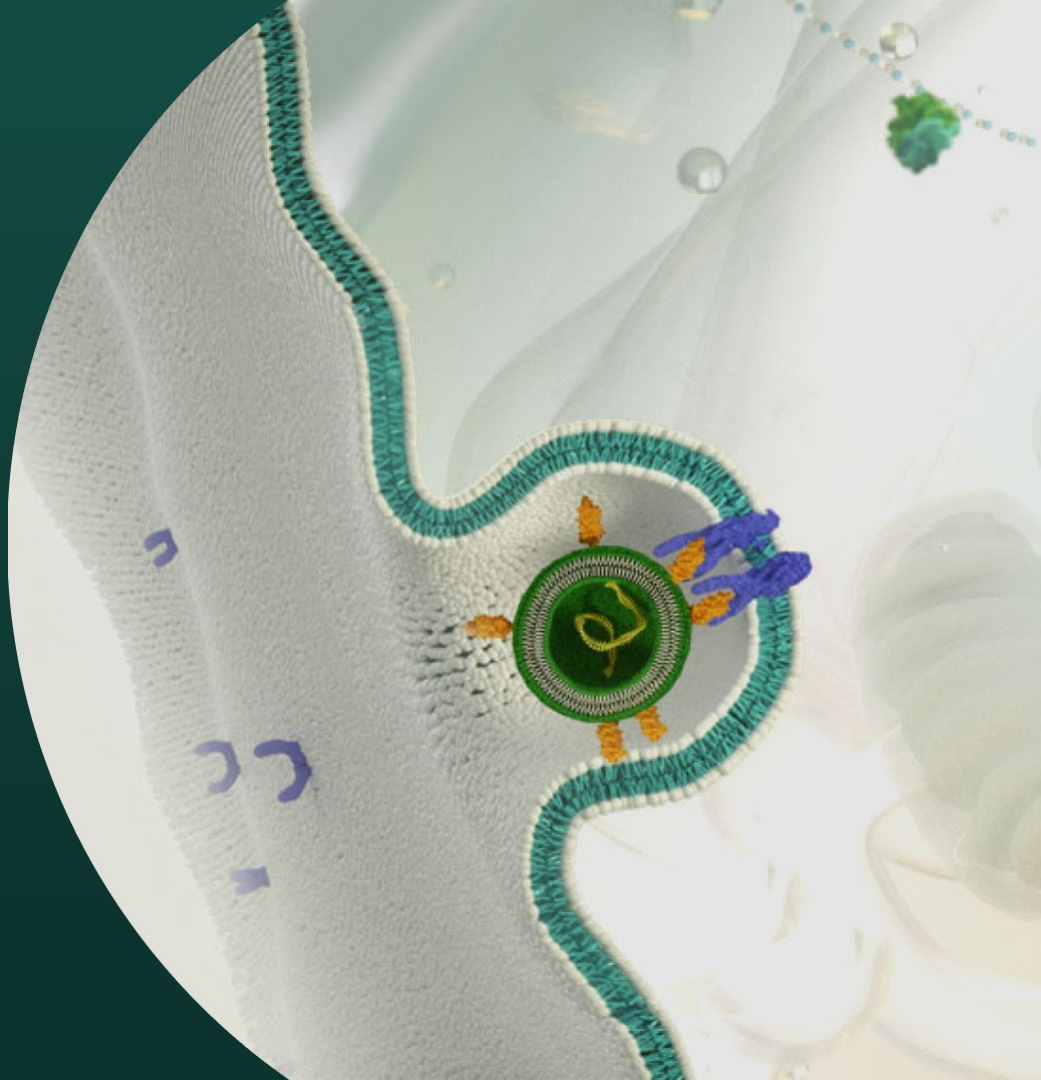
ACUITAS THERAPEUTICS

Advancing LNP Systems for Clinical Development of mRNA-based Medicines

18 November 2026



Our Partners' Success

Clinical Firsts

First Clinically Approved **RNA interference-based Medicine**

First Clinically Approved **mRNA Vaccine**

First **LNP enabled personalized CRISPR gene editing therapy**

Products in Clinic



22 in Phase 1

4 in Phase 2

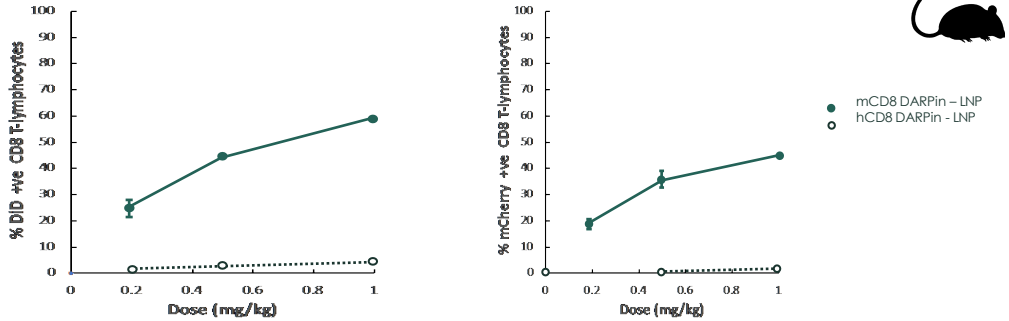
3 in Phase 3

Targeted LNP for Non-hepatic mRNA Delivery

mRNA LNP delivery to non-hepatic targets

DARPins* for in vivo targeting of mRNA LNP to murine CD8+ T lymphocytes

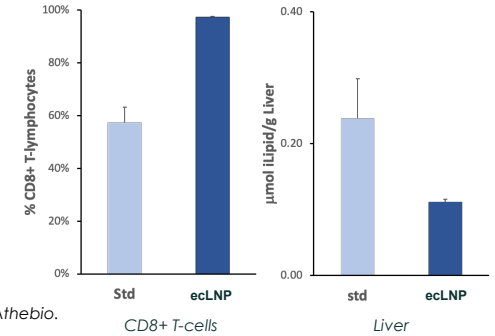
CD8 DARPin targeted mRNA LNP show dose dependent, target specific binding / uptake and transgene expression.



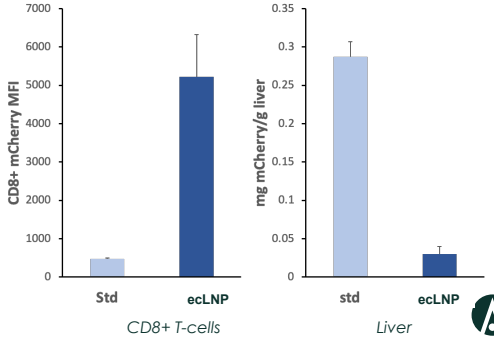
Optimized extended circulation (ec) LNP results in 2x increased binding / uptake and 10x increased expression.

Expression in liver is ~10x lower vs. standard LNP.

LNP Binding / Uptake



Reporter Gene Expression



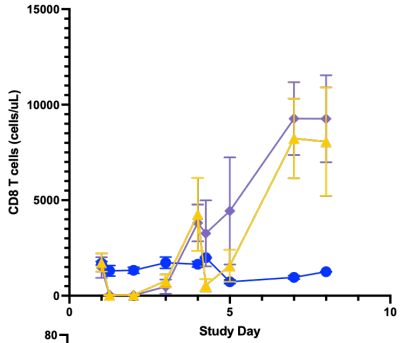
*Athebody® DARPin (Designed Ankyrin Repeat Proteins) are provided by Athebio.

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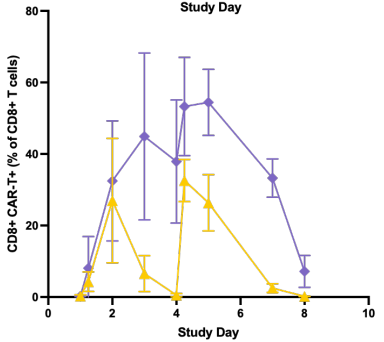
Targeted LNP for Non-hepatic mRNA Delivery

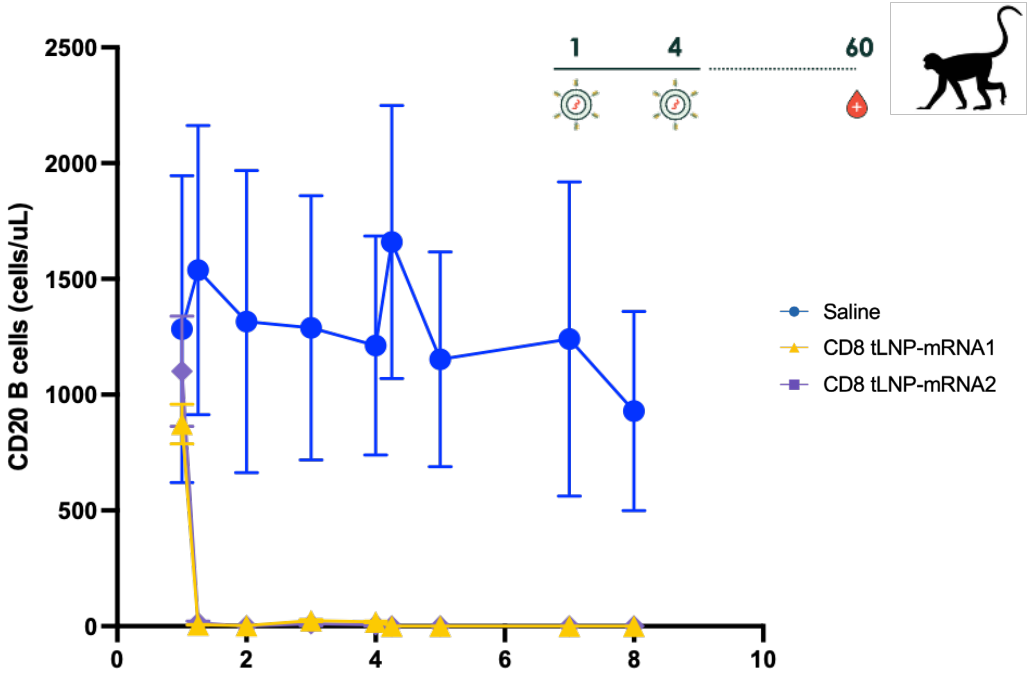
LNP delivery to T cells to generate *in vivo* CAR T cells. Data shows induced CD8+ T cell expansion with a high % of CAR expressing T cells which mediate significant and sustained B cell depletion.

CD8 T Cell Expansion



CAR+ CD8 T Cell



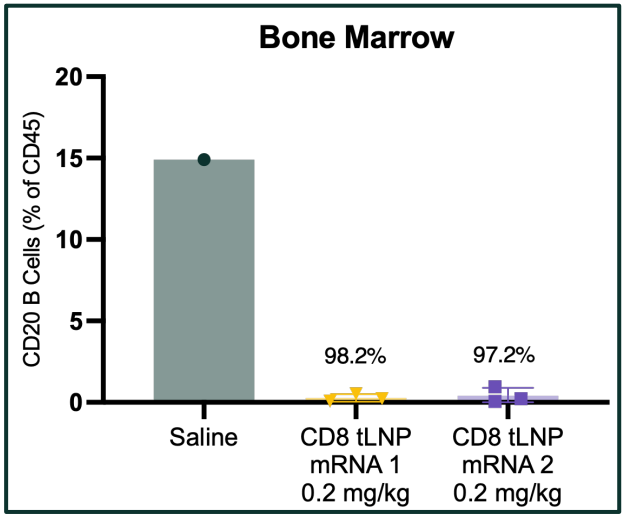


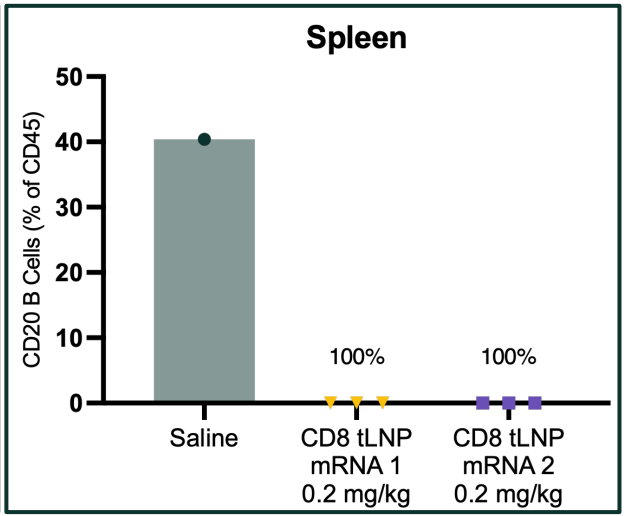
**In vivo CAR-T work conducted in collaboration with an RNA therapeutics partner*

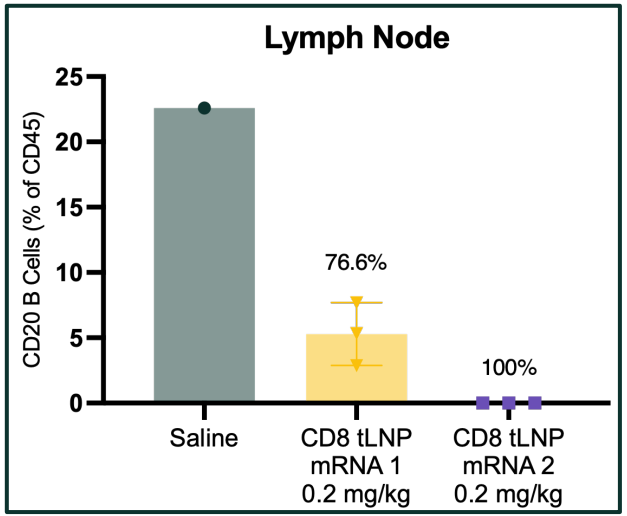
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Targeted LNP for Non-hepatic mRNA Delivery

mRNA LNP CAR delivery to CD8+ T cells results in significant depletion of B cells in spleen, bone marrow, and lymph nodes.







*In vivo CAR-T work conducted in collaboration with an RNA therapeutics partner

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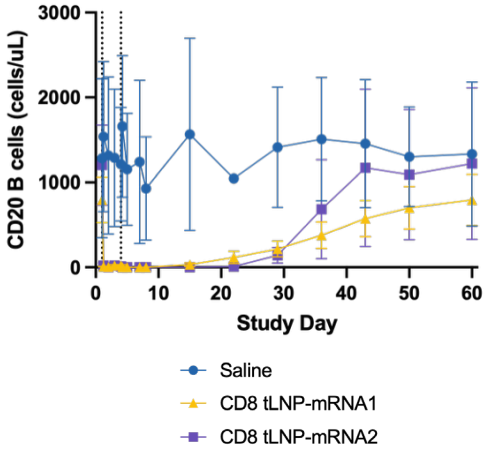
Targeted LNP for Non-hepatic mRNA Delivery

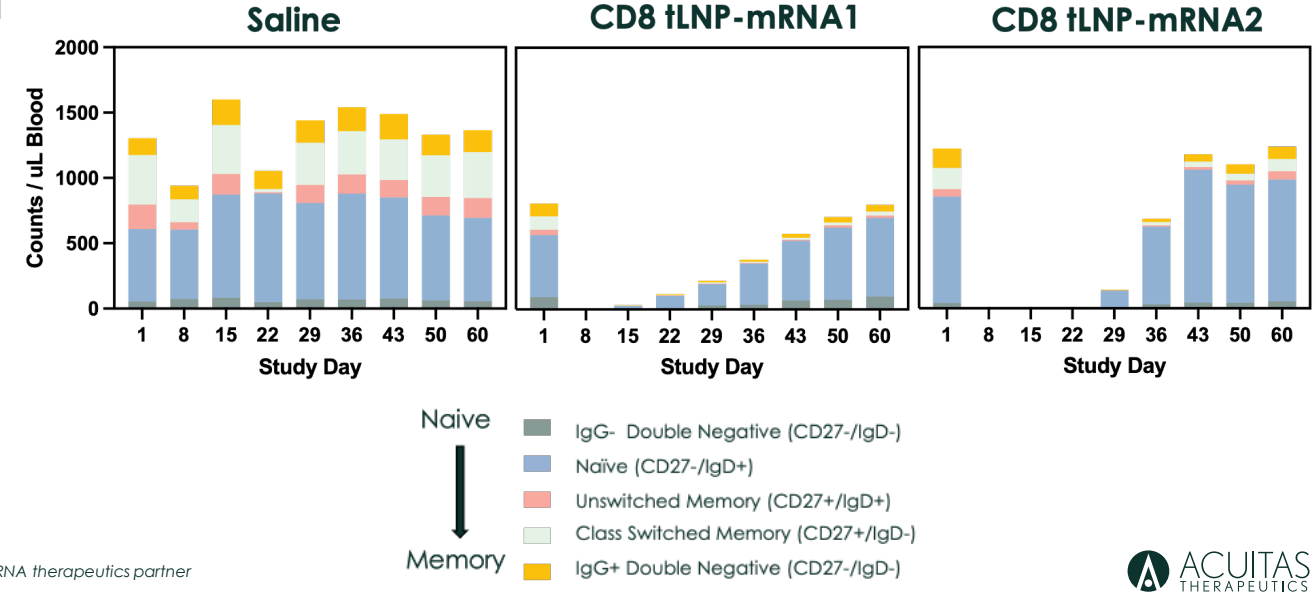
Upon repopulation, B cells have predominantly naive phenotype.





CD8 tLNP-mRNA | 0.2mg/kg



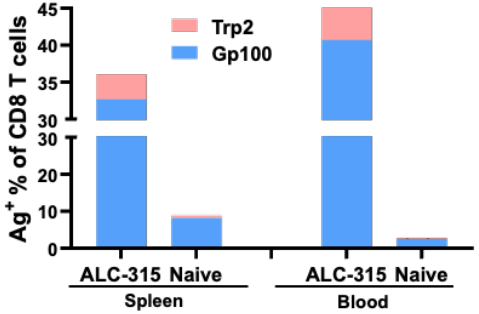


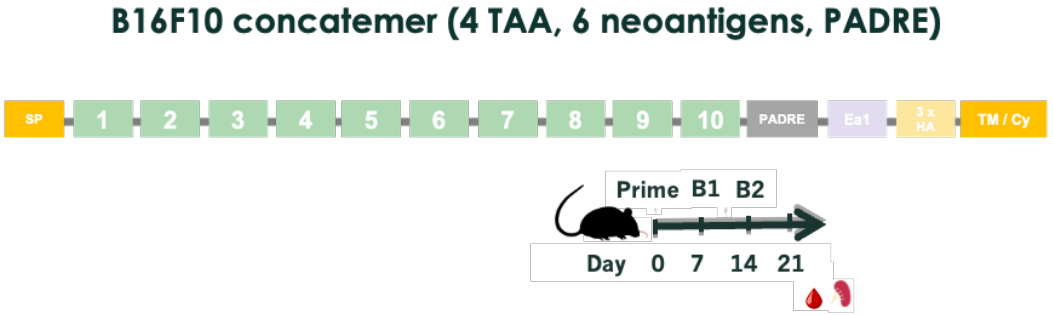
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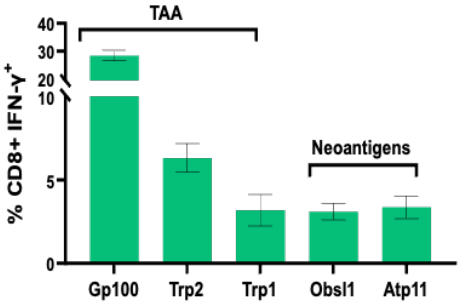
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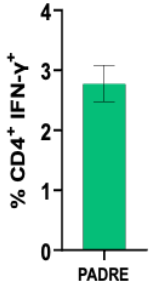
mRNA LNP Cancer Vaccines

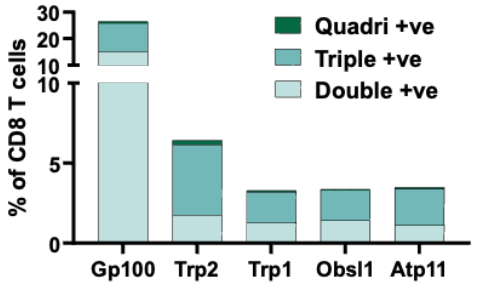
ALC-315® -based mRNA cancer vaccine elicits potent T cell response against syngeneic B16-F10 melanoma TAA and neoantigens.







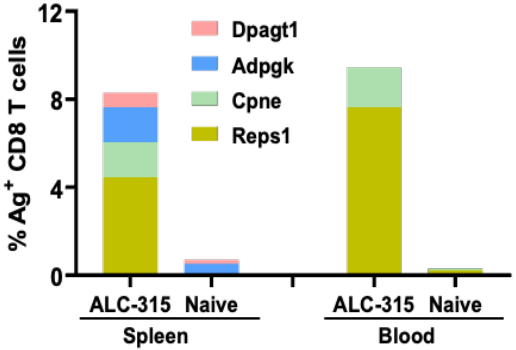


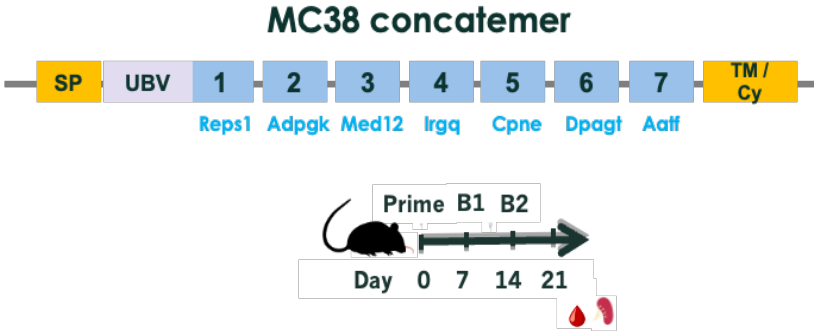


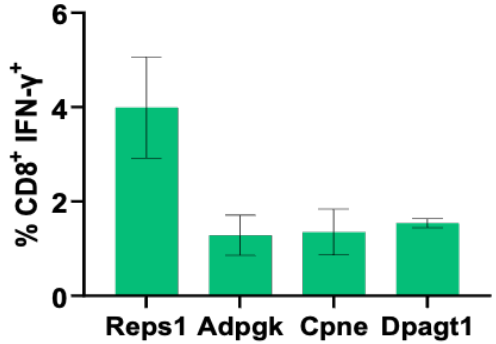
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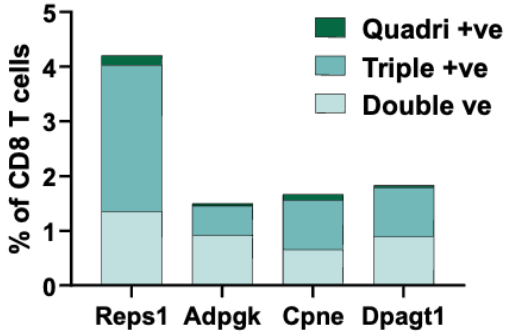
mRNA LNP Cancer Vaccines

ALC-315® -based mRNA cancer vaccine elicits potent T cell response against MC38 colon cancer neoantigens.





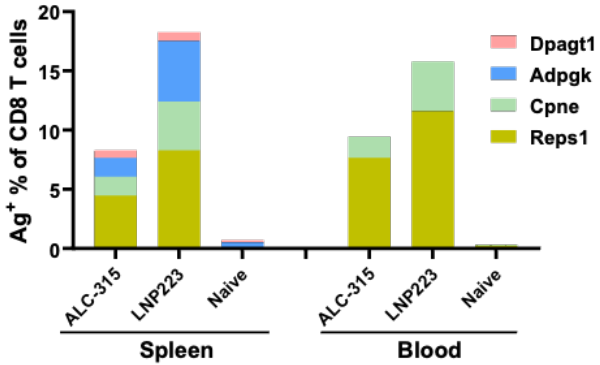


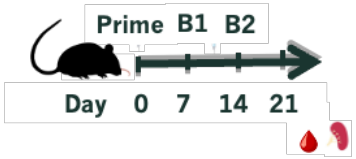


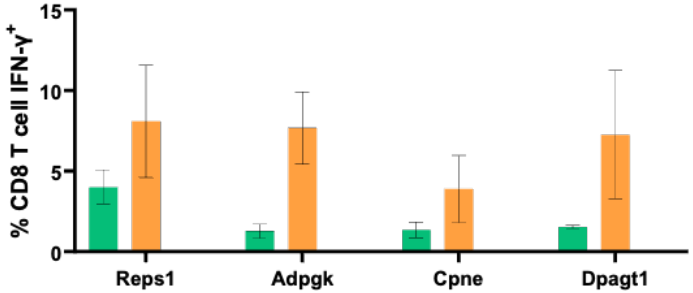
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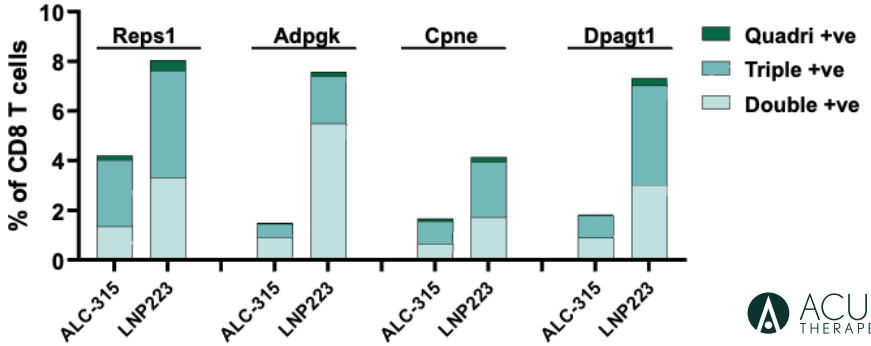
mRNA LNP Cancer Vaccines

LNP223, a novel LNP, has superior performance compared to an ALC-315® – based mRNA cancer vaccine against MC38 colon cancer neoantigens.





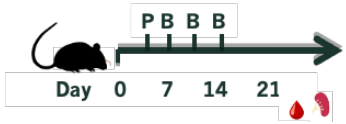


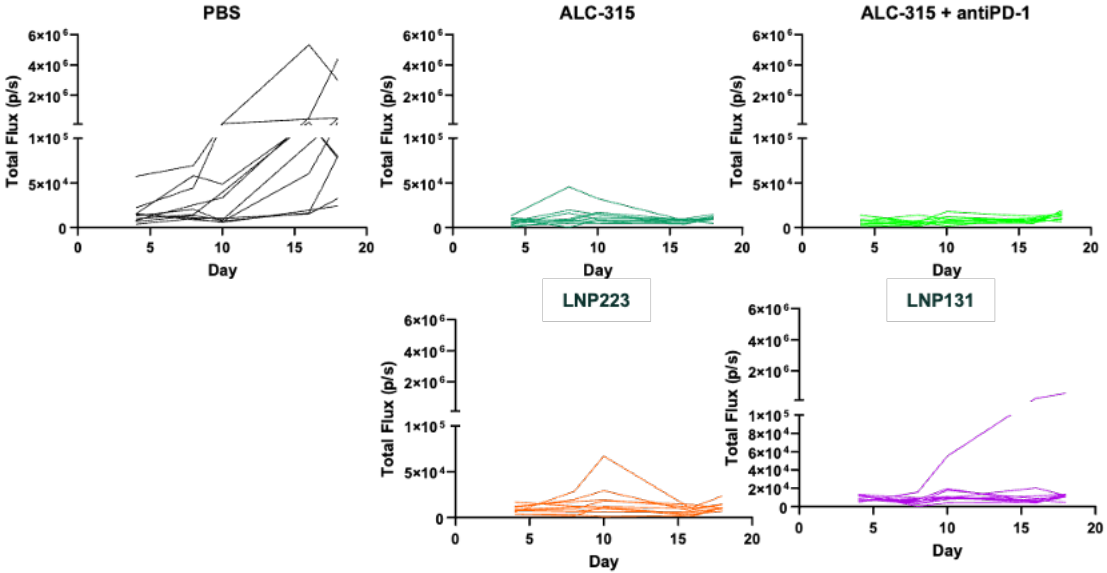


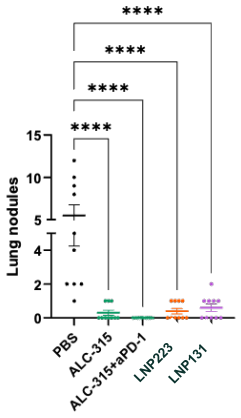
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mRNA LNP Cancer Vaccines

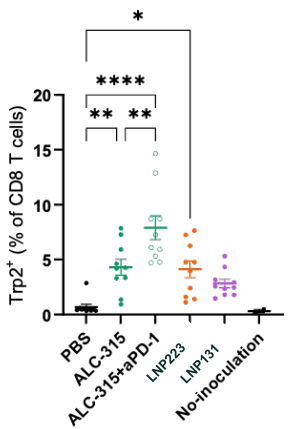
Anti-tumour efficacy following therapeutic vaccination with novel LNP-mRNA in mouse bearing syngeneic B16-F10 melanoma.







Lung nodules count at termination (Day 18)

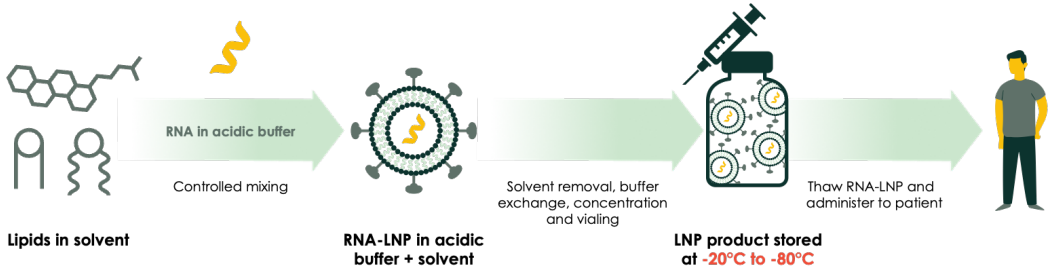


Blood TRP2 CD8 tetramers at termination

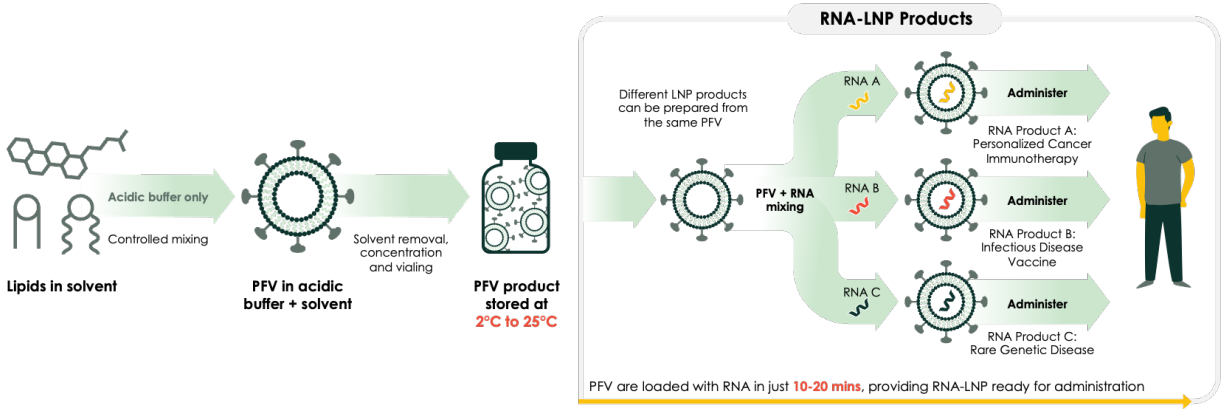
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mRNA LNP Manufacturing Using PFV

Conventional



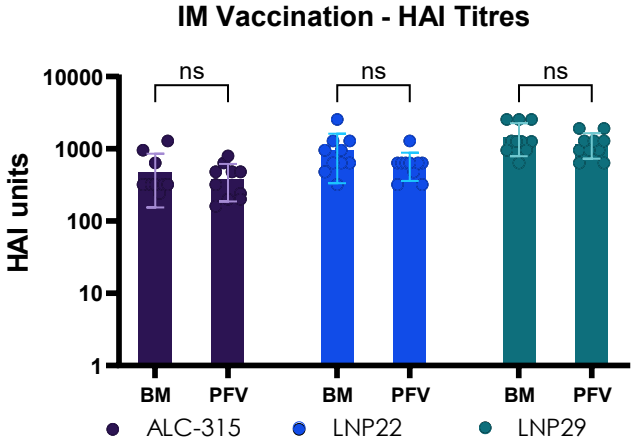
PFV



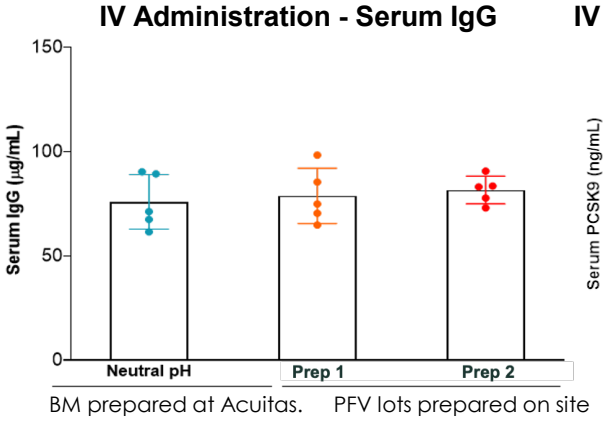
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mRNA LNP Manufacturing Using PFV

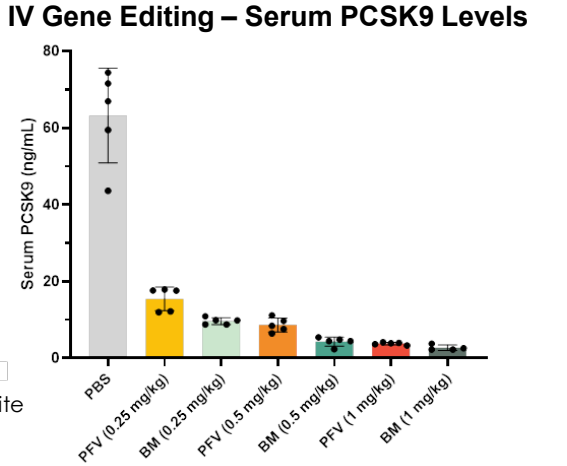
Relative potency of mRNA LNP manufactured by PFV following IM (prophylactic vaccine) and IV (mAb expression and base editing) administration.



Equivalent *in vivo* vaccine performance across 3 LNP following IM administration



Equivalent expression of mRNA encoded mAb following IV administration

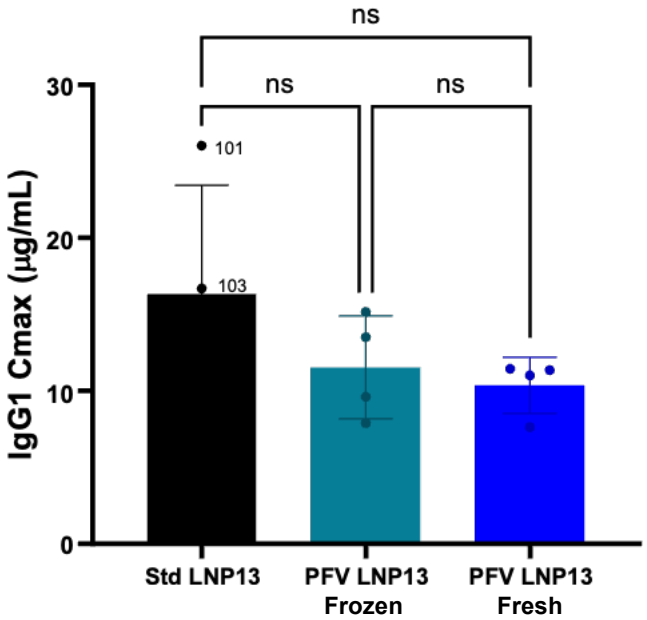


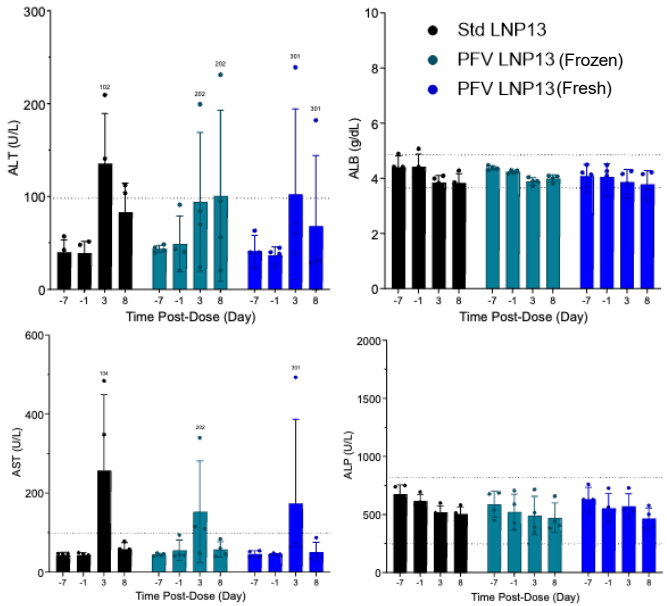
Comparable gene editing levels following IV administration

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mRNA LNP Manufacturing Using PFV

Relative potency and safety of mRNA LNP manufactured by PFV following IV (mAb expression) administration in NHP.





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Summary

- **Targeted LNP (tLNP) for extrahepatic delivery enables *in vivo* CAR T cells for autoimmune reset:**
  - Delivery of CAR mRNA to CD8 T cells using tLNP resulted in:
    - Expansion of CD8 T cells and high frequency/proportion of CAR-expressing CD8 T cells
    - Substantial depletion of B cells in peripheral blood, BM, spleen, and LN
    - Recovery of B cells within 30-60 days, predominantly with a naive phenotype

- **mRNA-LNP vaccines encoding syngeneic tumour-associated and/or neoantigens:**
  - Induced strong and broad T cell responses against the majority of syngeneic immunogens
  - Newly identified LNP induce superior, multifunctional T cell responses
  - Inhibited tumour growth and improved survival in animals with established tumours

- **PFV-based manufacturing:**
  - Enables modular, flexible, and cost-effective production amenable to patient specific therapies
  - Comparable physical/chemical properties, stability, and *in vivo* performance to conventionally manufactured LNP


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