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ClearPoint Neuro, Inc. Congratulates uniQure on Completion of Enrollment in First Cohort of Phase I/II Clinical Trial of AMT-130 for the Treatment of Huntington's Disease

IRVINE, Calif., April 06, 2021 (GLOBE NEWSWIRE) -- ClearPoint Neuro, Inc. (Nasdaq: CLPT) (the "Company"), a global therapy-enabling platform company providing navigation and delivery to the brain, today congratulates uniQure N.V. on the early completion of patient enrollment in the first dose cohort of a randomized, double-blinded, Phase I/II clinical trial of AMT-130 being conducted in the United States for the treatment of early-stage Huntington's disease.

Six patients out of ten who were enrolled in the first cohort were randomized to receive AMT-130 gene therapy and underwent administration procedures using the ClearPoint® Neuro Navigation System and SmartFlow® MRI-safe neuro ventricular cannulae under MRI guidance. The Company's clinical specialist team provided support during the administration and imitation surgery (sham) procedures to assist the study team in executing the clinical trial protocol. A second, higher-dose cohort in this study is expected to begin enrolling 16 patients in the second half of 2021. A separate, open-label Phase Ib/II clinical trial of AMT-130 for 15 patients at European trial sites is expected to be performed using the ClearPoint Neuro Navigation System and SmartFlow cannulae, with patient enrollment planned to begin later this year.

Huntington's disease is a rare, inherited neurodegenerative disorder impacting approximately 70,000 people in the U.S. and Europe.[1] It affects motor function and leads to behavioral symptoms and cognitive decline in young adults, resulting in total physical and mental deterioration. It is caused by the expansion of the CAG trinucleotide in exon 1 of a multifunctional gene coding for a protein called huntingtin. Despite the clear etiology of Huntington's disease, there are no currently approved therapies to delay the onset or to slow disease progression.

uniQure's gene therapy product candidate AMT-130 consists of an AAV5 vector carrying a gene that expresses a micro-RNA specifically tailored to block the production of huntingtin protein, including the toxic mutant variant. Using AAV vectors to deliver this treatment directly to affected areas in the brain under live MRI-guidance for non-selective knockdown

of the huntingtin mRNA represents a highly innovative and promising approach to treating Huntington's disease.

"ClearPoint is proud to help advance potential treatments for this devastating disorder," stated Jeremy Stigall, Vice President, Biologics and Drug Delivery. "At this time, we are actively pursuing the installation process in Europe to prepare for training and enrollment of the new Phase Ib/II clinical trial sites in the second half of 2021."

About ClearPoint Neuro

ClearPoint Neuro's mission is to improve and restore quality of life to patients and their families by enabling therapies for the most complex neurological disorders with pinpoint accuracy. Applications of the Company's current product portfolio include deep brain stimulation, laser ablation, biopsy, neuro-aspiration, and delivery of drugs, biologics, and gene therapy to the brain. The ClearPoint Neuro Navigation System has FDA clearance, is CE-marked, and is installed in over 60 active clinical sites in the United States, Canada, and Europe. The Company's SmartFlow® cannula is being used in partnership or evaluation with over 25 individual biologics and drug delivery companies in various stages – from preclinical research, to late-stage regulatory trials. To date, more than 4,000 cases have been performed and supported by the Company's field-based clinical specialist team, which offers support and services for our partners. For more information, please visit www.clearpointneuro.com.

Forward-Looking Statements

Statements herein concerning the Company's plans, growth and strategies may include forward-looking statements within the context of the federal securities laws. Statements regarding the Company's future events, developments and future performance, as well as management's expectations, beliefs, plans, estimates or projections relating to the future, are forward-looking statements within the meaning of these laws. Uncertainties and risks may cause the Company's actual results to differ materially from those expressed in or implied by forward-looking statements. Particular uncertainties and risks include those relating to: the impact of COVID-19 and the measures adopted to contain its spread; future revenues from sales of the Company's ClearPoint Neuro Navigation System products; and the Company's ability to market, commercialize and achieve broader market acceptance for the Company's ClearPoint Neuro Navigation System products. More detailed information on these and additional factors that could affect the Company's actual results are described in the "Risk Factors" section of the Company's Annual Report on Form 10-K for the year ended December 31, 2020, which has been filed with the Securities and Exchange Commission.

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[1] http://uniqure.com/gene-therapy/huntingtons-disease.php

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