Innovation in AML Treatment

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The neClectAML consortium, led by the co-coordinators Prof. Dr. Toni Cathomen and Prof. Dr. Evelyn Ullrich, is developing a promising new therapy for acute myeloid leukemia (AML), an aggressive and challenging form of blood cancer. Patients who are ineligible for stem cell transplantation often face limited treatment options, as conventional therapies are often ineffective or poorly tolerated.

At the core of the project is the development of genetically engineered natural killer (NK) cells that are equipped with a chimeric antigen receptor (CAR) that enables the NK cells to specifically recognize and destroy leukemia cells. This is achieved using a CAR based on a llama-derived nanobody that targets the CLEC12A molecule found on AML cells. To further enhance the activity of the NK cells, an NK cell-specific immune checkpoint will be selectively deactivated using CRISPR-Cas gene editing technology.

The consortium, which brings together renowned scientists from the Universities of Freiburg and Frankfurt and the Fraunhofer Institute for Cell Therapy and Immunology (IZI) Leipzig, aims to establish clinical-scale production of these gene-edited CAR NK cells and conduct preclinical testing to ensure their safety and efficacy. The ultimate goal is to prepare for a Phase I/II clinical trial, paving the way for a new therapeutic option. In the long term, the neClectAML team aims to bring this innovative therapy to market and provide AML patients with an effective and well-tolerated treatment. This breakthrough could fill a critical gap in the treatment landscape for this serious disease. In parallel, we are developing a synopsis for the first clinical trial and preparing the necessary documents for the application of an Investigational Medicinal Product Dossier (IMPD). Moreover, an active patient participation in the process is included by structured patient interviews and presentation of the study results in patient organizations. Based on these results, we will seek scientific advice from the Paul-Ehrlich-Institute to define the final preclinical work packages required for approval to conduct a clinical study.