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Cancer drug shows promise for treating Duchenne muscular dystrophy

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A drug commonly used to treat leukaemia is showing potential as a treatment that could slow the progression of the muscle-wasting condition, Duchenne muscular dystrophy.

Duchenne muscular dystrophy most commonly affects boys, with around 2,400 people in the UK affected by the condition. There is currently no cure and most patients are not expected to live past the age of 30.

Researchers at the University of Sheffield investigated a drug called dasatinib, which works by blocking certain chemical signals that stimulate the growth of cancer cells. They found the same drug will also switch off similar signals in a protein implicated in Duchenne Muscular Dystrophy (DMD). This protein, called dystroglycan, has a part to play in maintaining healthy muscle tissue.

The team tested the drug in zebrafish bred to carry DMD and recorded a 40 % improvement in the condition of the fish. Those fish treated with dasatinib were able to swim further and for longer than those in a control group. It could be that by combining the drug with other treatments currently under development, their effectiveness could be improved even further. The results are published in the journal *Human Molecular Genetics*.

"Dasatinib clearly has promise as a treatment for DMD," says Professor Steve Winder, who led the research. "From our understanding of how the drug works we believe it could be effective in slowing muscle

deterioration, prolonging patients' ability to walk and also protecting their heart and respiratory muscles. There is the potential that if the drug were taken immediately upon diagnosis, the disease progression could be dramatically reduced."

Because dasatinib is already cleared for clinical use, researchers hope that progress can be made more quickly towards trialling the drug in humans as a treatment for DMD. Experiments have already begun in mice, with promising results. Other drugs that work in a similar way to dasatinib are also under investigation by Professor Winder's team.

Dr Marita Pohlschmidt, Muscular Dystrophy UK's Director of Research, said:

"These are encouraging findings about a unique new avenue to treating Duchenne muscular dystrophy. It is a complex condition and we are of the view that it will take a combination of therapies to treat it effectively. Professor Winder's approach could complement potential therapies currently advancing through clinical trials, making them more effective.

"In time, we hope that its potential to treat other muscular dystrophies will also be investigated. For now, we are looking forward to seeing the results of further research into Duchenne muscular dystrophy to support these early promising results."

More information: 'Dasatinib as a treatment for Duchenne muscular dystrophy,' by Leanne Lipscomb, Robert W. Piggott, Tracy Emmerson and Steve J. Winder, is published in *Human Molecular Genetics*.

Provided by University of Sheffield