Alzheimer’s disease (AD), Parkinson’s disease (PD), Huntington’s disease (HD), amyotrophic lateral sclerosis (ALS) and frontotemporal dementia (FTD) are all devastating fatal neurodegenerative diseases. Despite years of effort, no therapeutic strategy has succeeded in significantly slowing the progression of any of these diseases, suggesting that the critical mechanisms of disease have not yet been targeted effectively. We have focused on using family-based whole genome analysis of patients, human neuron models of disease developed from patient stem cells, deep learning, and single cell analysis methods to understand the predictive value of specific pathways and molecules for neurodegeneration. Protein dyshomeostasis in general and autophagy in particular has emerged as an important pathway, and efforts to target it with small molecules show promise.

**PROGRESS IN NEUROSCIENCE**

**PINS**

Seminar Series of the Brain & Mind Research Institute Weill Cornell Medical College (WCMC) & The Graduate Program in Neuroscience of WCMC and Sloan Kettering Institute

Thursday, 1/28/16, 4 PM, coffee at 3:45 PM Weil Auditorium

“Insights from genomics, stem cells and artificial intelligence into neurodegenerative diseases and strategies to treat them”

Steve Finkbeiner, M.D., Ph.D. Professor, Departments of Neurology and Physiology, UCSF Senior Investigator & Associate Director, Gladstone Institute of Neurological Disease

**Abstract:**

Alzheimer’s disease (AD), Parkinson’s disease (PD), Huntington’s disease (HD), amyotrophic lateral sclerosis (ALS) and frontotemporal dementia (FTD) are all devastating fatal neurodegenerative diseases. Despite years of effort, no therapeutic strategy has succeeded in significantly slowing the progression of any of these diseases, suggesting that the critical mechanisms of disease have not yet been targeted effectively. We have focused on using family-based whole genome analysis of patients, human neuron models of disease developed from patient stem cells, deep learning, and single cell analysis methods to understand the predictive value of specific pathways and molecules for neurodegeneration. Protein dyshomeostasis in general and autophagy in particular has emerged as an important pathway, and efforts to target it with small molecules show promise.

**Recent relevant publications:**


