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Therapeutic target for treatment of ataxia

A newly identified pathway that represents a therapeutic target for the prevention and treatment of ataxia.

IP Status: Issued US Patent; Issued Patent No. 10,973,812

Applications

- Patients with ataxia
- Patients at risk of ataxia
- Damaged Purkinje cells

Benefits & Features

- Cholecystokinin receptor (Cck1R) agonist
- Treats and may even prevent ataxia
- Upregulates Cck gene
- Protects Purkinje cells against atrophy and death

Technology Overview

A Cck1R agonist may treat symptoms of ataxia. Cerebellar ataxia disorders occur when cerebellar Purkinje cells degenerate and die. The protective pathway identified involves upregulating the Cck gene by administering a Cck1R agonist. When Cck (or Cck1R) was knocked out in mice models, the mice showed progressive ataxia, Purkinje cell degeneration, and death, but administering a Cck1R agonist activated Cck1R to protect against progressive disease. The Cck-Cck1R pathway represents a therapeutic target for protecting the Purkinje cells against atrophy and death. It may be an effective treatment for patients suffering from ataxia as well as those at risk, even before symptoms occur.

Currently, no treatments can prevent or slow ataxia, and any treatments that do exist merely aim to alleviate symptoms. This method shows that upregulation of cholecystokinin (Cck) and the subsequent interaction with the cholecystokinin receptor (Cck1R) halts progressive Purkinje cell pathology and can effectively treat—or even prevent—ataxia.

Phase of Development

TRL: 2-3
Proof of concept. In vivo genetic proof of concept; in vivo pharmacology proof of concept in two genetic models of ataxia.

Desired Partnerships

This technology is now available for:

- License
- Sponsored research
- Co-development

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Researchers

Technology ID

20160307

Category

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