Advances in delivery of therapeutic genes to treat brain tumors

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Human Gene Therapy, the Official Journal of the European Society of Gene and Cell Therapy, British Society for Gene Therapy, French Society of Cell and Gene Therapy, German Society of Gene Therapy, and five other gene therapy societies is an authoritative peer-reviewed journal published monthly in print and online that presents reports on the transfer and expression of genes in mammals, including humans. Related topics include improvements in vector development, delivery systems, and animal models, particularly in the areas of cancer, heart disease, viral disease, genetic disease, and neurological disease, as well as ethical, legal, and regulatory issues related to the gene transfer in humans. Credit: © Mary Ann Liebert Inc., publishers

Novel tools and methods for delivering therapeutic genes to cells in the

central nervous system hold great promise for the development of new treatments to combat incurable neurologic diseases. Five of the most exciting developments in this rapidly advancing field are presented in a series of articles in the June issue of *Human Gene Therapy*, a peer-reviewed journal published by Mary Ann Liebert, Inc.

A review article by Betley and Sternson, "Adeno-Associated Viral Vectors for Mapping, Monitoring, and Manipulating Neural Circuits," highlights the latest genetic tools that are enabling cell type-specific delivery of transgenes for studying the structure and function of neuronal circuits.

Ryu et al. report on the successful use of stem cells derived from human umbilical cord blood to deliver a novel therapeutic gene —interleukin-12—to the brain for long-term anti-tumor activity against gliomas, a deadly type of brain tumor. Their innovative strategy is presented in the research report "Gene Therapy of Intracranial Glioma Using Interleukin 12-Secreting Human Umbilical Cord Blood-Derived Mesenchymal Stem Cells."

"High-Density Lipoprotein Facilitates In Vivo Delivery of α-Tocopherol-Conjugated Short-Interfering RNA to the Brain" by Uno et al. describes a combination strategy for dramatically improved delivery of siRNAs to the brain to silence genes involved in neurological disease.

Loss of the neurotrophic factor GDNF contributes to the development of neuropathic pain caused by trauma or neurodegenerative disease. Shi et al. present data to support this link and demonstrate the potential to replace GDNF via intramuscular gene delivery in the article "Glial Cell Line-Derived Neurotrophic Factor Gene Transfer Exerts Protective Effect on Axons in Sciatic Nerve Following Constriction-Induced Peripheral Nerve Injury."

The use of adenoviral vectors as carriers of therapeutic genes to the cerebrospinal fluid has the potential to enable long-term gene expression for the treatment of neurological diseases. In the brief report "Intrathecal Injection of Helper-Dependent Adenoviral Vectors Results in Long-Term Transgene Expression in Neuroependymal Cells and Neurons" Dindot et al. describe the successful use of adenoviral vectors to transduce neuronal cells.

"Treating neurologic diseases with traditional biologic products such as therapeutic proteins has been challenging due to limited access. The use of vectors helps to overcome these barriers," says James M. Wilson, MD, PhD, Editor-in-Chief, and Director of the Gene Therapy Program, Department of Pathology and Laboratory Medicine, Perelman School of Medicine, University of Pennsylvania, Philadelphia.

More information: The articles are available free online at www.liebertpub.com/hum

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