Raya Therapeutic Announces Publication in The Lancet Neurology of ROCK-ALS Study Data for Fasudil in ALS

Phase 2 trial showed Fasudil is safe and tolerable and provided evidence to support further investigation as a potential disease-modifying treatment for ALS.

Company Logo

MONTREAL, QUEBEC, CANADA, October 17, 2024 /EINPresswire.com/ -- Raya Therapeutic Inc., (“Raya”) a mission-driven company focused on the treatment of ALS and other neurodegenerative diseases, announces publication today in The Lancet Neurology of the Phase 2 ROCK-ALS study data for its lead compound, fasudil (RT1968) in the treatment of Amyotrophic Lateral Sclerosis (ALS). The publication is available on the Lancet Neurology website: LancetNeurology

Highlights from the ROCK-ALS Study:

• The ROCK-ALS study was a randomized, double-blind, placebo-controlled trial that enrolled 120 patients across sites in Germany, France, and Switzerland. Subjects were randomly assigned (1:1:1) to receive 30 mg of fasudil, 60 mg of fasudil, or placebo intravenously (iv) over 20 treatment days. Assessments were performed at 45, 90 and 180 days after treatment initiation.
• Primary endpoints were tolerability and safety, and secondary endpoints included, among others, motor unit number index (MUNIX), which estimates how many motor neurons innervate a muscle, and slow vital capacity (SVC), which is a measure of respiratory function.
• In the intention-to-treat population (118 participants), the primary endpoints were met.
• In the secondary endpoints, MUNIX showed a significantly reduced decline with fasudil 60 mg at 26 and 90 days, and for fasudil 30 mg at 90 days after treatment initiation. In addition, in a pre-specified subgroup analysis of female and male patients, there was a significantly slower SVC decline in females in the fasudil 60mg group at all timepoints.
• The molecular biomarkers NfL and p75ECD did not differ significantly between treatment groups, even though the absolute NfL values showed a directional decline in the fasudil 60 mg group from baseline to day 180. It is hypothesized that the magnitude of the effect would be larger with a longer treatment duration. Interestingly, a significant decrease in GFAP was noted at day 180 in the fasudil 60 mg group compared with placebo, potentially implying a decrease in astrogliosis. These results should be interpreted with caution and validated in a larger cohort.

The authors, who include the principal investigators of this investigator-initiated trial, concluded the following regarding the results of this trial: "These findings suggest that the neurophysiological MUNIX assessment can be used in multi-centre trials and might be more sensitive to change than scale-based measures, such as the well-established Revised Amyotrophic Lateral Sclerosis Functional Rating Scale (ALSFRS-R)." They further concluded that "Fasudil 30 mg and 60 mg delivered intravenously is safe and tolerable and that these findings support further investigation of this drug as a potential disease-modifying treatment for patients with amyotrophic lateral sclerosis."

Based on these encouraging findings, Raya plans to test fasudil in more extensive studies with a larger patient cohort to further assess the compound's efficacy in ALS. In addition, Raya has reformulated fasudil into a novel formulation to enhance ease of use, especially for the approximately 80% of ALS patients that suffer from dysphagia.

For more information about Raya and its innovative pipeline, visit www.rayatherapeutic.com

About Raya

Raya is a mission-driven company focused on the treatment of ALS, leveraging the latest techniques for the selection and development of disease-modifying therapies. The company has a robust pipeline of five distinct clinical stage compounds that each target different pathways involved in motor neuron degeneration seen in ALS patients. The compounds were in-licensed following a rigorous selection process based on biological plausibility, clinical target engagement and functional clinical effects reflective of efficacy. This diversified approach may enable the development of combination therapies which may have a significant impact on disease progression. To develop further combination therapies, Raya announced a strategic research collaboration with argenx on July 12, 2023: Link

This partnership explores synergistic effects of their combined drug candidates in the hope of developing more effective treatment options for ALS patients. Raya is further supported by global experts and will leverage a unique patient-friendly platform trial design offering new hope in the fight against ALS.

About Fasudil

Fasudil is a small molecule inhibitor of the rho-associated kinase (ROCK) and is approved for the treatment of subarachnoid hemorrhage in a few Asian countries. In preclinical studies, fasudil attenuates neurodegeneration, modulates neuroinflammation, and fosters axonal regeneration.

Contact Information for Media Inquiries: admin@rayatherapeutic.com

Anjan Aralihalli
Raya Therapeutic, Inc.
admin@rayatherapeutic.com
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