

CELL & GENE THERAPY STUDIES

Biodistribution Studies • GLP Safety and Toxicology • Bioanalytical Support

CELL AND GENE THERAPY CHALLENGES

Regulatory guidelines for toxicology and safety pharmacology studies for advancing new cell and gene therapies to human clinical trials are still evolving. Unlike other therapy classes such as small molecules or biologics, preclinical study requirements must be individually tailored to the characteristics of the therapy. Biodistribution studies remain critical for determining vector distribution and persistence in both target and non-target tissues. In addition, cell and gene therapy studies necessitate diverse approaches including ex vivo gene editing, and in vivo viral and non-viral vector treatments.

HISTORY OF TOXICOLOGY EXPERTISE

IITRI offers an experienced toxicology team to provide expert assistance in planning and executing gene therapy preclinical studies and programs for IND submissions. We have been designing and executing GLP-compliant preclinical studies in compliance with FDA requirements since these guidelines were first instituted in 1979.

ANALYTICAL AND MOLECULAR SUPPORT

Our in-house supporting bioanalytical laboratory and extensive expertise in viral vector biology and molecular biology forms the foundation of our study team. This allows us to provide the specialized attention needed for cell and gene therapy studies under one roof to save sponsors time and resources.

GENE THERAPY VECTOR TYPES

- Viral (lentivirus, adenovirus, AAV)
- Naked plasma DNA, nanoparticles, RNA

GENE THERAPY PRECLINICAL STUDY TYPES

- Proof of concept (efficacy or gene expression studies)
- Biodistribution studies (vector and/or gene product quantitation in blood and/or tissues)
- GLP toxicology and safety

TOXICOLOGY BIOANALYSIS ASSAYS

- qPCR (DNA), RT-qPCR (RNA)
- ELISA – anti-drug antibody analysis
- ELISpot – cytokine-secreting cell quantitation assay
- Cytokine profiling (Luminex)

MULTIPLE MODELS AND ROUTES AVAILABLE

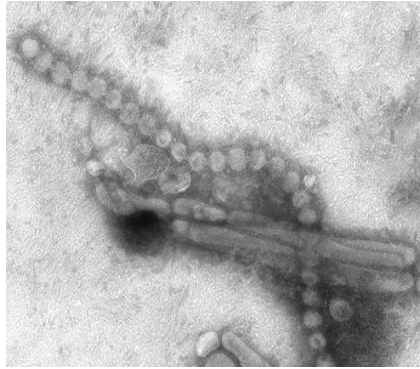
- All routes of administration available (including inhalation and electroporation)
- Extensive collection of animal models for efficacy studies (including in ABSL-2 and ABSL-3)
- Comprehensive offering of toxicology studies in all the typical research species including NHPs (marmoset, cynomolgus monkeys, rhesus macaques, african green monkeys)

THE IITRI ADVANTAGE

- A highly experienced and integrated study team from a broad range of disciplines including virology, bacteriology, immunology, molecular biology and toxicology
- Direct partnership with PhD-level study directors for seamless communication
- Proprietary bioaerosol program with BSL-2/3+ facilities and vivarium
- Flexible, collaborative approach to study design, optimizing cost and quality
- All studies conducted at one site, with all functions located under one roof



CORE SERVICES



DISCOVERY AND DEVELOPMENT

BSL-2/3+

Bacterial and viral strain library:

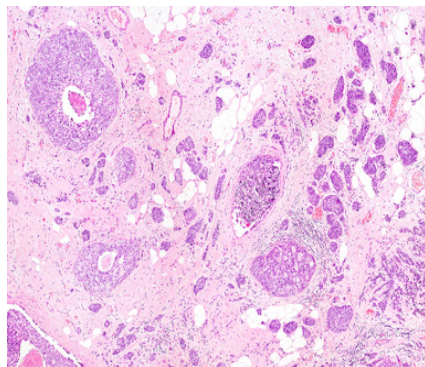
- Influenza, flavivirus (Zika, West Nile, dengue), VEE/ WEE/EEE, B. anthracis (anthrax), Y. pestis (plague) and Methicillin-resistant Staphylococcus aureus (MRSA) I

In vitro efficacy studies

- Viral, bacterial

In vivo efficacy studies

- Rodent, cotton rat, rabbit, ferret, minipig



GLP SAFETY & TOXICOLOGY

Biodistribution (non-GLP)

- ELISA
- qPCR, RT-qPCR

Repeat-dose GLP toxicology studies

- BSL-2
- All relevant routes of administration including inhalation
- Rodent, rabbit, minipig, canine, NHP • Histopathology, clinical chemistry
- Immunogenicity, immunotoxicology

Safety pharmacology



SUPPORTING ASSAYS (GLP AVAILABLE)

BSL 2/3+

Antibiotic potency

- MIC

Immunogenicity •

- ELISA • Plaque-reduction neutralization (PRN)
- Microneutralization (MN)
- Hemagglutination assay (HI)
- Yield-reduction assay

Modulation of cytokine expression

- Assessment of biomarkers
- Immunophenotyping (FACS)



MANUFACTURING SUPPORT

BSL 2/3+

Batch release testing (GMP)

Potency testing

- *In vitro* efficacy studies
- *In vivo* efficacy studie

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