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

(19) World Intellectual Property Organization
International Bureau

(43) International Publication Date
3 August 2006 (03.08.2006)

PCT

(10) International Publication Number
WO 2006/081429 A1

(51) International Patent Classification:
G01N 33/50 (2006.01)

(21) International Application Number:
PCT/US2006/002949

(22) International Filing Date: 27 January 2006 (27.01.2006)

(25) Filing Language: English

(26) Publication Language: English

(30) Priority Data:
60/647,477 27 January 2005 (27.01.2005) US

(71) Applicant *(for all designated States except US)*: **CHIRON CORPORATION** [US/US]; 4560 Horton Street, Emeryville, CA 94608-2916 (US).

(72) Inventor; and

(75) Inventor/Applicant *(for US only)*: **VADEVANTER, Donald** [US/US]; 12520 33rd Street Court E, Edgewood, WA 98372 (US).

(74) Agents: **COLLIER, Steven** et al.; Chiron Corporation, 4560 Horton Street, Emeryville, CA 94608-2916 (US).

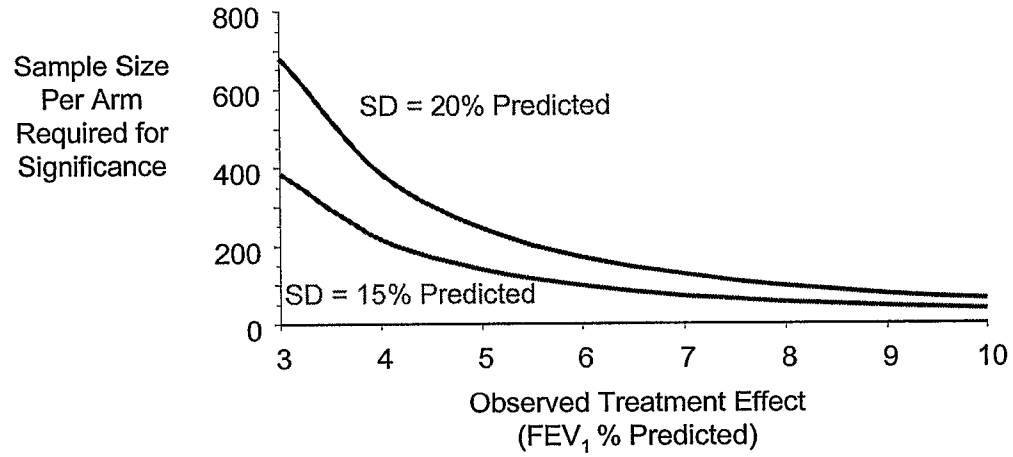
(81) Designated States *(unless otherwise indicated, for every kind of national protection available)*: AE, AG, AL, AM, AT, AU, AZ, BA, BB, BG, BR, BW, BY, BZ, CA, CH, CN, CO, CR, CU, CZ, DE, DK, DM, DZ, EC, EE, EG, ES, FI, GB, GD, GE, GH, GM, HR, HU, ID, IL, IN, IS, JP, KE, KG, KM, KN, KP, KR, KZ, LC,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

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.

(54) **Title:** A METHOD FOR CHARACTERIZING THE EFFICACY OF AN AGENT TARGETING A PRIMARY CYSTIC FIBROSIS DEFECT



(57) **Abstract:** Generally, the method for characterizing the efficacy of an agent targeting a primary cystic fibrosis defect comprises measuring a change in the status of lung infection in a sample population of subjects administered the agent in comparison to a control sample population of subjects; wherein the subjects in the sample population and the control sample population have the primary cystic fibrosis defect and are uninfected before the agent is administered; wherein a beneficial change in the presence of lung infection in the sample population in comparison to the control sample population is indicative of a treatment effect; and wherein the agent lacks intrinsic antibiotic activity.

WO 2006/081429 A1