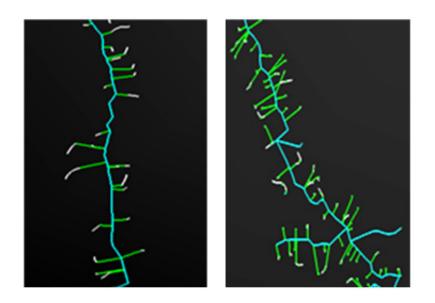


Effective drugs for Parkinson's reduce symptoms of Rett syndrome in mice

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The nerve endings (dendrites of neurons) are scarce in Rett syndrome (left). Treatment with the drugs described in the article increases the nerve endings, thereby enhancing neural activity (right). Credit: IDIBELL

IDIBELL researchers, led by the director of the Program for Epigenetics and Cancer Biology, ICREA researcher and Professor of Genetics at the University of Barcelona, Manel Esteller, have shown that a combination of effective drugs for Parkinson's disease in mice that are used as a model of human Rett syndrome reduces some of the symptoms associated with this disease. The results of the study are published in the journal *Neurophsycopharmacology*



Second leading cause of mental retardation in females

Rett syndrome is the second most common cause of <u>mental retardation</u> in women, after Down syndrome. It is a neurodevelopmental disease whose clinical picture begins to appear 6-18 months after birth and involves a loss of intellectual, social and motor skills, accompanied by autistic behaviors, such as repetitive movements of the hands.

The syndrome is usually due to the presence of a mutation in the MECP2 gene, an epigenetic gene which controls the activity of many other genes like a padlock. Today there is no effective treatment of the disease. Manel Esteller's group, in collaboration with the group of neurometabolic diseases IDIBELL led by Aurora Pujol, described this week in the journal Neuropsycopharmacology how drug treatment in mice used as models of Rett syndrome reduces some of the symptoms associated with the disease .

Altered production via Dopamine

"Six years ago, studying the brains of mice that faithfully present the same characteristics of human Rett syndrome, we found that there was an alteration in the way of production of dopamine, a neurotransmitter. Here, Rett syndrome bore some resemblance to Parkinson, which also presents defects in the same molecule.

There are effective drugs in Parkinson's so we decided to study whether they could also function in Rett syndrome, "says Manel Esteller. "We found that combined treatment with L-Dopa and Dopa decarboxylase inhibitor reduces typical manifestations of the disease and mobility defects, tremor and respiratory distress in these animals."

"This is not a panacea or a magic pill or" warns Esteller "but at least is a



starting point to study whether it may also be useful in controlling the symptoms of Rett syndrome in humans."

More information: Improvement of the Rett Syndrome Phenotype in a Mecp2 Mouse Model Upon Treatment with Levodopa and a Dopa Decarboxylase Inhibitor. Szczesna K, de la Caridad O, Petazzi P, Soler M, Roa L, Saez MA, Fourcade S, Pujol A, Artuch-Iriberri R, Molero-Luis M, Vidal A, Huertas D, Esteller M. *Neuropsychopharmacology*, <u>DOI: 10.1038/npp.2014.136</u>, 2014.

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