GENETIC DIAGNOSTICS AND THERAPY 21 LTD.

CELL AND GENE THERAPY LTD.

The Evolution of Drug Development in Bone Fractures Therapy

2 + 2 = 5

Why?

Innovative Synergy for a Supra-additive Effect!

GENETIC DIAGNOSTICS AND THERAPY 21 LTD.

A breakthrough approach to gene therapy

CELL AND GENE THERAPY LTD.

Founded in 2015 in London (UK), Cell and Gene Therapy Ltd. has emerged as a leading biotech company and a principal member of an international group specializing in the development of gene therapy drugs. The core concept of the products lies in the masterful combination of traditional and innovative elements, creating a supra-additive effect and forging a unique solution. Within this concept, the following components are envisioned:

Unique DNA Vectors

Our DNA vectors, developed and patented as a platform solution, elegantly combine efficacy, safety, and flexibility by integrating universal or tissue-specific and inflammation-activated promoters with coding sequences of target genes.

Multiplicity of Targets

Therapeutic diversity in achieved through the use of a composition of next-generation DNA vectors containing genes, each of which is directed at corresponding target.

Advanced Delivery Systems

The use of modern delivery systems enables the attainment of therapeutically significant concentrations of target proteins, thereby maximizing the drug's therapeutic effect.

Technological Excellence

The use of proven technologies allows the drug to be manufactured at various standard biotechnological facilities, achieving both competitive pricing and high profitability.

Focus on Pathology

Priority is given to the careful selection of targets within pathological biological processes, focusing on underlying mechanisms rather than merely addressing disease symptoms, to achieve effective and sustainable therapeutic outcomes.

Use of Native Genes

The use of native genes ensures harmonious integration with natural biological processes, reducing the risk of adverse reactions and enhancing the drug's biocompatibility.

Precision Delivery

The use of optimal promoters ensures accurate and efficient delivery of DNA vectors to target cells, enhancing overall therapy efficacy and minimizing off-target effects.

Regulatory Compliance

The vectors' structural elements, developed as part of our platform solution, fully comply with FDA and EMA requirements, ensuring strict safety and efficacy standards.

Our development priorities focus on diseases that currently have no effective treatments available, such as **Alzheimer's disease, Parkinson's disease, multiple sclerosis, liver fibrosis,** along with numerous other diseases. Furthermore, we are dedicated to addressing **type 2 diabetes mellitus and obesity,** as well as rare and orphan diseases.

GENETIC DIAGNOSTICS AND THERAPY 21 LTD.

Unique and innovative non-viral DNA vectors

CELL AND GENE THERAPY LTD.

Since 2015, an international team of scientists, spearheaded by our company, has dedicated extensive intellectual resources and cutting-edge research efforts to this project, ultimately leading to the development of a groundbreaking universal platform solution — non-viral DNA vectors series VTvaf17 and GDDT1.8NAS — for creating advanced genetic tools in the rapidly evolving fields of biomedical and genetic technologies. These DNA vectors incorporate the unique RNA-out regulatory element from the Tn10 transposon, thus enabling antibiotic-free positive selection, and offering the following key advantages:

Maximum Safety

The **absence of antibiotic resistance genes** and **viral genome sequences** in our DNA vectors, in accordance with EMA and FDA recommendations, ensures the highest safety. This distinct combination in a non-viral DNA vectors makes our solution **one-of-a-kind globally**.

Nature-like Mechanism

The use of **non-modified native genes** ensures seamless integration with natural biological processes, minimizing the risk of adverse reactions.

Precision Expression

By integrating **cell-specific and inflammation-activated promoters**, our drugs achieve precise and effective expression of genes in target cells while minimizing undesirable side effects.

Superior Performance

By incorporating **advanced delivery systems** into our drug, we achieve therapeutically significant concentrations of target proteins.

Multiply Therapeutic Targets

Creation of a **unique composition of genes** empowers our drugs to simultaneously target multiple therapeutic pathways, achieving a synergistic effect.

Technological Excellence

Implementation of high-tech manufacturing techniques optimizes production processes, achieving **exceptional efficiency** and **significant cost reductions**.

The intellectual property associated with this project is protected by **more than 30 patents** across various countries worldwide, highlighting the **unique** and **innovative** nature of the product.

GENETIC DIAGNOSTICS AND THERAPY 21 LTD.

Genoterosil - Gene therapy for bone regeneration

CELL AND GENE THERAPY LTD.

The overall concept of the project is based on a comprehensive integration of traditional elements, which have stood the test of time, and innovative components that leverage the latest advancements in the field, all meticulously combined to create a **supra-additive effect** that exceeds the sum of their individual contributions and delivers a unique, cutting-edge solution for bone regeneration in osteogenesis imperfecta, non- and delayed-union fractures, skeletal dysplasias, pseudarthrosis and osteoporosis.. Within this concept, the following components are included:

Focus on biological processes

Priority is given to selecting therapeutic targets within biological pathways that are directly involved in orchestrating bone tissue regeneration and the successful fracture healing.

Multiplicity of Targets

Therapeutic versatility is achieved through the use of a composition of genes responsible for a range of biological processes, including the acceleration of bone morphogenesis, bone matrix formation and mineralization, lamellar bone remodeling etc.

Optimal Delivery Systems

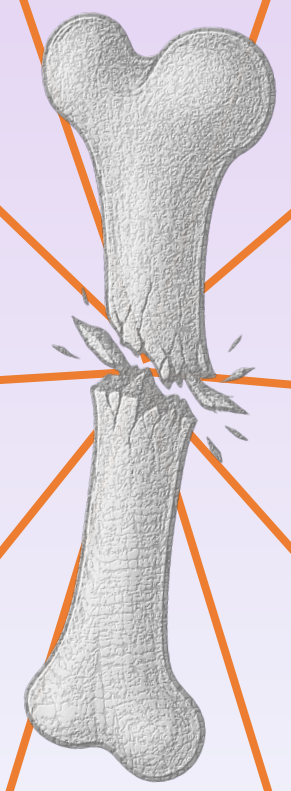
The use of calcium phosphate precipitate as the delivery vehicle ensures effective and safe delivery of genetic material into target cells while simultaneously promoting bone matrix mineralization and accelerating regeneration synergistically.

Technological Excellence

The use of proven technologies allows the drug to be manufactured in a variety of standard biotechnology facilities, achieving both competitive pricing and profitability.

Treatment Protocols

Three intramuscular injections, administered at 6-day intervals are aligned with the phases of active bone remodeling, increase expression of target proteins during patient's treatment period, effectively initiate key osteogenic processes and regeneration.



Unique DNA Vectors

Our unique therapeutic DNA vectors series VTvaf17 developed and patented as a platform solution, elegantly combine efficacy, safety, and the flexibility to vary with specifically selected promoters and coding sequences of human genes.

Use of Native Genes

The use of native human genes BMP-2,BMP-7,COL1A1, COL1A2 ensures harmonious integration with natural biological processes, significantly reducing the risk of adverse reactions and further enhancing the overall safety and biocompatibility of the drug.

Precision Expression

The use of specifically selected promoter and image - guided administration ensures precise and effective expression of delivered genes in target cells, that enhancing overall therapeutic efficacy and consequently minimizing ectopic effects.

Regulatory Compliance

The composition of structural elements of the vectors, developed and patented as part of a platform solution, fully complies with FDA and EMA requirements, guaranteeing adherence to strict safety and efficacy standards.

Enhanced Transfection

To further increase the effectiveness of gene transfer into target cells, several adjunctive physical methods were evaluated. These techniques aim to enhance the permeability of cell membranes or highly improve local conditions for gene uptake.