

Hepatoblastoma siRNA

Unmet Need

- Hepatoblastoma is the most common primary liver tumor in children (0–4 years).
- Annual incidence: 1.8 cases per million.
- Current treatments (surgery, chemotherapy, liver transplantation) have ~12% relapse rate.
- Relapsed tumors often show chemoresistance, linked to poor prognosis.

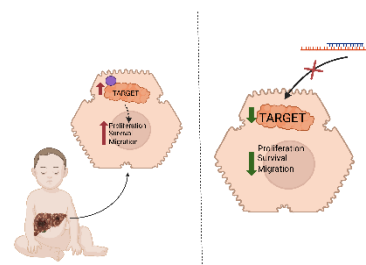
Current Therapeutics Options:

Modality	Role	Limitations
Surgical resection	Gold standard in localized disease; best chance for cure when complete removal is possible.	Many tumors are unresectable at diagnosis due to size, multifocality, or involvement of critical structures.
Liver transplantation	For unresectable but liver-confined disease; can achieve cure in some cases.	Donor shortage, surgical risk, need for lifelong immunosuppression, high cost.
Chemotherapy (cisplatin-based regimens etc.)	Used pre-operatively to shrink tumors; post-operatively to reduce recurrence; also in metastatic disease.	Toxicity; some tumors are or become resistant; side effects in young children.
Supportive care monitoring	For small/stable lesions or when treatment is not tolerated.	Does not reliably prevent progression or relapse; not curative.

Our Solution

We have developed a proprietary siRNA therapeutic that precisely silences a oncogenic driver of hepatoblastoma. Key benefits:

- **Precision targeting** of liver tumor cells.
- **Enhanced safety** – reduced systemic exposure vs. conventional nanoparticles.
- **Chemoresistance bypass** by silencing survival and resistance pathways.
- **Adaptability** – compatible with patient-specific oncogene profiles.
- **Clinically validated platform** – GalNAc conjugation already proven in approved siRNA drugs.



Technology Highlights

- Mechanism: siRNA triggers RNA interference to silence disease-causing mRNAs.
- Delivery: Nanoparticle/lipid carriers ensure liver-targeted action.
- Versatility: Applicable in both primary and relapsed hepatoblastoma.
- Personalization: Customizable to patient-specific tumor profiles.

PATENT STATUS **Priority filing March 2025**

STATE OF THE TECHNOLOGY R&D. in vivo Proof of concept generated

Collaboration Opportunities

We are seeking partnerships with:

- Pharma companies (pediatric oncology, RNA therapeutics)
- Clinical Research institutions (for preclinical & clinical validation)
- Investors (to accelerate translational development)

CONTACTS

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