

RNA Editing Therapeutic Platform

First Programmable RNA Cut-and-Repair Technology

THE NEAR-TERM OPPORTUNITY

40,000+ Disease-causing nonsense mutations in ClinVar

Current RNA approaches (RNAi, ASOs, Cas13) can only degrade transcripts—they cannot repair and restore protein function. DNA editing carries permanent genome modification risks.

BREAKTHROUGH TECHNOLOGY

Type III CRISPR RNA Editing

- ✓ Programmable RNA cut, stitch, and repair
- ✓ Precise excisions; >90% target RNA degradation
- ✓ Sequence insertion (oligo splints) capabilities
- ✓ Endogenous RTCB ligase repairs breaks
- ✓ Restores protein function
- ✓ Research tool → clinic commercial pathway

CRISPR PIONEER & PROLIFIC INNOVATOR



Blake Wiedenheft, PhD

- NIH-Funded CRISPR Pioneer
- Widely published in *Science*, *Nature*, *PNAS*, *Cell*, and more
- PECASE Award Recipient
- Doudna Lab Alumnus
- Endowed Chair, Montana State

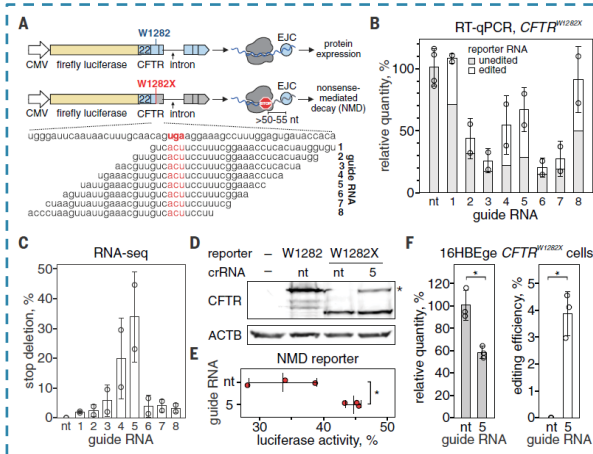
PROOF-OF-CONCEPT DATA

65.8%
Editing Efficiency

5.3%
Protein Restored

>30%
Improved Ligation Potential*

Programmable RNA excision rescues CFTR expression in cystic fibrosis cells harboring W1282X (Science, 2024):



LEAD INDICATION

Cystic Fibrosis (nonsense mutations)

- ✓ No approved therapy
- ✓ Defined patient population (high unmet need)
- ✓ MOA: Function CFTR protein translation
- ✓ Defined regulatory path

Regulatory Path: RNA Therapy (Spinraza, etc.)

Delivery: LNPs with repeat dosing

Proof-of-concept established; defined pathway for improved protein rescue

PLATFORM CAPABILITIES

R&D / Ex Vivo Tool Precise cell engineering (no collateral damage)

Duchenne MD Dystrophin reading frame

Retinal Diseases CEP290 mutations

Huntington's Disease CAG repeats

Oncology Fusion transcripts; Micropeptide regulation

PARTNERSHIP OPPORTUNITIES

- Licensing
- Sponsored Research
- Co-Development
- Strategic Investment



COMPETITIVE ADVANTAGE

	RNAi	Cas13	Type III
Protein Rescue	X	X	✓
Precision	Moderate	Low	High
Sequence Agnostic	N/A	X	✓
RNA Insertion	N/A	X	✓

Publication: Science | DOI: 10.1126/science.adk5518

*Additional references available upon request

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IP Status: 5 Issued / Active Patents & Applications

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