The Evolution of Drug Development in T2DM and Obesity 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, myocardial 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.

Pikvelis: Gene therapy for T2DM and obesity

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. Within this concept, the following components are included:

Focus on Pathology

Priority attention is given to the comprehensive normalization of biological processes related to metabolism, as well as the effective regulation and control of blood glucose levels.

Multiplicity of Targets

Therapeutic versatility is achieved through the use of 4 innovative DNA vectors containing a carefully designed and precisely optimized composition of genes responsible for a range of biological processes that are intricately associated with the disease.

Advanced Delivery Systems

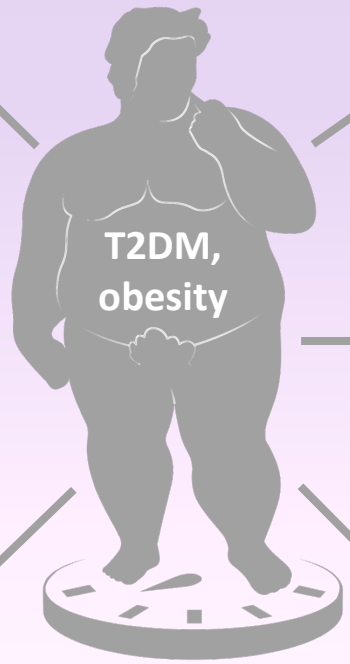
A complex of cationic liposomes + PEG with *** functionalization's been selected as the delivery system, which ensures the achievement of therapeutically significant concentrations of target proteins, thereby maximizing the therapeutic effect.

Technological Excellence

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

Treatment Protocols

The treatment regimen involves administering the drug once every 1 - 2 weeks, with regular monitoring of key parameters, including glycemic control, metabolic indicators and renal function. This treatment regimen ensures safety and minimizes adverse effects.



Unique DNA Vectors

Our therapeutic DNA vectors of GDDT1.8NAS series developed and patented as a platform solution, elegantly combine efficiency, safety, and the flexibility to vary with universal and tissue-specific promoters and coding sequences of genes.

Use of Native Genes

The use of four native human genome regions ensures a harmonious integration with natural biological processes,thereby significantly reducing the risk of adverse reactions and enhancing the drug's overall biocompatibility and therapeutic efficacy.

Precision Expression

The use of specifically selected and optimally designed promoters ensures precise and efficient delivery of DNA vectors into target cells, further enhancing overall therapeutic efficacy and significantly minimizing the risk of unintended 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.

Method of administration

The drug is administered orally in an enteric-coated capsule. Upon administration, the enteric-coated capsule dissolves in the small intestine, releasing lyophilized microspheres, which ensure maximum penetration of the delivered drug into the target cells.