

Help may be on the way for people with muscular dystrophy

March 27 2019, by Marcia Heroux Pounds



Credit: CC0 Public Domain

Researchers in Florida never expected this much success with a drug they're developing to treat muscular dystrophy.

"The results look better than we could have imagined," said Matthew Disney, the Scripps Florida scientist leading the research.

The [potential drug](#) improved muscle defects with no apparent side effects in tests using mice, Scripps researchers say. Human trials still have to be conducted.

The research targets the most common form of muscular dystrophy in adults, [myotonic dystrophy](#) type 1, which is estimated to affect around 1 in 8,000 people, according to the National Institutes of Health.

Doctors often misdiagnose the disease, meaning more people could actually be affected, said Molly White, CEO of the San Francisco-based Myotonic Dystrophy Foundation.

Genetic studies suggest the actual numbers are more than three times higher, around 1 in 2,500, she said.

Symptoms of the inherited disease emerge in late teens or early adulthood. They include muscle clenching lock-jaw, early-onset cataracts, brain fog, muscle wasting and weakness, digestive difficulties and sudden cardiac death, White said.

In 2018, Disney's [drug](#) development company Expansion Therapeutics became Scripps Florida's largest spinoff since the institute was founded in 2014. Expansion Therapeutics, which is developing drugs that target [muscular dystrophy](#), operates out of wet labs at Florida Atlantic University adjacent to Scripps Florida in Jupiter, as well as San Diego.

In 2015, Disney was a recipient of the "Pioneer Award" from the National Institute of Health, for scientists developing groundbreaking approaches.

In February, Disney and his research team announced the development of a compound that could help make breast cancer patients more receptive to treatment.

Scripps Florida opened in Jupiter in 2009, part of an effort to generate higher-paying science and technology jobs in Florida. Palm Beach County and the state invested more than \$600 million in taxpayer-backed incentives to bring the research institute to the area.

©2019 Sun Sentinel (Fort Lauderdale, Fla.)
Distributed by Tribune Content Agency, LLC.

Citation: Help may be on the way for people with muscular dystrophy (2019, March 27)
retrieved 2 May 2024 from

<https://medicalxpress.com/news/2019-03-people-muscular-dystrophy.html>

<p>This document is subject to copyright. Apart from any fair dealing for the purpose of private study or research, no part may be reproduced without the written permission. The content is provided for information purposes only.</p>
--