

(19) World Intellectual Property
Organization
International Bureau



(43) International Publication Date
18 May 2017 (18.05.2017)

(10) International Publication Number
WO 2017/083776 A9

(51) International Patent Classification:

A61K 48/00 (2006.01) *A61P 39/06* (2006.01)
A61P 21/00 (2006.01) *C07K 14/435* (2006.01)

(74) Agent: SINTICH, Sharon, M.; Marshall, Gerstein & Borun LLP, 233 S. Wacker Drive, 6300 Willis Tower, Chicago, IL 60606-6357 (US).

(21) International Application Number:

PCT/US2016/061703

(22) International Filing Date:

11 November 2016 (11.11.2016)

(25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data:

62/254,539 12 November 2015 (12.11.2015) US
62/419,793 09 November 2016 (09.11.2016) US

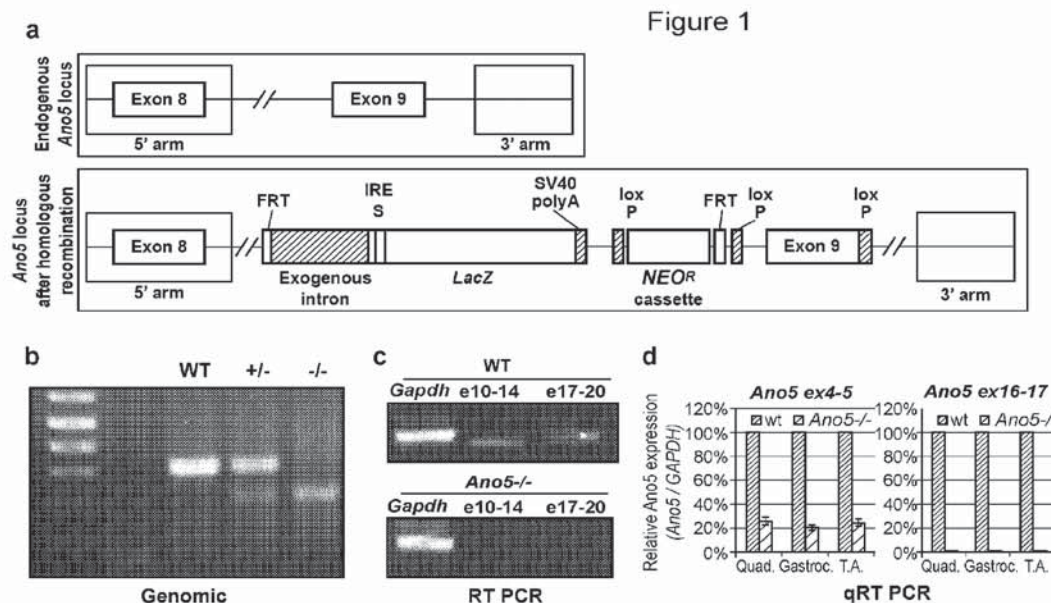
(81) Designated States (unless otherwise indicated, for every kind of national protection available): AE, AG, AL, AM, AO, AT, AU, AZ, BA, BB, BG, BH, BN, BR, BW, BY, BZ, CA, CH, CL, CN, CO, CR, CU, CZ, DE, DJ, DK, DM, DO, DZ, EC, EE, EG, ES, FI, GB, GD, GE, GH, GM, GT, HN, HR, HU, ID, IL, IN, IR, IS, JP, KE, KG, 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, SM, ST, SV, SY, TH, TJ, TM, TN, TR, TT, TZ, UA, UG, US, UZ, VC, VN, ZA, ZM, ZW.

(71) Applicant: RESEARCH INSTITUTE AT NATION-WIDE CHILDREN'S HOSPITAL [US/US]; 700 Children's Drive, Room W 172, Columbus, OH 43205 (US).

(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,

(72) Inventor: RODINO-KLAPAC, Louise; 4912 Bixby Ridge Drive, E. Groveport, OH 43125 (US).

(54) Title: METHODS OF TREATING MUSCULAR DYSTROPHY



(57) Abstract: The invention provides for AAV vectors expressing the ANO5 gene and antioxidant therapy as methods of inducing muscle regeneration and a method of treating muscular dystrophy.

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))*

(48) Date of publication of this corrected version:

31 May 2018 (31.05.2018)

(15) Information about Correction:

see Notice of 31 May 2018 (31.05.2018)