

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

(19) World Intellectual Property
Organization
International Bureau

(43) International Publication Date
16 February 2023 (16.02.2023)



(10) International Publication Number
WO 2023/018854 A3

(51) International Patent Classification:

C12N 15/86 (2006.01) C07K 14/435 (2006.01)

(21) International Application Number:

PCT/US2022/040030

(22) International Filing Date:

11 August 2022 (11.08.2022)

(25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data:

63/231,720 11 August 2021 (11.08.2021) US

(71) Applicant: **SOLID BIOSCIENCES INC.** [US/US]; 500 Rutherford Ave, Third Floor, Charlestown, MA 02129 (US).

(72) Inventors: **BROWN, Kristy, Jean**; 15104 Georgia Avenue, Rockville, MD 20853 (US). **GREEN, Jennifer**; 141 Portland Street, 5th Floor, Cambridge, MA 02139 (US).

(74) Agent: **LU, Yu et al.**; McCarter & English, LLP, 265 Franklin Street, Boston, MA 02110 (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).

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))

(54) Title: TREATMENT OF MUSCULAR DYSTROPHY

(57) Abstract: The invention described herein provides a microdystrophin-encoding, codon optimized polynucleotide with reduced CpG island, and use thereof in the treatment of muscular dystrophy such as DMD / BMD.

WO 2023/018854 A3