LOVELACE BIOMEDICAL

GENE THERAPY

16+ 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 leading scientists in the industry to advance gene therapy products to the clinic. In 2007, our Gene Therapy Pharmacology/Toxicology Center was established by the NIH and the University of Pennsylvania. We have maintained this core for over 16 years and have built on its foundation as a successful resource for biotechnology and pharmaceutical companies, aiming to advance their candidates from discovery to clinical stage.

GENE THERAPY EXPERTISE

SPECIALIZED DOSE DELIVERY
- Inhalation
- Intra-Articular
- Cerebral
- Epicardial
- Ocular
- Intrvenous Infusion
- Spinal

REGULATORY SUPPORT
- Pre-IND-enabling package development

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

DISEASE MODELS
- Animal Model Development
- Colony Breeding

END POINTS
- Biodistribution
- mRNA Concentration & Gene Expression
- Clinical Pathology/ Histopathology

NOTEABLE 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., Wand, G., Benson, J. and Crystal R.G. Intrapleaural Administration of an AAA Vrh. 10 vector Coding for Human a1-antitrypsin for the Treatment of a1-antitrypsi Deficiency. Human Gene Ther Clin Dev, 24(4): 161-173, 2013

query@lovelacebiomedical.org | (505) 549-8353 | www.lovelacebiomedical.org

LOVELACE BIOMEDICAL

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 performs preclinical studies in gene therapy that are run in compliance with GLP standards in species ranging from rodents to nonhuman primates generating final reports for submission to U.S. and European regulatory agencies.

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, mRNA concentration, and transgene expression
- Evaluate traditional and non-traditional dosing routes

ENDPOINTS THAT MATTER

- Efficacy
- General toxicity
- Vector Biodistribution
- mRNA concentration
- Gene expression
- Immunogencity

400,000
square feet of laboratory space, including GLP-compliant operations

250+
Lovelace Biomedical staff experienced with pre-IND gene therapy programs

16+ years
running complex IND-enabling programs to support gene therapy development

$42 billion
estimated sales of gene therapy products in the U.S. by 2030

query@lovelacebiomedical.org | (505) 549-8353 | www.lovelacebiomedical.org