

Raya Therapeutic unveils its Raya CARES pipeline of five clinical-stage compounds

Each new chemical entity (NCE) has substantial clinical safety data and a unique mechanism of action to treat ALS, PD, and other neurodegenerative diseases.

MONTREAL, QUEBEC, CANADA, December 4, 2025 /EINPresswire.com/ -- Raya Therapeutic Inc., ("Raya") a mission-driven company focused on the treatment of amyotrophic lateral sclerosis (ALS), Parkinson's Disease (PD) and other neurodegenerative diseases, announces the unveiling of its "Raya CARES" pipeline—a portfolio of five advanced clinical-stage compounds designed with differentiated mechanisms of action to address the multifactorial biology of neurodegenerative diseases.

The Raya CARES pipeline includes Cutamesine, fAsudil, Rovatirelin, Epertinib and Smilagenin. It reflects Raya's bold commitment to advancing therapies that protect neuronal health and survival. Each compound has a distinct mechanism of action, offering complementary approaches to slowing or halting disease progression. By leveraging compounds with established human safety data (each compound has completed at least Phase 2 development involving other CNS-related indications), Raya will accelerate their development.

With financing, Raya expects to advance all five compounds into large Phase 2 or Phase 3 clinical trials within 12-18 months.

Pipeline Overview

RT1972 (Cutamesine) – Sigma-1 receptor agonist (S1RA)

- RT1972 is neuroprotective by activating the sigma-1 receptor, a key regulator of endoplasmic reticulum-mitochondrial signaling.
- This drug has already completed two Phase 2 trials in other CNS indications demonstrating blood-brain barrier (BBB) penetration and safety.
- Raya believes that RT1972 is the most selective and potent S1RA in development and has potential in multiple indications including Parkinson's, Alzheimer's, Huntington's and in other neurodegenerative and rare diseases.

RT1968 (fAsudil) – ROCK (Rho-associated kinase) inhibitor

- Fasudil modulates cytoskeletal dynamics, reduces neuroinflammation, and promotes axonal regeneration, targeting both neurons and glia in ALS.

- By inhibiting ROCK, fasudil decreases microgliosis, enhances oligodendrocyte survival and remyelination.
- Approved in Asia for cerebral vasospasm; extensive human safety record.
- Demonstrated clinical proof of concept in a large Phase 2 study in people with ALS; the study results were published in The Lancet Neurology ([LINK](#)) in November 2024.

RT2010 (Rovatiorelin) – TRH (thyrotropin-releasing hormone) analog

- Rovatiorelin stimulates central nervous system pathways that enhance motor neuron outgrowth and provide protection.
- Raya is collaborating with Shionogi & Co., Ltd. which discovered the drug and Kissei Pharmaceutical Co., Ltd. which is developing it in a Phase 3 confirmatory study in patients with spinocerebellar ataxia (SCA) that is expected to complete enrolling in March 2027. ([NCT07040137](#)).

RT1978 (Eperitinib) – HER2/EGFR Tyrosine Kinase Inhibitor (TKI)

- Originally developed as an oncology agent that completed Phase 1/2 clinical trial development, eperitinib crosses the blood–brain barrier and modulates receptor tyrosine kinase signaling.
- In neurodegeneration, this mechanism may promote neuronal survival through trophic signaling and stimulating autophagy as well as reducing abnormal glial activation.
- Raya is collaborating with Shionogi & Co., Ltd., which discovered and initially developed the drug.

RT1999 (Smilagenin) – Neurotrophic factor modulator

- Orally active, non-peptide inducer of neurotrophic factors with blood–brain barrier penetration and extensive Phase 1/2 safety data.
- Enhances production of BDNF and GDNF, supporting neuronal survival and outgrowth.
- Differentiated mechanism with broad potential to treat neurodegenerative disorders.
- RT1999 completed Phase 2B development in Parkinson's Disease. While the drug missed its primary endpoints, a subsequent post-hoc analysis suggested a significant slowing of the progression of motor symptoms.

"The Raya CARES pipeline represents our conviction that ALS, PD and neurodegenerative diseases demand a multipronged therapeutic strategy," said Anjan Aralihalli, President & Founder of Raya Therapeutic. "In addition to advancing each compound for development in ALS and other neurodegenerative diseases, the company has identified synergistic combinations, which it will advance as subsequent life cycle management opportunities. Combination therapies will likely be necessary to fully treat heterogeneous diseases such as ALS, PD and AD."

Members of the Raya management team will attend the 36th International Symposium on ALS/MND December 4-7th in San Diego, California.

Members of the management team will also be available during the JP Morgan Annual Healthcare Conference January 12 – 15th, 2026 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.

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, PD and other neurodegenerative diseases, 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.

About Shionogi & Co., Ltd.

Shionogi & Co., Ltd. is a leading global research-driven pharmaceutical company dedicated to bringing benefits to patients based on its corporate philosophy of "supplying the best possible medicine to protect the health and well-being of the patients we serve." Shionogi has discovered and developed novel antibiotics, medicines for HIV and influenza and currently markets medicines for infectious diseases and central nervous system disorders. Shionogi's global pipeline includes research programs in infectious disease, pain/CNS, metabolic disorders, rare disease, oncology and stroke.

For more information, please visit <https://www.shionogi.com/global/en/>.

About Kissei Pharmaceutical Co., Ltd.

Kissei Pharmaceutical Co., Ltd. is a Japanese pharmaceutical company based on the management philosophy "contributing to society through high-quality, innovative pharmaceutical products" and "serving society through our employees." As a strong R&D-oriented corporation, it concentrates on providing innovative pharmaceuticals to patients worldwide. Kissei Pharmaceutical Co., Ltd. is engaged in bringing new drugs into the world through drug discovery and licensing activities in its focus fields of rare/intractable diseases, urology, and nephrology/dialysis.

For more information about Kissei Pharmaceutical Co., Ltd., please visit https://www.kissei.co.jp/e_contents/.

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