Scavenger Receptor Uptake for Fabry Disease
Enzyme Replacement Therapy

BACKGROUND

Fabry disease is a rare X-linked inborn error of glycolipid metabolism caused by a deficiency of the lysosomal enzyme, α-galactosidase A, which leads to early death in affected males due to occlusive disease of the heart, kidney, and brain. Several clinical trials of enzyme replacement therapy for Fabry disease patients in the last few years revealed clinical efficacy. However, 88% of patients developed potentially neutralizing IgG antibodies to α-galactosidase A with a therapeutic enzyme dose of 1 mg per kilogram of body weight while only 21% developed antibodies with a lower dose of 0.2 mg of enzyme per kilogram of body weight. Hence there exists a long standing need to provide a treatment regimen that requires lower doses of enzyme, targeted delivery within body and sufficient biological activity upon intracellular uptake.

INVENTION

The present invention relates to composition comprising a lysosomal enzyme conjugated to a negatively charged scavenger receptor ligand. This negatively charged scavenger receptor ligand can be any ligand that binds a scavenger receptor and is transported to the lysosome. The invention also relates to composition comprising lysosomal enzyme capsulated by a liposome and methods of treating lysosomal storage disease with such compositions.

APPLICATIONS

One of the aspects of this invention is to provide a method for treatment of lysosomal storage diseases. An effective amount of the composition can be administrated to one in need in a convenient manner such as oral, intravenous, intramuscular, intranasal, intradermal or subcutaneous routes.

ADVANTAGES

The present invention will lead to improvements in treatment and lives of Fabry disease patients and other patients receiving replacement therapy for lysosomal storage disease as it will lead to:

- Lower dosage requirement of enzymes
- More effective therapeutic effects
- Less frequent infusions, minimizing ill-defined infusion associated reactions (vomiting, rigors etc.)

MARKET

- During 2005-2010, Global Fabry’s disease therapeutics market grew at a compound annual growth rate (CAGR) of 9.9% and was worth $412.8m in 2010.
- The market is expected to record a CAGR of 12.8% from 2010-2018, to reach a value $1,078.8m by 2018.

TEAM

Professor David H. Calhoun; Department of Chemistry; City College of The City University of New York
Professor Lane Gilchrist; Department of Biomedical Engineering; City College of The City University of New York

Licensing Contact
Douglas Adams
TCO
The City University of New York
555 West 57th Street, Suite 1407
New York, NY 10019

T 646-758-7906
F 646.758.7907
douglas.adams@mail.cuny.edu
www.cuny.edu/research/oivcr/fo.html

Ref #: 12A0009
Lead Inventor: Professor David H. Calhoun

IP pending.
Licensing available.