

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

(19) World Intellectual Property
Organization
International Bureau

(43) International Publication Date
05 October 2023 (05.10.2023)



(10) International Publication Number
WO 2023/192290 A1

(51) International Patent Classification:

A61K 31/451 (2006.01) *A61K 47/26* (2006.01)
A61K 9/00 (2006.01) *A61K 47/38* (2006.01)
A61K 47/12 (2006.01) *A61P 25/28* (2006.01)

(21) International Application Number:

PCT/US2023/016581

(22) International Filing Date:

28 March 2023 (28.03.2023)

(25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data:

63/324,313 28 March 2022 (28.03.2022) US

(71) Applicant: **THERAVANCE BIOPHARMA R&D IP, LLC** [US/US]; 901 Gateway Boulevard, South San Francisco, California 94080 (US).

(72) Inventors: **VICKERY, Ross**; c/o Theravance Biopharma R&D IP, LLC, 901 Gateway Boulevard, South San Francisco, California 94080 (US). **BOURDET, David L.**; c/

o Theravance Biopharma R&D IP, LLC, 901 Gateway Boulevard, South San Francisco, California 94080 (US). **GUERIN, Tadhg**; c/o Theravance Biopharma R&D IP, LLC, 901 Gateway Boulevard, South San Francisco, California 94080 (US). **NORCLIFFE-KAUFMANN, Lucy**; c/o Theravance Biopharma R&D IP, LLC, 901 Gateway Boulevard, South San Francisco, California 94080 (US). **ZHENG, Beiyao**; c/o Theravance Biopharma R&D IP, LLC, 901 Gateway Boulevard, South San Francisco, California 94080 (US).

(74) Agent: **TANNER, Lorna L.** et al.; SHEPPARD MULLIN RICHTER & HAMPTON LLP, 650 Town Center Drive, 10th Floor, Costa Mesa, California 92626-1993 (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, CV, CZ, DE, DJ, DK, DM, DO, DZ, EC, EE, EG, ES, FI, GB, GD, GE, GH, GM, GT, HN, HR, HU, ID, IL, IN, IQ, IR, IS, IT, JM, JO, JP, KE, KG, KH, KN, KP, KR, KW, KZ, LA, LC, LK, LR, LS, LU, LY, MA, MD, MG, MK, MN, MU, MW, MX, MY, MZ, NA,

(54) Title: AMPRELOXETINE FOR USE FOR TREATING MULTIPLE SYSTEM ATROPHY

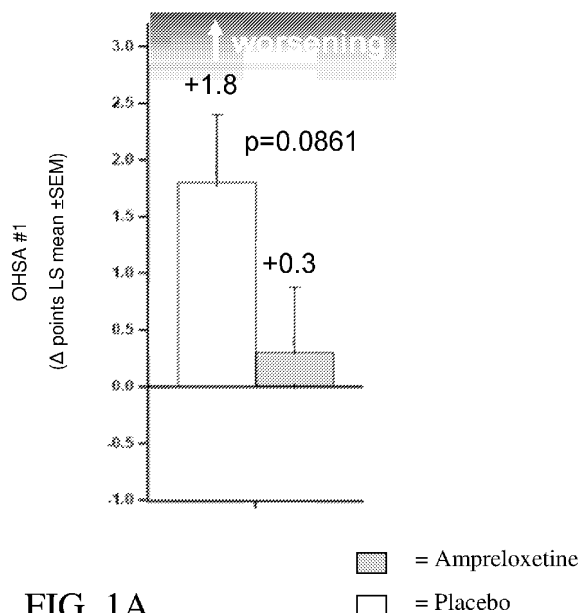


FIG. 1A

(57) Abstract: Disclosed are methods for treating a subject having multiple system atrophy (MSA) using ampreloxetine or a pharmaceutically acceptable salt thereof. The methods disclosed include using ampreloxetine or a pharmaceutically acceptable salt thereof to (i) treat the symptoms of neurogenic orthostatic hypotension in a subject having MSA; (ii) reduce the magnitude of decline in the quality of life a subject having MSA; and/or (iii) increase the level of norepinephrine in a subject having MSA.

[Continued on next page]

WO 2023/192290 A1

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, CV, GH, GM, KE, LR, LS, MW, MZ, NA, RW, SC, 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, ME, 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))*