



(51) International Patent Classification:

C12N 5/10 (2006.01) A61K 35/12 (2015.01)
C12N 15/11 (2006.01)

Published:

- with international search report (Art. 21(3))
- with sequence listing part of description (Rule 5.2(a))

(21) International Application Number:

PCT/IB2020/058281

(22) International Filing Date:

04 September 2020 (04.09.2020)

(25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data:

62/896,473 05 September 2019 (05.09.2019) US
62/979,771 21 February 2020 (21.02.2020) US

(71) Applicant: CRISPR THERAPEUTICS AG [CH/CH];

Baarerstrasse 14, 6300 Zug (CH).

(72) Inventors: REZANIA, Alireza; c/o Crispr Therapeutics,
610 Main Street, Cambridge, Massachusetts 02139 (US).

RAMOS-ZAYAS, Rebeca; c/o Crispr Therapeutics, 610
Main Street, Cambridge, Massachusetts 02139 (US).

(74) Agent: VOSSIUS & PARTNER; Patentanwälte Rechtsan-

wälte mbB, Siebertstrasse 3, 81675 München (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).

(54) Title: UNIVERSAL DONOR CELLS

(57) Abstract: Genetically modified cells that are compatible with multiple subjects, e.g., universal donor cells, and methods of generating said genetic modified cells are provided herein. The universal donor cells comprise at least one genetic modification within or near at least one gene that encodes a survival factor, wherein the genetic modification comprises an insertion of a polynucleotide encoding a tolerogenic factor. The universal donor cells may further comprise at least one genetic modification within or near a gene that encodes one or more MHC-I or MHC-II human leukocyte antigens or a component or a transcriptional regulator of a MHC-I or MHC-II complex, wherein said genetic modification comprises an insertion of a polynucleotide encoding a second tolerogenic factor.

