LOVELACE BIOMEDICAL

Gene Therapy Toxicology

Lovelace Biomedical has one of the **longest-running gene therapy toxicology 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's gene therapy toxicology program 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

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Gene Therapy Toxicology

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 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 Toxicology Expertise

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

Specialized Dose Delivery
- Inhalation
- Intra-Articular
- Cerebral
- Epicardial
- Ocular
- Intravenous Infusion
- Spinal

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

Regulatory Support
- Pre IND
- Pre IND Package Development

Disease Models
- Animal Model Propagation
- Animal Model Development

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 recombiantint 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. Intrapleaural Administration of an AAAVrh.10 vector Coding for Human a1-antitrypsin for the Treatment of a1-antitrypsi Deficiency. Human Gene Ther Clin Dev, 24(4): 161-173, 2013

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