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US FDA grants VICO therapeutics Orphan-Drug Designation for anti-neurodegenerative drug

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VO659, an investigational therapy for Spinocerebellar Ataxia



VICO Therapeutics, a Leiden, the Netherlands, based biotech company focusing on the development of RNA modulating therapies for rare neurological disorders, announced that the Office of Orphan Products Development (OOPD) of the U.S. Food and Drug Administration (FDA) has granted orphan-drug designation for VO659.

VO659 is a VICO's investigational antisense oligonucleotide (AON) therapy for the treatment of spinocerebellar ataxia (SCA). In February, VICO already received orphan drug designation for VO659 in SCA from the European Commission.

Rupert Sandbrink MD PhD., Chief Medical Officer at VICO, stated, "Spinocerebellar ataxia belongs to the group of polyglutamine disorders which are debilitating and progressive diseases, leading to significant impairment of mobility and multiple other daily activities of patients suffering from these conditions. Currently, no disease-modifying treatments are available for these patients. Our investigational RNA modulating therapy is designed to lower the mutant protein levels causing these neurodegenerative diseases. We look forward to advancing VO659, our therapy that holds great potential for addressing their unmet needs. It also recognizes the potential of our AON approach. This is yet another important step forward as we prepare for our first in human trials, expected to start in 2022."

FDA's Office of Orphan Products Development grants Orphan Drug Designation to drugs and biologics that are defined as those intended for the safe and effective treatment, diagnosis or prevention of rare diseases/disorders that affect fewer than 200,000 people in the U.S. The designation allows VICO to qualify for a number of incentives, including seven years of market exclusivity upon regulatory approval; exemption from FDA application fees for spinocerebellar ataxia; and tax credits for qualified clinical trials.