

# Amyloidosis Research Consortium: Research Roundtable

January 19<sup>th</sup> – 21<sup>st</sup>, 2024  
Miami, FL

## DAY 1: FRIDAY, JANUARY 19<sup>TH</sup>

7:00 am Breakfast (Marriott Bayfront Terrace South)

8:00 am Registration Opens

Amyloidosis Research Roundtable  
Double Tree by Hilton Biscayne Bay, Grand Ballroom

8:30am WELCOME AND INTRODUCTION (25 minutes)

*Isabelle Lousada, Ashutosh Wechalekar, Sarah Cairns-Smith, Kristen Hsu*

### Session 1

## WHAT HAS CHANGED OVER THE PAST 5 YEARS?

8:55am **Basic and Translational Research**

*Moderators: Jeff Kelly & Stefan Schoenland*

**Scientist's Perspective (20 minutes)**

*Jeff Kelly*

**Clinician's perspective (20 minutes)**

*Stefan Schoenland*

**Key Future Priorities (20 minutes)**

*Basic/Translational Research Group Discussion*

9:55 am Break (15 minutes)

10:10 am **AL Amyloidosis**

*Moderator: Morie Gertz*

**Update on natural history (10 minutes)**

*Vaishali Sanchorawala*

**Approval and impact of daratumumab in AL (10 minutes)**

*Shaji Kumar*

**Progress of other therapeutic approaches (e.g., anti-amyloid, CART, biospecifics) (10 minutes)**

*Murielle Roussel*

**What challenges have now emerged/remain? The perspective of industry (10 minutes)**

*Jessica Vermeulen*

**Discussion/Q&A (20 minutes)**

*All*

**11:10 am ATTR Amyloidosis***Moderator: Mat Maurer***Update on natural history (10 minutes)***Marianna Fontana, Tom Brannagan***New therapeutic options available, new trials ongoing (10 minutes)***Rodney Falk***Better diagnosis and new treatments have changed the characteristics of the population (10 minutes)***Paolo Milani***What challenges have now emerged/remain? Industry perspective (10 minutes)***Pushkal Garg***Discussion (20 minutes)***All***12:10 pm Group Photo (25 minutes)****12:35 pm Lunch (85 minutes - Marriott Bayfront Terrace South)****Session 2****ADVANCING THE SCIENCE OF CLINICAL TRIALS****2:00 pm AL Amyloidosis***Moderator: Morie Gertz***What are the lessons learned from recent clinical trials? (10 minutes)***Ray Comenzo***What additional clinical trials are needed? (10 minutes)***Stathis Kastritis***Development of a PRO/status of current PROs (10 minutes)***Vaishali Sanchorawala, Anita D'Souza***Definition of progression (10 minutes)***Angela Dispenzieri***Key future priorities (20 minutes)***AL Group Discussion***3:00 pm Break (15 minutes)****3:15 pm Breakout Session Sneak Peeks****Basic/Translational Science: pathway for non-biopsy diagnosis (10 minutes)***Stephan Harding, Gareth Morgan, Jon Wall***Development of a new staging system (10 minutes)***Shaji Kumar, Ashutosh Wechalekar***3:35 pm Hot Topic Discussion (25 minutes)***Moderators: Ashutosh Wechalekar & Sarah Cairns-Smith***4:00 pm Adjourn****6:15 pm Depart for Dinner**

## DAY 2: SATURDAY, JANUARY 20<sup>TH</sup>

7:00 am Breakfast (Marriot Bayfront Terrace South)

Amyloidosis Research Roundtable  
Miami Marriott Biscayne Bay, Watson Island

8:30 am Chairman Summary (10 minutes)

*Ashutosh Wechalekar*

### Session 2

## ADVANCING THE SCIENCE OF CLINICAL TRIALS (cont.)

8:40 am ATTR Amyloidosis

*Moderator: Rodney Falk*

What are the lessons learned from recent therapeutic clinical trials? (10 minutes)

*Spencer Guthrie, Adam Castano*

How do we approach eye or CNS involvement? (10 minutes)

*Jeff Kelly*

ATTR-PN and combination therapy (10 minutes)

*Sami Khella, Laura Obici*

Status of PROs (10 minutes)

*Kristen Hsu, Michelle Carty*

Key future priorities (20 minutes)

*ATTR Group Discussion*

9:40 am Breakout Session Sneak Peeks

How do we diagnose early? (10 minutes)

*Marianna Fontana*

9:50 am Break (15 minutes)

### Session 3

## IMPROVING CLINICAL CARE

*Moderators: Ashutosh Wechalekar & Mat Maurer*

10:05 am Focus on AL Amyloidosis

Improving management of stage 3b/4 AL patients (10 minutes)

*Vaishali Sanchorawala, Paolo Milani*

Addressing the poor/non-responders to daratumumab in AL (10 minutes)

*Angela Dispenzieri, Stathis Kastritis*

Key future priorities (15 minutes)

*AL Group Discussion*

10:40 am Focus on ATTR Amyloidosis

PYP/DPD scans – Challenges? Accuracy? What do changes mean? (10 minutes)

*Marianna Fontana*

**Prevention and when to start treatment? (10 minutes)**

*Adam Castano*

**Increased numbers of patients managed at specialized centers (10 minutes)**

*Renee Bullock-Palmer*

**Key future priorities (15 minutes)**

*ATTR Group*

#### 11:25 am **Measuring & Monitoring Disease**

**Use/potential of biomarkers (10 minutes)**

*Rodney Falk, Jeff Kelly*

**How can we monitor amyloid load? (10 minutes)**

*Marianna Fontana, Sharmila Dorbala, Cristina Quarta*

**How can we monitor disease progression/response to therapy? (10 minutes)**

*Ray Comenzo, Laura Obici*

**Key future priorities (15 minutes)**

*Measure & Monitor Discussion Group*

#### 12:10 pm **What Matters to Patients?**

**What matters most to patients (10 minutes)**

*Sabrina Rebello, Isabelle Lousada*

**Treatment and frailty of elderly patients, role of supportive care (10 minutes)**

*Ashutosh Wechalekar, Sami Khella, Mat Maurer*

**Health Disparities, access (10 minutes)**

*Renee Bullock-Palmer*

**Key future priorities (15 minutes)**

*Patient Care Discussion Group*

#### 12:55 pm **Lunch (50 minutes – Bayfront Terrace South)**

#### 1:45 pm **Breakout Session Sneak Peek**

**Federated Data Analytics: An approach to data sharing (10 minutes)**

*James Signorovitch*

### Session 4

## BREAKOUT SESSIONS

1:55 pm **Setting the stage: Aims of the breakout sessions (5 minutes)**

*Sarah Cairns-Smith*

2:00 pm **BREAKOUT SESSIONS: IDENTIFYING THE UNMET NEEDS AND KEY PRIORITIES (120 minutes)**

- Basic and translational Science (Group Leader *Gareth Morgan*)
- Shortening the time to diagnosis (Group Leader *Mat Maurer*)
- Unified staging systems for AL (Group Leader *Ashutosh Wechalekar*)
- Imaging – current challenges, future tools (Group Leader *Sharmila Dorbala*)
- Data sharing (including federated approach) (Group Leader *James Signorovitch*)



4:00 Adjourn

6:45 pm Depart for Dinner

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## DAY 3: SUNDAY, JANUARY 21<sup>ST</sup>

7:00 am Breakfast (Marriot Bayfront Terrace South)

Amyloidosis Research Roundtable  
Miami Marriott Biscayne Bay, Bayview Ballroom

8:00 am Welcome (5 minutes)

*Isabelle Lousada*

8:05 am A look back in history and forward into the future (15 minutes)

*Giampaolo Merlini*

### Session 5

## KEY PRIORITIES

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8:20 am AL Amyloidosis: Advancing the Science of Clinical Trials (10 minutes)

*Morie Gertz*

8:30 am ATTR Amyloidosis: Advancing the Science of Clinical Trials (10 minutes)

*Rodney Falk*

8:40 am Improving Clinical Care: AL and ATTR Amyloidosis, Measuring & Monitoring Disease, What Matters to Patients? (25 minutes)

*Ashutosh Wechalekar & Mat Maurer*

9:05 am Recommendations of Breakout Sessions (Presentations – 10 minutes, Discussion – 15 minutes)

*Open meeting*

11:05 am Summary: What have I heard? (20 minutes)

*Preston Dunnmon, Morie Gertz*

11:25 am Meeting Close (5 minutes)

*Isabelle Lousada*

11:30 am Coffee/Lunch/End of Meeting

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