IONIS-HTTRx

IONIS-HTTRx (RG6042), an antisense drug for the treatment of people with Huntington's disease.

IONIS-FB-LRx, an antisense drug using Ionis' advanced LIgand Conjugated Antisense (LICA) technology, reduces the production of FB, a key protein in the complement innate immune system. FB is predominately produced in the liver and circulates throughout the vascular system, including vessels in the eye and kidney. This complement protein plays a pivotal role in an innate immunogenic cascade that, when overactivated, has been associated with the development of several complement-mediated diseases, including dry AMD.

IONIS-HTTRx is a Generation 2+ antisense drug we designed to target the underlying cause of HD by reducing the production of the toxic mHTT protein. IONIS and Roche entered into a collaboration to develop and commercialize antisense drugs to treat HD in April 2013. Roche licensed IONIS-HTTRx from us in December 2017. Roche is now responsible for all IONIS-HTTRx development, regulatory and commercialization activities and costs, including managing the ongoing OLE and all future studies.

HD is a rare, inherited, genetic brain disorder that results in the progressive deterioration of mental abilities and physical control. In the U.S., there are approximately 30,000 individuals with symptomatic HD and more than 200,000 people at risk of inheriting HD. HD is a triplet repeat disorder and is one of a large family of genetic diseases in which the body mistakenly repeats certain gene sequences. The resulting mHTT protein is toxic and gradually damages neurons in the brain. Symptoms of HD usually appear between the ages of 30 to 50 years and continually worsen over a 10 to 25-year period. Ultimately, the weakened individual succumbs to pneumonia, heart failure or other complications. Presently, there is no effective disease-modifying treatment, and current approaches only focus on managing the severity of some disease symptoms.

In December 2017, announced that completed a randomized, placebo-controlled, dose escalation, Phase 1/2a clinical study of IONIS-HTTRx in patients with early stage HD. Dose-dependent reductions of mHTT were observed among patients treated with IONIS-HTTRx, with a safety and tolerability profile supporting continued development.

The FDA and EMA have granted Orphan Drug Designation for IONIS-HTTRx to treat people with HD.