Genetic Diagnostics and Therapy 21 Ltd.

Cell & Gene Therapy Ltd.

Innovative gene therapy drugs and medical products

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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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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
Neoinervation	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, impaired neurogenesis, for stimulation of neuronal growth, including 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
Neurodegenerative diseases: Alzheimer's disease, Parkinson's disease, Huntington's disease, multiple sclerosis, trauma to the brain and central nervous system, age-related 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 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, including Huntington's disease, inherited forms of amyotrophic lateral sclerosis, Parkinson's disease, Alzheimer's disease, trauma to the central nervous system, autoimmune demyelinating processes, including multiple sclerosis

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Immunomodulation	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 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
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

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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

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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.
Invecta Diabetes mellitus / obesity	Completion of: Drug concept development, bioinformatic and scientific analysis, and work is underway on DNA vector development	Gene therapy drug based on a non-viral DNA vector carrying a modified coding portion of glucagon-like peptide-1 (GLP-1) to counteract type 2 Diabetes mellitus and obesity by regulating insulin secretion and lowering blood glucose levels
Glycobact Diabetes mellitus / obesity	Completion of: Concept development, bioinformatic and scientific analysis	Gene therapy drug in the form of lyophilised recombinant lactobacillus in an enteric soluble shell producing glucagon-like peptide-1 (GLP-1) to counteract type 2 Diabetes mellitus and obesity by regulating insulin secretion and lowering blood glucose levels
Zeptogen Diabetes mellitus / obesity	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 modified coding parts of glucagon-like peptide-1 (GLP-1) / glucose-dependent insulinotropic polypeptide (GIP) to counteract diabetes mellitus type 2 and obesity by regulating insulin secretion and lowering blood glucose levels
Graciglybact Diabetes mellitus / obesity	Completion of: Concept development, bioinformatic and scientific analysis	Gene therapy product in the form of lyophilised recombinant lactobacillus in an enteric-soluble shell producing glucagon-like peptide-1 (GLP-1) / glucose-dependent insulinotropic polypeptide (GIP)
CGLE924 Fibrosis and cirrhosis in non-alcoholic/alcoholic fatty liver disease	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 HGF, IL-10, PPARα, FGF21, PRKAA1, EGF, and other genes for therapy of fibrosis and cirrhosis of non-alcoholic/alcoholic fatty liver disease

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	Medical products	
Bone osteoplastic material	Completion of: Development of a medical product concept, DNA vectors development, depositing producer strains in depositories, conducting a complete set of preclinical trials and phase I clinical trials of DNA vectors	Bone osteoplastic material based on non-viral DNA vectors carrying coding parts of BMP-2, BMP-7, COL1A1, COL1A2 genes for accelerated osteogenesis in dentistry
Parodontosis, periodontitis	Completion of: Development of a medical product concept, DNA vectors development, depositing producer strains in depositories, conducting preliminary trials of DNA vectors in vitro	Liniment (paste, ointment, application) based on non-viral DNA vectors carrying coding parts of BMP-2, BMP-7, OPG, PDGFA, PDGFB and other genes for treatment of periodontal disease, periodontitis, improvement of bone and cartilage tissue regeneration during maxillofacial surgeries/optimisation of osteoinductive properties of implants
Parodontosis, periodontitis	Completion of: Development of a medical product concept, DNA vectors development, depositing producer strains in depositories, conducting preliminary trials of DNA vectors in vitro	Liniment (paste, ointment, application) based on non-viral DNA vectors carrying coding parts of BMP-2, BMP-7, OPG, PDGFA, PDGFB and other genes for treatment of periodontal disease, periodontitis, improvement of bone and cartilage tissue regeneration during maxillofacial surgeries/optimisation of osteoinductive properties of implants
Prevention of aging, turgor, overall skin health	Completion of: Development of a medical product concept, DNA vectors development, depositing producer strains in depositories, conducting preliminary trials of DNA vectors in vitro	Biorevitalising complex based on non-viral DNA vectors carrying coding parts of COL1A1, COL1A2, P4HA1, P4HA2, COL7A1, CLCA2, ELN, PLOD1 and other genes for normalization of the extracellular matrix of the dermis, biorevitalisation and hydration of the skin, toning, structural rejuvenation, prevention of age-related skin changes

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Oxidative stress	Completion of: Development of a medical product concept, DNA vectors development, depositing producer strains in depositories, conducting preliminary trials of DNA vectors in vitro	Biorevitalising complex based on non-viral DNA vectors carrying coding parts of SOD1, SOD2, SOD3, CAT and other genes for treatment, prevention and protection of skin from oxidative stress, slowing down ageing processes, activation of intracellular processes
Alopecia	Completion of: Development of a medical product concept, DNA vectors development, depositing producer strains in depositories, conducting preliminary trials of DNA vectors in vitro	Biorevitalising complex based on non-viral DNA vectors carrying coding parts of SHH, CTNNB1, NOG, WNT7A and other genes for hair restoration in alopecia, normalisation of hair structure, stimulation of hair follicle metabolism
Veterinary drugs		
Accelerated muscle gain	Completion of: Concept development, bioinformatic and scientific analysis	Livestock drug for accelerated gain of muscle mass of farm animals in the form of a composition of lyophilised recombinant intestinal bacteria in an intestinal soluble shell, producing proteins for muscle mass gain

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