Advances in stem cell therapy to treat neurogenetic diseases

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Transplantation of therapeutic stem cells directly into the central nervous system (CNS) is a promising new approach to treating the neurological effects of lysosomal storage diseases (LSD), a group of at least 50 different monogenic inherited diseases. Translating the stem cell therapies developed in animal models of LSD to effective human therapies still faces significant challenges, which are described in an article in *Human Gene Therapy*.

The article entitled "Stem Cell Therapy for the Central Nervous System in Lysosomal Storage Diseases (online.liebertpub.com/doi/full … 10.1089/hum.2016.088)" is part of a special joint issue on stem cell gene therapy in Human Gene Therapy and Stem Cells & Development guest edited by Luigi Naldini, MD, Scientific Director, San Raffaele Telethon Institute for Gene Therapy, Milan, Italy. A special "upside-down" print issue will be distributed at ESGCT/ISSCR Florence 2016 in October.

Coauthors Faez Siddiqi and John Wolfe, Children's Hospital of Philadelphia, and Perelman School of Medicine and School of Veterinary Medicine, University of Pennsylvania (Philadelphia, PA), discuss the different types of stem cells that can be used to treat neurological diseases. They review the latest research and describe the efforts underway to engineer neural stem cells and deliver them to the CNS to optimize their therapeutic capacity in the brains of patients with LSD.

"Stem cell therapy for previously incurable lysosomal storage disorders

affecting the brain continues to progress, offering new hope to patients and families," says Editor-in-Chief Terence R. Flotte, MD, Celia and Isaac Haidak Professor of Medical Education and Dean, Provost, and Executive Deputy Chancellor, University of Massachusetts Medical School, Worcester, MA. "Many rodents studies have demonstrated the concept that direct injection of stem cells can reverse aspects of these diseases that are not treatable with enzyme replacement therapy. This review by Drs. Siddiqi and Wolfe provides a realistic and comprehensive assessment of the prospect for and limitations of this approach,"

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More information: Faez Siddiqi et al. Stem Cell Therapy for the Central Nervous System in Lysosomal Storage Diseases, *Human Gene Therapy* (2016). DOI: 10.1089/hum.2016.088