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

(19) World Intellectual Property Organization
International Bureau

(43) International Publication Date
4 April 2013 (04.04.2013)

WIPO | PCT

(10) International Publication Number
WO 2013/048558 A3

(51) International Patent Classification:
A61K 49/00 (2006.01)

(21) International Application Number:
PCT/US2012/028620

(22) International Filing Date:
9 March 2012 (09.03.2012)

(25) Filing Language: English

(26) Publication Language: English

(30) Priority Data:
61/542,100 30 September 2011 (30.09.2011) US
61/564,668 29 November 2011 (29.11.2011) US

(71) Applicant *(for all designated States except US)*: **HYPERION THERAPEUTICS, INC.** [US/US]; 2000 Sierra Point Parkway, Suite 400, Brisbane, CA 94005 (US).

(72) Inventors; and
(71) Applicants : **SCHARSCHMIDT, Bruce** [US/US]; 45 St. Francis Boulevard, San Francisco, California 94127 (US). **MOKHTARANI, Masoud** [US/US]; 725 Castle Rock Road, Walnut Creek, California 94598 (US).

(74) Agent: **MORRIS, Patrick D.**; Perkins Coie LLP, P.O. Box 1208, Seattle, Washington 98111-1208 (US).

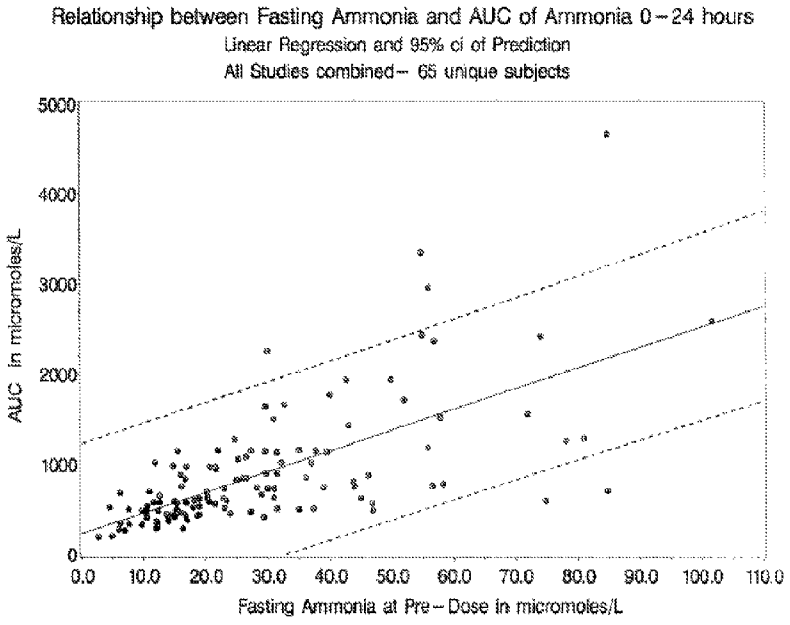
(81) Designated States *(unless otherwise indicated, for every kind of national protection available)*: AE, AG, AL, AM, AO, AT, AU, AZ, BA, BB, BG, BH, BR, BW, BY, BZ, CA, CH, CL, CN, CO, CR, CU, CZ, DE, DK, DM, DO, DZ, EC, EE, EG, ES, FI, GB, GD, GE, GH, GM, GT, HN, HR, HU, ID, IL, IN, IS, JP, KE, KG, 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).

[Continued on next page]

(54) **Title**: METHODS OF THERAPEUTIC MONITORING OF NITROGEN SCAVENGING DRUGS

Figure 2



(57) **Abstract**: The present disclosure provides methods for evaluating daily ammonia exposure based on a single fasting ammonia blood level measurement, as well as methods that utilize this technique to adjust the dosage of a nitrogen scavenging drug, determine whether to administer a nitrogen scavenging drug, and treat nitrogen retention disorders.

WO 2013/048558 A3

WO 2013/048558 A3

Published:

— *with international search report (Art. 21(3))*

(88) Date of publication of the international search report:
3 April 2014