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

(19) World Intellectual Property Organization
International Bureau

(43) International Publication Date
23 July 2020 (23.07.2020)

WIPO | PCT

(10) International Publication Number
WO 2020/150478 A1

(51) International Patent Classification:
A61K 35/12 (2015.01)

(21) International Application Number:
PCT/US2020/013887

(22) International Filing Date:
16 January 2020 (16.01.2020)

(25) Filing Language: English

(26) Publication Language: English

(30) Priority Data:
62/793,210 16 January 2019 (16.01.2019) US
62/852,573 24 May 2019 (24.05.2019) US

(71) Applicant: **THE TRUSTEES OF COLUMBIA UNIVERSITY IN THE CITY OF NEW YORK** [US/US]; 412 Low Memorial Library, 535 West 116th Street, New York, NY 10027 (US).

(72) Inventors: **MUKHERJEE, Siddhartha**; c/o Columbia University Medical Center, 701 W. 168 St., New York, NY 10032 (US). **BOROT, Florence**; c/o Columbia University Medical Center, 701 W. 168 St., New York, NY 10032 (US). **ALI, Abdullah, Mahmood**; c/o Columbia University Medical Center, 701 W. 168 St., New York, NY 10032 (US).

(74) Agent: **DAVITZ, Michael, A.** et al.; Leason Ellis LLP, One Barker Avenue, Fifth Floor, White Plains, NY 10601 (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).

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))*

(54) Title: COMPOSITIONS AND METHODS FOR INHIBITION OF LINEAGE SPECIFIC ANTIGENS

(57) Abstract: Disclosed herein are methods of administering an agent targeting a lineage-specific cell- surface antigen, e.g., CD33, and a population of hematopoietic cells that are deficient in the lineage-specific cell-surface antigen, e.g., CD33 for immunotherapy of hematological malignancies. Cells comprising mutations in CD33 are also provided, as are gRNAs targeting CD33.

WO 2020/150478 A1