PLL Therapeutics reports positive safety and tolerability results from phase I/II trial in ALS (Amyotrophic Lateral Sclerosis)

Stage one results from PLL Therapeutics' first-in-human, multi-stage phase I/II trial show favorable safety and tolerability of PLL001 in 12 ALS patients

Australian multi-stage study tested single ascending subcutaneous doses of PLL001 – in three dose cohorts - with placebo control

Villenave-d'Ornon (near Bordeaux), France, February 18, 2026 – PLL Therapeutics, a biopharmaceutical company developing a groundbreaking polypeptide delivery platform to treat the root cause of autoimmune and neurodegenerative diseases, today announces positive results in the first stage of its phase I/II clinical trial in Amyotrophic Lateral Sclerosis (ALS), demonstrating a favorable safety and tolerability profile for its investigational therapy PLL001.

The administration of a single ascending dose in the first stage of PLL Therapeutics' phase I/II study successfully met its primary objectives of showing safety and tolerability in 12 ALS patients. The study, led by Prof. Susan Mathers, lead investigator, was conducted across multiple sites in Australia: Alfred Health, Calvary Health Care and Wesley Research Institute. It was designed to evaluate the safety and tolerability of single ascending doses of PLL001, in which patients were assigned to one of three dose cohorts during the first seven days, with a single administration of PLL001 or placebo in a double-blind manner.

The first stage of the phase I/II study demonstrated a favorable safety and tolerability profile, with no Serious Adverse Event (SAE) reported and no Treatment Emergent Adverse Event (TEAEs) resulting in study discontinuation.

"We are very encouraged by the phase I results in the 12 patients with ALS, which validate the safety profile of PLL001," said Jean-Pascal Zambaux, co-founder and CEO of PLL Therapeutics. "This is a critical step forward in our mission to restore the intestinal epithelium barrier, thereby treating the root cause of ALS – a disease linked to the dysbiosis of the gut. Our teams in Australia, New Zealand and France are diligently advancing this program into phase II."

The successful completion of this clinical phase paves the way for a second-stage trial, involving 140 ALS patients in Australia and New Zealand, to test the efficacy of PLL001, and for future 'compassionate use' programs. This next phase, due to start in Q2 of 2026, is scheduled to run for one year, during which patients will receive a daily injection of either PLL001 or a placebo over a six-month treatment period.

PLL001 is designed to address ALS at its onset and slow or halt disease progression. It is derived from PLL Therapeutics' polypeptide delivery platform that leverages a Poly-L-Lysine conjugate, a peptide chain engineered to extend the *in vivo* half-life of active compounds. PLL001 aims at restoring the intestinal epithelium barrier through the delivery of targeted Small-Chain Fatty Acids (SCFAs) to gut epithelial cells and the Blood-Brain Barrier (BBB), helping restore the normal function of cells lining the gut, as well as the BBB. Tightening the junctions between cells, sealing the gut lining to prevent leakage of harmful substances into the bloodstream and Central Nervous System, helps reduce inflammation.

Although ALS is classified as a rare disease, research findings indicate that it will impact a growing proportion of the global population over the coming decades. There are approximately between 4 and 8 people per 100,000 living with ALS worldwide, with its prevalence projected to increase by 30% across multiple countries by 2040, when taking into account aging populations and improved survival rates.

About PLL Therapeutics

PLL Therapeutics, a biopharmaceutical company, is spearheading a unique therapeutic approach for treating the root cause of autoimmune and neurodegenerative diseases based on a groundbreaking polypeptide delivery platform. In parallel, through specific biomarkers, it is advancing the early-stage diagnosis of Amyotrophic Lateral Sclerosis (ALS), a fatal motor neuron disease, and proliferative disorders (i.e. colon cancer). PLL Therapeutics focuses on restoring gut integrity. Its lead candidate is PLL001, a 'poly-targeted' drug therapy that aims at tackling the disease at its inception.
Founded in 2019, PLL Therapeutics is led by a highly experienced management team with decades of expertise in the CNS (Central Nervous System) and rare diseases. The company recently completed a phase I clinical trial, with a phase II underway. PLL Therapeutics is located near Bordeaux, France, and operates two subsidiaries in Asia-Pacific.
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