Rare Disease Day: OneChain Immunotherapeutics has received 1.3M euro grant to advance a new promising CAR T therapy for a rare leukemia with no therapeutic alternatives

- **A consortium led by the Spanish company OneChain Immunotherapeutics has received 1.9M euros from the Spanish Ministry of Science and Innovation to advance a groundbreaking therapy for acute lymphoblastic leukemia type T into clinical phase.**
- **The therapy, based on CAR T technology, targets two new therapeutic markers with minimal presence in healthy tissues, enhancing treatment efficacy and the number of treatable patients.**
- **This therapeutic approach builds on the success of a previous therapy developed by the same company, which is currently being evaluated in the CARxALL Phase 1 clinical trial.**

Barcelona, February 29th 2024. The consortium led by the company **OneChain Immunotherapeutics** (OCI) located at the Barcelona Scientific Park (PCB) has received almost **2 million euros** from the Spanish Ministry of Science and Innovation to bring an **innovative therapy** to the clinical phase. This therapy could treat up to 80% of patients with **T-cell acute lymphoblastic leukemia** (T-ALL), a rare condition with few therapeutic options, affecting children (60%) and adults. The project, set to last three years, will involve **collaboration with the Josep Carreras Institute and the Blood and Tissue Bank** (Banc de Sang i Teixits).

The treatment of acute lymphoblastic leukemia type T, **one of the most aggressive forms of leukemia**, has historically relied on intensive chemotherapy. Despite improvements in survival rates, this therapy results in devastating effects for patients. Furthermore, a significant percentage of them **do not respond to treatment**. "For patients who have experienced a relapse, finding effective treatments is particularly challenging," explains Dr. Víctor M. Díaz, research director at the company. **"Our therapy brings hope to these patients."**

OCI's approach is based on **CAR T technology**, a type of immunotherapy that involves extracting immune cells from the patient to modify them in the laboratory, enhancing their ability to recognize and attack cancer cells and has shown great promise in other forms of leukemia and lymphoma. "**Our aim is** to develop this therapy over the three years of the project to **initiate a first clinical trial in humans,"** Dr. Diaz adds.

Attacking the same cell through different targets

The therapy developed by OCI will **simultaneously target two markers** present on cancer cells, making it **more effective** than single-target therapies and **expanding the number of treatable patients.** CAR T therapy has not yet become a consolidated option against T-cell leukemias, such as T-ALL, because tumor and healthy T cells exhibit almost the same molecules on their surface. As a result, CAR T therapies aimed at T-ALL destroy both types of cells, leading to severe immunosuppression in patients.

OCI has already developed a CAR-T therapy directed at the CD1a protein, a safe target with little presence in healthy cells. However, this molecule is present in cortical T-ALL (coT-ALL), a subset of T-ALL, representing only 30-40% of T-ALL patients. "The first CAR T therapy we developed, targeting the CD1a antigen, is already in clinical trials, demonstrating its safety and efficacy for a specific group of patients," Dr. Diaz explains. **"By incorporating a second target into our strategy, we not only increase the percentage of patients we could treat—up to 80%—but also enhance the effectiveness of the therapy."**

The grant has been awarded in the **call for public-private collaboration projects by the Spanish Ministry of Science and Innovation**. It is the result of recognition awarded to projects that have already reached an advanced phase of development and are ready to **transition to clinical application**. "We are in a unique position to make a real difference in the lives of patients, expanding our therapies to include those who previously had few options," Dr. Diaz says.

Acute Lymphoblastic Leukemia Type T: A Rare Disease

Acute lymphoblastic leukemia (ALL) is a type of **cancer affecting the bone marrow**, the soft tissue within our bones where blood cells are produced. It is characterized by the excessive production of immature lymphocytes (white blood cells) that do not function properly. These abnormal lymphocytes rapidly multiply, interfering with the production of healthy blood cells and can spread within a few months to different parts of the body, such as the lymphatic system, liver, or spleen.

It is **an aggressive and uncommon cancer**, with an incidence of around 1.5 cases per 100,000 inhabitants in high-income countries. Depending on the affected cell type, there are two types of ALL, B-cell ALL (ALL-B) and **T-cell ALL (ALL-T).** The latter, which this new therapy targets, is **even rarer**, with about **100 cases diagnosed per year** in Spain, accounting for approximately 10-15% of all acute leukemias in children and between 20-25% in adults.

It is a **highly heterogeneous disease** with many subtypes, which complicates research and the development of effective treatments. Therefore, innovation and the development of **targeted therapies are essential** to increase treatment options for these patients.

About OneChain Immunotherapeutics (OCI)

OCI was **founded** by the Josep Carreras Leukemia Research Institute, ICREA and Dr. Pablo Menéndez **in Barcelona in June 2020.** It is currently supported by a partnership that includes the venture capital firm **Invivo Partners**, the **Center for Technological Development and Innovation** (Ministry of Industry), the venture capital firms **Nara Capital** and **Clave Capital**, and the **Josep Carreras Leukemia Foundation.**

The company, **based at the Barcelona Science Park (PCB)**, is focused on the **development of 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.

Currently, OCI is developing **five projects that include** 1) the treatment of **T-cell precursor cortical acute lymphoblastic leukemia (coT-ALL)** with a CAR T **already in clinical phase** (OC-1). 2) A **dual CAR T** therapy (OC-1d) that could treat up to 80% of patients with **T-type acute lymphoblastic leukemia**. 3) A **CAR T candidate** (OC-2) for the treatment of **B-cell**

acute lymphoblastic leukemia. 4) A platform in development of **Vδ1 cells for allogeneic application** (OC-3), which will provide ready-to-use treatments at a lower cost than current autologous CAR T treatments. And, finally, 5) a **dual CAR T therapy** for the treatment of **glioblastoma multiforme**, a highly aggressive brain tumor with fatal prognosis.

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