



A Novel, Synthetic DNA for Cell and Gene Therapy Application

Alexander Pekarsky, Luca Distefano, Marco Guarrera, Ivana Pastierikova, David Wilson, Martin Cusack, Anna Krutyholowa, Roxanne Lourman, Fabian Trick, Gustavo Lou, Andreia M Silva, Ileana Guerrini, Nicolas Meier, J. Omar Yáñez-Cuna, Joel de Beer
Anjarium Biosciences AG, Wagistrasse 23, 8952 Schlieren, Switzerland



Scan to visit our website

Introduction

Demand for DNA as a critical starting material for viral vector manufacturing, mRNA production, and gene therapy delivery applications continues to rise, increasing the need for efficient, timely, and scalable DNA manufacturing.

Our One-pot Enzymatic DNA Synthesis

Anjarium's novel, cell-free enzymatic approach for producing linear, double-stranded DNA enables a complete range of applications with significantly faster delivery times than traditional methods.

Our enzymatic DNA synthesis provides multiple benefits:

- **Purity:** Synthetic DNA is devoid of bacterial sequences.
- **Scale:** DNA batches ranging from microgram to multigram produced in small bioreactors with minimal reagents.
- **Speed:** Production time takes just weeks from circular DNA template to vial delivery.
- **Stability:** Hairpin-ended structures, inspired by nature, protect the integrity of the DNA and provide specific functionality in certain applications.
- **Flexibility:** Complex and customized transgene sequences can be produced.

Anjarium's Synthetic DNA (ANJ-DNA)

ANJ-DNA is designed to catalyze advanced therapy research and clinical development programs across AAV, mRNA, Lentivirus and other applications.

Here we show the broad versatility of our synthetic DNA demonstrating its use as input for mRNA, rAAV, and lentivirus vector production, as well as for use in transgene expression *in vivo*.

Our synthetic DNA is well-positioned to replace plasmid DNA as key starting material for cell and gene therapy modalities.

ANJ-DNA allows the use of different nature-inspired ITRs

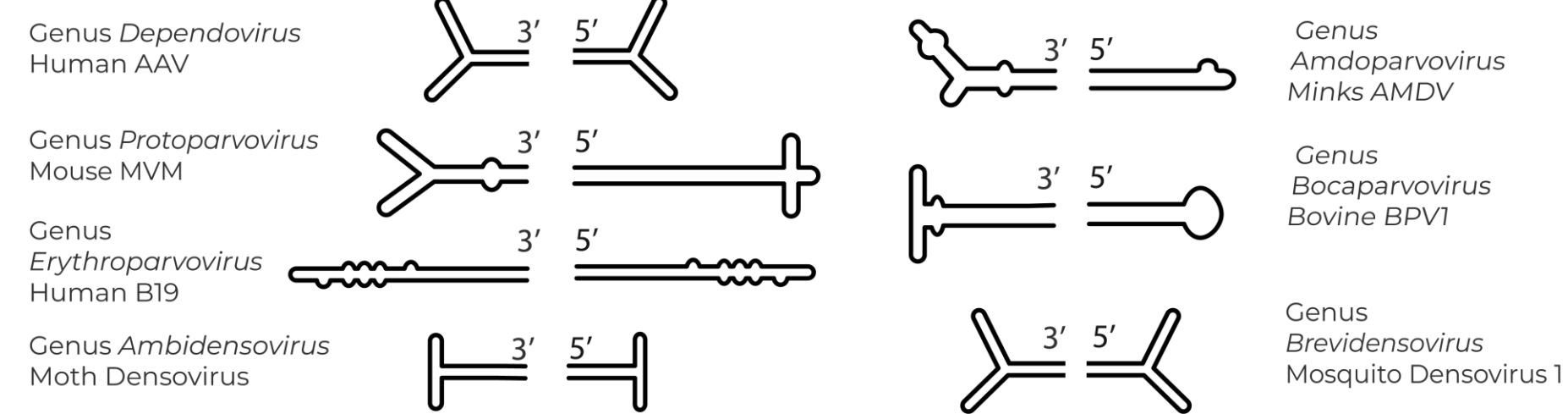
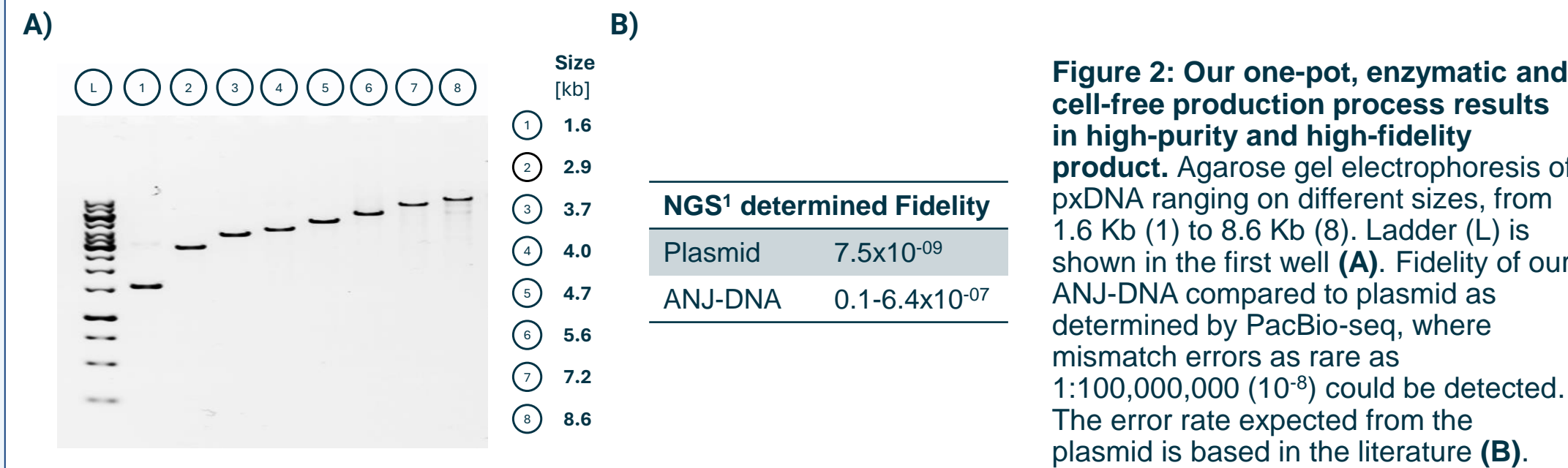
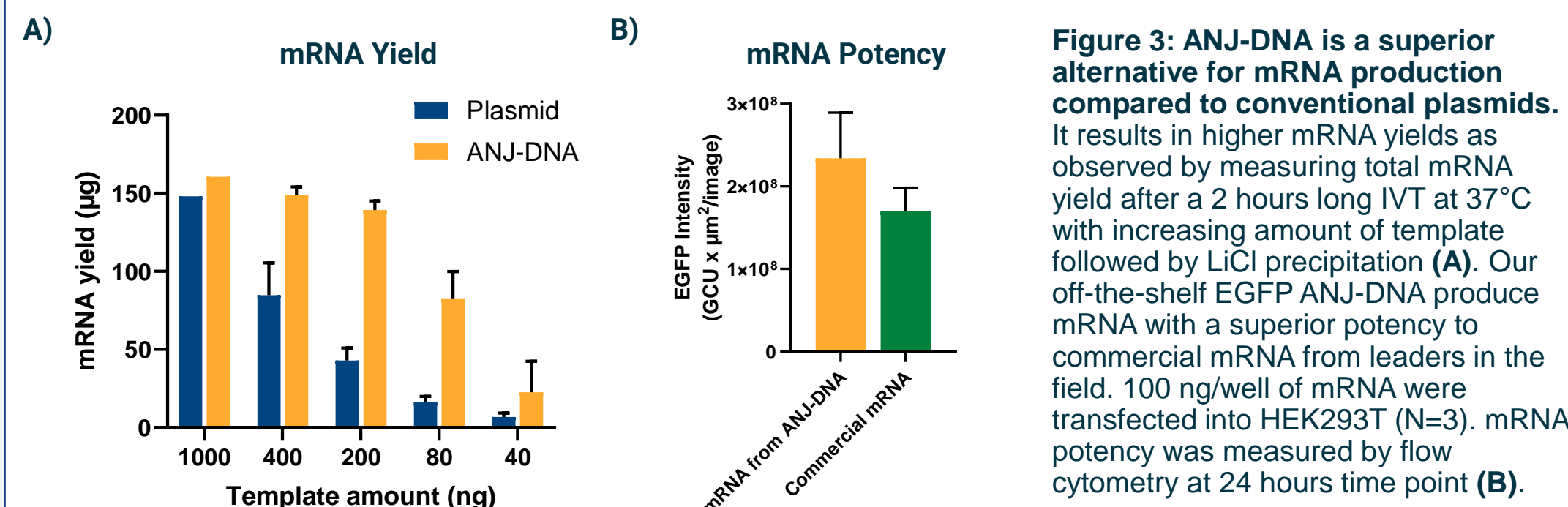


Figure 1: ANJ-DNA can leverage from different natural hairpin-ended structures and synthetic ITRs inspired by nature. Shown is a schema of an ANJ-DNA with the gene expression cassette (in blue) flanked by hairpin-ended structures (top). The hairpin ended structures can be customized to mimic ITRs from different viruses.

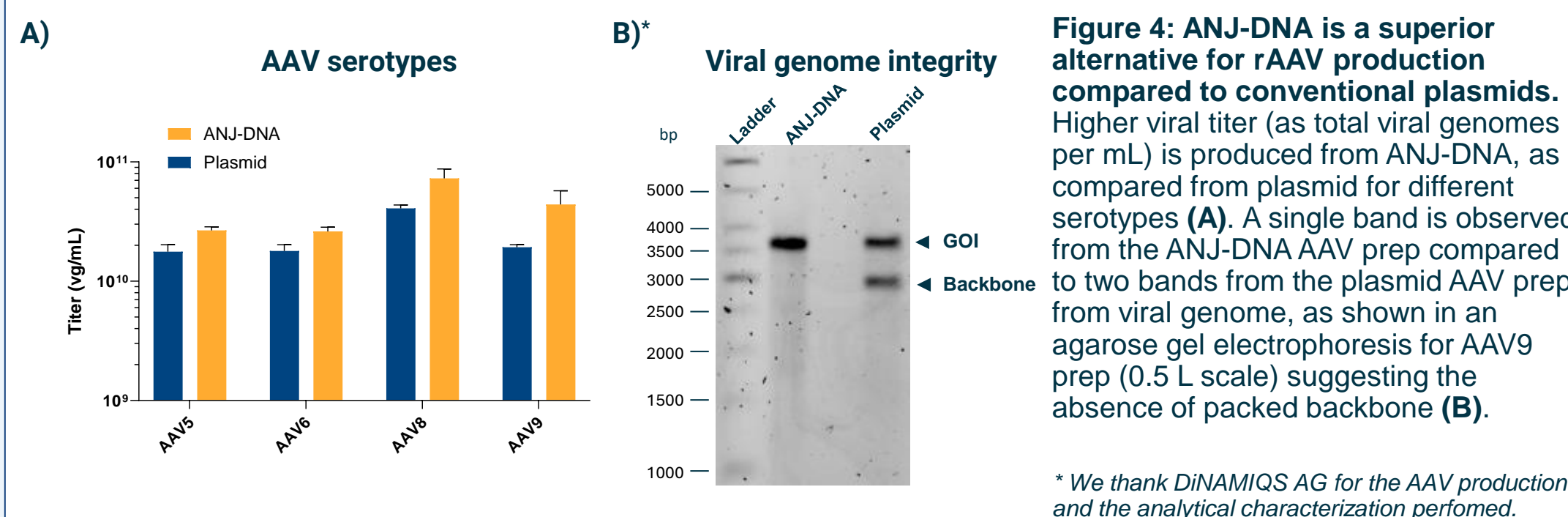
Anjarium's Process Results in High-purity and High-fidelity DNA Product



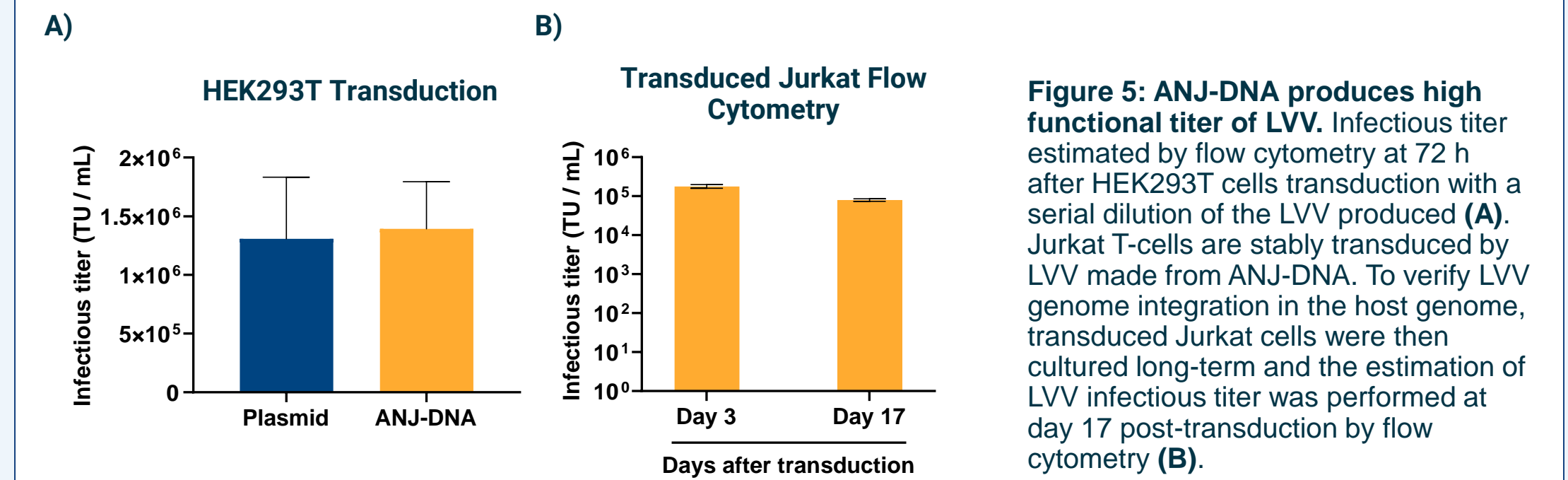
ANJ-DNA is a Superior Alternative for mRNA Production



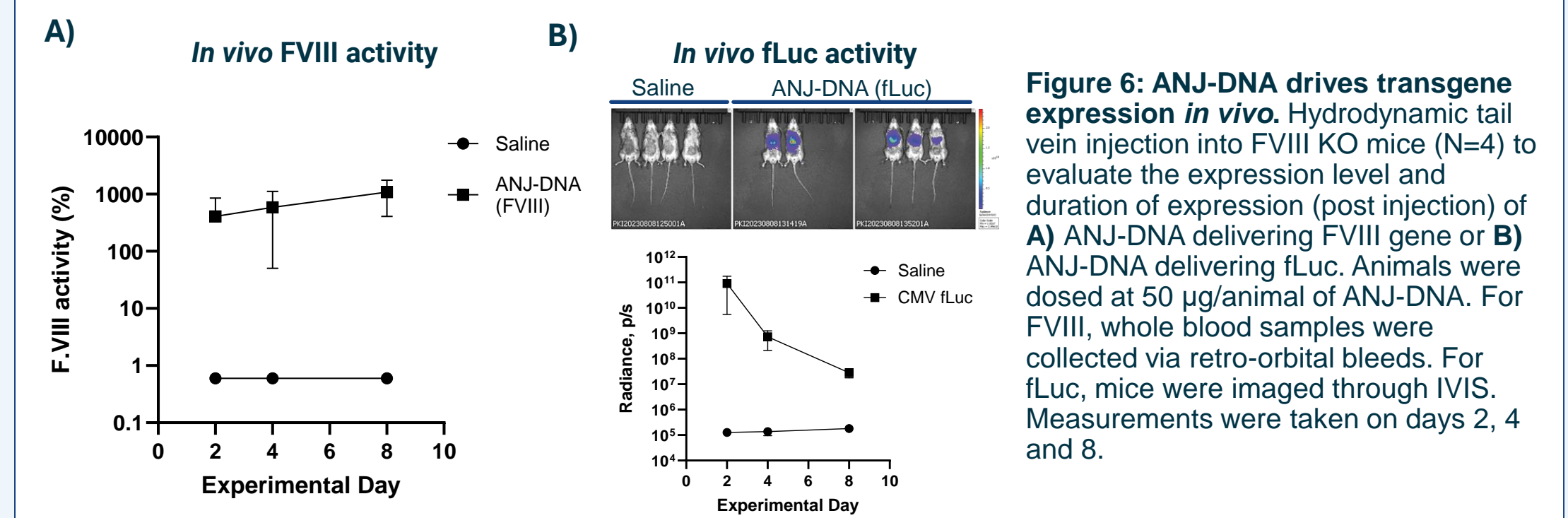
ANJ-DNA Outperforms Plasmid for rAAV Manufacturing



ANJ-DNA Produces High Functional Titer of Lentivirus Virus Vector



ANJ-DNA Drives Transgene Expression In Vivo



Conclusions

Our synthetic DNA proves to be a versatile input material for cell and gene therapy

- ANJ-DNA is a superior alternative as a starting material for AAV and mRNA production compared to conventional plasmid
- Our off-the-shelf ANJ-DNA produce mRNA with potency higher than commercial mRNA from leaders in the field
- Absence of packed backbone in the AAV produced from ANJ-DNA
- ANJ-DNA can be used for LVV production at a functional infectious titer comparable to plasmid