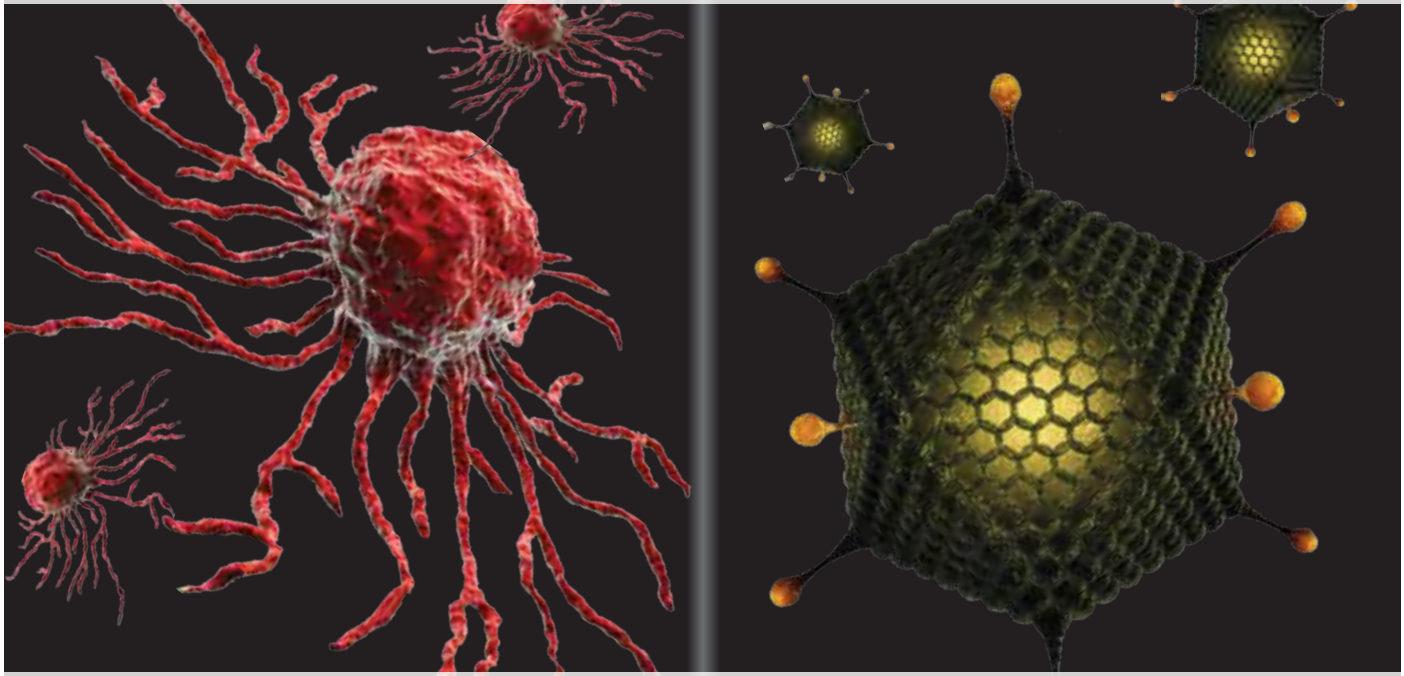
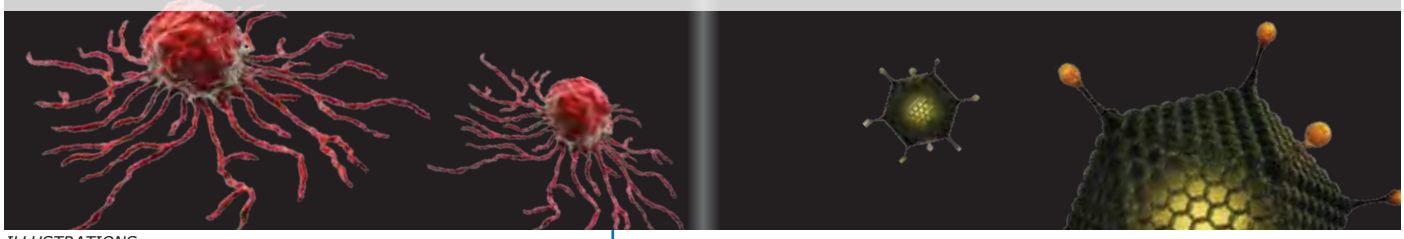
Cell and Gene Therapy Preclinical Services



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



ILLUSTRATIONS:
Stem cells and adenoviral vectors

药明康德
WuXi AppTec

Gene and Cell Therapy Preclinical Services

WuXi AppTec's preclinical services for cell and 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 advanced therapeutic development make WuXi AppTec the leader in preclinical, toxicology, and biodistribution studies worldwide.

Exceptional Expertise to Support Your Specific Study Design Needs

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

- On-staff surgeons, veterinarians, board-certified pathologists, Ph.D. toxicologists, immunologists, molecular biologists, and analytical chemists.
- Extensive experience in IND-enabling studies.
- Expert guidance in discovery studies for lead identification and optimizing pivotal GLP programs.
- Personalized consulting in a rapidly evolving regulatory landscape.
- Cell-based and bioanalytical methods development and validation for a wide variety of cell and viral vectored therapeutics.
- Design of PCR-based methods for cell therapy and viral vector biodistribution studies.

Comprehensive Preclinical Capabilities [GLP and Discovery Research]

- Efficacy and tK/pK studies
- Toxicology:
  Dose Finding (MTD) | Subacute | Subchronic | Chronic
- Biodistribution:
  Viral vectors | Human cells
- Tumorigenicity:
  Model development | ESC | iPSC | Adult SC | Cell lines

Discovery Research Capabilities

Proof-of-concept studies
Lead identification
Efficacy and dose analysis
Optimization of transgene expression
Comparing routes of administration
Preliminary toxicology
Biodistribution feasibility studies

Animal Models and ROA

Small-Animal Models

Mouse *(disease state, normal & immunodeficient)*
Rat *(normal & immunodeficient)*
Guinea Pig
Rabbit

Large-Animal Models

Dog | Pig | Mini-pig | Goat
Sheep | Non-human primate

Routes of Administration

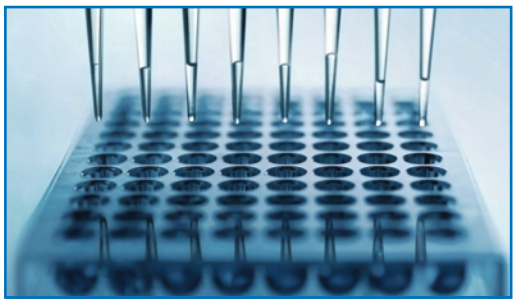
Intraveneous | Intra-arterial | Intradermal
Intramuscular | Subcutaneous | Oral
Intraperitoneal | Intraportal

Endpoint Analysis Capabilities

Clinical chemistry, coagulation and hematology
Histopathology and immunohistochemistry
Deep sequencing
Replication-competent virus detection
Neutralizing antibody assays
Total and infectious titer assays
High-throughput bioanalysis
Immunogenicity
Biomarker analysis

Technology Platforms

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



Facilities and Infrastructure

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

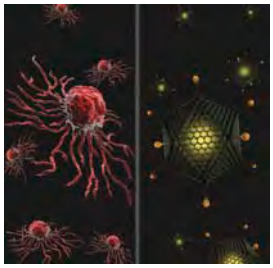
St. Paul, Minnesota

- ISO 17025 and AALAC accredited; USFDA HCT/Ps registered
- 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 pathology, hematology, clinical chemistry, and coagulation analysis
- Radiography / Fluoroscopy imaging capabilities

Philadelphia, Pennsylvania

- ISO 17025 Accredited; USFDA HCT/Ps registered
- GMP manufacturing of cellular therapeutics
- Dedicated laboratories
  Bioanalytical | Analytical | Immunology
  Molecular Biology | 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.



Cell and Gene Therapy Preclinical Services

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

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

PROGRAM FEATURES

- Strong scientific, technical and regulatory knowledge for a wide variety of cell and viral-based therapeutics
- Comprehensive R&D and GLP program support – from discovery to IND-enabling studies
- Study design, assay development, and validation expertise
- Broad expertise with a variety of animal models and routes of administration
- In-house clinical chemistry, pathology, hematology, bioanalytical, molecular biology (qPCR), virology, and cell biology laboratories
- On-staff pathologists, surgeons, veterinarians, DABT-certified toxicologists, molecular biologists, immunologists, and analytical chemists
- Virology expertise for critical safety studies such as replication-competent virus detection and product titer assays
- Global support through facilities in both the U.S. and China

ADDITIONAL SERVICES

WuXi AppTec has a 25-year history in providing biosafety and analytical testing for a wide variety of advanced cell-and gene-therapy-based products.

Services include:

- Comprehensive program support for GMP cell banking (eukaryotic/mammalian) and cell line characterization.
- GMP manufacture of cellular therapeutics.
- Potency assays for cell- and gene-therapy-based products.
- Genetic stability and characterization
- Assay development and validation
- Lot release
- Stability programs

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Contact WuXi AppTec for cell and gene therapy preclinical services:

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