# Cell and Gene Therapy Solutions

## *Analytical development, characterization and release testing for advanced therapy medicinal products (ATMPs)*

Cell and gene therapies are part of the fast-growing field of advanced therapy medicinal products (ATMPs). These innovative therapies offer huge potential for preventing and treating many diseases based on genes, tissues or cells. ATMPs come under the regulatory framework of biological products in both the EU and the US, although sub-classification varies between regions.

### HUMAN GENE THERAPY

Gene therapy is a technique in which a functioning gene is inserted into a human cell to correct a genetic error or to introduce a new function to the cell. Genetic material is administered to modify or manipulate the expression of a gene product, either RNA or protein, or to alter the biological properties of living cells for therapeutic use.

Human gene therapy offers new hope for the treatment of rare diseases, genetic disorders, cancers and infectious diseases. Advances in genetic engineering and recombinant viral vector development have resulted in several gene therapy products gaining approval.

| TYPE OF GENE THERAPY PRODUCT | THERAPEUTIC PRINCIPLE |
|---|---|
| Plasmid DNA | Circular DNA molecules can be genetically engineered to carry therapeutic genes into human cells |
| Viral vectors | Viruses have a natural ability to deliver genetic material into cells, and therefore some gene therapy products are derived from viruses. Once viruses have been modified and their ability to cause infectious disease removed, these modified viruses can be used as vectors (vehicles) to carry therapeutic genes into human cells |
| Genetically engineered microorganisms (e.g. bacterial vectors) | Bacteria can be modified to prevent them from causing infectious disease and then used as vectors (vehicles) to carry therapeutic genes into human tissues |
| Human gene editing technology | The goals of gene editing are to disrupt harmful genes or to repair mutated genes |
| Patient-derived cellular gene therapy products | Cells are removed from the patient, genetically modified (often using a viral vector) and then returned to the patient |

## CELL THERAPY

Cellular therapies contain cells or tissues which have either been manipulated to change their biological characteristics or which were not intended to be used for the same essential functions in the body. Tissue-engineered medicines contain cells or tissues that have been modified so they can be used to repair, regenerate or replace human tissue.

Cell therapies are an emerging therapeutic option that can be used to cure, diagnose or prevent disease. Recent advances in cell characterization, isolation and *ex vivo* manipulation, combined with a greater understanding of stem-cell biology, are enabling biotechnology companies to develop cell-based products with the potential to cure a variety of diseases and injuries.

## OUR CELL AND GENE THERAPY SOLUTIONS

Advanced therapeutic modalities like cell and gene therapies require particular methods and experience so as to ensure their safety, identity, quality, purity, and strength as well as potency. Solvias can support your advanced therapy development from early stage development (e.g. IND application) to commercial testing according to the latest FDA, EMA and ICH guidelines.

We offer a comprehensive range of orthogonal analytical methods for cell and gene therapies.

## CELL & GENE THERAPY BUILDING BLOCKS

### Raw Material Testing

- Contamination (Rapid Sterility Testing, Endotoxins, Mycoplasma, Bioburden)
- Purity (Process Related Impurities)
- Characterization (Cell Culture Media, Culture Media Components, Virus Detection & Identity)

### Cell Culture Media Specific Analysis

- Contamination & Chemical Characterization (Osmolarity & Density…)
- Supplement Analysis (e.g. Interleukins & Cytokines, Heparin, Insulin, Transferrin, Hydrocortisone, Riboflavin, …)
- Quantitative Amino Acid Analysis
- Vitamins & Preservatives (Antibiotics,…)
- PRI: Trace & Metal Analysis (33 Elements)

### Oligonucleotide Analytics

- Physicochemical Characterization
- Structural and Physicochemical Integrity, Biological Potency of Oligonucleotide Products
- Stability Programs for Oligonucleotides Incorporating ICH Storage & Testing to cGMP
- Routine & Customized QC Release Testing

## PRODUCT ANALYTICS

### Gene Therapy

- DNA Vector Creation & Sequencing
- AAV & LV Process-Related Impurities: Residual DNA Determination
- Vector Physical Titer Determination
- Vector Aggregation Formation & Capsid Content (AUC, SEC-MALS, FFF-MALS, DLS, TEM)
- Transfection Potency
  - Expression Control
  - mRNA, RNAi Analysis
  - mRNA Quantification RT-qPCR
  - Protein Characterization
  - Phenotypic Characterization

### Cell Therapy

- Vector Characterization: Impurity, Identification, Safety, Stability, Sterility
- Vector Transduction Efficiency Determination (Sequencing & qPCR)
- Process Related Impurities (e.g. Vector Related Contaminants by qPCR)
- Cell Counting & Viability
- Cell Identification (Flow Cytometry)
- Recombinant Protein Expression Determination (HPLC, MS, cIEF, SDS-PAGE, ELISA, SPR, Flow cytometry)
- Specific Potency Assay Development (Functional Characterization of Transgene)

## CELL & GENE THERAPY RELEASE TESTING

### Product Release Testing

- Impurity, Identify, Safety, Stability, Sterility
- Physical, Chemical, Cellular & Functional QC Testing
- Absence of Contamination QC
- Rapid Sterility Testing, Endotoxins, Mycoplasma, Bioburden
- Potency

### Container Testing

- Contamination (Rapid Sterility Testing, Endotoxins, Mycoplasma, Bioburden)
- Extractables & Leachables
- Integrity (CCIT)
- Physical Testing
- Break Loose & Glide Force

## WHY SOLVIAS?

Solvias brings more than 15 years' experience in chemistry, manufacturing and controls (CMC) and commercial testing of biopharmaceutical products to support leading pharma and biotech innovators in cell and gene therapies. Our multidisciplinary teams offer profound expertise in molecular and cellular biology, physical-chemistry, biochemistry, microbiology and virology.

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