

QurAlis Receives Health Canada Clinical Trial Application Authorization for QRL-201, a First-in-Class STATHMIN-2 Precision Therapy for ALS

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First ever study to evaluate a therapy that rescues STATHMIN-2 expression in ALS patients expected to initiate in Canada in early 2023 with other countries to follow

STATHMIN-2 is a well-known protein important for neural repair and axonal stability; its expression is significantly decreased in nearly all ALS patients and several other neurodegenerative diseases including frontotemporal dementia and Alzheimer's Disease

CAMBRIDGE, MASS., DECEMBER 8, 2022 – [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 the authorization of its clinical trial application (CTA) by Health Canada for QRL-201, a first-in-class STATHMIN-2 (STMN2) precision medicine for ALS. The ANQUR clinical trial is a global, multi-center, randomized, double-blind, placebo-controlled multiple-ascending dose Phase 1 study designed to evaluate the safety and tolerability of QRL-201 in patients with ALS.

QRL-201 is a therapeutic product candidate that aims to restore STATHMIN-2 expression in ALS patients. STATHMIN-2 (also known SCG10 or SCGN10) is a well-known protein important for neural repair and axonal stability, the expression of which is significantly decreased in nearly all ALS patients. QRL-201 rescues STMN2 loss of function in QurAlis ALS patient-derived motor neuron disease models of TDP-43 pathology.

“The authorization of our CTA in Canada is a significant milestone for our company, representing our first program to receive clearance by a regulatory body to enter the clinic and the first ever clinical trial to evaluate a therapy that rescues STMN2 expression in ALS patients,” said Kasper Roet, Ph.D., CEO and co-founder of QurAlis. “We are working diligently with our investigators in Canada to begin patient enrollment and dosing as quickly as possible. We will continue advancing the QRL-201 clinical program so that we can bring this potentially transformative treatment to patients rapidly and fulfill our mission to make a meaningful difference in patients’ lives.”

“ALS is a devastating, fatal neurodegenerative disease with a large unmet medical need. We applaud the team at QurAlis for developing promising novel precision medicine approaches such as QRL-201. These patients today have very limited treatment options,” said Geneviève Matte, M.D.C.M., FRCP(C), assistant clinical professor, Department of Neurosciences, University of Montréal; ALS clinic director, University of Montréal Hospital Centre (CHUM); principal investigator, University of Montréal Hospital Research Centre (CRCHUM); and an ANQUR study investigator.

This clearance from Health Canada is the first part of the global regulatory strategy and plan established by QurAlis for the clinical development of QRL-201; additional clearances across multiple jurisdictions are anticipated in the coming months.

About the ANQUR Clinical Trial

ANQUR (NCT05633459) is a first-in-human global, multi-center, randomized, double-blind, placebo-controlled multiple-ascending dose Phase 1 clinical trial designed to evaluate the safety, tolerability, and pharmacokinetics (PK) of QRL-201 versus placebo. The primary objective of the study is to determine the safety and tolerability of multiple doses of QRL-201 in people living with ALS. The study is expected to include 64 patients with ALS across sites in the U.S., Canada, the United Kingdom, Belgium, the Netherlands, Italy, Germany, and Ireland.

Visit www.clinicaltrials.gov for more information about the ANQUR study.

About STATHMIN-2 and TDP-43

STATHMIN-2 (STMN2) is a well-known protein important for neural repair and axonal stability, the expression of which is significantly decreased in nearly all ALS patients. Also known as SCG-10, STMN2 is a protein important for stabilization of microtubules which form an important component of the cytoskeleton of cells and axons. STATHMIN-2 is highly expressed in human motor neurons, the cells that primarily degenerate in patients suffering from ALS. In animal models, STMN2 deletion was found to cause axonal degeneration, which is the primary functional deficit that leads to paralysis in ALS patients.

Using human neuronal stem cell models from ALS patients, QurAlis co-founder and Harvard professor Kevin Eggan, Ph.D., discovered in 2019 that the expression of STMN2 is regulated by TDP-43. The Eggan Lab showed that loss of normal TDP-43 function leads to a highly significant decrease in expression of STMN2 and an impairment in neuronal repair which could be rescued by restoring STMN2 levels. These results were published in [Nature Neuroscience](#).

In addition to nearly all ALS patients, TDP-43 pathology is also associated with approximately 50 percent of patients with frontotemporal degeneration, the second most common form of dementia; about a third of Alzheimer's Disease patients; and up to seven percent of Parkinson's disease patients.

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](#).

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