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Lipophilic polyamines providing enhanced intracellular delivery of agents by a polyamine transport system

Background

A key requirement for successful gene therapy is the efficient in vivo transfer of DNA to specific cell types. Viral vectors have proven to be very efficient transfection agents and allow for the insertion of foreign DNA into many cell types. However, the inherent problems with viral vectors such as immunogenicity and the limited size of the DNA plasmid that can be transferred have led to interest in developing efficient non-viral vectors. Non-viral vectors are ideal because of their expected low toxicity and immunogenicity, ability to transfer large strands of DNA and simpler synthetic preparation. Typically, these vectors consist of a lipophilic component attached to a positively charged polar headgroup through the use of a spacer or linking motif and are a mixture of neutral and positively charged lipids.

Researchers at the University of Central Florida (UCF) synthesized homospermine and homospermidine cationic lipids that can be used as highly efficient and selective transfection agents. When used as plasmid DNA carriers, these lipopolyamines exhibited higher transfection and gene expression levels than vectors transfected with commercially-available lipophillic transfection reagents. In addition, these lipopolyamines can be used to selectively target specific cell types. The lipopolyamines utilize the polyamine transport system (PAT) on the surface of cells and can selectively target rapidly proliferating cancer cells in which the PAT system is often upregulated. These compounds can be used to deliver a therapeutic agent to afflicted cells while avoiding healthy cells. The disclosed invention can also be used for co-administration with anticancer toxin proteins, such as saporin, which is a ribosome-inactivating protein. When administered alone saporin demonstrates low in vivo activity, but when it is co-administered in the presence of these lipopolyamines, it results in a 60% reduction in protein synthesis in cancer cells.

Invention

This invention relates to an improved liposome for use in gene and protein therapy on cells having an active polyamine transport system. UCF ID#7353

Application

The current invention can be used for targeted delivery of a gene or protein therapy via a non-viral vector to cells exhibiting high levels of polyamine transporters on their cell surface (i.e., cancer cells).

Advantages

- Non-viral polyamine transport system that is simple to synthesize and that exhibits low toxicity and immunogenicity and an ability to transfer large strands of DNA
- The lipopolyamines can be co-administered with therapeutic agents or anti-cancer toxins for direct delivery to the cancer cell

Lead Inventor

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Selected References

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