FDA Gives Green Light to UniQure to Begin Clinical Trial of Gene Therapy for Huntington's Disease

Rare Daily Staff

The U.S. Food and Drug Administration cleared UniQure's application to begin a human clinical trial of AMT-130, the company's experimental gene therapy to treat Huntington's disease, a rare genetic neurodegenerative disorder.

Huntington's disease leads to loss of muscle coordination, behavioral abnormalities and cognitive decline, resulting in complete physical and mental deterioration. The disease is caused by a genetic mutation to the huntingtin gene, that leads to the production of a mutated protein that aggregates in the brain. There are no therapies available to treat the disease, delay onset, or slow progression of a patient's decline.

AMT-130 consists of a recombinant AAV5 vector carrying a DNA cassette encoding a microRNA that non-selectively lowers or knocks-down human huntingtin protein in Huntington's disease patients.

UniQure is now cleared to begin a phase 1/2 dose-escalating, randomized, controlled clinical trial to assess the safety, tolerability and efficacy of a one-time treatment of AMT-130 in Huntington's patients. The company expects to start dosing patients in the second half of 2019. The company said AMT-130 is on track to be the first AAV-based gene therapy to enter clinical trials for Huntington's disease.

"AMT-130 also represents the first clinical-stage AAV-based therapy specifically designed to silence an abnormal gene in the brain with a single administration, and we believe our proprietary miQURE gene silencing platform has the potential to be applied to many other diseases, such as spinocerebellar ataxia type 3 (SCA3)," said Matt Kapusta, CEO of UniQure. "This achievement is a major milestone for UniQure's research organization, who have dedicated years of effort with the hope we can one day offer treatment for the many patients waiting generations for an effective therapy."

January 22, 2019

Photo: Matt Kapusta, CEO of UniQure

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@lisettekingo & I were proud to attend the proclamation last year when our city recognized #WRDD and we are excited to work with our city again in 2019 to further the discussion & increase awareness. #babysteps @TheAngelProjec1

10h

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A3 I often share stories about my past & the friends that I've lost to rare disease. I speak about the importance for research, advances in medical science, & survival of those who are struggling to live. My friends are always on my mind in everything that I so. #USP7RareChats

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A5 When I was growing up, I felt alone much of the time. I didn't feel many people knew what CF was. Today, however, there are so many stories of people living with CF that I feel we are beginning to be heard! Never be afraid to use your voice to raise awareness!

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