Technology Offer

METHODS AND COMPOSITIONS FOR THE TREATMENT OF PROTEINOPATHIES

A novel first-in-class therapeutic platform based on proprietary New Chemical Entities (NCEs) targeting retroviral-like ARC capsids to halt neurodegenerative progression in ALS and FTD

Research goal

The progressive spreading of misfolded proteins within the CNS drives the relentless clinical decline in Amyotrophic Lateral Sclerosis (ALS) and Frontotemporal Dementia (FTD). Our main objective is to develop and commercialize a disruptive therapeutic platform centered on proprietary New Chemical Entities (NCEs) designed to target the neuronal protein ARC. By blocking ARC capsid assembly, these small molecules establish a vector-centric mechanism to intercept extracellular toxic transmission. This target-focused approach is fully validated by a solid, protected pre-clinical Proof of Concept (PoC) utilizing Lenacapavir as a structural tool compound.

Problem to solve

Current neurodegenerative treatments are purely palliative because they focus on a somatic paradigm, trying to repair dying neurons as isolated units while failing to stop the disease from spreading. We discovered that the protein ARC self-assembles into virus-like capsids that act as a pathological vector, encapsulating and transporting toxic protein seeds (TDP-43/tau) and transcripts between glia and neurons. No current therapies target this extracellular transmission vehicle. While we validated that this mechanism can be intercepted using a repurposed tool compound, overcoming blood-brain barrier (BBB) limitations requires dedicated, proprietary next-generation small molecules (NCEs).

Innovative Aspects

- **First-in-Class Target Platform**: ARC identified as the core retroviral-like vehicle driving intercellular toxic seeding and clinical progression in multiple proteinopathies.
- **Protected Repurposing PoC**: Robust *in vitro* (human cells) and *in vivo* (Drosophila) data prove that ARC inhibition via Lenacapavir rescues locomotor deficits and extends lifespan, with findings protected under our patent application.
- **Proprietary NCE Pipeline:** Active Structure-Based Drug Design (SBDD) and AlphaFold modeling to synthesize a unique library of small molecules optimized for human-ARC affinity and superior BBB penetration.
- **Validated Screening Assays:** Operational high-throughput protocols utilizing laboratory-produced self-assembling human ARC capsids to rapidly profile candidate compounds**.**

Market opportunity

ALS and FTD pose a catastrophic burden, with intensive care costing €80,000–€100,000 per patient annually. Halting this progression turns fatal trajectories into manageable chronic states, creating a highly sustainable commercial model. Our protected pre-clinical PoC significantly de-risks the asset, enabling fast-track orphan pathways before scaling the platform into massive markets like Alzheimer's. Orally available small molecules maximize cost-effectiveness over expensive, invasive biologics.

Intellectual Property:

- Priority European patent application filed (September 19, 2025) **EP25383004.6**
- Suitable for international extension (PCT application)

Development stage:

Available for: *Licensing, co-development*

Aims

Looking for a partner interested in a license and/or a collaboration agreement to develop and exploit this asset.

Contact details

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