



(51) International Patent Classification:

A61K 48/00 (2006.01) C07K 16/00 (2006.01)
C07K 14/005 (2006.01) C12N 15/86 (2006.01)

(21) International Application Number:

PCT/US2022/079339

(22) International Filing Date:

04 November 2022 (04.11.2022)

(25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data:

63/275,731 04 November 2021 (04.11.2021) US
63/369,761 28 July 2022 (28.07.2022) US

(71) Applicant: **REGENERON PHARMACEUTICALS, INC.** [US/US]; 777 Old Saw Mill River Road, Tarrytown, New York 10591 (US).

(72) Inventors: **SABIN, Leah**; c/o Regeneron Pharmaceuticals, Inc., 777 Old Saw Mill River Road, Tarrytown, New York 10591 (US). **STEC, Michael**; c/o Regeneron Pharmaceuticals, Inc., 777 Old Saw Mill River Road, Tarrytown, New York 10591 (US). **MURPHY, Andrew J.**; c/o Regeneron

Pharmaceuticals, Inc., 777 Old Saw Mill River Road, Tarrytown, New York 10591 (US). **KYRATSOUS, Christos**; c/o Regeneron Pharmaceuticals, Inc., 777 Old Saw Mill River Road, Tarrytown, New York 10591 (US). **MOLLER-TANK, Sven**; c/o Regeneron Pharmaceuticals, Inc., 777 Old Saw Mill River Road, Tarrytown, New York 10591 (US). **SAMAI, Poulami**; c/o Regeneron Pharmaceuticals, Inc., 777 Old Saw Mill River Road, Tarrytown, New York 10591 (US).

(74) Agent: **WU, Rita S.**; FisherBroyles/Regeneron/RW, 79 Willowleaf Drive, Littleton, Colorado 80127 (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, CV, CZ, DE, DJ, DK, DM, DO, DZ, EC, EE, EG, ES, FI, GB, GD, GE, GH, GM, GT, HN, HR, HU, ID, IL, IN, IQ, IR, IS, IT, JM, JO, JP, KE, KG, KH, KN, KP, KR, KW, KZ, LA, LC, LK, LR, LS, LU, LY, MA, MD, 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,

(54) Title: VIRAL PARTICLES RETARGETED TO SKELETAL MUSCLE

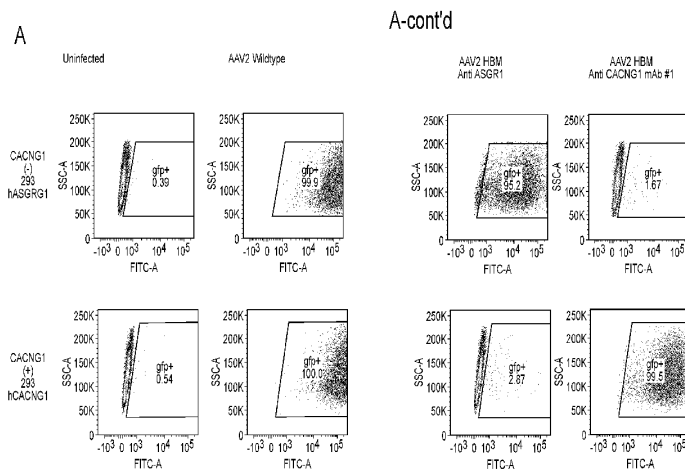


Figure 1

Figure 1 (cont'd)

Figure 1 (cont'd)

(57) Abstract: Provided herein are compositions and methods for retargeting viral particles, e.g. adeno-associated virus (AAV) particles, to muscle cells using muscle-specific surface proteins. AAV adapted accordingly may be a viable gene therapy platform for the treatment of a skeletal muscle related disorder (e.g., X-linked myotubular myopathy (XLMTM), Duchenne muscular dystrophy (DMD), myotonic dystrophy (DM1), Facioscapulohumeral muscular dystrophy Type 1 (FSHD), congenital muscular dystrophy type 1A (MDC1A), Limb girdle muscular dystrophy, dystroglycanopathy, etc.) in a patient in need thereof.

[Continued on next page]

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, CV, 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, ME, 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))*
- *before the expiration of the time limit for amending the claims and to be republished in the event of receipt of amendments (Rule 48.2(h))*
- *with sequence listing part of description (Rule 5.2(a))*