

Pioneering mRNA/LNP Delivery Platform-Based in vivo CAR-T Therapy for Autoimmune Diseases

Company Overview

*tia Therapeutics is an emerging biotechnology company operating in the U.S. and China, dedicated to transforming treatment paradigms for autoimmune diseases through cutting-edge mRNA and cellular immunotherapy technologies. Founded by veterans from leading biotech and global pharmaceutical companies, the team brings a strong record in patent licensing, biologics development, and mRNA/LNP engineering, positioning the company at the forefront of next-generation immune modulation therapies.

Innovative mRNA/LNP Delivery Platform

While lipid nanoparticles (LNPs) have revolutionized mRNA therapeutics, their intravenous (IV) application remains largely constrained by several challenges: efficacy and safety concerns limiting systemic use; instability issues that complicate scalable GMP manufacturing; liver-restricted biodistribution, since conventional LNPs predominantly accumulate in hepatocytes, with poor access to other tissues such as lungs, spleen, or brain. These hurdles have slowed the progress of RNA therapeutics targeting extra-hepatic diseases including autoimmune disorders.

Overcoming the Limitations of Current mRNA/LNP Delivery

*tia Therapeutics has developed a proprietary mRNA-LNP platform designed to overcome the core limitations of current mRNA delivery systems. The platform integrates multiple innovation layers:

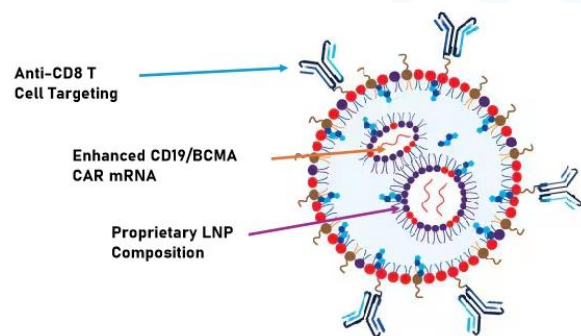
- **Enhanced efficacy and safety** through optimized lipid composition reducing immunogenicity and toxicity.
- **Improved manufacturability**, providing superior formulation stability and scalability under GMP conditions.
- **Antibody-LNP conjugation for extra-hepatic delivery**, enabling precise tissue-specific targeting of immune cell populations.

This novel delivery architecture establishes a versatile foundation for systemic RNA therapies and supports repeat dosing in chronic indications.

In vivo Anti-CD18/BCMA mRNA/LNP CAR-T for Autoimmune Diseases

The lead program, PTH-0239, is an in vivo mRNA/LNP CAR-T therapy that enables direct reprogramming of T cells within the body, bypassing the complexity of autologous ex vivo manufacturing.

PTH-0239 encodes anti-CD19/BCMA CAR constructs and leverages CD8-targeted LNPs to selectively transfect cytotoxic T cells. The result is an optimized CAR-T therapeutic index (TI) with superior safety, transient activity, and repeat-dose compatibility - a transformative profile for chronic autoimmune diseases such as lupus.



Key Advantages Over Traditional CAR-T Approaches

Limitation of Autologous CAR-T	Advantage of mRNA/LNP in vivo CAR-T
Cytokine Release Syndrome (CRS)	Flexible dosing regimen to mitigate CRS and enable repeat dosing
Complex, time-consuming cell manufacturing	Off-the-shelf, scalable GMP production
High production cost	Standardized, cost-efficient workflow
Supply chain and storage challenges	Consistent GMP lot release
Risk of secondary malignancies from genome integration	Transient mRNA expression ensuring safety
Lymphopenia, immunosuppression	Mild lymphodepletion

Business and Collaboration Opportunities

This company is actively seeking strategic partnerships and investment to accelerate the advancement of its platform and lead program. The company welcomes opportunities including:

- **Pre-seed funding** to expand preclinical and IND-enabling studies.
- **Out-licensing** of its proprietary **mRNA/LNP delivery technology and the in vivo anti-CD18/BCMA CAR-T asset**.
- **Co-development collaborations** with biotech or pharma partners exploring **in vivo CAR-T** or **extra-hepatic RNA delivery**.
- **Acquisition discussions** for companies interested in integrating differentiated RNA/LNP capabilities into their immunotherapy pipelines.

About Protheragen

Headquartered in New York, Protheragen is a US-based company specializing in the global pharmaceutical and biomedical sectors. Our core services aim to precisely connect innovative pharmaceutical assets with potential partners worldwide, efficiently facilitating diverse strategic collaborations including, but not limited to: Licensing-out, Financing, Co-development, and Mergers & Acquisitions.

These assets from the early discovery stage to the later clinical phases include first-in-class small molecules, ADCs, proteins, cell therapies, and vaccines, which are being developed for the treatment of key therapeutic areas, such as oncology, autoimmune diseases, or metabolic disorders.

More Cell Therapy Assets Seeking Out-licensing and Financing Opportunities

Asset	Phase	Description	Indication	Target
GCC-CART	Phase 1 (FDA and NMPA)	Human autologous T cells transduced with a lentiviral vector encoding a CAR targeting guanylate cyclase 2C	Recurrent, refractory and metastatic colorectal cancer	Guanylate cyclase 2C (GC-C)
PTH-0232	Phase 1 (NMPA)	Pioneering approach utilizing iPSC-derived endothelial progenitor cells (EPCs) to repair damaged vasculature	Acute ischemic stroke	
PTH-0426	IIT (China)	Targeting BCMA/NKG2DL/FAP TCR-T cells that possess the characteristics of TCR-T and the 4th-generation CAR-T cells showed no severe toxic side effects and exhibited excellent clinical efficacy	Multiple myeloma	BCMA; NKG2DL; Fibroblast activation protein (FAP)
PTH-0427	IIT (China)	Super-effective TCR-T cells	B/T cell malignancies; leukemia	CD19; CD37; Fibroblast activation protein (FAP)
PTH-0428	IIT (China)	Super-effective TCR-T cells	Solid tumors	Nectin4; NKG2DL; FAP
PTH-0429	IIT (China)	Super-effective TCR-T cells	Solid tumors	Nectin4; B3H7; TROP2; FAP
PTH-0430	IIT (China)	Super-effective TCR-T cells	Lupus erythematosus	CD19; BCMA
PTH-0089	IIT (China)	NK cell therapy derived from induced pluripotent stem cells (iPSCs) through the programming and modification with the CD16 gene (vCD16)	Solid tumor	CD16
PTH-0091	Preclinical	iPSC-Derived CAR-NK Cells	Solid tumor	
PTH-0092	Preclinical	iPSC-Derived CAR-NK Cells	Autoimmune disease	
PTH-0096	Preclinical	Anti-CD19/CD22 CAR-T Cells (anti-CD19 scFv combined with anti-CD22 nanobody)	Lymphoma	CD19; CD22
EDB-TCR-T	Preclinical	T cells equipped with duraCAR demonstrated antigen-induced metabolism profile suitable for sustained in vivo efficacies.	Solid tumor	Fibronectin extra domain B (EDB)
PTH-0097	Discovery	Autologous/Allogeneic CAR-T Cells	Acute and chronic graft-versus-host disease (GVHD)	
TCR-T012	Discovery	TCR-T Cells	Blood tumors, ovarian cancer	WT1
TCR-T013	Discovery	TCR-T Cells	Nasopharyngeal cancer, gastric cancer	EBV
TCR-T014	Discovery	TCR-T Cells	Cervical cancer	HPV-16
TCR-T015	Discovery	TCR-T Cells	Lung cancer, pancreatic cancer	KRAS
TCR-T016	Discovery	TCR-T Cells	Melanoma, non-small cell lung cancer and other types of tumors	NY-ESO-1
PTH-0192	Discovery	TCR-T Cells	NSCLC, lung adenocarcinoma, gastric cancer, colon cancer	Tumor neoantigen