

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

(19) World Intellectual Property
Organization
International Bureau

(43) International Publication Date
17 February 2022 (17.02.2022)



(10) International Publication Number
WO 2022/036179 A3

(51) International Patent Classification:
A61K 48/00 (2006.01) *C12N 5/07* (2010.01)
A61P 7/04 (2006.01)

TR), OAPI (BF, BJ, CF, CG, CI, CM, GA, GN, GQ, GW,
KM, ML, MR, NE, SN, TD, TG).

(21) International Application Number:
PCT/US2021/045881

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))

(22) International Filing Date:
13 August 2021 (13.08.2021)

(25) Filing Language: English

(26) Publication Language: English

(88) Date of publication of the international search report:
24 March 2022 (24.03.2022)

(30) Priority Data:
63/066,011 14 August 2020 (14.08.2020) US

(71) Applicant: **ORCHARD THERAPEUTICS (EUROPE) LIMITED** [GB/GB]; 108 Cannon Street, London EC4N 6EU (GB).

(72) Inventor; and

(71) Applicant: **WOLFE, Jia, L.** [US/US]; 28 Hollywood Road, Winchester, MA 01890 (US).

(72) Inventors: **GASPAR, Bobby**; 5, Ardberg Road, London SE24 9JL (GB). **LEMOINE, Adrien**; 4 Acton Street, Flat 7, London WC1X 9NA (GB). **MARKS-BLUTH, Jonathon, Simeon**; 102b Hanley Road, London N4 3DW (GB). **SAGOO, Pervinder**; 18 St Vincent Drive, St Albans AL1 5S1 (GB). **PENNUCCI, Valentina**; 109 Buckler Court, Eden Grove, London N78GQ (GB).

(74) Agent: **BELLIVEAU, Michael, J.**; Clark & Elbing LLP, 101 Federal Street, 15th Floor, Boston, MA 02110 (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,

(54) Title: COMPOSITIONS AND METHODS FOR TREATING OR PREVENTING HEREDITARY ANGIOEDEMA

(57) Abstract: Described herein are compositions and methods for treating a subject having or at risk of developing hereditary angioedema. Using the compositions and methods of the disclosure a patient may be provided one or more agents that elevate the expression and/or activity levels of C1-esterase inhibitor (C1-INH). Exemplary agents that may be used in conjunction with the compositions and methods of the disclosure for this purpose include cells, such as pluripotent cells, that express C1-INH.

WO 2022/036179 A3