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

(19) World Intellectual Property Organization
International Bureau

(43) International Publication Date
09 March 2023 (09.03.2023)

WIPO | PCT

(10) International Publication Number
WO 2023/031440 A1

(51) International Patent Classification:
A61K 31/44 (2006.01) *A61P 25/18* (2006.01)
A61P 25/00 (2006.01) *A61P 25/28* (2006.01)
A61P 25/16 (2006.01) *C07D 417/04* (2006.01)

(21) International Application Number:
PCT/EP2022/074536

(22) International Filing Date:
02 September 2022 (02.09.2022)

(25) Filing Language: English

(26) Publication Language: English

(30) Priority Data:
21194937.5 03 September 2021 (03.09.2021) EP

(71) Applicant: **INSUSENSE APS** [DK/DK]; Scherfigsvej 7, 2100 København (DK).

(72) Inventors: **LITTLE, Paul Brian**; 2100 Copenhagen (DK). **CASES-THOMAS, Manuel Javier**; 2100 Copenhagen (DK). **KJØLBY, Mads Fuglsang**; 2100 Copenhagen (DK). **NYKJÆR, Anders**; 2100 Copenhagen (DK).

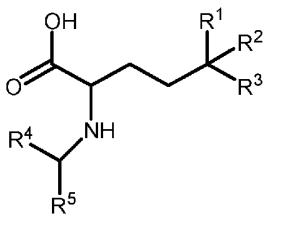
(74) Agent: **GILL JENNINGS & EVERY LLP**; The Broadgate Tower 20 Primrose Street, London, EC2A 2ES (GB).

(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, 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, 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))*
— *with sequence listing part of description (Rule 5.2(a))*
— *in black and white; the international application as filed contained color or greyscale and is available for download from PATENTSCOPE*

(54) Title: 2-AMINO-5,5-DIMETHYLHEXANOIC ACID DERIVATIVES AS SORTILIN MODULATORS FOR USE IN THE TREATMENT OF DISEASE OF THE CENTRAL NERVOUS SYSTEM



(I)

(57) Abstract: The present invention relates to a compound that binds to and modulates the activity of sortilin, wherein the compound has a blood-to-brain K_{puu} greater than 0.1. The compounds are capable of crossing the blood brain barrier and are suitable for use in the treatment of a disease of the central nervous system. The invention also relates to compounds of formula (I), which are modulators of sortilin activity, pharmaceutical compositions comprising these compounds and the use of these compounds in the treatment or prevention of medical conditions where modulation of sortilin activity is beneficial.

WO 2023/031440 A1