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

(19) World Intellectual Property Organization
International Bureau

(43) International Publication Date
3 May 2012 (03.05.2012)

PCT

(10) International Publication Number
WO 2012/055826 A1

(51) International Patent Classification:
*A61K 31/00* (2006.01) *A61K 38/13* (2006.01)
*A61K 31/145* (2006.01) *A61P 43/00* (2006.01)
*A61K 31/436* (2006.01) *A61P 25/00* (2006.01)
*A61K 38/00* (2006.01)

(21) International Application Number:
PCT/EP2011/068576

(22) International Filing Date:
24 October 2011 (24.10.2011)

(25) Filing Language: English

(26) Publication Language: English

(30) Priority Data:
61/406,248 25 October 2010 (25.10.2010) US

(71) Applicants *(for all designated States except US)*: **UNIVERSITE DE LA MEDITERRANEE (AIX-MARSEILLE II)** [FR/FR]; 58 boulevard Charles Livon, F-13007 Marseille (FR). **INSERM (Institut National de la Santé et de la Recherche Médicale)** [FR/FR]; 101 rue de Tolbiac, F-75654 Paris Cedex 13 (FR). **INSTITUT CURIE** [FR/FR]; 26 rue d'Ulm, F-75248 Paris cedex 05 (FR). **CENTRE NATIONAL DE LA RECHERCHE SCIENTIFIQUE** [FR/FR]; 3 rue Michel Ange, F-75016 Paris (FR).

(72) Inventors; and
(75) Inventors/Applicants *(for US only)*: **ROUX, Jean-Christophe** [FR/FR]; 1 rue des Genats, F-13190 Allauch (FR). **VILLARD, Laurent** [FR/FR]; Le Manoir C, 151 Chemin de la Colline Saint -Joseph, F-13009 Marseille (FR). **SAUDOU, Frédéric** [FR/FR]; 24 Chemin des Vignes, F-91400 Orsay (FR).

(74) Agents: **CHAJMOWICZ, Marion** et al.; Becker & Associes, 25 rue Louis Le Grand, F-75 002 Paris (FR).

(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, BR, BW, BY, BZ, CA, CH, CL, CN, CO, CR, CU, CZ, DE, DK, DM, DO, DZ, EC, EE, EG, ES, FI, GB, GD, GE, GH, GM, GT, HN, HR, HU, ID, IL, IN, IS, JP, KE, KG, KM, KN, KP, KR, KZ, LA, LC, LK, LR, LS, LT, LU, LY, MA, MD, ME, MG, MK, MN, MW, MX, MY, MZ, NA, NG, NI, NO, NZ, OM, PE, PG, PH, PL, PT, QA, RO, RS, RU, RW, 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, SZ, TZ, UG, ZM, ZW), Eurasian (AM, AZ, BY, KG, KZ, MD, 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, ML, MR, NE, SN, TD, TG).

**Declarations under Rule 4.17**:

— *of inventorship (Rule 4.17(iv))*

**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))*

(54) Title: TREATMENT OF MeCP2-ASSOCIATED DISORDERS

(57) Abstract: The invention relates to the use of cystamine, cysteamine, or a salt thereof, or of calcineurin inhibitors for treating a MeCP2-associated disorder such as Rett syndrome.

WO 2012/055826 A1