Raya Therapeutic Announces 9 Orphan Drug Designations Across its 5 Clinical Stage Compounds

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

Company Logo

MONTREAL, QUEBEC, CANADA, January 9, 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 9 orphan drug designations (ODD) by the U.S. Food and Drug Administration (FDA) and the European Medicines Agency (EMA) across its portfolio of clinical stage, oral, small molecule, new chemical entities (NCEs). The company anticipates receiving additional ODDs through 2025.

“Raya Therapeutic is committed to addressing the need of persons living with rare neurodegenerative diseases, such as ALS, and we are grateful to have received (thus far) 9 ODDs from the FDA and EMA,” commented Anjan Aralihalli, President of Raya Therapeutic. “In addition to the positive results from preclinical studies, the ODDs provide support for the therapeutic potential of Raya’s pipeline of 5 clinical stage compounds, for which each is neuroprotective by targeting multiple biological mechanisms simultaneously.”

Members of the management team will be available during the JP Morgan Annual Healthcare Conference, held on January 13 – 16th, 2025 in San Francisco, California.

The J.P. Morgan Healthcare Conference is the industry's leading global investment forum, bringing together international pharma and biotech companies as well as investors across the healthcare ecosystem.

In December 2024 Raya announced the presentation of the Phase 2 ROCK-ALS study data for its lead compound, fasudil (RT1968) at the 35th International Symposium on ALS/MND in Montreal. The results of the Phase 2 ROCK-ALS trial were previously published in the Lancet Neurology on November 1st.

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 additional combination therapies, Raya announced a strategic research collaboration with argenx in July 2023. 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 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 steadily 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)

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