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

(19) World Intellectual Property Organization
International Bureau

(43) International Publication Date
19 May 2016 (19.05.2016)

WIPO | PCT

(10) International Publication Number
WO 2016/077687 A1

(51) International Patent Classification:
C12N 15/11 (2006.01) *C12N 15/85* (2006.01)
C12N 15/113 (2010.01)

(21) International Application Number:
PCT/US2015/060562

(22) International Filing Date:
13 November 2015 (13.11.2015)

(25) Filing Language: English

(26) Publication Language: English

(30) Priority Data:
62/079,588 14 November 2014 (14.11.2014) US
62/211,992 31 August 2015 (31.08.2015) US
62/234,466 29 September 2015 (29.09.2015) US

(71) Applicant: **VOYAGER THERAPEUTICS, INC.** [US/US]; 75 Sidney Street, Cambridge, Massachusetts 02139 (US).

(72) Inventors: **SAH, Dinah Wen-Yee**; 15 Huckleberry Road, Hopkinton, Massachusetts 01748 (US). **HOU, Jinzhao**; 49 Hill Road, Apt 55, Belmont, Massachusetts 02478 (US). **NONNENMACHER, Mathieu E.**; 98 Queensberry Street, Apt. 10, Boston, Massachusetts 02215 (US). **ZHOU, Pengcheng**; 17 Banks Ave., Lexington, Massachusetts 02421 (US). **HOSSBACH, Markus**; Weiherer Str. 30, 95326 Kulmbach (DE). **DECKERT, Jochen**; Oberhacken 12, 95326 Kulmbach (DE).

(74) Agents: **WARD, Donna T.** et al.; DT WARD, PC, 142A Main Street, Groton, Massachusetts 01450 (US).

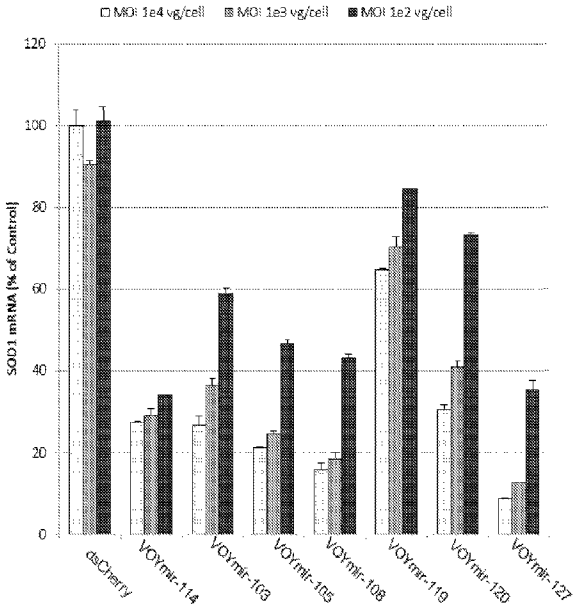
(81) Designated States *(unless otherwise indicated, for every kind of national protection available)*: AE, AG, AL, AM, AO, AT, AU, AZ, BA, BB, BG, BH, BN, BR, BW, BY, BZ, CA, CH, CL, CN, CO, CR, CU, CZ, DE, DK, DM, DO, DZ, EC, EE, EG, ES, FI, GB, GD, GE, GH, GM, GT, HN, HR, HU, ID, IL, IN, IR, IS, JP,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

[Continued on next page]

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

FIG. 1



(57) Abstract: The present invention relates to small interfering RNA (siRNA) molecules against the SOD1 gene, adeno-associated viral (AAV) vectors encoding siRNA molecules and methods for treating amyotrophic lateral sclerosis (ALS) using the siRNA molecules and AAV vectors.

WO 2016/077687 A1

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))*