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Ionis, Roche initiates pivotal study of RG6042 for Huntington's disease

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Ionis Pharma, Inc. the leader in antisense therapeutics, has announced that its partner Roche has enrolled the first patient in a pivotal study of RG6042 (IONIS-HTT$_{Rx}$) for people living with symptoms of Huntington's disease (HD), a hereditary neurodegenerative disorder for which there is currently no approved disease-modifying treatment.

RG6042 is the first therapy in pivotal trials designed to target the underlying cause of HD by reducing production of the toxic mutant huntingtin protein (mHTT). Ionis has earned a $35 million milestone payment for the initiation of the study.

"We are pleased that RG6042 has progressed to a Phase 3 study. It is the first of four or more Ionis programs that we anticipate will advance to pivotal studies this year, further proof that we are realizing the potential of our novel antisense technology to deliver transformative medicines to those who need them," said Brett P. Monia, Ph.D., Ionis' chief operating officer. "Our commitment to developing antisense medicines for neurological diseases has led to the commercialization of SPINRAZA, the standard of care treatment for people with all forms of spinal muscular atrophy. Enrollment of the first patient in this pivotal trial represents substantial hope for people living with Huntington's disease and their families."

Roche and Ionis are collaborating to develop antisense drugs to treat HD. In December 2017, Roche licensed IONIS-HTT$_{Rx}$ from Ionis and has renamed the investigational molecule RG6042.

In total, Ionis has generated $135 million in up-front, milestone and license payments and is eligible to receive additional milestone payments as RG6042 progresses in development, as well as royalties on sales of the medicine if it is commercialized. Roche is responsible for all RG6042 development, regulatory and commercialization activities and costs.