



THE FORUM
For Collaborative ResearchSM

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: <i>Veronica Miller, Forum for Collaborative Research</i>	
8:30 AM	PSC Forum Project Updates	<i>Jessica Weber, Forum for Collaborative Research</i>
	Introductory Remarks 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	PSC Updates	
	<ul style="list-style-type: none">• IPSCSG• Meta-Analysis Project	<i>Gideon Hirschfield, University of Toronto</i>
	<ul style="list-style-type: none">• PSC Endpoints Working Group	<i>David Assis, Yale University</i>
	<ul style="list-style-type: none">• IQ DILI Position Paper	<i>Melissa Palmer, Takeda</i>
	<ul style="list-style-type: none">• PSC Partners Seeking a Cure	<i>Ricky Safer, PSC Partners Seeking a Cure</i>
9:30 AM	Discussion	<i>All</i>
10:10 AM	Break	

Agenda

10:30 AM **Session II: PSC Regulatory & Clinical Trials Update**

Moderator: Kris Kowdley, Swedish Medical Center

10:30 AM **PSC Regulatory Updates**

Elmer Schabel, BfArM/ European Medicines Agency Ruby Mehta, U.S. Food and Drug Administration

10:55 AM **Current Status of PSC Trials**

Keith Lindor, Arizona State University

11:10 AM **Phase 3 Trial Endpoints**

Gideon Hirschfield, University of Toronto

11:25 AM **Panel Discussion**
PSC Trials & Execution

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*

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**

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”

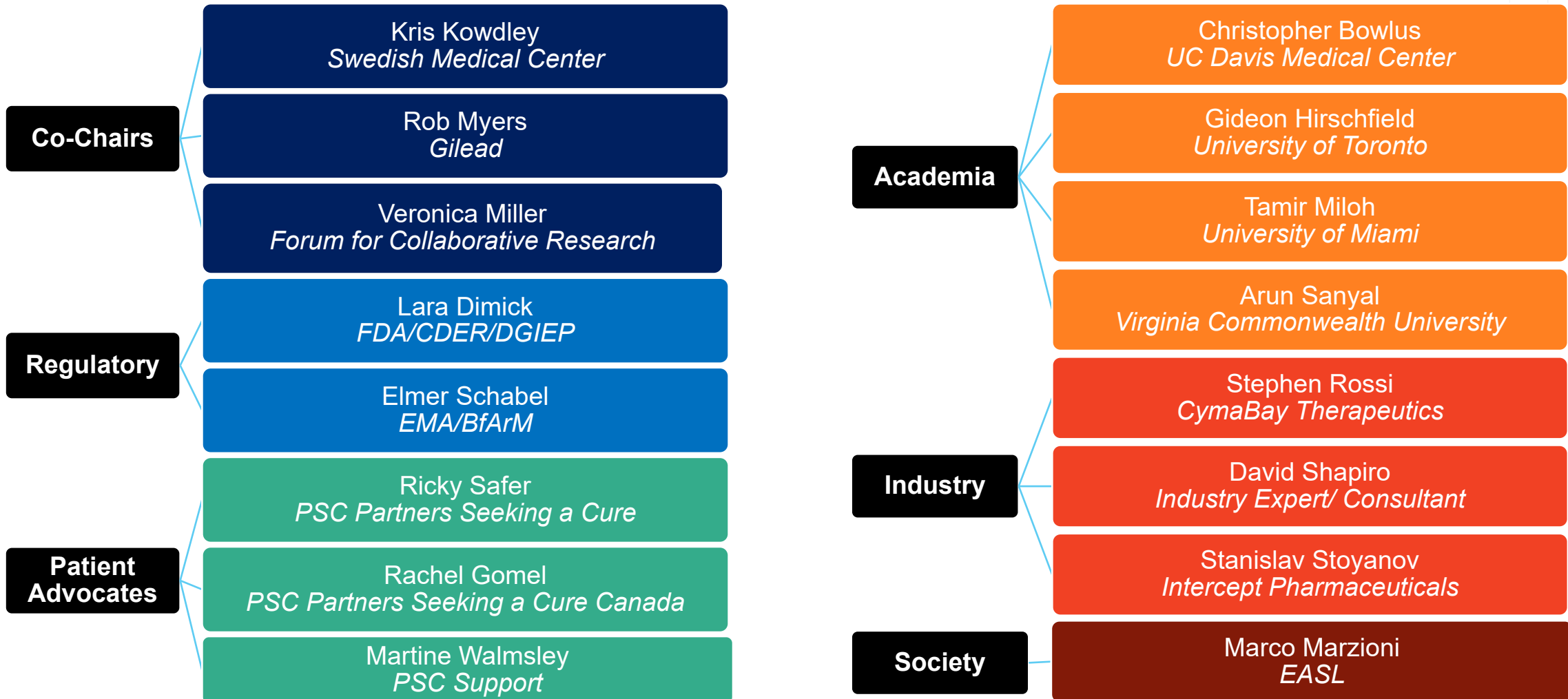
Operating Principles

- 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:

- 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

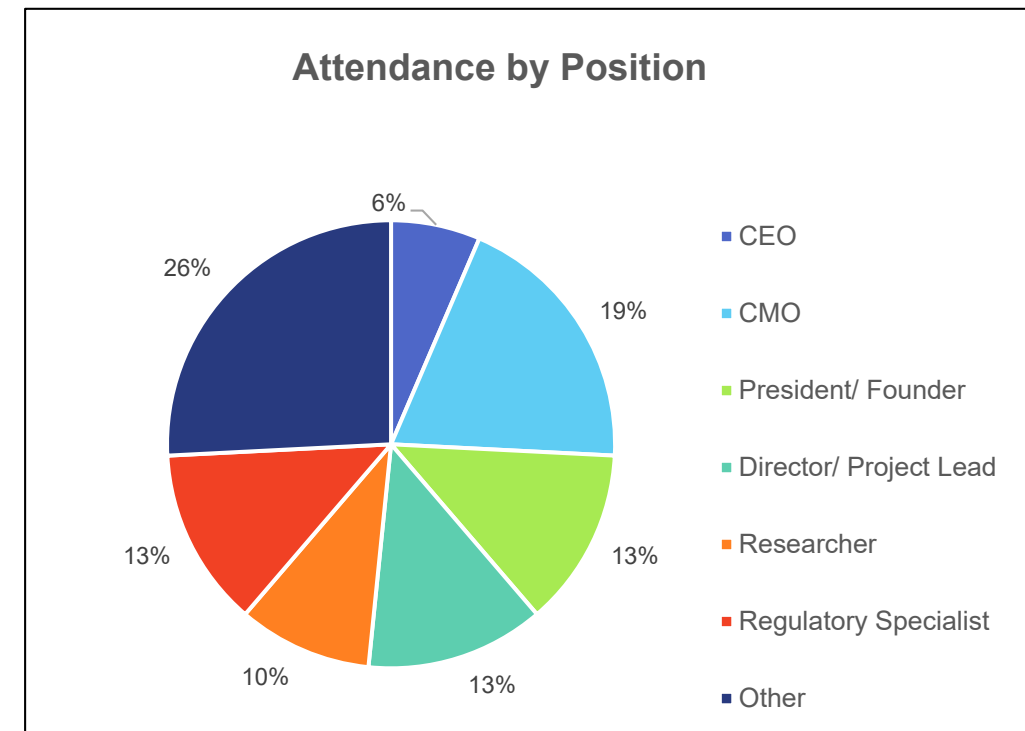
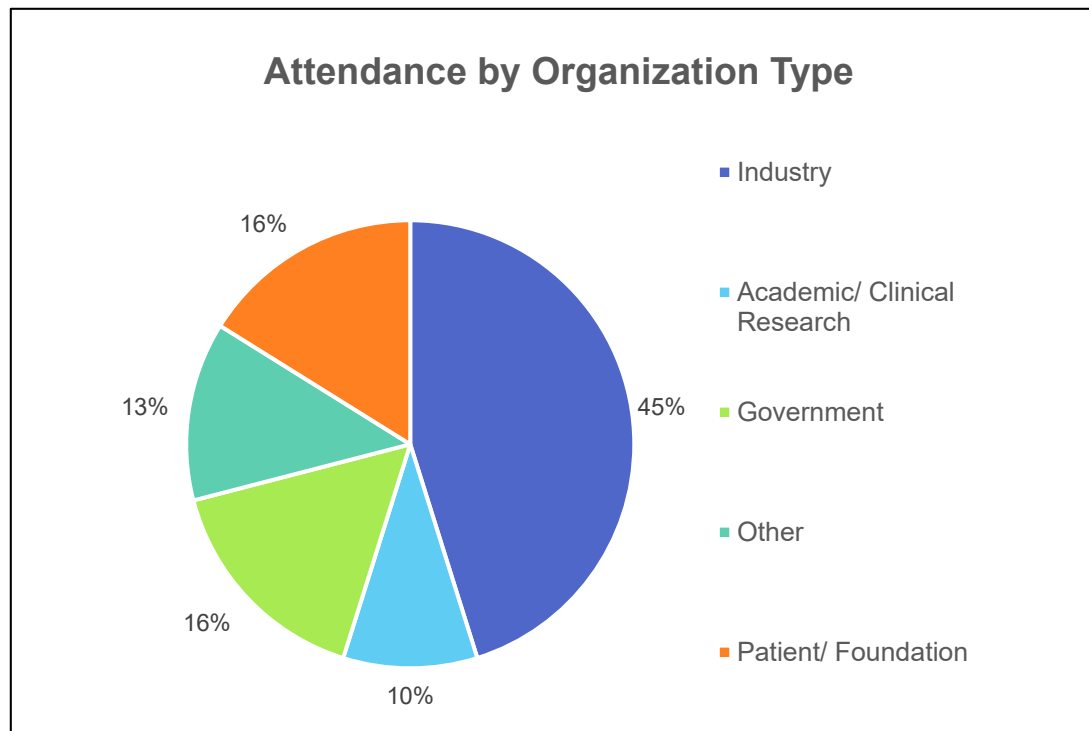
- **Membership:**
 - We restrict participation to experts with the necessary scientific knowledge from organizations or entities with a clear commitment to advancing the therapeutic field related to PSC.
 - We recruit project members meeting the scientific expertise criteria from the various stakeholder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

Updates Since PSC Forum 2

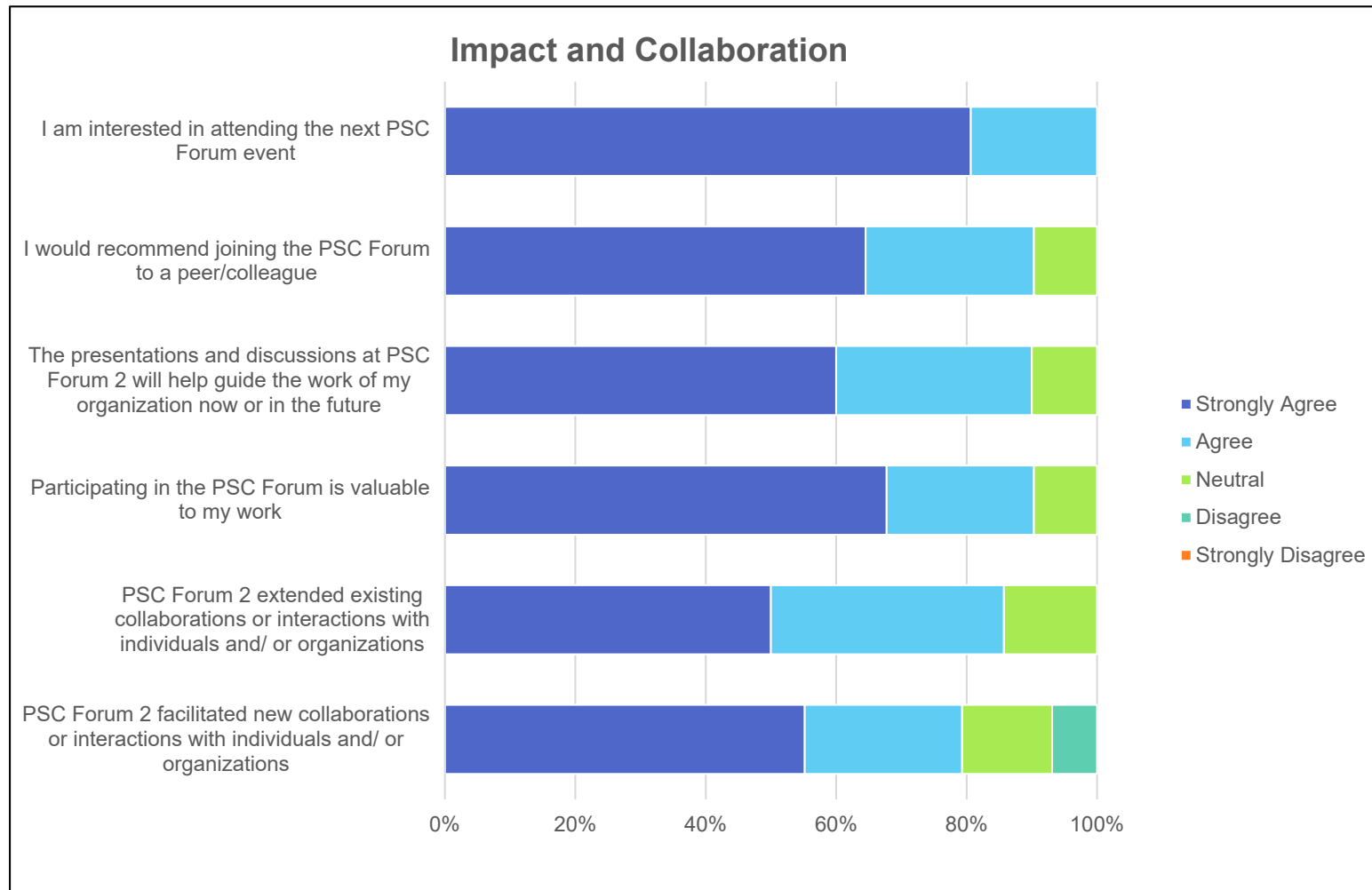
- The Forum is now operating as a membership organization, in relation to industry stakeholders.
- The PSC Forum is now part of the Liver Forum.

PSC Forum 2

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



PSC Forum 2: Evaluation Results





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

Funders



UK-PSC QoL Measure

Phase	Status		
1 Identification of QoL issues	Review of the literature and discussions (PSC patients + clinicians)		
2 Provisional questionnaire construction			
3 Pilot testing in UK participants	Inc debriefing interviews (acceptability, clarity, relevance, and redundancy of the items and measure) and analysis		
International development - 3 Centres (USA, Europe)	Administrative	Identify centres Local costs Local research approvals	Jan 2020
	Phase 1	Local interviews/ transcript translations	Oct 2020
	Phase 3	Local provisional measure test and debrief interviews	Analysis
4 Large scale questionnaire validation			

UK-PSC QoL Measure

Thank you. Any questions?
(or email martine@pscsupport.org.uk)

Options for Discussion

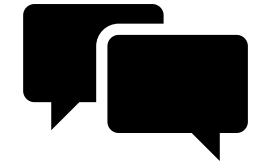
■ Microphones

- 1 mic per table
- Signal moderator
- Introduce self



■ Submit Questions

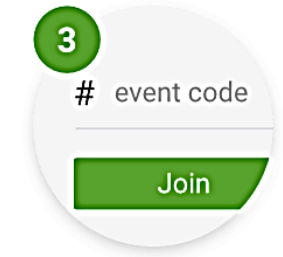
- <https://www.sli.do/>
- **Event Code: LF10**



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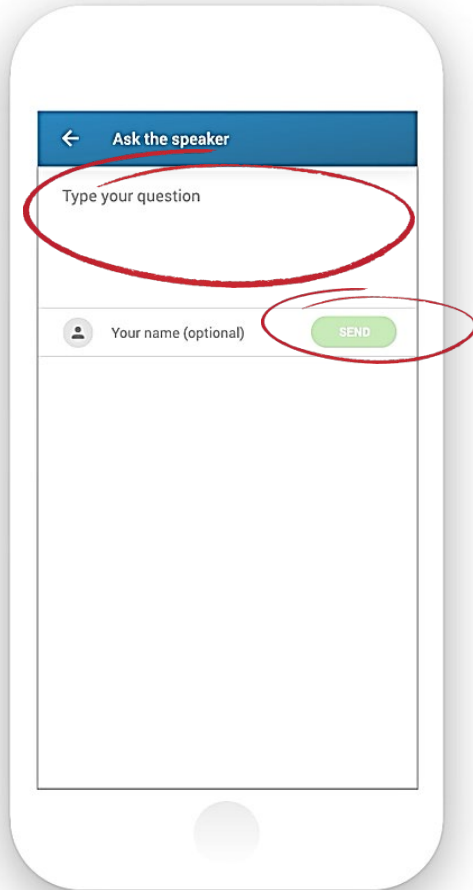
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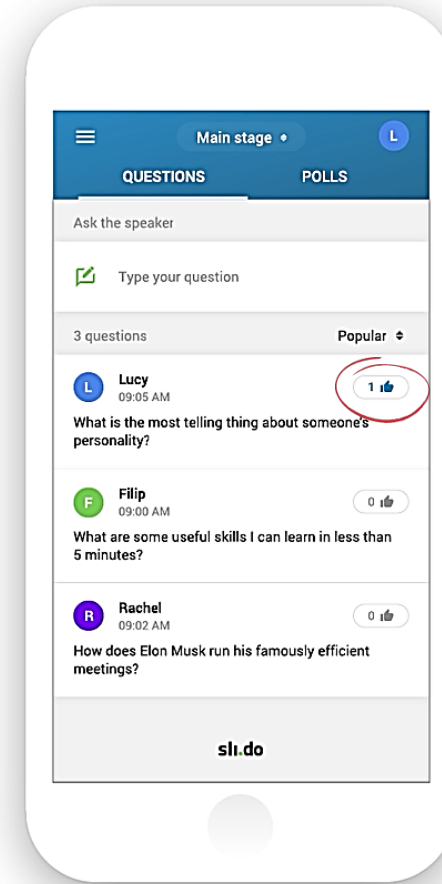
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Type questions & submit

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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:

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