Drug discovery based on Alzheimer's disease gene mutations

Technology #538

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Gene mutations for Alzheimer’s drug screening: Alzheimer’s disease is a devastating disease afflicting millions, and its prevalence is rapidly increasing. Although a number of treatments are available, they function almost exclusively to palliate symptoms, rather than reverse damage or halt disease progression. A number of common and rare genetic mutations have been associated with the disease, and these are promising targets for drug development. In particular, mutations of presenilin 1 and 2 have been associated with familial early-onset AD, inherited in an autosomal dominant manner. In these cases, the alteration of presenilin’s function leads to the excessive accumulation of amyloid-beta that is synonymous with AD.

Gene mutations used as a screening tool in Alzheimer’s drug development: The inventors have discovered and characterized two genes, hop-1 and sel-12, in C. elegans that display high homology to human presenilins. The inventors then created mutations in these genes homologous to those that cause AD in humans. These mutations lead to an observable phenotype, constitutive egg-laying. This phenotype can then be utilized as a screening mechanism to identify novel compounds that may be useful in treating AD.

Applications: • Novel target and biological screen for Alzheimer’s drug development • Sequence data and antibodies may be useful for development of Alzheimer’s diagnostics

Advantages: • Organism-based screen: using a phenotypic screen can identify drug candidates that would be missed in protein target-based screens • High-throughput: C. elegans is the multicellular model organisms best suited for high-throughput screening


Licensing Status: Available for Licensing


Inventors

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