

# FTD Research Roundtable 2024 Agenda



**AUGUST 27, 2024**

**5:00–7:00 PM**  
Reception

**AUGUST 28, 2024**

**7:00–8:00 AM**  
Breakfast

**8:05–8:30 AM**  
Event Welcome

- **2024 Co-Chairs:** Serena Hung, MD, Atalanta Therapeutics & Howard Feldman, MDCM, FRCP(C), UC San Diego
- **FDA:** Office of Neuroscience
- **EMA Representative:** Steffen Thirstrup, MD, PhD
- **FTD Family Advocate:** Joseph Marquez, MD

**8:30–10:45 AM**

**Session 1: FTD Biomarker Landscape**

**Moderator:** Howard Feldman, MDCM, FRCP(C), UC San Diego

8:30–8:45 AM

The Status of FTD Drug Development  
*Adam Boxer, MD, PhD, UC San Francisco*

8:45–9:05 AM

Pathophysiology and Biomarkers in FTD  
*Jonathan Rohrer, MD, PhD, University College London*

9:05–10:25 AM

Pathophysiological Rationale & Analytical Validation of Specific Biomarkers

- **Fluid & PET Tau Detection**  
*David Irwin, MD, University of Pennsylvania*
- **NfL & GFAP**  
*Tania Gendron, PhD, Mayo Clinic*
- **TDP-43 Measurements**  
*Leonard Petrucelli, PhD, Mayo Clinic*
- **Lysosomal Lipid Biomarkers**  
*Yohannes Ambaw, PhD, Memorial Sloan Kettering*

10:25–10:45 AM

Panel Discussion  
*Session Speakers*

**10:45–11:00 AM**  
Coffee Break

**11:00 AM–12:20 PM**

**Session 2: Inclusion Criteria for FTD Trials**

**Moderator:** Jonathan Rohrer, MD, PhD, University College London

11:00–11:05 AM

Session Introduction

11:05–11:20 AM

Predicting Clinical Progression in Active Disease  
*James Rowe, MD, PhD, University of Cambridge*

11:20–11:35 AM

NfL Qualification as an Inclusion Criteria for FTD-GRN Trials  
*Tania Gendron, PhD, Mayo Clinic*

11:35–11:50 AM

Predicting Symptom Onset in At-Risk Genetic Carriers - Beyond NfL  
*Howie Rosen, MD, UC San Francisco*

11:50 AM–12:20 PM

Panel Discussion  
*Session Speakers*

**12:30–1:30 PM**

Lunch

**1:30–3:10 PM**

**Session 3: Disease Age and Other Modeling Approaches**

**Moderator:** Arthur Simen, MD, PhD, Takeda Pharmaceuticals

1:30–1:35 PM

Session Introduction

1:35–1:55 PM

*Modeling from a Related Disease Space*  
*Klaus Romero, MD, MS, Critical Path Institute*

1:55–2:20 PM

Disease Progression Model as an Analysis Tool  
*Melanie Quintana, PhD, Berry Consulting*

2:20–2:40 PM

Assessing Disease Progression and Treatment Effects Using Multiple Outcomes  
*Sam Dickson, PhD, Pentara Corporation*

2:40–3:10 PM

Panel Discussion  
*Session speakers; FDA Office of Neuroscience; Steffen Thirstrup, MD, PhD, EMA*

**3:10–3:25 PM**

Coffee Break

**3:25–4:25 PM**

**Session 4: Reflections on Participant Recruitment and Retention**

**Moderators:** Laura Mitic, PhD, The Bluefield Project to Cure FTD & Niraj Shanbhag, MD, PhD, Takeda Pharmaceuticals  
**Patient Identification Working Group**

3:25–3:30 PM

Session Introduction

3:30–3:45 PM

Patient Identification Working Group  
*Niraj Shanbhag, MD, PhD, Takeda Pharmaceuticals*

3:45–4:10 PM

Real-World Representation in FTD Research

- **International Landscape for Research**  
*Hilary Heuer, PhD, UC San Francisco*
- **BEYONDD Study**  
*Monica Rivera Mindt, PhD, ABPP-CN, Fordham University*

**AFTD's 2024 FTD Research Roundtable**

August 27–29 • Crystal Gateway Marriott, Arlington, VA

# FTD Research Roundtable 2024 Agenda



## AUGUST 28, 2024 (continued)

4:10-4:25 PM

Panel Discussion

*Session Speakers & Dementia Advocate*

### 4:25-4:35 PM

Day 1 Closing Remarks

### 5:00-7:00 PM

Reception

## AUGUST 29, 2024

### 7:30-8:30 AM

Breakfast

### 8:30-8:35 AM

Opening Remarks

*Susan Dickinson, MSGC, AFTD*

### 8:35-10:05 AM

Session 5: Defining Meaningful In-Person Change

Moderators: *Olga Uspenskaya, MD, PhD, Eli Lilly/Prevail*

*Therapeutics & Larry Carter, PhD, Alector*

*Tool Development Working Group*

8:35-8:40 AM

Session Introduction

8:40-9:00 AM

Perspectives from People with Lived Experience

- *Voices of the Patient – Insights on FTD*  
*Shana Dodge, PhD, AFTD*
- *Q&A with Community Advocates*  
*Joseph Marquez, MD, FTD Family Advocate & Rita Choula, MA, FTD Family Advocate*

9:00-9:20 AM

FDA Guidance and Lessons Learned from Related Neurodegenerative Diseases

*FDA Office of Neuroscience*

9:20-9:35 AM

Patient Experience Data and Qualification of New Methodologies - EMA perspective

*Steffen Thirstrup, MD, PhD, EMA*

9:35-9:45 AM

Defining Clinical Meaningfulness: Progress and Plans

*Jonathan Rohrer, MD, PhD, University College London*

9:45-10:05 AM

Panel Discussion

*Session Speakers & Collin Hovinga, PharmD, MS, FCCP, Critical Path Institute*

### 10:05-10:20 AM

Coffee Break

### 10:20 AM-12:50 PM

Session 6: Infrastructure for Developing FTD Biomarkers

Moderator: *Serena Hung, MD, Atalanta Therapeutics*

10:20-10:25 AM

Session Introduction

10:25-11:20 AM

Progress in Related Fields on Specific Biomarkers of Relevance to FTD

- *TDP-43 and Other Pathway Biomarkers in ALS*  
*Sami Barmada, MD, PhD, University of Michigan*
- *Synaptic Density Biomarkers*  
*Barbara Bendlin, PhD, MA, University of Wisconsin*
- *Panel Discussion*  
*Sami Barmada, MD, PhD & Barbara Bendlin, PhD, MA*

11:20-11:35 AM

Emerging Biomarkers in Natural History Studies – ALLFTD

*Adam Staffaroni, PhD, UC San Francisco*

11:35-11:50 AM

Emerging Biomarkers in Natural History Studies – GENFI

*Arabella Bouzigues, University College London*

11:50 AM-12:05 PM

Biomarker Performance Across Ethnocultural and Genetic Backgrounds

*Carmela Tartaglia, MD, FRCPC, University of Toronto*

12:05-12:20 PM

Digital Assessment Tools for Detection and Monitoring of Symptom Development

*Adam Staffaroni, PhD, UC San Francisco*

12:20-12:50 PM

Panel Discussion

*Adam Staffaroni, PhD, UC San Francisco; Arabella Bouzigues, University College London; Carmela Tartaglia, MD, FRCPC, University of Toronto*

### 12:50-1:50 PM

Lunch

### 1:50-3:20 PM

Developing Emergent Biomarkers for Use in Clinical Trials

Moderators: *Serena Hung, MD, Atalanta Therapeutics & Howard Feldman, MDCM, FRCP(C), UC San Diego*

- *Lessons Learned from FDA Feedback on FNIH NfL Qualification*  
*Laura Mitic PhD, Bluefield Project to Cure FTD*
- *Discussion on Shared Opportunities*

### 3:20-3:30 PM

Closing Remarks

Moderator: *Penny Dacks, PhD, AFTD*

### 3:30 PM

Adjourn

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August 27-29 • Crystal Gateway Marriott, Arlington, VA