

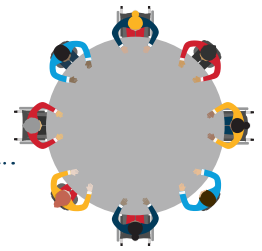


# ALS VALUE & ACCESS ROUNDTABLE EXECUTIVE MEETING SUMMARY



**October 28, 2019**  
**Washington, DC**

# OVERVIEW



**O**n October 28, 2019, The ALS Association convened its second Roundtable meeting in Washington, D.C. Forty-four participants, representing key ALS and health care system stakeholders (see Appendix B), joined the one-day session to consider the topics of assessing value for and ensuring access to ALS therapies.

The Roundtable discussion was an opportunity for collective brainstorming and problem solving about the challenges, potential solutions, and near term action steps the community can take to ensure people with ALS are able to access and pay for current drug therapies and those that could reach the marketplace in the near future. In the meeting, ALS care providers, people living with ALS, caregivers, industry, representatives of public and private payor organizations, health policy experts, and Association leadership actively engaged in the discussion. The meeting was facilitated by Ilisa Halpern Paul, President of the District Policy Group at Drinker Biddle.

## BACKGROUND & CONTEXT

In providing context for the day's discussion, ALS Association Executive Vice President of Mission Strategy Neil Thakur, Ph.D., reviewed recent activities undertaken by the Association since the March 2019 Roundtable agenda setting meeting.

- A **working group on increasing clinical trial participation** has been established and is evaluating **clinical trial engagement strategies and development of measurement tools**.
- The Association is establishing a performance measure and ways to **identify bottlenecks and points of intervention in the many diagnostic pathways** people with ALS face on their way to an ALS specialist.
- **ALS Focus, a survey platform to determine what is most important to people with ALS**

**and their caregivers**, has launched with the first survey to be issued in the coming months.

- The **ALS Voice of the Patient Report to the FDA**, stemming from the IMPACT ALS survey and involving more than 1,500 people, was published.
- **FDA has issued new Guidance on the development of drugs and treatments for ALS** related to improvements in ALS clinical trial methodology, and design principles.

Dr. Thakur noted how each of these activities has helped to better identify and communicate the needs and values of people with ALS and their caregivers, to inform drug developers, regulatory officials, and payors.

The October 28<sup>th</sup> Roundtable builds on this work by zeroing in on ways to ensure that people with ALS have access to current and future drug therapies that could help meet their needs. Given the progress being made in multiple drug development programs aimed at disease-modifying treatments for ALS, it appears to be only a matter of time before such therapies are commercialized. This raises key questions about coverage, reimbursement, pre-authorization, and access. By convening a Roundtable meeting focused on these topics, the Association is laying a foundation from which the community can work together to ensure rapid adoption of new treatments into standard practice for people with ALS.

## KEY TAKEAWAYS

Roundtable participants agreed that there is a critical need to reduce the current timing between regulatory approval of a new therapy and access to treatment for people with ALS. They collectively identified, discussed, and prioritized a series of challenges.

- **COVERAGE CRITERIA:** Payers require specific types of evidence to evaluate new therapies and make determinations about drug formularies and coverage.
  - Sometimes what payors require to make these decisions do not align with drug developers’ clinical trial outcome measures, which were used in the regulatory process for FDA approval.
  - Current clinical trial exclusion criteria create an evidence gap as payors look to restrict access to approved therapies based on the patient populations studied in the trial.
- **VALUE:** There is a need to better define and measure what people with ALS need and value from therapies (beyond survival), recognizing that these aspects will likely evolve as people move through their journey with the disease.
  - The patient voice can inform favorable coverage decisions and access to care by defining a “360-degree” understanding of the elements that matter to people with ALS to improve their quality of life.
  - Defining value and determining coverage require specific information, which takes time, adding to the delay a person with ALS might experience accessing a new treatment.
- **FINANCIAL BURDEN:** People with ALS and their families face high costs of care that are likely to be exacerbated by the availability of new, expensive drug-therapy options.
  - There is a need to address uneven availability of co-pay assistance and inconsistent current coverage and benefits, which creates access challenges.
- **AUTHORIZATION PROCESSES AND ADMINISTRATIVE BURDEN:** The current “system” for gaining access to needed therapies is burdensome

for everyone (people with ALS, caregivers, providers, sponsors) to navigate. This involves pre-authorization, denials, appeals, utilization review, and more.

- People with ALS, their families, and caregivers spend a burdensome amount of time appealing denials and coverage exceptions for many elements of care and disease management throughout their ALS journey.
- Clinicians and their staff also experience administrative burden advocating for their patients.
- **BENEFITS NAVIGATION:** Navigating insurance coverage for existing therapies is extremely complex. New therapies that are on the horizon, when approved, will add to the complexity.
  - Education around how the therapy works, how it is administered, who is eligible to take the drug, and clear options for insurance coverage are important to help to avoid confusion and allow access to therapies as quickly as possible.



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