



ALS ASSOCIATION ROUNDTABLE

STRATEGIC PRIORITIES FOR ALS:
A COMMUNITY DISCUSSION

NOVEMBER 3, 2021

ALS ASSOCIATION ROUNDTABLE PROGRAM OVERVIEW

On November 3, 2021, The ALS Association (the Association) virtually convened our sixth multistakeholder Roundtable. This meeting focused on identifying research priorities for the Association with an emphasis on feasibility and impact in supporting the Association's goal of making ALS a livable disease. Association staff presented the current draft research plan to gain input and recommendations from all key stakeholders — people living with ALS, caregivers, clinicians, researchers, public and private funders, and industry partners — about what to prioritize given finite resources and capacity and the unique role the Association plays in advancing ALS research.

ROUNDTABLE CONTEXT & FRAMING CONVERSATIONS

To provide insight into the current research landscape, speakers representing people living with ALS, caregivers, federal agencies and other nonprofit organizations presented overviews of their respective research interests and current programs. In the weeks prior to the Roundtable, Association staff met individually with representatives from each industry partner to discuss top interests and identify gaps within research. This information was anonymized and summarized in a pre-read document shared with all Roundtable participants.

KEY ROUND TABLE THEMES

Throughout the Roundtable, panelists and participants highlighted several important themes for the Association to consider as it continued to refine its research plan. Top priority topics that emerged for focus by the Association within its plan included:

- Updating/refining the ALSFRS-R.
- Funding biomarker research.
- Addressing disease heterogeneity — both clinical and pathological.
- Bolstering clinical trial infrastructure.
- Developing new “patient-important” outcome measures.

Advancing these efforts will require multi-stakeholder and multi-organizational collaborative efforts. The community as a whole must focus on accelerating progress and shortening the time it takes to impact the lives of people with ALS. It is important to recognize that, while we are aiming for a “home run” through research, making significant incremental progress is also valuable.



PANEL & TOWN HALL GROUP DISCUSSION

The Roundtable included three panel discussions to further elicit input from a diverse set of stakeholders and provide additional insights, recommendations and context to inform the Association's research strategy/plan.

Panel 1: People Living with ALS

Panelists included four individuals living with ALS. Each panelist was asked about their top priorities for ALS research based on their own experiences. Key themes that emerged during this panel and group discussion included:

- Accelerating development of effective treatments with "multiple shots on goal."
- Developing ALS biomarkers.
- Addressing heterogeneity within the disease to design trials that are effective for people with ALS having varying rates of progression.
- Streamlining the process for people with ALS to gather information, understand their eligibility and enroll in clinical trials; and developing a checklist to help this process.
- Leveraging telehealth/telemedicine to expand access to clinical trials.

Panel 2: Government Agency Funders

During this panel, six representatives of federal agencies that fund ALS and related research activities provided an overview of their respective organization's ALS research programs and efforts. They also were asked to follow up after the meeting with additional insights about how their agencies could engage with the Association to help make progress toward its goal of making ALS a livable disease by 2030.

Key themes emerged during this panel and group discussion.

- Collaboration across government funding agencies, as well as with the nonprofit and for-profit sectors is vital.
- Increasing federal appropriations have permitted federal funders to expand their programs for ALS, neuroscience and rare diseases.
- There is agreement about the importance of developing biomarkers for ALS.
- Understanding the genetic underpinnings of ALS and its various subtypes is a priority.
- There is a need to enhance standardization of data elements across research programs, including terminology and definitions of various types of ALS.
- Federal funders are prioritizing efforts to expand diversity, equity and inclusion within their research programs, including within their workforces, among investigators and among clinical trial participants.
- Improving coding of types of research project would help ensure complementarity (and avoid duplication) across funders of ALS research (e.g., a form of Global Unique Identifier [GUID] system).
- Panelists highlighted the importance and impact of the Association's efforts in supporting federal efforts for ALS.



Panel 3: Nonprofit Funders

The third panel included four speakers representing nonprofit organizations that support ALS research. Each panelist was asked to provide an overview of their organization's ALS research programs and approach.

Key themes from this panel and group discussion included:

- There is alignment on the importance making ALS livable, while also trying to cure and prevent it.
- Efficiency and speed matter in providing research funding. The traditional grantmaking timeline is "inconsistent with the ALS clock."
- Progress in developing biomarkers will help accelerate impact.
- It is a priority to reduce time to diagnosis.
- Organizations need to be flexible and willing to sunset programs that are not working.
- Imaging programs – we do not have the tools yet for this.
- It is a priority to build the field, including supporting the next generation of scientists and funding those in the mid-career stage.

Town Hall Discussion

The final session of the Roundtable offered the opportunity for an open discussion about the Association's draft research plan. Using a facilitated town hall format, all participants were able to comment on their top priority for what the Association can do through its research programs to advance progress toward the goal of making ALS a livable disease.

A robust list of topics emerged from this session, including topics that are included in the current draft Association strategic plan and others that may need to be incorporated in a future version of the plan:

- Balance the research portfolio.
- Build the ALS research and drug development field (human capital).
- Improve clinical trials.
- Update standards of care.
- Expand understanding of ALS in underrepresented parts of the U.S. and the world.

NEXT STEPS

In addition to developing a detailed meeting summary from the Roundtable meeting, Association staff will engage with key stakeholders to review all recommendations made by participants and determine best next steps to prioritize those actions that can be operationalized into programs in the near-term and those that may require additional consideration, time, resources and/or potential collaborations.

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