



ALS ASSOCIATION ROUNDTABLE
DEVELOPING ACTION PLANS TO EXPAND CLINICAL
TRIAL CAPACITY & IMPROVE INCLUSIVITY
ACROSS THE ALS COMMUNITY

April 19, 2023

ALS ASSOCIATION ROUNDTABLE PROGRAM OVERVIEW

On April 19, 2023, The ALS Association (the Association) convened our ninth multi-stakeholder Roundtable. This meeting provided a forum for a deeper dive into priorities and recommendations for the Association stemming from the two 2022 Roundtable meetings on Inclusion in ALS and Enhancing ALS Clinical Trial Capacity, respectively.

In January 2023, the Association convened two multi-stakeholder working groups, including representatives from ALS clinics and research settings, individuals living with ALS, and industry, to identify and prioritize near-term action areas within inclusion and clinical trial capacity. Working group representatives presented their recommendations to the April 19 Roundtable participants, who then met in small groups to further prioritize and refine proposed next steps. Additionally, the Roundtable provided an opportunity for a retrospective across Roundtable focus areas since the program's inception in 2019.

KEY ROUNDTABLE THEMES

What Life is Like at ALS Clinics

Several leading ALS clinicians and clinical trialists described current challenges associated with conducting research and their vision for the future. They noted the need to increase access to clinical trials for more people, including by leveraging telemedicine; align incentives for conducting clinical trials; and streamline regulatory requirements and processes.

Focus on Inclusion

A multi-stakeholder group presented its recommendations for priority actions by the community to advance inclusion in diagnosing ALS. These included:

- A. Expanding/accelerating referral to neuromuscular specialists from health care providers (HCPs) in underserved communities
- B. Supporting HCPs in under-represented communities
- C. Defining thoughtful engagement with non-traditional organizations (e.g., faith-based institutions, community organizations, businesses, sports, etc.)
- D. Calling attention to gaps in engagement/current methods of ALS data collection

After small group discussions, the Roundtable participants prioritized two recommended near-term next steps for focus:

- Expanding/accelerating referral to neuromuscular specialists among HCPs in underserved communities.
- Defining thoughtful engagement with non-traditional organizations (e.g., faith-based institutions, community organizations, businesses, sports, etc.)

Focus on Clinical Trial Capacity

The multi-stakeholder Clinical Trial Working Group presented its work with a focus on building site and investigator capacity. The group emphasized the importance of speeding up ALS trials by 1) facilitating/expanding trial sites' ability to run effective studies and 2) supporting referrals to trial sites from clinics that do not conduct this research.

Specifically, the Clinical Trial Working Group brought four recommendations to the Roundtable for further prioritization and refinement:

- A. Collecting data on barriers to starting and conducting trials (including staffing issues) to support addressing those barriers
- B. Supporting referrals of patients to trials from all ALS specialists to available trial sites
- C. Supporting development of an ALS Research Coordinator Network
- D. Developing a model of "what good looks like" for ALS clinical trial infrastructure in the U.S.

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- Developing a model of "what good looks like" for ALS clinical trial infrastructure in the U.S.

As immediate next steps for both Inclusion and Clinical Trial Capacity, the Association will conduct a feasibility, impact, and time (FIT) assessment to further prioritize and map out

FIVE-YEAR RETROSPECTIVE

As 2023 marks the fifth year since the launch of the Roundtable program, Roundtable participants reviewed the progress made during that time.

Fall 2022

- Topic: [Increasing the Number of, and Access to, High-Quality Clinical Trials in ALS](#)
- Outcomes:
 - o Creation of working group
 - o Refinement of next steps during Spring 2023 Roundtable

Spring 2022

- Topic: [Developing Action Plans to Advance Inclusion in ALS Diagnosis, Care, and Research](#)
- Outcomes:
 - o Creation of working group
 - o Refinement of next steps during Spring 2023 Roundtable



Fall 2021

- Topic: [Strategic Priorities for ALS: A Community Discussion](#)
- Outcomes:
 - Input into the Association's research strategy and plans for advancing the "livable" goal
 - Development of new research funding opportunity focused on increasing clinical trial capacity

Spring 2021

- Topic: [Access to Essential Care, Services, and Supports](#)
- Outcomes:
 - Focus on consistency and access in care services and advocacy
 - Development of an interactive [patient journey web tool](#) that helps prepare and empower people with ALS as they are faced with making critical decisions

Fall 2020

- Topic: [Decreasing ALS Complications](#)
- Outcomes:
 - Renewed effort to promote materials on managing hospital visits
 - Incorporation of "preventing harms of ALS" into the Association's pillars
 - Implementation of new ways to track complications for people with ALS treated at ALS Association clinics to help guide intervention development
 - Development of new research funding opportunity focused on quality of care

Spring 2020

- Topic: [Reducing Time to Diagnosis](#)
- Outcomes:
 - Creation of two working groups: Time to Diagnosis and Genetic Testing
 - Development of the [thinkALS](#) tool
 - [Educational webinar](#) about importance of early diagnosis
 - Development and dissemination of an Association [statement on timely diagnosis](#)
 - Creation of a genetic counseling and genetic testing awareness and education campaign

Fall 2019

- Topic: [Access to New Therapies](#)
- Outcomes:
 - ALS Focus survey on [Understanding Insurance Needs and Financial Burdens](#)
 - Partnership with the [Patient Advocate Foundation](#), which provides direct benefits to people with ALS such as overturned insurance denials; grants for copays, deductibles, premiums, living expenses, disability awards (SSDI, SSI); and other financial support
 - Strengthened strategic engagement with the FDA, including developing an Association [policy](#) on supporting approval of experimental therapies

Spring 2019

- Topic: [Setting ALS Association Strategic Priorities](#)
- Outcomes:
 - Key input on three priority areas – finding new treatments and cures, optimizing care, and preventing ALS and its harms – for the Association’s [strategic plan](#)
 - Topic ideas for future Roundtables and ALS Focus surveys

NEXT STEPS

Dr. Neil Thakur and Dr. Kuldip Dave from The ALS Association introduced several topics that are under consideration for future Roundtable meetings.

The first relates to real world evidence (RWE) and evaluating the changing treatment landscape now that there are multiple approved therapies for ALS. There is a need to collect and evaluate data about what happens when people are taking the available therapies in combination or sequentially. The Roundtable could evaluate examples in other diseases (e.g., SMA) where similar issues have arisen. Key questions include determining how data will be collected, what the pitfalls and costs may be, and how long this might take.

A second topic under consideration relates to quality of care and collecting better data on why people with ALS are hospitalized or visit the ER. In addition to developing a more comprehensive understanding of the impact of falls, respiratory complications, and other medical emergencies, there is a need to develop better strategies to prevent and minimize them.

Additional topics raised by Roundtable participants included:

- Expanding and improving data collection on diagnosis (understanding gaps)
- Evaluating data on outcomes to determine if recent approvals have led to more people living with ALS
- Addressing caregiver burden
- Determining steps to address additional inclusion and clinical trial topics (beyond those prioritized in this meeting)
- Conducting an after action review of the Act for ALS and expanded access
- Evaluating nursing home care



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