PORTEFOLIO DE THÉRAPIES ARN

Dans le cadre du Pôle ARN du Québec – AReNA promu par le gouvernement du Québec, Axelys et son équipe ARN dédiée à favoriser la valorisation de la recherche publique et la création d'entreprises dans le secteur des thérapies ARN, a identifié à travers l'écosystème de recherche, des projets prometteurs qui présentent un potentiel scientifique et commercial.

Rassemblées dans ce portefolio, cette vitrine en ligne offre des opportunités de développement, de partenariat et d'investissement et constitue une source d'innovations pour les investisseurs, les partenaires académiques et industriels ainsi que les acteurs de l'écosystème. Grâce à ce portefolio, Axelys accélère le passage de la découverte à la commercialisation et contribue à positionner le Québec comme pôle d'excellence et d'attractivité internationale en ARN thérapeutique.

RNA THERAPEUTICS PORTFOLIO

As part of the Quebec RNA Hub – AReNA, promoted by the Government of Quebec, Axelys and its dedicated RNA team, working to support scientific translation of public research and company creation in the RNA therapeutics sector, have identified promising projects with scientific and commercial potential throughout the research ecosystem.

As part of this portfolio, this online showcase offers development, partnership and investment opportunities and serves as a source of innovation for investors, academic and industrial partners, as well as ecosystem stakeholders. Through this portfolio, Axelys accelerates the translation from discovery to commercialization and contributes to positioning Quebec as a hub of excellence and international attractiveness in RNA therapeutics.

Cell-Immune Pharma (CIP)

Engineering Human-Derived T-Cell Solutions to Supercharge Vaccines & Immunotherapies

$10-15B	100 s	>90%	C$2M Cap	Location: Montréal, QC
Global RNA therapies	Epitopes / month	HLA population reach	Seed safe round	Founded: August 2022

BUSINESS MODEL & EXIT STRATEGY

CIP is pursuing licensing and co-development opportunities with RNA and vaccine partners.

Early agreements could generate $6–12M in upfront and milestone payments by 2027–28, with royalties from future commercial products.

Collaboration with HDT Bio (CQDM program) and early engagement with Sanofi and Daiichi Sankyo highlight industry alignment around T-cell–enhanced vaccine design.

NEXT MILESTONES (Q1 2026 – Q1 2027)

2026

- **Q3** — **Complete CMV epitope mapping**
- **Q4** — **Finalize cassette** for preclinical validation

2027

- **Q1** — **First partner demo with HDT Bio** validating clinical integration (GBMprogram)
- **Q3** — **Expand platform to second indication** and initiate preclinical validation

THE PROBLEM

Most current vaccines and immunotherapies stimulate antibodies only, leaving out the immune system's most powerful defense: T-cells.

Without robust T-cell activation, protection fades, viral reactivation occurs, and cancers remain "invisible" to the immune system.

This gap is critical in transplant patients, hard-to-treat cancers and emerging infections.

OUR SOLUTION

CIP's human PBMC platform identifies natural T-cell targets and converts them into compact, manufacturable immune modules.

These have been shown to drive strong, balanced T-cell activation—a key predictor of durable protection and therapeutic response—and can be rapidly adapted across multiple pathogens.

PROVEN PLATFORM

Human-validated workflow demonstrates compact multi-epitope, multi-HLA constructs that drive strong, balanced T-cell activation while maintaining safety.

The platform can screen hundreds of epitopes monthly across multiple HLA types and is ready to power discovery of new immune targets such as CMV, enabling rapid expansion to multiple targets.

$10–15B GLOBAL CMV MARKET

$2B: Transplants | **$5B:** Oncology | **$5–10B:** Congenital

CIP is uniquely positioned to solve CMV's core challenge, restoring durable T-cell immunity where antibody vaccines fail. The CMV program serves as the first validation use-case for the platform—de-risking expansion to other viral and pathogen-linked oncology applications.

COMPETITIVE LANDSCAPE

Company	Focus	Limitation	CIP Advantage
Moderna	Antibody-based CMV vaccine	Limited T-cell	Human-validated T-cell targets
Hookipa Biotech	Viral-vector CMV vaccine	Narrow HLA coverage	>90% HLA population reach
CIP	**T-cell platform**	**Early stage**	**Multi-pathogen potential**

INVESTMENT OPPORTUNITY

This seed round funds completion of the CMV discovery program and positions CIP for its first licensing deal within 24 months, establishing early nondilutive revenue and validating the platform's commercial model.

- **Seed SAFE @ C$2M cap (+25% discount)**
- **$500K open** to complete $1.3M round
- **$800K secured** from angels and grants (VRQ, CQDM, Axelys)
- **24-month runway** to first licensing revenue and IP-driven exit

CIP Seed Round Goal:
Launch a high-throughput, human-validated T-cell discovery engine targeting pathogen and pathogen-linked cancers, driving CMV completion and first licensing within 24 months.

TEAM

David Lapointe, CEO
Biotech CEO | $50M+ raised
Global BD track record

Dr. Réjean Lapointe, CSO
Director, CRCHUM Cancer Research
25 yrs T-cell immunology

Dr. Jean-François Cailhier, Chief Medical Officer
Clinician-scientist
Transplant & inflammation

Pôle ARENA du Québec
invest@arenapole.ca
axelys
axelys.ca

Espoir Biosciences

Reactivating the Brain's Repair Mechanisms to Reverse Neurodegeneration

$3B → $6B
ALS market by 2030

CAD $1.3M
Non-dilutive raised
(IRSC, RQRM, ALS Canada, Huntignton Canada)

> 90% Knockdown
Fist-in-Class
Target Modality: ASO

Pre-Seed
CAD $1-2M Raise

Location: Montréal, QC
Incorporation: 2025

EXIT STRATEGY

Primary Path: Out-licensing to Big Pharma post-Phase I safety/POC (2029-2030)

- **Target Partners:** Biogen, Sanofi, Ionis, Roche (established neuro franchises)
- **Deal Structure:** Geographic or indication-based licensing;
  $50-150M upfront + milestones typical for Phase I neuro assets
- **Precedent:** Ionis/Biogen Spinraza deal ($75M upfront, $1B+ in milestones)

Alternative:
M&A by pharma seeking regenerative neuro pipeline (2029-2030)

18-MONTH MILESTONES & USE OF FUNDS

Pre-Seed Round: CAD $1-2M

Lead Optimization:
- Finalize ASO sequence
- Confirm >90% knockdown & safety profile

In Vivo POC:
- ALS mouse model efficacy
- Demonstrate neuroprotection and axonal repair

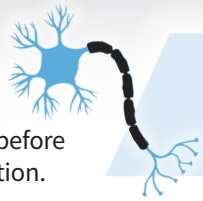
Expanded Indications:
- Parkinson's & Huntington's model validation

IND Prep:
- Pre-IND meetings (FDA/Health Canada)
- Tox study design

2026				2027			
Q1	Q2	Q3	Q4	Q1	Q2	Q3	Q4
	Lead ASO locked		ALS mouse data	Multi-indication POC			Series A ready Pre-IND meetings

THE PROBLEM

Neurodegeneration starts with disconnection, not death.
In ALS, Parkinson's, and Huntington's, synapses and axons deteriorate before neurons die—causing memory loss, motor decline, and cognitive dysfunction.



Current drugs protect neurons but cannot restore connections.
This is why they fail to halt disease progression.

OUR SOLUTION

First-in-class RNA therapeutic modulating a novel target
A master regulator of axonal repair discovered through genetic screening at CRCHUM

- **Mechanism:** Antisense oligonucleotide (ASO) reactivates neurons' intrinsic regeneration machinery
- **Validated Platform:** ASOs are clinically proven (Spinraza®, Tofersen)—de-risked regulatory path
- **Intrathecal Delivery:** Standard CNS administration route

PRECLINICAL VALIDATION

- **Target Discovery** via in vivo genetic screens, also identified as risk factor for PD and AD via GWAS.
- **POP Demonstrated:** Target upregulated in ALS iPSCs and in blood samples of ALS patients
- **Lead ASO:** >90% knockdown in ALS patient-derived motor neurons
- **Safety:** Target knockout in mice is safe

MARKET OPPORTUNITY

300K+ ALS Patients

70K New cases per year

- **Current SOC ineffective:** Riluzole, edaravone increase lifespan by 2-6 months
  Current gene therapies apply only to small sub-groups of patients : Tofersen less than 2% of ALS patients
- **Platform potential:** Pathway is implicated across multiple neurodegenerative diseases (Parkinson's, Huntington's, Alzheimer's).

IP & REGULATORY STRATEGY

- **Intellectual Property:** Discovery declared at CRCHUM (2023, updated 2025)
  Provisional patent filing planned 2026, PCT to follow. Exclusive worldwide license to Espoir
- **Regulatory Path:** Orphan Drug (ALS), Fast Track (FDA), ODD (FDA and EMA)
  Priority Review (Canada/EMA)— could accelerate approval by 12-18 months.

TEAM

Supported by: Axelys, AReNA, CRCHUM and a network of clinical & regulatory advisors

Constantin Bretonneau, CEO
PhD Candidate
Neuroscience & biotech venture creation

Dr. Gilles Tossing, CSO
Postdoc, CRCHUM
ASO design & neurobiology specialist

Dr. Alex Parker, Scientific Advisor
Professor, CRCHUM
Leading expert in ALS genetics & axon regeneration

01-2026-001

Nanofacile™ Symphony Kit

Accelerating RNA therapy discovery & development

$50B
Global RNA therapies

30 sec
Encapsulation time

28
Parallel formulations

$500K USD
Dilutive and non dilutive funding

Location: Montréal, QC
Founded: August 2023

BUSINESS MODEL

Monthly Subscription

2025: • $7.5K/month includes 28 units + systemic delivery recipes
• Access to the Nanofacile Maestro Formulation Companion (NMFC) as a service
2027: Subscription adds AI functions and targeted delivery recipes to the NMFC
2028: Joint ventures with customers (IP share, royalties)

Revenue Milestone

Paid beta customers in 2025

TIMELINE

2026

Launch: • RUO commercial product with 50+ customers
• Formulation companion

2027

Scale-Up: • GLP to GMP transition
• Clinical scale-up, Version 2.0 and AI features
• +100 commercial customers
• >$2M+ ARR, Seed Round $5-10M
• cGMP certification & Two-sided marketplace

THE PROBLEM

Current LNP formulation solutions are expensive, slow, and expertise-dependent, creating a major bottleneck in RNA therapeutic development.

- **Low-end options:** Cost up to $40K per formulation, take up to 60 days, with success rates <10%
- **High-end platforms:** Require up to $300K, extend timelines by 180+ days, demand specialized expertise
- **Success rates:** Rarely exceed 50%, leaving significant technical and financial risk

Critical unmet need: Existing solutions lack speed, scalability, and reproducibility, forcing companies to choose between high cost with low success or high capital investment with long development cycles.

OUR SOLUTION

Nanofacile™ Symphony Kit is a turnkey platform that uses Centrifuge-driven consumables run RNA formulations in parallel, paired with proven recipes and a learning software layer that improves with every experiment.

Result:
Reduces preclinical development timelines from weeks to days.

Key Features:

- **30-second encapsulation** per sample
- **Up to 28 parallel formulations** in a single run
- **Single-use sterile devices** ensuring downstream compatibility
- **Easy integration:** Compatible with standard lab facilities; no new costly equipment
- **Highly reproducible** with high-quality results

MARKET OPPORTUNITY

Applications: Oncology, Infectious Diseases, Rare Genetic Diseases, Inflammatory & Autoimmune Conditions, Respiratory Diseases.
Momentum: 340 clinical trials currently recruiting. 17+ mRNA therapies and vaccines already approved.

COMPETITIVE POSITIONING

Nanofacile is the only solution explicitly designed for accelerated preclinical R&D with cost-effective subscription pricing.

Platform	Speed	Cost	Throughput	Sterility
Nanofacile	**30 sec**	**Zero Cap Ex.**	**28 samples**	**Yes**
Spark PNI (Cytiva)	1 min	$60K + $5/sample	Limited	Yes
Sunscreen	3 min	$300K system	96 well samples	No

IP STRATEGY

- First provisional patent submitted in 2025
- PCT filings: 2026 (hardware + AI formulation companion)
- Patent-pending platform technology

TEAM

Rubén López, Ph.D., CEO & Founder
Business Development
Industry Experience in Nanomedicine

Angel Valerio, Ph.D., COO (Advisor)
Engineering Physics
Business Operations

Alejandro Forigua, Ph.D., R&D Chemistry Lead
Ph.D. Chemistry

Prof. Christine DeWolf, Scientific Advisor
Ph.D. Chemistry & Biochemistry

01-2026-001

Pôle ARENA du Québec
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axelys
axelys.ca

RNABioPrecision: Reprogramming disease networks with synthetic RNA molecules inspired by natural microRNAs

ONCOLOGY
FIBROTIC & NEURODEGENERATIVE DISEASES
INMMUNE DISORDERS

$468K CAD
Research funds obtained

$2.3M CAD
Research funds pending

TRL 3

Pre-clinical stage
Software validation complete; in vitro validation ongoing

IRIC Université de Montréal

Performance
- Up to 20 targets with a single syncRNA
- Wide variety of optimization endpoints

Software copyrights & trade secrets
Asset patents upcoming

incorporation: Q1 2026

Business Opportunity:
Co-development, Pre-seed investment, In-licensing

Market Opportunity:
Global market: $9.4B USD (2025) for RNA therapeutics
CAGR: 17.6% for RNA therapeutics

Q1-Q2 2026	Q3 2026	Q4 2026	Q4 2026 - Q1 2027	Q1-Q2 2027	Q3 2027
In vitro validation of KRAS-network core genes (PDAC, NSCLC, CRC)	PK/PD, biodistribution, and toxicology	Preliminary efficacy and validation	Seed fundraising	Pharma collaborations	Pre-IND and regulatory consultations

THE PROBLEM

In many diseases, particularly cancer, cells abnormally proliferate not because a single gene is dysregulated, but because multiple pathways converge to support the same essential function – often with redundancy.

Many tumors rely on networks of growth factor receptors, transcription factors, and survival signals that overlap in function. Inhibiting one component rarely collapses the network; instead, cells rewire their signaling to maintain growth. This adaptability is a major reason why single-target therapeutics (small molecules, monoclonal antibodies, or traditional RNAi) often produce initial responses followed by relapse.

OUR SOLUTION

RNABioPrecision's syncRNA platform introduces a new therapeutic category built on natural microRNA principles, enabling programmable modulation of multi-gene disease networks rather than isolated targets.

This stands in clear contrast to existing RNA design platforms, which rely on perfect complementarity and are limited to single-gene silencing. Our syncRNAs instead leverage partial complementarity, the mechanism used by endogenous microRNAs to regulate complex post-transcriptional circuits. We uniquely consider essential features such as transcript structure, target site accessibility, local interaction context, dissociation constant (KD), GC content, and off-target propensity, each of which contributes to specificity, stability, and efficacy. This yields a physiologically grounded and inherently flexible intervention, allowing a single molecule to reshape an entire oncogenic network.

MARKET

While dominant players in the RNA therapeutics space have developed clinically validated therapies for monogenic and hepatic diseases, these approaches fail to address the combinatorial complexity of polygenic diseases such as cancer or fibrosis. Attempts to move beyond single-gene strategies include multi-target siRNAs (Sirnaomics), miRNA mimics and anti-miRNAs (miRagen, Regulus), and AI-assisted siRNA design platforms (Deep Genomics, Verge Genomics, Profluent Bio). While promising, these efforts are often constrained by limited structural modeling, black-box designs, or platform-specific delivery – all adressed by the syncRNA platform.

RNABioPrecision is adopting a diversified business architecture that integrates software-driven platform licensing, strategic pharmaceutical collaborations, and proprietary therapeutic development.

TEAM

Elham Dianati, PhD: Cofounder, CEO
IRIC, Université de Montréal
Research Officer

François Major, PhD: Cofounder, CSO
IRIC, Université de Montréal
Full Professor of Computer Science

Claude Larose, MSc: Cofounder, CBO
IRICoR, Université de Montréal
VP Business Development

v_01-2026-001

Novel Treatment for Microvessel Rarefaction

RENAL TRANSPLANTATION, VASCULAR DISEASES

CRCHUM
CENTRE DE RECHERCHE

1 Provisional patent
March 2025

TRL 3

[Pre-]clinical stage
In vivo validation in ischemia-reperfusion models

Business Opportunity:
Licensing and Co-development

Market Opportunity:
Global market: $883M USD (2028) for miRNA
CAGR: 5.9% for miRNA

TIMELINE

DIAGNOSTIC			THERAPEUTIC		
miRNA signature confirmed on post-transplant patient samples	Correlation between renal function and miRNA expression levels ongoing	miRNA signature validation on larger cohort (including samples from healthy donors)	In vivo proof-of-concept validation on ischemia-reperfusion and leg ischemia models completed	Reduced microvessel rarefaction and prevention of renal fibrosis 21 days following ischemia-reperfusion (completed)	Identification of downstream targets and validation of MOA ongoing

THE PROBLEM

During major surgery, sepsis, trauma, or kidney transplantation, interruption of renal blood flow followed by kidney reperfusion (ischemia-reperfusion) causes tubular injury and endothelial cell damage that leads to renal dysfunction, acute kidney injury and to chronic or progressive renal failure.

An estimated 35.5 million Americans have kidney disease and about 815 000 Americans are living with kidney failure (1 in 7).
Similarly, blockage in the blood flow of the lower limbs, associated with smoking, diabetes, high blood pressure, aging, injury, or hereditary factors, causes a slowing of blood flow to the legs and feet that can cause pain and fatigue and even lead to ulcers, gangrene, and amputations. It is estimated that nearly 200 million people suffer from peripheral vascular disease worldwide, including nearly 45 million Americans.

Since no biomarkers exist to measure capillary reserve, it would be necessary to identify new markers to predict microvessel rarefaction and renal failure in order to quickly identify and initiate appropriate treatments.

OUR SOLUTION

Dr. Marie-Josée Hébert's team has identified specific miRNAs that could be used as diagnostic and therapeutic agents in the context of microvessel rarefaction.

They have shown that serum measurements of these miRNA markers by PCR predict renal microvessel rarefaction and, thus, the risk of progressive renal failure in humans. Moreover, they have also shown that the administration of these miRNAs in mice prevents renal microvessel rarefaction and accelerates new vessel formation after renal ischemia-reperfusion and leg ischemia.

MARKET

Market application:
- Diagnostic
- Microvessel rarefaction
- Renal transplantation
- Peripheral vascular diseases

It is estimated that the market for miRNAs as a research tool, **diagnostic tool, and therapeutic agent** could reach nearly $US 883M by 2028 with a CAGR of 5.9% for the period 2023-2028.

The annual cost for actual treatments can range from 56K $CA to 107K $CA per patient for dialysis and it is estimated that nearly 200 million people suffer from peripheral vascular disease worldwide.

Market sizes for major therapeutic indications, such as renal insufficiency and peripheral vascular diseases, range from $3.6 Billion USD (CAGR 8.2% 2029) to $9.2 Billion USD (CAGR 8.35% 2031), respectively.

TEAM

Marie-Josée Hebert
CRCHUM, Lead PI

Francis Migneault
CRCHUM

Héloïse Cardinal
CRCHUM

Alain Rivard
CRCHUM

Hyunyun Kim
CRCHUM

01-2026-001

Smart Anti-Infective Antisense Strategy and Molecules

INFECTIOUS DISEASES, ANIMAL ANTIMICROBIALS

McGill

Provisional patent
Filing Q1 2026

TRL 2

Research funds raised
Pending results in March 2026

Pre-clinical stage
In vivo efficacy, delivery validation, safety, scalability in progress

Performance
Antisense PNAs shown to downregulate toxin-related genes in vitro

Business Opportunity:
Licensing and Co-development

Market Opportunity:
Global market: $30.8 billion USD in 2025 for Necrotic enteritis
CAGR: 7.2% for Necrotic enteritis

TIMELINE →	Q4 2025	Q1 2026	Q1 2027
Fundamental in vitro principles demonstrated	Completion of final prototype & validation	Filing of Provisional Patent Application	In vivo efficacy & safety data
Antisense PNAs shown to downregulate toxin-related genes			
Clear biological rationale and differentiated mechanism			

THE PROBLEM

Necrotic enteritis (NE), caused by Clostridium perfringens, remains a major economic and animal-health burden in the global poultry industry, with annual losses estimated at US$5–6B.

Historically controlled through antibiotics, NE management is increasingly constrained by regulatory restrictions and antimicrobial resistance (AMR) concerns, while existing alternatives (vaccines, probiotics, feed additives) show variable efficacy.

There is a strong unmet need for non-antibiotic, targeted anti-infective strategies that reduce bacterial virulence while preserving host microbiota and limiting resistance development.

OUR SOLUTION

An antisense peptide nucleic acid (PNA) platform designed to suppress Clostridium perfringens virulence by selectively inhibiting genes involved in toxin production, without impacting bacterial viability.

The technology consists of CPP-conjugated antisense PNAs targeting key regulatory genes at the mRNA level, blocking toxin expression while preserving bacterial growth.

Key elements:

1. PNA antisense oligonucleotides targeting toxin-related mRNAs
2. Cell-penetrating peptide (CPP) conjugation to enhance bacterial uptake
3. Selective inhibition of virulence without bactericidal effect
4. In vitro proof-of-concept demonstrating reduced infectivity with preserved viability

MARKET

Target users include:
- Animal health companies
- Nutrition companies and
- Veterinary pharmaceuticals

Market application:
- Veterinary therapeutics for necrotic enteritis (poultry)
- Antibiotic-alternative solutions for animal health
- Anti-virulence strategies in livestock production
- Longer-term potential extension to other bacterial pathogens (animal or human health)

The innovation developed by Prof. Zhao's team targets the animal antimicrobial market (USD 678.5 million in 2022, 3.6% CAGR). More specifically, the necrotic enteritis treatment market is estimated at USD 30.8 billion in 2025 and is projected to reach USD 61.6 billion by 2035 (7.2% CAGR), with annual poultry industry losses of USD 5–6 billion.

TEAM

Xin Zhao
McGill, Lead PI

Mohamed Elfateh
McGill, Ph.D. candidate

01-2026-001

DNA-Encoded Functionalized Aptamers

ONCOLOGY, INFECTIOUS AND IMMUNE DISEASES

McGill

2 Patents
USA & Canada

TRL 4

Pre-clinical stage
Ex vivo validation in oncological models

$500K CAD
Research funds raised (Médicament Québec grant)

Business Opportunity:
Licensing and Co-development

Market Opportunity:
Global market: $342.5 Billion USD (2026) for aptamers
CAGR: 17.7% for aptamers

TIMELINE

~300,000-member libraries synthesized and screened	High-affinity, nuclease-resistant binders identified	Ongoing target validation in cancer cell models

THE PROBLEM

While aptamers offer a promising alternative to antibodies due to their stability and ease of synthesis, their broader impact is limited by the narrow chemical diversity of natural nucleotides.

This restricted chemical space reduces their ability to engage challenging targets such as protein-protein interactions. Existing strategies to chemically modify aptamers are constrained by enzymatic compatibility, limiting the scope and diversity of non-natural building blocks that can be incorporated.

OUR SOLUTION

A novel type of DNA-encoded library (DEL) in which the ligands are aptamer-like molecules (“alenomers”).

Unlike conventional DELs, these alenomers can explore targets that are typically inaccessible and are designed with improved features compared to standard aptamers.

Our technology allows for the augmentation of the ‘alphabet’ of aptamers beyond 4 DNA bases, creating DELs of alenomers with full freedom in their chemical modifications, as each modification is encoded by a DNA code. This approach generates molecules diversity-superior to antibodies, while also improving their selectivity, binding strength, and nuclease resistance all without the enzymatic compatibility limitations of traditional aptamer discovery.

MARKET

DNA-encoded functionalized aptamers are versatile molecular tools with applications in diagnostics, therapeutics, and targeted drug delivery. Their programmable structure and ability to incorporate diverse chemical modifications allow high-specificity binding and screening of vast chemical spaces in a modular and scalable way.

They can be integrated into biosensors (aptasensors) for rapid, sensitive detection of disease biomarkers, pathogens, and toxins, and combined with nanoparticles for enhanced imaging in PET, MRI, and optical techniques. Their flexibility supports point-of-care diagnostics, microfluidic platforms, and multiplexed assays for simultaneous detection of multiple analytes.

The global aptamer market, including DNA-encoded functionalized aptamers, is rapidly expanding.
Valued at USD 2.34–3.27 billion in the early 2020s, it is projected to grow to ~USD 10.9 billion by 2030, ~USD 17.9 billion by 2032, and potentially USD 23–26 billion by 2034–2035, with CAGR ranging from 19 % to 24.6 %.

This growth reflects the increasing adoption of aptamers in various applications, highlighting their high commercial potential and versatility in biotechnology and medicine.

TEAM

Hanadi Sleiman
McGill University, Co-PI

Maureen McKeague
McGill University, Co-PI

Fiona Ebanks
McGill University

01-2026-001

Layer-by-Layer Lipid Nanoparticles for Enhanced Gene and Drug Delivery

ONCOLOGY, IMMUNOTHERAPY

CHU Sainte-Justine
Le centre hospitalier universitaire mère-enfant
Université de Montréal

US Patent pending
US19/160,019

TRL 3

Pre-clinical stage
Extra-hepatic delivery in vivo of dual payloads

$300K
Research funds raised

Business Opportunity:
Licensing and Co-development

Market Opportunity:
Global market: $101.2B USD for lipid nanoparticles
CAGR: CAGR 9.3% (2028)

TIMELINE → **Q1 2026** **Q1 2027**

In vitro and in vivo proof-of-concept of targeting efficiency (Done)	LNPs stability after lyophilization validated (Done)	Validation of LNP platform on first indication: CAR-M	Validation of LNP platform on second indication: Glioblastoma	Scalability potential under investigation

THE PROBLEM

The development of nanocarriers capable of encapsulating, targeting, and delivering Drug Substances (DS) has become the main challenging issue during the last three decades for biotechnology and pharmaceutical industries.

Ideally, an optimal drug delivery system would be able to promote high encapsulation efficiency and adequate stability of DS in both storage and physiological conditions, while maintaining bioactive properties of loaded cargo.

Moreover, the carrier platform would be easily modulated to target specific sites, allowing controlled spatial and temporal delivery of DS. Successful accomplishment of those hurdles has been expected to allow improvement of bioavailability of DS as well as to minimize side effects for patients. Lipid-based nanocarriers have been frequently proposed to achieve such mentioned requirements.

OUR SOLUTION

A system that combines the advantages of LNPs and layer-by-layer assembly (LbL)

The LbL technique allows surface modification of LNPs, thus creating an organized multilayered structure with tunable properties, functionalities, and applications. It results in a more efficient targeting process and gene delivery as compared to plain LNPs, or nanoparticles in general.

The system also provides a simple and effective way to develop drug combinations. Such combinations may include encapsulated nucleic acids and hydrophobic drugs for dual targeted treatment. In vitro and in vivo results obtained with these proposed LbL assembled LNPs demonstrate superior targeting ability as compared to commercially available delivery agents, enhanced gene delivery when compared to non-modified LNPs (current gold standard for gene delivery) and blood barrier crossing properties.

What sets our LbL apart is their remarkable stability after lyophilization, maintaining size, charge, and functionality. This ensures extended shelf life, efficient rehydration, and structural integrity. Additionally, their design supports hepatic bypass, reducing clearance and enhancing the bioavailability of active molecules for targeted clinical use.

MARKET

Target users include:
- Biotech and pharma companies
- Research centres and academia
- CRO and CDMO

Market application:
- Drug development and formulation
- Drug delivery systems
- Gene therapy

- The global market for nanoparticles for life science applications reached $102.7B USD in 2023 and is expected to grow to $156.8B USD in 2028.
- The main application is drug delivery systems, which in 2023 accounted for just over 50% of the market, or $53.8B US.
- In terms of the type of nanoparticles, liposomes represent the largest market (65%; $101.2B US in 2028) and the fastest growth rate (CAGR 9.3% in 2028).

TEAM

Pierre Hardy
CHUSJ, Lead PI

Xavier Banquy
Université de Montréal

Houda Tahiri
CHUSJ

Victor Passos Gibson
CHUJ

Chun Yang
CHUSJ

01-2026-001

Prevention of Cancer/Metastasis Through an Antibody-Oligo-Conjugate (AOC) Platform

ONCOLOGY AND IMMUNOTHERAPY

IRCM

3 patents
1 active, 2 filed

TRL 4

[Pre-]clinical stage
In vivo validation of inhibition via a developed AOC

$1.55M CAD
Research funds raised

Performance:
- 90% pancreatic tumor reduction in KO mice
- 50-80% checkpoint inhibition in KO model and in human cells

Incorporated: April 2025

Business Opportunity:
Co-development and Investment

Market Opportunity:
Global market: $161 Billion USD (2034) for T-cell immunotherapy
CAGR: 35% for T-cell immunotherapy

TIMELINE

Q3 2025	Q1 2026	Q2 2026	Q2 2026	Q2-Q3 2026
In vitro and in vivo proof-of-concept for inhibition completed in KO	Selection and optimization of oligos is finalized and patent filed	AOC developed for drug candidate	Ex-vivo inhibition	In vivo AOC mice studies

THE PROBLEM

Current immune checkpoint inhibitor therapies (i.e. Keytruda) fail in nearly 80% of patients with solid tumors and are ineffective in most T-cell malignancies.

Resistance results from the redundancy of control pathways (PD-1, TIM-3, TIGIT, etc.), while antibody-combining strategies result in systemic toxicity.

OUR SOLUTION

Silengenics' AOCs will deliver oligonucleotides directly into immune cells to inhibit from within via inhibition of a main regulatory gene that has an effect on the degradation of several immune checkpoints, thus bypassing surface targeting.

1. Targeted administration: AOCs use disease-specific markers (PD-1, TIM-3, CD5); thus contributing to the therapeutic effect
2. Intracellular silencing (via new patent-protected target): Oligonucleotide payload reduces the expression of multiple checkpoints by up to 80% via inhibition of a single gene (PD-1, TIM-3, LAG-3, TIGIT)
3. Precision reprogramming: reverses immune exhaustion and suppressive phenotypes without systemic cytokine storm
4. The platform can then be used to deliver other immunotherapies in vivo.

MARKET

Target users include:
- Biotech and pharma companies
- Research centres and academia

Multiple commercialization pathways:
- Pancreatic cancers
- Colorectal cancers
- Melanoma cancers
- T cell immunotherapy
- Any cancer for which immunotherapies currently exist

The pancreatic cancer treatment market reached $US 3B in 2023 and is expected to grow to $US 9.2B in 2032, showing a CAGR of 13.2%.

The overall colorectal cancer treatment market reached $US 12.23B in 2023 and is expected to reach $US 18.25B in 2032, with a CAGR of 4.5%.

The CAR-T therapy market was valued at $US 5.5B for 2024 and is expected to grow to $US 29B in 2029, following a very significant CAGR of 39.6%.

TEAM

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01-2026-001

RNOVA Tx: Innovative antisense strategies targeting SRSF3 to reprogram immune response in neurodegenerative disorders

ALS, NEURODEGENERATIVE DISEASES

UNIVERSITÉ LAVAL

5 Patents
2 active, 3 pending

TRL 4

Performance
ALS: 11% lifespan increase
AD: 43% Aβ plaque decrease, 47% pTau decrease

Pre-clinical stage
In vivo mouse ALS/FTD & AD model

$1.5M CAD
Research funds raised

$80K CAD
Private funds raised

RNOVA Tx
Incorporation: Q1 2026

Business Opportunity: **Licensing and Co-development**
Pre-seed investment

Market Opportunity: **Global market:** $667M USD (2023) for ALS
CAGR: 5.8% for ALS

TIMELINE → **Q3 2026** **Q2 2027**

Principle of SRSF3 knockdown demonstrated in vitro and in vivo	Intellectual property secured	In vitro efficacy assays with optimized formulation completed	Formulation design completed; CMC ongoing	In vivo efficacy to be completed	Pre-clinical proof of concept

THE PROBLEM

ALS is a neurodegenerative disorder characterized by a progressive muscle weakness followed by a lethal paralysis, currently affecting ~300,000 individuals worldwide.

There are no efficient treatments against this devastating disease, as the only 2 drugs currently available in Canada (riluzole and edaravone) have demonstrated modest benefit to survival and/or function in ALS patients. They have been shown to increase survival by 2-3 months against the harsh reality that ALS patients face: they are typically given a 2-to-5-year prognosis upon diagnosis. Although no known causes are identified, recent evidence revealed that over the progression of disease, brain immune cells such as microglia gradually lose their immune functions and develop unconventional toxic phenotypes. This suggests the implication of pathogenic molecular pathways that can be targeted to restore microglial function, slow disease progression, and maximize quality of life.

OUR SOLUTION

We identified a novel and previously unknown role for the RNA binding protein SRSF3 as a master suppressor/regulator of innate immune gene translation.

Targeting the expression of SRSF3 in microglial cells and macrophages represents an entirely novel immunomodulatory approach for treatment of neurodegenerative diseases. Initial studies revealed that therapeutic knockdown of SRSF3 alleviates translational suppression of selected immune genes and significantly impacts microglial activation, i.e., restores the phagocytic capacity and protective profile of microglia and substantially extends survival – even in late-stage treatment – in the aggressive SOD1G93A model of ALS. These data collectively show that targeting SRSF3 can modulate and reprogram the innate immune response in microglia/macrophages and thereby provide potential for a transformative therapeutic effect in ALS and neurodegenerative diseases more generally – a departure from conventional approaches based on blocking and/or attenuating inflammation.

MARKET

Neurodegenerative disorders are among the most serious health problems facing modern society today. Many of these disorders become more common with advancing age, including amyotrophic lateral sclerosis (ALS), Alzheimer's disease (AD), Parkinson's disease (PD), and frontotemporal lobar degeneration (FTLD). The burden of these diseases is growing inexorably as the population ages, with enormous potential economic and human costs. In terms of economics, the global ALS market was estimated around $667M USD in 2023 and growing (CAGR of 5.8%), while the value of the global dementia therapeutics market size was $17B USD in 2023 and is expected to grow to around $28B USD by 2030 (CAGR of 7.4%).

Compounds in ongoing clinical trials targeting various disease mechanisms may provide some additional functional benefits, though none have yet shown the potential to have a transformative impact on disease. An exception may be argued for Tofersen, although it is indicated only for patients positive for mutant SOD1 disease, which represents roughly 2% of the ALS patient population.

Effective pharmacologic therapies with potential for meaningful impact on the broader population of ALS patients (as well as for patients suffering from other neurodegenerative disorders) are still desperately needed.

TEAM

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Chemically Modified CRISPR crRNA for Advanced Gene Editing Applications

GENE THERAPY, ONCOLOGY

McGill

2 Provisional patents
Filed Summer 2025

TRL 3

Pre-clinical stage
In vitro editing efficacy completed

Business Opportunity:
Licensing or Co-development

Market Opportunity:
Global market: gRNA market ~3.17 billion USD by 2034
CAGR: 18% for gRNA

TIMELINE

Chemically modified crRNAs synthesized	In vitro cell-based validation of gene-editing capability	Strong mechanistic rationale and differentiation vs unmodified crRNAs	Optimal patterns for full modification are close but not yet selected. No in vivo efficacy, PK, or safety data	Head-to-head benchmarking vs industry-standard crRNAs

THE PROBLEM

CRISPR-based gene editing faces persistent limitations related to RNA instability, off-target effects, immune activation, and toxicity, which restrict therapeutic applicability.

CRISPR-Cas9 and CRISPR-Cas12a systems, both of which we have made significant progress in, offer unique features for development but their guide RNAs remain highly susceptible to nuclease degradation, limiting in vivo performance. There is a strong unmet need for next-generation crRNA chemistries that improve stability and performance of Cas9 and Cas12a systems while maintaining editing efficiency and low toxicity.

OUR SOLUTION

The developped technologies are chemically modified CRISPR-Cas12a and CRISPR-Cas9 crRNAs designed to enhance RNA stability, reduce toxicity, and preserve high gene-editing efficiency.

The invention combines specific chemical modifications at defined positions of the crRNA backbone, including:
2'-AraOH, 2'-5'-RNA, 2'F-ANA, 2'-OMe, 4'-OMe, 4'-F-RNA, 2'-Fluoro, and phosphorothioate (PS)

These modifications are used in combinatorial and position-specific patterns to:

- Protect crRNA from exonuclease degradation
- Maintain or enhance editing efficiency
- Reduce off-target effects and cellular toxicity

Newly synthesized modified crRNAs have been validated in vitro for gene-editing performance.

MARKET

Target users include:
- CRISPR reagent manufacturers
- Gene-editing biotechnology companies
- Pharmaceutical companies developing CRISPR-based therapeutics

Multiple commercialization pathways:
- Sale of modified crRNA products
- Licensing of proprietary crRNA chemistry
- Integration into CRISPR platform companies

- The gRNA market includes products and services and should reach 3.17 billion USD by 2034, accelerating through a high CAGR (18%) for the period.
- North America represents 54% of this market, reaching 323 million USD in 2024.
- The research-use segment dominates the gRNA market and the "custom gRNA synthesis services" segment is expected to grow significantly.
- The GMP-grade segment, required for CRISPR-based therapeutics or clinical trials, is also expected to grow significantly.
- The gRNA market is highly influenced by the CRISPR market, which is expected to increase at 15.6% growth rate (CAGR) between 2023 and 2028.
- In 2028, the segment of the services should reach 1.6 billion USD and represent 23% of the CRISPR market.

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