Médicament Québec Pilot Project / DePTAQ
CRISPR/dCas13Rx-Based Identification and Correction of Splicing Defects

A significant proportion—at least 20%—of disease-causing missense mutations disrupt RNA splicing, leading to the production of non-functional proteins and worsening disease outcomes. Despite this, splicing defects remain an underexploited therapeutic target, leaving a vast patient population underserved.

Our Solution: Precision Splicing Rescue Platform

We have developed a screening platform based on the CRISPR/dCas13Rx system that simplifies the identification and correction of splicing defects caused by missense mutations. This platform enables:

- Rapid and Cost-Effective Screening: Our system uses guide RNAs to selectively block splicing regulatory proteins (RBPs) on pre-mRNAs, allowing us to pinpoint which missense mutations disrupt splicing—without altering the RNA sequence itself.
- Therapeutic Innovation: Once splicing-disruptive mutations are identified, the same platform screens for guide RNAs that restore normal splicing. These guides inform the design of splice-switching antisense oligonucleotides (ASOs) that can rescue mRNA processing, restoring the production of full-length, functional proteins.
- Versatile Therapeutic Modalities: Beyond ASOs, our approach supports the development of targeted mRNA variant delivery using lipid nanoparticles (LNPs), expanding therapeutic options for diverse indications.

Why Partner With Us?

- First-Mover Advantage: By focusing on splicing rescue, we address a critical and previously overlooked mechanism in genetic disease, opening new therapeutic avenues with high unmet medical need.
- Scalable Pipeline: Our platform enables the rapid identification of a multitude of ASO candidates with disease-modifying potential, creating a robust pipeline for preclinical and clinical development.
- Translational Readiness: The technology is poised for preclinical validation, with a clear path to clinical translation and commercialization.
- Transformative Impact: We offer pharma partners and investors the opportunity to lead in the development of next-generation RNA therapeutics, targeting a broad spectrum of genetic disorders that currently lack effective treatments.

Join Us in Pioneering the Next Wave of RNA Therapeutics

Over 100,000 missense mutations have been catalogued as disease-causing in the HGMD and ClinVar databases. More than 7,000 rare diseases are recognized worldwide, and a substantial proportion of these are linked to missense mutations in one or more genes.

Opportunity for Collaboration

This call is open to a range of collaboration opportunities including funded research collaborations, in-kind contributions, licensing of intellectual property and building expertise in AI, SOPs and ASO design.

DePTAQ is le *Réseau de développement et de production de thérapies ARN*

Led by the Institut de Recherches Cliniques de Montréal (IRCM), McGill University, and the Université de Sherbrooke, this Network is one of the five key initiatives of the flagship RNA therapies project announced by the Government of Quebec. This initiative aims to strengthen Quebec's RNA therapy ecosystem and propel the province to the rank of world leader in this field.

DePTAQ provides a full range of services, from the initial design of RNA therapies to preclinical-scale production, including formulation and delivery. Researchers, as well as companies, will thus benefit from cutting-edge expertise to accelerate the development of their most promising RNA therapies, helping them move more quickly towards clinical trials and, ultimately, benefit patients. It is worth noting that the services offered by this new Network will complement the service offerings of Quebec companies, and partnerships are to be expected.

DePTAQ relies on three complementary platforms located in partner institutions:

- The RNomics Platform at the University of Sherbrooke
- The Sidney-Altman Therapeutic RNA Hub at IRCM
- The Therapeutic Messenger RNA Platform at the RNA Science Center of McGill University