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 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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# Rare Diseases

## Recent Programs Include

cystic fibrosis • idiopathic pulmonary fibrosis • Pompe disease
α1 antitrypsin deficiency • Tay-Sachs • tuberous sclerosis complex • Kawasaki disease

Off-the-shelf solutions for preclinical research simply don't exist for many of the pharmaceutical industry's more complex drug development challenges — **including the challenge of creating treatments for rare diseases.**

There is still no cure for the majority of rare diseases, most of which are severe or life-threatening. But that is changing as today's DNA technologies enable greater insights into genetic disease pathways, leading to new therapeutic approaches.

## What We Do

- Integrate animal breeding, pharmacology and toxicology at a single site for seamless study conduct
- Investigate mechanisms of action using appropriate biomarker endpoints
- Evaluate non-traditional drug types often needed to treat rare diseases, including gene therapy, antisense oligonucleotides, RNAi, enzyme replacement therapy and more

## Need to Know

- Lovelace Biomedical is an experienced partner for advancing rare disease candidates into the clinic
- Published 12+ peer-reviewed journal articles since 2010 showcasing new rare disease research

**7,000+**
rare diseases, according to NIH

**30+ million**
Americans affected by a rare disease

**50%**
of patients are children

**$209 billion**
in worldwide orphan drug sales by 2022

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