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

(19) World Intellectual Property Organization
International Bureau

(43) International Publication Date
08 October 2020 (08.10.2020)

WIPO | PCT

(10) International Publication Number
WO 2020/205720 A2

(51) International Patent Classification:
A61K 35/32 (2015.01)

(21) International Application Number:
PCT/US2020/025689

(22) International Filing Date:
30 March 2020 (30.03.2020)

(25) Filing Language: English

(26) Publication Language: English

(30) Priority Data:
62/826,553 29 March 2019 (29.03.2019) US

(71) Applicant: **KOLON TISSUEGENE, INC.** [US/US]; 9713 Key West Ave, Suite 300, Rockville, MD 20850 (US).

(72) Inventors: **NOH, Moon, Jong**; 9713 Key West Ave., Suite 300, Rockville, MD 20850 (US). **YI, Youngsuk**; 9713 Key West Ave., Suite 300, Rockville, MD 20850 (US). **SONG, Sun, Uk**; 9713 Key West Ave., Suite 300, Rockville, MD 20850 (US). **LEE, Kwan, Hee**; 9713 Key West Ave., Suite 300, Rockville, MD 20850 (US).

(74) Agent: **KIM, Joseph, Hypsuk**; Nixon & Vanderhye P.C., 901 North Glebe Road, 11th Floor, Arlington, VA 22203-1808 (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, BN, BR, BW, BY, BZ, CA, CH, CL, CN, CO, CR, CU, CZ, DE, DJ, DK, DM, DO, DZ, EC, EE, EG, ES, FI, GB, GD, GE, GH, GM, GT, HN, HR, HU, ID, IL, IN, IR, IS, JO, JP, KE, KG, KH, KN, KP, KR, KW, KZ, LA, LC, LK, LR, LS, LU, LY, MA, MD, ME, MG, MK, MN, MW, MX, MY, MZ, NA, NG, NI, NO, NZ, OM, PA, PE, PG, PH, PL, PT, QA, RO, RS, RU, RW, SA, SC, SD, SE, SG, SK, SL, ST, SV, SY, TH, TJ, TM, TN, TR, TT, TZ, UA, UG, US, UZ, VC, VN, WS, 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, ST, SZ, TZ, UG, ZM, ZW), Eurasian (AM, AZ, BY, KG, KZ, 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, KM, ML, MR, NE, SN, TD, TG).

Declarations under Rule 4.17:
— *of inventorship (Rule 4.17(iv))*

Published:
— *without international search report and to be republished upon receipt of that report (Rule 48.2(g))*

WO 2020/205720 A2

(54) Title: MIXED-CELL GENE THERAPY

(57) Abstract: The subject invention is directed to a mixed cell composition to generate a therapeutic protein at a target site by providing a first population of mammalian cells transfected or transduced with a gene that is sought to be expressed, and a second population of mammalian cells that have not been transfected or transduced with the gene, wherein endogenously existing forms of the second population of mammalian cells are decreased at the target site, and wherein generation of the therapeutic protein by the first population of mammalian cells at the target site stimulates the second population cells to induce a therapeutic effect.