

# GENE & CELL THERAPY



## Transgene Expression (*in vitro* and *in vivo*)

- mRNA (target & off-target tissues, clinical/preclinical)
- Protein (intracellular, secreted or membrane expression)

## Biodistribution (pharmacokinetics)

- qPCR/RT-qPCR (various tissues and matrices, animal/human)
- Automated tissue processing/nucleic acid extractions

## Vector Shedding & Toxicity

- qPCR/RT-qPCR (secreta/excreta)
- Cytokine release panels (MSD, ELISA, ProteinSimple)

## Immunogenicity and Pharmacodynamics

- Transgene or vehicle immunogenicity (TAb & NAb)
- Cellular immunity (T cell activation, granzyme B, IFN $\gamma$ )
- Evaluation of target pathway(s) through RT-qPCR or LBA

## Modalities

### Gene Therapy

- AAV
- mRNA/LNP
- Lentivirus
- Oncolytic viruses

### Cell Therapy

- CAR-T
- TCR
- TILs
- Non-T cell



## Technologies

- QuantStudio™ 7 Pro Real-Time PCR
- KingFisher™ Flex Purification System
- CytoFLEX S Flow Cytometer