



OneChain Immunotherapeutics achieves FDA Orphan Drug designation for OC-1, a CAR T therapy against an aggressive subtype of T-cell leukemia

- This designation provides the company with benefits such as market exclusivity, scientific advice, and significant economic benefits.
- OC-1 is a CAR T targeting the CD1a antigen, a protein found in patients' tumors with cortical T-cell acute lymphoblastic leukemia, an aggressive subtype of leukemia with few therapeutic options.
- The company has already initiated the CARxALL clinical trial to evaluate the safety and efficacy of the product in humans.

Barcelona, October 3rd, 2023. Spanish biotech OneChain Immunotherapeutics' (OCI) product OC-1 has received **Orphan Drug designation from the U.S. Food and Drug Administration (FDA)** for the treatment of acute lymphoblastic leukemia (ALL). This designation, granted to drugs and biologics intended to treat, diagnose, or prevent diseases affecting fewer than 200,000 people in the United States, **will provide the company with several benefits** such as tax credits, exemption from certain regulatory fees, market exclusivity and mentoring during clinical development.

"The Orphan Drug designation is a significant recognition that underscores the urgency and **need for innovative treatments for this disease**," says **Dr. Wilmar Castillo**, the company's director of Clinical Operations. "This is a crucial step in our journey to bring hope to patients who have no other therapeutic options," she adds.

Acute lymphoblastic leukemia is a type of hematological cancer characterized by the excessive production of immature lymphocytes, which invade the blood, bone marrow and lymphatic tissues, with the ability to spread to other parts of the body within a few months. This rare, aggressive cancer accounts for **less than one-half of 1% of all cancers in the United States**, and can be classified into several subtypes, each with even lower prevalence, depending on factors such as the type of lymphocyte affected and the stage of cell development. OCI's therapy is focused on one of these subtypes.

"The reality is that **our product is directed at a very specific target, the CD1a antigen**, which is expressed almost exclusively on the tumor cells of patients with **cortical T-cell acute lymphoblastic leukemia**," explains Dr. Castillo. "For these patients, the first line of treatment is usually effective, but the prognosis for those who do not respond to existing therapies is very unfavorable."

This recognition for OC-1 is in addition to the designation it already received from the European Medicines Agency (EMA) in 2021, both granted based on **strong preclinical data supporting the efficacy and safety of the treatment**. "The granting of these designations reflects the confidence and potential that the regulatory agencies see in our product. It is a testament to the robustness of our data and the value OC-1 can bring in the fight against this pathology," she says.

The company initiated the **CARxALL clinical trial in January 2023** to evaluate the safety and efficacy of the product in patients with cortical T-cell acute lymphoblastic leukemia with no therapeutic alternative. As Dr. Castillo notes, "These achievements are a testament to the company's progress and the potential of its approach to treating malignancies," Castillo concludes.

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About OneChain Immunotherapeutics

OCI was founded by the **Josep Carreras Leukemia Research Institute, ICREA and Dr. Pablo Menéndez** in Barcelona in June 2020, with a first round of funding led by Invivo Ventures with CDTI-Invierte (Ministry of Industry) and the Josep Carreras Foundation.

The company based at the Barcelona Science Park (PCB) aims to develop **immunotherapy-based treatments for malignant neoplasms**, based on the research results of the group led by Dr. Pablo Menéndez, ICREA research professor and researcher at the Josep Carreras Leukemia Research Institute.

In addition to the above and most advanced, the company is developing three projects: 1) The OC-2 product, a **CAR T** that will be used to treat patients with **B-cell acute lymphoblastic leukemia**. 2) A platform in development of **V δ 1 cells for allogeneic application**, which will provide ready-to-use treatments at a lower cost than current autologous CAR T treatments. And 3) Finally, a **dual CAR T** is being developed for the treatment of **Glioblastoma**, a highly aggressive brain tumor with a fatal prognosis.

OCI has recently closed a **pre-series A investment round of 6.7 million euros**. The deal involved venture capital firm **Invivo Capital, the Center for Technological Development and Innovation**, venture capital firms **Nara Capital and Clave Capital**, and the **Josep Carreras Leukemia Foundation**.