

## Gene Therapy Naming Scheme

---

The gene therapy naming scheme applies only to noncellular products produced by insertion of genetic material into a vector and where altered genetic material is administered to patients as a biologic drug. Three elements are important in distinguishing a gene therapy drug and conveying safety information: Indication of the drug's mechanism or pharmacological class, the vector used in transfection and complete identification of the genes carried by the vector.

## Nomenclature Scheme for Gene Products Therapies

### Scope

Scheme would apply only to noncellular products produced by insertion of genetic material (transgene) into a vector (virus or plasmid) and where altered genetic material is administered to patients as a biologic drug.

Please note: The following are not covered by the Cell and Gene therapies scheme: minimally manipulated hematopoietic elements including minimally manipulated umbilical cord blood and peripheral blood stem cells for transplant; combination products, which include combinations of cells with non-cellular pharmaceutical products (cell/device, cell/drug combination products); prophylactic vaccines; tissue engineered products; induced pluripotent stem (iPS) cells; embryonic-derived cell therapies; and veterinary cellular therapies.

### Elements of Name

Three elements have been identified that are important in distinguishing a gene therapy drug and conveying safety information to the user physician. These are:

1. Indication of the drug's mechanism of action or pharmacologic class. [Note that administration of these gene therapy products frequently results in expression of a biologically active protein];
2. The vector used in transfection; and
3. Complete identification of the genes carried by the vector if there are 1 or 2 transgenes; for

vectors containing >3 transgenes, 1 or 2 genes will be chosen to be included in the gene qualifier based on an analysis of the impact that they have on preventing product confusion or conveying safety information.

Additional nomenclature elements would be a fantasy syllable(s) that serves as a unique identifier for the molecular entity and a stem indicating the gene therapy class of products or the ability of the vector to replicate. Based on these elements, the following is proposed:

## First Word: Corresponds to the Gene Component

**Prefix:** Fantasy element to provide unique identification; to contribute to the distinct name.

**Infix:** Element to denote the gene's mechanism of action (pharmacologic class) such as:

-ald- [adrenoleukodystrophy (ALD) protein]

-beglo- [A-t87Q-globin]

-bermin- [vascular endothelial growth factor]

-cabta- [cell expressed antibody and T cell activation]

-cima- [cytosine deaminase]

-ermin- [growth factor]

-etid- [eczema-thrombocytopenia-immunodeficiency syndrome]

-far- [interferon]

-fermin- [fibroblast growth factor]

-kin- [interleukins]

-lim- [immunomodulator]

-lip- [human lipoprotein lipase]

-mul- [multiple gene]

- naco- [coagulation factor IX]
- nad- [NADH dehydrogenase]
- nermin- [tumor necrosis factor (TNF)]
- octoco- [coagulation factor VIII]
- pap- [human papilloma virus]
- papkino- [human papilloma virus and IL-2]
- permin- [hepatocyte growth factor]
- repi- [REP-1 gene]
- reti- [retinal pigment]
- semn- [SMN]
- stim- [colony stimulating factor]
- tima- [thymidine kinase]
- tusu- [tumor suppression]

**Stem:** Element to indicate gene.

-gene

## Second Word: Corresponds to the Vector Component

**Prefix:** Fantasy element to provide unique identification; to contribute to the distinct name

**Infix:** Element to denote the type of viral vector such as:

- adeno- [adenovirus]
- cana- [canarypox virus]

-foli- [fowlpox virus]

-herpa- [herpes virus]

-lenti- [lentivirus]

-morbilli- [paramyxoviridae morbillivirus]

-parvo- [adeno-associated virus (parvoviridae dependovirus)]

-retro- [other retro viruses]

-vaci- [vaccinia virus]

**Stem:** Element to identify type of vector

-vec [non-replicating viral vector]

-repvec [replicating viral vector]

-plasmid [plasmid vector]