

Real-life spider men using protein found in venom to develop muscular dystrophy treatment

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UB researchers are developing a treatment for muscular dystrophy using a peptide found in the venom of a Chilean rose tarantula. Credit: University at Buffalo

While Spider-Man is capturing the imagination of theatergoers, real-life spider men in Upstate New York are working intently to save a young boy's life.

It all began in 2009, when Jeff Harvey, a stockbroker from the Buffalo suburbs, discovered that his grandson, JB, had Duchenne muscular dystrophy. The disease is fatal. It strikes only boys, causing their muscles to waste away.

Hoping to help his grandson, Harvey searched <u>Google</u> for promising muscular dystrophy treatments and, in a moment of serendipity,



stumbled upon University at Buffalo scientist Frederick Sachs, PhD.

Sachs was a professor of physiology and <u>biophysics</u> who had been studying the medical benefits of venom. In the venom of the Chilean rose <u>tarantula</u>, he and his colleagues discovered a protein that held promise for keeping <u>muscular dystrophy</u> at bay. Specifically, the protein helped stop <u>muscle cells</u> from deteriorating.

Within months of getting in touch, Harvey and Sachs co-founded Tonus Therapeutics, a pharmaceutical company devoted to developing the protein as a drug. Though the treatment has yet to be tested in humans, it has helped dystrophic mice gain strength in preliminary experiments.

The therapy is not a cure. But if it works in humans, it could extend the lives of children like JB for years -- maybe even decades.

Success can't come quickly enough.

JB, now four, can't walk down the stairs alone. When he runs, he waddles. He receives physical therapy and takes steroids as a treatment. While playing tee ball one recent day, he confided to his grandfather, "When I grow up, I want to be a baseball player."

It was a heartbreaking moment.

"Oh, I would be thrilled if you could be a baseball player," Harvey remembers replying. He's doing everything he can to make sure that JB -- and other boys like him -- can live out their dreams.

Provided by University at Buffalo

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