ALS ASSOCIATION ROUNDTABLE

ENSURING FDA-APPROVED THERAPIES REACH PEOPLE WITH ALS

April 10, 2024

EXECUTIVE SUMMARY

OVERVIEW

Since 2019, the ALS Roundtable Program has regularly assembled members of the ALS community for thoughtful, facilitated discussions about issues of priority for people living with the disease, helping shape the ALS Association's strategic planning efforts and identifying actions to continuously improve ALS care, advocacy, and research. Roundtable meetings convene a diverse group of individuals who reflect a range of ALS expertise and experiences, including people living with ALS, caregivers, clinicians, researchers, government officials, and industry partners.

On April 10, 2024, the Association convened its 11th multi-stakeholder Roundtable, providing a forum for members of the ALS community to examine gaps in and barriers to accessing therapies approved by the U.S. Food and Drug Administration (FDA) to treat ALS, with a focus on prioritizing action steps to eliminate or minimize those gaps. The meeting focused on the four therapies approved by the FDA for the treatment of ALS: riluzole (various brand names), Radicava (edaravone), Relyvrio (AMX0035),[1] and Qalsody (tofersen).

KEY TAKEAWAYS

Based on available data, less than a third of people living with ALS in the U.S. are taking an FDA-approved therapy. To better understand the factors that contribute to this low utilization rate, the agenda was designed to explore the impact of "the four P's"—providers/physicians, people living with ALS, payors, and pharmaceutical companies. However, multiple additional "P's" surfaced throughout the day that affect access to and utilization of FDA-approved therapies. These include perspective, pricing, processes, procedures, prior authorization, pharmacy benefit managers, progression of disease, paternalism, philosophy of practice, personalized, pressure, persistence, partnership, and policy.

Other overarching themes that emerged included:

- Providers face challenges determining the most effective treatment for individuals amidst a landscape of evolving data and few treatment guidelines.
- Defining optimal utilization goals for FDA-approved ALS therapies is crucial for assessing comparators and improving treatment strategies.
- Administrative barriers and cost issues contribute to undertreatment of people living with ALS. Specialty clinics may have better success overcoming these barriers due to greater capacity and experience compared to neurologists practicing in the community.
- Comparisons with diseases like Alzheimer's, epilepsy, and multiple sclerosis highlight the importance of efficacy, standard of care, and institutional buy-in for treatment optimization.

[1] The Roundtable occurred just after the release of disappointing results from the phase 3 PHOENIX trial of Relyvrio and the subsequent decision by its manufacturer, Amylyx, to voluntarily withdraw the drug from the market.

- There is a need for robust clinical trials to inform treatment decisions (including data about the impact of combination treatment), as there are differing opinions among physicians about the efficacy of FDA-approved therapies for ALS.

Overall, participants articulated that a complex, degenerative, and ultimately terminal illness like ALS serves to magnify the gaps, limitations, and challenges of the broader U.S. health care system. Therefore, with the robust drug development pipeline that is emerging for ALS, it is imperative to begin to chip away at the systematic changes that need to occur to prepare for what's to come.

A key solution to many of the issues cited throughout the Roundtable relates to enhancing timely access for all people living with ALS to multidisciplinary care and specialty clinics—both in-person and via telemedicine.[2] Policy changes also are needed to address the cost of care and require payors to provide access. Additional recommendations included:

- Developing and disseminating data that define or illustrate access gaps.
- Advocating for quality-of-care standards to enhance ALS treatment outcomes.
- Evaluating formulary patterns.
- Creating alignment among providers for how benefits and risks of approved therapies are described.
- Educating/empowering people living with ALS and their families to support shared decision-making.

NEXT STEPS

The Association will review the proposed actions generated and prioritized during the Roundtable, with the potential to create one or more working groups.

[2] The Association is addressing these issues through advocacy and other initiatives.

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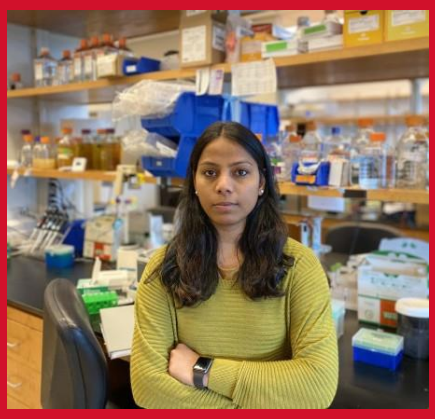

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