

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

(19) World Intellectual Property
Organization
International Bureau

(43) International Publication Date
17 March 2022 (17.03.2022)



(10) International Publication Number
WO 2022/055791 A1

(51) International Patent Classification:

A61P 21/00 (2006.01) C12N 15/86 (2006.01)
C07K 14/47 (2006.01)

(21) International Application Number:

PCT/US2021/048957

(22) International Filing Date:

03 September 2021 (03.09.2021)

(25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data:

63/075,697 08 September 2020 (08.09.2020) US

(71) Applicant: **SAREPTA THERAPEUTICS, INC.**
[US/US]; 215 First Street, Cambridge, MA 02142 (US).

(72) Inventors: **RODINO-KLAPAC, Louise**; 215 First Street,
Cambridge, MA 02142 (US). **POZSGAI, Eric**; 215 First
Street, Cambridge, MA 02142 (US).

(74) Agent: **VERNI, Christopher** et al.; Sarepta Therapeutics,
Inc., 215 First Street, Cambridge, MA 02142 (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, 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.

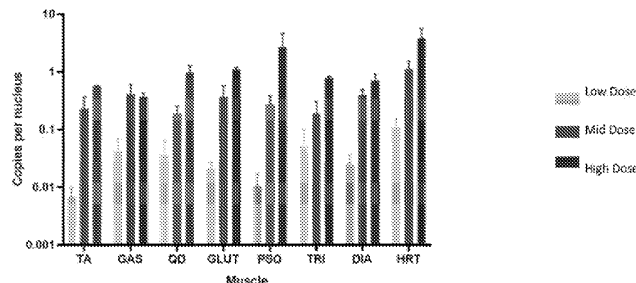
(84) Designated States (unless otherwise indicated, for every
kind of regional protection available): ARIPO (BW, GH,
GM, KE, LR, LS, MW, MZ, NA, RW, SD, SL, ST, SZ, TZ,
UG, ZM, ZW), Eurasian (AM, AZ, BY, KG, KZ, RU, TJ,
TM), European (AL, AT, BE, BG, CH, CY, CZ, DE, DK,
EE, ES, FI, FR, GB, GR, HR, HU, 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).

Published:

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

(54) Title: SYSTEMIC DELIVERY OF ADENO-ASSOCIATED VIRUS VECTOR EXPRESSING G-SARCOGLYCAN AND THE TREATMENT OF MUSCULAR DYSTROPHY

Fig. 1



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

WO 2022/055791 A1