

Slowing Huntington's disease: how science advice enabled a breakthrough treatment

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A coordinated research effort shows how science advice and shared infrastructure can help turn new ideas into meaningful progress for people and communities.

Around 8,000 people in the UK are living with Huntington's disease, a rare and fatal condition caused by a single genetic mutation. Until now, there has been no treatment proven to slow its progression. Through science advice and publicly funded research infrastructure, that is beginning to change.

The National Institute for Health and Care Research (NIHR), funded by the Department of Health and Social Care, supported a landmark clinical study into a new gene therapy, AMT-130 - the first gene therapy to be tested in people with Huntington's disease. The study, funded by the life sciences company uniQure, showed that participants receiving a higher dose of the treatment experienced 75% less disease progression after three years, alongside slower decline in movement and thinking. For patients and families, this represents long-awaited hope.

An NIHR Biomedical Research Centre and NIHR Clinical Research Facility provided expert scientific advice, specialist NHS facilities, and skilled research staff. The NIHR Research Delivery Network provided further research staff and supported a long-term observational research study, which provided a suitable comparison group of those with Huntington's who were not receiving the treatment, ensuring the results were robust.

This study is one of nearly 18,000 projects enabled by NIHR research infrastructure in a single year. By bringing together trusted science advice, the NHS, and industry, NIHR helps turn cutting-edge ideas into real-world treatments that improves the health and wealth of the nation. It also strengthens the UK's position as a global leader in life sciences and innovation.

This is science advice in action - supporting better decisions, safer research, and breakthroughs that can save lives. It Celebrates Science Advice, highlighting how evidence and expertise underpin progress across public services and deliver real benefits for people and communities.

<https://www.gov.uk/government/case-studies/slowing-huntingtons-disease-how-science-advice-enabled-a-breakthrough-treatment>