

Basic Research, Clinical Trial Infrastructure, and Community Engagement to Support Drug Development for ALS

January 27-28, 2021

Virtual Day 1: Introduction and State of Research

January 27, 2021

12:00pm-3:00pm ET

Meeting Objective: Most cases of ALS, a progressive neurodegenerative disease, have no known cause or cure. Disease pathophysiology is incompletely characterized—approximately 10% of ALS cases are linked to genetic biomarkers, while the other 90% of cases are classified as sporadic—presenting challenges in trial design and patient enrollment. While much progress has been made in basic and clinical ALS research to date, there is still an outstanding need for enhanced evidence generation on the safety and effectiveness of investigational ALS therapeutics across disease stages. In this workshop, the first in a two-part series, participants will discuss challenges impeding therapeutic development for ALS (e.g., issues associated with disease characterization, endpoint development, and the maintenance of a robust research infrastructure) as well as ways to overcome them.

12:00 p.m. Welcome and Overview

Mark McClellan, Duke-Margolis Center for Health Policy

12:05 p.m. Fireside Chat: Perspectives from FDA Leadership

- **Mark McClellan**, Duke-Margolis Center for Health Policy
- **Billy Dunn**, US Food & Drug Administration
- **Patrizia Cavazzoni**, US Food & Drug Administration
- **Peter Marks**, US Food & Drug Administration

12:30 p.m. Where Are We Now with Drug Development in ALS?

Objective: Provide an overview of the lifecycle of ALS research and drug development and provide an update on the status of current innovation in the field.

Presentation:

- **Jinsy Andrews**, Columbia University

12:45 p.m. Session 1: Importance and Limitations of Basic and Preclinical Research

Moderator: Mark McClellan, Duke-Margolis Center for Health Policy

Objective: Advancements in basic and preclinical research are fundamental to developing effective treatments for ALS. This research provides essential information about potential drug targets and characterization of the disease across sub-populations of patients who may benefit from different types of treatment. In this session, participants will discuss gaps and challenges in basic and preclinical research and the importance of collaborative approaches to addressing these issues to support drug development for ALS.

Panel:

- **Amelie Gubitz**, National Institutes of Health
- **Erin Fleming**, Project ALS
- **Michael Benatar**, University of Miami
- **Lois Freed**, US Food & Drug Administration

Open Discussion

1:40 p.m. Break (10min)

1:50 p.m. Session 2: Considerations for Innovative Trial Designs

Moderator: Mark McClellan, Duke-Margolis Center for Health Policy

Objective: While robust evidence generation is necessary to facilitate access to safe and effective treatments, the heterogenous, quick-progressing, and rare nature of ALS presents challenges that might be addressed with the use of efficient, innovative trial designs. This session will begin with a presentation on the utility of platform trial design for the assessment of new and repurposed therapeutics for ALS. Session participants will discuss scientific and practical considerations for implementing innovative trial designs in ALS drug development (e.g., approaches to increasing patient recruitment and enrollment, parameters associated with patient eligibility criteria, the use of digital tools to support data collection, the utility of remote monitoring, and the feasibility of decentralized trials).

Presentation:

- **Sabrina Paganoni**, MGH Neurological Clinical Research Institute

Panel:

- **Melanie Quintana**, Berry Consultants
- **Jeremy Shefner**, Barrow Neurological Institute
- **Richard Bedlack**, Duke University
- **Phillip Green**, ALS Research Ambassador
- **Lei Xu**, US Food & Drug Administration
- **Teresa Buracchio**, US Food & Drug Administration

Open Discussion

2:55 p.m. Closing Remarks

Mark McClellan, Duke-Margolis Center for Health Policy

3:00 p.m. Adjournment

Improving Basic Research, Clinical Trial Infrastructure, and Community Engagement to Support Drug Development for ALS

Virtual Day 2: Looking to the Future

January 28, 2021

12:00pm-3:30pm ET

12:00 p.m. Welcome and Overview of Day 2
Mark McClellan, Duke-Margolis Center for Health Policy

12:05 p.m. Session 3: Research Infrastructure and Data Sharing for ALS
Moderator: Mark McClellan, Duke-Margolis Center for Health Policy

Objective: The development and maintenance of a shared clinical research infrastructure to support efficient evidence generation as well the widespread adoption of tools and mechanisms for the sharing of data across studies are key components of effective, collaborative drug development efforts. Participants in this session will discuss existing efforts to build and maintain an enhanced research infrastructure that is supportive of data sharing. Participants will also discuss the impact of siloed, proprietary data on basic and clinical research, and approaches to ensuring quality data collection for input into shared data resources.

Presentation:

- **Alexander Sherman**, MGH Neurological Clinical Research Institute

Panel:

- **Jane Larkindale**, Critical Path Institute
- **Paul Mehta**, Centers for Disease Control and Prevention
- **Carolina Mendoza-Puccini**, National Institutes of Health
- **Neil Thakur**, ALS Association

Open Discussion

1:05 p.m. Session 4: Understanding What is Meaningful for Patients - Recruitment, Patient Experience Data, and Expanded Access
Moderator: Mark McClellan, Duke-Margolis Center for Health Policy

Objective: Integrating patient input in the design and conduct of ALS trials is vital to advancing quality and innovation in ALS drug development, as well as to expanding patient access to effective treatments. In this session, participants will discuss trial recruitment and enrollment from a patient perspective, pathways for the contribution of patient experience data to inform clinical trials, and access to treatments outside of clinical trials.

Panel:

- **Fernando Vieira**, ALS Therapy Development Institute
- **Sandy Morris**, I AM ALS
- **Paul Melmeyer**, Muscular Dystrophy Association
- **Kristina Bowyer**, Ionis Pharmaceuticals
- **Julia Tierney**, US Food & Drug Administration

Open Discussion

2:05 p.m. **Break (10 min)**

2:15 p.m. **Session 5: Coordination, Collaboration, and Shared Strategy**
Moderator: Mark McClellan, Duke-Margolis Center for Health Policy

Objective: While significant progress has been made toward understanding ALS and developing potential treatments, a continued collaborative drug development approach is needed. In this session, participants will discuss next steps for continued coordination of ALS research efforts across a variety of stakeholders to support treatment development and access.

Panel:

- **Merit Cudkowicz**, Sean M. Healey & AMG Center for ALS at MGH
- **Toby Ferguson**, Biogen
- **Bryan Traynor**, National Institutes of Health
- **Rick White**, National Organization for Rare Disorders
- **Wilson Bryan**, US Food & Drug Administration
- **Eric Bastings**, US Food & Drug Administration

Open Discussion

3:25 p.m. **Closing Remarks**
Mark McClellan, Duke-Margolis Center for Health Policy

3:30 p.m. **Adjournment**