

Nicholas J. Maragakis, M.D

Dr. Maragakis is a Professor of Neurology and the Director of the ALS Center for Cell Therapy and Regeneration Research at the Johns Hopkins University School of Medicine. He also serves as the Co-Director of the Johns Hopkins ALS Clinic. A common theme to his research has been in the study of the astrocyte biology and its role in disease pathogenesis with a particular emphasis in how astrocytes may contribute to Amyotrophic Lateral Sclerosis (ALS).

A second major theme has been focused on the role of glial progenitor biology and, particularly, his laboratory's interest in using glial restricted precursors (GRPs) for transplantation into the spinal cords of ALS rodent models. This strategy is founded in the concept that astrocytes play a role in ALS disease progression. His group has provided *in vivo* support for this hypothesis and also demonstrated that the transplantation of wildtype GRPs can be neuroprotective in ALS models. This work has progressed from the most fundamental studies of glial progenitor biology to the point of bringing a Phase I/IIa clinical trial proposal to the FDA for the transplantation of human GRPs into the spinal cords of ALS patients.

His laboratory, in collaboration with others, has been interested in the development of induced pluripotent stem cells from ALS patients. We have collected over 150 ALS patient fibroblast samples and created over 50 iPSC cell lines from familial and sporadic ALS patients as well as controls. His current efforts have been to characterize iPSC-derived motor neurons and astrocytes both *in vitro* and *in vivo* with an effort towards understanding disease mechanisms; in particular modeling ALS disease heterogeneity with regard to disease progression.

Dr. Maragakis has also been heavily involved in clinical research as the principal investigator, site principal investigator, or co-investigator of numerous clinical trials in ALS, many coordinated by the Northeast ALS Consortium on which he served an Executive Board member. He is currently the principle investigator of a multicenter clinical trial to study the role of resistance and endurance exercise in ALS and has launched an ALS Telemedicine Program at Johns Hopkins to deliver care to ALS patients remotely. He is also the Clinical Co-Principal Investigator of the multicenter "Answer ALS" research program. The goal of "Answer ALS" is to create a large repository of iPSCs for genomic, proteomic, and metabolomics analysis. Bio-fluid samples, gene sequencing, and carefully collected longitudinal clinical phenotype information will also be obtained.