



(51) International Patent Classification:

A61K 38/46 (2006.01) *A61P 21/00* (2006.01)
A61P 3/00 (2006.01) *C07K 19/00* (2006.01)
A61P 9/00 (2006.01)

(21) International Application Number:

PCT/US2021/032451

(22) International Filing Date:

14 May 2021 (14.05.2021)

(25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data:

63/024,941 14 May 2020 (14.05.2020) US
 63/109,677 04 November 2020 (04.11.2020) US
 63/180,379 27 April 2021 (27.04.2021) US

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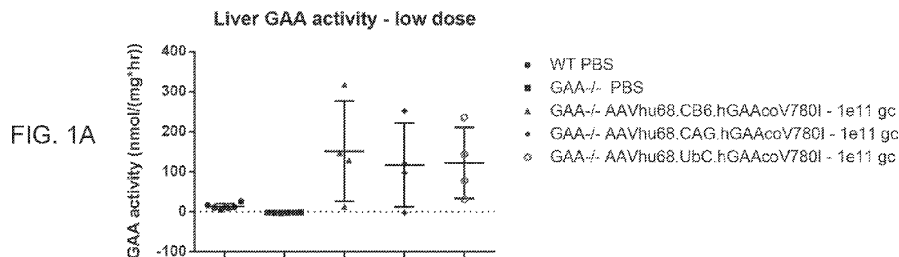
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(81) Designated States (unless otherwise indicated, for every kind of national protection available): AE, AG, AL, AM, AO, AT, AU, AZ, BA, BB, BG, BH, BN, BR, BW, BY, BZ, CA, CH, CL, CN, CO, CR, CU, CZ, DE, DJ, DK, DM, DO, DZ, EC, EE, EG, ES, FI, GB, 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,

(54) Title: COMPOSITIONS USEFUL FOR TREATMENT OF POMPE DISEASE



(57) Abstract: Provided herein is a method for reducing the progression of abnormal muscle pathology and/or reversing abnormal muscle pathology in a patient, wherein the patient has been diagnosed with Pompe disease or is suspected of having Pompe disease. The method comprising administering to the patient a recombinant AAV (rAAV) having an AAV capsid and a vector genome packaged therein, wherein the vector genome comprises: (a) a 5' inverted terminal repeat (ITR); (b) a promoter; (c) a nucleotide sequence encoding a chimeric fusion protein comprising a signal peptide and a vIGF2 peptide fused to a human acid- α -glucosidase (hGAA), (d) a poly A; and (e) a 3' ITR. Also provided are pharmaceutical composition comprising an rAAV described herein for use in treating a patient having or suspected of having Pompe disease.

[Continued on next page]

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))*