



PRESS RELEASE

ILTOO Pharma announces that ILT-101 has achieved Orphan Drug Designation

Paris, 23 May 2023

ILTOO Pharma SAS today announced that the European Medicines Agency (EMA) and the U.S. Food and Drug Administration (FDA) have **granted Orphan Drug Designation (ODD) status for ILT-101**, the company's lead drug for the treatment of amyotrophic lateral sclerosis (ALS).

Orphan drug designation is a special status granted by both the EMA and FDA to drugs and biologics intended for the treatment, diagnosis or prevention of rare, life-threatening or chronically debilitating diseases or conditions that impact fewer than 5 in 10,000 patients in the European Union (EU) or fewer than 200,000 people in the United States (US) respectively. To qualify, an investigational medicine must show sufficient non-clinical or clinical data to suggest it may produce clinically relevant outcomes. This designation allows companies certain benefits including scientific advice and assistance with drug approval processes, reduced regulatory fees and market exclusivity following regulatory approval for 10 years in the EU and 7 years in the US.

The use of ILT-101 represents an unprecedented therapeutic approach for treating the deleterious neuro-inflammatory component of ALS. Low dose IL-2 (IL-2_{LD}) represents a new class of immunomodulatory treatment for ALS patients. ILT-101 is available as a proprietary **liquid, low dose and ready-to-use formulation** of IL-2, **more suitable for long-term treatment** and providing a **better usage experience for patients**.

The active substance of ILT-101 is human recombinant interleukin-2 (IL-2). When administered at low dose, IL-2 selectively expands and activates regulatory T cells (Tregs) while having no impact on effector T cells (Teffs). This dose-driven mechanism of action allows the patient's own immune system to reinstate a proper Tregs/Teffs balance. Tregs play an indispensable role in maintaining immune homeostasis and so-called self-tolerance (*Klitzmann & Abbas 2015*).

Professor David Klitzmann, founder of ILTOO Pharma and its Chairman of the Scientific Advisory Board, pioneered the field of IL-2_{LD} for treating autoimmune and inflammatory diseases by conducting the first study ever in patients with HCV-induced vasculitis (*New England Journal of Medicine*, 'VASCU-IL2', *N Engl J Med 2011;365:2067-77*).

Commenting on ODD status, Prof. Klitzmann said, *"Since the publication of this seminal research in the New England Journal of Medicine in December 2011, we have been hard at work establishing the scientific basis for the treatment of several autoimmune disorders (Klitzmann & Abbas 2015). In particular, recent **clinical trial results in ALS showing a large and significant effect on survival** are an encouraging sign for patients afflicted with this terrible disease."*

Jeff Lyons, CEO of ILTOO Pharma, also commented, *"Attaining Orphan Drug Designation for ILT-101 for treating Amyotrophic Lateral Sclerosis is a great achievement for ILTOO Pharma. It will accelerate our development timeline and enhance the approval pathway for ILT-101. Now we will continue to work closely with the relevant agencies in order to achieve full regulatory approval to supply ILT-101 to patients suffering from ALS."*



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About Amyotrophic lateral sclerosis:

Amyotrophic lateral sclerosis (ALS), also known as Charcot's disease or Lou Gehrig's disease, is an adult-onset motor neuron disease and is usually diagnosed between the ages of 40 and 70. It is a life-limiting neurodegenerative disease that affects about 42,000 patients in Europe and approximately 20,000 patients in the US. There is currently no cure for ALS and no effective treatment to stop or reverse the progression of the disease.

About ILTOO Pharma:

ILTOO Pharma is dedicated to the development of immunotherapies with IL-2_{LD} 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