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uniQure and Apic Bio enter into global licensing agreement for APB-102, a clinical stage gene therapy for patients with ALS caused by mutations in SOD1

~ License of APB-102 further strengthens uniQure's pipeline of innovative gene therapies to treat neurological disorders and miRNA-based gene silencing programs ~

~ APB-102 and uniQure's c9orf72-ALS program have the potential to address most inherited forms of ALS ~
~ uniQure plans to initiate a Phase I/II trial of APB-102 in the second half of 2023 ~

LEXINGTON, Mass. and AMSTERDAM and CAMBRIDGE, Mass., Jan. 31, 2023 (GLOBE NEWSWIRE) -- uniQure N.V. (NASDAQ: QURE), a leading gene therapy company advancing transformative therapies for patients with severe medical needs, and Apic Bio, an innovative gene therapy company developing novel treatment options for patients with rare genetic diseases, today announced that they have entered into a global licensing agreement for APB-102 to treat superoxide dismutase 1 (SOD1) amyotrophic lateral sclerosis (ALS), a rare, genetic form of ALS. Under the agreement, uniQure acquires global rights for the development and commercialization of APB-102, adding to its pipeline of gene therapies to treat neurological disorders. The U.S. Food and Drug Administration has cleared the investigational new drug (IND) application for APB-102 and has granted Orphan Drug and Fast Track designations.

APB-102 is designed to be a novel, one-time, intrathecally administered gene therapy for ALS caused by mutations in SOD1, a rapidly progressing, rare motor neuron disease that leads to loss of everyday functions and is uniformly fatal. Mutations in the SOD1 gene of ALS account for approximately one-fifth of all inherited forms of this fatal disease[1]. APB-102 is comprised of a recombinant AAVrh10 vector that expresses a micro ribonucleic acid (miRNA) designed to knock down the expression of SOD1 with the goal of slowing down or potentially reversing the progression of ALS in patients with SOD1 mutations.

"The licensing of APB-102 provides uniQure with another clinical stage program that is strategically aligned with our current pipeline and highly complementary with our AMT-161 program for the treatment of ALS caused by mutations in the c9orf72 gene," stated Ricardo Dolmetsch, Ph.D. president of research and development at uniQure. "Together, these ALS gene therapy candidates have the potential to address most familial forms of ALS and transform the lives of thousands of patients around the world suffering from this devastating disease. We look forward to initiating a Phase I/II clinical study of APB-102 in the second half of 2023."

The clinical development of APB-102 is based on nearly 30 years of research demonstrating the link between the SOD1 gene mutation and ALS. Preclinical studies in a SOD1-ALS mouse model demonstrated that APB-102 greatly enhanced survival in affected mice. Relevant SOD1 reduction in spinal cord motor neurons also was demonstrated in rodents, as well as in non-human primates at proposed clinical doses.

"I am very proud of the contributions Apic Bio has made to bring APB-102 to the cusp of clinical development," stated John Reilly, co-founder and chief executive officer of Apic Bio. "uniQure is at the forefront of the field of miRNA gene therapies for neurological disorders and is the ideal partner to achieve the goal of rapidly advancing the clinical development of APB-102 for the potential benefit of SOD1-ALS patients."

Under the terms of the agreement, uniQure will make an initial cash payment of $10 million. In addition, uniQure will pay Apic Bio up to $45 million in milestones upon achievement of regulatory approvals in the U.S. and Europe and pre-specified annual net sales, and a tiered royalty on net sales ranging from the mid-single digits to low double digits.

SVB Securities acted as exclusive financial advisor to uniQure in the transaction.
Torreya Partners acted as exclusive financial advisor to Apic Bio in the transaction.

About SOD1-ALS

Amyotrophic lateral sclerosis (ALS) is a fatal neurodegenerative disorder characterized by loss of motor neurons, leading to muscle weakness and eventual paralysis. Most patients face mortality within five years of disease onset due to respiratory failure[2]. ALS can be caused by multiple genetic mutations and can be sporadic (spontaneous mutations) or familial (inherited mutations). Familial mutations account for approximately ten percent of ALS cases, and of these, approximately twenty percent are linked to a mutation in the SOD1 gene that codes for the enzyme superoxide dismutase 1[1]. SOD1-linked ALS is most likely caused by toxic mutant forms of the superoxide dismutase 1 (SOD1) protein (a gain-of-function mutation)[1]. Current approved ALS treatments only delay disease progression without addressing the underlying genetic causes of the disease.

About uniQure

uniQure is delivering on the promise of gene therapy – single treatments with potentially curative results. The recent approval of our gene therapy for hemophilia B – an historic achievement based on more than a decade of research and clinical development – represents a major milestone in the field of genomic medicine and ushers in a new treatment approach for patients living with hemophilia. We are now leveraging our modular and validated technology platform to rapidly advance a pipeline of proprietary gene therapies for the treatment of patients with Huntington's disease, refractory temporal lobe epilepsy, ALS, Fabry disease, and other severe diseases. www.uniQure.com

About Apic Bio

Apic Bio is an innovative gene therapy company focused on developing first-in-class treatment options for rare, undertreated neurological and liver diseases. The Company's lead program to date has been an adeno-associated (AAV)-based gene therapy for the treatment of SOD1 ALS. Preclinical studies of additional genetic forms of ALS (C9orf72) and Alpha-1 Antitrypsin Deficiency (Alpha-1) are ongoing. The Company is also advancing discovery programs for two undisclosed CNS indications that leverage its proprietary silence and replace THRIVE™ platform. The Company is backed by leading and disease-centric investors, including Morningside Venture Investments, ALS Investment Fund, and The Alpha-1 Project (TAP). For more information, please visit www.apic-bio.com.

uniQure Forward-Looking Statements

This press release contains forward-looking statements. All statements other than statements of historical fact are forward-looking statements, which are often indicated by terms such as "anticipate," "believe," "could," "establish," "estimate," "expect," "goal," "intend," "look forward to", "may," "plan," "potential," "predict," "project," "seek," "should," "will," "would" and similar expressions. Forward-looking statements are based on management's beliefs and assumptions and on information available to management only as of the date of this press release. These forward-looking statements include, but are not limited to, statements about whether uniQure's ALS gene therapy candidates have the potential to address most familial forms of ALS or transform the lives of thousands of patients around the world, whether uniQure will initiate a Phase I/II clinical study of APB-102 in the second half of 2023, and whether uniQure will be able to rapidly advance the clinical development of APB-102 for the potential benefit of SOD1-ALS patients. The Company's actual results could differ materially from those anticipated in these forward-looking statements for many reasons, including, without limitation, risks associated with the regulatory approval and commercial launch of HEMGENIX®, our clinical trial for Huntington's disease, the impact of financial and geopolitical events on our Company and the wider economy and health care system, our Commercialization and License Agreement with CSL Behring, our clinical development activities, clinical results, collaboration arrangements, regulatory oversight, product commercialization and intellectual property claims, as well as the risks, uncertainties and other factors described under the heading "Risk Factors" in the Company's periodic securities filings, including its Annual Report on Form 10-K filed February 25, 2022. Given these risks, uncertainties and other factors, you should not place undue reliance on these forward-looking statements, and the Company assumes no obligation to update these forward-looking statements, even if new information becomes available in the future.

References:

1. Brown CA, Lally C, Kupelian V, Flanders WD. Estimated Prevalence and Incidence of Amyotrophic Lateral Sclerosis and SOD1 and C9orf72 Genetic Variants. Neuroepidemiology. 2021
2. Brown RH, Al-Chalabi A. Amyotrophic Lateral Sclerosis. N Engl J Med. 2017 Jul 13

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