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

(19) World Intellectual Property Organization
International Bureau

(43) International Publication Date
08 November 2018 (08.11.2018)

WIPO | PCT

(10) International Publication Number
WO 2018/204786 A1

(51) International Patent Classification:
A61K 31/713 (2006.01) *C12N 15/11* (2006.01)
A61K 48/00 (2006.01) *C12N 15/113* (2010.01)
A61P 25/28 (2006.01) *C12N 15/86* (2006.01)

(21) International Application Number:
PCT/US2018/031089

(22) International Filing Date:
04 May 2018 (04.05.2018)

(25) Filing Language: English

(26) Publication Language: English

(30) Priority Data:

62/501,788	05 May 2017 (05.05.2017)	US
62/507,927	18 May 2017 (18.05.2017)	US
62/520,100	15 June 2017 (15.06.2017)	US
62/566,609	02 October 2017 (02.10.2017)	US

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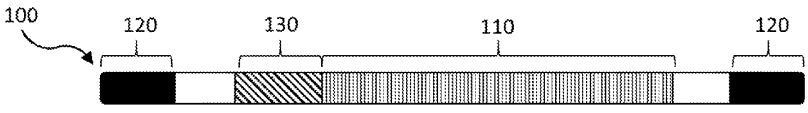
(81) Designated States *(unless otherwise indicated, for every kind of national protection available)*: AE, AG, AL, AM, AO, AT, AU, AZ, BA, BB, BG, BH, BN, BR, BW, BY, BZ, CA, CH, CL, CN, CO, CR, CU, CZ, DE, DJ, DK, DM, DO, DZ, EC, EE, EG, ES, FI, GB, GD, GE, GH, GM, GT, HN, HR, HU, ID, IL, IN, IR, IS, 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))*

(54) Title: COMPOSITIONS AND METHODS OF TREATING AMYOTROPHIC LATERAL SCLEROSIS (ALS)

FIG. 1



(57) Abstract: The present invention relates to adeno-associated viral (AAV) particles encoding siRNA molecules and methods for treating amyotrophic lateral sclerosis (ALS). The present invention relates to compositions, methods and processes for the design, preparation, manufacture, use and/or formulation of AAV particles comprising modulatory polynucleotides, e.g., polynucleotides encoding at least one small interfering RNA (siRNA) molecules which target the superoxide dismutase 1 (SOD1) gene. Methods for using the AAV particles to inhibit the expression of the SOD1 gene in a subject with a neurodegenerative disease (e.g., amyotrophic lateral sclerosis (ALS)) are also disclosed.

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