All-in-one delivery of gene-editing system into primary cells and in vivo using LentiFlash®, an MS2-chimeric viral RNA delivery tool designed for clinical applications.





### A. Gene disruption



- Activated T cells were transduced by a range of LentiFlash® vector (from 0 to 5 pg of p24/cell) expressing Cas9 and a sgRNA targeting the human PD1 gene.
- Lentiflash® delivering Cas9 is used as negative control of PD1 editing.
- PD1 expression is analyzed by FACS 6 days after transduction, as well as cell viability and expression of TCR and CD25.

Human T lymphocytes display high KO efficiency using highly purified and concentrated LentiFlash<sup>®</sup> vectors without affecting viability and proliferation, and preserving the original cell phenotype.



## **B. Exon skipping**







#### **Duchenne disease model:**

- No cure, and current pre-clinical strategies use AAVs (DNA delivery) to deliver gene editing systems
- Restoration of DMD activity using LentiFlash®

FACS

analysis

- Transient expression of gene editing enzymes minimizes the off-target activity
- Collaboration with Institut Clinique de la Souris (ICS)
- A highly promising therapeutic strategy to repair the defective dystrophin gene



# LENTIFLASH® : A NEW TOOL FOR KNOCK-IN APPLICATIONS



## PRODUCTION OF HIGHLY PURIFIED AND CONCENTRATED LENTIFLASH® PARTICLES



VSVG

Lentiviral vector







- Harvesting
- Concentration
- Purification

**LentiFlash**®