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CNS targeted peptides and proteins for the treatment of neurological disorders

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Delivery of proteins by vascular distribution to the central nervous system (CNS) is significantly impaired by the blood-brain barrier (BBB). The BBB is composed of a tightly packed layer of endothelial cells and numerous astrocytic processes that regulate the passage and diffusion of proteins and growth factors from the blood stream to the CNS. Transport of almost all particles to the CNS occurs via binding to specific receptors on the vascular side of the endothelial cell followed by endocytosis and transport to the CNS. One well-characterized receptor is the low-density lipoprotein (LDL) receptor, which binds and transports apolipoprotein B (ApoB). We fused the LDL receptor-binding domain of ApoB to select therapeutic proteins and then utilized a viral vector delivery system to deliver the fusion gene to the liver for expression and secretion into the blood. These fusion proteins were taken up by neurons and astrocytes in the CNS and proved to be functional in preventing neuronal degeneration. In an attempt to generate a more clinical viable route of administration, recombinant proteins produced in eukaryotic cells have been generated, and these show similar transport properties as the virus vector expressed proteins. This demonstrates a feasible approach for delivery of proteins to the CNS and could possibly be used as a general method for delivery of therapeutic proteins to targeted organs. We have applied this technology to the delivery of therapeutic proteins for Parkinson's disease, Alzheimer's disease, Gaucher's disease and epilepsy.

Biography

Brian Spencer received his PhD in Medical Microbiology and Immunology from the University of Wisconsin. He then completed post-doctoral training with Inder Verma at the Salk Institute in La Jolla, CA. Following this, he was appointed as a project scientist at the University of California, San Diego. Recently, he started his own company, NeuroTransit, Inc.; to develop CNS targeted therapeutics for neural-degenerative disorders.

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