



(51) International Patent Classification:

C07K 16/00 (2006.01) C07K 17/14 (2006.01)

C07K 17/00 (2006.01) C12P 21/08 (2006.01)

(21) International Application Number:

PCT/US2012/037251

(22) International Filing Date:

10 May 2012 (10.05.2012)

(25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data:

61/485,677 13 May 2011 (13.05.2011) US

(71) Applicant (for all designated States except US): EDIMER PHARMACEUTICALS, INC. [US/US]; 1 Broadway, 8th Floor, Cambridge, MA 02142 (US).

(72) Inventors; and

(75) Inventors/Applicants (for US only): SCHNEIDER, Pascal [CH/CH]; Chemin des Ormeaux 10, 1066 Epalinges (CH). KOWALCZYK, Christine [CH/CH]; Av. de Grat-ta-Paille 15, 1018 Lausanne (CH).

(74) Agent: WARD, Donna, T.; Dt Ward, P.C., 292 Main Street, Suite 2, Groton, MA 01450 (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, 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:

— as to applicant's entitlement to apply for and be granted a patent (Rule 4.17(ii))

— as to the applicant's entitlement to claim the priority of the earlier application (Rule 4.17(iii))

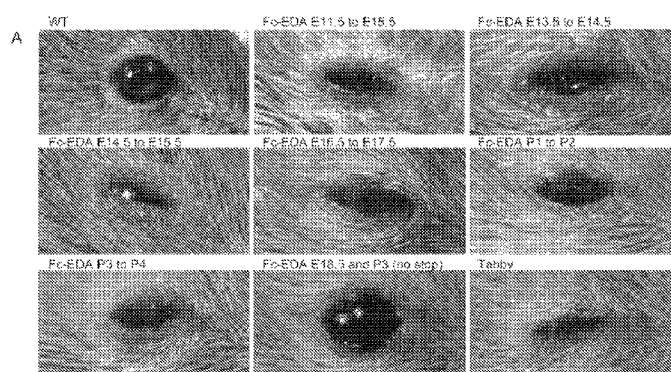
Published:

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

[Continued on next page]

(54) Title: COMPOSITIONS AND METHODS FOR THE ALTERATION OF XLHED PHENOTYPES

FIGURE 5



B Eye phenotype correction in Tabby mice with Fc-EDA1

WT	Fc-EDA E11.5 to E15.5	Fc-EDA E13.5 to E14.5
Fc-EDA E14.5 to E15.5	Fc-EDA E15.5 to E17.5	Fc-EDA P1 to P2
Fc-EDA P3 to P4	Fc-EDA E18.5 and P3 (no stop)	Tabby

(57) Abstract: The invention relates to methods for the temporal administration of EDA agonists, in particular EDI200, which correlate to optimal therapeutic response windows required for the formation of any EDA-dependent structures such as ectodermal appendages. Use of the methods described allow for the design of targeted therapeutic dosing and administration regimens in order to correct or alter abnormal phenotypes associated with genetic disorders, in particular, XLHED.



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