

(12) International Application Status Report

Received at International Bureau: 13 October 2005 (13.10.2005)

Information valid as of: (..)

Report generated on: 15 May 2021 (15.05.2021)

(10) Publication number:

WO2006/007560

(43) Publication date:

19 January 2006 (19.01.2006)

(26) Publication language:

English (EN)

(21) Application Number:

PCT/US2005/023529

(22) Filing Date:

01 July 2005 (01.07.2005)

(25) Filing language:

English (EN)

(31) Priority number(s):

60/584,648 (US)

(31) Priority date(s):

01 July 2004 (01.07.2004)

(31) Priority status:

Priority document received (in compliance with PCT Rule 17.1)

(51) International Patent Classification:

A61K 38/46 (2006.01); **A61K 38/47** (2006.01); **C12N 9/14** (2006.01); **C12N 9/20** (2006.01); **C07H 21/04** (2006.01)

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(54) Title (EN): TARGETED PROTEIN REPLACEMENT FOR THE TREATMENT OF LYSOSOMAL STORAGE DISORDERS

(54) Title (FR): REMPLACEMENT DE PROTEINES CIBLEES POUR LE TRAITEMENT DE MALADIES LYSOSOMALES

(57) Abstract:

(EN): The present invention relates to compositions and methods for delivering lysosomal proteins. The compositions and methods described herein permit the targeted delivery of exogenous lysosomal proteins to cell surface proteins that allow their internalization via non-clathrin pathways. The present invention further relates to the use of the compositions and methods for enzyme replacement therapy of lysosomal storage diseases. Nucleic acids, recombinant cells and kits useful for making and using the compositions of the invention are also provided.

(FR): La présente invention concerne des compositions et des méthodes de transport de protéines lysosomales. Les compositions et les méthodes de l'invention permettent d'acheminer de façon ciblée des protéines lysosomales exogènes vers des protéines de surface permettant leur internalisation par des voies autres que celles de la clathrine. L'invention concerne également l'utilisation desdites compositions et méthodes destinées au traitement par remplacement enzymatique de maladies lysosomales. Elle concerne en outre des acides nucléiques, des cellules recombinantes, et des trousseaux utiles servant à produire et mettre en oeuvre les compositions de l'invention.

International search report:

Received at International Bureau: 02 August 2006 (02.08.2006) [US]

International Report on Patentability (IPRP) Chapter II of the PCT:

Not available

(81) Designated States:

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, KP, KR, KZ, LC, LK, LR, LS, LT, LU, LV, 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

European Patent Office (EPO) : 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

African Intellectual Property Organization (OAPI) : BF, BJ, CF, CG, CI, CM, GA, GN, GQ, GW, ML, MR, NE, SN, TD, TG

African Regional Intellectual Property Organization (ARIPO) : BW, GH, GM, KE, LS, MW, MZ, NA, SD, SL, SZ, TZ, UG, ZM, ZW

Eurasian Patent Organization (EAPO) : AM, AZ, BY, KG, KZ, MD, RU, TJ, TM