

# Committee on ALS: Accelerating Treatments and Improving Quality of Life

September 1, 2023: ALS Research and Therapeutic Development  
Public Workshop Agenda

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## Purpose

- Hold a public workshop to receive information and engage in discussion with ALS experts and experts in other disease areas and fields, about how to improve ALS research and therapeutic development.

## OPEN SESSION

### 12:00—12:05 Welcome and Introduction to the Workshop

ALAN I. LESHNER, *Committee Chair*  
Chief Executive Officer, Emeritus  
American Association for the Advancement of Science

### 12:05—12:30 Medicare Coverage Challenges in ALS

Describe the major challenges and opportunities in Medicare coverage of ALS clinical care and home-based services.

*7 min presentation, followed by 17-minute discussion with committee*

KATHLEEN HOLT  
Associate Director/Attorney  
Center for Medicare Advocacy

### 12:30—1:05 ALS Epidemiology and Research

Describe key epidemiological studies and registries in ALS.

Describe insights/trends in the data – what populations have disproportionate prevalence rates?  
Are there any clusters of particular interest?

Consider congressionally directed medical research programs in ALS.

*7 min presentations each, followed by 20-minute discussion with committee*

#### The National ALS Registry

PAUL MEHTA  
Principal Investigator  
National ALS Registry

#### Congressionally Directed Medical Research Programs in ALS

SARAH FONTAINE  
Program Manager  
Congressionally Directed Medical Research Programs  
ALS Research Program

Department of Defense

**1:05—1:15**     **Break**

**1:50—2:35**     **ALS Drug Development**

What biomarkers currently exist for ALS, and what biomarkers are being researched?  
What disease models are being used or developed to study the natural history of ALS?  
Describe some of the current activity in ALS drug development – what collaborations are underway and what are the possible outcomes and timelines for each collaborative endeavor?  
Consider the challenges and opportunities facing academic research to inform the ALS drug development pipeline.  
*7-minute presentations each, followed by 2 minute reactions/remarks from discussants, followed by 20-minute discussion with committee*

**Academic Research and the ALS Drug Development Pipeline**

JUSTIN ICHIDA  
Professor of Stem Cell Biology and Regenerative Medicine  
University of Southern California

**FDA and NIH Public Private Partnership – Critical Path for Rare Neurodegenerative Disease (CP-RND)**

COLLIN HOVINGA  
Vice President, Rare and Orphan Disease Programs  
Critical Path Institute

**ALS Therapy Development Institute (TDI)**

FERNANDO VIEIRA  
Chief Executive Officer and Chief Scientific Officer  
ALS Therapy Development Institute

**Discussants:**

- MICHAEL BENATAR, Professor of Neurology and Executive Director, The ALS Center, University of Miami
- NEIL THAKUR, Chief Mission Officer, ALS Association

**2:35—3:05**     **Designing Clinical Trials in ALS**

Describe the current landscape of ALS clinical trials – how many succeed in reaching enrollment targets? How are innovative trial designs being used?  
What level of scientific rigor is expected for an ALS research project to be promoted to the clinical trial phase?  
What are the major challenges to improving clinical trial enrollment?  
What would persons living with ALS like to see changed about clinical trials?  
*7-minute presentations each, followed by 15-minute discussion with committee*

JAMES BERRY  
Director, Neurological Clinical Research Institute  
Massachusetts General Hospital

BOB HEBRON

Chair, Clinical Trials Team  
I AM ALS

**3:05—3:10**    **Break**

**3:10—3:45**    **Lessons Learned from Other Disease Areas**

Describe how the Cystic Fibrosis Foundation measures the overall health of the cystic fibrosis population over time – what are the challenges faced in gaining a clear picture of population health?

How are patient preferences and patient-reported outcomes measured and implemented in other disease areas?

Consider how pediatric oncology has integrated clinical research and care – what are the lessons learned that could apply to ALS?

*7-minute presentations each, followed by 15-minute discussion with committee*

**Lessons Learned – The Evolution of Cystic Fibrosis Care**

ALBERT FARO  
Vice President, Clinical Affairs  
Cystic Fibrosis Foundation

**Patient-Reported Outcomes and Preferences**

NORAH CROSSNOHERE  
Assistant Professor, General Internal Medicine  
Ohio State University College of Medicine

**Integrating Research and Clinical Care – Pediatric Oncology**

WILLIAM WOODS  
Professor Emeritus, Department of Pediatrics  
Emory University School of Medicine

**3:45**    **Closing Remarks**

ALAN I. LESHNER, *Committee Chair*  
Chief Executive Officer, Emeritus  
American Association for the Advancement of Science

**END WEBINAR**