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Development of new protein vectors for the delivery of large therapeutic compounds to treat CNS disorders

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The central nervous system (CNS) is a sanctuary protected by barriers among them the blood-brain barrier (BBB). The BBB is formed by the specific nature of the endothelial cells of the brain capillaries only allowing brain access to nutrients necessary for brain cell survival and function. These properties of the BBB result in the incapacity of therapeutic compounds small and large to reach the brain at therapeutic concentrations. Various strategies are now being developed to enhance the amount and concentration of these compounds in the brain parenchyma. The development of new technologies such as peptide vectors will achieve the delivery of active agents in therapeutic concentration across the BBB to treat brain diseases such as cancer or neurodegenerative disorders. BiOasis Technologies Inc is dedicated to the development of new platform technologies for the delivery of therapeutics in the brain. The use and development of new active proteins or peptides for drug brain delivery using physiological approaches will be presented. BiOasis Technologies is developing a new vector, Transcend or also known as melanotransferrin (MTf) and its second generation vector MTfpep for the delivery of biologics across the BBB. The application of the MTfpep vector for the delivery of lysosomal enzyme, antibodies and nucleic acids such as siRNA will be presented. We have demonstrated that the MTfpep vector associated to antibodies and to siRNA can deliver a therapeutic concentration of active compounds in the CNS. These studies demonstrate that Transcend can be used as a vector for the transport of biologics across the BBB and capable of shuttling therapeutic levels of a variety of compounds from small anti-cancer agent to larger biologics such as antibodies and nucleic acids across the BBB for the treatment of neurological disorders.

Biography

Reinhard Gabathuler has obtained his PhD in Plant Biochemistry at the Université de Lausanne, Switzerland, in 1982 and Post-doctoral studies at the University of Washington, Seattle. He has held various research positions at the Swiss Institute for Experimental Cancer Research, Lausanne; the Ludwig Institute for Cancer Research at the Karolinska Institutet, Stockholm, Sweden and the Biotechnology Laboratory of the University of British Columbia, Vancouver, Canada. His research on new vector for delivery of therapeutics to the brain led to the creation of Synapse Technologies Inc., where he began as Vice-President Research (1998). The company was later acquired by BioMarin Pharmaceutical Inc., where he assumed the position of Vice President of Brain Research (2002). He has joined AngioChem Inc., in 2005 as its Chief Scientific Officer and has applied his extensive knowledge in biochemistry, cell biology and immunology to directing the R&D programs, advancing the company's product ANG1005 to IND application and clinic. He has joined BiOasis Technologies Inc., in 2010 as its Chief Scientist.

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