

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

(19) World Intellectual Property
Organization
International Bureau



(10) International Publication Number
WO 2020/028466 A1

(43) International Publication Date
06 February 2020 (06.02.2020)

(51) International Patent Classification:

A61P 25/00 (2006.01) C12N 15/63 (2006.01)
C12N 15/09 (2006.01) C12N 15/86 (2006.01)
C12N 15/12 (2006.01)

(21) International Application Number:

PCT/US2019/044290

(22) International Filing Date:

31 July 2019 (31.07.2019)

(25) Filing Language: English

(26) Publication Language: English

(30) Priority Data:

62/712,669 31 July 2018 (31.07.2018) US

(72) Inventor; and

(71) Applicant: **KAPLITT, Michael G.** [US/US]; 395 Pine
Treet Road, Suite 310, Ithaca, New York 14850 (US).

(74) Agent: **LANG, Allen, R.** et al.; Schwegman, Lundberg &
Woessner, P.A., P.O. Box 2938, Minneapolis, Minnesota
55402 (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).

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))

(54) Title: GENE THERAPY METHODS TO CONTROL ORGAN FUNCTION

(57) Abstract: Methods and compositions for controlling visceral organ function are provided. For example, the methods and compositions are useful to prevent, inhibit or treat disease as a result of controlling, e.g., regulating, organ function. In one embodiment, viral vectors are delivered to an organ, and the virus infects a nerve that regulates a function of the organ. In one embodiment, the viral vector is a retrograde vector. In one embodiment, the viral vector encodes a gene product, the activity of which is controlled by an exogenously delivered agent or energy. The delivery of the agent or energy thus controls the organ function.

WO 2020/028466 A1