AUTOLOGOUS HUMAN T CELLS EX VIVO TRANSDUCED WITH A LENTIVIRAL VECTOR ENCODING A CHIMERIC ANTIGEN RECEPTOR AGAINST ErbB FAMILY

A research group from CIEMAT/CIBERER/IIS-FJD has developed a new therapeutic application for Fanconi anemia based on immunotherapy with specific CAR T cells

The Need

Fanconi anemia is a rare genetic disorder characterized by congenital abnormalities, bone marrow failure and increased risk for malignancy. Solid tumors, mainly head and neck squamous cell carcinoma (HNSCC), among other types, are common in older individuals with FA.

Treatment of HNSCC in FA patients is challenging because these patients have a hypersensitivity to standard chemo-or radiotherapy.

For this reason, challenging new non-genotoxic treatments are urgently needed in FA patients.

The Solution

Immunotherapy with specific CAR T cells constitute a promising non-toxic treatment for patients with FA that develop HNSCCs.

The solution consists on the use of autologous anti-panErbB CAR T cells generated by a lentiviral vector that carries a modified second generation, intended to eliminate HNSCC overexpressing ErbB molecules.

This new vector is expected to achieve greater antitumor activity in a safer manner in FA patients with HNSCC compared to previous vectors developed.

Innovative Aspects

- Since early tumor resection is considered the only current efficient therapy, the therapy proposed offers **a novel therapeutic and non-genotoxic approach in inoperable stages.**
- Chemotherapy reported in FA patients **produces** severe or fatal toxicities and poor treatment outcomes. The use of a non-genotoxic immunotherapy with CAR T cells would be highly beneficial for the treatment of HNSCC tumors in FA patients based on **the local effect that is expected from these cells, therefore avoiding the circulation of CAR T and possible off-target interactions in healthy tissues**.
- This approach is a **safe therapy** for HNSCC that could efficiently improve the treatment of these FA patients.
- In addition, the treatment with **intratumorally CAR T can be very helpful for inoperable tumors** due to several factors, such as tumor extension, invasion of critical structures or the presence of metastases.

Stage of Development: In vitro efficacy and in vivo efficacy in immunodeficient mice model.

Intellectual Property:

- Orphan Drug Designation in 2025 (EU/3/25/3181)

Aims

Looking for partners interested in a license and/or collaboration agreement to develop and exploit this asset.

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