Technology Offer

ARGININE-FUNCTIONALIZED NANOPARTICLES FOR TARGETED SIRNA DELIVERY IN GLIOBLASTOMA

An innovative nanoparticle-delivered siRNA therapy targeting key oncogenic pathways for the precision treatment of aggressive brain tumors

Research goal

To advance a first-in-class, non-viral RNAi therapeutic designed to cross the blood-brain barrier and treat glioblastoma. By combining proprietary, highly specific siRNA sequences with synthetic arginine-functionalized scaffolds, the formulation self-assembles into stable nanoparticles. This complete therapeutic candidate protects the nucleic acid payload from degradation and achieves targeted gene silencing of critical oncogenic drivers (p42-MAPK/Rheb) directly within malignant brain cells to halt tumor progression.

Problem to solve

Approved therapeutic options for aggressive brain tumors like glioblastoma suffer from dismal prognoses due to poor drug accumulation within target tissues. While siRNA offers massive potential for personalized gene modulation, naked nucleic acids exhibit zero membrane permeability and are rapidly degraded by biological fluids. Current non-viral viral vectors lack tissue specificity, introduce severe safety risks, or completely fail to bypass the endothelial tight junctions of the BBB, creating a prominent bottleneck for effective CNS disease management.

Innovative Aspects

- **Targeted Therapeutic Payload:** Integrated with highly specific siRNAs designed for the targeted knockdown of key glioblastoma drivers (p42-MAPK/Rheb), driving oncogenic suppression.
- **Exceptional Antitumor Efficacy:** Demonstrated potent target knockdown (below 50%) in patient-derived glioblastoma organoids (dPDOs) without causing toxicity in healthy primary cultures.
- **Effective CNS Delivery & Target Engagement:** *In vivo* mouse imaging confirms successful crossing of the BBB and stable therapeutic accumulation within the brain and spinal cord after standard systemic administration.
- **Favorable Safety Profile:** High biocompatibility with zero hemolysis, normal coagulation parameters, and no systemic liver or metabolic toxicity in animal models

Market opportunity

Glioblastoma represents a high-priority orphan indication with urgent clinical needs and fast-track regulatory eligibility. This platform de-risks drug development by turning undruggable genomic targets into accessible precision therapies. Formulated as stable, scalable small molecules for routine intravenous administration, it completely avoids the complex manufacturing and prohibitive costs associated with traditional gene therapies or invasive neurosurgical biologics.

Intellectual Property:

- Priority European patent application filed (June 24, 2026) **EP26382886.5**
- 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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