

Gene Therapy Testing Services

**Utilizing over 20 years of experience
to guide you safely into clinical trials**

*ILLUSTRATION:
Model of DNA double helix*

 **WuXi AppTec**

**THE
VIROLOGY
EXPERTS**



Gene Therapy Testing Services

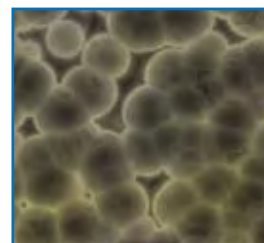
A full spectrum of services to speed therapeutics from discovery to commercialization

WuXi AppTec offers GLP/GMP testing programs for somatic cell, viral, and plasmid DNA-based gene therapies. With many years of experience in providing fully integrated biosafety testing programs for gene therapy programs, we have supported a vast number of successful regulatory submissions.

GMP CELL BANKING AND CRYOPRESERVATION



Cell banks are manufactured to client specifications – under exacting standards and QA oversight – in one of our seven closely controlled, monitored and secure GMP cell banking suites. GMP cell banks can be stored long term at WuXi AppTec in highly controlled and regulated GMP facilities. Various levels of segregation can be reserved for cell banks within the vapor phase of liquid nitrogen.



CELL LINE CHARACTERIZATION

Whether mammalian cells (for virus-based therapies) or microbial cells (for plasmid-based therapies), we provide complete biosafety, identification and characterization assessment of cell banks. All testing is conducted under FDA and ICH guidelines. The comprehensive battery of test methodologies – including genetic stability analysis required for most species – is conducted by our in-house laboratories.

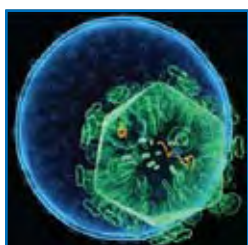
VIRAL BANK SAFETY TESTING & CHARACTERIZATION

WuXi AppTec offers all of the assays mandated by regulatory authorities worldwide for the biosafety analysis of viral banks (stocks) used in gene therapy programs. Custom identity, purity and potency testing is also available for viral vector stocks using molecular biology or virology methodologies.



PLASMID STOCK CHARACTERIZATION

Molecular biology and analytical tests can be performed to assess identity, concentration and purity of plasmid stocks. Additionally, WuXi AppTec offers sterility and endotoxin testing to ensure stocks are free of contaminants, as well as residual DNA and protein assays to assess residual host cell elements.



REPLICATION-COMPETENT VIRAL VECTOR TESTING

Sensitive *in vitro* cell culture or molecular biology assays are used to detect replication-competent viral vectors (e.g., adenovirus, retrovirus, lentivirus and AAV). WuXi AppTec can work with clients to provide custom replication-competent virus detection assays for a particular vector construct.

Technical & Regulatory Guidance

From a foundation of over 20 years of industry experience and in-depth knowledge of global regulatory guidances, WuXi AppTec's technical experts will provide you with the necessary information to assure that your product meets the highest expectations of regulatory authorities worldwide.



LOT RELEASE

*For Unprocessed Bulk/Viral Harvest,
Bulk Drug Substance and Final Drug Product*

WuXi AppTec can develop or perform technology transfer from the Sponsor for the various biosafety and analytical methods required for these lot release program criteria:

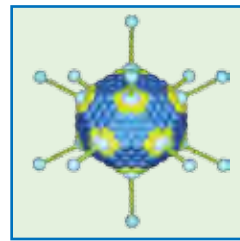
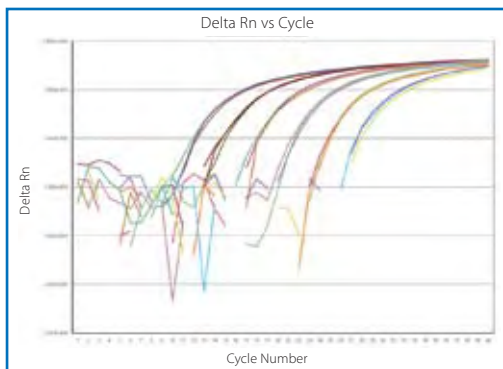
- Purity • Concentration • Potency • Identity • Consistency
- Host Cell and Process Residuals • Adventitious Virus Detection
- Mycoplasma • Sterility/Microbial Enumeration

BIODISTRIBUTION

Comprehensive in-life and molecular biology capabilities are offered to support biodistribution programs. Experience with a wide variety of animal models and routes of administration have been implemented for viral, plasmid- and cell-based programs.



In addition, using real-time qPCR technology, our program provides the appropriate tools for quantifying the copy number of a DNA or RNA target in a defined set of organs and tissues. If toxicology studies are required, WuXi AppTec has experience with performing these as well and coordinating them with the biodistribution studies for time- and cost-saving advantages.



PRODUCT STABILITY

A wide variety of analytical or biosafety methodologies are available for use in product stability studies. We maintain ICH validated chambers at a variety of temperature and humidity settings. Stability programs are conducted under GMP guidelines.

Additional Services

• RAW MATERIALS TESTING AND SUPPLY CHAIN ASSESSMENT

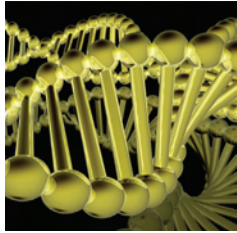
Biosafety testing of raw materials includes mycoplasma testing and 9CFR assays for detection of adventitious virus from animal-derived raw materials. Supply chain assessments include written risk evaluations in support of regulatory filings or for review by Sponsor's product development partners.

• ASSAY DEVELOPMENT AND VALIDATION

Assays can be developed or we can perform technology transfer of assays from Sponsor. Validations/qualifications can be conducted to perform assays under GMP conditions.

• VECTOR PURIFICATION PROCESS VALIDATION FOR VIRAL CLEARANCE

To validate the removal of potential contaminants, WuXi AppTec can provide custom design and performance of viral clearance studies for various vector purification processes.



Gene Therapy Testing Services

Ever since the first gene therapy programs were initiated, WuXi AppTec has been providing the technical testing competencies and regulatory expertise to help navigate novel biologics from early-stage development through to late-stage clinical trials. We will work with you to develop comprehensive biosafety testing programs that meet the highest expectations of regulatory authorities worldwide.

EXPERIENCE

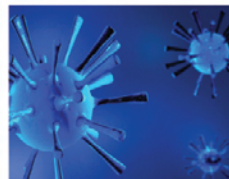
- Expertise to provide biosafety programs for all types of viral gene therapy vectors (adenovirus, lentivirus, AAV, herpes, etc).
- Hundreds of cell banks and viral banks characterized.
- Thousands of clinical lots tested under GLP and GMP.
- Over 50 cell lines and 60 viruses available as indicator cell lines or positive controls for potency, identity or biosafety assays.
- Over 80 PCR assays developed for detection of specific adventitious or contaminating viruses.

PROGRAM FEATURES

- Extensive knowledge and multiple assay methodologies for the testing of replication-competent vectors.
- Development of custom vector potency and identity assays for lot release or clinical trials.
- Both in-house animal facilities and molecular biology laboratories available for biodistribution and toxicology studies.
- Established protocols for GMP-suite cleaning validations and environmental monitoring.
- Ph.D.-level virologists with industry expertise to develop custom assays from novel vectors.
- Access to industry-leading gene therapy consultants.



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Contact WuXi AppTec for your gene therapy testing:

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