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, 12/12/13, 4 PM, coffee at 3:45 PM
Weill Auditorium

Emerging Therapies for Inherited Neurodegenerative Disease

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Abstract:

Davidson has pioneered the development and application of brain-targeted gene-silencing technologies to treat neurodegenerative diseases. Gene silencing harnesses a powerful natural process called RNA interference to turn off production of toxic proteins. Davidson's group has developed reagents for expressing inhibitory RNA in vivo. This approach improved disease phenotypes in relevant models of dominantly inherited human neurodegenerative diseases. Davidson's work may help lead to therapies for conditions such as Huntington's disease and Spinocerebellar ataxia. For recessive diseases, strategies to supplement the missing protein are being developed. Novel approaches for tackling the CNS manifestations of these different forms of inherited diseases will be presented.

Recent relevant publications:

Recent advances in RNA interference therapeutics for CNS diseases. Ramachandran, P.S., Keiser, M.S., Davidson, B.L. *Neurotherapeutics*. 2013 Jul;10(3):473-85.

Molecular signatures of disease brain endothelia provide new sites for CNS-directed enzyme therapy. Chen, Y.H., Davidson, B.L., *Nat Med*. 2009 Oct;15(10):1215-8. doi: Epub 2009 Sep 13.

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