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

(19) World Intellectual Property Organization
International Bureau

(43) International Publication Date
14 June 2012 (14.06.2012)

WIPO | PCT

(10) International Publication Number
WO 2012/079000 A1

(51) International Patent Classification:
C07H 21/04 (2006.01) *A61K 39/00* (2006.01)

(21) International Application Number:
PCT/US2011/064191

(22) International Filing Date:
9 December 2011 (09.12.2011)

(25) Filing Language: English

(26) Publication Language: English

(30) Priority Data:
61/421,470 9 December 2010 (09.12.2010) US
61/502,649 29 June 2011 (29.06.2011) US

(71) Applicant *(for all designated States except US)*: **THE TRUSTEES OF THE UNIVERSITY OF PENNSYLVANIA** [US/US]; Center For Technology Transfer, 3160 Chestnut Street, Suite 200, Philadelphia, PA 19104-6283 (US).

(72) Inventors; and

(75) Inventors/Applicants *(for US only)*: **JUNE, Carl, H.** [US/US]; 409 Baird Road, Merion Station, PA 19066 (US). **LEVINE, Bruce, L.** [US/US]; 1258 Liberty Bell Drive, Cherry Hill, NJ 08003 (US). **PORTER, David, L.** [US/US]; 821 Crum Creek Road, Springfield, PA 19064 (US). **KALOS, Michael, D.** [US/US]; 716 Carpenter Lane, Philadelphia, PA 19119 (US).

(74) Agents: **NGUYEN, Quang, D.** et al.; Riverside Law, LLP, 300 Four Falls Corporate Center, Suite 710, 300 Conshohocken State Road, West Conshohocken, PA 19428 (US).

(81) Designated States *(unless otherwise indicated, for every kind of national protection available)*: AE, AG, AL, AM, AO, AT, AU, AZ, BA, BB, BG, BH, BR, BW, BY, BZ, CA, CH, CL, CN, CO, CR, CU, CZ, DE, DK, DM, DO, DZ, EC, EE, EG, ES, FI, GB, GD, GE, GH, GM, GT, HN, HR, HU, ID, IL, IN, IS, JP, KE, KG, KM, KN, KP, KR, KZ, LA, LC, LK, LR, LS, LT, LU, LY, MA, MD, ME, MG, MK, MN, MW, MX, MY, MZ, NA, NG, NI, NO, NZ, OM, PE, PG, PH, PL, PT, QA, RO, RS, RU, RW, SC, SD, SE, SG, SK, SL, SM, ST, SV, SY, TH, TJ, TM, TN, TR, TT, TZ, UA, UG, US, UZ, VC, VN, ZA, ZM, ZW.

(84) Designated States *(unless otherwise indicated, for every kind of regional protection available)*: ARIPO (BW, GH, GM, KE, LR, LS, MW, MZ, NA, RW, SD, SL, SZ, TZ, UG, ZM, ZW), Eurasian (AM, AZ, BY, KG, KZ, MD, RU, TJ, TM), European (AL, AT, BE, BG, CH, CY, CZ, DE, DK, EE, ES, FI, FR, GB, GR, HR, HU, IE, IS, IT, LT, LU, LV, MC, MK, MT, NL, NO, PL, PT, RO, RS, SE, SI, SK, SM, TR), OAPI (BF, BJ, CF, CG, CI, CM, GA, GN, GQ, GW, ML, MR, NE, SN, TD, TG).

Published:

— *with international search report (Art. 21(3))*

— *before the expiration of the time limit for amending the claims and to be republished in the event of receipt of amendments (Rule 48.2(h))*

— *with sequence listing part of description (Rule 5.2(a))*

WO 2012/079000 A1

(54) Title: USE OF CHIMERIC ANTIGEN RECEPTOR-MODIFIED T CELLS TO TREAT CANCER

(57) Abstract: The present invention provides compositions and methods for treating cancer in a human. The invention includes relates to administering a genetically modified T cell to express a CAR wherein the CAR comprises an antigen binding domain, a transmembrane domain, a costimulatory signaling region, and a CD3 zeta signaling domain.