

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

(19) World Intellectual Property
Organization

International Bureau

(43) International Publication Date
09 November 2017 (09.11.2017)



(10) International Publication Number
WO 2017/192930 A1

(51) International Patent Classification:

C07D 487/14 (2006.01) *A61P 3/10* (2006.01)
C07D 513/04 (2006.01) *A61P 13/12* (2006.01)
C07D 495/04 (2006.01) *A61P 25/24* (2006.01)
C07D 491/048 (2006.01) *A61P 25/28* (2006.01)
C07D 498/04 (2006.01) *A61P 25/08* (2006.01)
A61K 31/519 (2006.01) *A61P 25/18* (2006.01)
A61P 25/16 (2006.01) *A61P 25/22* (2006.01)
A61P 27/06 (2006.01) *A61P 43/00* (2006.01)

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))

(21) International Application Number:

PCT/US2017/031189

(22) International Filing Date:

05 May 2017 (05.05.2017)

(25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data:

62/332,151 05 May 2016 (05.05.2016) US

(71) Applicant: **LYSOSOMAL THERAPEUTICS INC.**
[US/US]; 19 Blackstone Street, Cambridge, MA 02139 (US).

(72) Inventors: **SKERLJ, Renato, T.**; 12 Crocker Circle, West Newton, MA 02465 (US). **BOURQUE, Elyse Marie Josee**; 162 Ch Bouffard, L'etang-du-Nord, Quebec, G4T 3G6 (CA). **GREENLEE, William, J.**; 115 Herrick Avenue, Teaneck, NJ 07666 (US). **LANSBURY, Peter, T.**; 24 Elm Street, Brookline, MA 02445 (US).

(74) Agent: **DAVIS, Chad, E.** et al.; Goodwin Procter LLP, 100 Northern Avenue, Boston, MA 02210 (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, DJ, 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, 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,

(54) Title: SUBSTITUTED IMIDAZO[1,2-*b*]PYRIDAZINES, SUBSTITUTED IMIDAZO[1,5-*b*]PYRIDAZINES, RELATED COMPOUNDS, AND THEIR USE IN THE TREATMENT OF MEDICAL DISORDERS

(57) Abstract: The invention provides substituted imidazo[1,2-*b*]pyridazine compounds, substituted imidazo[1,5-*b*]pyridazine compounds, related compounds, compositions containing such compounds, medical kits, and methods for using such compounds and compositions to treat medical disorders, e.g., Gaucher disease, Parkinson's disease, Lewy body disease, dementia, or multiple system atrophy, in a patient. Exemplary substituted imidazo[1,2-*b*]pyridazine compounds described herein include substituted imidazo[1,2-*b*]pyridazine-3-carboxamide compounds and variants thereof.

WO 2017/192930 A1