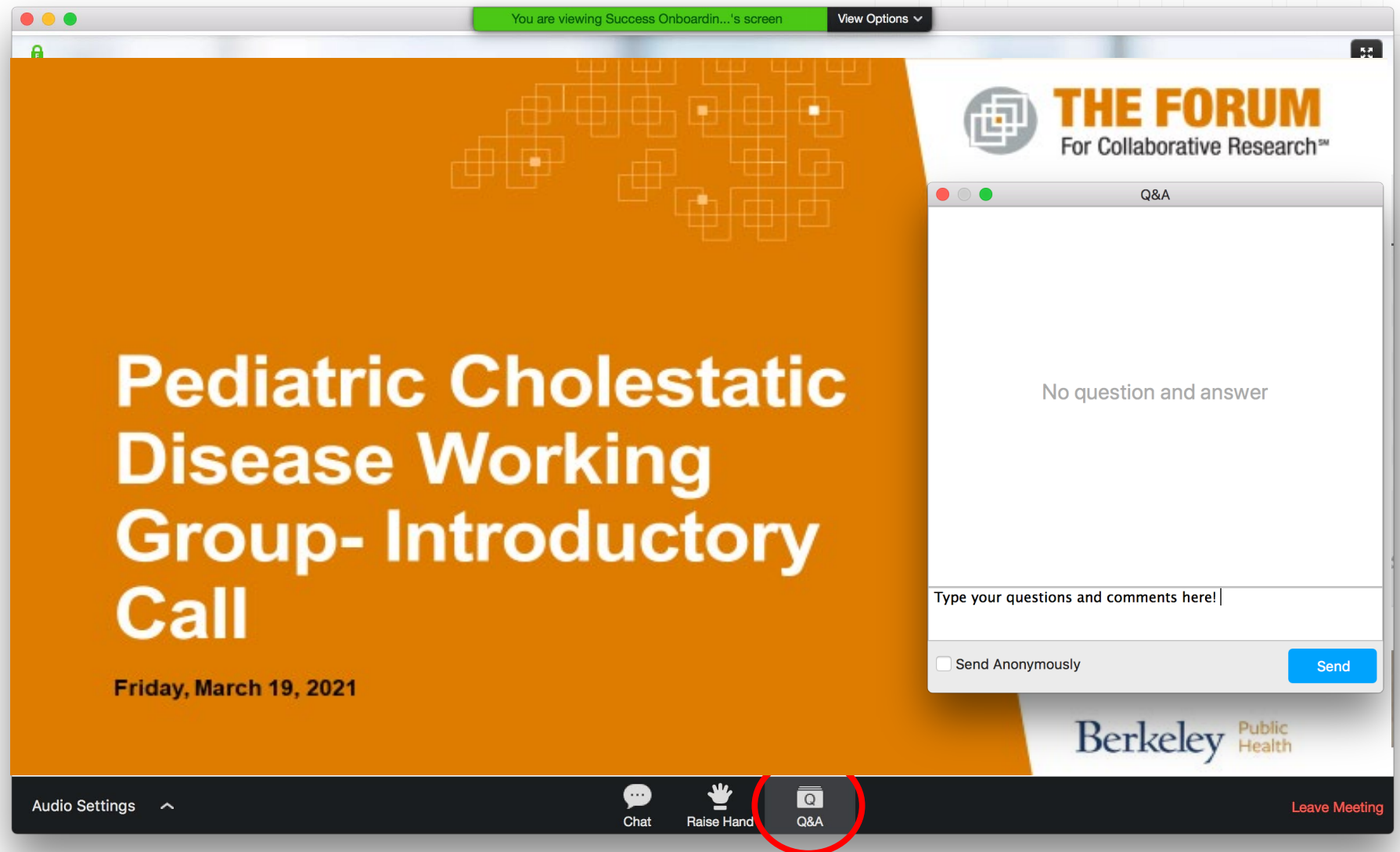
Agenda

12:00 PM ET		Begin	
			Veronica Miller, <i>Forum for Collaborative Research</i> Jessica Weber, <i>Forum for Collaborative Research</i> Saul Karpen, <i>Emory University School of Medicine/</i> <i>Children's Healthcare of Atlanta</i> Henkjan Verkade, <i>University of Groningen</i> Pam Vig, <i>Mirum Pharma</i>
12:00 PM		Welcome and Meeting Goals	
12:20 PM		Stakeholder Perspectives on Demonstrating Efficacy and Safety in Clinical Trials	
		<ul style="list-style-type: none">• Patient Representative• FDA• EMA• Industry	Roberta Smith, <i>Alagille Syndrome Alliance</i> Ruby Mehta, <i>U.S. Food and Drug Administration</i> Chrissi Pallidis, <i>European Medicines Agency</i> Pam Vig, <i>Mirum Pharma</i>
1:00 PM		Next Steps	Jessica Weber, <i>Forum for Collaborative Research</i>
		<ul style="list-style-type: none">• Logistics• Subgroups• Outputs	
1:10 PM		Discussion	<i>All</i>
1:30 PM ET		Adjourn	

How to Participate

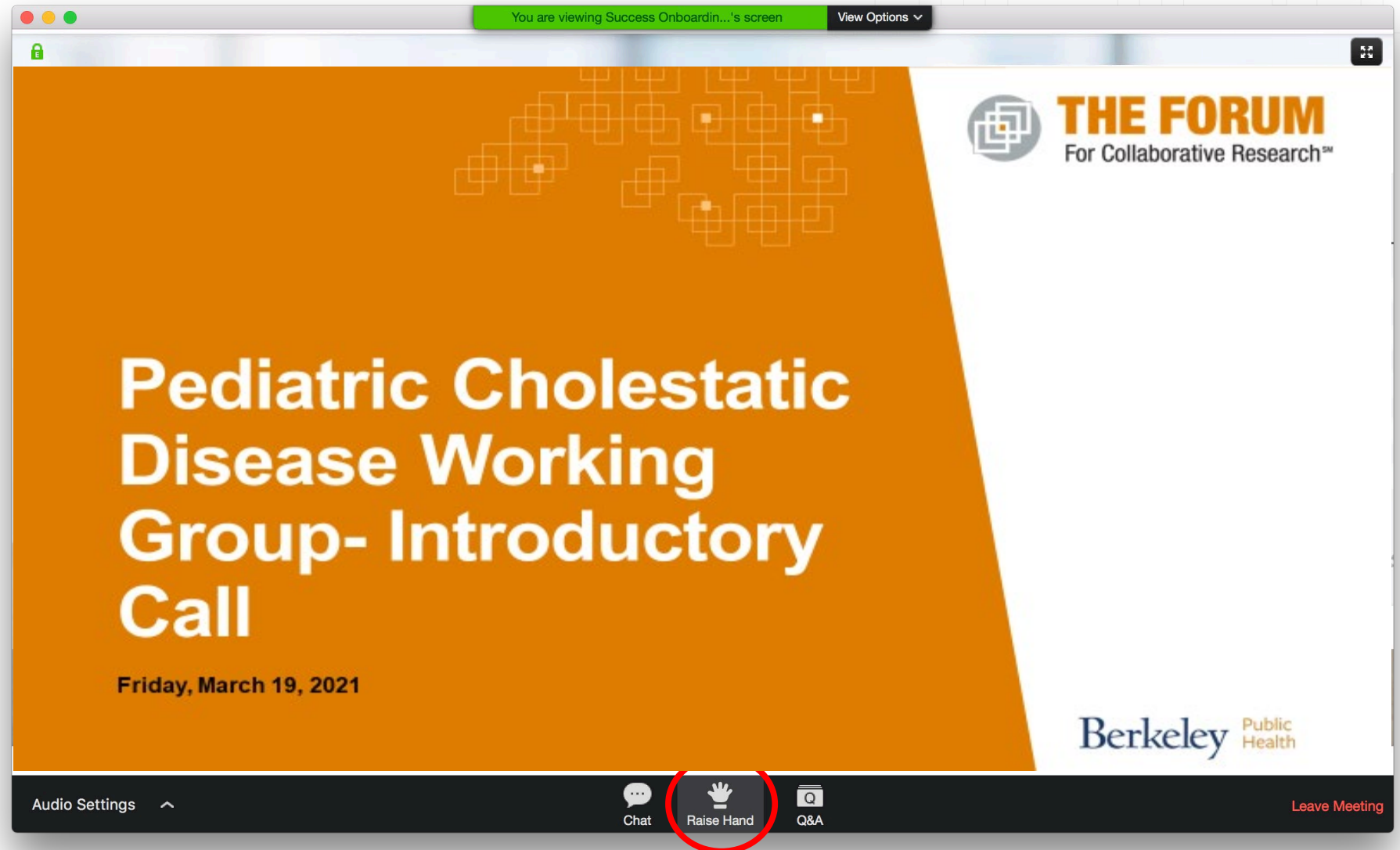
■ Q&A

- 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



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”

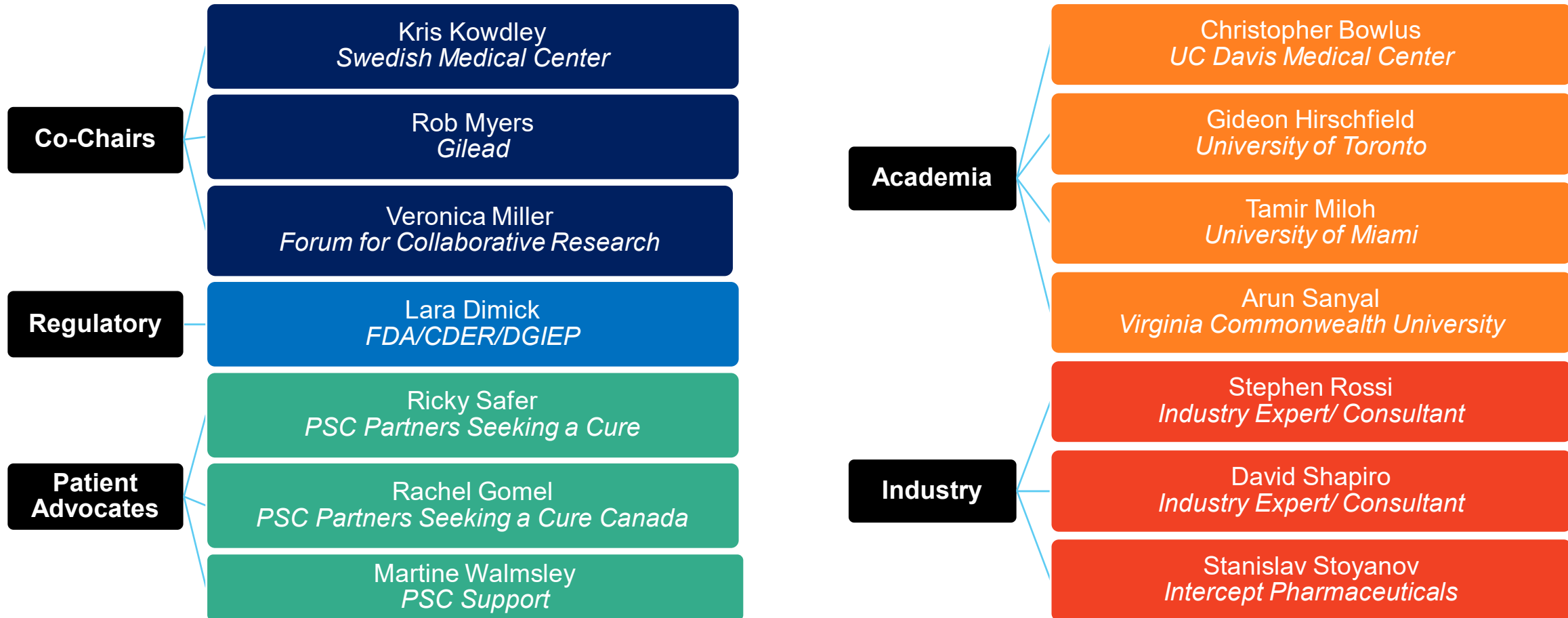
Operating Principles

- 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



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

Diamond Sponsor:



The following sponsors contribute at their assigned membership levels:



Thank You to our Sponsors

Diamond Sponsor:



The following sponsors contribute at their assigned membership levels:



Thank you!

Contact Information:

Jessica Weber

jweber@forumresearch.org