



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, 1/8/15, 4 PM, coffee at 3:45 PM
Weill Auditorium

"Amyotrophic Lateral Sclerosis: Update on Genetics and New Animal Models"

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Abstract:

Amyotrophic lateral sclerosis (ALS) is a progressive motor neuron disease that is uniformly fatal, typically in less than five years. More than 40 genetic variants have been detected that either cause modify the course of ALS. This talk will describe recent themes from ALS genetics and new models based on ALS genes (C9orf72 and TDP43). The development of new ALS models *in vitro* and *in vivo* supports the hope that meaningful treatments for ALS will be found in the near term.

Recent relevant publications:

1. Rosen DR, Siddique T, Patterson D, Figlewicz DA, Sapp P, Hentati A, Donaldson D, Goto J, O'Regan, Den H, Rahamani Z, Krisus A, Berger R, Tanzi RE, Halperin JJ, Herzfeldt B, Van den Bergh R, Hung W, Bird T, Deng G, Mulder DW, Smyth C, Nigel G, Soriano E, Pericak-Vance MA, Haines J, Rowleau GA, Gusella JS, Horvitz RH, Brown RH Jr. Mutations in Cu/Zn superoxide dismutase gene are associated with familial amyotrophic lateral sclerosis. *Nature* 1993;362:59-62. PMID: 8446170
2. Kwiatkowski TJ, Bosco DA, LeClerc AL, Tamrazian E, VandenBerg CR, Russ C, Davis A, Gilchrist J, Kasarskis EJ, Munsat T, Valdmanis P, Rouleau GA, Hosler BA, Cortelli P, de Jong PJ, Yoshihaga Y, Haines JL, Pericak-Vance MA, Yan J, Ticozzi N, Siddique T, McKenna-Yasek D, Sapp, PC, Horvitz HR, Landers JE, Brown RH, Jr. Mutations in the FUS/TLS gene on chromosome 16 cause familial amyotrophic lateral sclerosis. in a novel ALS gene. *Science* 2009; 323:1205-8. PMID: 19251627
3. Wu C-H, Fallini C, Ticozzi N, Keagle PJ, Sapp PC, Piotrowska K, Lowe P, Koppers M, McKenna-Yasek D, Baron D, Kost E, Gonzalez-Perez P, Fox AD, Adams J, Taroni F, Tiloca C, Leclerc AL, Chafe SC, Mangroo D, Moore MJ, Zitzewitz J, Xu Z-S, van den Berg LH, Glass JD, Siciliano G, Cirulli ET, Goldstein DB, Salachas F, Meninger V, Rossoll W, Ratti A, Gellera C, Bosco DA, Bassell GJ, Silani V, Drory VE, Brown RH Jr., Landers JE. Mutations in the profilin 1 gene cause familial amyotrophic lateral sclerosis. *Nature*. 2012 Aug 23;488(7412):499-503. PMID: 22801503



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