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

(19) World Intellectual Property Organization
International Bureau

(43) International Publication Date
23 April 2020 (23.04.2020)

WIPO | PCT

(10) International Publication Number
WO 2020/081843 A1

(51) International Patent Classification:
C12N 15/113 (2010.01) *C12N 15/90* (2006.01)
A61K 48/00 (2006.01)

(21) International Application Number:
PCT/US2019/056779

(22) International Filing Date:
17 October 2019 (17.10.2019)

(25) Filing Language: English

(26) Publication Language: English

(30) Priority Data:
62/747,128 17 October 2018 (17.10.2018) US

(71) Applicant: **CASEBIA THERAPEUTICS LIMITED LIABILITY PARTNERSHIP** [US/US]; 455 Mission Bay Boulevard South, San Francisco, California 94158 (US).

(72) Inventors: **BROOKS, Alan Richard**; c/o 455 Mission Bay Boulevard South, San Francisco, California 94158 (US). **VO, Karen**; c/o 455 Mission Bay Boulevard South, San Francisco, California 94158 (US).

(74) Agent: **MANDRAGOURAS, Amy E.** et al.; Nelson Mullins Riley & Scarborough LLP, One Post Office Square, Boston, MA 02109-2127 (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, 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:
— *as to applicant's entitlement to apply for and be granted a patent (Rule 4.17(ii))*

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 DELIVERING TRANSGENES

(57) Abstract: Provided herein are compositions, methods, and systems for targeted delivery of nucleic acids, including DNA and RNA, to a target cell. Also provided are compositions, methods, and systems for expressing a transgene in a cell by genomic editing. Further provided are compositions, methods, and systems for knocking in a gene-of-interest (GOI) into a target genomic locus in the genome, in particular the locus of albumin gene. Also provided are compositions, methods, and systems for treating a subject having or suspected of having a disorder or health condition employing ex vivo and/or in vivo genome editing.

WO 2020/081843 A1