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

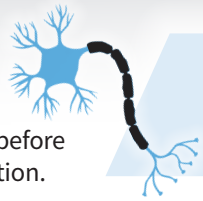
Expanded Indications:
- Parkinson's & Huntington's model validation

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

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