



(51) International Patent Classification:

A61K 48/00 (2006.01) A61P 25/28 (2006.01)
A61K 35/76 (2015.01) C12N 15/86 (2006.01)
A61P 25/00 (2006.01)

(21) International Application Number:

PCT/US2023/078864

(22) International Filing Date:

06 November 2023 (06.11.2023)

(25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data:

63/423,481 07 November 2022 (07.11.2022) US

(71) Applicant: **OCUGEN, INC.** [US/US]; 11 Great Valley
Parkway, Malvern, PA 19355 (US).

(72) Inventor: **UPADHYAY, Arun, Kumar**; 403 Blue Welsh
St., Malvern, PA 19355 (US).

(74) Agent: **CHA, Don, D.**; 14405 W Colfax Ave, Suite 305,
Lakewood, CO 80228 (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, CV, DE, DJ, DK, DM, DO, DZ, EC, EE, EG, ES, FI, GB, GD, GE, GH, GM, GT, HN, HR, HU, ID, IL, IN, IQ, IR, IS, IT, JM, JO, JP, KE, KG, KH, KN, KP, KR, KW, KZ, LA, LC, LK, LR, LS, LU, LY, MA, MD, MG, MK, MN, MU, 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, CV, GH, GM, KE, LR, LS, MW, MZ, NA, RW, SC, 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, ME, 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: TREATMENT OF NEUROLOGICAL DISORDER USING NHR

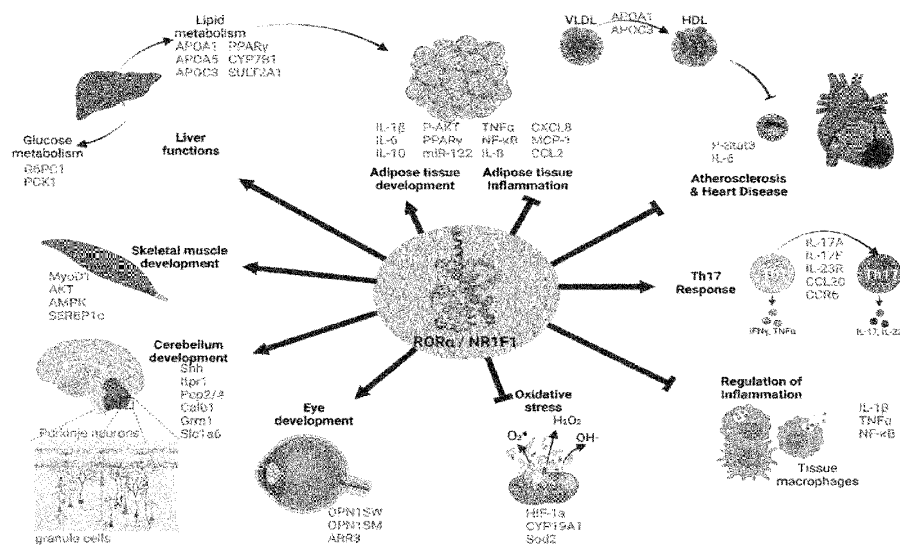


FIG. 1

(57) Abstract: The present disclosure provides methods for ameliorating or treating a neurological condition or disease in a subject in need of treatment thereof by administering to the subject a therapeutically effective amount of a composition comprising (i) a recombinant DNA (rDNA), wherein the rDNA comprises a human nuclear hormone receptor (hNHR) gene or a fragment thereof; and (ii) a delivery vehicle adapted for delivering said rDNA to neurological cells or tissues to ameliorate or treat said neurological condition or disease.

[Continued on next page]

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))*

(88) Date of publication of the international search report:

27 June 2024 (27.06.2024)