Transcript Details
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Advancements in Understanding ALS Treatment

Announcer
Welcome to *Neurofrontiers* on ReachMD. This medical industry feature, titled "Advancements in Understanding ALS Treatment" is sponsored by Mitsubishi Tanabe Pharma America. Here's your host, Dr. Charles Turck.

Dr. Turck:
Did you know that on average, 5,000 people are newly diagnosed with amyotrophic lateral sclerosis, also known as ALS or Lou Gehrig's disease, every year? In fact, the National ALS Registry reports there are approximately 30,000 people living with ALS in the U.S alone.[1] So given that prevalence, what are we learning about approaches we can use to treat this growing patient population?

This is ReachMD, and I'm Dr. Charles Turck. Joining me is Dr. Gustavo Suarez Zambrano, Vice President of Medical Affairs at Mitsubishi Tanabe Pharma America, to discuss clinical trial results and new real-world evidence about an established ALS treatment option. Dr. Suarez, thanks for being here today.

Dr. Suarez:
Thank you, Dr. Turck! I'm glad to be here.

Dr. Turck:
To start us off, Dr. Suarez, can you give us some background on ALS and how it affects patients?

Dr. Suarez:
Of course. ALS is a progressive neurodegenerative disease that currently has no cure. , It affects mostly the nerve cells in the brain and spinal cord, causing weakness and loss of movement.[2,3] Most cases occur without any known family history or genetic cause, this is known as "sporadic" ALS, and the cases that occur within families are known as "familial" ALS.[1] Sadly, the majority of patients with ALS die within two to five years of diagnosis,[3], which underscores the importance of early intervention.

Dr. Turck:
Yes, it's clear there's a need for that so we can work to reduce that heavy burden. And so, with that in mind, let's talk about the therapies available for ALS. What does the current treatment landscape look like?

Dr. Suarez:
Well, there are only a few treatments available for ALS. For a long time, riluzole was the only FDA-approved treatment option. But the team at Mitsubishi Tanabe Pharma America is constantly working to address this. We zeroed in on one particular therapy, edaravone, which was FDA approved as RADICAVA® IV in 2017. We then worked on developing the oral formulation, which was FDA approved as RADICAVA ORS® in 2022.[5], Recently, the FDA recognized RADICAVA ORS with Orphan Drug Exclusivity based on its major contribution to patient care due to its oral suspension route of administration that provides a less burdensome option for patients verses intravenous administration.

Dr. Turck:
Would you tell us about the clinical data that supported the FDA approval of RADICAVA?

Dr. Suarez:
Yes, in a Phase 3 trial, RADICAVA was shown to slow down the rate of functional decline by 33 percent as measured by the revised ALS Functional Rating Scale, compared with placebo at 24 weeks.[5], Patients who did not receive RADICAVA declined more rapidly in

physical function.[5] In terms of safety, the most common side effects of RADICAVA include bruising, problems walking, and headache.[5] Fatigue was also reported in patients taking RADICAVA ORS.[5] In addition, positive results from a Phase 3 study evaluating the long-term safety of RADICAVA ORS showed no new safety concerns, and that it was well-tolerated during the 96-week study period. But as always, it's important for physicians and their patients to discuss the benefits and risks associated with the treatment.

Dr. Turck:
For those just tuning in, you're listening to *Neurofrontiers* on ReachMD. I'm Dr. Charles Turck, and today I'm speaking with Dr. Gustavo Suarez Zambrano, Vice President of Medical Affairs at Mitsubishi Tanabe Pharma America, about the treatment option RADICAVA for people living with ALS. So, Dr. Suarez, if we shift over to new data, what are you finding out about RADICAVA?

Dr. Suarez:
We've conducted several extension and real-world studies that demonstrate the potential impact of RADICAVA,which can be found in the science section of our website, MPTA.com. Recently, we presented results from a Phase 3b extension study, which explored the superiority of investigational once-daily dosing versus the FDA approved on-and-off regimen of RADICAVA ORS in people living with ALS. The result was that the daily dosing did not show superiority to the approved on-and-off dosing. No significant difference in ALS Functional Rating Scale Revised Score with reductions between the two groups reinforces that the on-and-off dosing of RADICAVA ORS is the most appropriate regimen for patients with ALS. We also shared results from a Phase 3 safety extension study that further evaluated the long-term safety and tolerability of RADICAVA ORS in patients with ALS. The results found no new safety concerns and showed that RADICAVA ORS was well tolerated during the 96-week study period.

Dr. Turck:
Thanks for breaking down all of that data for us, Dr. Suarez. And as we come to the end of our conversation, do you have any final thoughts for our listeners?

Dr. Suarez:
ALS is a complex neurodegenerative disease that requires ongoing research. We at MTPA are building on a 300-year-old legacy of one of the first pharma companies in the world, and we have faced ALS head on for more than 20 years. We are committed to advancing our understanding of ALS and generating new data about RADICAVA to support healthcare providers and the ALS community in making informed treatment decisions.

Dr. Turck:
Well, with those final comments in mind, I want to thank my guest, Dr. Gustavo Suarez Zambrano, for detailing the clinical and real-word data on RADICAVA ORS. Dr. Suarez, it was great speaking with you today.

Dr. Suarez:
Thank you for having me. It was a pleasure being here. For additional details about our studies, visit the science section of MTPA.com.

Dr. Turck:
For ReachMD, I'm Dr. Charles Turck. Please stay tuned to hear some important safety information.

IMPORTANT SAFETY INFORMATION

Hypersensitivity Reactions
RADICAVA (edaravone) and RADICAVA ORS (edaravone) are contraindicated in patients with a history of hypersensitivity to edaravone or any of the inactive ingredients of this product. Hypersensitivity reactions (redness, wheals, and erythema multiforme) and cases of anaphylaxis (urticaria, decreased blood pressure, and dyspnea) have occurred with RADICAVA.

Patients should be monitored carefully for hypersensitivity reactions. If hypersensitivity reactions occur, discontinue RADICAVA or RADICAVA ORS, treat per standard of care, and monitor until the condition resolves.

Sulfite Allergic Reactions
RADICAVA and RADICAVA ORS contain sodium bisulfite, a sulfite that may cause allergic-type reactions, including anaphylactic symptoms and life-threatening or less severe asthmatic episodes in susceptible people. The overall prevalence of sulfite sensitivity in the general population is unknown but occurs more frequently in asthmatic people.

Adverse Reactions
The most common adverse reactions (≥10%) reported in RADICAVA-treated patients were contusion (15%), gait disturbance (13%), and headache (10%). In an open label study, fatigue was also observed in 7.6% of patients receiving RADICAVA ORS.

Pregnancy

Based on animal data, RADICAVA and RADICAVA ORS may cause fetal harm.
To report suspected adverse reactions or product complaints, contact Mitsubishi Tanabe Pharma America, Inc., at 1-888-292-0058. You may also report suspected adverse reactions to the FDA at 1-800-FDA-1088 or www.fda.gov/medwatch.

INDICATION
RADICAVA and RADICAVA ORS are indicated for the treatment of amyotrophic lateral sclerosis (ALS).

Please see full Prescribing Information.

Announcer:
This program was sponsored by Mitsubishi Tanabe Pharma America. If you missed any part of this discussion or to find others in this series, visit *Neurofrontiers* on ReachMD.com, where you can Be Part of the Knowledge.

1. National Institute of Neurological Disorders and Stroke. Accelerating Medicines Partnership® for Amyotrophic Lateral Sclerosis (AMP® ALS). https://www.ninds.nih.gov/current-research/focus-disorders/amyotrophic-lateral-sclerosis/accelerating-medicines-partnershipr-amyotrophic-lateral-sclerosis-ampr-als. Accessed September 2024.
2. ALS.org. What is ALS? https://www.als.org/understanding-als/what-is-als. Accessed April 2024.
3. National Institute of Neurological Disorders and Stroke. Amyotrophic Lateral Sclerosis (ALS). https://www.ninds.nih.gov/health-information/disorders/amyotrophic-lateral-sclerosis-als. Accessed April 2024.
4. Mehta P, Kaye W, Bryan L, et al. (2016). Prevalence of Amyotrophic Lateral Sclerosis in the United States using established and novel methodologies, 2017 Amyotroph Lateral Scler Frontotemporal Degener. 2023 Feb;24(1-2):108-116.
5. RADICAVA and RADICAVA ORS Prescribing Information. Jersey City, NJ: Mitsubishi Tanabe Pharma America, Inc.; 2022
6. Edaravone (MCI-186) ALS 19 Study Group. Safety and efficacy of edaravone in well defined patients with amyotrophic lateral sclerosis: a randomised, double-blind, placebo-controlled trial. Lancet Neurol. 2017;16(7):505-512
7. Shimizu H, et al. Bioequivalence study of oral suspension and intravenous formulation of edaravone in healthy adult subjects. Clin Pharmacol Drug Dev. 2021;10(10):1188-1197.

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