Muscular dystrophy: New drug promises benefit without risk of infection

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A new drug being studied for the treatment of muscle degenerating diseases has shown promising results. According to a study published today in the *British Journal of Pharmacology*, Debio 025 is as effective as current drugs but, crucially, does not cause unwanted immunosuppressive effects.

Bethlem Myopathy and Ullrich Congenital Muscular Dystrophy (UCMD) are muscle wasting diseases caused by deficiencies in collagen VI, a component of connective tissue. Patients are usually diagnosed at birth and suffer from muscle weakness that worsens over time. UCMD patients often also suffer respiratory failure, which is complicated by lung infections. Although the drug cyclosporin A (CsA) offers some benefit for these patients, its long term use may be undesirable because it interacts with calcineurin, an important immunoregulatory protein.

"Long-term treatment with CsA is risky because it suppresses the immune system, making patients more susceptible to life-threatening lung infections," says Paolo Bernardi, who led the team at the University of Padova in Italy. "Our findings suggest Debio 025 may provide a safer alternative".

In the study, mice suffering from a muscle-wasting disease similar to human muscular dystrophy were protected when treated with Debio 025. The researchers studied muscle cells from mice given the drug for five days and found decreases in numbers of abnormal muscle fibres similar to those reported in studies of treatment with CsA. They were also able

to show that Debio 025, although related to CsA, was not targeting calcineurin.

"This drug has no effects on the immune system and therefore could be used for prolonged periods of time without increasing risk of infection. We should be able to treat children affected by these forms of muscular dystrophy and possibly slow down or even stop the progression of the disease," says Bernardi.

Source: Wiley-Blackwell