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

CORRECTED VERSION

(19) World Intellectual Property Organization
International Bureau

(43) International Publication Date
11 September 2025 (11.09.2025)

WIPO | PCT

(10) International Publication Number
WO 2025/188755 A8

(51) International Patent Classification:
C12N 15/86 (2006.01) *C12N 15/11* (2006.01)
C07K 14/47 (2006.01)

(21) International Application Number:
PCT/US2025/018343

(22) International Filing Date:
04 March 2025 (04.03.2025)

(25) Filing Language: English

(26) Publication Language: English

(30) Priority Data:

65/561,130	04 March 2024 (04.03.2024)	US
63/666,899	02 July 2024 (02.07.2024)	US
63/676,780	29 July 2024 (29.07.2024)	US
63/688,024	28 August 2024 (28.08.2024)	US

(71) Applicant: **KATE THERAPEUTICS, INC.** [US/US]; 10675 Sorrento Valley Road, Suite 150, San Diego, California 92121 (US).

(72) Inventors: **MONAVARFESHANI, Aboozar**; KATE THERAPEUTICS, INC., 10675 Sorrento Valley Road, Suite 150, San Diego, California 92121 (US). **SESHADRI, Saurav**; KATE THERAPEUTICS, INC., 10675 Sorrento Valley Road, Suite 150, San Diego, California 92121 (US). **TABEBORDBAR, Mohammadsharif**; KATE THERAPEUTICS, INC., 10675 Sorrento Valley Road, Suite 150, San Diego, California 92121 (US). **TABEBORDBAR, Shayan**; KATE THERAPEUTICS, INC., 10675 Sorrento Valley Road, Suite 150, San Diego, California 92121 (US). **VELEZ, Danielle**; KATE THERAPEUTICS, INC., 10675 Sorrento Valley Road, Suite 150, San Diego, California 92121 (US). **YE, Simon**; KATE THERAPEUTICS, INC., 10675 Sorrento Valley Road, Suite 150, San Diego, California 92121 (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, MU, 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, UY, 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, SC, 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 non-prejudicial disclosures or exceptions to lack of novelty (Rule 4.17(v))*

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))*

(48) Date of publication of this corrected version:
02 October 2025 (02.10.2025)

(15) Information about Correction:
see Notice of 02 October 2025 (02.10.2025)

(54) Title: ADENO-ASSOCIATED VIRUS COMPOSITIONS FOR THE TREATMENT OF DUCHENNE MUSCULAR DYSTROPHY

(57) Abstract: The present invention provides novel gene therapy compositions for use in treatment of Duchenne Muscular Dystrophy (DMD).

WO 2025/188755 A8