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

(19) World Intellectual Property Organization
International Bureau

(43) International Publication Date
17 November 2016 (17.11.2016)

WIPO | PCT

(10) International Publication Number
WO 2016/183280 A1

(51) International Patent Classification:
A61K 38/18 (2006.01) *A61P 7/06* (2006.01)
A61P 7/00 (2006.01)

(21) International Application Number:
PCT/US2016/031999

(22) International Filing Date:
12 May 2016 (12.05.2016)

(25) Filing Language: English

(26) Publication Language: English

(30) Priority Data:

62/161,136	13 May 2015 (13.05.2015)	US
62/173,836	10 June 2015 (10.06.2015)	US
62/243,457	19 October 2015 (19.10.2015)	US

(71) Applicants: **CELGENE CORPORATION** [US/US]; 86 Morris Avenue, Summit, NJ 07901 (US). **ACCELERON PHARMA INC.** [US/US]; 128 Sidney Street, Cambridge, MA 02139 (US).

(72) Inventors: **ATTIE, Kenneth, M.**; 228 Marlborough Street, Boston, MA 02116 (US). **LAADEM, Abderrahmane**; 12 Matthews Farm Rd., Belle Mead, NJ 08502 (US). **CHOPRA, Rajesh**; 54 Eggers Court, Summit, NJ 07901 (US). **BACKSTROM, Jay**; 15400 Aberdeen, Leawood, KS 66224 (US).

(74) Agents: **MARTINEK, Sebastian** et al.; Jones Day, 250 Vesey Street, New York, NY 10281-1047 (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, DK, DM, DO, DZ, EC, EE, EG, ES, FI, GB, GD, GE, GH, GM, GT, HN, HR, HU, ID, IL, IN, IR, IS, JP, KE, KG, KN, KP, KR, 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, SM, ST, SV, SY, TH, TJ, TM, TN, TR, TT, TZ, UA, UG, US, UZ, VC, VN, ZA, ZM, ZW.

[Continued on next page]

WO 2016/183280 A1

(54) Title: TREATMENT OF BETA-THALASSEMIA USING ACTRII LIGAND TRAPS

(57) Abstract: Provided herein are methods of treating beta-thalassemia by subcutaneous administration of about 0.8 mg/kg of an ActRII signaling inhibitor. Also provided herein are methods of adjusting the dose of the ActRII signaling inhibitor administered to the subject.

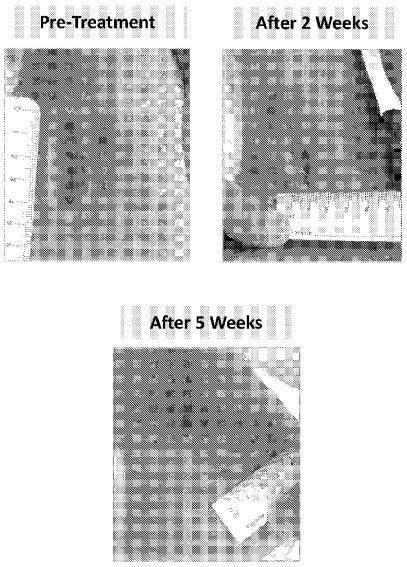


Figure 1

(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))*

— *with sequence listing part of description (Rule 5.2(a))*