11/04/03
200202053 4

(12) INTERNATIONAL APPLICATION PUBLISHED UNDER THE PATENT COOPERATION TREATY (PCT)

(19) World Intellectual Property Organization
International Bureau

(43) International Publication Date
18 April 2002 (18.04.2002)

PCT

(10) International Publication Number
WO 02/31110 A3

(51) International Patent Classification[7]: A61P 31/18, 37/00, 37/04, 37/06, A61K 31/18, 35/28, 35/54

(21) International Application Number: PCT/IB01/02739

(22) International Filing Date: 12 October 2001 (12.10.2001)

(25) Filing Language: English

(26) Publication Language: English

(30) Priority Data:

09/795,286	13 October 2000 (13.10.2000)	US
09/795,302	13 October 2000 (13.10.2000)	US
PR 0745	13 October 2000 (13.10.2000)	AU
09/758,910	10 January 2001 (10.01.2001)	US
09/966,576	26 September 2001 (26.09.2001)	US
09/969,510	1 October 2001 (01.10.2001)	US

(71) Applicant and
(72) Inventor: BOYD, Richard [AU/AU]; Department of Pathology & Immunology, Monash Medical School, Commercial Road, Prahran, VIC 3181 (AU).

(74) Agent: DAVIES COLLISON CAVE; Level 3, 303 Coronation Drive, Milton, Queensland 4064 (AU).

(81) Designated States *(national)*: AE, AG, AL, AM, AT, AU, AZ, BA, BB, BG, BR, BY, BZ, CA, CH, CN, CO, CR, CU, CZ, DE, DK, DM, DZ, EC, EE, ES, FI, GB, GD, GE, GH, GM, HR, HU, ID, IL, IN, IS, JP, KE, KG, KP, KR, KZ, LC, LK, LR, LS, LT, LU, LV, MA, MD, MG, MK, MN, MW, MX, MZ, NO, NZ, PH, PL, PT, RO, RU, SD, SE, SG, SI, SK, SL, TJ, TM, TR, TT, TZ, UA, UG, UZ, VN, YU, ZA, ZW.

(84) Designated States *(regional)*: ARIPO patent (GH, GM, KE, LS, MW, MZ, SD, SL, SZ, TZ, UG, ZW), Eurasian patent (AM, AZ, BY, KG, KZ, MD, RU, TJ, TM), European patent (AT, BE, CH, CY, DE, DK, ES, FI, FR, GB, GR, IE, IT, LU, MC, NL, PT, SE, TR), OAPI patent (BF, BJ, CF, CG, CI, CM, GA, GN, GQ, GW, ML, MR, NE, SN, TD, TG).

Declarations under Rule 4.17:

--- *as to applicant's entitlement to apply for and be granted a patent (Rule 4.17(ii)) for all designations*

— *as to the applicant's entitlement to claim the priority of the earlier application (Rule 4.17(iii)) for all designations*

Published:

— *with international search report*

-- *before the expiration of the time limit for amending the claims and to be republished in the event of receipt of amendments*

(88) Date of publication of the international search report:
20 June 2002

For two-letter codes and other abbreviations, refer to the "Guidance Notes on Codes and Abbreviations" appearing at the beginning of each regular issue of the PCT Gazette.

162162

ACTION

WO 02/31110 A3

(54) Title: HEMATOPOIETIC STEM CELL GENE THERAPY

(57) Abstract: The present disclosure provides methods for gene therapy utilizing hematopoietic stem cells, lymphoid progenitor cells, and/or myeloid progenitor cells. The cells are genetically modified to provide a gene that is expressed in these cells and their progeny after differentiation. In a preferred embodiment the cells contain a gene or gene fragment that confers to the cells resistance to HIV infection and/or replication. The cells are administered to a patient in conjunction with treatment to reactivate the patient's thymus. The cells may be autologous, syngeneic, allogeneic or xenogeneic, as tolerance to foreign cells is created in the patient during reactivation of the thymus. In a preferred embodiment the hematopoietic stem cells are $CD34^+$. The patient's thymus is reactivated by disruption of sex steroid mediated signaling to the thymus. In a preferred embodiment, this disruption is created by administration of LHRH agonists, LHRH antagonists, anti-LHRH receptor antibodies, anti-LHRH vaccines or combinations thereof.