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

(19) World Intellectual Property Organization
International Bureau

(43) International Publication Date
28 August 2025 (28.08.2025)

WIPO | PCT

(10) International Publication Number
WO 2025/176843 A1

(51) International Patent Classification:
A61K 9/00 (2006.01) *C12N 15/88* (2006.01)
A61K 9/51 (2006.01) *A61K 8/14* (2006.01)
A61K 31/7088 (2006.01) *A61K 36/05* (2006.01)
A61K 48/00 (2006.01) *C12N 1/12* (2006.01)

(21) International Application Number:
PCT/EP2025/054721

(22) International Filing Date:
21 February 2025 (21.02.2025)

(25) Filing Language: English

(26) Publication Language: English

(30) Priority Data:
63/556,325 21 February 2024 (21.02.2024) US
63/562,941 08 March 2024 (08.03.2024) US
63/663,030 21 June 2024 (21.06.2024) US

(71) Applicant: **AGS THERAPEUTICS SAS** [FR/FR]; 10 Rue Greneta, 75003 Paris (FR).

(72) Inventors: **DRITTANTI, Lila**; 89 av. Philippe Auguste, 75011 Paris (FR). **VEGA, Manuel**; 89 av. Philippe Auguste, 75011 Paris (FR).

(74) Agent: **BOULT WADE TENNANT LLP**; Salisbury Square House, 8 Salisbury Square, London Greater London EC4Y 8AP (GB).

(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, CZ, 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, UY, UZ, VC, VN, WS, ZA, ZM, ZW.

WO 2025/176843 A1

(54) Title: MICROALGAE EXTRACELLULAR VESICLE BASED GENE THERAPY VECTORS (MEV-GTVS), THEIR PREPARATION, AND USES THEREOF

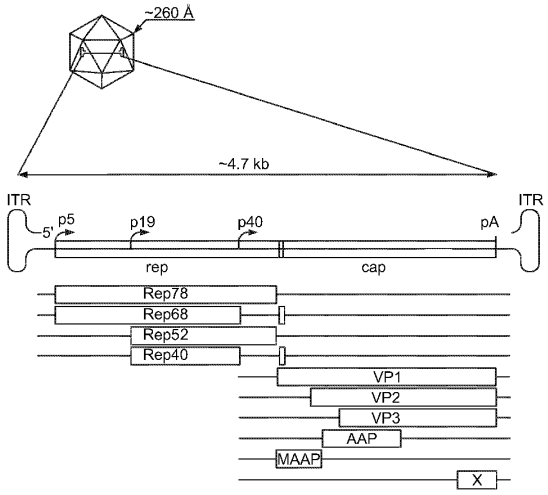


FIG. 1

(57) Abstract: Provided are gene therapy vectors designated MEV-GTVs, which are MEVs that contain an ITR-containing plasmid (also referred to as a minigene plasmid) that comprises Inverted Terminal Repeats (ITRs), such as viral ITRs, and nucleic acid encoding a product of interest, and optionally regulatory sequences. The ITRs serve to circularize the minigene plasmid. The plasmids do not contain additional viral components, so that resulting DNA is not encapsulated in a viral capsid or envelop and is not replicated by viral genes. The plasmids can be inserted into bacterial plasmids for propagation.

[Continued on next page]

(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).

Published:

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