Transgenic Rodent Models of Parkinson's Disease: Human LRRK2 Knockin

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Technology description

Parkinsons disease (PD) is a degenerative disease of the brain that often impairs motor skills, speech, and other functions. PD is both chronic and progressive. Currently there is no cure, and animal models for studying the disease are poor.

PD may be inherited or acquired. Although PD patients who inherit the disease are only 10% of PD population, the study of what is going on genetically in people who inherit PD is very important for understanding how the disease works.

A few years ago, investigators learned that a certain protein, leucine-rich repeat kinase2 (LRRK2), is mutated in a large set of inherited cases of PD (those with the "Park8" type of PD). Further, the mutation in the gene makes it more active than normal (instead of less active). This makes LRRK2 an attractive target for drug development, since it is easier to make a drug turn something off, than it is to make a drug turn something on.

Using funding from the Michael J Fox Foundation, investigators at the Weill Cornell Medical College have generated mice and rats that contain the mutated, human LRRK2 gene. These animals faithfully reproduce the hallmarks of human PD, including drug response, making them excellent models for testing drugs to treat PD.

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