



National Heart, Lung,
and Blood Institute



Gene Therapy Resource Program

What is the NHLBI Gene Therapy Resource Program (GTRP)?

The GTRP provides resources to US-based investigators registered with the Program to assist in the translation of gene therapy products from preclinical testing to early phase clinical trials.

Services are provided at no cost to investigators.

To be eligible, investigators must be conducting research in heart, lung, and blood diseases at a US-based institution.

What services does the Program provide?

- Preclinical Vector Production
- Immunology Testing of Preclinical Materials
- Pharmacology/Toxicology Testing
- Assistance with Regulatory Activities
- Supplemental Funds for Clinical Trials

For further information visit the Program website at www.gtrp.org or contact the GTRP Clinical Coordinating Center at gtrpccc@s-3.com.

Clinical Coordinating Center (CCC) – Social & Scientific Systems, Inc.

Capabilities include:

- Regulatory Assistance to Investigators
 - Arrange and prepare for INTERACT and pre-IND meetings with FDA
 - Assist in preparation of submission of materials to oversight bodies (i.e., FDA, DSMB, OBA/RAC)
 - Provide guidance in compilation of documents required by IBCs & IRBs
 - Assist in securing an IBC for institution without standing IBC
 - Assist clinical sites with preparation of site management materials (e.g., Regulatory Binder, MOP) for trial conduct
 - Assist with study monitoring services at clinical trial sites for investigators whose protocol receives supplemental GTRP funds
- Disbursement of Supplemental Funds for Clinical Trials

Visit <https://www.s-3.com> for additional information about Social & Scientific Systems, Inc.





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Preclinical Vector Production Core – University of Pennsylvania

Capabilities include:

- Production of research-grade vectors – Adenoviral, lentiviral, and AAV (conventional & novel serotypes)
- Design and construction of vectors
- Characterization of plasmid DNA source material
- Bio-distribution analysis
- Immunology testing services – including neutralizing antibody & ELISpot assays
- GMP process-comparable AAV vectors for approved IND-enabling pharmacology/toxicology studies

The Preclinical Vector Core offers AAV preparations in a variety of scales ranging from small scale (5E+12 GC per prep) to mega scale (1E+14 GC per prep or greater).

Visit <http://www.med.upenn.edu/gtp> for additional information about the University of Pennsylvania Vector Core.

Pharmacology/Toxicology Testing Core – Lovelace Biomedical and Environmental Research Institute

Capabilities include:

- Consult on design of pivotal preclinical safety and bio-distribution studies
- Support development of pre-IND package information
- Conduct pivotal safety and bio-distribution studies in compliance with GLP standards with various animal models, dose delivery routes, and various endpoint evaluation (see table below)
- Develop final study reports for submission to FDA

Animal Models	Dose Delivery Routes	Endpoint Evaluation
<ul style="list-style-type: none"> • NHP • Canine • Swine • Rabbit • Guinea Pig • Ferret • Rat • Mouse • Disease models (various species) 	<ul style="list-style-type: none"> • IV, IM, SQ • Inhalation • Intra-articular • Epicardial • Intravenous infusion • Spinal • Cerebral • Ocular 	<ul style="list-style-type: none"> • General Toxicity • Clinical Pathology • Histopathology • Biodistribution • Gene Expression • Immunogenicity • Other protocol-specific endpoints

Visit <http://www.gtrp.org> for additional information about the Lovelace Pharmacology/Toxicology Core.

