

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

(19) World Intellectual Property
Organization
International Bureau

(43) International Publication Date
09 March 2023 (09.03.2023)



(10) International Publication Number
WO 2023/034818 A4

(51) International Patent Classification:

A61K 47/64 (2017.01) C12N 15/113 (2010.01)
C07K 7/64 (2006.01) C12N 15/11 (2006.01)

(21) International Application Number:

PCT/US2022/075693

(22) International Filing Date:

30 August 2022 (30.08.2022)

(25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data:

63/239,671	01 September 2021 (01.09.2021)	US
63/244,915	16 September 2021 (16.09.2021)	US
63/290,960	17 December 2021 (17.12.2021)	US
63/298,565	11 January 2022 (11.01.2022)	US
63/268,577	25 February 2022 (25.02.2022)	US
63/337,574	02 May 2022 (02.05.2022)	US
63/354,454	22 June 2022 (22.06.2022)	US

(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 amended claims (Art. 19(1))

Date of publication of the amended claims:

19 May 2023 (19.05.2023)

(71) Applicant: ENTRADA THERAPEUTICS, INC.
[US/US]; 6 Tide Street, Boston, Massachusetts 02210 (US).

(72) Inventors: LI, Xiang; 6 Tide Street, Boston, Massachusetts 02210 (US). QIAN, Ziqing; 6 Tide Street, Boston, Massachusetts 02210 (US). KHEIRABADI, Mahboubeh; 6 Tide Street, Boston, Massachusetts 02210 (US). ZHOU, Ming; 6 Tide Street, Boston, Massachusetts 02210 (US). GIRGEN-RATH, Mahsweta; 6 Tide Street, Boston, Massachusetts 02210 (US). ESTRELLA, Nelsa; 6 Tide Street, Boston, Massachusetts 02210 (US). PEDDIGARI, Suresh; 6 Tide Street, Boston, Massachusetts 02210 (US). PATHAK, Anushree; 6 Tide Street, Boston, Massachusetts 02210 (US). SETHURAMAN, Natarajan; 6 Tide Street, Boston, Massachusetts 02210 (US). LIAN, Wenlong; 6 Tide Street, Boston, Massachusetts 02210 (US). LIU, Nanjun; 6 Tide Street, Boston, Massachusetts 02210 (US).

(74) Agent: PERDOK, Monique M. et al.; P.O. Box 2938, Minneapolis, Minnesota 55402 (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.

(54) Title: COMPOSITIONS AND METHODS FOR SKIPPING EXON 45 IN DUCHENNE MUSCULAR DYSTROPHY

(57) Abstract: Described herein in various embodiments are compositions comprising (a) a cell penetrating peptide (CPP) sequence; and (b) an antisense compound, wherein the antisense compound targets exon 45 of the DMD gene.

WO 2023/034818 A4