QurAlis Grants Lilly Exclusive Global License for QRL-204, a Potentially First-in-Class Precision Therapy That Restores UNC13A Function in ALS and FTD

QRL-204 is a splice-switching ASO generated through QurAlis' FlexASO™ Platform; represents Lilly's first program targeting UNC13A in ALS and FTD

Parties to also collaborate to leverage QurAlis' ALS and ASO development expertise to advance QRL-204 and next-generation compounds

UNC13A is an essential regulator of neurotransmitter release at synapses; mis-splicing is a critical RNA alteration occurring in up to 63 percent of all ALS patients and up to one-third of all FTD cases

CAMBRIDGE, Mass., *June 3, 2024* – QurAlis Corporation ("QurAlis") today announced that it has entered into an exclusive license agreement with Eli Lilly and Company ("Lilly") in which QurAlis is granting Lilly global rights to develop and commercialize QRL-204, a potentially best-in-class splice-switching antisense oligonucleotide (ASO) designed to restore UNC13A function in amyotrophic lateral sclerosis (ALS), frontotemporal dementia (FTD), and other neurodegenerative diseases.

Under the terms of the agreement, QurAlis granted Lilly an exclusive, worldwide license to develop and commercialize QRL-204 and other UNC13A-targeting compounds in exchange for an upfront payment of $45 million to QurAlis, plus an additional equity investment. QurAlis is also eligible for future milestone payments of up to $577 million and tiered royalties on net sales.

The agreement includes a research and development (R&D) collaboration to identify and develop additional candidates targeting UNC13A, leveraging QurAlis' proprietary FlexASO™ Splice Modulator Platform. The QurAlis FlexASO Splice Modulator Platform was developed to generate splice-switching ASOs with improved potency and increased therapeutic index. QurAlis' ASOs correct UNC13A mis-splicing, restore UNC13A protein production and reduce cryptic exons that may contribute to disease progression.

"We are determined at Lilly to uncover the next great idea, the next collaboration, the next advancement in technology that will drive us toward meaningful treatments for ALS and FTD patients. It's all for one reason – to make life better for even more people," said Andrew Adams, senior vice president, neurodegeneration research, and director, Lilly Institute for Genetic Medicine. "Genetic precision medicines like QRL-204 that target specific causal components of disease pathology hold great promise for delivering meaningful advances against a range of neurodegenerative diseases like ALS and FTD. We look forward to collaborating with QurAlis to identify and develop additional next-generation candidates targeting UNC13A."

"On behalf of the entire team at QurAlis, we are extremely pleased to partner with Lilly, an innovation-led company advancing transformative medicines to help make life better for people around the world," said Kasper Roet, chief executive officer and co-founder of QurAlis. "At QurAlis, we are driven to explore the deepest aspects of human neurology to find the treatments patients urgently need. This partnership enables QRL-204 to rapidly move toward the clinic, while we continue to advance our other lead programs. We look forward to combining our complementary strengths to uncover additional candidates that target UNC13A that have the potential to transform the treatment of neurodegenerative diseases like ALS and FTD and beyond."

Amyotrophic lateral sclerosis is a progressive neurodegenerative disease characterized by the loss of neurons in the spinal cord, brainstem, and brain. A defining feature of both sporadic and familial disease is the cytoplasmic mis-localization of TAR DNA Binding Protein-43 (TDP-43). TDP-43 pathology is implicated in 90 percent of ALS cases and approximately 50 percent of FTD cases.

UNC13A is an essential regulator of neurotransmitter release at synapses and is one of several pre-mRNAs that becomes mis-spliced due to loss of nuclear TDP-43 in disease. Up to 63 percent of ALS patients and up to one-third of FTD patients carry a single nucleotide polymorphism in the UNC13A gene or show TDP-43 pathology, which greatly exacerbates UNC13A mis-splicing leading to loss of function of the UNC13A protein.

Preclinical data recently presented at the AD/PD™ 2024 International Conference on Alzheimer's and Parkinson's Diseases and Related Neurological Disorders showed QurAlis' UNC13A splice-switching ASOs modulate UNC13A splicing and restore normal synaptic activities in ALS and FTD. QurAlis' ASOs prevented cryptic exon inclusion in UNC13A transcripts, increased UNC13A protein levels, and normalized localization of UNC13A protein at the synapse.

About QurAlis Corporation
At QurAlis, we are neuro pioneers on a quest to cure. We work with a relentless pursuit of knowledge, a precise attention to craft, and an optimistic mindset to discover and develop effective precision medicines that will alter the trajectory of amyotrophic lateral sclerosis (ALS), frontotemporal dementia (FTD), and other neurodegenerative diseases. Founded by an internationally recognized team of neurodegenerative biologists from Harvard Medical School and Harvard University, QurAlis is advancing a pipeline with therapeutic candidates that target specific components of ALS and FTD pathology and defined patient populations based on both disease-causing genetic mutation(s) and clinical biomarkers. For more information, please visit www.quralis.com or follow us on X @QurAlisCo or LinkedIn.

Contact:

Kathy Vincent
kathy.vincent@quralis.com
310-403-8951

# # #