

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

(19) World Intellectual Property
Organization
International Bureau

(43) International Publication Date
10 October 2024 (10.10.2024)



(10) International Publication Number
WO 2024/211416 A2

- (51) **International Patent Classification:**
C12N 15/861 (2006.01) *A61K 31/711* (2006.01)
- (21) **International Application Number:**
PCT/US2024/022843
- (22) **International Filing Date:**
03 April 2024 (03.04.2024)
- (25) **Filing Language:** English
- (26) **Publication Language:** English
- (30) **Priority Data:**
63/494,925 07 April 2023 (07.04.2023) US
63/586,227 28 September 2023 (28.09.2023) US
- (71) **Applicant: 4D MOLECULAR THERAPEUTICS INC.**
[US/US]; 5858 Horton Street, Suite 455, Emeryville, California 94608 (US).
- (72) **Inventors: CALTON, Melissa;** c/o 4D Molecular Therapeutics Inc., 5858 Horton Street, Suite 455, Emeryville, 94608 (US). **CROZE, Roxanne;** c/o 4D Molecular Therapeutics Inc., 5858 Horton Street, Suite 455, Emeryville, 94608 (US). **KANTARDZHIEVA, Albena;** 5980 horton st. suite 455, Emeryville, 94608 (US).
- (74) **Agent: CABRAL, Christopher M.;** Much Shelist PC, 191 N. WACKER DR. STE 1800, CHICAGO, IL 60606 (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, 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:

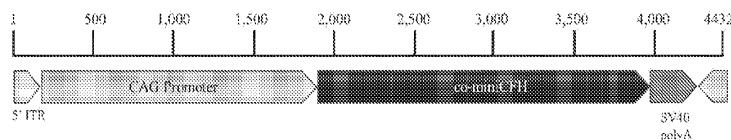
- *as to applicant's entitlement to apply for and be granted a patent (Rule 4.17(ii))*
- *as to the applicant's entitlement to claim the priority of the earlier application (Rule 4.17(iii))*

Published:

- *without international search report and to be republished upon receipt of that report (Rule 48.2(g))*
- *with sequence listing part of description (Rule 5.2(a))*

(54) **Title:** AAV VARIANTS FOR TREATMENT OF COMPLEMENT DISORDERS

FIG. 1



(57) **Abstract:** Recombinant AAV (rAAV) comprising a variant adeno-associated virus (AAV) capsid and a transgene encoding a human factor H variant are provided. Also provided are methods of delivering the transgene to the retina and methods of treating dry age-related macular degeneration and geographic atrophy secondary to age-related macular degeneration disorders by contacting retinal cells with the rAAV.

WO 2024/211416 A2