QurAlis Announces Exclusive License on Novel Mechanism for Fragile X Syndrome (FXS) to Enable Development of First Potential Disease-Modifying Therapy

Collaboration with UMass Chan Medical School confirmed FMR1-217 as well as an RNA-targeted mechanism to restore functional FMRP protein to develop potential treatments for FXS

QurAlis' preliminary data suggest feasibility of biomarker to detect mis-splicing of FMR1 in FXS; company advancing FMR1-217 as precision medicine target in up to 80 percent of FXS patients

Company applying its FlexASO® platform and expertise of splicing targets toward having a candidate nominated for IND-enabling studies in the near future

CAMBRIDGE, Mass., *May 15, 2025* – QurAlis Corporation ("QurAlis"), a clinical-stage biotechnology company driving scientific breakthroughs into powerful precision medicines that have the potential to alter the trajectory of neurodegenerative and neurological diseases, today announced it has entered into an exclusive license agreement with UMass Chan Medical School ("UMass Chan") on a novel RNA-targeted mechanism confirmed to restore functional protein for Fragile X syndrome (FXS).

Fragile X syndrome is the leading inherited form of intellectual disability and the most common single genetic cause of autism. It is a genetic condition caused by a mutation of a single gene – Fragile X messenger ribonucleoprotein 1 (FMR1) – on the X chromosome. This mutation of FMR1 causes a range of developmental problems including learning disabilities, behavioral challenges, and cognitive impairment.

QurAlis' exclusive license agreement is a result of its 2024 partnership and collaboration with UMass Chan to explore the biology of FXS to determine and confirm relevant targets that could enable antisense oligonucleotide (ASO)-mediated correction for FXS. QurAlis leveraged its deep understanding, knowledge and expertise in developing ASOs as part of the collaboration. QurAlis confirmed the findings from the original publication of the UMass Chan researchers and is advancing FMR1 as a precision medicine target in up to 80 percent of FXS patients. The mis-spliced form of FMR1, designated as FMR1-217, is widely expressed throughout cortical brain areas affected in FXS and can be measured in blood and cerebrospinal fluid. Preliminary data suggest biomarker feasibility to detect mis-splicing of FMR1 in patients with FXS.

"FXS is a devastating neurodevelopmental disorder with no effective disease-modifying therapies available. Our initial partnership with UMass Chan confirmed that FMR1-217 is a validated genetic target for FXS," said Kasper Roet, Ph.D., chief executive officer and co-founder of QurAlis. "This groundbreaking discovery of a novel RNA-targeted mechanism to restore functional protein for FXS and the feasibility of a biomarker to detect mis-splicing of FMR1 in FXS patients opens up a completely new type of therapeutic approach through splice correction. We look forward to applying QurAlis' FlexASO® platform and deep knowledge and expertise of ASO splicing targets toward having a candidate nominated for IND-enabling studies in the near future, so that we can bring a potential new precision medicine option to patients."

Joel Richter, Ph.D., the Arthur F. Koskinas Chair in Neuroscience and professor of molecular medicine at UMass Chan, and colleagues Sneha Shah, Ph.D., and Jonathan K. Watts, Ph.D., together with Elizabeth Berry-Kravis, M.D., Ph.D., at Rush University Medical Center, have shown that aberrant alternative splicing, or mis-splicing, of messenger RNA (mRNA) plays a fundamental role in FXS. In a seminal publication by the group, it was revealed that in FXS patients, FMR1 mRNA is still being expressed, but is mis-spliced, comprising a short, truncated alternative mRNA variant called FMR1-217 which results in non-functional FMRP protein expression. Working with patient-derived cells, Dr. Richter's lab and Dr. Berry-

Kravis initially demonstrated that ASOs can successfully inhibit the mis-splicing, reduce expression of FMR1-217, rescue proper FMR1 mRNA, and restore FMRP protein expression.

“This is a meaningful step in the process of taking basic biological discoveries and turning them into practical therapies that can benefit patients in the clinic,” said Dr. Richter. “QurAlis’ platform and expertise in neurodegenerative disorders are industry leading and well positioned to address the mis-splicing of FMR1 RNA and restore functional FMRP protein expression. This partnership has not only validated our years-long research but also has resulted in the confirmation of a novel target for FXS, which we hope will lead to much-needed treatment options for FXS patients and their families.”

Dr. Berry-Kravis added, “I am very excited that we will be able to continue development of this potential genetically based disease-modifying FMRP-restoring therapy that is expected to have a major impact on the FXS field and the spectrum of treatment options available to improve function in people with FXS.”

An orphan disease, FXS affects approximately 87,000 individuals in the U.S. alone – one in 4,000 men and one in 6,000 women. Though FXS occurs in both genders, males are more frequently affected than females, and generally with greater severity. In addition to intellectual disability, FXS patients endure a wide range of disabling symptoms including severe anxiety, social aversion, hyperactivity and attention deficit, sensory hypersensitivity, aggression, developmental seizures, and others. There are no effective disease-modifying therapies currently available for FXS.

ASOs are short, engineered single-stranded DNA/RNA molecules that can selectively bind RNA to regulate its expression in the cell. ASO technology has been leading in the field of protein regulation and has since allowed us to develop treatments for neurodegenerative disease by changing the expression of genes connected to the disease.

QurAlis’ FlexASO® platform was developed to generate splice-switching ASOs with improved potency, increased therapeutic index and improved bio-distribution. This bespoke platform has the potential to tackle the spectrum of neurodegenerative and neurological diseases.

About QurAlis Corporation
At QurAlis, we are neuro pioneers on a quest to cure, boldly seeking to translate scientific breakthroughs into powerful precision medicines. We work collaboratively with a relentless pursuit of knowledge, precise attention to craft, and compassion to discover and develop medicines that have the potential to transform the lives of people living with neurodegenerative and neurological diseases. QurAlis is the leader in development of precision therapies for amyotrophic lateral sclerosis (ALS). In addition to ALS, QurAlis is advancing a robust precision medicine pipeline to bring effective disease-modifying therapeutics to patients suffering from severe diseases defined by genetics and clinical biomarkers. For more information, please visit www.quralis.com or follow us on X @QurAlisCo or LinkedIn.

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