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Early statin treatment may help children with Fragile X

Children with an inherited form of intellectual disability and autism could be helped by a medicine commonly used to lower cholesterol, if used early in life.

The drug – called lovastatin – corrected learning and memory problems in rats with a form of Fragile X Syndrome, tests revealed.

Rats were treated with lovastatin for four weeks during infancy but the benefits persisted for months afterwards.

Researchers say this suggests learning problems in children with Fragile X might be prevented by a similar treatment in early life.

Fragile X Syndrome is one of the most common genetic causes of intellectual disability. It is often associated with autism and attention deficit and hyperactivity disorder, or ADHD. Many affected individuals also have seizures.

The condition occurs when a particular gene is disrupted leading to altered communication between brain cells.

Previous studies in mice and rats have shown that this disruption can be treated with drugs, but it was not known how long treatment might be effective for.

Researchers at the University of Edinburgh studied rats with a genetic alteration similar to that found in people with Fragile X Syndrome. These rats have problems completing certain memory tasks when compared with typical rats.

Treatment with lovastatin between five and nine weeks of age – the precise window when they are developing these memory abilities – restored normal development in the rats.

The animals were able to complete the memory tasks more than three months after treatment ended, indicating the effects of the drug were long-lasting.

Children with Fragile X Syndrome are usually diagnosed around the age of three, typically because they are late in learning to speak. Genetic tests have enabled earlier diagnosis, which raises the possibility of starting treatments sooner.

Current medications help manage specific symptoms – such as hyperactivity and seizures – but there are not yet any treatments that tackle the underlying brain changes leading to Fragile X Syndrome.

Statins are widely prescribed to both children and adults to control high blood cholesterol and to reduce the risk of heart disease.

The study, published in *Science Translational Medicine*, was led by researchers at the University of Edinburgh's Patrick Wild Centre and the Simons Initiative for the Developing Brain.

Professor Peter Kind, Director of the Patrick Wild Centre and Simons Initiative for the Developing Brain at the University of Edinburgh, said: "Children with Fragile X Syndrome need special education and, although some will live semi-independently, most require some form of lifelong support.

"We have found that early intervention for a limited period during development can lead to persistent beneficial effects, long after treatment ends, in a rat model of Fragile X Syndrome. Our future experiments will focus on whether there is a critical time-window during development when treatment is more effective."

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