

LIPOSOMES AND ITS USE FOR ENZYME DELIVERY

The Need

Certain diseases can be caused by the lack of activity of a particular protein or of a certain enzyme, either by being defective or absent, as in lysosomal diseases such as Fabry disease.

The Solution

The present invention relates in general to the field of liposomes which are useful in the delivery of enzymes, in particular of the alpha-galactosidase enzyme.

Innovative Aspects

The inventors have developed a robust tool for α -galactosidase A (GLA) enzyme delivery, allowing the transported enzyme to perform its activity *in vivo* with high efficacy which is useful in the treatment of Fabry disease.

The liposomes of the present invention outperformed previously known liposomal systems.

The improvements in the colloidal stability, entrapment efficiency, and biological activity of GLA described in the invention allowed a reduction in dose and volume of GLA-conjugated liposomes to be administered *in vivo*, a necessary step to demonstrate the significant benefit of these systems versus the current enzymatic replacement therapy in Fabry patients.

Good tolerability and no adverse side effects were observed in mice after repeated administrations of liposomes.

The liposomes of the present invention are safe can be used as enzyme-replacement therapy system for treatment of Fabry disease.

Stage of Development:

In Vivo

Intellectual Property

European patent application (Priority date: January 27, 2021)

International extension (PCT application)

Available for:
Licensing



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