Raya Therapeutic Announces Oral Presentation and Poster at International Symposium on ALS/MND in Montreal December 6-8

Dr. Paul Lingor to present Phase 2 ROCK-ALS study. Raya also presenting promising new preclinical data of a combination of two new chemical entities (NCEs).

Company Logo

MONTREAL, QUEBEC, CANADA, December 5, 2024 /EINPresswire.com/ -- Raya Therapeutic Inc., (“Raya”) a mission-driven company focused on the treatment of ALS and other neurodegenerative diseases, announces the presentation of the Phase 2 ROCK-ALS study data for its lead compound, fasudil (RT1968) for the treatment of Amyotrophic Lateral Sclerosis (ALS) by Dr. Paul Lingor (Principal Investigator) on Friday, December 6th at the 35th International Symposium on ALS/MND in Montreal. The results were previously published in the Lancet Neurology on November 1st. LancetNeurology

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. The authors 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."

The company would also like to announce a poster presentation by Kim Staats, PhD, on December 7th, entitled: "The novel combination of 2 clinical-stage molecules demonstrates a beneficial effect in preclinical models of ALS."

Raya is also developing 2 novel small molecules, RT1972 and RT1999, each of which have neuroprotective effects in ALS preclinical models, are oral small molecules, cross the blood-brain-barrier, have been tested in patients in other indications, have favorable safety profiles, and are not yet approved for any indication. Both drugs are neuroprotective in many different preclinical models of ALS and of neurodegeneration in general by affecting processes such as ER stress, autophagy, and growth factor production.

In co-culture studies with a neurotoxic stressor, the combination of RT1972 and RT1999 protects the innervation of muscle fibers more than each drug separately. In addition, the combination also slowed disease progression and affected the transcriptome in a pilot study with a mouse model of ALS to a greater extent than each drug on its own.

Raya believes that this is the first presentation of a combination of two new chemical entities across multiple preclinical models of ALS.

In addition to testing fasudil (RT1968) in more extensive studies with a larger patient cohort to further assess the drug's efficacy in ALS, Raya also plans to advance the clinical development of RT1972 and RT1999, both as monotherapies and in combination with each other, for the treatment of ALS.

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 enables 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, 2024: 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 supports axonal regeneration.

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