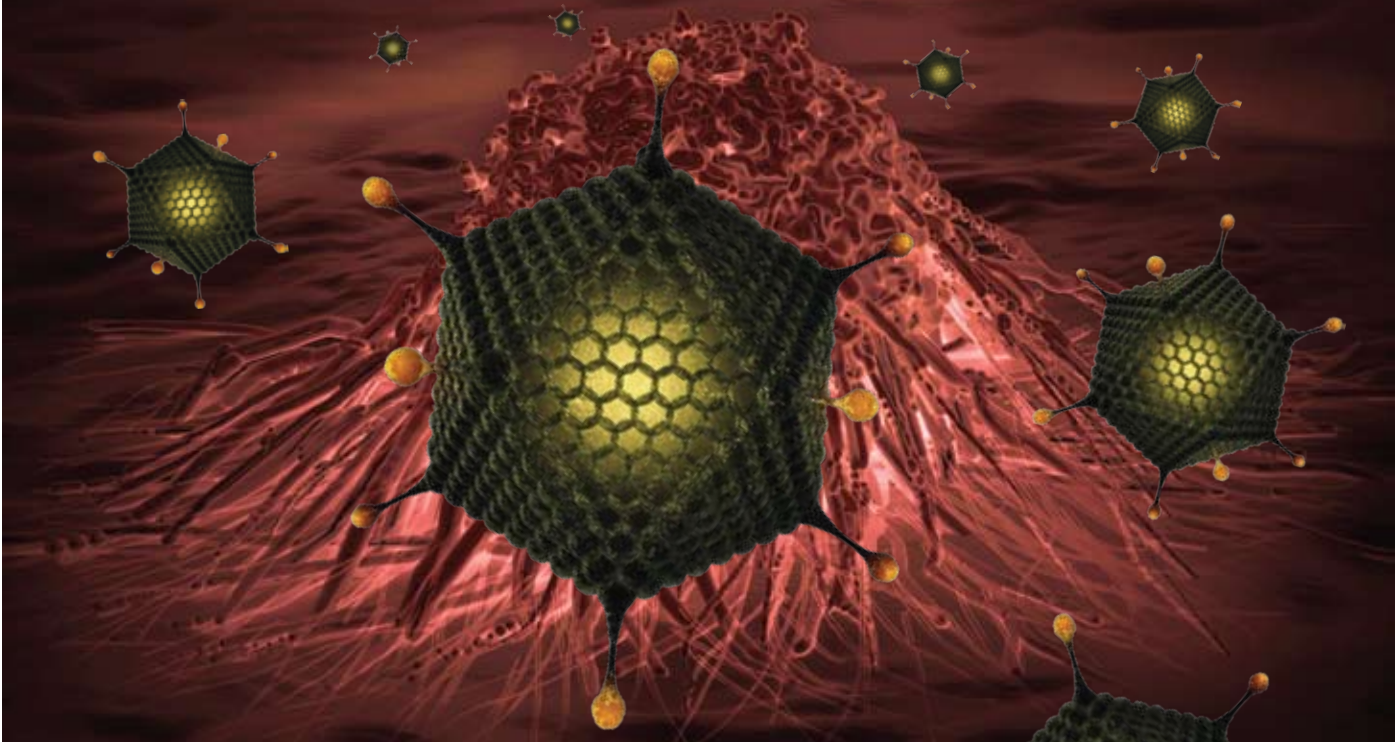
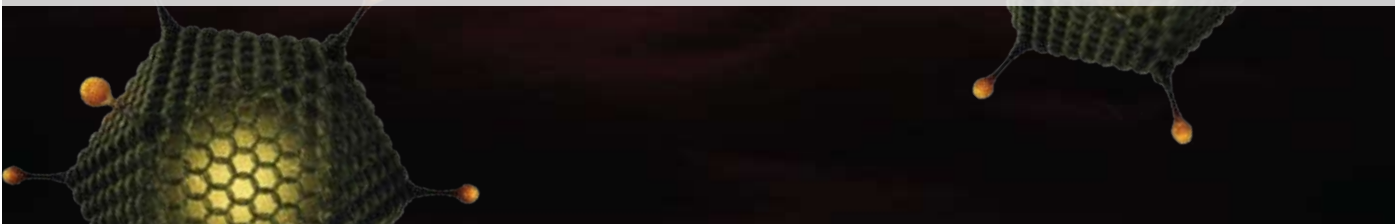


Gene Therapy Preclinical Services



**Program design, testing, and expertise
for your critical product evaluations**



*ILLUSTRATION:
Adenoviral vectors targeting a tumor*

 **药明康德**
WuXi AppTec

Gene Therapy Preclinical Services

WuXi AppTec's preclinical services for gene therapy products leverage our unique mix of scientific, technical and regulatory expertise in virology, in-life studies and bioanalytical assays. Our staff experience, established facilities and broad understanding of virus-based therapeutics makes WuXi AppTec the leader in preclinical, toxicology and biodistribution studies for gene therapy programs.



Exceptional Expertise to Support Your Specific Study Design Needs

Technical and regulatory experts – with a wide variety of skill sets – are available to assist in all facets of your program, including study and protocol design, assay development and validation and end-point analysis.

- On-staff surgeons, veterinarians, board-certified pathologists, Ph.D. toxicologists, molecular biologists, and analytical chemists
- Strong background in GLP and non-GLP/research projects
- Consulting and personalized expert guidance in uncertain regulatory environments
- Cell-based and bioanalytical methods development and validation for a wide variety of viral-based therapeutics
- Design of PCR-based methods for vector biodistribution studies

Comprehensive Toxicology & Biodistribution Capabilities

- Acute Toxicity
Range Finding (MTD) | Fixed Dose | Local Tolerance
- Repeat Exposure
Range Finding (MTD) | Subacute | Subchronic | Chronic
- pK/tK/Vector Biodistribution
Blood & tissue sampling | PCR analysis of vector from tissues
- Tumorigenicity
- Non-GLP Exploratory Studies
- Implant or Efficacy Studies

Animal Models, Treatment Modalities & Routes of Administration

Small Animal

Mouse (*disease state, normal and immunocompromised*)
Rat (*normal and immunocompromised*)
Guinea Pig
Rabbit

Large Animal

Dog
Non-human primate
Pig
Mini-pig
Sheep
Goat

Injection

Intravenous
Intraarterial
Intramuscular
Subcutaneous
Intraperitoneal
Oral

Surgical

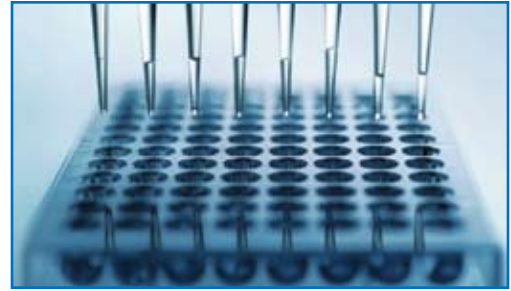
Intramuscular
Subcutaneous
Intraperitoneal
Skeletal

Endpoint Analysis Capabilities

Clinical Chemistry
Hematology
Histopathology
Gene Detection or Sequencing
Replication Competent Virus Detection
Neutralizing Antibody Assays
Cell-based and Infectious Titer Assays
High-throughput Bioanalysis
Immunoassays
Immunogenicity
Biomarkers

Technologies

ELISA | ELISPOT | qPCR | DNA Sequencing
Flow Cytometry | MSD | ICS | UPLC (Q-TOF/MS)
HPLC | Q-Trap Mass Spectrometry (LC/MS/MS)



Facilities & Infrastructure

Our St. Paul and Philadelphia facilities provide all the necessary suites and laboratories to conduct your critical preclinical studies.

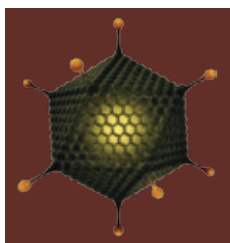
St. Paul, Minnesota

- AAALAC and ISO 17025 Accredited
- Dedicated BSL-2 suites/laboratories for both in-life and endpoint analysis
- 4 state-of-the-art surgical suites
- 54 animal study rooms
- Dedicated isotope suite
- Formulation and cell culture laboratories
- On-site necropsy and histopathology
- On-site clinical chemistry, pathology, hematology and coagulation analysis
- Radiography / Fluoroscopy imaging capabilities

Philadelphia, Pennsylvania

- ISO 17025 Accredited
- Bioanalytical
- Analytical
- Immunology
- Molecular Biology (PCR, Sequencing)
- Cell Biology
- Virology

In addition, WuXi AppTec's Suzhou, China facility is one of Asia's largest and most comprehensive preclinical facilities for large-animal work, should dog or non-human primate studies be required.



Gene Therapy Preclinical Services

WuXi AppTec is the leader in preclinical services for gene therapies.

By focusing on quality, scientific expertise, flexibility and responsiveness, WuXi AppTec partners with our Sponsors to provide a wide range of IND-enabling preclinical, toxicology and laboratory services that meet global regulatory standards.

PROGRAM FEATURES

- Strong scientific, technical and regulatory knowledge for a wide variety of virus-based therapeutics.
- Study design and assay development and validation expertise.
- Broad expertise with a variety of animal models and treatment modalities (ROA).
- In-house clinical chemistry, pathology, hematology, bioanalytical, molecular biology (qPCR), virology and cell biology laboratories.
- On-staff pathologists, surgeons, veterinarians and Ph.D. toxicologists, molecular biologists and analytical chemists.
- Virology expertise for critical safety assays such as replication competent virus detection and product titer assays.
- Comprehensive program support including GMP cell banking and characterization, lot release and stability studies for bulk and final product.
- Global support through facilities in both the U.S. and China.

ADDITIONAL SERVICES

WuXi AppTec has a twenty-year history in providing biosafety and analytical testing for a wide variety of viral and plasmid-based gene therapy products.

Services include:

- GMP Cell Banking (eukaryotic/mammalian) and Cell Line Characterization
- Genetic Stability and Characterization
- Assay Development and Validation
- Lot Release
- Stability Programs

Contact WuXi AppTec for gene therapy preclinical services:

(1) 651-675-2000 / 888-794-0077 • info@wuxiapptec.com • www.wuxiapptec.com