

(19) World Intellectual Property
Organization
International Bureau

(43) International Publication Date
07 May 2020 (07.05.2020)



(10) International Publication Number
WO 2020/092404 A8

(51) International Patent Classification:

A61P 31/00 (2006.01) C07D 417/14 (2006.01)

(21) International Application Number:

PCT/US2019/058616

(22) International Filing Date:

29 October 2019 (29.10.2019)

(25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data:

62/752,659 30 October 2018 (30.10.2018) US
62/769,078 19 November 2018 (19.11.2018) US
62/878,368 25 July 2019 (25.07.2019) US

(71) Applicants: **MACROGENICS, INC.** [US/US]; 9704 Medical Center Drive, Rockville, MD 20850 (US). **NANOSTRING TECHNOLOGIES, INC.** [US/US]; 530 Fairview Ave. North, Seattle, WA 98109 (US). **NOTTINGHAM TRENT UNIVERSITY** [GB/GB]; 50 Shakespeare Street, Nottingham NG1 4FQ (GB).

(72) Inventors: **DAVIDSON, Jan, Kenneth**; 9704 Medical Center Drive, Rockville, MD 20850 (US). **CHURCH,**

Sara; 6311 Rockefeller Ave., Everett, WA 98203 (US). **RUTELLA, Sergio**; 50 Shakespeare Street, Nottingham NG1 4FQ (GB).

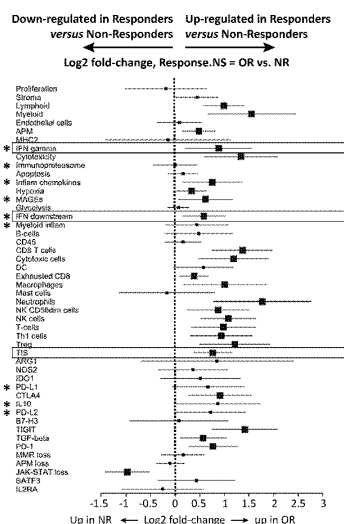
(74) Agent: **AUERBACH, Jeffrey, I.** et al.; AuerbachSchrodt LLC, 2200 Research Blvd., Suite 560, Rockville, MD 20850 (US).

(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, 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, 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,

(54) Title: BISPECIFIC CD123 X CD3 DIABODIES FOR THE TREATMENT OF HEMATOLOGIC MALIGNANCIES

(57) Abstract: The present invention is directed to a method of treating a hematologic malignancy such as acute myeloid leukemia (AML) or myelodysplastic syndrome (MDS), including hematologic malignancies that are refractive to chemotherapeutic and/or hypomethylating agents. The method concerns administering a CD123 x CD3 bispecific binding molecule to a patient in an amount effective to stimulate the killing of cells of said hematologic malignancy in said patient. The present invention is additionally directed to the embodiment of such method in which a cellular sample from the patient evidences an expression of one or more target genes that is increased relative to a baseline level of expression of such genes, for example, a baseline level of expression of such genes in a reference population of individuals who are suffering from the hematologic malignancy, or with respect to the level of expression of a reference gene.



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

(48) Date of publication of this corrected version:

20 May 2021 (20.05.2021)

(15) Information about Correction:

see Notice of 20 May 2021 (20.05.2021)