Innovative gene therapy medicines

Genetic Diagnostics and Therapy 21 Ltd.

Cell & Gene Therapy Ltd.

Drug name / nosology, area of use	Progress by stages	Description
	Pharmaceuticals	
Genoterosil Osteogenesis in bone fractures	Completion of: DNA vectors development, depositing of producer strains in depositories, development of industrial production technology, conducting preclinical trials and phase I clinical trials, approval obtained for phase II-III combined clinical trials	Gene therapy drug based on non-viral DNA vectors carrying coding parts of BMP-2, BMP-7, COL1A1, COL1A2 genes for accelerated bone tissue regeneration in fractures
Epidermolysis bullosa	Completion of: DNA vectors development, depositing of producer strains in depositories, development of industrial production technology, conducting preliminary preclinical trials in vitro	Gene therapy drug based on non-viral DNA vectors carrying coding parts of KRT5, KRT14, LAMB3, COL7A1 genes for epidermolysis bullosa therapy
Cystic fibrosis	Completion of: DNA vectors development, depositing of producer strains in depositories, development of industrial production technology, conducting preliminary preclinical trials in vitro	Gene therapy drug based on non-viral DNA vectors carrying coding parts of CFTR, NOS1, AQ1, AQ3, AQ5 genes for cystic fibrosis therapy
Haemochromatosis	Completion of: DNA vectors development, depositing of producer strains in depositories, development of industrial production technology, conducting preliminary preclinical trials in vitro	Gene therapy drug based on a non-viral DNA vector carrying the coding part of the target gene HFE for the therapy of haemochromatosis

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CG-AD211 Alzheimer's disease	Completion of: Drug concept development, bioinformatic and scientific analysis, and work is underway on DNA vector development	By leveraging native human genes, our approach includes two stages: first stage directly targets the accumulation of intracellular tau aggregates—recognized as a primary cause of neurodegeneration and cognitive decline in Alzheimer's disease; next stage actively stimulates multiple regeneration processes within the brain. It supports damaged neurons in their recovery and enhances their capability to re-establish functional synaptic connections
CG-PD118 Parkinson's disease	Completion of: Drug concept development, bioinformatic and scientific analysis, and work is underway on DNA vector development	By leveraging native human genes, our approach includes two stages: first stage directly targets the accumulation of intracellular α-synuclein aggregates—recognized as a primary cause of neurodegeneration and cognitive decline in Parkinson's disease; next stage actively stimulates multiple regeneration processes within the brain. It supports damaged neurons in their recovery and enhances their capability to re-establish functional synaptic connections
CG-MS750 Multiple sclerosis	Completion of: Drug concept development, bioinformatic and scientific analysis, and work is underway on DNA vector development	By leveraging native human genes, our approach includes two stages: first stage directly targets the glial scars formation—recognized as a primary cause of neurodegeneration and cognitive decline in multiple sclerosis; next stage actively stimulates multiple regeneration processes within the brain, especially the process of remyelination
Other neurodegenerative diseases	Completion of: DNA vectors development, depositing of producer strains in depositories, development of industrial production technology, conducting preliminary preclinical trials in vitro	Gene therapy drug based on non-viral DNA vectors carrying coding parts of DDC, IL10, IL13, IFNB1, TNFRSF4, TNFSF10, BCL2, HGF, IL2, BDNF, VEGF, HIF1A, IGF1, GDNF, TGFB3 and other genes for therapy of a group of neurodegenerative diseases, the causes of which are associated with genetic factors, inherited forms of amyotrophic lateral sclerosis, trauma to the central nervous system, etc.

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CG-LF481 Liver fibrosis	Completion of: Drug concept development, bioinformatic and scientific analysis, and work is underway on DNA vector development	Gene therapy drug based on non-viral DNA vectors carrying coding parts of genes for therapy of liver fibrosis
CG-FM319 Post-infarction myocardial fibrosis	Completion of: Drug concept development, bioinformatic and scientific analysis, and work is underway on DNA vector development	Gene therapy drug based on non-viral DNA vectors carrying coding parts of genes for myocardial regeneration after infarction.
Pickvelis Diabetes mellitus / obesity	Completion of: Concept development, bioinformatic and scientific analysis	Gene therapy drug in the form of lyophilised complex of LNP+non-viral DNA vector in an enteric soluble shell to counteract type 2 Diabetes mellitus and obesity by regulating insulin secretion and lowering blood glucose levels
Neovascularisation	Completion of: DNA vectors development, depositing of producer strains in depositories, development of industrial production technology, conducting preliminary preclinical trials in vitro	Gene therapy drug based on non-viral DNA vectors carrying coding parts of ANG, ANGPT1, VEGFA, FGF1, HIF1α, HGF, SDF1, KLK4, PDGFC, PROK1, PROK2 and other genes. for therapy of a variety of diseases characterized by impaired vascularisation and tissue trophism, angiogenesis and haematopoiesis, hypoxia, impaired regeneration of various tissues, for treatment of ischemic lesions of myocardium, brain and spinal cord, limb muscle tissues, including diabetes, for treatment of oncological and neurodegenerative diseases, including amyotrophic lateral sclerosis
Neoinnervation	Completion of: DNA vectors development, depositing of producer strains in depositories, development of industrial production technology, conducting preliminary preclinical trials in vitro	Gene therapy drug based on non-viral DNA vectors carrying coding parts of BDNF, VEGFA, BFGF, NGF, GDNF, NT3, CNTF, IGF1 and other genes for therapy of diseases characterized by impaired functions of the central and peripheral nervous system, for enhancing the potential of cell therapy and allogeneic transplants, for enhancing neurogenesis, as well as after acute ischemia, trauma, in neurodegenerative diseases, diabetic neuropathy, for enhancing cognitive functions

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Spondylodesis	Completion of: DNA vectors development, depositing of producer strains in depositories, development of industrial production technology, conducting preliminary preclinical trials in vitro	Gene therapy drug based on non-viral DNA vectors carrying coding parts of BMP-2, BMP-7, LMP-1, NELL-1, etc. genes to increase efficiency and in rehabilitation after spondylodesis
Female idiopathic infertility	Completion of: DNA vectors development, depositing of producer strains in depositories, development of industrial production technology, conducting preliminary preclinical trials in vitro	Gene therapy drug based on non-viral DNA vectors carrying coding parts of IL11, LIF, DICER, HOXA10, WT1 and other genes for therapy of female idiopathic infertility associated with aspects of blastocyst attachment failure
Erectile dysfunction	Completion of: DNA vectors development, depositing of producer strains in depositories, development of industrial production technology, conducting preliminary preclinical trials in vitro	Gene therapy drug based on non-viral DNA vectors carrying coding parts of NOS2, NOS3, VIP, KCNMA1, CGRP and other genes for therapy of erectile dysfunction that is not caused by organic disorders or diseases
Age-related skin changes	Completion of: DNA vectors development, depositing of producer strains in depositories, development of industrial production technology, conducting preliminary preclinical trials in vitro	Gene therapy drug based on non-viral DNA vectors carrying coding parts of COL1A1, COL1A2, P4HA1, P4HA2, COL7A1, CLCA2, ELN, PLOD1 and other genes for therapy of a set of pathologies characterized by impaired formation of the extracellular matrix of the skin and other organs, for prevention of skin aging caused by external and internal factors, for therapy of hereditary connective tissue diseases, including Ehlers-Danlo Syndrome.
Immunomodulation	Completion of: DNA vectors development, depositing of producer strains, development of industrial production technology, conducting preliminary preclinical trials in vitro	Gene therapy drug based on non-viral DNA vectors carrying coding parts of IFNB1, IFNA14, IFNA2, IL12A, IL12B and other genes for therapy of a set of pathologies associated with disorder of innate and adaptive immunity, for therapy of autoimmune, oncological, viral diseases associated with imbalance of the immune system

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Parodontosis, periodontitis	Completion of: DNA vectors development, depositing of producer strains in depositories, development of industrial production technology, conducting preliminary preclinical trials in vitro	Gene therapy drug based on non-viral DNA vectors carrying coding parts of BMP-2, BMP-7, OPG, PDGFA, PDGFB and other genes for therapy of periodontal disease and periodontitis
Oxidative stress	Completion of: DNA vectors development, depositing of producer strains in depositories, development of industrial production technology, conducting preliminary preclinical trials in vitro	Gene therapy drug based on non-viral DNA vectors carrying coding parts of SOD1, SOD2, SOD3, CAT and other genes. The drug is intended to target a set of disorders of the energetic balance of the organism, deficiency of enzymes of the family of superoxide dismutases and catalases, to prevent the development of atherosclerosis, to protect the organism from oxidative stress and toxic effects of reactive oxygen species, to prevent the development of a spectrum of diseases including, but not limited to, abnormalities in which oxidative stress plays a pathogenetic role in the development of hereditary cardiovascular, oncological, degenerative and autoimmune diseases. The drug is also expected to be used as a preventive agent
Scar tissue correction	Completion of: DNA vectors development, depositing of producer strains in depositories, development of industrial production technology, conducting preliminary preclinical trials in vitro	Gene therapy drug based on non-viral DNA vectors carrying coding parts of SKI, TGFB3, TIMP2, FMOD and other genes for therapy and prevention of scar complications in wound healing
Alopecia	Completion of: DNA vectors development, depositing of producer strains in depositories, development of industrial production technology, conducting preliminary preclinical trials in vitro	Gene therapy drug based on non-viral DNA vectors carrying coding parts of SHH, CTNNB1, NOG, WNT7A and other genes for alopecia therapy