Therapeutic targets for Spinal Muscular Atrophy and Amyotrophic Lateral Sclerosis

Technology #cu13238

This technology identifies target genes that can be modulated to treat Amyotrophic Lateral Sclerosis (ALS) and Spinal Muscular Atrophy (SMA).

Unmet Need: Improved therapeutics for the treatment of SMA and ALS

SMA and ALS are motor neuron diseases characterized by muscle weakness and motor neuron degradation that can ultimately lead to death by respiratory failure. Current treatment options are limited, carry the risk of serious side effects, and only slow the progression of symptoms. As such, there is a need for improved therapeutics with reduced side effect profiles that can treat both diseases and prevent disease progression.

The Technology: Target genes identified for the versatile treatment of SMA and ALS

This technology identifies over 37 genes as possible therapeutic targets for the treatment and prevention of SMA and ALS. Identification of the therapeutic targets was achieved via a mouse model of SMA, autopsies of SMA patients, and analysis of previously published gene expression data. This technology has identified both vulnerability gene variants, that are indicative of SMA, and resistant gene variants, that confer resistance to SMA. Therapeutics that inhibit vulnerability genes may reduce susceptibility of motor units to disease development, whereas activation of resistance genes may improve motor unit resistance to disease. Consequently, this technology could be used to design therapeutics that both treat and prevent the progression of debilitating symptoms associated with SMA and ALS.

Applications:

- Therapeutic targets for the treatment and prevention of SMA and ALS
- Diagnostic assay to predict genetic susceptibility to SMA and ALS
- Identified targets may be also be relevant to additional neurodegenerative diseases
Advantages:

- Identifies specific gene targets involved in disease susceptibility and resistance
- May improve speed and accuracy of drug development for neurodegenerative diseases

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Patent Information:

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Related Publications:


Tech Ventures Reference:

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