

**Gene therapy drugs:
Nomenclature (gene therapy drugs, cell
therapy drugs, cell-based gene therapy drugs
and virus- based therapy drugs).**

Gene therapy drugs are drugs whose pharmaceutical substance is a recombinant nucleic acid or includes a recombinant nucleic acid that allows for the regulation , repair, replacement, addition or removal of a genetic sequence.

Federal Law of 12.04.2010 No. 61-FZ "On the circulation of medicines"

Gene therapy drug is a biological drug: containing an active substance containing a recombinant nucleic acid or consisting thereof, used or administered to a human being for the purpose of regulating , restoration , replacement, addition or deletion of a genetic sequence; therapeutic, the prophylactic or diagnostic effects of which are directly due to the sequence of the recombinant nucleic acid it contains, or to the product of the genetic expression of this sequences .

Gene therapy LP does not include vaccines against infectious diseases

Decision of the EEC Council dated 03.11.2016 No. 78
"On the Rules for the Registration and Expertise of Medicines for Medical Use"

Somatic cell-based medicinal product is a biological medicinal product: containing or consisting of cells or tissues , which have been substantially manipulated so that their biological characteristics, physiological functions , or structural properties relevant for clinical use have been altered; or consisting of cells or tissues that are not intended to be used to perform the same basic functions functions in the recipient and the donor.

Not considered as essential manipulations: cutting , crushing , shaping, centrifugation, treatment with antibiotic or antiseptic solutions , sterilization , irradiation, cell separation, concentration or purification, filtration, lyophilization, freezing, cryopreservation , vitrification, in particular, are not considered as essential manipulations ; used in humans for the purpose of treating, preventing or diagnosing a disease by means of pharmacological , immunological or metabolic action of the cells or tissues that comprise it

Decision of the EEC Council dated 03.11.2016 No. 78
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BMCP (biomedical cell product)

- a complex consisting of cell line (cell lines) and auxiliary substances, or from a cell line (cell lines) and excipients in in combination with state-registered medicinal products and (or) pharma . substances

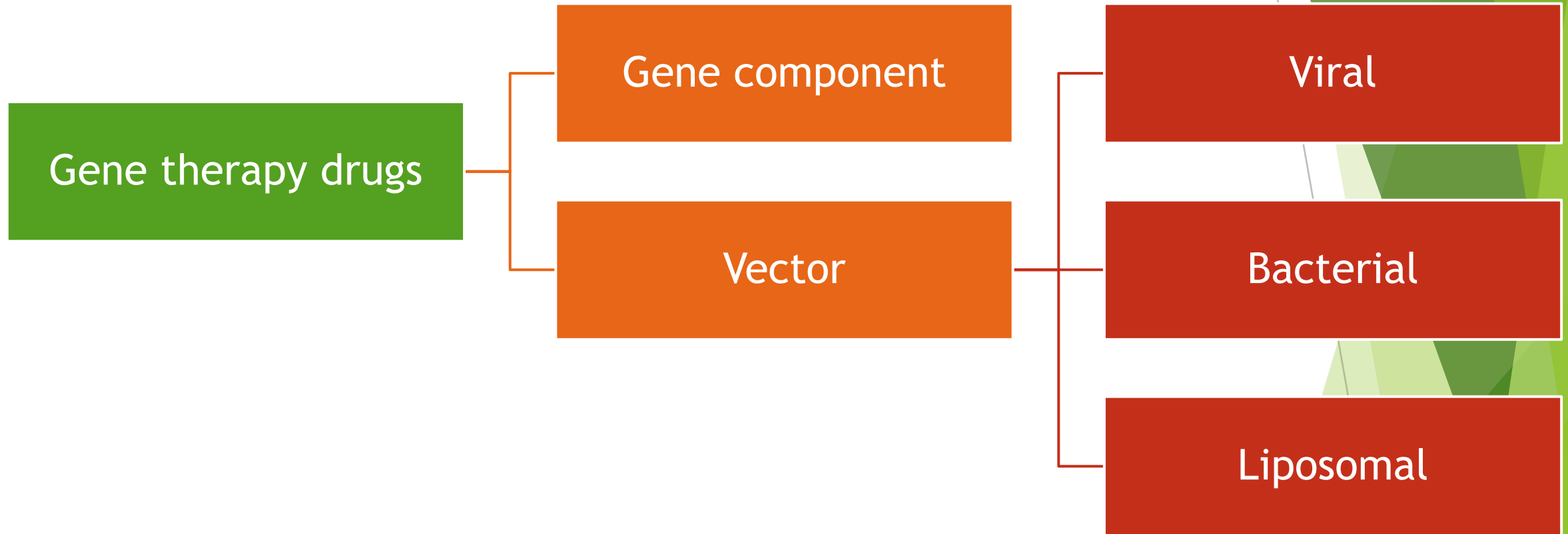
Autologous BMCP - BMCP , containing in its composition cell line (cell lines), obtained from bio . material of a certain human being , and intended for applications to the same person

Allogeneic BMCP - BMCP , containing in its composition a cell line (cell lines) obtained from biological material a certain person, and intended for use to other people

Combined BMCP - BMCP , containing in its composition cell lines derived from biological material several people, and intended for use one of them

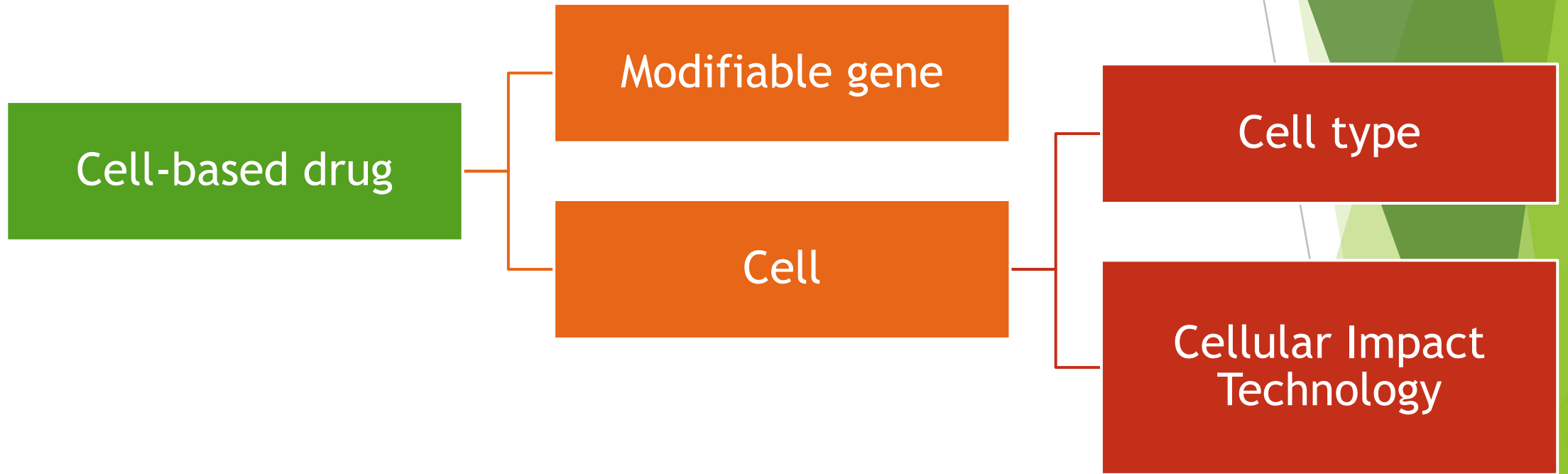
Federal Law of 23.06.2016 No. 180-FZ "On biomedical cell products

One of the elements of the system of development, state registration, production, promotion and medical use of gene therapy medicinal products is the formation of a national **nomenclature** and its harmonization with international requirements .



Scheme names substances For gene therapy on basis plasmids , viral vectors and bacteria using two words

	Prefix	Infix	Suffix
WORD 1 (genetic component)	Arbitrary For receipt euphonious and distinctive names	For designations used gene at availability are used existing infixes For biological drugs or similar infixes How For squirrel which is being coded genome , For example :	-(vowel letter) gene /-(g.b.)gene For example , - (o)
		- cima -/- cima - cytosine deaminase	
		- term -/- term - factor growth	
		- kin -/-kin- interleukin	
		- lim -/- lim - immunomodulator	
		- lip -/-lip- lipase lipoprotein human	
		- mule -/- mul - plural gene	
		- stim -/-stim- colony-stimulating factor	
		- tima -/- tima - thymidine kinase	
		- tisu -/- tisu - suppression tumors	
WORD 2 (cellular component)	Arbitrary For receipt euphonious and distinctive names	For designations type viral vectors , for example :	- century -/- vec (non-replicating viral vector) - repvec (replicating viral vector)
		- adeno -/-adeno- adenovirus	
		- cana -/- cana - virus smallpox canaries	
		- foli -/- foli - virus smallpox chicken	
		- erpa -/- erpa - virus herpes	
		-lenti-/-lenti- lentivirus	
		-morbilli-/morbilli- measles paramyxovirus	
		- parvo -/- parvo - adeno-associated virus	
		- retro -/-retro- other retrovirus	
		- vaccine -/- vaci - virus vaccines	
		For designations type bacterial vectors , for example : - lis -/- lis - Listeria monocytogenes	- tank -/-bac (bacterial vector)
			- plasmid / -plasmid (plasmid vector)



Examples of nomenclature names of medicinal products or drug For gene therapy

Trade name	INN	Developer
IMLYGIC	Talimogenelaherparepvec	Talimogen laherparepvek — is a genetically modified herpes virus (oncolytic herpes virus). Two genes were removed — one that disables individual cell defenses and one that helps the virus evade the immune system — and the human GM-CSF gene was added. The drug works by replicating in cancer cells and causing them to die; it was also designed to stimulate the patient's immune response to cancer, which has been demonstrated by numerous data, including regression of tumors that were not injected with talimogen laherparepvek .

scheme for genetically modified cell-

	Prefix	Infix		Suffix
WORD 1 (genetic component)	Arbitrary For receipt euphonious and distinctive names	To indicate the gene used, if available existing infixes for biologicals are used drugs, for example:		-(vowel letter) gen / -(g.b.)gene For example, -(o)gen/ -(o) gene
		- cima -/- cima - cytosine deaminase		
		- ermin -/- ermin - growth factor		
		- kin -/- kin - interleukin		
		- lim -/- lim - immunomodulator		
		-lip-/- lip - lipoprotein lipase human		
		-mul-/-mul- plural gene		
		-stim-/-stim- colony-stimulating factor		
		-tima-/-tima- thymidine kinase		
		- tisu -/- tisu - suppression tumors		
WORD 2 (vector component)	Arbitrary For obtaining a harmonious and distinctive name	Infix 1: Processing To indicate, if applicable, species processing which subjected to cells , with using , where This possibly existing infixes to handle, For example : - fus -/- fus-merge cells	Infix 2: Type cells To indicate the main cell type using , where This possibly existing infixes For types cells	- cel (cell)

Infixes that reflect cell type/source:

chondrocytes :	-ho(n)-/- co(n)-
Primary cord cells:	- cor -/- cor
differentiated stem cells (not included in any other category):	- deficit -/- deficit
dendritic cells:	- den -/- den
endothelial cells:	-end(o)-/- end(o)-
hepatocytes :	- ep (a)-/- ep(a)-

fibroblasts:	-fi(b)-/- fi(b)-
islet cells :	-isle-/- isle
keratinocytes :	-ker(a)-/- ker(a)-
lymphocytes/monocytes/antigen presenting cells (leukocytes):	-lei-/- leu
mesenchymal stromal cells:	-mestro-/- mestro
myoblasts:	-myo(b)-/- mio(b)-
ovarian cells:	- ova -/- ova
placental cells:	- pla (c)-/- pla (c)-
renal tubular cells:	- ren -/- ren
retinal epithelial cells:	- ret -/- ret
c stem cells:	-theme-/- tem
testicular cells:	- tesi -/- tesi
tumor cells:	-tu-/- tu
urothelial cells:	- ur -/- ur

Examples of nomenclature names of medicinal products or drug For gene therapy

Trading Name	INN	Developer
KYMRIAH	Tisagenlecleucel	Tisagenlecleucel is a CAR T-cell drug for the treatment of B-cell acute lymphoblastic leukemia that uses the body's own T cells to fight cancer (adoptive cell transfer)