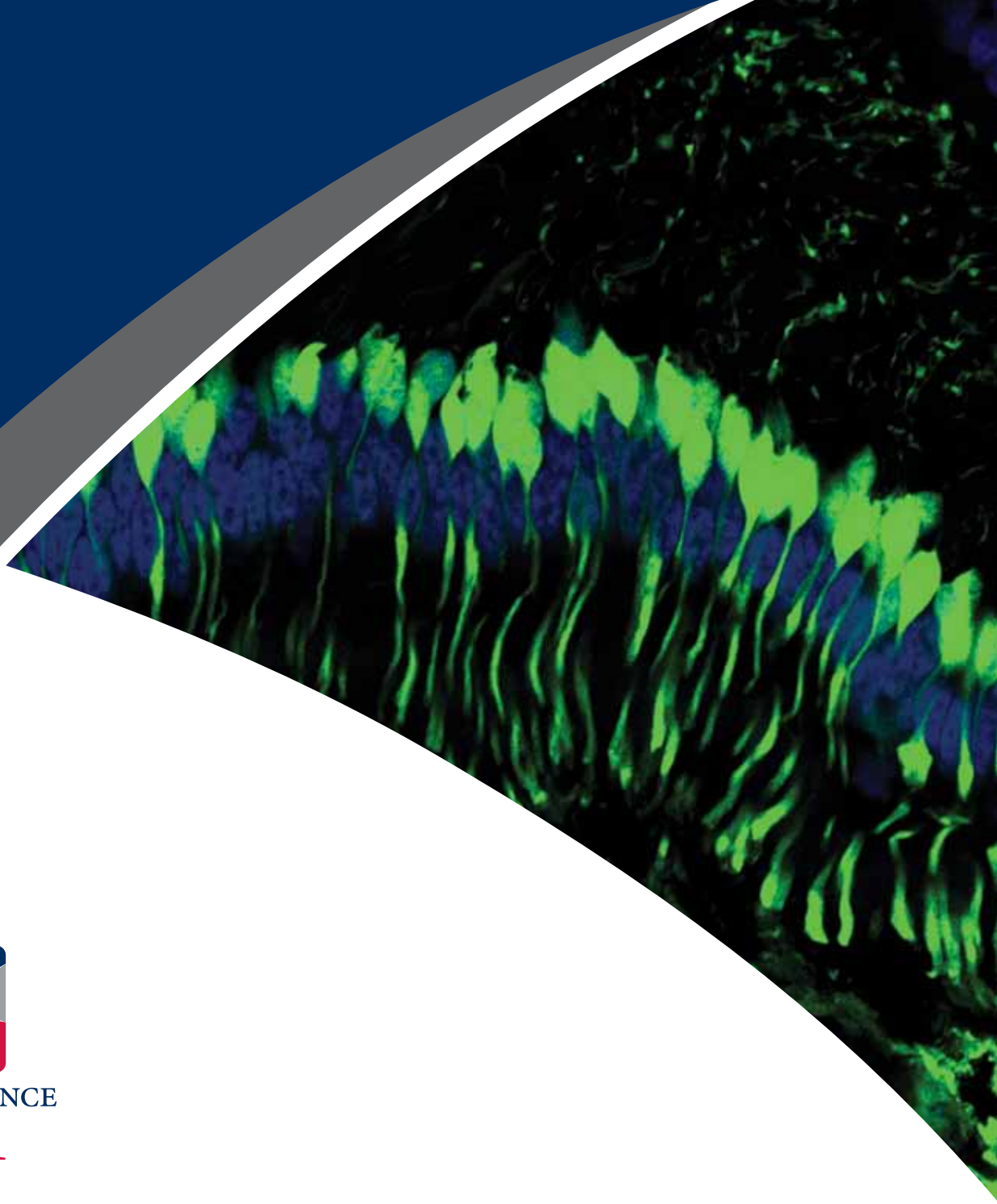

NEUROSCIENCE 2011

SHORT COURSE I

Gene Vector Design and Application to Treat Nervous System Disorders

Organized by Joseph C. Glorioso, PhD

SOCIETY FOR NEUROSCIENCE

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[AUTHOR'S LAST NAME, AUTHOR'S FIRST & MIDDLE INITIALS] (2011)
[CHAPTER TITLE] In: Gene Vector Design and Application to Treat Nervous System Disorders.
(Glorioso J, ed) pp. [xx-xx]. Washington, DC: Society for Neuroscience.

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Introduction

The neurobiology of human disease is a rapidly progressing field and encompasses a broad range of pathological processes that include abnormal development, metabolism, cognitive and emotional states, memory and sensory functions. The nervous system is also afflicted with unique forms of autoimmune processes, malignancy, infections, and trauma. These disease processes can affect peripheral and central nervous system functions either independently or through their connected activities and related structures. The nervous system is by far the most complex and difficult to study of the organ systems and most of our most elusive and pervasive disease processes arise in the nervous system. The nervous system detects our environment, interprets our world, and directs our activities.

Many of the nervous system's pathological states are coming to light, although tremendous gaps in our understanding of disease mechanisms remain. Indeed, fundamental brain processes such as memory storage and recall, consciousness, and integrative functions are nearly completely mysterious. Most serious diseases that involve degenerative processes such as Huntington's, Parkinson's, and Alzheimer's disease are without lasting or even palliative treatments. Therapies that involve the use of pharmaceutical approaches often have nonspecific and global unwanted effects and many drugs in use to treat brain-related diseases are used for off-target applications. Our best insights into disease processes center on single gene defects and metabolic disturbances. However, even in these instances, interventions to correct disease processes are disappointingly limited. Moreover, the protective blood-brain barrier (BBB) can inhibit treatment approaches even though the BBB is crucial to protecting the brain. The application of genetic approaches to develop nervous system therapies may offer an important approach to correction of nervous system diseases, and clearly there is a great deal of interest in disease prevention, progression, and nervous system repair through the delivery of functional genes to affected nerve tissues. These interventions generally fall under the purview of gene therapy.

The goal of this workshop is to provide information on the latest gene therapy vehicles and delivery methods in which treatment of animal models of nervous system disease has proven effective and early human clinical trials suggest that applied gene therapy can be translated to humans. While we are still in the early phases of the development of this technology, gene therapy will very likely become standard medical practice for many nervous system-related diseases where well-accepted treatment practices simply may not work. This is largely because nervous system disease often involves multiple cellular systems where drug-related therapy will not be sufficient to tackle problems inherent in complex systems.

We have brought together experts in the gene therapy field that include those in vector biology and design, in the application of gene therapy to clinical problems, and gene therapy pioneers who can provide insight into the potential future of gene therapy applications to human disease. The goal of this workshop is to open discussion of the utility of gene therapy for nervous system disease conditions, the future of clinical research, and prospects for first successes.

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