

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

(19) World Intellectual Property
Organization
International Bureau

(43) International Publication Date
08 August 2019 (08.08.2019)



(10) International Publication Number
WO 2019/152474 A1

(51) International Patent Classification:

A61K 35/76 (2015.01) A61M 1/36 (2006.01)
A61K 38/17 (2006.01) A61M 5/14 (2006.01)
A61K 48/00 (2006.01)

(21) International Application Number:

PCT/US2019/015779

(22) International Filing Date:

30 January 2019 (30.01.2019)

(25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data:

62/624,616 31 January 2018 (31.01.2018) US

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

(72) Inventor: **RODINO-KLAPAC, Louise R.**; c/o Research
Institute at Nationwide Children's Hospital, 700 Children's
Drive, Columbus, Ohio 43205 (US).

(74) Agent: **KONSKI, Antoinette F.** et al.; Foley & Lardner
LLP, 3000 K Street NW, Suite 600, Washington, District of
Columbia 20007 (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, SM, ST, SV, SY, TH, TJ, TM, TN,
TR, TT, TZ, UA, UG, US, UZ, VC, VN, 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).

Declarations under Rule 4.17:

- as to applicant's entitlement to apply for and be granted a
patent (Rule 4.17(ii))
- as to the applicant's entitlement to claim the priority of the
earlier application (Rule 4.17(iii))

Published:

- with international search report (Art. 21(3))
- with sequence listing part of description (Rule 5.2(a))

(54) Title: GENE THERAPY FOR LIMB-GIRDLE MUSCULAR DYSTROPHY TYPE 2C

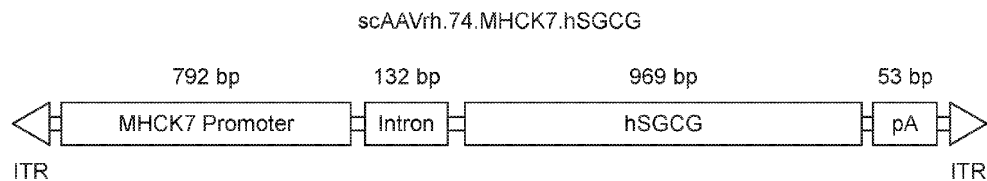


FIG. 1

(57) Abstract: The disclosure relates to gene therapy vectors, such as AAV vectors, comprising a polynucleotide encoding γ -sarcogly-
can (SGCG) and methods of using such gene therapy vectors to treat subjects suffering from a muscular dystrophy, e.g. limb girdle
dystrophy type 2C (LGMD2C).

WO 2019/152474 A1