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

(19) World Intellectual Property Organization
International Bureau

(43) International Publication Date
22 October 2020 (22.10.2020)

WIPO | PCT

(10) International Publication Number
WO 2020/214609 A1

(51) International Patent Classification:
C12N 15/52 (2006.01) *C12N 15/85* (2006.01)
A61K 48/00 (2006.01) *C12N 9/22* (2006.01)
C12N 15/11 (2006.01)

(21) International Application Number:
PCT/US2020/028148

(22) International Filing Date:
14 April 2020 (14.04.2020)

(25) Filing Language: English

(26) Publication Language: English

(30) Priority Data:
62/833,760 14 April 2019 (14.04.2019) US

(71) Applicant: **DUKE UNIVERSITY** [US/US]; 2812 Erwin Road, Suite 306, Durham, NC 27705 (US).

(72) Inventors: **GERSBACH, Charles A.**; 3220 Wilderness Road, Durham, NC 27712 (US). **BULAKLAK, Karen**; c/o Duke University, 2812 Erwin Road, Suite 306, Durham, NC 27705 (US). **ROBINSON-HAMM, Jacqueline N.**; c/o Duke University, 2812 Erwin Road, Suite 306, Durham, NC 27705 (US).

(74) Agent: **COX, Julia M.** et al.; Michael Best & Friedrich LLP, 100 East Wisconsin Avenue, Suite 3300, Milwaukee, WI 53202-4108 (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, 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: AAV VECTOR-MEDIATED DELETION OF LARGE MUTATIONAL HOTSPOT FOR TREATMENT OF DUCHENNE MUSCULAR DYSTROPHY

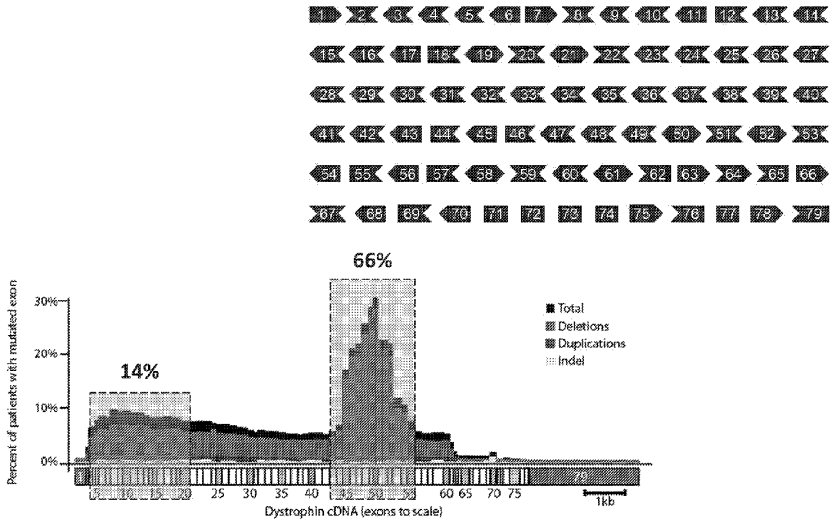


FIG. 1

(57) Abstract: Disclosed herein are therapeutic targets for the correction of the human dystrophin gene by gene editing and methods of use.

[Continued on next page]

WO 2020/214609 A1

Published:
— *with international search report (Art. 21(3))*