Therapeutic Target for Treatment of Ataxia

Elevated Cck expression protects Purkinje cells

A cholecystokinin receptor (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.

Treats and prevents ataxia

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 Cck1 receptor halts progressive Purkinje cell pathology and can effectively treat—or even prevent—ataxia.

BENEFITS AND FEATURES:

- Cholecystokinin receptor (Cck1R) agonist
- Treats and may even prevent ataxia
- Upregulates Cck gene

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• Protects Purkinje cells against atrophy and death

APPLICATIONS:
• Patients with ataxia
• Patients at risk of ataxia
• Damaged Purkinje cells

Phase of Development - Proof of concept. In vivo genetic proof of concept; in vivo pharmacology proof of concept in two genetic models of ataxia.

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<td>The University relies on industry partners to scale up technologies to large enough production capacity for commercial purposes. The license is available for this technology and would be for the sale, manufacture or use of products claimed by the issued patents. Please contact Kevin Anderson to share your business needs and technical interest in this technology and if you are interested in licensing the technology for further research and development.</td>
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