

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

(19) World Intellectual Property
Organization
International Bureau

(43) International Publication Date
12 August 2021 (12.08.2021)



(10) International Publication Number
WO 2021/158858 A1

(51) International Patent Classification:
C12N 15/113 (2010.01) A61K 31/7125 (2006.01)
A61K 31/712 (2006.01)

(21) International Application Number:
PCT/US2021/016730

(22) International Filing Date:
05 February 2021 (05.02.2021)

(25) Filing Language: English

(26) Publication Language: English

(30) Priority Data:
62/971,906 07 February 2020 (07.02.2020) US
62/981,965 26 February 2020 (26.02.2020) US
63/019,076 01 May 2020 (01.05.2020) US

(71) Applicant: INTELLIA THERAPEUTICS, INC.
[US/US]; 40 Erie Street, Cambridge, Massachusetts 02139 (US).

(72) Inventors: ODATE, Sobu; c/o Intellia Therapeutics, Inc.,
40 Erie Street, Cambridge, Massachusetts 02139 (US).
SEITZER, Jessica Lynn; c/o Intellia Therapeutics, Inc., 40
Erie Street, Cambridge, Massachusetts 02139 (US).

(74) Agent: BAUR, Amelia Feulner et al.; MCNEILL BAUR
PLLC, 125 Cambridge Park Drive, Suite 301, Cambridge,
Massachusetts 02140 (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, 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: COMPOSITIONS AND METHODS FOR KALLIKREIN (KLKB1) GENE EDITING

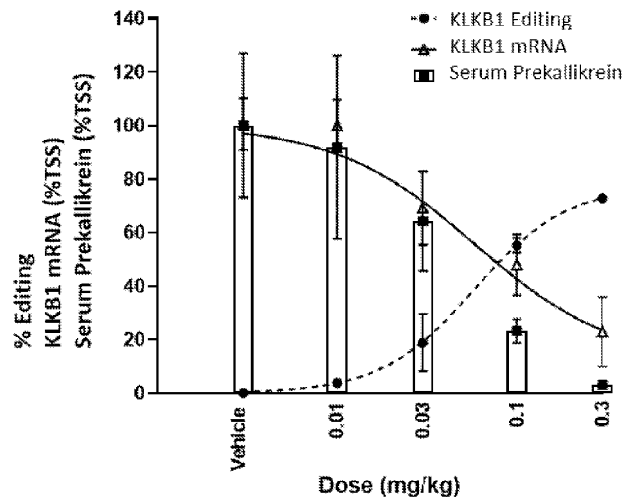


Fig. 10

(57) Abstract: Compositions and methods for editing, e.g., introducing double-stranded breaks, within the *KLKB1* gene are provided. Compositions and methods for treating subjects having hereditary angioedema (HAE), are provided.

[Continued on next page]

Published:

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