Philip C. Wong, Ph.D.
Departments of Pathology and Neuroscience
The Johns Hopkins University School of Medicine

“The RNA-binding Protein, TDP-43, in Health and Disease”

DP-43 proteinopathy is a pathological feature occurring in a number of human diseases including amyotrophic lateral sclerosis (ALS), frontotemporal dementia (FTD), Alzheimer’s disease and inclusion body myositis (IBM). A poor understanding of TDP-43’s disease mechanism, however, has limited our ability to design effective therapies for these disorders. We recently revealed that TDP-43 represses non-conserved cryptic exons and this critical role of TDP-43 is compromised in ALS and FTD. In addition, we have identified a set of RNA targets of TDP-43 in cell culture models. However, it is not known whether TDP-43 also regulates a set of tissue-specific targets. Using mice lacking TDP-43 in either neurons or skeletal muscles, we plan to identify a set of neuron- or skeletal muscle-specific RNA targets of TDP-43 associated with non-conserved cryptic exons by RNA-seq analysis. Results of these ongoing studies will be presented. We believe that outcomes from these studies will have important implications for human diseases such as IBM and neurodegenerative diseases.

TUESDAY December 1, 2015
3:00-4:15 PM
JC Meeting Room F