

In A Nutshell
October 2017

Gene Therapy

One of the most promising advancements in MS research is a gene therapy which has reversed MS in mice. And, it was based on research done at our own University of Florida.

By combining a brain-protein gene and an existing medication, the researchers were able to prevent the mouse version of multiple sclerosis. Likewise, the treatments produced near-complete remission in the animal models.

The researchers used a harmless virus, known as an adeno-associated virus, to deliver a gene responsible for a brain protein into the livers of the mouse models. The virus sparked production of so-called regulatory T cells, which suppress the immune system attack that defines multiple sclerosis. The gene was targeted to the liver because it has the ability to induce immune tolerance.

A group of five mouse models that received the gene therapy did not develop experimental autoimmune encephalomyelitis, which is the mouse equivalent of multiple sclerosis in humans. In another experiment, all but one mouse model showed a significant reversal of the disease eight days after a single gene therapy treatment.

When the protein was combined with rapamycin -- a drug used to coat heart stents and prevent organ transplant rejection -- its effectiveness was further improved, the researchers found. The drug was chosen because it allows helpful regulatory T-cells to proliferate while blocking undesirable effector T-cells'.

Before the therapy can be tested in humans during a clinical trial, further research involving other preclinical models will be needed.

Just For Fun

Can we dare to be too hopeful about this therapy?
Here are some songs that deal with hope. Do you have a favorite?

"Here Comes The Sun," Beatles
"Things can Only Get Better," Howard Jones.
"Don't Stop," Fleetwood Mac
"Don't Stop Believin'" Journey
"I Can See Clearly Now," Jimmy Cliff
"Rise," Eddie Vedder
"We are the World," Various
"Keep Holding On," Avril Lavigne
"Don't Give Up," Peter Gabriel and Kate Bush
"I Believe I can Fly," R Kelly

<https://www.news-medical.net/news/20170921/Novel-gene-therapy-can-inhibit-reverse-multiple-sclerosis-in-mouse-models.aspx>

<http://news.ufl.edu/articles/2017/09/unique-gene-therapy-prevents-reverses-multiple-sclerosis-in-animal-model.php>

[http://www.cell.com/molecular-therapy-family/molecular-therapy/fulltext/S1525-0016\(17\)30413-6](http://www.cell.com/molecular-therapy-family/molecular-therapy/fulltext/S1525-0016(17)30413-6)

<http://www.mademan.com/mm/10-best-songs-about-hope.html>