

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

(19) World Intellectual Property
Organization

International Bureau

(43) International Publication Date
23 December 2021 (23.12.2021)



(10) International Publication Number
WO 2021/257668 A1

(51) International Patent Classification:

A61P 7/00 (2006.01) C07K 16/28 (2006.01)

A61K 45/06 (2006.01)

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))

(21) International Application Number:

PCT/US2021/037575

(22) International Filing Date:

16 June 2021 (16.06.2021)

(25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data:

63/040,381 17 June 2020 (17.06.2020) US

63/135,998 11 January 2021 (11.01.2021) US

63/152,085 22 February 2021 (22.02.2021) US

(71) Applicant: THE TRUSTEES OF THE UNIVERSITY

OF PENNSYLVANIA [US/US]; 3600 Civic Center Blvd.,
9th Floor, Philadelphia, PA 19104 (US).

(72) Inventors: **HINDERER, Christian**; 4723 Laurel Street,
New Orleans, LA 70115 (US). **HORIUCHI, Makoto**;
413 Spruce Street, Apt. 1, Philadelphia, PA 19106 (US).
WILSON, James, M.; 1831 Delancey Street, Philadelphia,
PA 19103 (US).

(74) Agent: **KODROFF, Cathy, A.** et al.; Howson & Howson
LLP, 325 Sentry Parkway East, Five Sentry East, Suite 160,
Blue Bell, PA 19422 (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 TREATMENT OF GENE THERAPY PATIENTS

(57) Abstract: Provided herein are compositions useful for co-administering with a gene therapy vector to a patient having pre-existing neutralizing antibodies to the viral source of the gene therapy vector capsid. The compositions comprise an FcRn ligand which inhibits specific binding between FcRn and IgG.

WO 2021/257668 A1