Advisory Panel on Clinical Trials Fall 2016 Meeting

Washington, DC

October 26, 2016

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PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE

Welcome and Plans for the Day

Anne Trontell, MD, MPH

Associate Director, Clinical Effectiveness Research, PCORI

Elizabeth A. Stuart, PhD, AM (Chair)

Professor of Mental Health and Biostatistics, The Johns Hopkins Bloomberg School of Public Health

John D. Lantos, MD (Co-Chair)

Professor of Pediatrics, Children's Mercy Hospital

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Housekeeping

- Today's webinar is open to the public and is being recorded.
- Members of the public are invited to listen to this teleconference and view the webinar.
- Anyone may submit a comment through the webinar chat function or by emailing advisorypanels@pcori.org.
- Visit www.pcori.org/events for more information.
- Chair Statement on COI and Confidentiality

Today's Agenda

Start Time	Item	Speaker
9:00 a.m.	Welcome, New Panelists and Plans for the Day	A. Trontell E. Stuart J. Lantos
9:45 a.m.	CTAP Guidances & Potential Publications from the Work of the SCCT Subcommittee	A. Trontell
10:00 a.m.	Welcome from PCORI's Chief Science Officer	E. Whitlock
10:15 a.m.	Future Directions & Priority Setting	A. Trontell E. Stuart J. Lantos
10:45 a.m.	Break	
11:00 a.m.	Future Directions & Priority Setting Continued	A. Trontell E. Stuart J. Lantos
12:00 p.m.	Lunch & Survey to Prioritize Activities	

Today's Agenda (cont.)

Start Time	Item	Speaker
1:00 p.m.	Post-Award Management of Targeted and Pragmatic Clinical Trials	A. Trontell
1:30 p.m.	PCORI Annual Meeting Update	A. Trontell
1:45 p.m.	Update on the Work of the Subcommittee on Recruitment, Accrual, and Retention	J. Gerson
2:00 p.m.	Break	
2:15 p.m.	Future Directions: Prioritized Activities	A. Trontell E. Stuart J. Lantos
3:00 p.m.	Recap and Next Steps	E. Stuart J. Lantos A. Trontell
3:15 p.m.	Adjourn	

CTAP Guidances and Potential Publications from the Work of the SCCT Subcommittee

Anne Trontell, MD, MPH

Associate Director, Clinical Effectiveness Research, PCORI

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CTAP Articulation of Advice on Behalf of PCORI

Options

- PCORI Guidance
- Peer-reviewed publication
- Coordinated peer-reviewed publication with PCORI Guidance

(Independent publications always possible without reference to PCORI or to CTAP membership)

Options for CTAP to Articulate Advice on Behalf of PCORI: Guidance

- Represents PCORI's viewpoint for a topic area
- Contents are reviewed and cleared by PCORI senior leadership
- Published on PCORI website
- Example: PCORI's Guidance on Research in Rare Diseases

  http://www.pcori.org/sites/default/files/PCORI-Guidance-on-Research-in-Rare-Diseases.pdf

Options for CTAP to Articulate Advice on Behalf of PCORI: Peer-reviewed Publication

- If potentially construed as a PCORI viewpoint, must be reviewed/cleared by PCORI's Publication Committee which includes members of PCORI's Board of Governors
- Policies and procedures being finalized; recommend advance consultation prior to developing full content

Welcome from PCORI's Chief Science Officer

Evelyn P. Whitlock, MD, MPH

Chief Science Officer, PCORI

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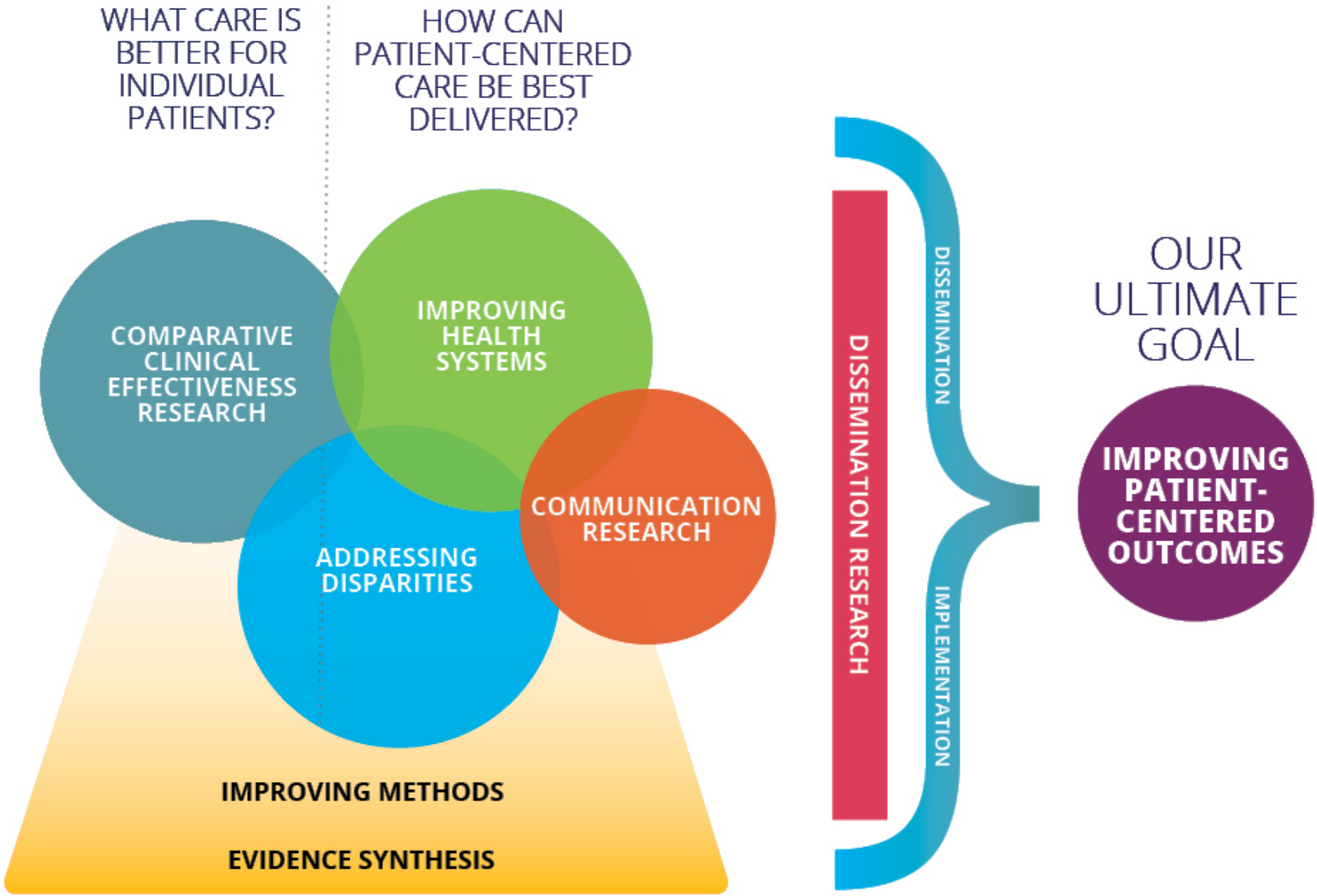
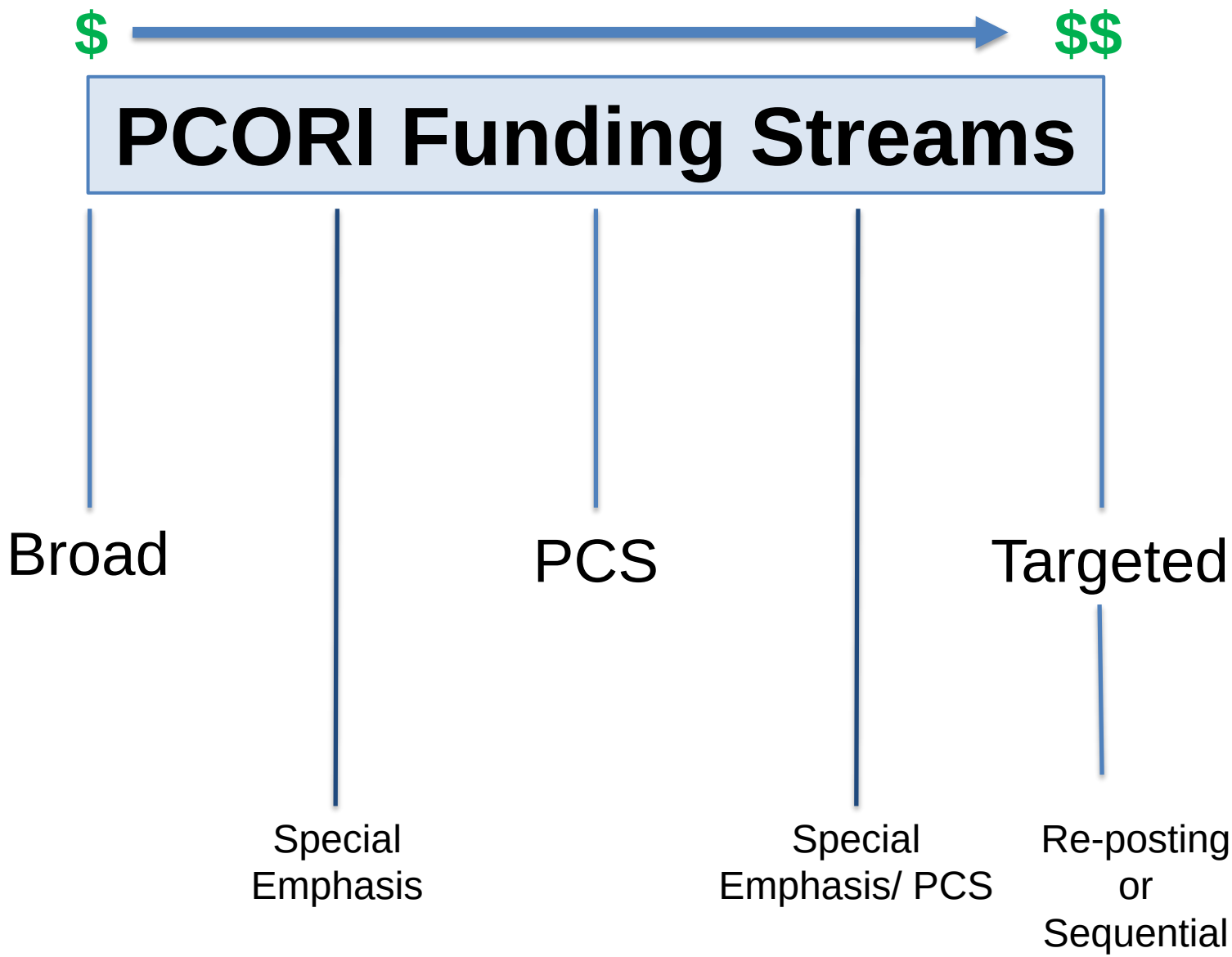
PCORI RESEARCH FRAMEWORK

Producing the comparative clinical effectiveness research (CER) evidence to improve patient-centered outcomes and inform value considerations in healthcare decisions by patients, clinicians, payers, and policy makers.

PCORI RESEARCH FRAMEWORK

APPLICABLE EVIDENCE ▶ ▶ ▶ ▶ ▶ ▶ ▶ ▶ **INFORMED DECISION MAKING**

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PCORI Funding Streams
Broad
Special Emphasis
PCS
Special Emphasis/ PCS
Targeted
Re-posting or Sequential

Thank You

Evelyn P. Whitlock, MD, MPH
Chief Science Officer

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Future Directions & Priority Setting

Anne Trontell, MD, MPH

Senior Program Officer, Clinical Effectiveness Research, PCORI

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Motivation for Discussion

- Strategic use of CTAP time and efforts
- Establishment of annual goals
- Clarification of effective mechanisms for CTAP to proffer advice
  - As a deliberative advisory group whose input is considered in PCORI decisions and actions
  - As Subcommittees or working groups that articulate a PCORI viewpoint or scientific recommendations
  - As consultants to specific PCORI research studies

CTAP Advisory Opportunities

- Direct engagement with PCORI funded clinical trials
- Advice on issues related to successful implementation/conduct of clinical trials
- Collaboration with Methodology Committee

Direct Engagement with PCORI-funded Trials

- As ad hoc consultants (e.g. ADAPTABLE)
- As a member of > 1 Study Advisory Committees (SAC) for large pragmatic or targeted clinical studies
  - SAC (or equivalent executive steering committee) now required by PCORI for its large pragmatic and targeted funding announcement (> $10 million direct costs, 5 yr length)
  - SACs meet in person at least twice a year and otherwise virtually
  - Supports the research team with refining the study questions, outcomes, and protocol.
- Benefits of direct engagement in studies
  - Offers direct experience in PCORI study planning and conduct
  - Identifies issues for CTAP attention
  - Aligns with the intended CTAP function outlined in PCORI's founding legislation.

Advisory Input on Issues in Clinical Trial Implementation

- Commentary and advice in CTAP meetings on PCORI policies, procedures, decisions, or actions on clinical trials
- Expert advice or presentations outside of CTAP meetings on issues nominated by multiple funded PCORI investigators
- Participation in PCORI guidance development for one or more aspects of clinical trial conduct
  - Best practices
  - Pitfalls to avoid

Advisory Input on Issues in Clinical Trial Implementation: Quality Scientific Conduct and Management of Trials

- Outcomes ascertainment (e.g. when might adjudication or blinding be warranted)
- Appropriateness of proposed adjustments to a study's protocol, analysis plan, outcomes, or other key study features after a study has been initiated
- Requisite preparatory data on candidate patients, clinical workflow, or other common study obstacles to support realistic assessments of feasibility and timely completion
- Productive milestones and monitoring to anticipate, detect, and mitigate risks of study delay or compromise
- Criteria/principles to aid balancing tradeoffs in pragmatic vs. tightly controlled designs as they affect internal and external validity

Advisory Input on Issues in Clinical Trial Implementation: Quality Scientific Conduct and Management of Trials

- When/how is it advisable to assess treatment adherence within a trial?
- How to handle variability in a comparator that represents the standard of care or 'usual care'?
- Do current regulations and processes for informed consent merit additional guidance to be more patient-centered?
- Can trials at high risk of delays or performance problems be identified early for enhanced monitoring and early intervention or remediation as needed?
- What are appropriate measures and benchmarks for PCORI to use in evaluating the performance of its funded clinical trials?

CTAP Collaboration with Methodology Committee

- Recommendation for new, revised, or expanded methodology standards
- Periodic or annual reporting on CTAP activities and findings

Break

10:45 – 11:00 a.m.

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Future Directions & Priority Setting Continued

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Lunch & Survey to Prioritize Activities

12:00 – 1:00 p.m.

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Post-Award Management of Targeted and Pragmatic Clinical Trials

Anne Trontell, MD, MPH

Associate Director, Clinical Effectiveness Research, PCORI

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PCORI's New Integrated Platform for Funding Research

- Built on FoundationConnect/Salesforce
- Will integrate pre-award, post-award, peer review/dissemination, contractual, and finance activities
- Will include application submission and merit review
- Post-award
  - Now being rolled out for PCORI awardee use
  - Will replace pdf submissions by January 2017
  - Supports dashboards and a variety of reporting options

Recruitment Reporting for Large Pragmatic and Targeted Studies

- Monthly reporting overall and by site
- Individual site activity status reporting
- Screening and cumulative accrual by site
- Configured to allow investigators to download/export their own tracking data for PCORI receipt and upload
- Piloting use during transition period to required use of post-award system

Anticipated Report

Cumulative screening and enrollment by site					Interval change in site's cumulative enrollment since last report
Site ID	Site active? (Enter 1 if yes, 0 if no)	Site inactivity reason (Use drop down menu)	Cumulative total of patients who have been screened	Cumulative total enrollment	
[Site ID]					
[Site ID]					
[Site ID]					
[Site ID]					

[Total # planned sites]	[Total active sites]	[Column total]	[Column total]	[Column total]

PCORI Annual Meeting Update

Anne Trontell, MD, MPH

Associate Director, Clinical Effectiveness Research, PCORI

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http://www.cvent.com/events/2016-pcori-annual-meeting/event-summary-93e8e6737e104ec8932fdac8877f1cd5.aspx

PCORI Annual Meeting 2016: Plenary Sessions

November 17	3:30 PM	How Can We Make Patient Needs and Values Central to Health Research and Decision Making? *Ronnie Sharp*
November 18	8:30 AM	Taking Stock: How Is Patient-Centered Outcomes Research Advancing Patient-Centered Care? *Harvey Fineberg*
	12:30 PM	How Can PCOR/CER Improve Care for People With Multiple Chronic Conditions? *Patrick Conway*
November 19	8:30 AM	A New Vision for Health Research: Finding Common Ground Among Stakeholders *Risa Lavizzo-Mourey*

PCORI Annual Meeting 2016: Highlighted Concurrent Sessions

Know Before You Go: Planning Upstream for Successful Recruitment in PCOR and Clinical Trials

- November 18, 10:30 AM - Noon
- Cynthia Girman (Moderator)

Management of Human Subjects Protections in Conducting Pragmatic Clinical Studies

- November 19, 10:30 AM - Noon
- John Lantos, Stephanie Morain, Julia Slutsman (Speakers)

Update on the Work of the Subcommittee on Recruitment, Accrual, and Retention

Jason Gerson, PhD

Senior Program Officer, CER Methods, PCORI

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Alignment of Proposed Standards with Methodology Standards on Patient Centeredness

Methodology Standard PC-1

PC-1: Engage people representing the population of interest and other relevant stakeholders in ways that are appropriate and necessary in a given research context

ALIGNS WITH

RAR-4: Form partnerships to increase referrals for and inquiry about the trial.

- Proposal is to include RAR-4 language (both the above bullet and additional sub-bullets) into the explanation of PC-1 in the next version of the PCORI Methodology Report.

Alignment of Proposed Standards with Methodology Standards on Patient Centeredness

Methodology Standard PC-2

PC-2: Identify, select, recruit, and retain study participants representative of the spectrum of the population of interest and ensure that data are collected thoroughly and systematically from all study participants

ALIGNS WITH

RAR-1: Ensure the trial has a systematic process in which a broad diversity of potential participants are informed and approached about trial participation

RAR-3: Ensure the provision of adequate support to encourage retention throughout the trial

- Proposal is to include RAR-1 and RAR 3 language (both the above bullet and additional sub-bullets) into the explanation of PC-2 in the next version of the PCORI Methodology Report.

Discussion about Proposed RAR-2

RAR-2: Ensure (potential) participants are supported in decision-making throughout the trial accrual process

Examples of issues relevant to RAR-2 (as applicable to a proposed study) include:

- Describe the process for in person informed consent
- Describe the process for e-consent
- Describe how comprehension is assessed (e.g. teach back)
- Describe how informed decision making is supported
- Describe how a range of literacy levels will be accommodated as part of the consent process
- Describe how the consent process will adequately and appropriately address appropriate language access, translation and interpretation needs of potential participants
- Describe how stakeholders are involved with design and implementation around these issues

MC members did not feel that the informed consent-related issues at the core of RAR-2 were suitable for a Methodology Standard, due to the large amount of existing guidance on informed consent from regulatory bodies.

Break

2:00 – 2:15 p.m.

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Future Directions: Prioritized Activities

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Recap and Next Steps

Elizabeth A. Stuart, PhD, AM (Chair)

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Thank You!

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