

# Gene Therapy



Lovelace Biomedical has one of the **longest-running gene therapy programs of any contract research organization**, with more than a decade of experience conducting preclinical studies to evaluate safety, efficacy and biodistribution of gene therapy products.

Lovelace consults on experimental design and content of pivotal, pre-IND packages and provides final reports for submission to U.S. and European regulatory agencies. Preclinical studies in gene therapy are run in compliance with GLP standards in species ranging from rodents to nonhuman primates.

## What We Do

- Develop novel study designs to concurrently measure safety and efficacy of gene therapies
- Monitor immune responses to vector or expressed protein
- Measure biodistribution of vector and transgene expression
- Evaluate traditional and non-traditional dosing routes

## Endpoints That Matter

- Efficacy
- General toxicity
- Vector biodistribution (qPCR)
- Gene expression
- Immunogenicity



10+ years

running complex IND-enabling programs to support gene therapy development



50+

Lovelace Biomedical scientific staff experienced with pre-IND gene therapy programs



300,000

Square feet lab space, including GLP-compliant operations



\$204 million

estimated sales of gene therapy products in the U.S. by 2020

# Gene Therapy



## 10+ Years



**IND Enabling Gene Therapy Program  
Development Experience**

**Lovelace Biomedical** is the leading CRO for performing Gene Therapy nonclinical development. We have been working with the FDA and scientists with leading pioneers in the industry to advance gene therapy products to the clinic for over 12 years. In 2007 our Gene Therapy pharmacology/toxicology Center was established by the NIH along with pioneers at the University of Pennsylvania. We have maintained this core for over 12 years, and have built on its foundation a successful resource for biotechnology and pharmaceutical companies aiming to advance their candidates from discovery to clinical stage by GLP enabling studies as rapidly as possible.

## Gene Therapy Expertise

### End Points

- PCR Development & Validation
- Biodistribution & Gene Expression
- Clinical Pathology/Histopathology

### Regulatory Support

- Pre IND
- Pre IND Package Development

### Specialized Dose Delivery

- Inhalation
- Intra-Articular
- Cerebral
- Epicardial
- Ocular
- Intravenous Infusion
- Spinal

### Disease Models

- Animal Model Propagation
- Animal Model Development

### Animal Models

- NHP
- Canine
- Swine
- Rabbit
- Ferret
- Guinea Pig
- Rat
- Mouse

## Notable Publications

- McDonald, C.L., Benson, J., Cornetta, K., Diggins, M., Johnston, J.C., Sepelak, S., Wang, G., Wright, J.F., Skarlatos, S.I. Advancing Translational Research Through the NHLBI Gene Therapy Resource Program (GTRP). *Human Gene Ther Clin Dev* 24(1): 5 – 10, 2013
- Chulay J.D. Ye, G.J., Thomas, D.L., Knop, D.R. Benson, J.M., Hutt, J.A., Wang, G, Humphries, M., Flotte, T.R. Preclinical Evaluation of a Recombinant Adeno-associated virus Vector Expressing Human alpha-1 antitrypsin Made Using a recombinant-tint Herpes Simplex Virus Production Method. *Hum Gene Ther.* 22(2) 155-165, 2011.
- Chiuchiolo, M.J., Kaminsky, S.M., Sondhi, D., Hackett, N.R., Rosenberg, J.B. Frenk, e.Z. Hwant, Y. Vand de Graff, B.G., Hutt, J.A., Wang, G., Benson, J. and Crystal R.G. Intrapleural Administration of an AAVrh.10 vector Coding for Human a1-antitrypsin for the Treatment of a1-antitrypsi Deficiency. *Human Gene Ther Clin Dev*, 24(4): 161-173, 2013