

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

(19) World Intellectual Property
Organization
International Bureau

(43) International Publication Date
20 January 2022 (20.01.2022)



(10) International Publication Number
WO 2022/015715 A1

(51) International Patent Classification:
C12N 15/86 (2006.01) C12N 15/11 (2006.01)
A61P 25/00 (2006.01)

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).

(21) International Application Number:
PCT/US2021/041406

Published:

(22) International Filing Date:
13 July 2021 (13.07.2021)

- 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))

(25) Filing Language: English

(26) Publication Language: English

(30) Priority Data:
63/051,336 13 July 2020 (13.07.2020) US
63/173,045 09 April 2021 (09.04.2021) US

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(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,

(54) Title: COMPOSITIONS USEFUL FOR TREATMENT OF CHARCOT-MARIE-TOOTH DISEASE

(57) Abstract: Provided herein are rAAV and other vectors and compositions useful for treating a patient having CMT2 comprising: (a) a recombinant nucleic acid sequence encoding an engineered human mitofusin 2 coding sequence operably linked to regulatory sequences which direct expression thereof in a human target cell. Also provided are rAAV and other vectors and compositions useful for treating a patient having CMT2 comprising: (b) a nucleic acid sequence encoding at least one miRNA specific for an endogenous human mitofusin 2 sequence in a human CMT2A subject, wherein the miRNA coding sequence is operably linked to regulatory sequences which direct expression thereof in the subject. Further provided are compositions containing both the engineered hMfn2 coding sequence and the at least one miRNA coding sequence, wherein the engineered human mitofusin 2 coding sequence has a sequence which differs from endogenous human mitofusin 2 in the CMT2A patient in the target site of the encoded miRNA.



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