

## **QurAlis Expands its Industry-Leading Precision Medicine Approach in Splicing Targets Beyond Neurodegeneration Into Fragile X Syndrome (FXS)**

*Company initiates partnership and collaboration with UMass Chan Medical School to characterize relevant targets that enable ASO-mediated correction for FXS*

*Fragile X syndrome is a rare, genetic neurological disease that is the leading inherited form of intellectual disability and a known cause of autism*

**CAMBRIDGE, Mass., June 25, 2024** – [QurAlis Corporation](#) (“QurAlis”), a clinical-stage biotechnology company driving scientific breakthroughs into powerful precision medicines that have the potential to alter the trajectory of amyotrophic lateral sclerosis (ALS), frontotemporal dementia (FTD), and other neurodegenerative and neurological diseases, today announced it is expanding its industry-leading precision medicine antisense oligonucleotide (ASO) splicing expertise beyond neurodegeneration into Fragile X syndrome (FXS).

As part of this strategy, QurAlis has initiated a partnership and collaboration with UMass Chan Medical School to explore the biology of FXS to determine and confirm relevant targets that could enable ASO-mediated correction for FXS. QurAlis can leverage its deep understanding, knowledge and expertise in developing ASOs including its proprietary FlexASO™ Splice Modulator Platform as part of the collaboration.

“QurAlis’ deep knowledge and expertise of ASO splicing targets combined with our FlexASO™ Splice Modulator Platform gives us the latitude to go beyond neurodegeneration to explore the potential of bringing much-needed precision medicines for other serious neurological diseases like FXS,” said Kasper Roet, Ph.D., chief executive officer and co-founder of QurAlis. “Our partnership and collaboration with UMass Chan in FXS underscores the enormous value of partnerships between academia and industry to drive innovation forward to help fulfill unmet medical needs for patients. We are excited to expand our scope into a new therapeutic area and to make use of our expertise in splicing targets so that QurAlis can continue the goal of making a real difference in patients’ lives.”

“We are excited to be partnering with QurAlis in the next step of this years-long research,” said Joel Richter, Ph.D., the Arthur F. Koskinas Chair in Neuroscience and professor of molecular medicine at UMass Chan Medical School in Worcester, MA. “QurAlis’ translational knowledge and clinical trial experience in ASO splicing for neurodegenerative disorders is an important component to bridging the gap between biomedical discoveries made in the laboratory and delivering therapies to patients in the clinic. With their help, we are hopeful that one day we may be able to offer patients with FXS meaningful treatment options.”

### **About Fragile X Syndrome (FXS)**

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

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 disease-modifying therapies currently available for FXS.

#### **About QurAlis' FlexASO™ Splice Modulator Platform**

Antisense oligonucleotides (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™ Splice Modulator 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. 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 have the potential to alter the trajectory of amyotrophic lateral sclerosis (ALS), frontotemporal dementia (FTD), and other neurodegenerative and neurological diseases. Founded by an internationally recognized team of neurodegenerative biologists from Harvard Medical School and Harvard University, QurAlis is advancing a robust precision medicine pipeline with therapeutic candidates aimed at modifying severe disease pathology in defined patient populations based on both disease-causing genetic mutation(s) and clinical biomarkers. For more information, please visit [www.quralis.com](http://www.quralis.com) or follow us on X @QurAlisCo or [LinkedIn](#).

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