Cell & Gene Therapy Overview

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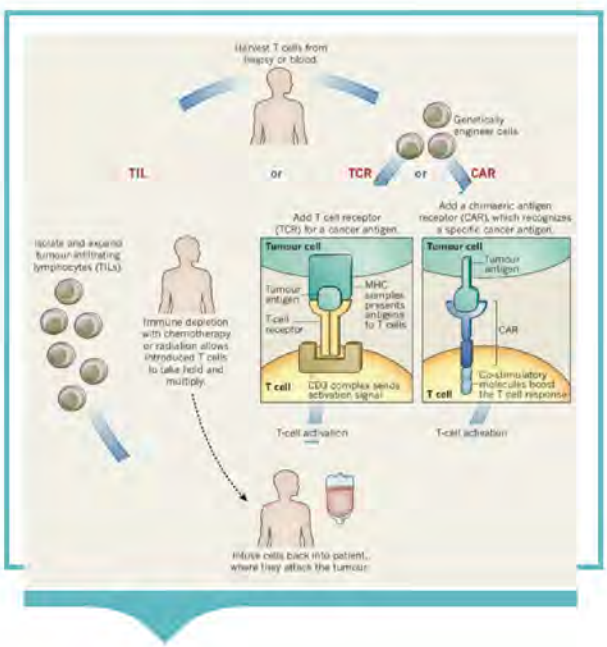
Cell & Gene Therapy

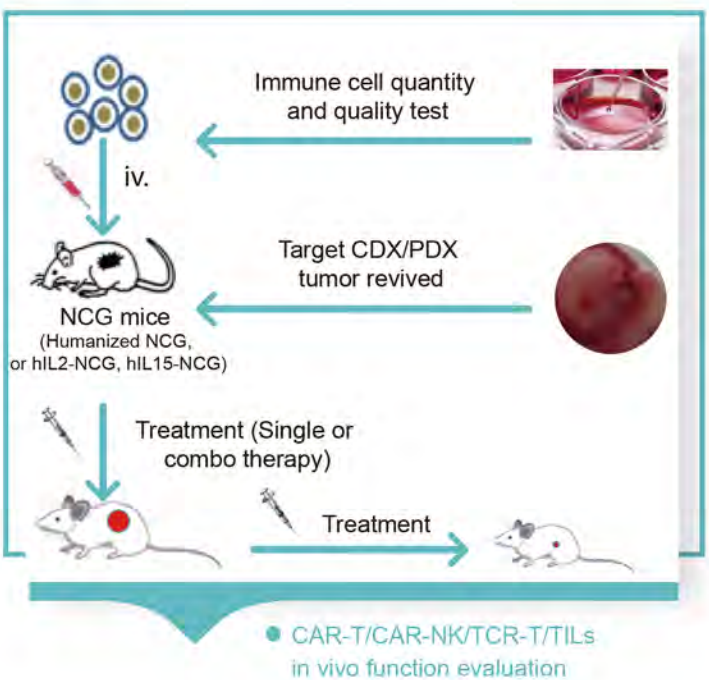
Cell & gene therapies are used to treat both rare and common diseases. Cellular therapy products include cellular immunotherapies, cancer vaccines, and other types of both autologous and allogeneic cells for certain therapeutic indications, including hematopoetic stem cells and adult and embryonic stem cells. Human gene therapy seeks to modify or manipulate the expression of a gene or to alter the biological properties of living cells for therapeutic use.

Continuing advances in cell and gene therapies are transforming how we treat and potentially cure certain diseases and dramatically changing healthcare outcomes.

CAR-T Cell Therapy

For treatment applications in Oncology, CAR-T (Chimeric Antigen Receptor T Cell) is a type of cell therapy which involves the introduction of T cells that have been genetically modified into the body of a cancer patient. CAR-T therapeutic development is now commonplace following recent approvals of drugs in CD19 expressing disease. These CAR-T products have been approved by the US FDA for certain types of leukemia and lymphoma, and both pharmaceutical and biotech companies are investing globally in this approach to treating cancer.

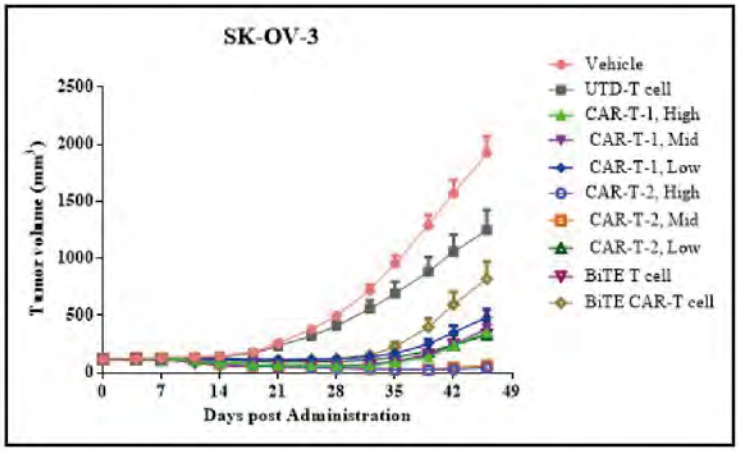


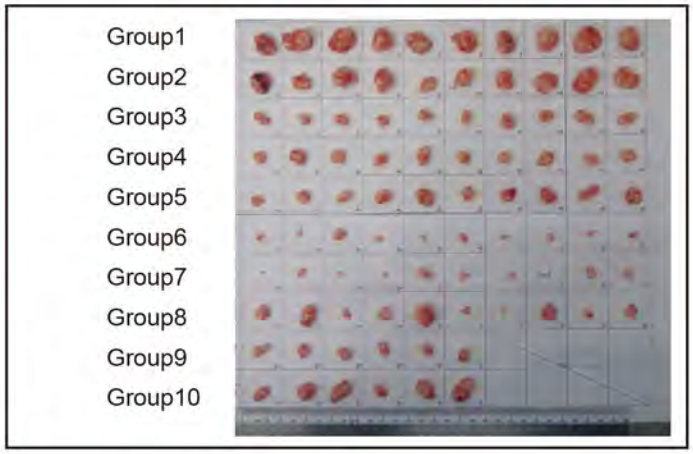


- CAR-T/CAR-NK/TCR-T/TILs in vivo function evaluation

Part I-1 In vivo efficacy evaluation of CAR-T in CDX model

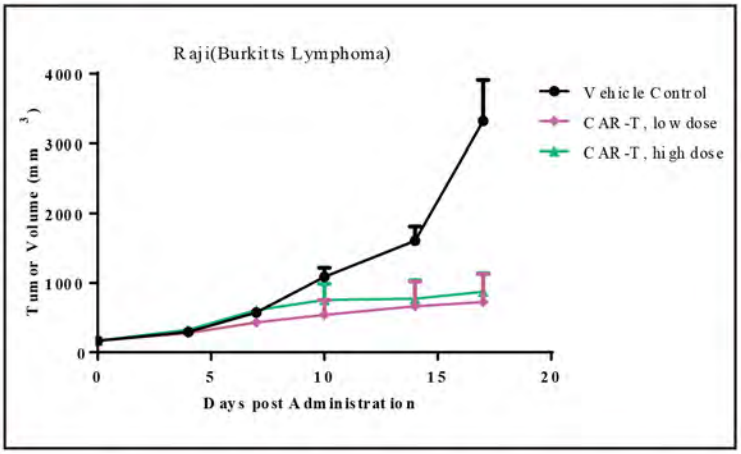
SKOV3

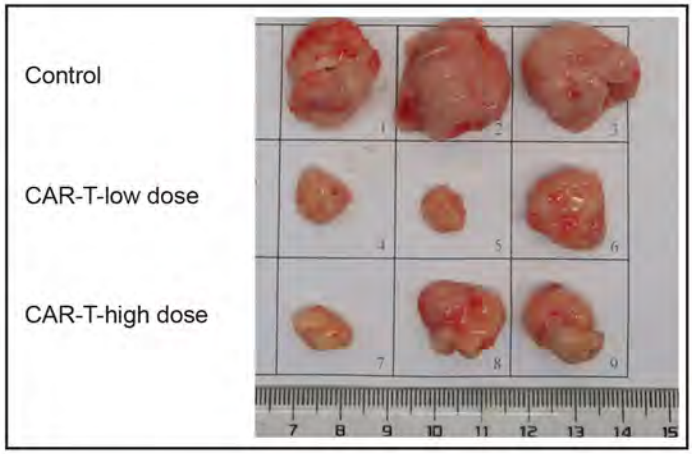




▶ Different types of CAR-T cells efficiently inhibited ovarian cancer development in NCG mice.

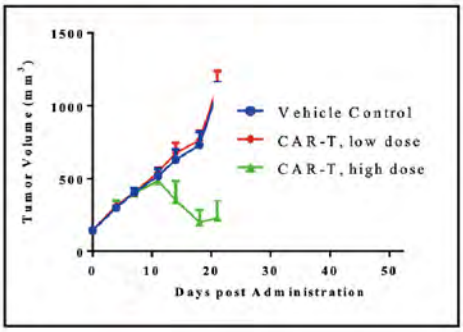
Raji



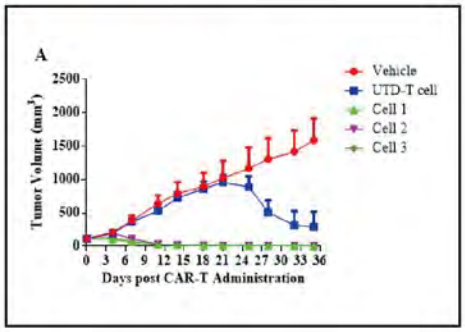


▶ Even low dose of CAR-T cells effectively killed Burkitt's lymphoma in NCG mice.

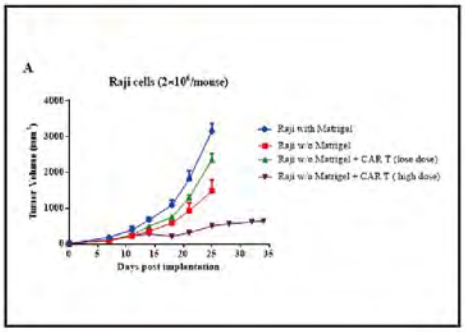
NALM-6 CDX



SNU620 CDX

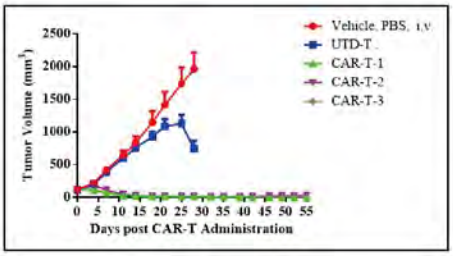


Raji CDX

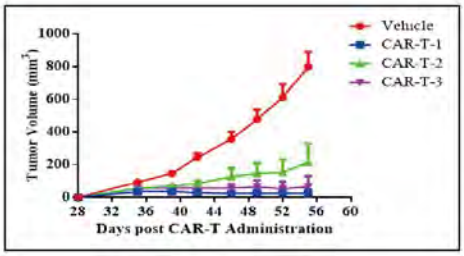


▶ CAR-T therapy showed nice in vivo effect in blood cancer as well as gastric cancer.

Long-term efficacy evaluation of CAR-T cells



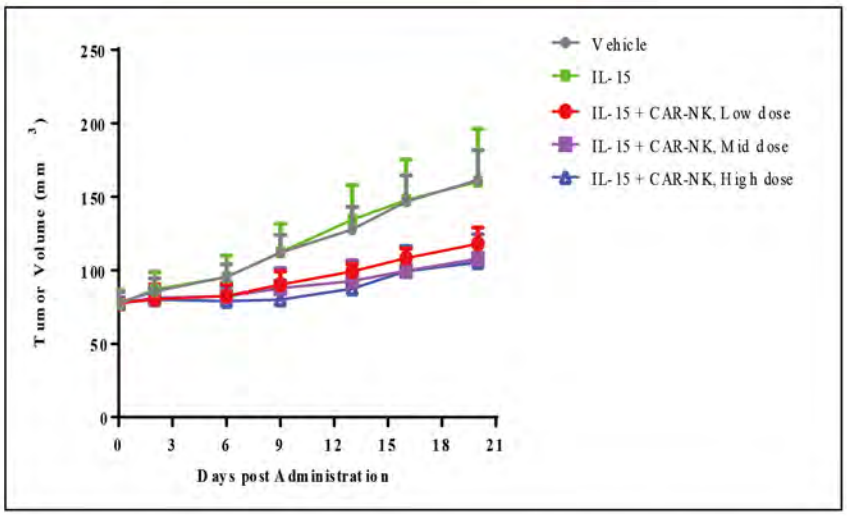
First Efficacy Study



Second Efficacy Study

To verify the long-term efficacy of the CAR-T cells, tumor cells were seeded on the other side of the mice after the end point of the first experiment. Experimental results show that CAR-T can still inhibit tumor growth for a long time.

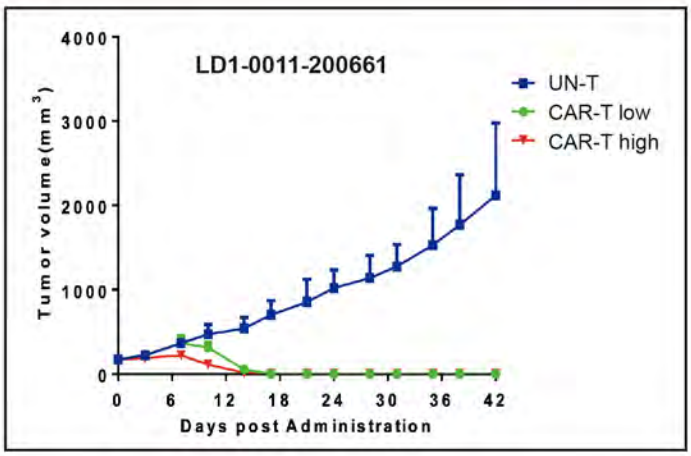
In vivo efficacy evaluation of CAR-NK in CDX model

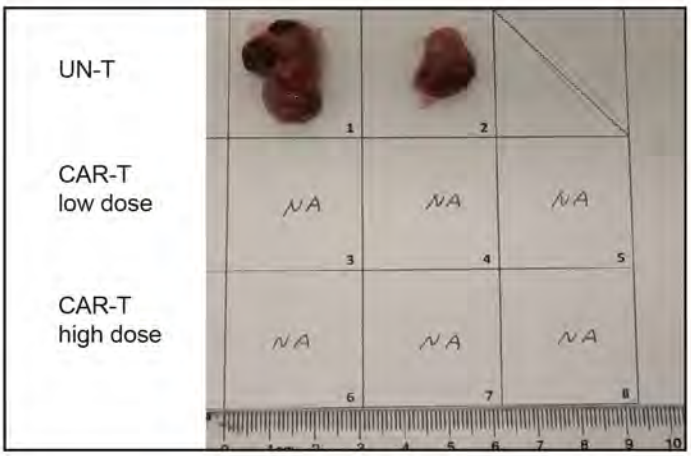


MDA-MB-468
+ CAR-NK/ IL15

▶ CAR-NK cell therapy did well for triple negative breast cancer MDA-MB-468 suppression.

Part I-2: In vivo efficacy evaluation of CAR-T in PDX model

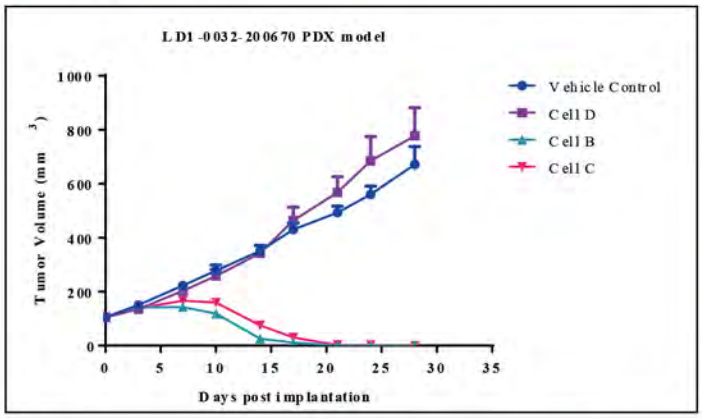


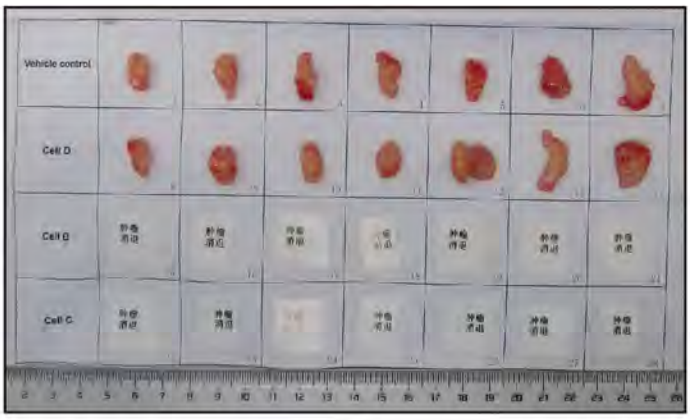


▶ Functional CAR-edited T cells fully stopped hepatic cell cancer growth in NCG mice.

In vivo efficacy evaluation of CAR-T in PDX model

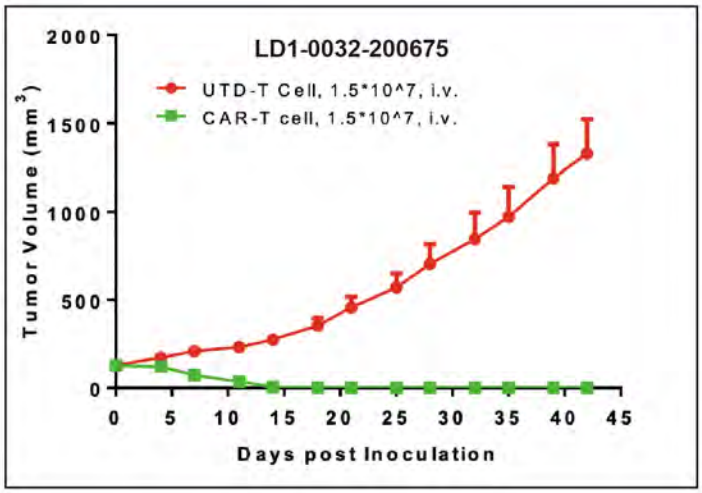
Ovarian Cancer PDX

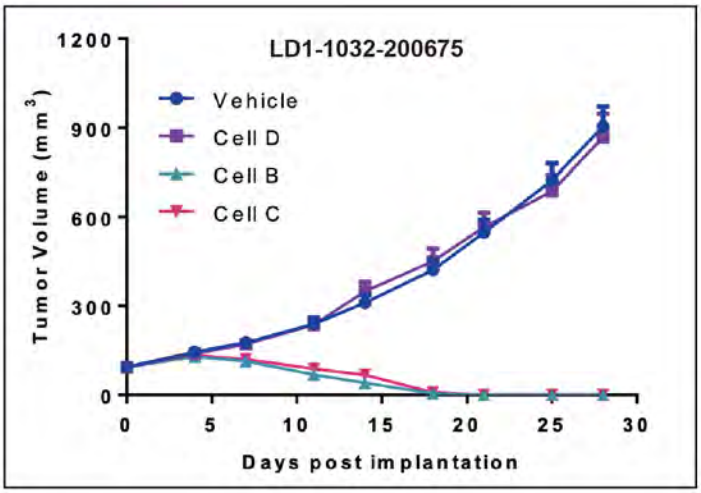


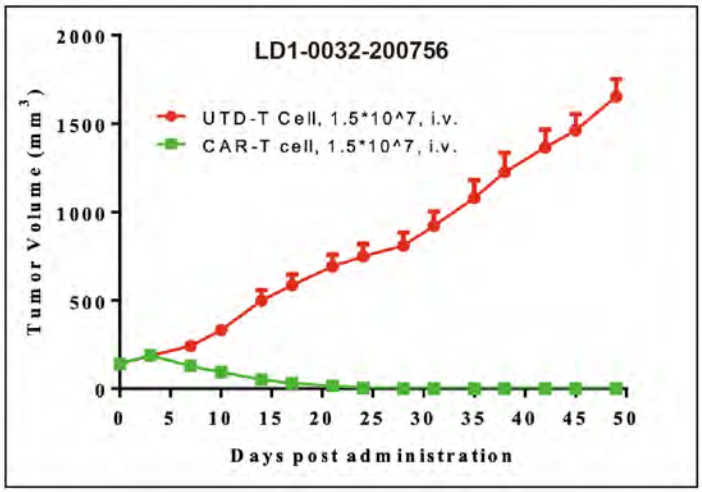


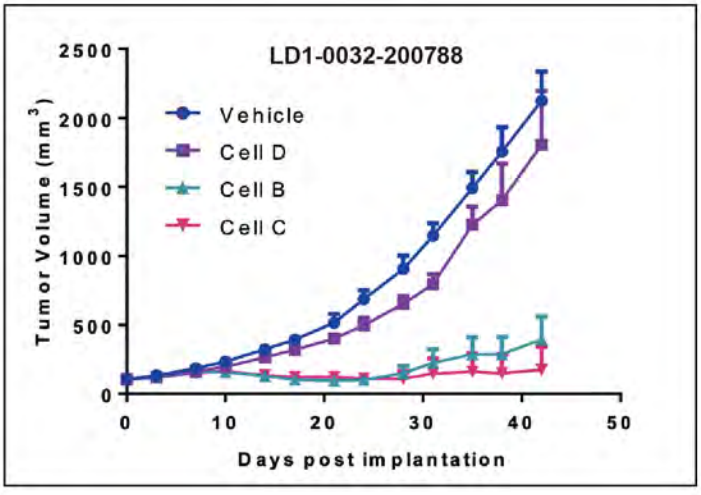
▶ Functional CAR-edited T cells fully stopped hepatic cell cancer growth in NCG mice.

Excellent in vivo efficacy of CAR-T therapy In ovarian cancer PDX models

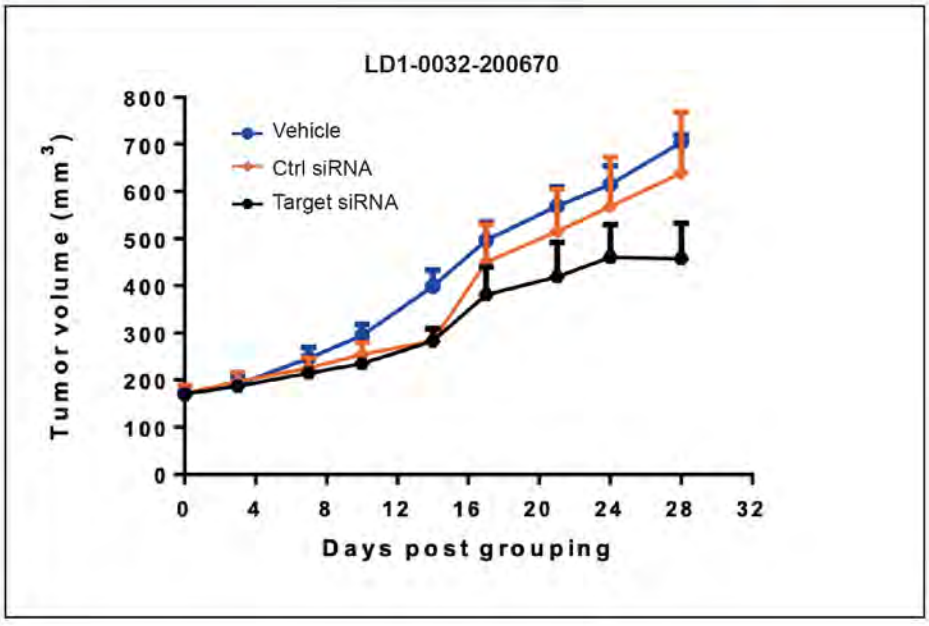






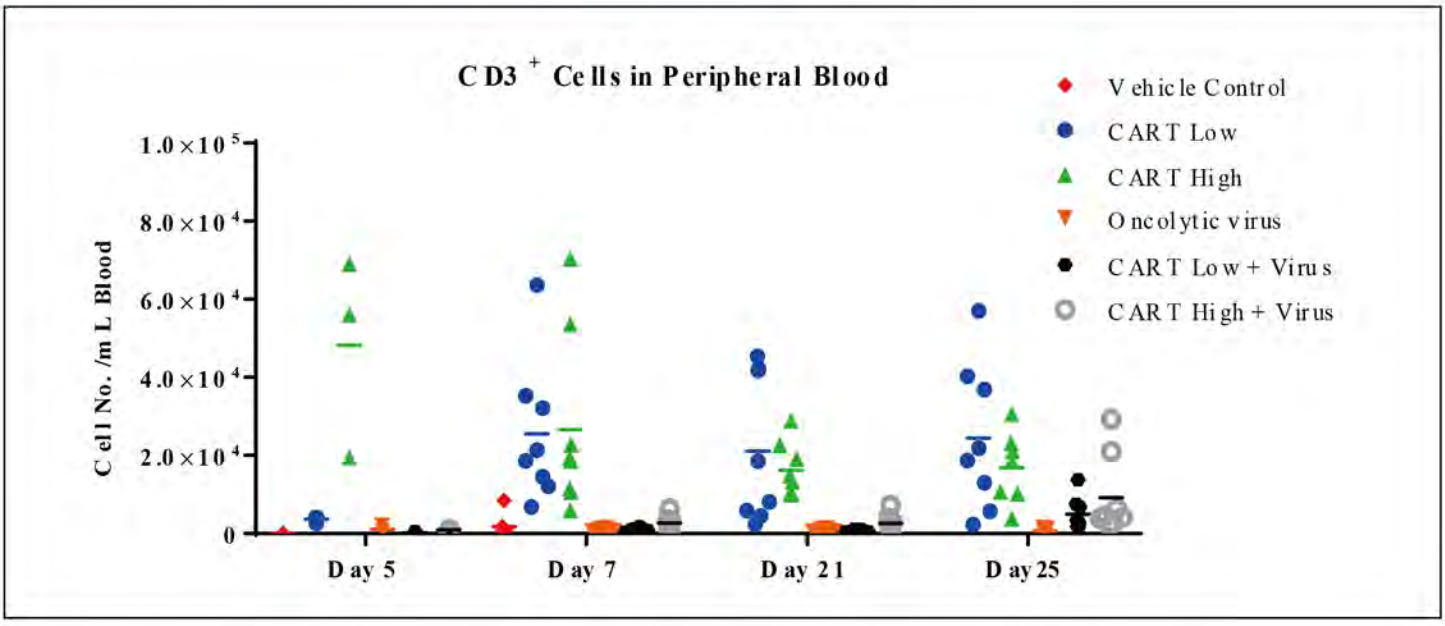


Gene modified immune cell therapy in ovarian cancer PDX models



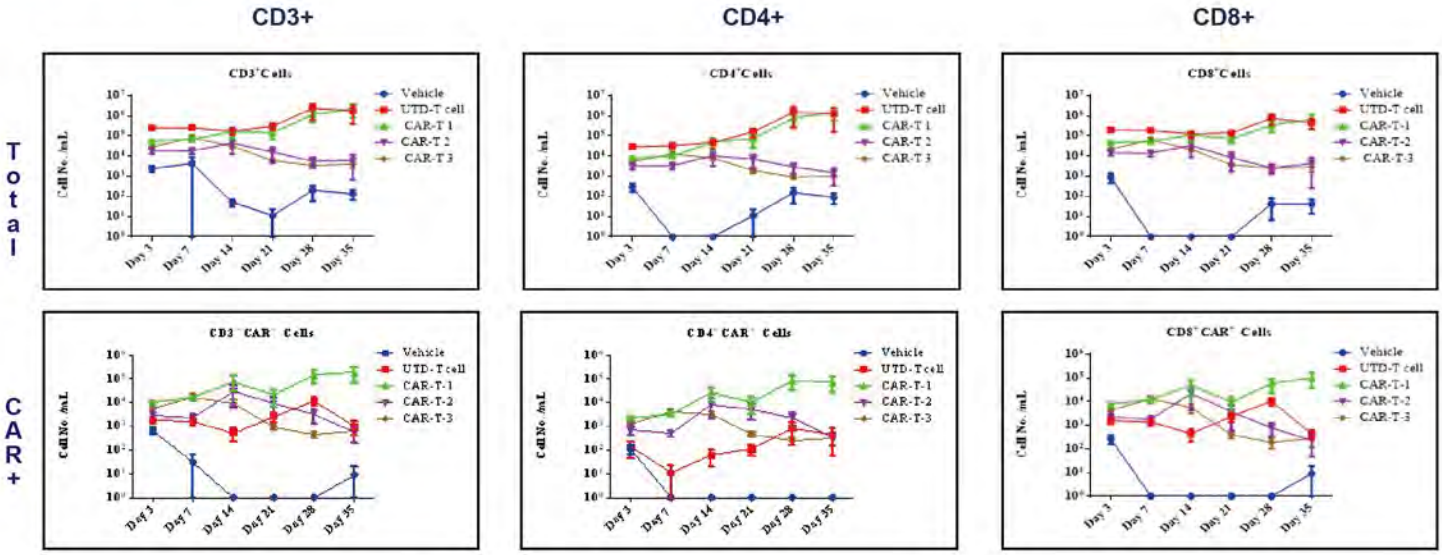
▶ Specific gene silent immune cells therapy showed some effect in ovarian cancer PDX model.

Part I-3: PK and immuno-phenotyping of CAR-T cell therapy



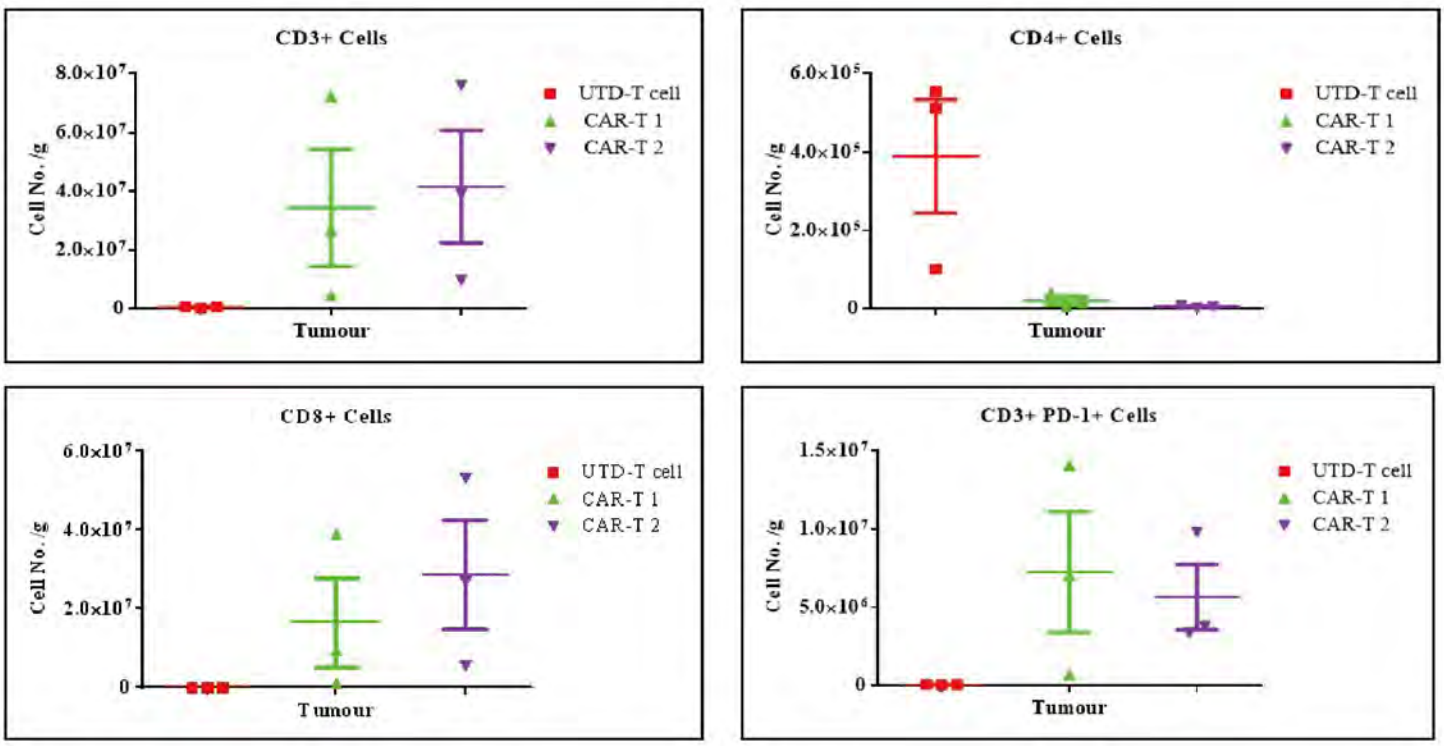
▶ Ex vivo tracking of human CD3+ (including CAR-T) in peripheral blood of NCG mice after different treatments.

PK study of CAR-T therapy



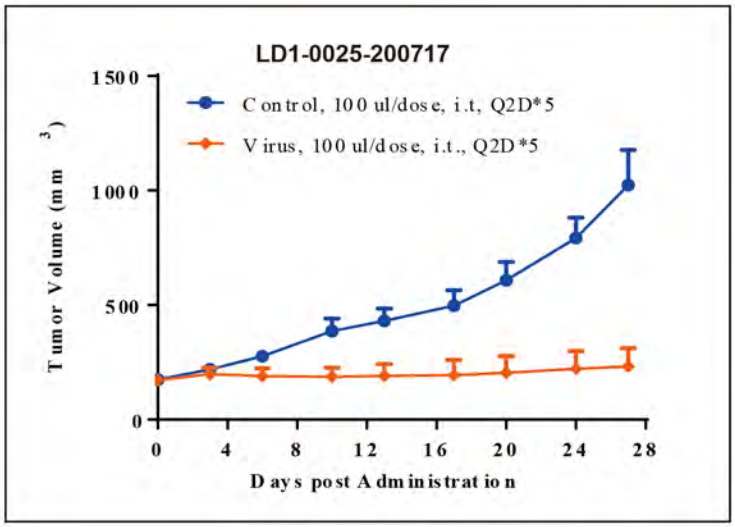
▶ Dynamic analysis of in vivo human CAR+ T and total T cells in peripheral blood of NCG mice.

CAR-T immuno-phenotyping in tumor microenvironment

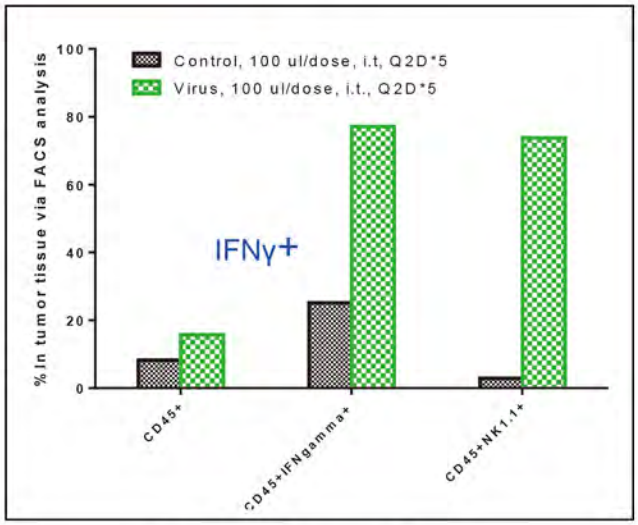


▶ PD1 was obviously up-regulated in CAR-T cells after in vivo transfer, compared to untransduced T cells.

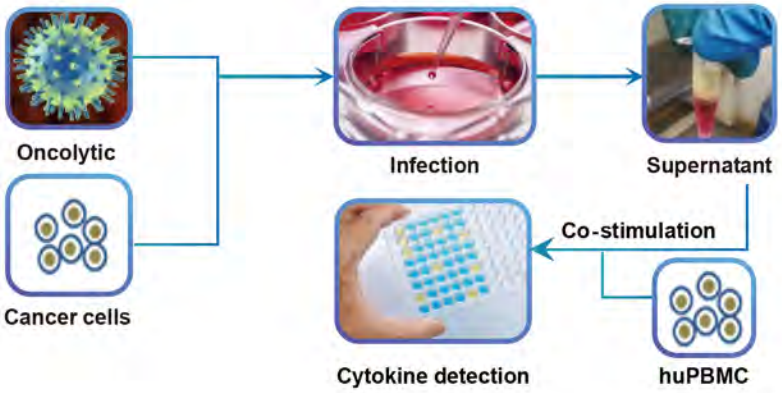
Part II: In vivo efficacy, biomarker and in vitro CRS assay of oncolytic virus

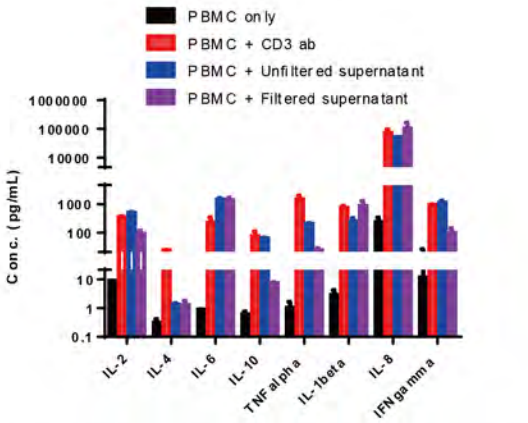


Tumor size



Functional Immune subsets

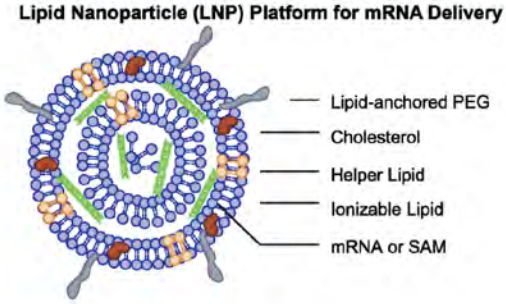




▶ Multiple cytokines detection by ELISA from human PBMC after oncolytic virus treatment.

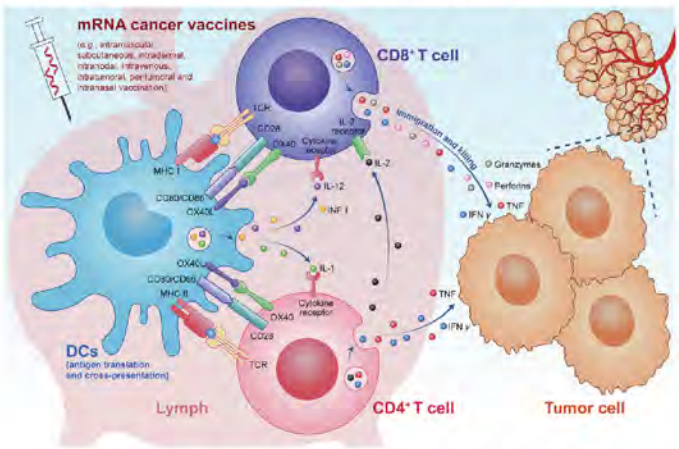
Part III: RNA vaccine in humanized CDX models

Lipid Nanoparticle (LNP) Platform for mRNA Delivery



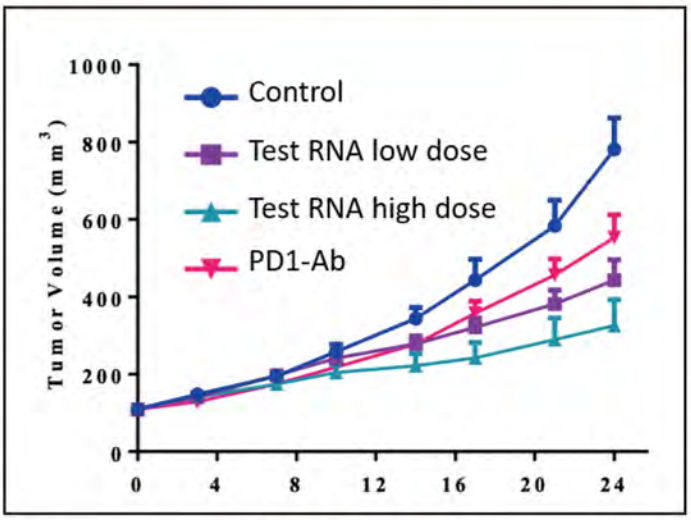
● Miao et al. Molecular Cancer (2021) 20:41

mRNA cancer vaccines potential working mechanism

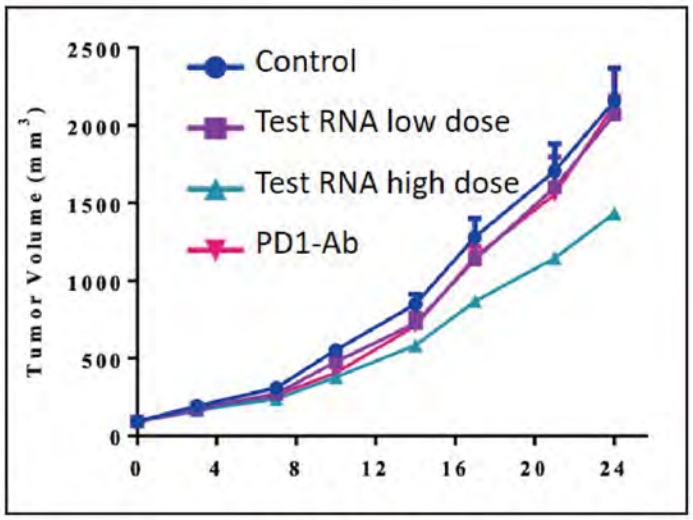


● Qing He, et al. Acta Pharm Sin B. 2022 Mar 23.

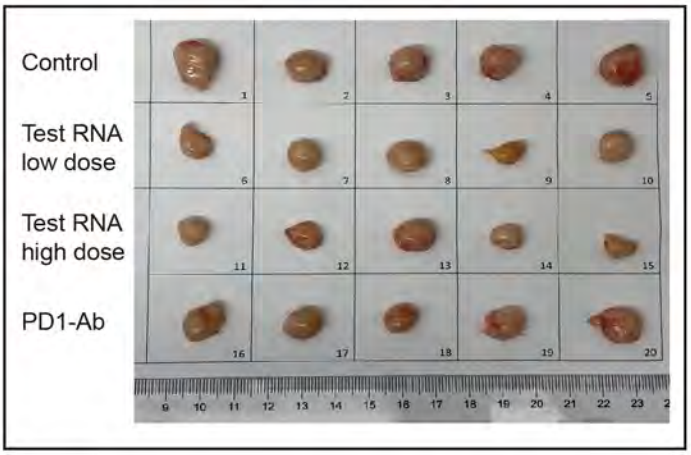
RNA vaccine boost human immune system to eradiate cancer

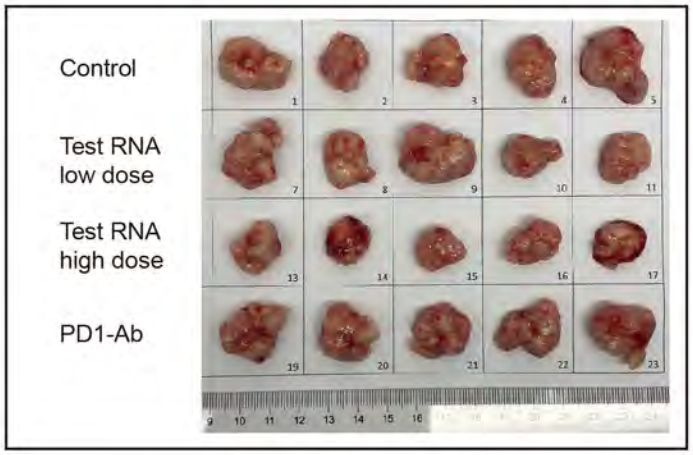


MDA-MB-231+ PBMC

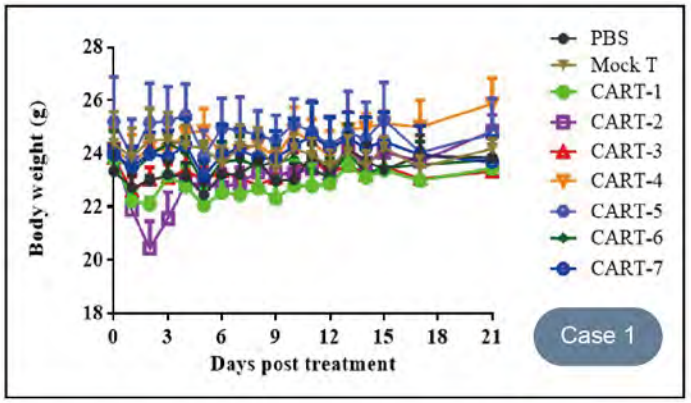


A375+ PBMC

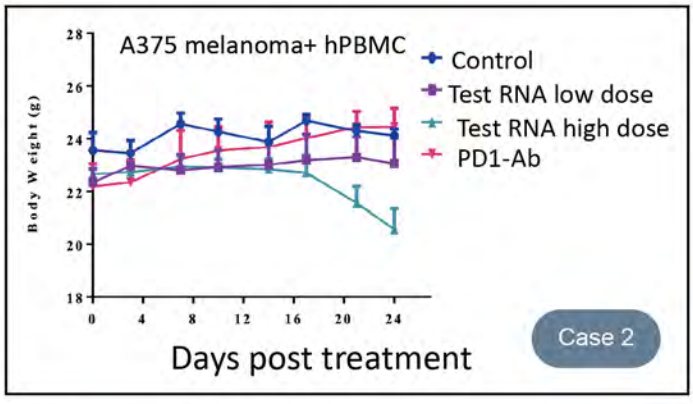




Part-IV: CAR-T cell and mRNA vaccine safety evaluation

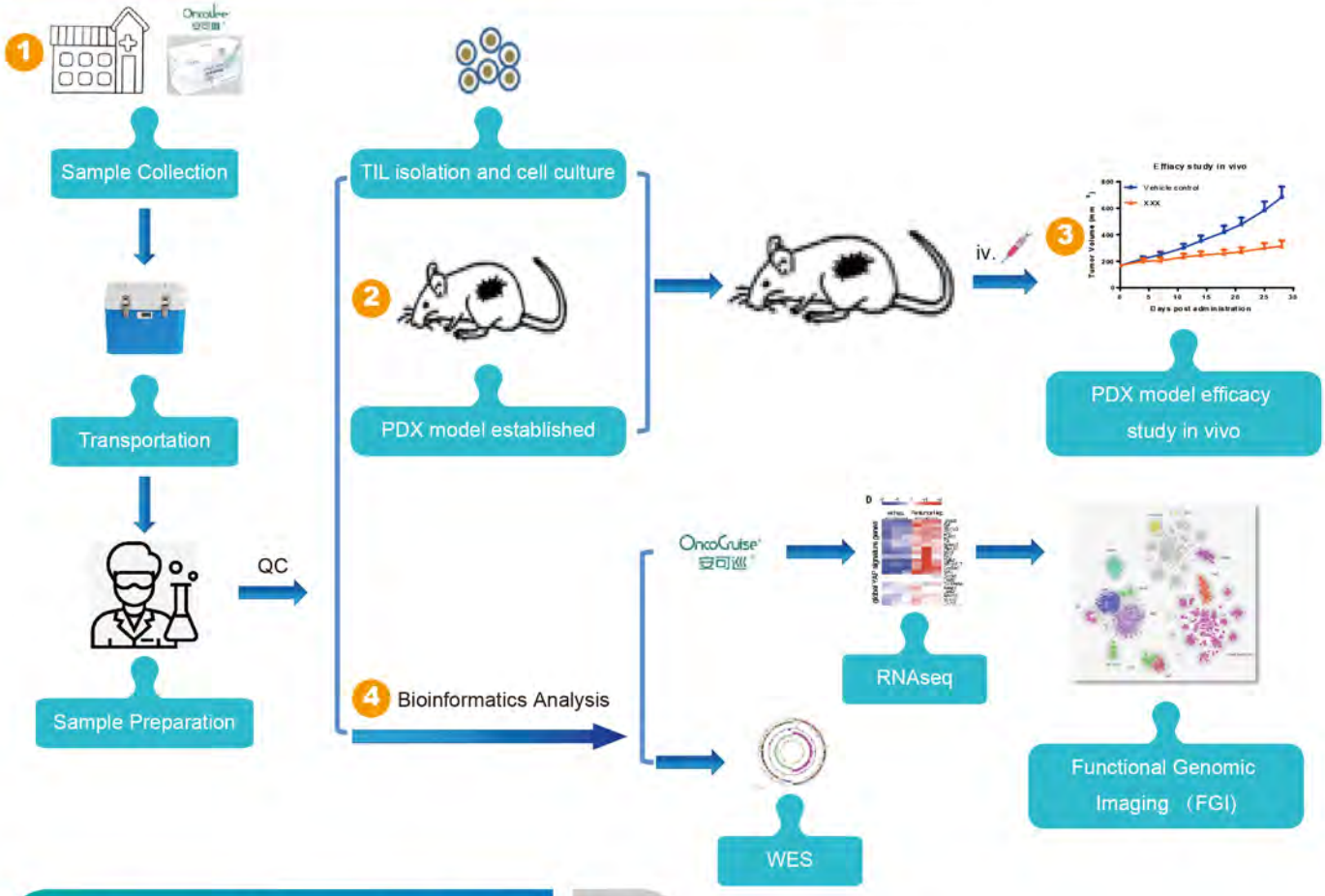


▶ No obvious mice body weight changed after a single dose treatment of different types of CAR-T cell therapy.



▶ High dose of RNA vaccine may induce gradually Weight Loss.

Part V: Functional Evaluation of TIL based on clinical samples



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