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

(19) World Intellectual Property Organization
International Bureau

(43) International Publication Date
28 December 2017 (28.12.2017)

WIPO | PCT

(10) International Publication Number
WO 2017/221145 A1

(51) International Patent Classification:
C07K 14/47 (2006.01)

(21) International Application Number:
PCT/IB2017/053656

(22) International Filing Date:
20 June 2017 (20.06.2017)

(25) Filing Language: English

(26) Publication Language: English

(30) Priority Data:

62/352,675	21 June 2016 (21.06.2016)	US
62/516,449	07 June 2017 (07.06.2017)	US

(71) Applicants: **BAMBOO THERAPEUTICS, INC.** [US/US]; 6101 Quadrangle Drive, Suite 200, Chapel Hill, NC 27517 (US). **THE UNIVERSITY OF NORTH CAROLINA AT CHAPEL HILL** [US/US]; 100 Europa Drive, Suite 430, Chapel Hill, NC 27517 (US).

(72) Inventors: **MCPHEE, Scott W.J.**; 1313 Arboretum Drive, Chapel Hill, NC 27517 (US). **QIAO, Chunping**; 119 Woodshire Lane, Chapel Hill, NC 27514 (US). **LI, Juan**; 4205 Taylor Hall Place, Chapel Hill, NC 27517 (US). **XIAO, Xiao**; 4205 Taylor Hall Place, Chapel Hill, NC 27517 (US). **MCINTYRE, Maritza**; 13512 Oriental Street, Rockville, MD 20853 (US). **SAMULSKI, Richard J.**; 201 Southwind Lane, Hillsborough, NC 27278 (US).

(74) Agent: **WALDRON, Roy F.** et al.; Pfizer INC., 235 East 42nd Street, MS 235/9/S20, New York, NY 10017 (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, 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, SM, ST, SV, SY, TH, TJ, TM, TN, TR, TT, TZ, UA, UG, US, UZ, VC, VN, 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))*

WO 2017/221145 A1

(54) Title: OPTIMIZED MINI-DYSTROPHIN GENES AND EXPRESSION CASSETTES AND THEIR USE

(57) Abstract: This invention relates to polynucleotides encoding mini-dystrophin proteins, viral vectors comprising the same, and methods of using the same for delivery of mini-dystrophin to a cell or a subject.