New muscular dystrophy treatment shows promise in early study

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A preclinical study led by researchers in the United States has found that a new oral drug shows early promise for the treatment of muscular dystrophy. The results, which are published today in *EMBO Molecular Medicine*, show that VBP15 decreases inflammation in mice with symptoms similar to those found in patients with Duchenne muscular dystrophy. The authors found that the drug protects and strengthens muscle without the harsh side effects linked to current treatments with glucocorticoids such as prednisone.

Duchenne muscular dystrophy results in severe muscle degeneration and affects approximately 180,000 patients worldwide, mostly children. Treatment with the current standard therapy, glucocorticoids, can only be used for a short time due to serious side effects leading to fragile bones and suppression of both the immune system and growth hormone production.

The researchers also observed that VBP15 inhibits the transcription factor NF-kB, a key cell-signaling molecule found in most animal cell types that plays a role in inflammation and tissue damage.

The study authors previously found out that NF-kB is active in dystrophin-deficient muscle years before the onset of symptoms, suggesting that very early treatment of Duchenne Muscular Dystrophy patients with VBP15 may prevent or delay the onset of some clinical symptoms.

"It is becoming increasingly clear that membrane integrity and repair are crucial factors in muscle, cardiovascular, neurodegenerative and airway disorders. The chemical properties of VBP15 also suggest potential for the treatment of other diseases." remarked Kanneboyina Nagaraju, DVM, PhD, the lead author of the study and a principal investigator in the Center for Genetic Medicine Research, Children's National Medical Center in Washington, DC. The authors conclude that VBP15 merits further investigation for efficacy in clinical trials.

More information: VBP15, a novel anti-inflammatory and membrane-stabilizer, improves muscular dystrophy without side effects, DOI: 10.1002/emmm.201302621

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