

Gene Therapy in Otorhinolaryngology

Essay

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Abstract

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The dramatic increases in the knowledge of the molecular and genetic basis of various diseases combined with advances in technology have resulted in novel molecular therapies for these diseases. Gene therapy, in utero or adults, which involves the transfer of genetic material to target cells via viral or non-viral vectors to produce a therapeutic effect, is a promising approach for treatment of a variety of diseases as hearing loss, paranasal sinus disease, recurrent respiratory papillomatosis, head and neck cancer, maxillofacial injuries and cleft palate.

Keywords: gene therapy, in utero gene therapy, vectors, hearing loss, paranasal sinus disease, recurrent respiratory papillomatosis, head and neck cancer, maxillofacial injuries, cleft palate.

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Adel Said El-Antably...

Lists

List of abbreviations

5-FC	5-fluorocytosine.
A	Adenine.
AAV	Adeno-associated virus.
Ad	Adenovirus.
APC	Antigen presenting cells.
Ath1 or Atoh1	Atonal homologue 1.
β-gal	β-galactosidase.
Bak	Bcl-2 homologous antagonist/killer.
Bax	Bcl-2–associated X protein.
BDNF	Brain-derived neurotrophic factor.
bFGF	Basic FGF.
BMP	Bone morphogenetic protein.
C	Cytosine.
CAG	Cytomegalovirus IE enhancer and chicken β-actin promoter.
cAMP	Cyclic adenosine monophosphate.
CAR	Coxsackie and adenovirus receptor.
Caspase	Cytosolic aspartate-specific protease.
CD	Cytosine deaminase.
cDNA	Complementary DNA.
CF	Cystic fibrosis.
CFTR	Cystic fibrosis transmembrane conductance regulator.
CGT	Cancer gene therapy.
CMV	Cytomegalovirus.
COM	Chronic otitis media.
Ctbp2	Carboxy-terminal binding protein 2.
CTL	Cytotoxic T lymphocytes.
DFNA2	Nonsyndromic sensorineural deafness type 2.
DNA	Deoxyribonucleic acid.
DSB	Double-strand breaks.
dsDNA	Double-stranded DNA.
DTH	Delayed type hypersensitivity.
E	Embryonic day.
EBV	Epstein-Barr virus.
EF1A	Elongation factor 1-α gene.

EGF	Epidermal growth factor.
EIAV	Equine infectious anaemic virus.
FasL	Fas ligand.
FGF	Fibroblast growth factor.
FITC	Fluorescein isothiocyanate.
FIV	Feline immunodeficiency virus.
G	Guanine.
GAM	Gene-activated matrix.
GCV	Gancyclovir.
GDEPT	Gene-directed enzyme prodrug therapy.
GDNF	Glial cell-line-derived neurotrophic factor.
GFP	Green fluorescent protein.
GM-CSF	Granulocyte-macrophage colony stimulating factor.
GTP	Guanosine triphosphate.
Hath1	Human atonal homologue 1.
HIV	Human immunodeficiency virus.
HL	Hearing loss.
HmR	Hammerhead ribozyme.
HNC	Head and neck cancer.
hnRNA	Heterogeneous nuclear RNA.
HNSCC	Head and neck squamous cell cancer.
HpR	Hairpin ribozyme.
HPV	Human papilloma virus.
HSV	Herpes simplex virus.
HSVtk	Herpes simplex virus thymidine kinase.
HVJ	Haemagglutinating virus of Japan.
I¹³¹-MIBG	I ¹³¹ -meta-iodobenzylguanidine.
IFs	Initiation factors.
Kbp	Kilo base pair.
KCN	Potassium channel.
KTP	Potassium titanium phosphate.
Luc	Luciferase.
Math1	Mouse atonal homologue 1.
MHC	Major histocompatibility complex.
MLV	Murine leukemia virus.
MOI	Multiplicity of infection.
mRNA	Messenger RNA.
MSCs	Mesenchymal stromal stem cells.

mtDNA	Mitochondrial DNA.
Myo7a	Myosin 7a promoter.
Nd:YAG	Neodymium:yttrium-aluminium-garnet.
NDV	Newcastle disease virus.
NIS	Sodium iodide symporter.
NT	Neurotrophin.
OC	Organ of Corti.
ODN	Oligodeoxynucleotide.
OP-1	Osteogenic protein-1.
Oris	Origins of replication.
PDGF	Platelet-derived endothelial growth factor.
PEG	Polyethylene glycol.
PET	Positron emission tomography.
PSA	Prostate specific antigen.
PSCC	Posterior semicircular canal.
PTH	Parathyroid hormone.
RCAV	Replication-competent adenovirus.
RCHSV	Replication-competent herpes simplex viruses.
RDAV	Replication-defective adenovirus.
RDRV	Replication-defective retrovirus.
RF	Release factor.
RNA	Ribonucleic acid.
rRNA	Ribosomal RNA.
RRP	Recurrent respiratory papillomatosis.
RV	Retrovirus.
RWM	Round window membrane.
S value	Sedimentation coefficient.
SCID	Severe combined immune deficiency syndrome.
SGC	Spiral ganglion cell.
snRNA	Small nuclear RNA.
SSCC	Superior semicircular canal.
T	Thymine.
TAA	Tumour-associated antigens.
TGF-β	Transforming growth factor beta.
tk	Thymidine kinase.
TM	Tympanic membrane.
TNF	Tumour necrosis factor.
TRAIL	TNF-related apoptosis-inducing ligand.

tRNA	Transfer RNA.
TRPA1	Transient receptor potential, member A1.
TSG	Tumour suppressor gene.
U	Uracil.
UTR	Untranslated region.
VEGF	Vascular endothelial growth factor.
VSV	Vesicular stomatitis virus.
VSVG	Vesicular stomatitis virus glycoprotein.

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Introduction