



Tat-Utrophin as a Protein Therapy for Muscular Dystrophies

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The Wisconsin Alumni Research Foundation (WARF) is seeking commercial partners interested in developing a fusion protein for the treatment of muscular dystrophies.

Overview

No effective therapy currently exists for the most common and severe form of muscular dystrophy disease, Duchenne Muscular Dystrophy (DMD). This disease, which is characterized by the weakening of voluntary muscles, is caused by the lack of a functional version of the muscle protein dystrophin.

Another muscle protein, utrophin, has a similar sequence and has been used to compensate for the loss of dystrophin in a mouse model. However, up-regulating utrophin or increasing the expression of functional dystrophin in muscle cells is difficult and will not be a feasible treatment option until clinically approved methods of gene therapy are developed.

The Invention

UW-Madison researchers have developed a fusion protein between utrophin and the TAT sequence from HIV, which is used to transport molecules into cells, for the treatment of muscular dystrophies, including DMD. This protein-based therapeutic provides the first effective treatment for these currently fatal conditions.

The TAT sequence provides a cell signal that leads to the internalization of utrophin in muscle cells. Once in the muscle cells, the utrophin is able to compensate for the lack of dystrophin, reducing or eliminating the disabling effects of dystrophinopathic conditions.

The inventors successfully treated a mouse model for DMD by injecting the fusion protein intraperitoneally. The TAT-utrophin localized to the target area of muscle, resulting in significant improvement in several key disease parameters.

Applications

- Treatment of muscular dystrophies, including DMD

Key Benefits

- Because it is protein-based, this treatment circumvents the need for gene therapy.
- Relatively low cost and low risk
- Because utrophin is not toxic, the fusion protein can be administered in relatively high doses, making it easier to transduce therapeutically effective amounts into muscle cells

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- Truncated versions of the fusion protein may be used to improve protein transduction *in vivo*.

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Additional Information

Related Intellectual Property

- [View Continuation Patent in PDF format.](#)

Tech Fields

- [Therapeutics & Vaccines : Musculoskeletal](#)

For current licensing status, please contact Andy DeTienne at adetienne@warf.org or 608-960-9857

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