**BBC News**

# Drug offers hope for treating muscular dystrophy

**By Helen Briggs** Health reporter, BBC News

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A drug that can boost muscle strength in mice shows promise as a possible treatment for Duchenne muscular dystrophy, research suggests.

The work could one day lead to a daily pill to treat all patients with the muscle-wasting disease, say Oxford University scientists.

About 100 boys are born with the condition in the UK each year.

It causes progressive muscle weakness, with most patients having to use a wheelchair by the age of 12.

The study, published in the journal PLoS ONE, was led by Professor Dame Kay Davies, of Oxford University.

She said: "We've shown that the drug can dramatically reduce muscle weakness in mice.

"These results give us everything we need to go forward into initial clinical trials in humans."

There is no effective treatment for the inherited disease - steroid and growth hormones help manage the symptoms but cannot protect muscles from decline.

**Muscular dystrophy**

* Muscular dystrophies are a group of more than 20 different genetic neuromuscular disorders
* The most common, Duchenne muscular dystrophy (DMD), affects about one in 3,500 boys
* Duchenne muscular dystrophy is caused by problems in a gene on the X chromosome that makes a protein called dystrophin, found in muscle fibres
* Muscle fibres break down and are gradually lost
* Another form - Becker muscular dystrophy - has similar but milder symptoms

The drug was identified by screening thousands of therapeutic compounds for the ability to raise levels of a substance that boosts muscle strength.

The substance - utrophin - seems to compensate for the lack of a key protein, dystrophin, which does not work properly in muscular dystrophy.

The most promising candidate, named SMT C1100, was tested in a mouse model of muscular dystrophy at three laboratories in Oxford, Italy and the US.

Mice given the drug developed stronger muscles that did not tire as easily and could run 50% further in exercise tasks, the PLoS ONE study found.

Preliminary tests in healthy human volunteers raised no safety concerns but suggest a need for further work to improve the drug's formulation.

Professor Max Parmar, of the Medical Research Council, said: "This study, without necessarily providing us with the final solution, does gives us an important platform from which to move forward and really make a serious progression through clinical trials."

Dr Marita Pohlschmidt, director of research at the Muscular Dystrophy Campaign, told the BBC: "At this stage in the game this is very hopeful as it really works in the mice very, very well.

"It's hopeful although you can never tell a family [affected by the disease] when or whether a treatment will come out of it."

The research was partly funded by the Muscular Dystrophy Campaign and the Medical Research Council.