

Raya Therapeutic Announces Selection of one of its 5 compounds onto the EXPERTS-ALS Clinical Platform Trial

Positive data from this clinical study could lead to a registration trial of the compound for the treatment of ALS/MND.

MONTREAL, QUEBEC, CANADA, September 2, 2025 /EINPresswire.com/ -- Raya Therapeutic Inc., ("Raya") a mission-driven company focused on the

treatment of ALS and other neurodegenerative diseases, announces that one of its five clinical stage compounds has been selected for inclusion in the EXPERTS-ALS (EXPERimental medicine Route To Success in ALS) Platform Trial with Sheffield University.



This marks a key milestone as Raya advances this compound towards approval for the treatment of amyotrophic lateral sclerosis (ALS) also known as motor neuron disease (MND) and independently validates the compound's potential as an ALS treatment.

EXPERTS-ALS is led by Professor Chris McDermott at the University of Sheffield and Professor Martin Turner at the University of Oxford. It involves 11 clinical centres around the UK and is sponsored by Sheffield Teaching Hospitals NHS Foundation Trust. It is a flagship study within the broader portfolio of the UK MND Research Institute and is funded by the National Institute for Health and Care Research (NIHR) and Biomedical Research Centre Capacity Building funding schemes. Patient charities MND Association, My Name's Doddie Foundation, the Darby Rimmer Foundation, and medical research charity LifeArc are providing additional support.

The mission of the National Institute for Health and Care Research (NIHR) is to improve the health and wealth of the nation through research. This is done by:

- Funding high quality, timely research that benefits the NHS, public health and social care;
- Investing in world-class expertise, facilities and a skilled delivery workforce to translate discoveries into improved treatments and services;
- Partnering with patients, service users, carers and communities, improving the relevance, quality and impact of our research;
- Attracting, training and supporting the best researchers to tackle complex health and social

care challenges;

- Collaborating with other public funders, charities and industry to help shape a cohesive and globally competitive research system;
- Funding applied global health research and training to meet the needs of the poorest people in low and middle income countries.

NIHR is funded by the Department of Health and Social Care. Its work in low and middle income countries is principally funded through UK international development funding from the UK government.

Raya's compound 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 company 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 this compound.

"We are proud that one of Raya's 5 clinical stage New Chemical Entities (NCE), has been selected for inclusion in the EXPERTS-ALS Platform Trial. This milestone underscores our conviction that the compound's differentiated mechanism is uniquely suited to address the complexity of ALS. More information will be shared at a future scientific meeting." said Anjan Aralihalli, President and Founder of Raya Therapeutic.

Professor Christopher McDermott, co-lead of EXPERTS-ALS, co-director of the new UK MND Research Institute, Professor of Translational Neurology at the University of Sheffield and Honorary Consultant Neurologist at Sheffield Teaching Hospitals NHS Foundation Trust, said: "MND is a cruel and devastating disease and we need new approaches to identify more effective treatments to help patients. EXPERTS-ALS is a pioneering project to prioritise the drugs which have the best chance of success in halting the progression of this terrible degenerative disease. Over five years, we will be able to screen drugs faster, on a larger scale and identify which ones should proceed into phase 3 trials based on signals found in people living with MND."

Mr. Aralihalli will be presenting Raya at the UCL Queen Square MND Centre Sixth ALS/MND International Symposium in Partnership with the UK MND Research Institute on Friday, September 5, 2025.

[Event information.](#)

For more information about Raya and its innovative pipeline and to listen to a recent podcast from Anjan Aralihalli telling the Raya story and his thoughts on ALS, 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 Amyotrophic Lateral Sclerosis (ALS) or Motor Neuron Disease (MND)

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. There are about 5,000 people in the UK who are thought to have the condition (NIHR). 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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