

FULL SPEAKER BIOGRAPHY and ABSTRACT

Steven Goldman, MD, PhD Rochester University

Dr. Steven A. Goldman is the Edward and Alma Vollertsen Rykenboer Professor and Chairman of the Department of Neurology at the University of Rochester, and Neurologist-in-Chief of Strong Memorial Hospital. He is also Chief of the Department's Division of Cell and Gene Therapy, and Co-Director, with Dr. Maiken Nedergaard, of the Center for Translational Neuromedicine at Rochester. Goldman holds additional appointments as a Professor of Neurosurgery, and as the Dean Zutes Chair in Biology of the Aging Brain. Goldman moved to Rochester in 2003 from the Weill Medical College of Cornell University, where he was the Nathan Cummings Professor of Neurology and Neuroscience, and Attending Neurologist at New York Presbyterian Hospital. A summa cum laude graduate of the University of Pennsylvania, he obtained his PhD from Rockefeller University in 1983, and his MD from Cornell in 1984. His thesis work, with Fernando Nottebohm at Rockefeller, included the first report of neuronal production and migration in an adult vertebrate brain, specifically of neurogenesis in the adult songbird brain. Dr. Goldman then interned in Medicine and completed his residency in Neurology at New York Hospital-Cornell and at the Memorial Sloan-Kettering Cancer Center. In 1988, after serving as Chief Resident in Neurology, he joined the faculty at Cornell, where he worked until moving to Rochester, and where he remains an Adjunct Professor of Neurology. Dr. Goldman is interested in cell genesis and neural regeneration in the adult brain and spinal cord, with a focus on the use of neural stem and progenitor cells in treating demyelinating and neurodegenerative diseases. His lab focuses on both the induction of endogenous progenitor cells for the treatment of neurodegenerative disorders, such as Huntington's Disease, and the transplantation of lineage-restricted progenitors for the treatment of glial and neuronal diseases of single phenotype, such as the pediatric leukodystrophies and Parkinson's Disease. He also has a strong interest in the signaling dysregulation that occurs in the conversion of endogenous stem and progenitor cells into brain cancers. He has published over 200 papers in his field, and over 100 as first or senior author. Dr. Goldman is a recipient of the Jacob Javits Neuroscience Investigator Award of the National Institutes of Health, and has been elected to the Association of American Physicians, as well as the American Neurological Association and the American Society for Clinical Investigation. He is also a member of the American Academy of Neurology and American College of Physicians, the Society for Neuroscience, the American Society for Gene Therapy, the Society for Neuro-oncology, and the ISSCR. Dr. Goldman remains active clinically, with subspecialty interests in neuro-oncology, stroke and the demyelinating diseases. His current work is supported by the NINDS, as well as by the Mathers, Adelson, McDonnell and CHDI Foundations, the National Multiple Sclerosis Society, the NY State Embryonic Stem Cell Research Program, and by Sanofi-Aventis. He and his wife Maiken Nedergaard, a neurobiologist and Professor of Neurosurgery at Rochester, live in Webster, New York with their 5 children, aged 9-19.

Stem and progenitor-cell based treatment of myelin disorders

Diseases of glial cells – which include both astroglia and myelin-producing oligodendroglia - may provide readily accessible targets for cell-based therapies, given the relative uniformity of CNS glial populations. The myelin diseases, which involve the loss or dysfunction of oligodendroglia in the brain and spinal cord, are among the most prevalent and disabling conditions in neurology. This talk will focus on the potential utility of glial progenitor cell transplantation as a therapeutic strategy for both congenital and acquired diseases of myelin. In addition, it will cover the molecular control of human glial progenitor cells, from the standpoint of establishing strategies for their mobilization and directed differentiation *in vivo*. We will also discuss the utility of human glial chimeric mice, as models by which to study the physiology of human glia and their progenitors in the live adult brain.

What is the central hypothesis of my presentation?

That glial progenitor cells may be suitable for cell-based treatment of glial disorders, in particular those of central myelin. As a secondary hypothesis, that animals engrafted neonatally to yield human glial chimeric brains may comprise appropriate models for assessing human glial physiology and pathology *in vivo*.

What is the most important observation I will discuss?

That congenitally hypomyelinated animals may be completely remyelinated and phenotypically rescued by neonatal transplantation of human glial progenitor cells.

What is the translational significance?

This strategy may comprise a feasible approach for treating both congenital and acquired diseases of myelin, as well as of other hereditary-metabolic diseases of the nervous system.