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Ferrer reports Top-line Results from Phase III ADORE study in ALS

Ferrer reports that Phase III ADORE clinical trial of oral edaravone formulation (FAB122) in amyotrophic lateral sclerosis (ALS) patients **did not meet primary or key secondary endpoints**.

The ADORE clinical trial is a multicenter, multinational, double-blind, randomized, placebo-controlled Phase III study to investigate the efficacy and safety of 100 mg edaravone (FAB122) once daily as oral formulation in ALS patients, during a 48-week period. Study participants were randomized in a 2:1 ratio to receive oral edaravone or placebo while continuing to receive their existing standard of care treatment for ALS. It was conducted with the support of TRICALS, the largest European research initiative focused on finding a cure for ALS.
Data from ADORE indicates that product did not show significant benefit over placebo in patients with ALS in slowing the disease progression as measured by change from baseline in the ALSFRS-R score after 48 weeks of daily dosing with the oral edaravone formulation. No improvement over placebo on long-term survival was observed as measured by CAFS at 48 weeks and 72 weeks for a subgroup of patients. The results of the study also concluded that **the product was safe and well-tolerated**.

Tatjana Naranda, Ferrer's Chief R&D Officer, explains "*we are disappointed with this outcome and would like to thank the people with ALS, caregivers, investigators and clinical trial staff for their participation in ADORE clinical trial. Although the results are not what we hoped for, our work to advance potentially transformative therapies in areas of high unmet need for people living with ALS will not stop*".
Prof. Leonard H. van den Berg, Chair of TRICALS, Professor of Neurology at the University Medical Centre Utrecht, the Netherlands, and Principal Investigator of the study declares: "*At TRICALS, we had hoped for a different outcome of the ADORE study and are disappointed that this is not the positive result we all wanted. We will continue to do our utmost to find a treatment for ALS as soon as possible. We want to thank all the patients who dedicated their time and effort to this trial and Ferrer for the excellent collaboration, which we hope to repeat in the future*."

The company also announces that its open-label extension **study ADOREXT will be now concluded**, based on the lack of efficacy of the oral edaravone formulation (FAB122).

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Amyotrophic lateral sclerosis (ALS) [1], the most frequent motor neuron disease, is a progressive neurodegenerative disease of motor neurons in the brain and spinal cord, resulting in progressive paralysis, with death typically within 2 to 5 years of diagnosis. ALS is a rare disease with multifactorial etiology, and the precise pathogenic mechanism is still unknown. ALS typically occurs in people between 40-70 years old, slightly more men than women. It is caused by a multitude of factors: 10-15% familiar ALS, 85-90% sporadic ALS.

[1] Masrori and Van Damme; Amyotrophic lateral sclerosis: a clinical review. European Journal of Neurology 2020, 27: 1918– 1929

Source: **Ferrer**

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