CAR T-cells Targeting the CD4 Protein Granted Orphan Drug Designation for the Treatment of Peripheral T-cell Lymphoma (PTCL)

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iCell Gene Therapeutics today announced that the US Food and Drug Administration (FDA) has granted Orphan Drug Designation for its chimeric antigen receptor engineered T-cells directed against the target protein CD4 (CD4CAR) for the treatment of peripheral T-cell lymphoma (PTCL). The Orphan Drug Designation program provides orphan status, and associated development incentives, to drugs and biologics intended for the safe and effective treatment, diagnosis or prevention of rare diseases or disorders that affect fewer than 200,000 people in the US.

Yupo Ma, MD, PhD, Professor of Pathology at Stony Brook University & Chairman and Chief Scientific Officer at iCell Gene Therapeutics, said: “CD4CAR could significantly enhance currently available treatment options for these patients. The Orphan Drug Designation is an important achievement as we advance our development plans for this promising treatment in T-cell hematologic cancers.”

About CAR T-cell Technology

A "chimeric antigen receptor" (CAR) engineered T-cell is a patient's T-cell (a component of the immune system) that has been genetically modified to express a protein on its surface with the capability to bind to a target protein on another cell. Upon binding, the CAR protein will send a signal across the cell membrane to the interior of the T-cell to set in motion mechanisms to selectively kill the targeted cell.

About PTCL

Although there are clinical development programs ongoing with CAR T-cells for CD19+ cell hematological malignancies, CD4+ peripheral T-cell lymphomas (PTCLs) have not been targeted by a CAR therapy in a human trial. PTCLs account for 10–15% of all non-Hodgkin’s lymphomas (NHLs) and are more difficult to treat in comparison to B-cell NHLs. Furthermore, and with few exceptions, T-cell NHLs have poorer outcomes, lower response rates, shorter times to progression, and shorter median survival in comparison to B-cell NHLs. As a result, the standard of care for PTCLs is not well-established and the only potential curative regimen is Bone Marrow Transplant (BMT). Not only is BMT poorly-tolerated, but is not an option for a significant subset of patients with resistant disease. This leaves many patients with no curative options.

William Tse, MD, FACP and Chief of the Blood and Marrow Transplantation Division, Department of Medicine at University of Louisville School of Medicine, said “we are very

excited to have this opportunity to partner with the iCell Gene Therapeutics and to lead this cutting-edge immunotherapy into first-in-human clinical trial for patients suffering this extremely difficult to treat T cell lymphoma."

About CD4CAR

CD4CAR is in development for CD4+ T-cell malignancies. The novel CD4-specific chimeric antigen receptor engineered T-cells are properly-matched allogeneic human T-cells engineered to express an anti-CD4scFV antibody domain. An initial Phase I clinical study is being planned through collaboration between iCell Gene Therapeutics, the National Institutes of Health, Indiana Clinical and Translational Sciences Institute, Stony Brook Hospital, the Blood and Marrow Transplantation Division and the Clinic Trial Research Unit at James Graham Brown Cancer Center at University of Louisville.

About iCell Gene Therapeutics

iCell Gene Therapeutics is developing CAR T and NK cells that target cancer with very poor prognoses and clear unmet medical need. Diseases covered by our proprietary CAR technologies include B and T cell lymphoma and leukemia, myeloproliferative neoplasms, myeloid dysplastic syndrome (MDS), acute myeloid leukemia (AML), multiple myeloma (MM), non-hematological (non-blood) cancers and autoimmune disorders. For more information please visit: www.icellgene.com

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