Recent patents in hematopoietic cell gene therapy

Patent number	Description	Assignee	Inventor	Publication date
US9029520	A DNA vaccine containing a recombinant expression vector that contains exotoxin fusion gene B7-1-PE40KDEL, which is effectively ligated into selected eukaryotic expression vectors; useful for the treatment or prevention of allogeneic tissue/organ transplant rejection and hematopoietic stem cell transplantation rejection such as graft-versus-host disease.	Affiliated Hospital of Academy of Military Medical Sciences (Beijing)	Xi Y, Luo Y	5/12/2015
US9023646	HIV-derived lentivectors useful for expressing transgenes for genetic treatments such as inherited and acquired lympho-hematological disorders, gene-therapies for cancers, especially the hematological cancers, as well as for the study of hematopoiesis via lentivector-mediated modification of human hematopoietic stem cells.	Research Development Foundation (Carson City, NV, USA)	Trono D, Salmon P	5/5/2015
US8933082	Materials and methods for suppressing and/or treating neurofibroma and related tumors by therapeutically effective doses of a tyrosine kinase inhibitor, such as the compound imatinib mesylate to a patient in need thereof to treat tumors in a human patient.	Indiana University Research & Technology Corp. (Indianapolis)	Clapp DW, Ingram D, Yang FC	1/13/2015
US8900567	A method of preserving cone cells in the eye of a mammal suffering from a retinal degenerative disease, comprising isolating from the bone marrow of the mammal a lineage-negative hematopoietic stem cell population that includes endothelial progenitor cells, transfecting cells from the stem cell population with a gene that operably encodes an antiangiogenic fragment of human tryptophanyl tRNA synthetase (TrpRS), and subsequently intravitreally injecting the transfected cells into the eye of the mammal in an amount sufficient to inhibit the degeneration of cone cells in the retina of the eye.	The Scripps Research Institute (La Jolla, CA, USA)	Friedlander M, Otani A, Da Silva K, Moreno S	12/2/2014
US8748169	HIV-derived lentiviral vectors comprising promoters that promote expression specific to cell types or tissues and that are useful for expressing transgenes for human gene therapy, especially in human hematopoietic progenitor cells as well as in all other blood cell derivatives.	Research Development Foundation (Carson City, NV, USA)	Trono D, Wiznerowicz M	6/10/2014
US8889419	A method to increase the efficiency of transduction of hematopoietic and other cells by retroviruses includes infecting the cells in the presence of fibronectin or fibronectin fragments. The fibronectin and fibronectin fragments significantly enhance retroviral-mediated gene transfer into the cells, particularly hematopoietic cells including committed progenitors and primitive hematopoietic stem cells.	Indiana University Research & Technology Corp. (Indianapolis)	Williams DA	11/18/2014
US8772028	Human progenitor T cells able to successfully engraft a murine thymus and differentiate into mature human T and NK cells. The human progenitor T cells have the phenotype CD34+CD7+CD1a–CD5– or CD34+CD7+CD1a–CD5+ and are derived from human hematopoietic stem cells, embryonic stem cells and induced pluripotent stem cells b\ coculture with cells expressing a Notch receptor ligand (OP9-DL1 or OP9-DL4); useful in a variety of applications including immune reconstitution, the treatment of immunodeficiencies and as carriers for genes used in gene therapy.	Sunnybrook Health Sciences Centre (Toronto, ON, Canada)	Zuniga-Pflucker JC, Awong G, La Motte-Mohs R	7/8/2014
US8692052	Genetically modified mice and engraftment, including a mouse with a humanization of the mIL-3 gene and the mGM-CSF gene, a knockout of a mRAG gene, and a knockout of a mII2rg subunit gene; and optionally a humanization of the TPO gene; a RAG/II2rg KO/hTPO knock-in mouse; and a mouse engrafted with human hematopoietic stem cells (HSCs) that maintains a human immune cell (HIC) population derived from the HSCs and that is infectable by a human pathogen, e.g., (*S. typhi* or *Mycobacterium tuberculosis*.	Regeneron Pharmaceuticals (Tarrytown, NY, USA), Yale University (New Haven, CT, USA), Institute for Research in Biomedicine (Bellinzona, Switzerland)	Stevens S, Murphy AJ, Flavell R, Eynon E, Galan J, Willinger T, Manz M, Rongvaux A, Yancopoulos GD	4/8/2014
US8461127	A method of expanding a cell population, cells with decreased p27 and/or p21 activity, transgenic animals with a disrupted p27 and/or p21 gene, pharmaceutical compositions comprising the cells of the invention, and methods of using these cells in gene therapy (e.g., stem cell gene therapy) and bone marrow transplantation.	The General Hospital Corp. (Boston)	Scadden DT, Cheng T	6/11/2013
US8258100	Amphipathic lytic peptides ideally suited to use in a ligand-cytotoxin combination to specifically inhibit cells that are driven by or are dependent upon a specific ligand interaction; may be used in gene therapy to treat malignant or nonmalignant tumors, and other diseases caused by clones or populations of "normal" host cells bearing specific receptors (such as lymphocytes), because genes encoding a lytic peptide or encoding a lytic peptide or peptide hormone fusion may readily be inserted into hematopoietic stem cells or myeloid precursor cells.	Board of Supervisors of Louisiana State University and Agricultural and Mechanical College (Baton Rouge, LA, USA)	Enright FM, Jaynes JM, Jesse M, Hansel W, Koonce KL, McCann SM, Yu WH, Melrose PA, Foil LD, Elzer PH	9/4/2012

Source: US Patent and Trademark Office (http://www.uspto.gov); European Patent Office (http://www.epo.org).