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

(19) World Intellectual Property Organization
International Bureau

(43) International Publication Date
19 October 2006 (19.10.2006)

PCT

(10) International Publication Number
WO 2006/110483 A1

(51) International Patent Classification:
A61K 31/4245 (2006.01) *A61P 11/00* (2006.01)
A61P 3/10 (2006.01) *A61P 25/00* (2006.01)
A61P 7/00 (2006.01) *A61P 25/28* (2006.01)
A61P 9/00 (2006.01) *A61P 35/00* (2006.01)

(21) International Application Number:
PCT/US2006/012887

(22) International Filing Date: 6 April 2006 (06.04.2006)

(25) Filing Language: English

(26) Publication Language: English

(30) Priority Data:
60/669,789 8 April 2005 (08.04.2005) US
60/787,395 30 March 2006 (30.03.2006) US

(71) Applicant *(for all designated States except US)*: **PTC THERAPEUTICS, INC.** [US/US]; 100 Corporate Court, Middlesex Business Center, South Plainfield, New Jersey 07080 (US).

(72) Inventors; and
(75) Inventors/Applicants *(for US only)*: **HIRAWAT, Samit** [IN/US]; 602 Margaret Court, South Plainfield, New Jersey 07080 (US). **MILLER, Langdon** [US/US]; 13 Strawberry Lane, Lebanon, New Jersey 08833 (US).

(74) Agents: **INSOGNA, Anthony, M.** et al.; JONES DAY, 222 East 41st Street, New York, New York 10017-6702 (US).

(81) Designated States *(unless otherwise indicated, for every kind of national protection available)*: AE, AG, AL, AM, AT, AU, AZ, BA, BB, BG, BR, BW, BY, BZ, CA, CH, CN, CO, CR, CU, CZ, DE, DK, DM, DZ, EC, EE, EG, ES, FI, GB, GD, GE, GH, GM, HR, HU, ID, IL, IN, IS, JP, KE, KG, KM, KN, KP, KR, KZ, LC, LK, LR, LS, LT, LU, LV, LY, MA, MD, MG, MK, MN, MW, MX, MZ, NA, NG, NI, NO, NZ, OM, PG, PH, PL, PT, RO, RU, SC, SD, SE, SG, SK, SL, SM, SY, TJ, TM, TN, TR, TT, TZ, UA, UG, US, UZ, VC, VN, YU, ZA, ZM, ZW.

(84) Designated States *(unless otherwise indicated, for every kind of regional protection available)*: ARIPO (BW, GH, GM, KE, LS, MW, MZ, NA, SD, SL, SZ, TZ, UG, ZM, ZW), Eurasian (AM, AZ, BY, KG, KZ, MD, RU, TJ, TM), European (AT, BE, BG, CH, CY, CZ, DE, DK, EE, ES, FI, FR, GB, GR, HU, IE, IS, IT, LT, LU, LV, MC, NL, PL, PT, RO, SE, SI, SK, TR), OAPI (BF, BJ, CF, CG, CI, CM, GA, GN, GQ, GW, ML, MR, NE, SN, TD, TG).

Published:
— *with international search report*
— *before the expiration of the time limit for amending the claims and to be republished in the event of receipt of amendments*

For two-letter codes and other abbreviations, refer to the "Guidance Notes on Codes and Abbreviations" appearing at the beginning of each regular issue of the PCT Gazette.

WO 2006/110483 A1

(54) Title: COMPOSITIONS OF AN ORALLY ACTIVE 1,2,4-OXADIAZOLE FOR NONSENSE MUTATION SUPPRESSION THERAPY

(57) Abstract: The present invention relates to specific doses of and dosing regimens for using a 1,2,4-oxadiazole benzoic acid compound in treating or preventing diseases associated with nonsense mutations. In particular, the invention relates to specific doses and dosing regimens for the use of 3-[5-(2-fluoro-phenyl)-[1,2,4]oxadiazol-3-yl]-benzoic acid in mammals having diseases associated with nonsense mutations.