



## GENE THERAPY

### GENE THERAPY OVERVIEW

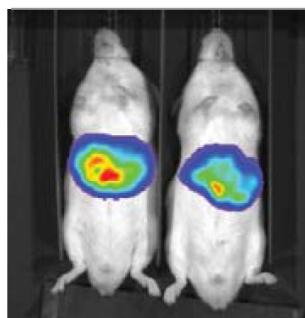
- Housing and breeding programs for transgenic lines
- Experience with administration of various client's test articles for multiple gene therapy methods e.g. CRISPR, AAV, Oligo, mRNA, RNAi, siRNA, ceDNA, antisense
- Proficient in multiple routes of administration of test materials for gene therapy
- Multiple tissue collections according to client requests
- Established and developed in-vitro assays to assess the efficiency of the gene therapy treatments e.g. ammonia, hEPO, FIX, FVIII, PHE, Luciferase
- Evaluate test compound toxicity by clinical observations and laboratory test e.g. liver enzymes

### ROUTES OF ADMINISTRATION

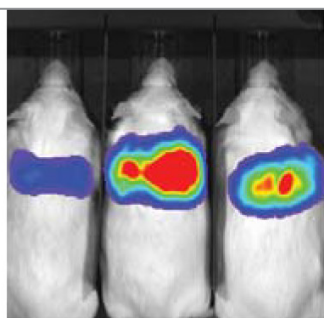
- Intravenous
- Hydrodynamic intravenous
- Subcutaneous
- Oral gavage
- Intramuscular
- Nebulization
- Intravitreal
- Intrathecal
- Intratracheal
- Intraarticular
- Intranasal
- Intracerebral ventricular

### SUMMARY

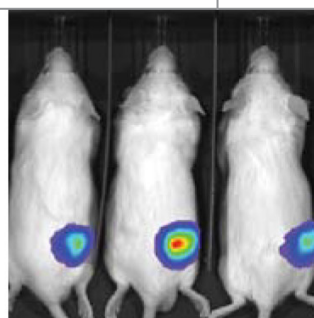
- Experience with multiple transgenic strains
- Proficient with multiple dose administration methods
- Established various in-vitro assays
- IVIS imaging capabilities



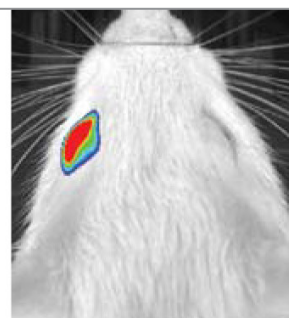
Luciferase via  
intravenous injection,  
targeting the liver



Luciferase via  
intratracheal  
administration,  
targeting the lungs



Luciferase via  
intramuscular  
injection



Luciferase via  
intravitreal  
injection