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

(19) World Intellectual Property Organization
International Bureau

(43) International Publication Date
13 August 2020 (13.08.2020)

WIPO | PCT

(10) International Publication Number
WO 2020/163245 A1

(51) International Patent Classification:
A61K 31/439 (2006.01) *A61K 45/06* (2006.01)
A61P 25/00 (2006.01) *A61K 38/46* (2006.01)

(21) International Application Number:
PCT/US2020/016441

(22) International Filing Date:
03 February 2020 (03.02.2020)

(25) Filing Language: English

(26) Publication Language: English

(30) Priority Data:

62/800,996	04 February 2019 (04.02.2019)	US
62/851,433	22 May 2019 (22.05.2019)	US
62/894,167	30 August 2019 (30.08.2019)	US
62/937,618	19 November 2019 (19.11.2019)	US
62/962,647	17 January 2020 (17.01.2020)	US

(71) Applicant: **GENZYME CORPORATION** [US/US]; 50 Binney Street, Cambridge, Massachusetts 02142 (US).

(72) Inventors: **CRAWFORD, Nigel Patrick Somerville**; c/o Sanofi, 55 Corporate Drive, Bridgewater, New Jersey 08807 (US). **FISCHER, Tanya Zaremba**; c/o Sanofi, 55 Corporate Drive, Bridgewater, New Jersey 08807 (US).

(74) Agent: **POKER, Cory**; Hoxie & Associates LLC, 75 Main Street, Suite 203, Millburn, New Jersey 07041 (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, 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))*

(54) Title: METHODS FOR TREATING SYMPTOMS AND DISORDERS ASSOCIATED WITH LYSOSOMAL STORAGE DISEASES

(57) Abstract: This disclosure to methods for treating or preventing particular symptoms and disorders which are associated with lysosomal storage diseases using quinuclidine compounds of formula (I), optionally in combination with enzyme replacement therapy. This includes supranuclear gaze palsies, including horizontal and vertical saccadic gaze palsies, and cognitive deficits or gait disorders, such as in a patient having Gaucher disease or Niemann-Pick disease Type C. Also disclosed is a pharmaceutical composition comprising a quinuclidine compound for use in said methods.

WO 2020/163245 A1