At last, hope for families living in the shadow of Huntington’s disease

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March 10, 2019

▲ Matt Ellison with his wife Marianna and son Joey, who was born free from Huntington's. Photograph: Courtesy of Matt Ellison

An innovative drug may soon offer new ways to fight this cruel inherited condition

Matt Ellison was seven when his father was diagnosed with Huntington’s disease. The condition – which is progressive, incurable and invariably fatal – took 15 years to kill John Ellison.

The impact on Matt’s life was profound. His father, who had inherited the disease from his mother, found he could no longer concentrate enough to hold down his job as an engineer at Jaguar. Later he began to lose the power of movement and, eventually, lost his ability to speak. At his local school Matt was mocked because of his father’s odd, uncoordinated gait. The taunting got so bad that Matt stopped attending. “I stayed at home and helped Mum look after Dad,” he recalls.

Then in 2007, when Matt reached 18, he decided to find out whether he faced a similar fate. He was tested and told: yes, he had the Huntington’s gene. A few years later his father died, aged 55.

“I had had time to prepare myself, but it still hits you hard when you are told you are positive,” says Matt. “I had wanted to be negative as much for my mum, who had gone through enough pain.”

For Matt, and thousands of others who havebeen told they have inherited this affliction, the future would appear bleak, a prospect of inexor able physical and mental decline. The Huntington's gene is remorseless in its impact.

But recently this dark outlook has brightened. Scientists believe they are closing in on a treatment to control Huntington's worst effects.

"We recently tested a new drug, called RG6042, that appears to be safe from our first phase-one trial and which reduces levels of the toxic protein that builds up in patients' brain cells and is believed to trigger the disease," says Huntington's expert Professor Sarah Tabrizi of University College London. "It's a great step forward."

A big hurdle remains, however: scientists still have to show that RG6042 actually delays or halts the progression of the disease once it has taken hold of patients' brain cells. It may reduce levels of the mutant protein, but will it actually slow or stop the onset of symptoms? Will it help those in the later stages of the disease, or aid those who have just begun to display symptoms?

These critically important questions should be answered by a major trial that has just been launched. More than 600 patients will be involved – the first has already been recruited – and the trial, backed by the pharmaceuticals giant Roche, is set to begin in Europe in a few months' time.

"It will take two years to complete, but it should tell us if we have a winner on our hands: a drug that could potentially improve patients' lives," says Tabrizi. "I am rightly cautious but hopeful."…..

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Source: *TheGuardian*

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