

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

(19) World Intellectual Property
Organization

International Bureau

(43) International Publication Date
23 September 2021 (23.09.2021)



(10) International Publication Number
WO 2021/188449 A1

(51) International Patent Classification:

C12N 7/00 (2006.01) C12N 5/00 (2006.01)

(21) International Application Number:

PCT/US2021/022396

(22) International Filing Date:

15 March 2021 (15.03.2021)

(25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data:

62/990,099 16 March 2020 (16.03.2020) US

(71) Applicant: **ULTRAGENYX PHARMACEUTICAL INC.** [US/US]; 60 Leveroni Court, Novato, CA 94949 (US).

(72) Inventors: **PANTELI, Jan, Thomas**; 100 Claremont Ave., Arlington, MA 02476 (US). **FULCO, Cameron, Tage**; 12 Slocumb Pl, Medway, MA 02053 (US). **ALLEN, John, Everett**; 10 Hemlock Rd, Reading, MA 01867 (US). **JIANG, Mingyang**; 275 Winchester Ave., Unit 204, New Haven, CT 06511 (US). **HOGAN, Rachael, C.**; 1 Bowdoin St., Apt. #319, Everett, MA 02149 (US).

(74) Agent: **NGUYEN, Suzanne, P.** et al.; Goodwin Procter LLP, 100 Northern Avenue, IP Docket Dept./7th Floor, Boston, MA 02210 (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, IT, 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).

(54) Title: METHODS FOR ENHANCING RECOMBINANT ADENO-ASSOCIATED VIRUS YIELD

(57) Abstract: The invention provides methods for the production of recombinant adeno-associated virus vectors (rAAV), comprising contacting a host cell with a solution comprising at least one compound of formula (I), (I-A), (I-B), (II), (III), or (IV), or a salt thereof, or a vitamin B, or any combination(s) thereof. Also provided are methods for increasing the production of rAAV by a host cell, comprising contacting a host cell with a solution comprising at least one compound of formula (I), (I-A), (I-B), (II), (III), or (IV), or a salt thereof, or a vitamin B, or any combination(s) thereof.

WO 2021/188449 A1