Raya Therapeutic Announces FDA and EMA Orphan Drug Designations for ALS with RT1978 (epertinib)

The orphan drug designations (ODDs) are from both the FDA & EMA and for the treatment of Amyotrophic Lateral Sclerosis (ALS).

Company Logo

MONTREAL, QUEBEC, CANADA, March 3, 2025 /EINPresswire.com/ -- Raya Therapeutic Inc., (“Raya”) a mission-driven company focused on the treatment of ALS and other neurodegenerative diseases, announces that it has received orphan drug designations (ODD) from the U.S. Food and Drug Administration (FDA) and the European Medicines Agency (EMA) for the treatment of ALS with RT1978 (epertinib).

The company now has 11 ODDs across its portfolio of 5 clinical stage, oral, small molecule, new chemical entities (NCEs).

Epertinib (RT1978) was originally discovered and developed by Shionogi & Co., Ltd., a leading pharmaceutical company based in Japan. This molecule has completed Phase 2 clinical development in another indication and has demonstrated significant promise in multiple preclinical models in ALS, a progressive neurodegenerative disease with no known cure. The ODDs will provide Raya with various development incentives, including market exclusivity, tax credits for clinical research, and fee waivers, which will expedite the advancement of epertinib to benefit ALS patients.

"We are pleased to receive Orphan Drug Designations from both the FDA and EMA for the treatment of ALS with epertinib," said Anjan Aralihalli, President and Founder of Raya Therapeutic. "We are excited about the drug’s potential to treat ALS by EGFR inhibition.”

“The preclinical data is promising, and we are hopeful to see such a well-developed compound now openly enter ALS development,” said Professor Leonard van den Berg, Chairman of TRICALS (Treatment Research Initiative to Cure ALS) and Clinical Faculty Professor at UMC Utrecht, “moreover, we are hopeful for upcoming clinical development, as this compound is already clinical-stage and as ALS represents such an urgent unmet need.”

Members of the management team will be available during the 25th annual Packard Center (for ALS Research) Symposium March 3 – 5th in Baltimore, Maryland and the Muscular Dystrophy Association's Annual Scientific Conference March 16 – 19th in Dallas, Texas.

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 in ALS. 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, 2023: Link. This partnership explores combinatorial effects of combined drug candidates in the hope of developing more effective treatment options for ALS. Raya is also supported by global experts and leverages unique patient-friendly trial designs offering new hope in the fight against ALS.

About Shionogi & Co., Ltd.

Shionogi & Co., Ltd. is a Japanese pharmaceutical company committed to discovering and developing novel medications to address global health challenges. Since its founding in 1878, Shionogi has been dedicated to improving patient lives through scientific innovation and collaboration.

About Amyotrophic Lateral Sclerosis

Amyotrophic lateral sclerosis (ALS) is a rare, progressive and fatal neurodegenerative disease that results in the loss of motor neurons in the brain and the spinal cord that are responsible for controlling voluntary muscle movement. People with ALS experience muscle weakness and atrophy, causing them to lose independence as they progressively lose the ability to move, speak, eat, and eventually breathe. Average life expectancy for people with ALS is three to five years from time of symptom onset. (National Institute of Neurological Disorders and Stroke. Amyotrophic Lateral Sclerosis (ALS). Available at: https://www.ninds.nih.gov/health-information/disorders/amyotrophic-lateral-sclerosis-als. Accessed: January 2025)

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