



# ALS ASSOCIATION ROUNDTABLE

LEVERAGING REAL WORLD DATA  
TO IMPROVE COMBINATION  
THERAPIES AND PREVENT  
COMPLICATIONS

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October 23, 2023

# Executive Summary



## ROUNDTABLE PROGRAM OVERVIEW

On October 23, 2023, we convened our 10th multi-stakeholder Roundtable, celebrating 5 years of this innovative and impactful program. The meeting served as a forum for stakeholders to discuss real-world data (RWD) and real-world evidence (RWE) in the context of ALS research and care.

**Real-World Data (RWD)** – Data relating to patient health status and/or the delivery of health care routinely collected from a variety of sources.

**Real-World Evidence (RWE)** – Clinical evidence about the usage and potential benefits or risks of a medical product derived from analysis of RWD.

Source: FDA. (2023). [Considerations for the Use of Real-World Data and Real-World Evidence to Support Regulatory Decision-Making for Drug and Biological Products: Guidance for Industry.](#)

During the meeting, participants highlighted the significance of RWD and RWE in advancing ALS research and care, particularly given the inherent limitations of clinical trial data. They emphasized the need for RWD and RWE to:

- Support more timely guidelines and treatment protocols/standard of care changes.
- Inform better insurance coverage and payment policies.
- Improve the lives of people living with ALS.

Additionally, the use of RWD for regulatory decision-making and the challenges associated with collecting and interpreting data from clinical trials and real-world settings were discussed. **The call to action for the ALS community was to work together to harness RWD for the benefit of patients and to accelerate progress in the field.**



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## OVERARCHING THEMES

Throughout the day-long Roundtable meeting, expert speakers helped paint a picture of the need for RWD/RWE in the ALS field, as well as the current landscape, gaps and opportunities for progress. Roundtable sessions included:

- A conversation about the potential impact of RWD/RWE for advancing progress and improving outcomes for people living with ALS by developing new care approaches and ensuring that those approaches are made available to the community.
- A presentation by FDA officials about the applications of RWD in the agency's neuroscience activities and its RWE framework.
- A panel with various non-profit and academic organizations describing current data collection initiatives within the ALS ecosystem and the importance of avoiding duplication and leveraging opportunities for collaboration to speed and enhance impact.
- A discussion about gaps in the current RWD landscape for ALS, which emphasized the importance of clear goals, collaboration and patient-centered approaches.
- Small group brainstorming sessions, which focused on opportunities and barriers to the collection and effective use of RWD in ALS.

Multiple overarching themes emerged for advancing the use of RWD and RWE in the ALS space, such as:

- Addressing data fragmentation and collaboration.
- Defining data sufficiency.
- Turning RWD into RWE.
- Enhancing data analysis capacity and tools.
- Incorporating new treatments and dynamism.
- Enhancing patient engagement and reducing burdens on people living with ALS.
- Ensuring equity and access.
- Promoting clinician buy-in and sharing lessons learned.



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## IDEAS FOR CONSIDERATION

During the second half of the Roundtable, participants broke into small groups to consider and brainstorm:

- Unique efforts or activities the ALS Association could undertake to contribute to the collection of RWD in ALS.
- Barriers to the effective use of RWD in ALS that could potentially be address by the Association.

Ideas that emerged from those discussions included:

- Promoting and/or funding efforts to analyze and get answers from existing data.
- Raising awareness about RWD and data collection efforts to help encourage more people living with ALS to participate.
- Addressing barriers that result in a lack of representativeness in ALS RWD.
- Supporting the standardization of data elements and the coordination of data collection.
- Defining evidence-based quality of care metrics and collaborating with similar disease associations to identify best practices.
- Advocating for privacy regulations that facilitate data sharing while still protecting participants.

## NEXT STEPS

We will engage with key stakeholders to review the recommendations made by participants and determine the best next steps to prioritize any actions that could be operationalized into programs for the near-term and those that may require additional consideration, time, resources and/or potential collaborations.

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