Aniqa Ali

aniqaa689@gmail.com

TeenMedX

Breakthrough Treatment shown to slow Huntington's Disease progression after promising Phase I/II clinical trials.

A leader in the field of biotechnology, UniQure has developed a revolutionary treatment which has been shown to slow the progression of Huntington's Disease, a genetic disease which causes nerve cells in the brain to die. However, the company now faces new hurdles in their attempts to receive licensing from the FDA to market their treatment.

1. Huntington's Disease

According to the National Institute of Health, Huntington's Disease is an "inherited disorder that causes nerve cells (neurons) in parts of the brain to gradually break down and die", leading to involuntary movements, personality changes, slurred speech, and a progressive loss of independent function.A notable first sign is chorea, defined as 'uncontrollable dance-like movements and abnormal body postures' that often take the form of jerky, uncontrollable movements. Symptoms typically develop in middle-aged adults but can also emerge before the age of 20, a form known as juvenile Huntington's disease.

Due to the progressive brain cell degeneration the disease causes, the Cleveland Clinic states that life expectancy of an adult with Huntington's after symptoms develop is typically estimated to be around fifteen to twenty years. The disease is caused by a change in the HTT gene, which normally makes huntingtin protein to facilitate communication in the brain and to support

movement, learning, and emotional balance. However, a mutation in the HTT gene leads to the misfolding of huntingtin proteins, leading to the protein clumping in the brain. These clumps damage brain cells that control movement and disrupt communication between brain cells. There is currently no cure for Huntington's Disease.

2. UniQure and Their Solution

"According to its website, UniQure is a biotechnology company specializing in gene therapy using adeno-associated virus (AAV)–based technology. In addition to pursuing a treatment for Huntington's disease, the company has developed a gene therapy for hemophilia B, a hereditary disorder caused by a deficiency in an essential clotting factor. The company is currently developing AMT-130, an 'AAV gene therapy to be investigated in Huntington's disease.'

Clinical outcomes for 29 treated patients demonstrated that AMT-130, at the high-dose treatment, showed a 75 percent slowing of disease progression with a p-value of 0.003, meaning that the results were statistically significant when compared to an external control as measured by the composite Unified Huntington's Disease Rating Scale at 36 months. This is one of the first indications of a successful treatment for Huntington's Disease.

UniQure is currently developing AMT-130, an AAV-based gene therapy under investigation for Huntington's disease. Clinical outcomes from 29 treated patients indicated that high-dose AMT-130 was associated with a 75 percent slowing of disease progression, with a p-value of 0.003, suggesting statistical significance when compared with an external control group as measured by the composite Unified Huntington's Disease Rating Scale at 36 months. These findings represent an early but promising signal of therapeutic benefit."

3. Challenges with the FDA

On November 3rd, 2025, UniQure announced that "based on the discussions at the meeting, UniQure believes that the FDA currently no longer agrees that data from the Phase I/II studies of AMT-130 in comparison to an external control. . . may be adequate to provide the primary evidence in support of a BLA submission." The 'external control' being referred to consisted of participants from Enroll-HD, an observational study for Huntington's Disease families. It monitors the progression of the disease in people without testing new drugs. The FDA appears unconvinced that the data is enough to prove the gene therapy's effectiveness.

4. Future Potential

While the decision by the FDA represents a setback, it doesn't mean that the treatment's development and testing has been cancelled. However, UniQure may need to produce more data and evidence in favor of the treatment, potentially through further clinical trials with a placebo control group as opposed to an external one. AMT-130 remains a promising potential treatment to slow the progression of Huntington's Disease in a meaningful way, possibly extending the lives of the people with this devastating disease. Just in 2022, the FDA approved UniQure's first gene therapy for hemophilia B, a historic achievement that allows certain patients living with Hemophilia B to produce their own clotting factors, highlighting the rapid progression of gene therapies and UniQure's history of success in gene therapy development.

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