

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

(19) World Intellectual Property
Organization
International Bureau

(43) International Publication Date
01 July 2021 (01.07.2021)



(10) International Publication Number
WO 2021/130223 A1

(51) International Patent Classification:

A61K 39/00 (2006.01) *A61P 35/00* (2006.01)
C07K 14/725 (2006.01) *A61K 9/127* (2006.01)
A61K 35/17 (2015.01) *A61K 39/395* (2006.01)
C07K 16/28 (2006.01) *C12N 15/62* (2006.01)
A61K 31/7105 (2006.01)

(21) International Application Number:

PCT/EP2020/087623

(22) International Filing Date:

22 December 2020 (22.12.2020)

(25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data:

PCT/EP2019/086950

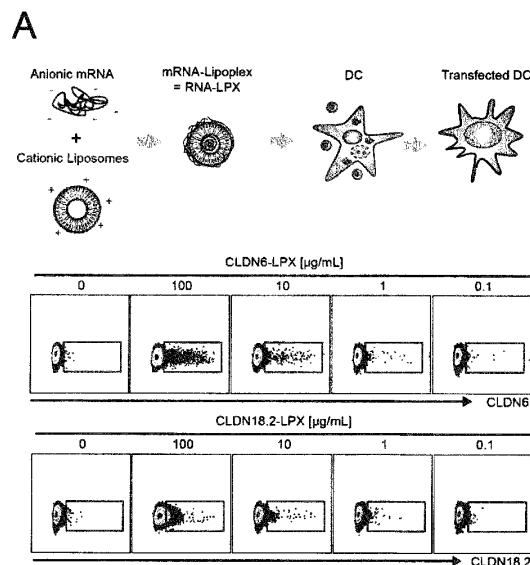
23 December 2019 (23.12.2019) EP

(71) Applicant: **BIONTECH CELL & GENE THERAPIES GMBH** [DE/DE]; An der Goldgrube 12, 55131 Mainz (DE).

(72) Inventors: **BILLMEIER, Arne**; c/o BioNTech SE, An der Goldgrube 12, 55131 Mainz (DE). **BIRTEL, Matthias**; c/o BioNTech SE, An der Goldgrube 12, 55131 Mainz (DE). **HAYDUK, Nina**; c/o BioNTech SE, An der Goldgrube 12, 55131 Mainz (DE). **JAHNDEL, Veronika**; c/o BioNTech SE, An der Goldgrube 12, 55131 Mainz (DE). **KLEIN, Oliver**; c/o BioNTech SE, An der Goldgrube 12, 55131 Mainz (DE). **KUNA, Kathrin**; c/o BioNTech SE, An der Goldgrube 12, 55131 Mainz (DE). **MICHEL, Kristina**; c/o BioNTech SE, An der Goldgrube 12, 55131 Mainz (DE). **OEHM, Petra**; c/o BioNTech SE, An der Goldgrube 12, 55131 Mainz (DE). **OUCHAN, Yasmina**; c/o BioNTech SE, An der Goldgrube 12, 55131 Mainz (DE). **REINHARD, Katharina**; c/o BioNTech SE, An der Gold-

(54) Title: TREATMENT INVOLVING IMMUNE EFFECTOR CELLS GENETICALLY MODIFIED TO EXPRESS ANTIGEN RECEPTORS

Figure 2



(57) Abstract: The present disclosure relates to methods for enhancing the efficiency of therapies involving immune effector cells such as T cells engineered to express antigen receptors such as T cell receptors (TCRs) or chimeric antigen receptors (CARs). It is demonstrated herein that such antigen receptor-engineered immune effector cells, even when provided to a subject in sub-therapeutic amounts, are extremely effective in the treatment of cancer diseases, even those cancer diseases that are known to be difficult to treat with antigen receptor-engineered immune effector cells, such as solid tumors or cancers, if additionally target antigen for the antigen receptor is provided to the subject. Immune effector cells may be engineered ex vivo or in vitro and subsequently the immune effector cells may be administered to a subject in need of treatment, or immune effector cells may be engineered in vivo in a subject in need of treatment.

[Continued on next page]

grube 12, 55131 Mainz (DE). **RENGSTL, Benjamin**; c/o BioNTech SE, An der Goldgrube 12, 55131 Mainz (DE). **SAHIN, Ugur**; c/o BioNTech SE, An der Goldgrube 12, 55131 Mainz (DE).

(74) **Agent: SCHNAPPAUF, Georg**; Zwicker, Schnappauf & Partner Patentanwälte PartG mbB, Hansastr. 32, 80686 Munich (DE).

(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, IT, 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))