

Infographic: Gene therapy drugs that silence the effects of faulty genes could help tackle Huntington's and other neurodegenerative diseases

Huntington's disease (HD) is an inherited condition that causes widespread deterioration in the brain and disrupts thinking, behaviour, emotion and movement. The disease usually begins in midlife, with subtle changes such as mood swings and difficulty in staying focused. As it progresses, people develop dementia and an inability to speak or move.

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There are no treatments available to stop or slow the progression of Huntington's, even though its genetic cause has been clear since 1993. Most other neurodegenerative diseases also lack effective therapies and, although their genetic roots are less clear-cut than for Huntington's, many of the genes associated with conditions such as motor neuron disease (amyotrophic lateral sclerosis, or ALS), Alzheimer's and Parkinson's have been known for decades.

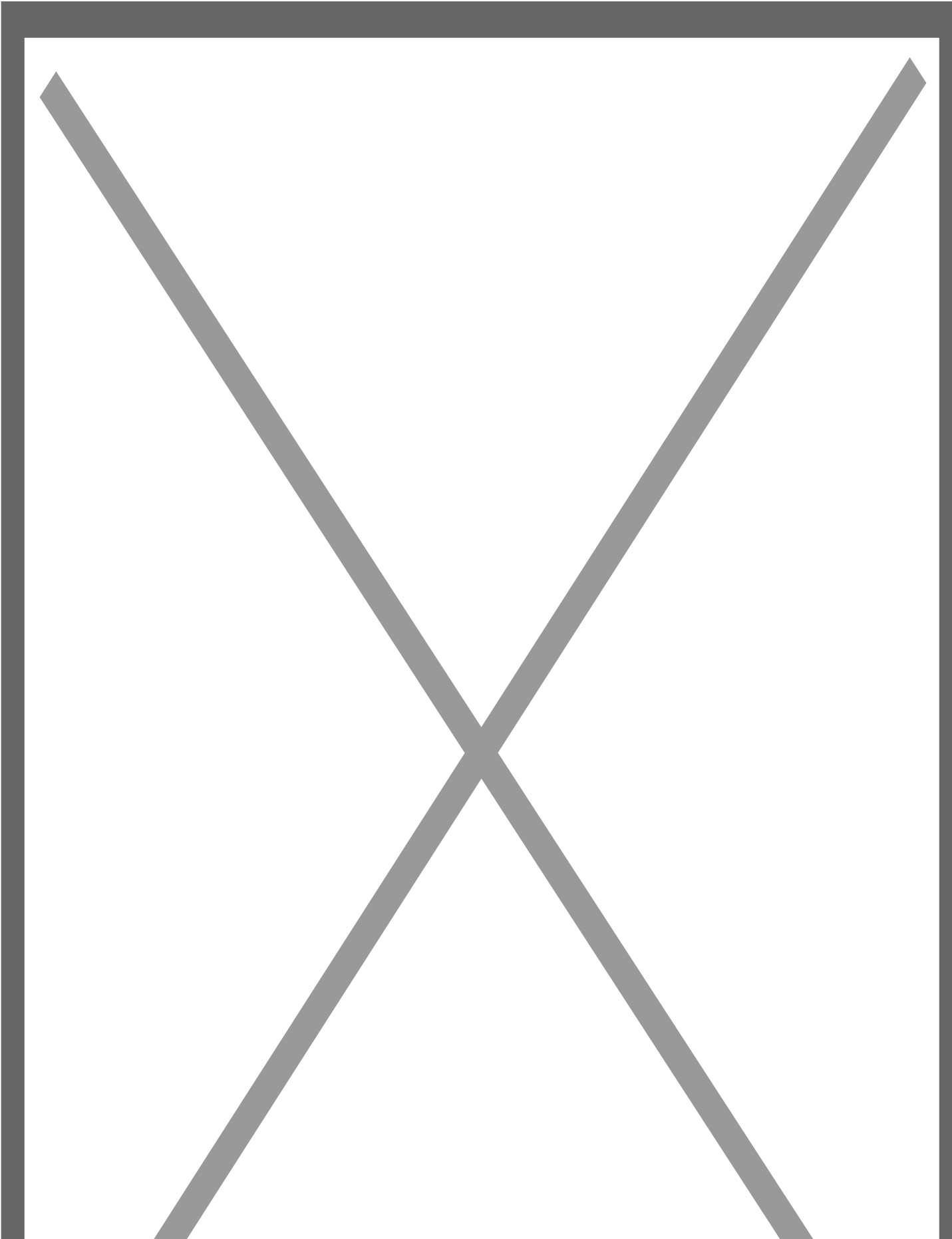
Now, the tide might be turning for treating these kinds of diseases. Many researchers are hopeful about drugs known as antisense oligonucleotides (ASOs). These are short strings of DNA or RNA letters that are designed to cling to particular sequences of RNA made by faulty genes, and to rebalance the levels of proteins they produce — boosting missing proteins or quashing faulty ones.

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The US Food and Drug Administration (FDA) approved the first ASO for a neurological disease in 2016, and there has since been an explosion of activity in this area.

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