

# RNA Editing Therapeutic Platform

Fist 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

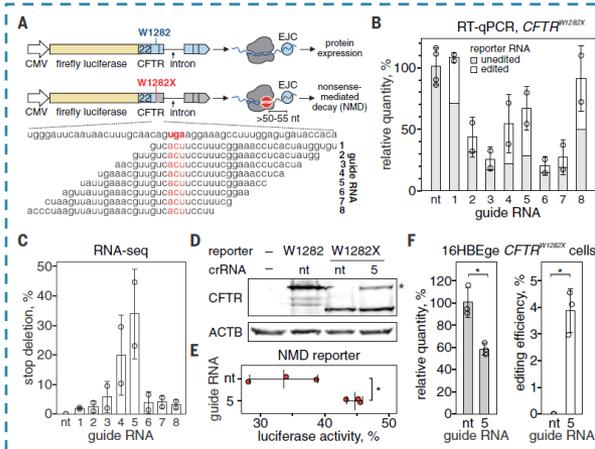
## PROOF-OF-CONCEPT DATA

**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	✓