

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

(19) World Intellectual Property
Organization

International Bureau

(43) International Publication Date
03 September 2020 (03.09.2020)



(10) International Publication Number
WO 2020/176614 A1

(51) International Patent Classification:

A61K 48/00 (2006.01) C07K 14/47 (2006.01)

(21) International Application Number:

PCT/US2020/019892

(22) International Filing Date:

26 February 2020 (26.02.2020)

(25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data:

62/810,917	26 February 2019 (26.02.2019)	US
62/834,012	15 April 2019 (15.04.2019)	US
62/858,644	07 June 2019 (07.06.2019)	US
62/881,901	01 August 2019 (01.08.2019)	US
62/909,564	02 October 2019 (02.10.2019)	US
62/910,779	04 October 2019 (04.10.2019)	US

(71) Applicant: **RESEARCH INSTITUTE AT NATION-WIDE CHILDREN'S HOSPITAL** [US/US]; 700 Children's Drive, Room W172, Columbus, OH 43205 (US).

(72) Inventors: **RODINO-KLAPAC, Louise**; 4912 Bixby Ridge Drive, E. Groveport, OH 43125 (US). **MENDELL, Jerry R.**; 8176 Copperfield Drive, Columbus, OH 43235 (US).

(74) Agent: **SINTICH, Sharon M.**; Marshall, Gerstein & Borun LLP, 233 S. Wacker Drive, 6300 Willis Tower, Chicago, IL 60606-6357 (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: ADENO-ASSOCIATED VIRUS VECTOR DELIVERY OF B-SARCOGLYCAN AND THE TREATMENT OF MUSCULAR DYSTROPHY

(57) Abstract: Described herein are methods of treating muscular dystrophy comprising administering a recombinant AAV (rAAV) scAAVrh74.MHCK7.hSGCB vector, methods of expressing beta-sarcoglycan gene in a patient, pharmaceutical compositions comprising the rAAV, and methods of generating the rAAV.

WO 2020/176614 A1