A Method for Drug Screening In Vivo Using a Drosophila Model of ALS

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Technology description

Invention
UA13-079 is a method for performing drug screens to test candidate compounds for their therapeutic effectiveness in treating locomotor and neurodegenerative defects in a drosophila model of the human disease Amyotrophic Lateral Sclerosis (ALS). The drug screen consists of testing the ability of candidate compounds to rescue deleterious phenotypic effects induced by mutations/functional alterations to TDP-43 in the drosophila model.
Background
Amyotrophic Lateral Sclerosis (ALS) is an adult onset, progressive neurological disorder characterized by selective degeneration and death of motor neurons in the motor cortex and the spinal cord. The ALS pathology includes ubiquitin positive cytoplasmic bodies, which have been shown to contain a 28 kDa fragment corresponding to the C-terminus domain of TDP-43 protein together with the full length TDP-43. Several missense mutations have been identified in TDP-43, the majority of which lie within the C-terminal region, indicating that this domain can be involved in the pathogenesis of ALS. Pathways and compounds with neuroprotective potential for TDP-43-associated phenotypes can be determined, which can impact a wide spectrum of ALS cases. Despite advances in understanding the physiology and pathophysiology of amyotrophic lateral sclerosis, there is still a scarcity of compounds that are potent, efficacious, and safe in the treatment or amelioration of amyotrophic lateral sclerosis.

Application area

Used as potential therapeutic targets to develop new drugs or design clinical trials.

Advantages

The potential to identify novel therapeutics for other neurodegenerative diseases which have been linked to TDP-43, including Alzheimer’ s and Fronto-Temporal Lobar Degeneration (FTLD).
May result in the ability to treat a wide variety, if not the majority of ALS patients

Institution

University of Arizona

Inventors

Patricia Estes
Assistant Staff Scientist
Molecular & Cellular Biology
Daniela Zarnescu
Professor
Molecular & Cellular Biology