

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

(19) World Intellectual Property
Organization
International Bureau

(43) International Publication Date
25 July 2024 (25.07.2024)



(10) International Publication Number
WO 2024/153769 A1

(51) International Patent Classification:

A61K 35/761 (2015.01) C12N 15/00 (2006.01)
A61K 47/00 (2006.01) C12N 15/86 (2006.01)
A61K 48/00 (2006.01) C12N 15/861 (2006.01)
C12N 15/63 (2006.01)

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))

(21) International Application Number:

PCT/EP2024/051203

(22) International Filing Date:

19 January 2024 (19.01.2024)

(25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data:

23152369.7 19 January 2023 (19.01.2023) EP

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

(72) Inventors: **BERTHIER, Eddy**; c/o uniQure biopharma B.V., Paasheuvelweg 25, 1105 BP Amsterdam (NL). **APETRI, Mihaela**; c/o uniQure biopharma B.V., Paasheuvelweg 25, 1105 BP Amsterdam (NL). **FAIMAN, Gabriel**; c/o uniQure biopharma B.V., Paasheuvelweg 25, 1105 BP Amsterdam (NL). **LJUBOVIC-COUTEAU, Edina**; 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, 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, 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,

(54) Title: PHARMACEUTICAL FORMULATIONS OF GENE DELIVERY VEHICLES

(57) Abstract: 41 P6105182WO ABSTRACT The invention relates to formulations comprising miRNA that have improved stability for the treatment of diseases including neurodegenerative diseases such as spinocerebellar ataxias type 3. 5

WO 2024/153769 A1