

Targeted miRNA Approach to Pediatric Diffuse Midline Glioma

Tech ID: 34827 / UC Case 2026-407-0

ABSTRACT

Researchers at the University of California, Davis have developed a treatment method leveraging let-7 microRNA restoration to combat aggressive pediatric diffuse midline gliomas with H3K27M mutations.

FULL DESCRIPTION

This technology involves a novel therapeutic strategy targeting pediatric diffuse midline gliomas harboring the H3 K27M mutation, a form of aggressive brain tumor with poor prognosis. Utilizing miRNA sequencing, it was found that the tumor suppressor let-7 family of microRNAs is significantly downregulated in these mutant glioma cells compared to wild-type. The method delivers synthetic let-7 miRNA molecules, stabilized oligonucleotides, or gene vectors encoding let-7 precursors directly to tumor cells to restore their expression, thereby repressing oncogene activity, inhibiting tumor growth, and improving patient outcomes. Delivery systems include lipid nanoparticles, viral vectors, and polymer-based carriers designed for targeted, controlled administration. The approach also explores modulation of LIN28, a protein that blocks let-7 maturation, as an adjunct therapeutic intervention.

APPLICATIONS

- ▶ Therapeutic development for pediatric diffuse midline gliomas and other H3K27M mutant gliomas.
- ▶ RNA-based therapies targeting oncogenic pathways in brain tumors.
- ▶ Precision medicine approaches in pediatric neuro-oncology.
- ▶ Development of delivery platforms for microRNA therapeutics in CNS cancers.
- ▶ Potential expansion into treatments for other cancers with let-7 microRNA dysregulation.

FEATURES/BENEFITS

- ▶ Restores tumor-suppressor let 7 microRNAs reduced by the H3K27M mutation.
- ▶ Inhibits core oncogenic programs that control tumor-cell growth and differentiation.
- ▶ Enables precise spatial and temporal control by leveraging versatile delivery modalities.
- ▶ Amplifies therapeutic impact by combining multiple let 7 family members and/or complementary regulatory RNAs.
- ▶ Provides a potential systemic treatment option beyond radiation.
- ▶ Addresses the lack of effective systemic therapies for aggressive pediatric diffuse midline gliomas with H3K27M mutation.
- ▶ Counteracts mutation-driven suppression of let 7 tumor-suppressor microRNAs that drives uncontrolled proliferation.

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OTHER INFORMATION

KEYWORDS

brain tumor, diffuse
 midline glioma, h3 k27m,
 let-7 microRNA, lin28,
 miRNA therapy, pediatric
 cancer, RNA delivery,
 tumor suppressor, viral
 vectors

CATEGORIZED AS

- ▶ **Biotechnology**
- ▶ Genomics
- ▶ Health

RELATED CASES

2026-407-0

► Targets underlying genetic and epigenetic disease drivers that current options do not directly treat.

PATENT STATUS

Patent Pending

ADDITIONAL TECHNOLOGIES BY THESE INVENTORS

► [Calcium Signaling as a Target for Medulloblastoma](#)

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