

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

(19) World Intellectual Property
Organization
International Bureau



(10) International Publication Number
WO 2021/198508 A1

(43) International Publication Date
07 October 2021 (07.10.2021)

(51) International Patent Classification:

C12N 5/07 (2010.01) C12N 15/87 (2006.01)

(21) International Application Number:

PCT/EP2021/058794

(22) International Filing Date:

02 April 2021 (02.04.2021)

(25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data:

20167813.3 02 April 2020 (02.04.2020) EP

(71) Applicant: **UNIQUIRE BIOPHARMA B.V.** [NL/NL];
Paasheuvelweg 25, 1105 BP Amsterdam (NL).

(72) Inventors: **DU PLESSIS, David Johannes Francois**; c/o uniQure biopharma B.V., Paasheuvelweg 25, 1105 BP Amsterdam (NL). **ANGGAKUSUMA, -**; c/o uniQure biopharma B.V., Paasheuvelweg 25, 1105 BP Amsterdam (NL). **BOSMA, Sebastiaan Menno**; c/o uniQure biopharma B.V., Paasheuvelweg 25, 1105 BP Amsterdam (NL). **LUBELSKI, Jacek**; c/o uniQure biopharma B.V., Paasheuvelweg 25, 1105 BP Amsterdam (NL).

(74) Agent: **NEDERLANDSCH OCTROOIBUREAU**;
P.O.Box 29720, 2502 LS The Hague (NL).

(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: DUAL BIFUNCTIONAL VECTORS FOR AAV PRODUCTION

(57) Abstract: The present invention relates novel combinations of nucleic acid constructs for the production of recombinant parvoviral gene therapy vectors. In particular the invention relates a combination preferably no more than two construct, the first construct expressing both the parvoviral Cap and Rep proteins, and the second construct at least comprising the transgene flanked ITRs and optionally again comprising an expression cassette for the Cap proteins. The nucleic acid constructs are preferably baculoviral vectors for the production of rAAV in insect cells.



WO 2021/198508 A1