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(71) Applicant(s):

UNIVERSITY OF PENNSYLVANIA [US/US]; Center for Technology Transfer Suite 200 Philadelphia, PA 19104-6283 (US) *(for all designated states except US)*

MOUNT SINAI SCHOOL OF MEDICINE [US/US]; One Gustave L. Levy Place Box 1675 New York, NY 10029 (US) *(for all designated states except US)*

GALINDO, Silvia, Muro [ES/US]; 4247 Locust St., Apt. 402 Philadelphia, PA 19104 (US) *(for US only)*

MUZYKANTOV, Vladimir, R. [US/US]; 101 White Pine Court Warminster, PA 18974 (US) *(for US only)*

SCHUCHMAN, Edward, Howard [US/US]; 300 Lakeshore Drive Haworth, NJ 07641 (US) *(for US only)*

(72) Inventor(s):

GALINDO, Silvia, Muro; 4247 Locust St., Apt. 402 Philadelphia, PA 19104 (US)

MUZYKANTOV, Vladimir, R.; 101 White Pine Court Warminster, PA 18974 (US)

SCHUCHMAN, Edward, Howard; 300 Lakeshore Drive Haworth, NJ 07641 (US)

(74) Agent(s):

ABRAMS, Samuel, B.; Jones Day 222 East 41st Street New York, NY 10017-6702 (US)

(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.

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