



## PRESS RELEASE

### ILTOO Pharma announces the results of a Phase IIb trial for ALS using Low-Dose IL-2

Paris, 6 December 2022

ILTOO Pharma SAS today announced the results of MIROCALS, a Phase IIb trial in amyotrophic lateral sclerosis (ALS) using low-dose Interleukine-2 (IL-2<sub>LD</sub>), which were presented by Dr. Gilbert Bensimon at the International Symposium on ALS / Motor Neuron Diseases.

The results of the MIROCALS trial demonstrate a **statistically significant efficacy of IL-2<sub>LD</sub>** in the treatment of patients with ALS. Clinical analyses, adjusted on the pNFH biomarker that reflects brain tissue damage, showed a **large and significant effect on survival** and **slowed disease progression** quantified as **48% decrease in risk of death** over 21 months and a **24% reduction in functional decline** (3.12 pts/yr on the ALSFRS scale) for **patients with low pNFH representing 70% of the trial population**.

Professor David Klatzmann, who pioneered the use of IL-2<sub>LD</sub> for the treatment of autoimmune diseases and is a founder of ILTOO Pharma and its Chairman of the Scientific Advisory Board, said, *“Low-dose IL-2 immunotherapy represents a whole new class of treatment for ALS by its ability to harness the regulatory T cell neuroprotective activities. This represents an unprecedented therapeutic approach. The MIROCALS results strongly support a therapeutic benefit of IL-2<sub>LD</sub> as an add-on therapy to Riluzole in the treatment of patients suffering from ALS.”*

Commenting on the results, José Achache, Chairman of ILTOO Pharma, said, *“As early as 2012, ILTOO Pharma began pioneering the treatment of autoimmune and inflammatory diseases using low-dose Interleukin-2 leading to a deep pipeline of activity. We are now extremely satisfied and proud of the results of the MIROCALS study showing the efficacy of our treatment in ALS. The team at ILTOO Pharma will now be wholly focused on satisfying regulatory requirements in order to provide the treatment as soon as possible to serve this major unmet medical need.”*

#### About MIROCALS:

MIROCALS (NCT03039673) is an acronym for Modifying Immune Response Outcomes in ALS. It is a randomized, placebo-controlled, double-blind, parallel group phase IIb study. Its main objective was to evaluate the clinical efficacy on survival, functional decline and safety of IL-2<sub>LD</sub> as an add-on therapy to Riluzole to establish that modifying immune responses through the enhancement of regulatory T cells modifies the rate of ALS progression. MIROCALS was funded by an EU *Horizon 2020* grant.

#### About ALS:

ALS is an adult-onset motor neuron disease and is usually diagnosed between the ages of 40 and 70. It is a rare and life-threatening neurodegenerative disease. There is currently no cure for ALS and no effective treatment to stop or reverse the progression of the disease. The disease affects about 42,000 patients in Europe and approximately 20,000 patients in the US.

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ILTOO Pharma is dedicated to the development of immunotherapies with IL-2<sub>LD</sub> for the treatment of auto/neuroimmune and inflammatory disorders. The company is based in central Paris near to a thriving community of Bio Pharma research and commercialisation.

For further information, please visit our website at: [www.iltoopharma.com](http://www.iltoopharma.com)