

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

(19) World Intellectual Property
Organization
International Bureau

(43) International Publication Date
04 November 2021 (04.11.2021)



(10) International Publication Number
WO 2021/221995 A1

(51) International Patent Classification:

A61K 35/761 (2015.01) *C07K 14/075* (2006.01)
A61K 38/00 (2006.01) *C12N 15/12* (2006.01)
A61K 48/00 (2006.01) *C12N 15/86* (2006.01)

(21) International Application Number:

PCT/US2021/028658

(22) International Filing Date:

22 April 2021 (22.04.2021)

(25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data:

63/016,507 28 April 2020 (28.04.2020) US
63/077,144 11 September 2020 (11.09.2020) US

(71) Applicant: **PFIZER INC.** [US/US]; 235 East 42nd Street,
New York, NY 10017 (US).

(72) Inventors: **LEONE, Paola**; c/o Rowan University, 2 Medical Center Drive, Stratford, NJ 08084 (US). **FRANCIS, Jeremy**; c/o Rowan University, 2 Medical Center Drive, Stratford, NJ 08084 (US). **ASSAF, Basel, Tariq**; c/o Pfizer Inc., 610 Main Street, Cambridge, MA 02139 (US). **ASANO,**

Shoh; c/o Pfizer Inc., 610 Main Street, Cambridge, MA 02139 (US). **HALES, Kathrine**; c/o Pfizer Inc., 610 Main Street, Cambridge, MA 02139 (US). **BERG, Allison, P.**; c/o Pfizer Inc., 610 Main Street, Cambridge, MA 02139 (US).

(74) Agent: **HAO, Jianming** et al.; Fox Rothschild LLP, 997 Lenox Drive, Lawrenceville, NJ 08648-2311 (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,

(54) Title: MODIFIED NUCLEIC ACIDS ENCODING ASPARTOACYLASE (ASPA) AND VECTOR FOR GENE THERAPY

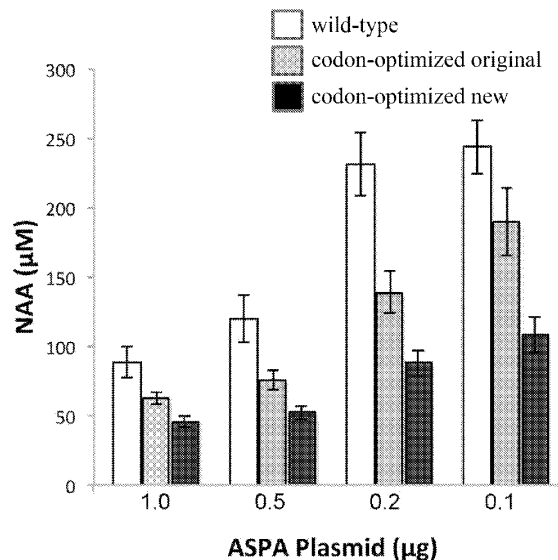


FIG. 1

(57) Abstract: The present disclosure relates to recombinant nucleic acids and gene therapy vectors comprising a modified nucleic acid encoding aspartoacylase (ASPA), and variants thereof, for use in the treatment of diseases and disorders associated with a deficiency or dysfunction of ASPA, and in particular, Canavan disease.

[Continued on next page]

WO 2021/221995 A1

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))*