QurAlis' selective Kv7 opener, a drug candidate for ALS, demonstrates up to 20-fold increase in potency and dramatic reduction in side effects compared to Retigabine in preclinical models

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Reduced Off-target Side Effects Make QRA-244 a Strong Drug Candidate to Treat Hyperexcitability-induced Disease Progression in ALS Patients

CAMBRIDGE, Mass., December 11, 2020— QurAlis Corporation, a biotech company focused on developing precision medicines for amyotrophic lateral sclerosis (ALS) and other neurologic diseases, today announced the results of extensive pre-clinical studies on its lead program, QRA-244, for ALS patients. QRA-244 is a Kv7.2/7.3 ion channel opener optimized for both safety and efficacy as a potential treatment for motor neuron hyperexcitability-induced neurodegeneration in ALS patients. The data were presented at the Motor Neuron Disease Association (MNDA) 31st International Symposium on ALS/MND.

The poster presentation, "QRA-244 a Potent, Selective KCNQ2/3 Opener and a Potential Therapy for Motor System Hyperexcitability induced Disease Progression in ALS Patients," showed that QRA-244 has comparable effects to retigabine (ezogabine) at up to 20-fold lower concentration in motor neurons differentiated from patient-derived iPSCs as measured by an all-optical electrophysiology experiment. Importantly, QRA-244 showed dramatic reductions in side effects associated with retigabine including fatigue/somnolence, dizziness and bladder retention in head-to-head pre-clinical studies.

"Degeneration of motor neurons in ALS patients leads to loss of innervation of muscles, which leads to paralysis and eventually fatality. These data, combined with the recent results published in JAMA Neuro showing that Kv7 modulation can decrease cortical and spinal motor neuron excitability, which are correlated to poor survival and could lead to protection of muscle innervation, make QRA-244 a very promising drug candidate for the almost 50% of ALS patients who present with hyperexcitability in their motor systems," said Daniel Elbaum, Ph.D., Chief Scientific Officer of QurAlis. "The preclinical data show that the improved channel specificity of our novel Kv7 opener could translate into an improved clinical safety profile with significant reduction in off-target adverse events." QurAlis plans on initiating first-in-human studies at the end of 2021 or beginning of 2022.

"If we can reduce motor neuron hyperexcitability in ALS patients, we may be able to slow the progression of the disease by reducing neuronal damage in hyperexcitable ALS patients," commented Merit Cudkowicz, M.D., M.Sc. (Massachusetts General Hospital). "These data combined with the recent JAMA Neuro clinical study represent an exciting glimmer of hope to treat this serious disease."

About QRA-244

QRA-244 is QurAlis' lead drug candidate intended to treat motor neuron hyperexcitability-induced disease progression in ALS patients, which is estimated to be up to 50% of all ALS cases. A Kv7 opener, QRA-244 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. QurAlis plans to initiate first-in-human studies of QRA-244 at the end of 2021 or beginning of 2022.

About 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. ALS breaks down nerve cells, reducing muscle function and causing loss of muscle 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. Its average life expectancy is three years, and there is currently no cure for the disease.

About QurAlis Corporation

QurAlis is bringing hope to the ALS community by developing breakthrough precision medicines for this devastating disease. Our stem cell technologies generate proprietary human neuronal models that enable us to more effectively discover and develop innovative therapies for genetically validated targets. We are advancing three antisense and small molecule programs addressing sub-forms of the disease that account for the majority of patients. Together with a world-class network of thought leaders, drug developers and patient advocates, our team is rising to the challenge of conquering ALS. www.quralis.com

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