

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

(19) World Intellectual Property
Organization

International Bureau

(43) International Publication Date
01 February 2018 (01.02.2018)



(10) International Publication Number
WO 2018/022608 A3

(51) International Patent Classification:

C07K 14/005 (2006.01) *A61K 48/00* (2006.01)
C12N 15/86 (2006.01)

(21) International Application Number:

PCT/US2017/043703

(22) International Filing Date:

25 July 2017 (25.07.2017)

(25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data:

62/366,838 26 July 2016 (26.07.2016) US

(71) Applicant: **BIOMARIN PHARMACEUTICAL INC.**
[US/US]; 105 Digital Drive, Novato, CA 94949 (US).

(72) Inventors: **COLOSI, Peter, Cameron**; 105 Digital Drive,
Novato, CA 94949 (US). **LOCHRIE, Michael**; 105 Digital
Drive, Novato, CA 94949 (US). **NG, Robert**; 105 Digital
Drive, Novato, CA 94949 (US).

(74) Agent: **SINTICH, Sharon, M.**; Marshall, Gerstein &
Borun LLP, 233 S. Wacker Drive, 6300 Willis Tower,
Chicago, IL 60606-6357 (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))
- 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))

(88) Date of publication of the international search report:

01 March 2018 (01.03.2018)



WO 2018/022608 A3

(54) Title: NOVEL ADENO-ASSOCIATED VIRUS CAPSID PROTEINS

(57) Abstract: The invention relates to novel adeno-associated virus (AAV) capsid proteins, AAV particles comprising a novel capsid protein, polynucleotides encoding these capsid proteins and AAV vectors expressing these capsid proteins. The invention also relates to methods of making the herein described AAV vectors expressing the novel capsid proteins of the invention and associated therapeutic uses of thereof.