

Candidate selection of a first-in-class RNA therapeutic for the treatment of BRAF mutant pediatric brain tumors



ONCOLOGY	Candidate
Product Type	Antisense oligonucleotide (ASO)
Indication	BRAF mutant pediatric glioma
Target	BRAF mRNA
MoA (Mechanism of Action)	RNase H1-dependent degradation of the target, BRAF mRNA
Competitiveness	VS. BRAF kinase inhibitors <ul style="list-style-type: none"> ① Proven delivery to the brain (bypassing BBB) ② Effective against various BRAF mutations ③ Low risk of drug resistance ④ Low risk of systemic exposure/adverse effects ⑤ Long duration of effect (monthly to quarterly dosing)
Development Stage	Candidate
Route of Administration	Intrathecal injection