



uniQure Collaborates with UCSF on GDNF Gene Therapy in Parkinson's Disease

Collaborators Partner with the National Institute of Neurological Disorders and Stroke (NINDS), Patient Enrollment into Clinical Study Expected to Begin Mid 2012

Amsterdam, The Netherlands – June 20, 2012 – uniQure, a leader in the field of human gene therapy, announced today the signing of a collaborative agreement with two leading neurology experts to develop further a gene therapy incorporating uniQure's GDNF (glial cell derived neurotrophic factor) gene for the treatment of Parkinson's disease.

Professor Krystof Bankiewicz at the University of California, San Francisco (UCSF), a world expert in GDNF gene therapy, and Professor Howard Federoff of Georgetown University, a preeminent physician-neuroscientist, have developed a product approved to start clinical trials in the U.S. using uniQure's GDNF gene incorporated into an adeno-associated virus-2 (AAV-2) delivery vector. The GDNF gene contains the information to produce a protein necessary for the development and survival of nerve cells. The positive effect of GDNF on nerve cells has already been demonstrated in early research by uniQure in collaboration with the University of Lund, Sweden.

UCSF entered into a collaboration with Dr. Russell Lonser, neurosurgeon and Chief of the Neurosurgical Branch of the NINDS, a division of the National Institutes of Health, to commence a Phase I study of the gene therapy in patients with Parkinson's disease. Patient enrollment is expected to begin mid-2012. Collaborating on the study will be Drs. Krystof Bankiewicz of UCSF, Howard Federoff of Georgetown University and NINDS co-investigator neurologists Drs. Mark Hallett and Walter Koroshetz.

"This agreement provides uniQure with access to the data from a Parkinson's disease GDNF clinical study conducted by two of the world's leading medical researchers in the field. If successful, we intend to manufacture the vector construct ourselves and with a partner progress the product into advanced clinical studies," said Jörn Aldag, CEO of uniQure. "GDNF has been shown to be involved in several other CNS disorders so if we reach the proof of concept stage in Parkinson's, we can potentially expand product development quickly and efficiently into clinical trials for other indications, such as Huntington's and Multiple System Atrophy (MSA)."

"The development of AAV2-GDNF, sponsored by both NIH and by Parkinson's foundations, has taken us 10 years to complete. We are very pleased that a path for clinical development of AAV2-GDNF as a possible treatment for PD is now in place," said Dr. Krystof Bankiewicz, UCSF Principal Investigator.

Under the terms of uniQure's agreement with UCSF, uniQure holds the exclusive commercial rights to all UCSF preclinical data and to IND enabling Phase I clinical data provided to UCSF by NINDS. In the event that the Phase 1 study shows proof of concept, uniQure will use its proprietary manufacturing system for future production of the AAV construct and take responsibility for future development of the gene therapy product. uniQure holds the exclusive license to the GDNF gene from Amgen.

About uniQure

uniQure is a world leader in the development of human gene based therapies. uniQure has a product pipeline of gene therapy products in development for hemophilia B, acute intermittent porphyria, Parkinson's disease and SanfilippoB. Using adeno-associated viral (AAV) derived vectors as the delivery vehicle of choice for therapeutic genes, the company has been able to design and validate probably the world's first stable and scalable AAV manufacturing platform. This proprietary platform can be applied to a large number of rare (orphan) diseases caused by one faulty gene and allows uniQure to pursue its strategy of focusing on this sector of the industry. Further information can be found at www.uniqure.com.

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