

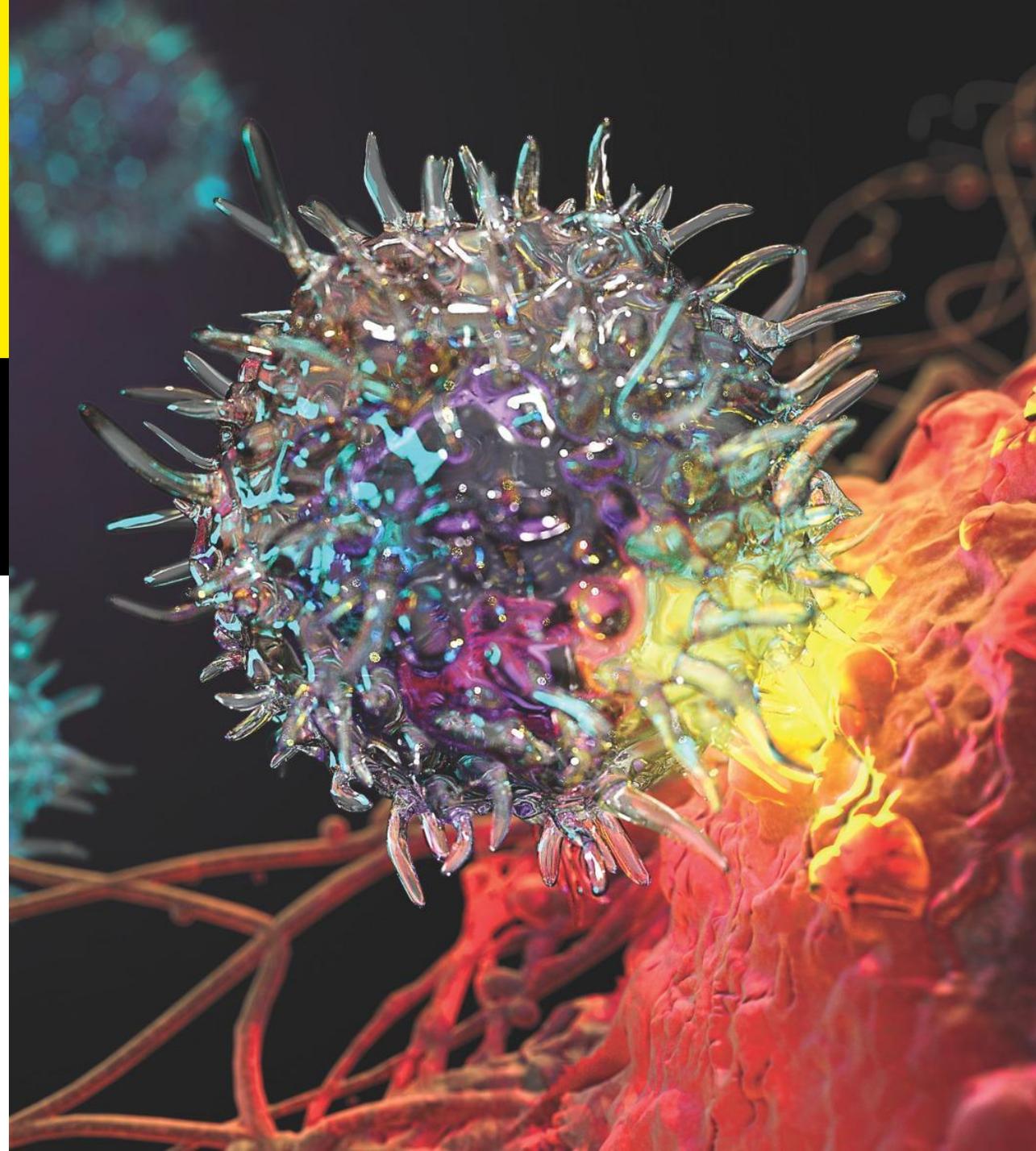
SARTORIUS

Simplifying Progress

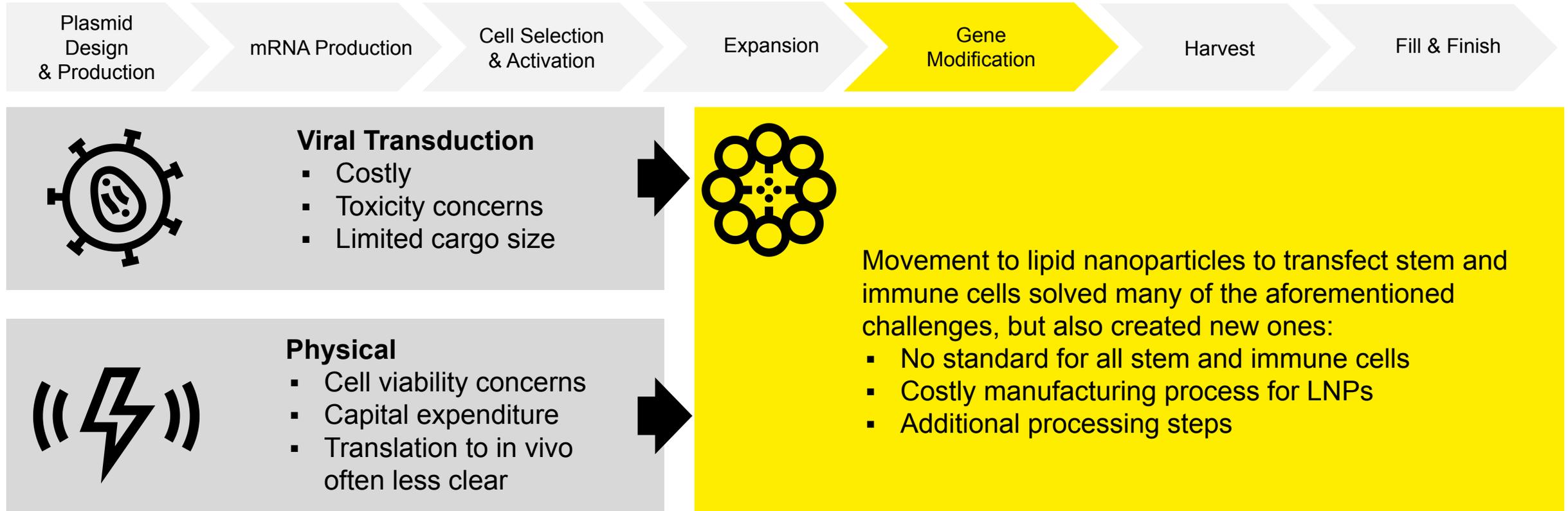
Democratizing Gene-Modified Cell Therapy Through Lipid-Based Transfection

February 2026

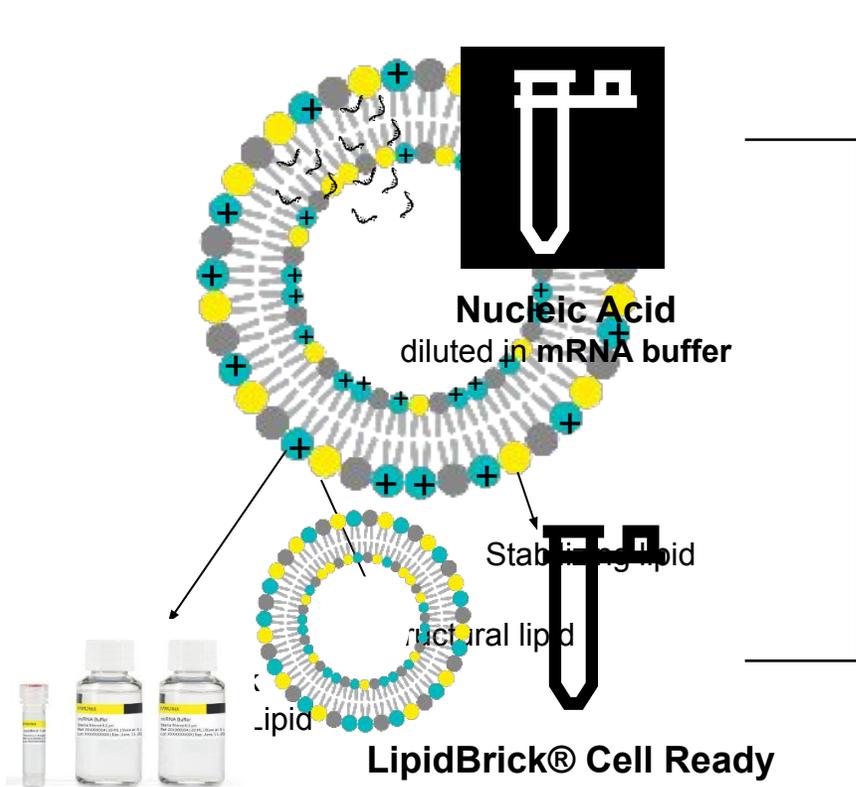
Ian Villamagna, PhD



Challenges in the Gene Modified Cell Therapy Process



Decoupling Gene Modification from Manufacturing Complexity

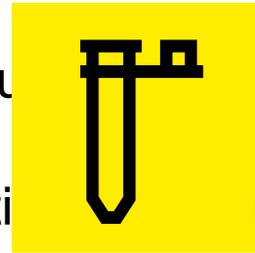


Pre-formed vesicles provide LNP-like delivery performance without the manufacturing complexity inherent to LNP formulation

• Encapsulation
No manufacturing

• 15 min at RT
One formulation
workflows

• Loadable with various payloads: mRNA, sgRNA, pDNA, oRNA, etc.



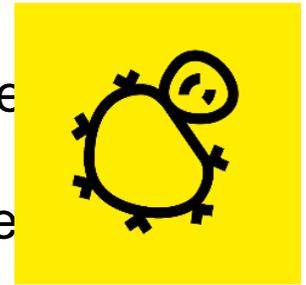
Transfection mix

Can be stored:

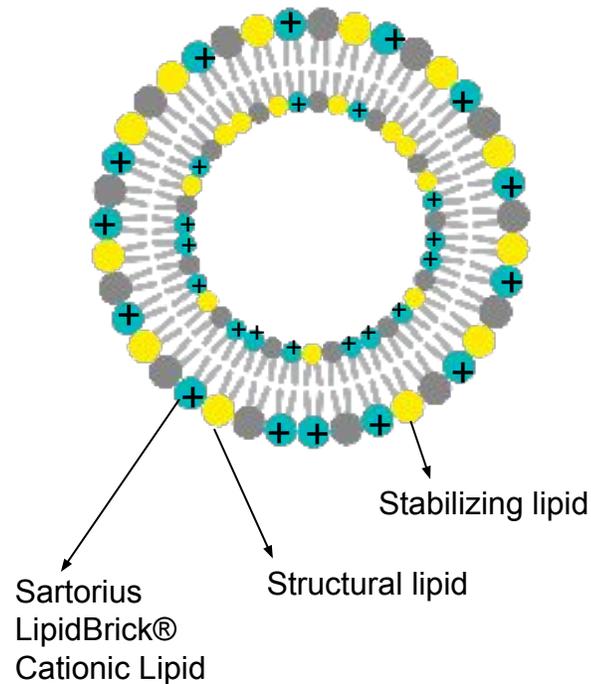
- Up to 1 month at 4°C
- Up to 1 month at -80°C (using a cryoprotectant)

Transfection
ready-to-use

• Cable to multiple cell



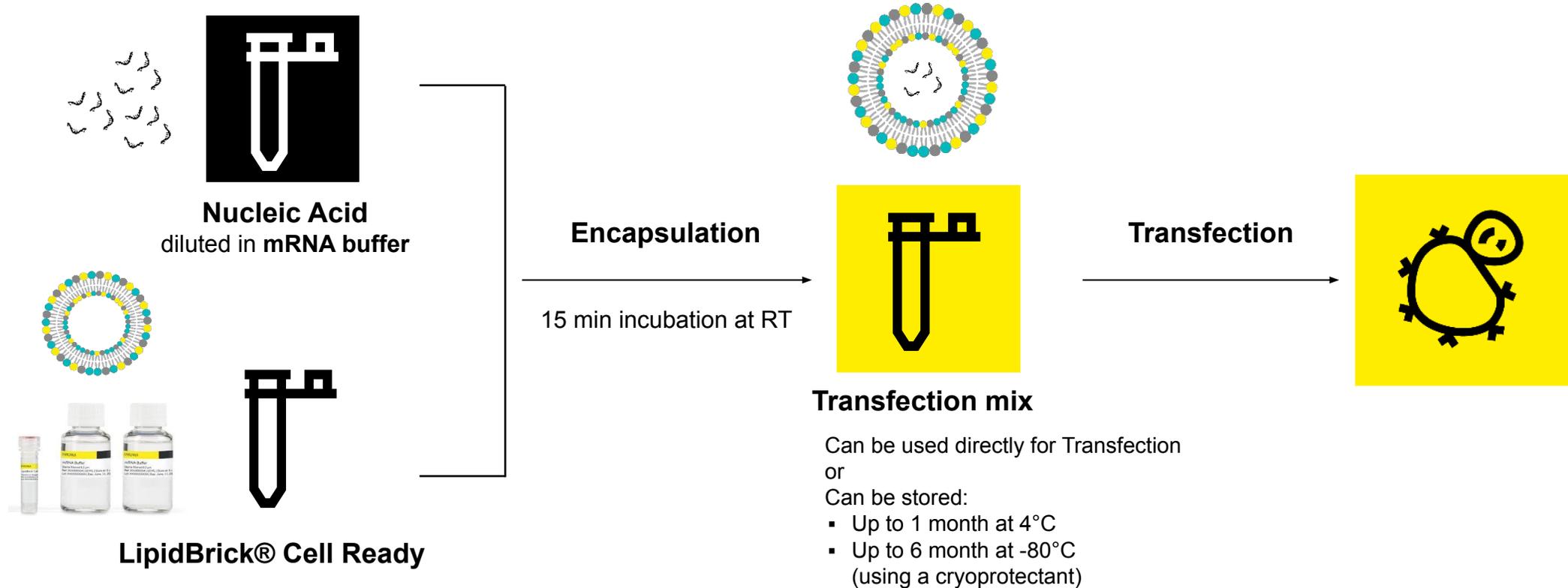
Decoupling Gene Modification from Manufacturing Complexity



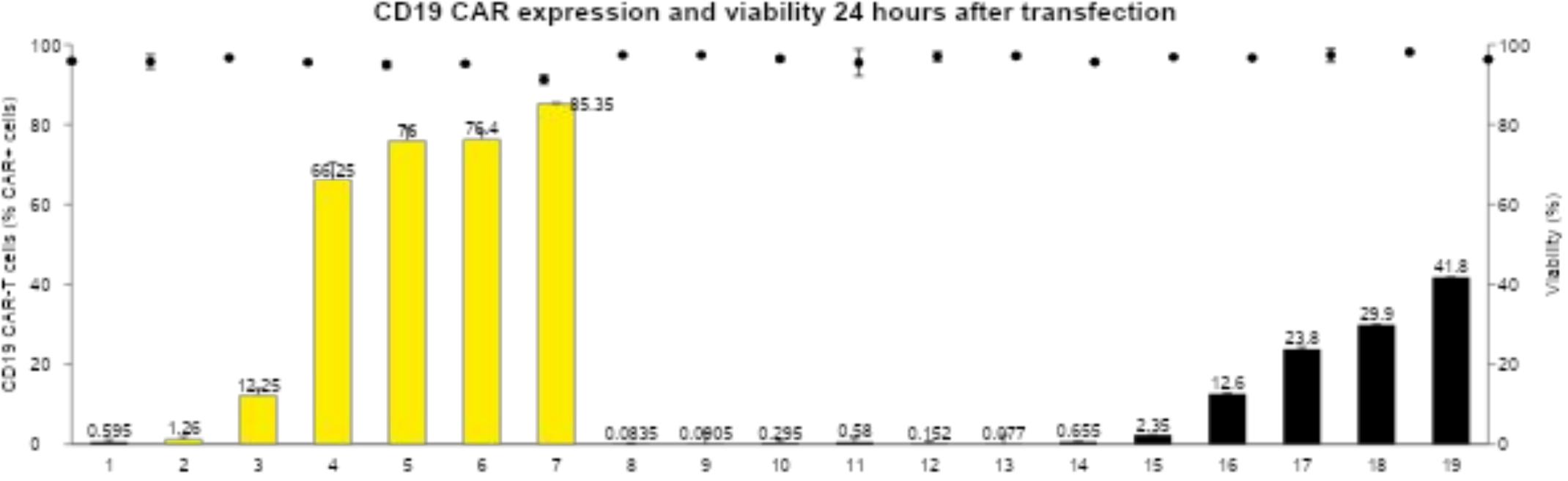
Pre-formed vesicles provide LNP-like delivery performance without the manufacturing complexity inherent to LNP formulation

- No manufacturing constraints, ready-to-load solution
- One formulation is applicable to multiple cell types and workflows
- Loadable with various payloads: mRNA, sgRNA, pDNA, oRNA, etc.

Decoupling Gene Modification from Manufacturing Complexity



Enhanced CAR mRNA Expression Compared to LNP Kit

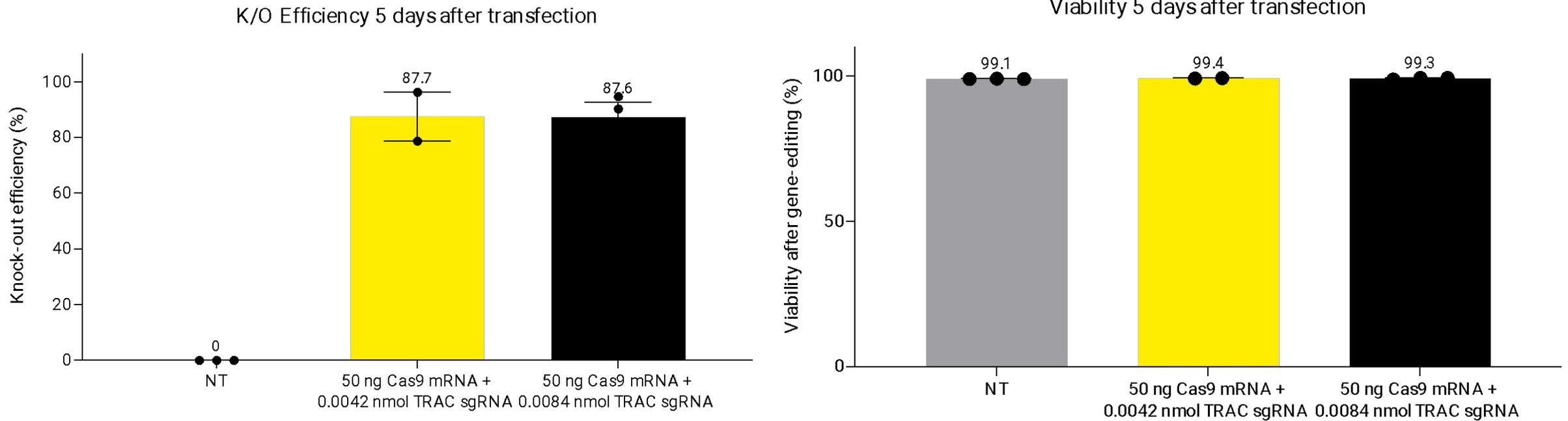


- **Efficient CAR Expression:** >85% CAR T generation
- **High Cell Viability:** >90%, ensuring healthy and functional cells
- **Low mRNA Use:** 0.8 µg per 10⁶ T cells, optimizing resource use
- **No ApoE Supplementation Needed:** Unlike standard LNPs

Dose: µg mRNA/million cells

LipidBrick® Cell Ready- PFVs for Cell Therapy Applications: Complex Payloads and Various Cell Types

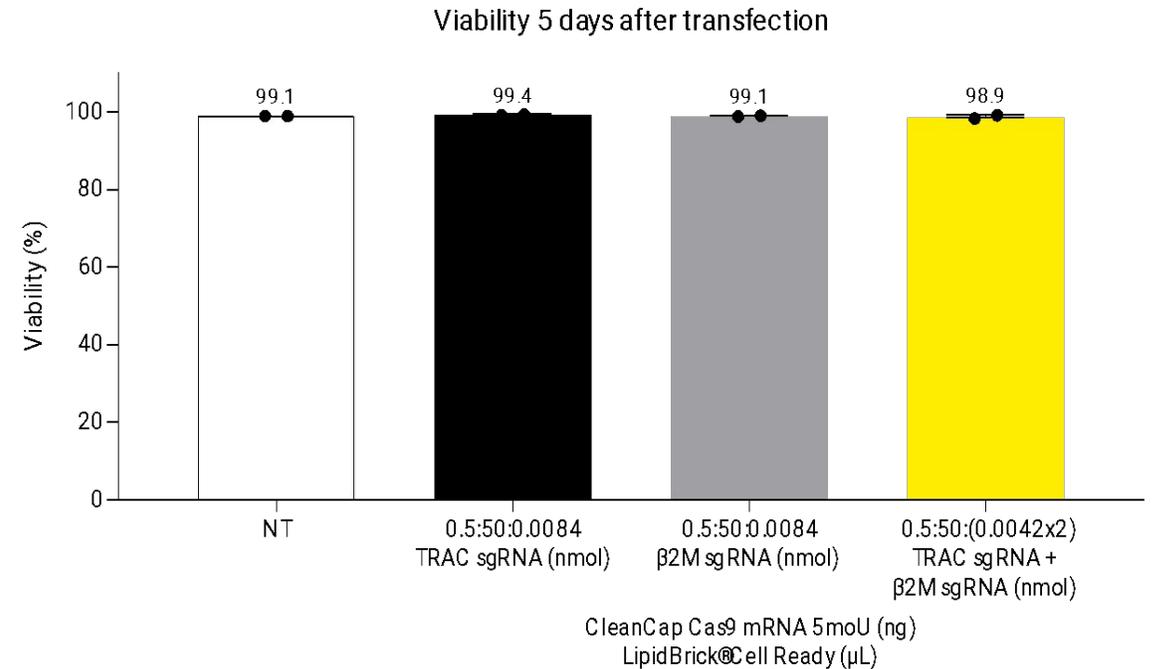
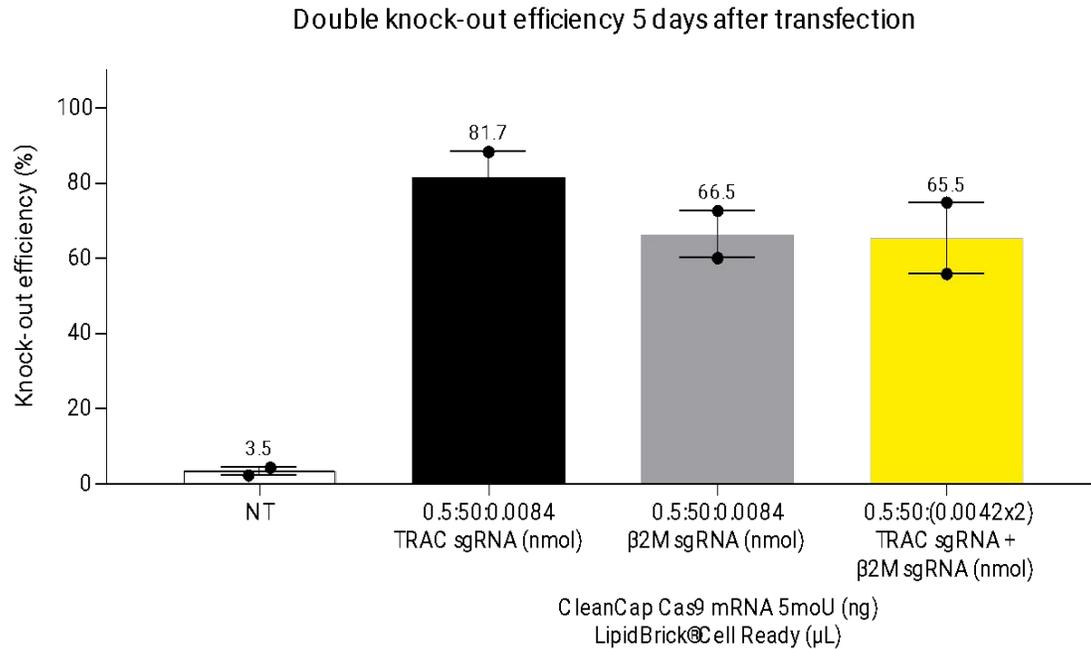
Efficient Stable Editing: Optimized TRAC Knockout in Primary T Cells



LipidBrick® Cell Ready delivers complex cargos while maintaining viability of cells: Further Cas9mRNA/sgRNA Ratio Optimization & total mRNA amount could increase the KO efficacy

50 ng Cas9 mRNA + 0.0042 or 0.0084 nmol sgRNA + 0.6 μ L of LipidBrick Cell Ready/187 500 cells (96WP) □ 0.27 μ g Cas9 mRNA + 0.73 μ g or 1.45 μ g sgRNA + 3.2 μ L of LipidBrick Cell Ready/million cells
n=2 or 3 donors

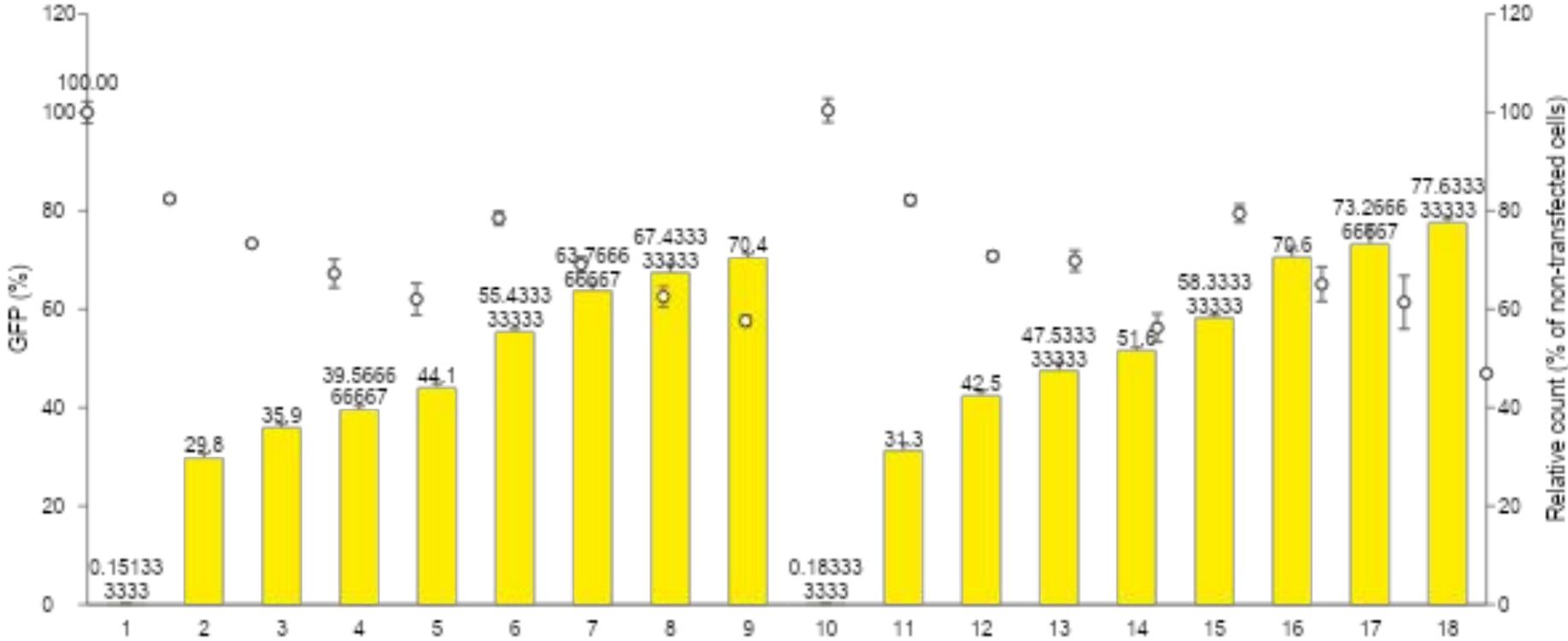
Efficient Stable Editing: Double Knockout TRAC & β 2M in Primary T Cells



High double KO efficacy obtained with LipidBrick® Cell Ready while maintaining high cell viability (>90%)

50 ng Cas9 mRNA + 0.0084 nmol sgRNA + 0.5 μ L LipidBrick® Cell Ready per well (96-well plate)
 □ 0.27 μ g Cas9 mRNA + 1.44 μ g TRAC sgRNA + 2,67 μ L LipidBrick® Cell Ready /million cells
 n=2 donors

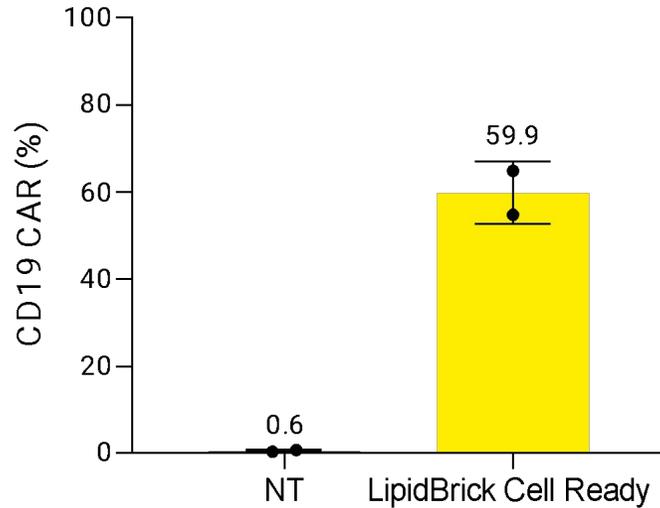
Advancing Early-stage DNA Delivery in T Cells: pDNA & Nanoplasmid



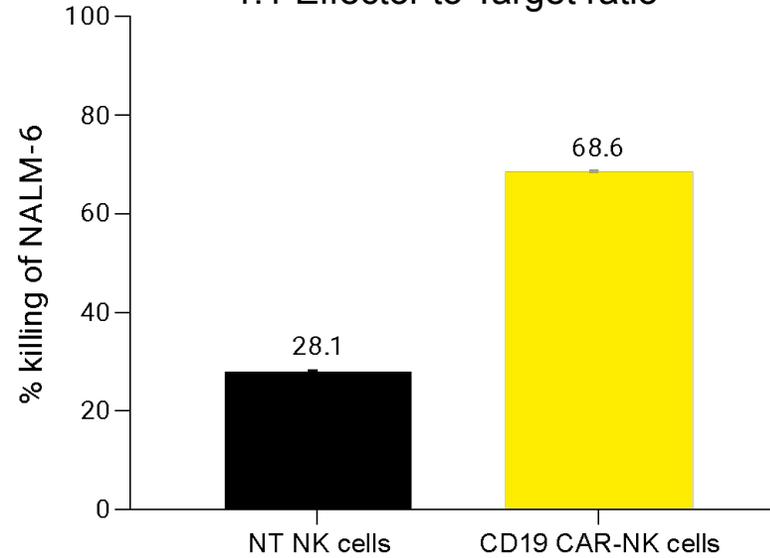
- Nanoplasmid delivery leads to higher efficacy
 - Variability is higher with DNA than with RNA
- Advancement for Knock-In delivery purposes

NK Cells: Efficient CD19 CAR NK Production and TIGIT Knock Out

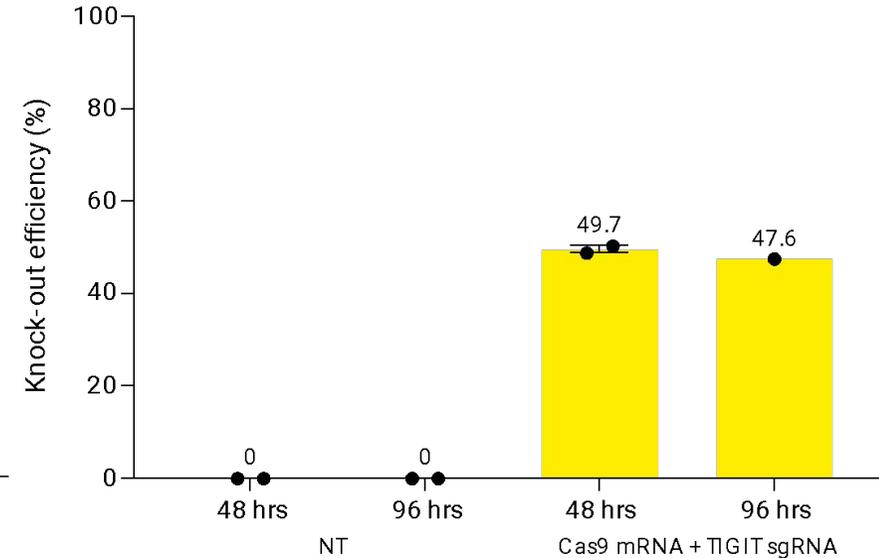
CD19 CAR expression in Primary NK Cells after 24 hours



Killing Assay onto NALM-6 cells 1:1 Effector to Target ratio



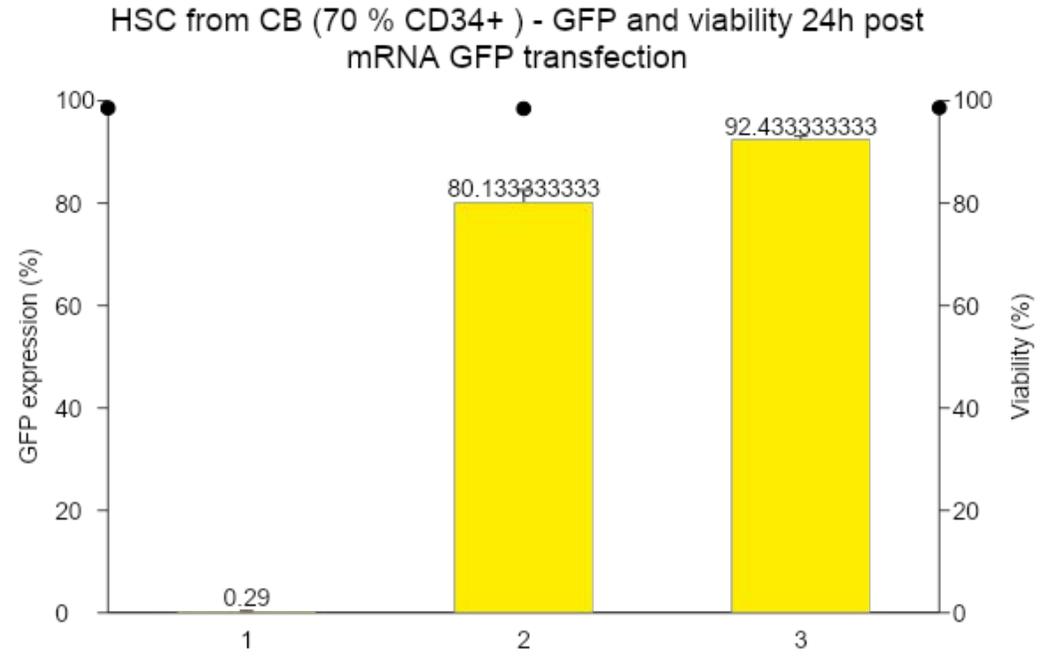
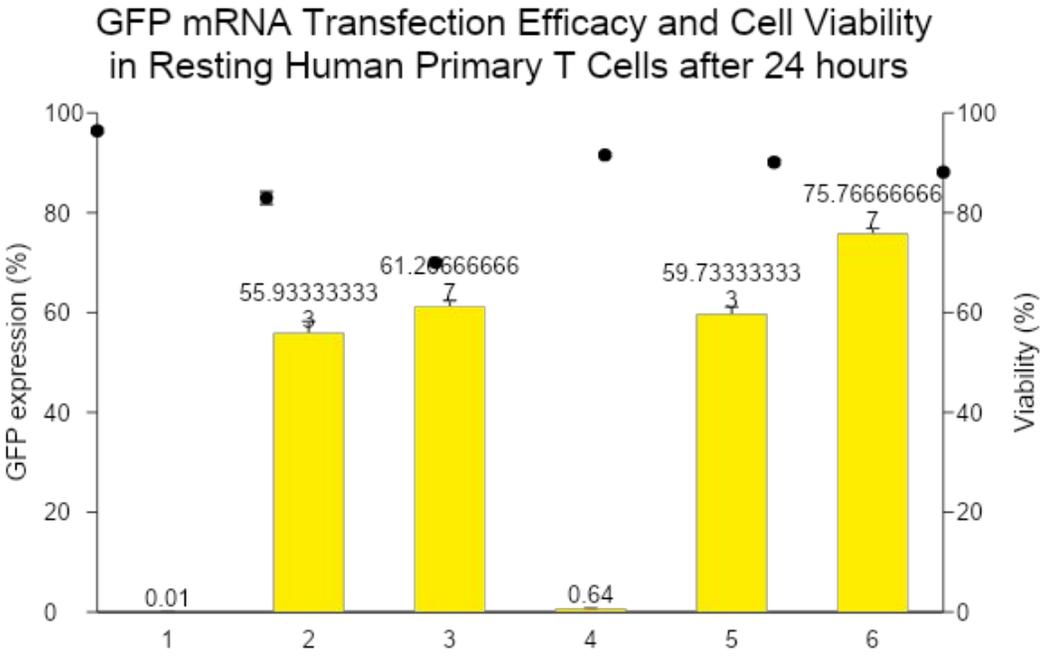
TIGIT KO Efficiency



- Efficient CAR expression in additional cell types: primary NK cells
- Efficient killing of target cells by CAR-NK cells generated with LipidBrick® Cell Ready
- Efficient editing with Cas9 mRNA + TIGIT sgRNA

Dose: 0.8 µg/million cells

Efficient Transfection of Various Cell Types: Unactivated T-Cells and CD34+ HSCs

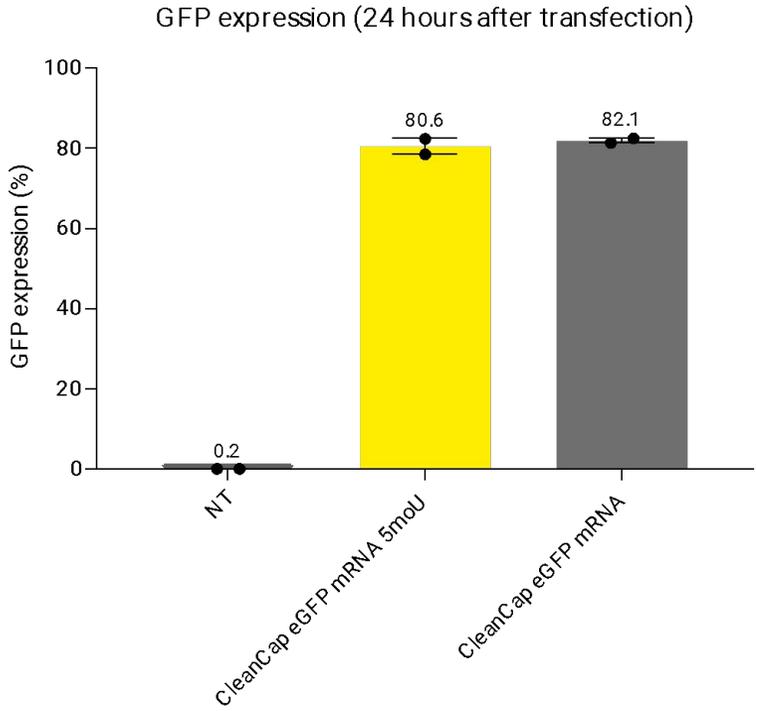


- Non-activated human primary T-Cells showed similar levels of expression as compared to activated after 24 hours
- LipidBrick® Cell Ready shows robust effectiveness in both immune and stem cells

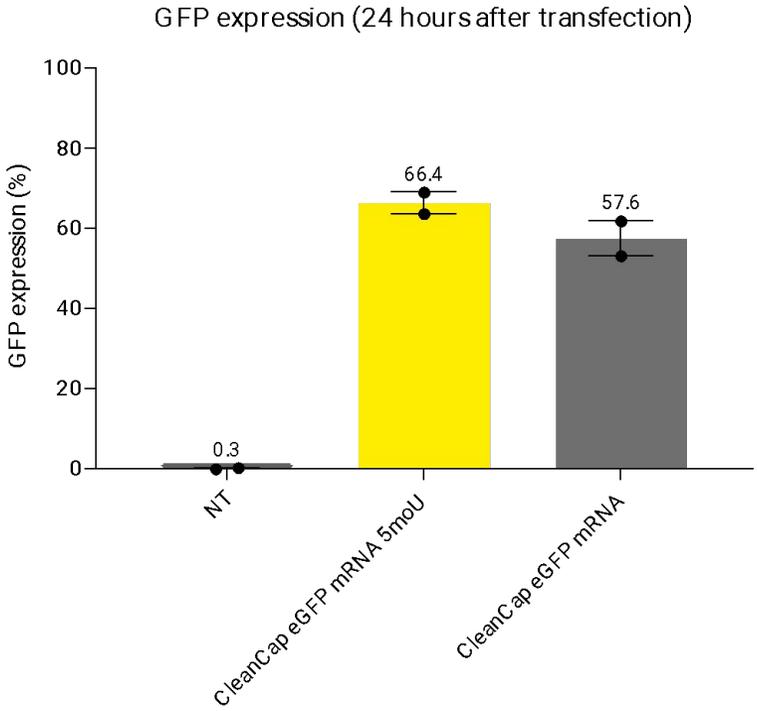
0.3 µg eGFP mRNA + 0.6 µL LipidBrick® Cell Ready per well (96-well plate)
 1.6 µg eGFP mRNA + 3.2 µL LipidBrick® Cell Ready per million cells
 n=2 donors

Efficient Transfection of Various Cell Types: MoDCs and M2 Macrophages

MoDCs



M2 Macrophages



- Early work has shown promise in transfecting less common immune cell types, including:
 - MoDCs
 - M2 Macrophages
 - B-Cells

0.3 µg eGFP mRNA + 0.6 µL LipidBrick® Cell Ready per well (96-well plate)
1.6 µg eGFP mRNA + 3.2 µL LipidBrick® Cell Ready per million cells
n=2 donors

Summary & Conclusions

LipidBrick® Cell Ready Enables Cell Therapy Development at Rapid Pace



Cost-Efficiency:

Ready-to-use, no specific equipment nor consumables, use low amount of nucleic acid



High Performance: Excels in transient expression & stable modification using gene editing



High Cell Viability: Gentle, preserve cell growth and function



Scalability: From well-plate screenings to bioreactor production



Versatility:

Diverse nucleic acid payloads across various cell types

- Pre-formed vesicles (PFVs) allow for a streamlined process that can replace traditional (viral, electroporation), and even less traditional (LNP) approaches to cell modification and engineering in cell therapy processes.
- PFV process allows for the benefits of LNP transfection, but without LNP development, strenuous analytics, manufacturing concerns
- Simple process is scalable, and translates to GMP readiness easily



LipidBrick
®
Cell Ready

Want to Learn More?

Explore our Solutions for Cell Therapy at Booth #263



Posters On Display

- Innovative Lipid-Based Delivery Solution: Unlocking New Opportunities in Manufacturing Genetically Modified Immune Cell Therapies
- Elevate Bio Poster Presentation: Automating Cell Therapy: A Closed, Digital Platform Delivers Consistent, Sterile, Scalable T Cell Production
- Perfusion-Enabled CAR-T Manufacturing in Stirred-Tank Bioreactors Achieving 100+ Doses Per Batch
- Scalable Hematopoietic Stem Cell Expansion in Stirred-Tank Bioreactors for Advancing Cell Immunotherapies

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