QurAlis Announces First-in-Human Dose in Phase 1 Clinical Trial of QRL-101, a First-in-Class Kv7 Precision Therapy for ALS

QRL-101 aims to reduce hyperexcitability-induced motor neuron degeneration

CAMBRIDGE, Mass., *January 5, 2023* – QurAlis Corporation, a clinical-stage biotechnology company developing breakthrough precision medicines for amyotrophic lateral sclerosis (ALS) and other neurodegenerative diseases with genetically validated targets, today announced that the company recently initiated dosing in a Phase 1 clinical trial of QRL-101 (QRA-244), a first-in-class selective Kv7.2/7.3 ion channel opener for the treatment of hyperexcitability-induced disease progression in ALS.

“We are excited to initiate dosing in this clinical trial of QRL-101, marking our second program to enter the clinic in less than a month,” said Kasper Roet, Ph.D., CEO and co-founder of QurAlis. “ALS is one of the most devastating, fatal neurodegenerative diseases and no therapies significantly extend patients’ lives. The validity of Kv7.2/7.3 as a drug target for the treatment of ALS comes from research using human ALS motor neurons and is strongly supported by the results of a clinical study published in *JAMA Neuro* showing that Kv7 modulation can decrease spinal and cortical motor neuron excitability, both of which have been linked to patient survival. QRL-101 has been shown in preclinical studies to have strong potential to control motor neuron hyperexcitability induced excitotoxicity with significantly fewer side effects than other drug candidates. QRL-101 has the potential to be a first-in-class effective therapy for ALS patients suffering from hyperexcitability-induced motor neuron degeneration.”

“Motor system hyperexcitability occurs in approximately 50 percent of all ALS patients and is linked to potassium channel dysfunction, recent studies

found that the gene coding for the potassium channel Kv7.2 is dysregulated in sporadic ALS," said Leonard H. van den Berg, M.D., Ph.D., professor of neurology and chair, TRICALS. "Previously, targeting these potassium channels in ALS patients showed promising results but that particular drug is no longer available due to side effects experienced in its marketed indication, epilepsy, which is thought to be the result of its non-selective profile. Developing a safer, more selective drug that targets the potassium channel Kv7.2/7.3 is a promising, clinically validated therapeutic strategy and we applaud QurAlis for advancing the QRL-101 program."

This first-in-human Phase 1 study (NCT05667779) is a randomized, double-blind, placebo-controlled, single-ascending-dose, single-site clinical trial designed to evaluate the safety, tolerability, and pharmacokinetics of QRL-101 in adult healthy volunteers. More information about this clinical trial can be found at www.clinicaltrials.gov.

About Amyotropic Lateral Sclerosis (ALS)

Amyotrophic lateral sclerosis (ALS), also known as Lou Gehrig's disease, is a progressive neurodegenerative disease impacting nerve cells in the brain and spinal cord, reducing muscle function and control. ALS can be traced to mutations in more than 25 different genes and is often caused by a combination of multiple sub-forms of the condition. Cases usually cannot be predicted, although a small percentage are inherited. ALS has a devastating impact on patients and families. ALS patients' average life expectancy is three years, and there is currently no cure for the disease.

About Kv7

Kv7.2/7.3 is a hetero-tetrameric voltage-gated potassium channel in cell membranes which is encoded by *KCNQ2* and *KCNQ3*. *KCNQ2* is dysregulated in sporadic ALS patients. Kv7.2/7.3 is the dominant component of the neuronal M-current which stabilizes the membrane potential and controls neuronal excitability. Therapies that reduce abnormal electrical activity in the brain by activating or opening the Kv7.2/7.3 ion channel show the potential to decrease spinal and cortical motor neuron excitability in patients with amyotrophic lateral sclerosis (ALS) and the potential to positively effect compound muscle action potential (CMAP) amplitude, suggesting that this may be an effective therapeutic approach for patients suffering from hyperexcitability-induced motor neuron degeneration.

About QurAlis Corporation

QurAlis is trailblazing the path to conquering amyotrophic lateral sclerosis (ALS) and other neurodegenerative diseases with genetically validated targets with next-generation precision medicines. QurAlis' proprietary platforms and unique biomarkers enable the design and development of drugs that act directly on disease-causing genetic alterations. Founded by an internationally recognized team of neurodegenerative biologists from Harvard Medical School and Harvard University, QurAlis is advancing a deep pipeline of antisense oligonucleotides and small molecule programs including addressing sub-forms of ALS that account for the majority of ALS patients. For more information, please visit www.quralis.com or follow us on Twitter @QurAlisCo.

Media contact:
Kathy Vincent
kathy@kathyvincent.com
310-403-8951