



(51) International Patent Classification:

A61K 31/713 (2006.01) *C07K 16/28* (2006.01)
A61P 21/00 (2006.01) *A61P 9/00* (2006.01)
A61K 47/68 (2017.01) *C12N 15/113* (2010.01)

(21) International Application Number:

PCT/US2022/073541

(22) International Filing Date:

08 July 2022 (08.07.2022)

(25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data:

63/220,016 09 July 2021 (09.07.2021) US
 63/316,466 04 March 2022 (04.03.2022) US

(71) Applicant: **DYNE THERAPEUTICS, INC.** [US/US];
 1560 Trapelo Road, Waltham, MA 02451 (US).

(72) Inventors: **DESJARDINS, Cody, A.**; 1560 Trapelo Road,
 Waltham, MA 02451 (US). **TANG, Kim**; 1560 Trapelo
 Road, Waltham, MA 02451 (US). **MCSWIGGEN, James**;
 19395 W. 94th Avenue, Arvada, CO 80007 (US). **SUBRA-
 MANIAN, Romesh, R.**; 9 Alan Street, Framingham, MA
 01701 (US). **WEEDEN, Timothy**; 1560 Trapelo Road,
 Waltham, MA 02451 (US). **QATANANI, Mohammed, T.**;
 1560 Trapelo Road, Waltham, MA 02451 (US). **QUINN,
 Brendan**; 45 East Newton Street #320, Boston, MA 02118
 (US). **NAJIM, John**; 1560 Trapelo Road, Waltham, MA
 02451 (US).

(74) Agent: **BUSS, Colin, G.** et al.; WOLF, GREENFIELD
 & SACKS, P.C., 600 Atlantic Avenue, Boston, MA
 02210-2206 (US).

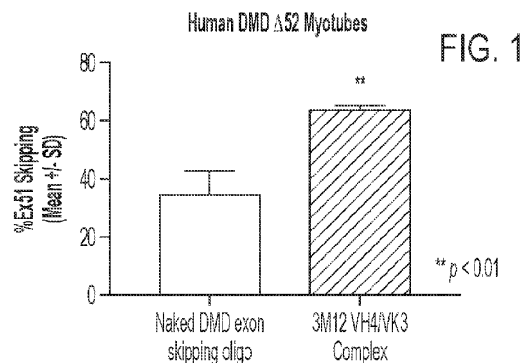
(81) Designated States (*unless otherwise indicated, for every
 kind of national protection available*): AE, AG, AL, AM,
 AO, AT, AU, AZ, BA, BB, BG, BH, BN, BR, BW, BY, BZ,
 CA, CH, CL, CN, CO, CR, CU, CV, CZ, DE, DJ, DK, DM,
 DO, DZ, EC, EE, EG, ES, FI, GB, GD, GE, GH, GM, GT,
 HN, HR, HU, ID, IL, 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, 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: MUSCLE TARGETING COMPLEXES AND USES THEREOF FOR TREATING DYSTROPHINOPATHIES



(57) Abstract: Aspects of the disclosure relate to complexes comprising a muscle-targeting agent covalently linked to a molecular payload. In some embodiments, the muscle-targeting agent specifically binds to an internalizing cell surface receptor on muscle cells. In some embodiments, the molecular payload promotes the expression or activity of a functional dystrophin protein. In some embodiments, the molecular payload is an oligonucleotide, such as an antisense oligonucleotide, e.g., an oligonucleotide that causes exon skipping in a mRNA expressed from a mutant DMD allele.

[Continued on next page]

(88) Date of publication of the international search report:
16 February 2023 (16.02.2023)