

# Tarantula venom-based MD therapy to be advanced by UB scientists' biotech company

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University at Buffalo biophysicists have found a protein in tarantula venom that shows promise as a potential therapy for muscular dystrophy (MD). They have formed a start-up biotech company in Buffalo -- Rose Pharmaceuticals -- to advance the drug to clinical trials.

Fredrick Sachs, PhD, professor of physiology and biophysics at the University at Buffalo, and colleagues in his laboratory, discovered the peptide, called GsMTx4.

Therapies for [muscular dystrophy](#) are classed as "orphan drugs" by the FDA, allowing a shorter testing period than normal drugs. Sachs said he anticipates Rose Pharmaceuticals may be able to obtain FDA approval of the peptide for human use within two years.

The new company is named for "Rose," the pet tarantula that has been in residence in Sachs' lab for nearly 20 years.

The first target of the peptide is MD, a condition Sachs has been investigating for several years, but the peptide also has potential as a therapy for several other conditions, such as neuropathic pain and atrial fibrillation. Formation of the company was motivated by the goal of finding an MD therapy for the grandson of Sachs' friend Jeffrey Harvey.

The start-up is a collaboration between Sachs, Harvey, Thomas Suchyna, PhD, and Philip Gottlieb, PhD. Suchyna and Gottlieb, UB research scientist and UB research associate professor, respectively, have been

working with Sachs at the university for several years to develop the peptide. Their work was supported by a grant from UB's Interdisciplinary Research and Creative Activities Fund.

In collaboration with Eric Hoffman, PhD, director of the Wellstone Muscular Dystrophy Center at Children's National Medical Center in Washington, D.C., the team tested the effect of GsMTx4 on MD mice extensively. Results showed that the drug increased muscle strength and caused no [mortality](#), [morbidity](#) or toxicity.

Rose Pharmaceuticals now is concentrating on developing methods to administer the drug. The peptide and its mirror image are covered by U.S. patents obtained by UB's Office of Science, Technology Transfer and Economic Outreach (STOR), and licensed to Rose Pharmaceuticals. Sachs noted that there are no other drugs known to act specifically on mechanosensitive ion channels, the target of GsMTx4.

"Unlike most drugs, GsMTx4 seems to generate only positive side effects," said Sachs. "In addition to its effectiveness in MD, it inhibits atrial fibrillation, a cardiac arrhythmia that affects 2 million Americans, and for which there currently is no reliable drug therapy.

"In a second application, research groups in Korea and UC San Francisco have shown that GsMTx4 can inhibit mechanically induced pain (pain originating in nerve fibers)," he said. "This therapy is at least half as effective as morphine, but does not act on the brain, only at the site of increased sensitivity." Mark Kristal, PhD, UB professor of psychology, has been collaborating on the pain testing.

GsMTx4 appears to have additional applications. Robert Plunkett, MD., UB associate professor of neurosurgery, has shown that the peptide stimulates neuronal growth, and may be useful for the treatment of Parkinson's disease.

Provided by University at Buffalo

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