

The background features a complex network diagram with nodes and edges in various colors (pink, yellow, blue, red) forming a large, intricate structure on the right side. The overall background has a light gray hexagonal pattern.

emendo<sup>bio</sup>

# OMNI<sup>TM</sup> Technology Platform

*Superior Performance through AI-Driven Design*

An  es

# About EmendoBio

- Founded in U.S. in 2016 by scientists from the Weizmann Institute, Israel
- Founding investors: OrbiMed and Takeda Ventures
- AnGes became a majority shareholder in December 2020

## Management

**Naoya Satoh, PhD**  
President & CEO

**Assaf Sarid**  
CFO

**Idit Buch, PhD**  
VP, Computational  
Biology

**Ella Segal**  
VP, Research and  
Analytical Sciences

## Board of Directors

**Ei Yamada, PhD**  
AnGes

**Naoya Satoh, PhD**  
AnGes

**David C. Dale, MD**  
Former Dean  
UW Medical School



**Stephen Tsang, MD**  
Clinical Geneticist  
Columbia University



**Harry Malech, MD**  
Chief Genetic  
Immunotherapy, NIH



**David Rawlings, MD**  
Director Immunity and  
Immunotherapies, SCRI



**Andrew Kung, MD PhD**  
Chair Dept. Peds. Sloan  
Kettering



Memorial Sloan Kettering  
Cancer Center..

# Current Limitations of Gene Editing



## Safety

- Off-target effects
- Translocations



## Editing Strategy

- PAM availability
- Allele specific editing
  - Mutations
  - SNPs
  - Enhancer sites
  - Splice donor / acceptor



## Delivery

- Packaging limitations
- Tissue specificity



## Immunogenicity

- Anti-nuclease antibodies
- Cytotoxic T cells



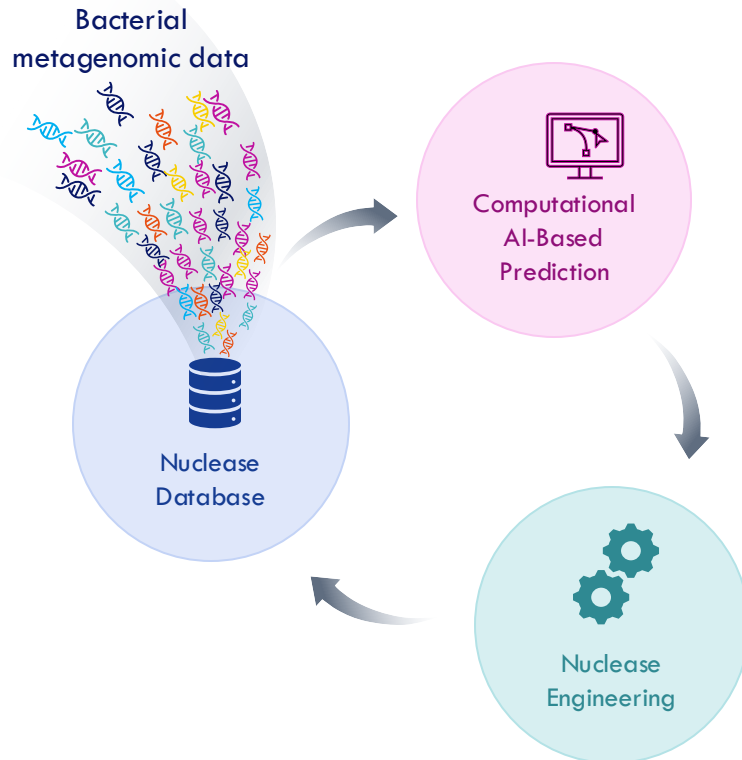
## IP

- Nuclease
- Guide RNA (gRNA)

# OMNI™ Platform Offers a Variety of Gene-Editing Solutions

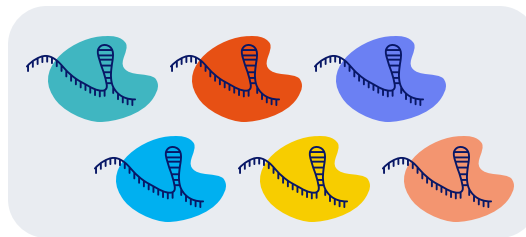
Synergistic discovery, engineering and computational technologies combine to produce a portfolio of high-performance OMNI™ type-II nucleases

## EmendoBio's Platform



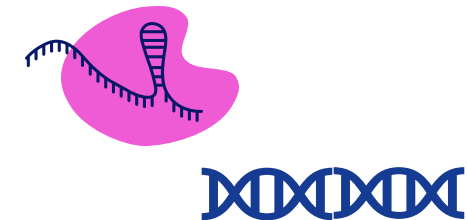
## Panel of Engineered OMNI™ Nucleases

- ✓ Novel
- ✓ Highly active
- ✓ Highly specific



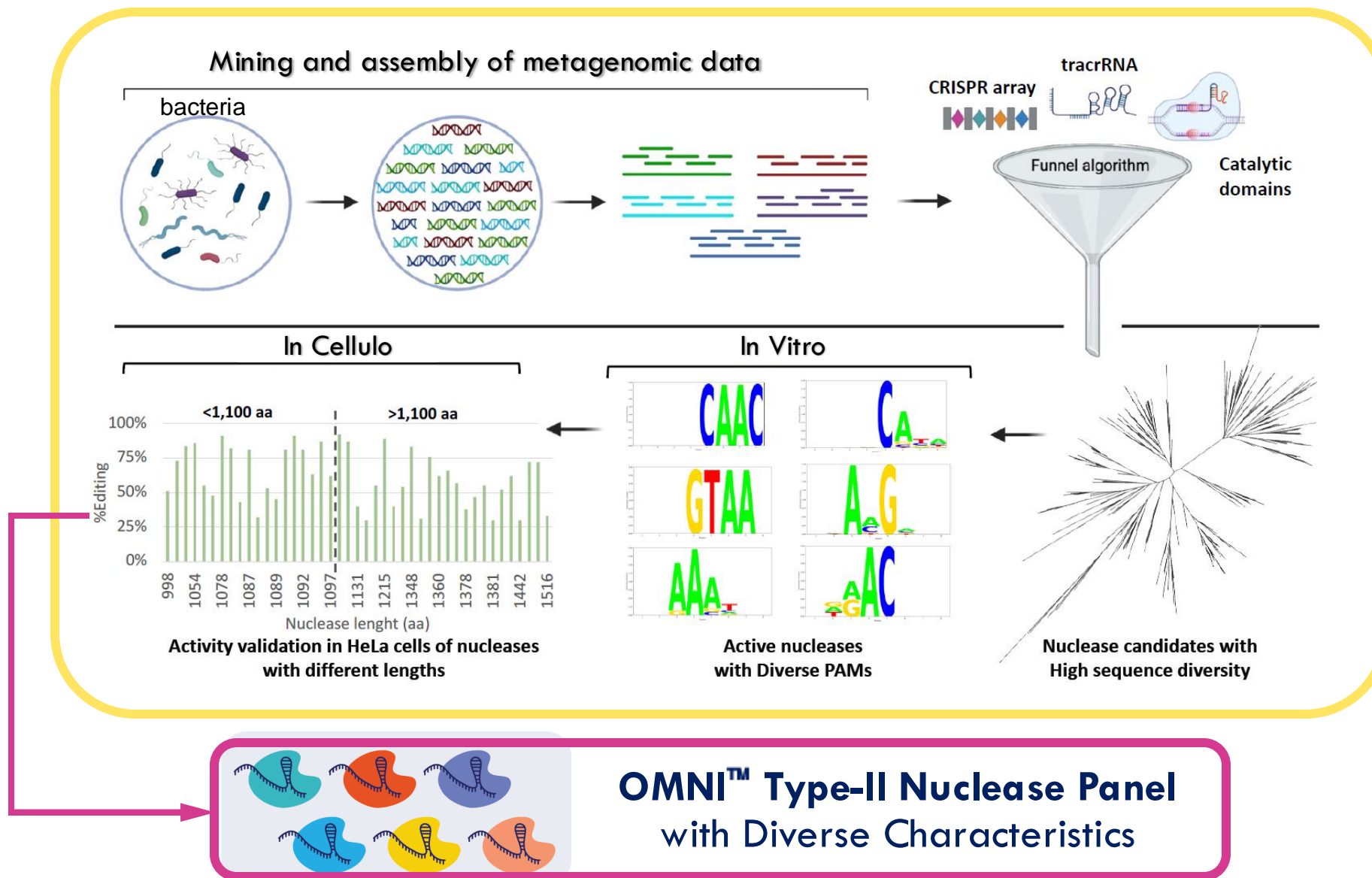
## Optimal Therapeutic Compositions per target

- ✓ High safety profile
- ✓ Expanded range of applications
- ✓ Freedom to operate





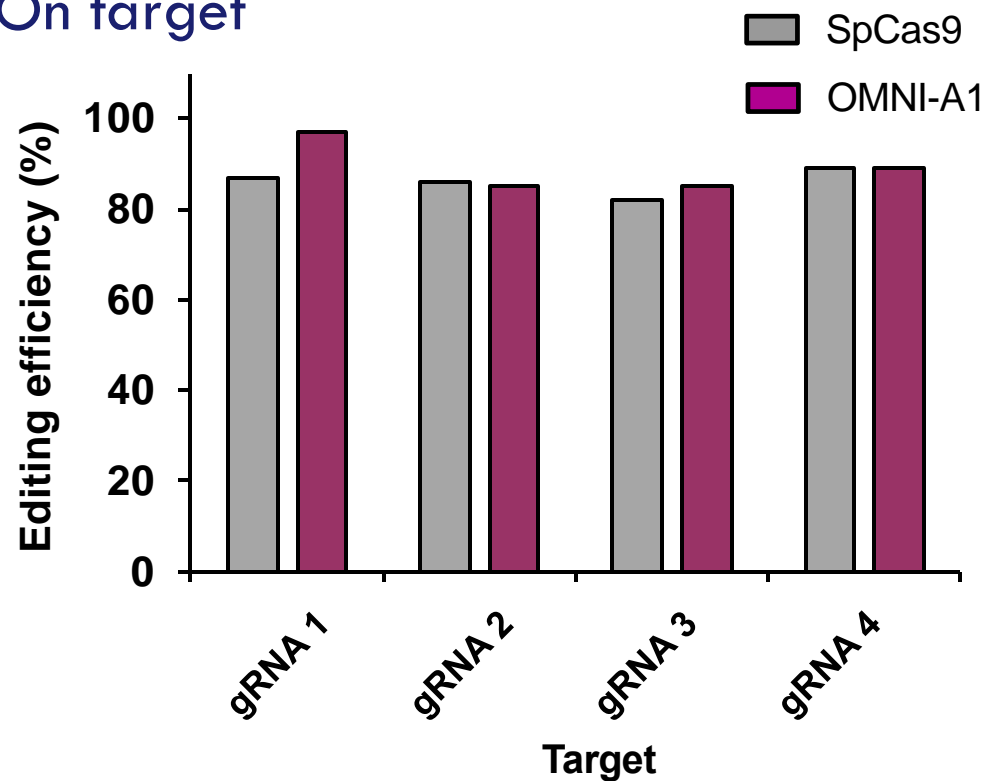
# Nuclease Discovery



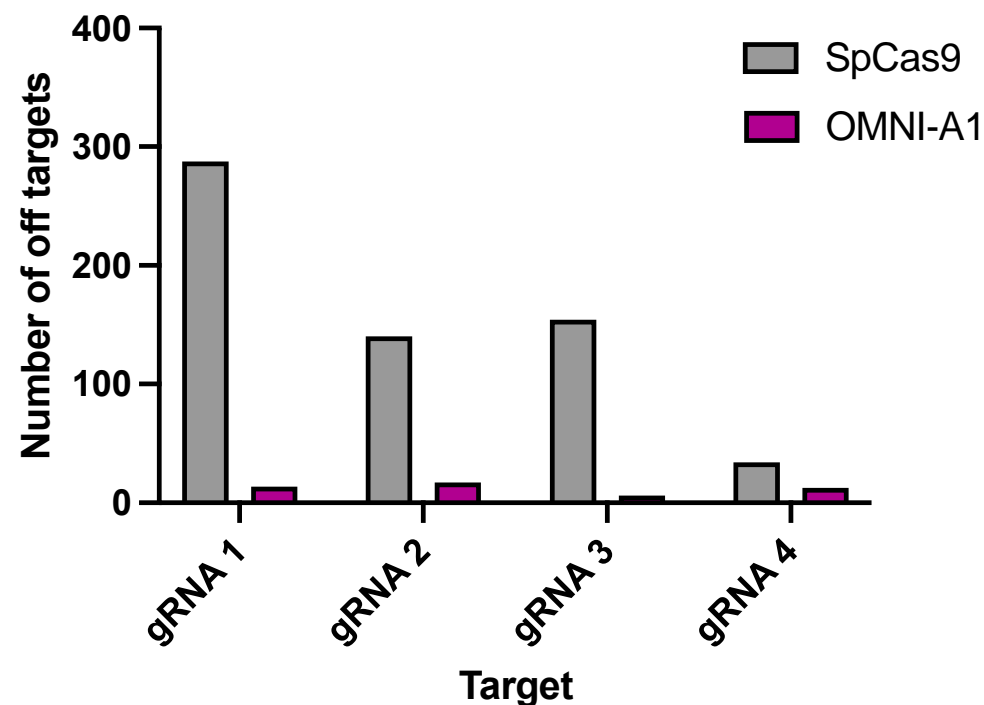
# Activity and Specificity of OMNI-A1™ (1,370aa)

## OMNI-A1™ vs SpCas9

### On target



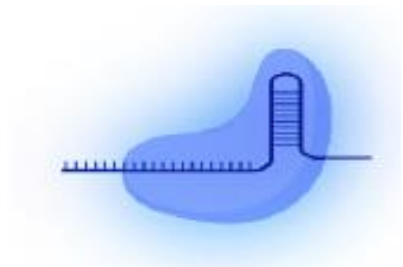
### Off target



OMNI-A1™ has higher specificity compared to SpCas9

# Nuclease Engineering Platform

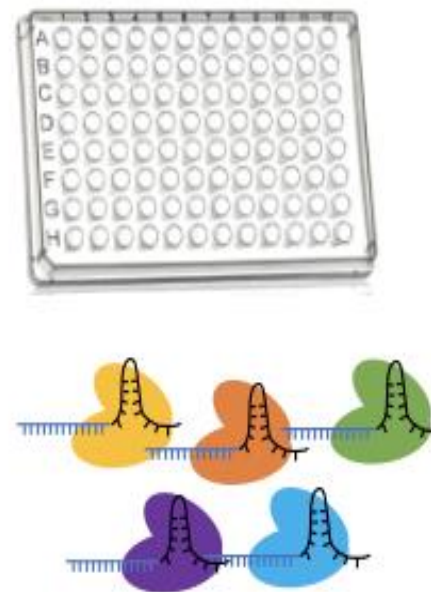
OMNI™ nuclease  
(from panel)



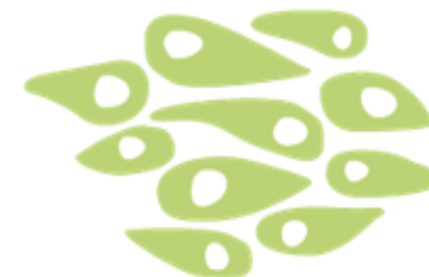
AI based engineering for  
variant library generation



Libraries of nuclease  
variants



Screening in mammalian  
cell line

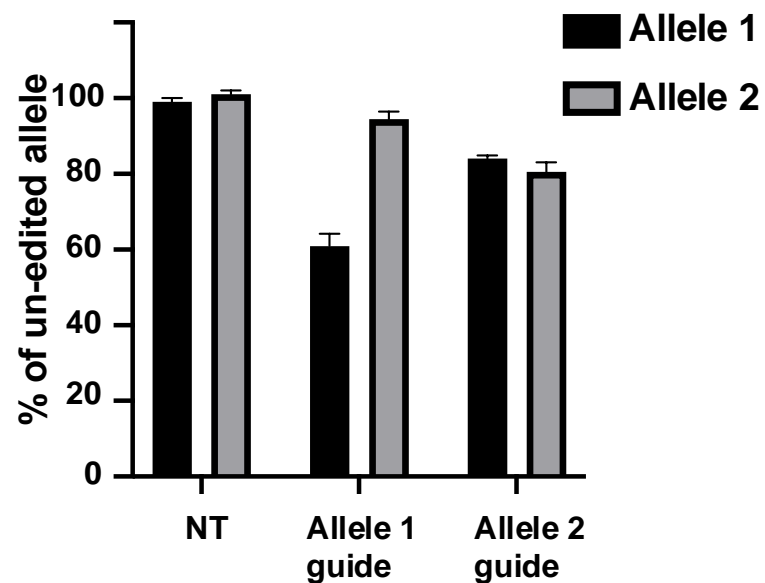


Highly Active and Specific  
**Optimized OMNI™ Variants**

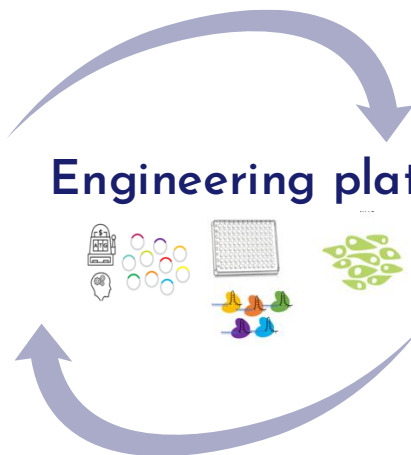
# Increased Specificity

OMNI-A1™ – powerful engineering platform

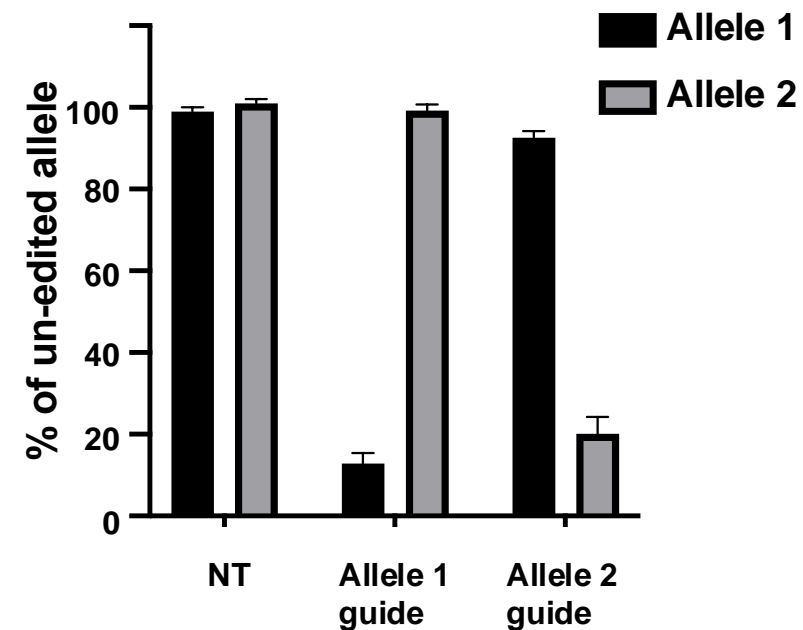
OMNI-A1™



Engineering platform



OMNI-A1 V10™





# Non-Compromised Nuclease Safety

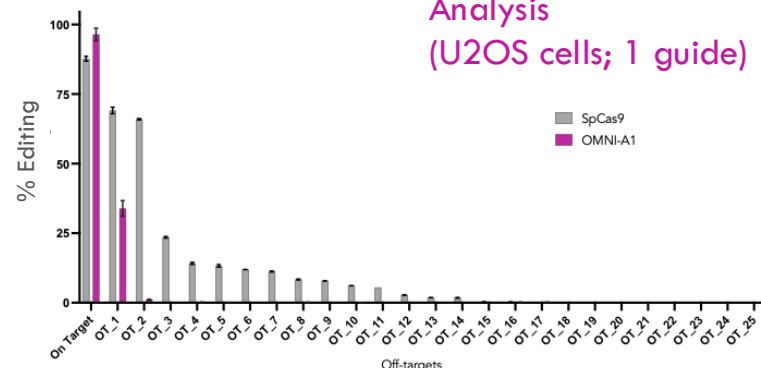
Engineering platform achieves systematic elimination of off-targets

Optimized to be highly active and specific

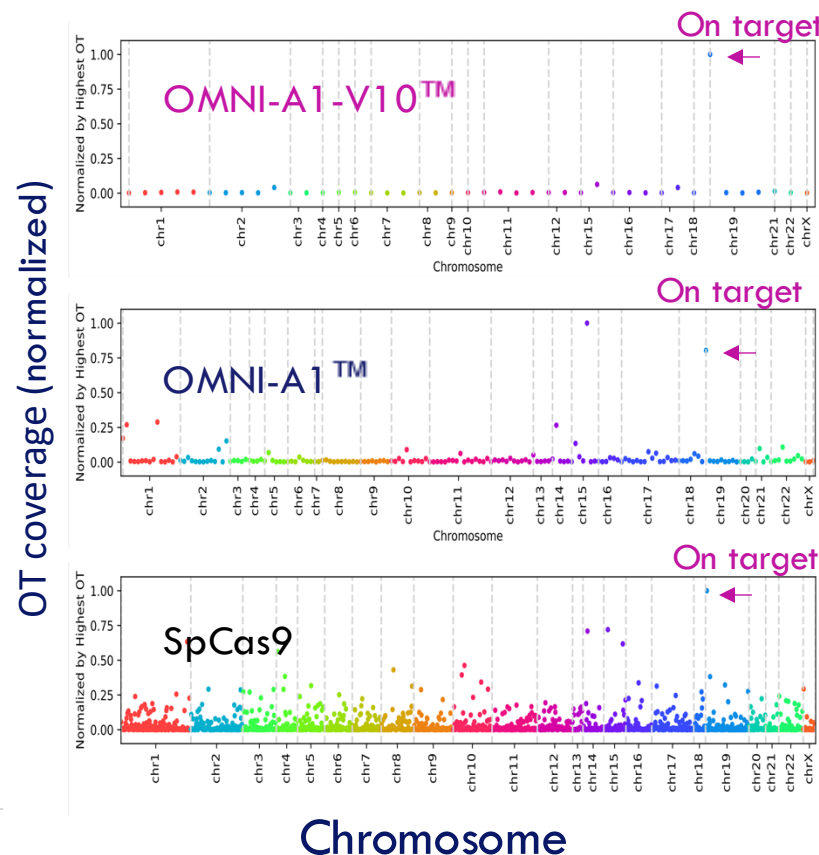
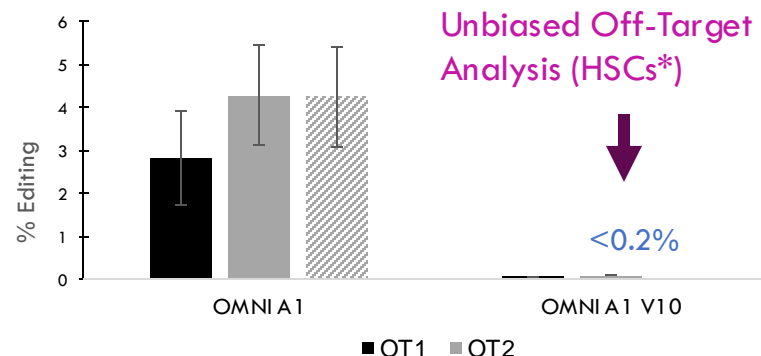
Engineering further eliminates off-targets

Limits potential for off-target mediated translocations (OMTs)

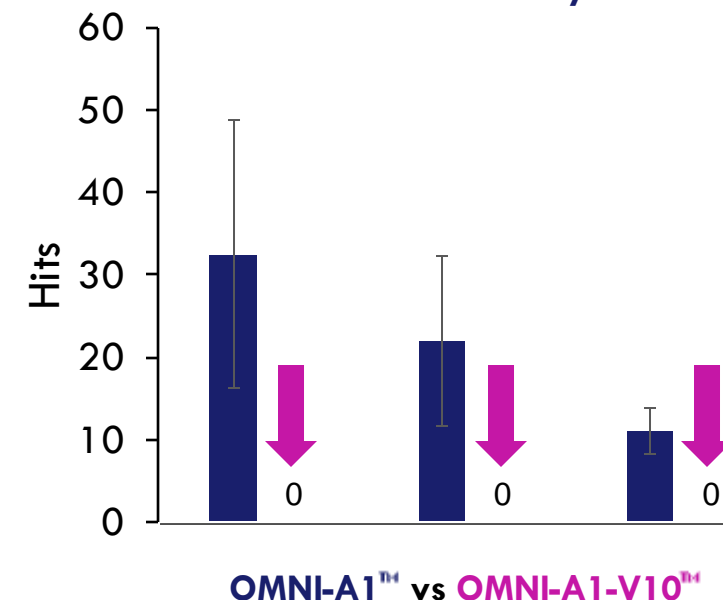
## OMNI-A1™ vs SpCas9



## OMNI-A1™ vs OMNI-A1-V10™



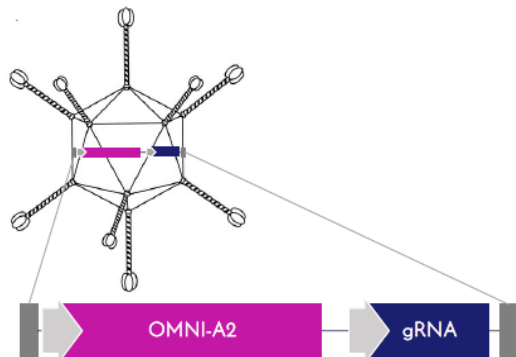
## Unbiased OMT analysis



# OMNI-A2™ (1,050aa): Short AAV-Deliverable Nuclease

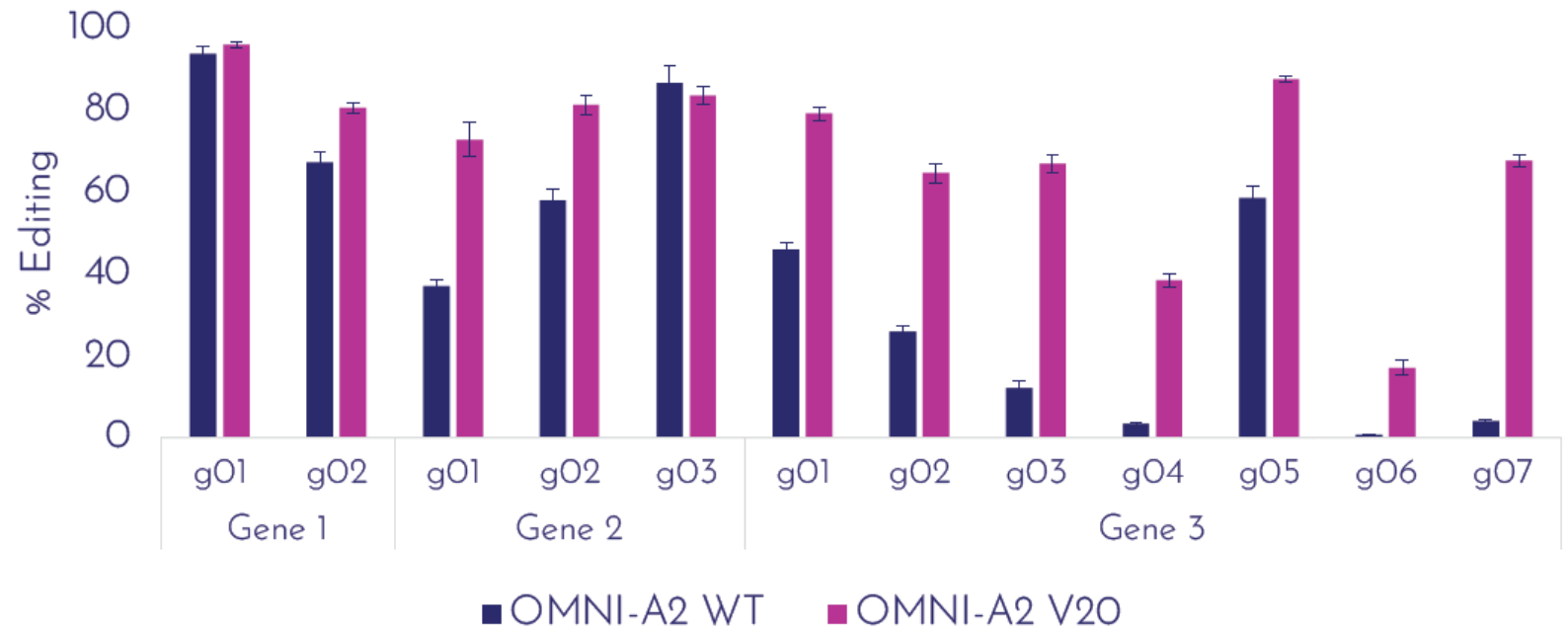
Short, highly active, AAV packaging compatible nucleases available

## AAV-based vectors



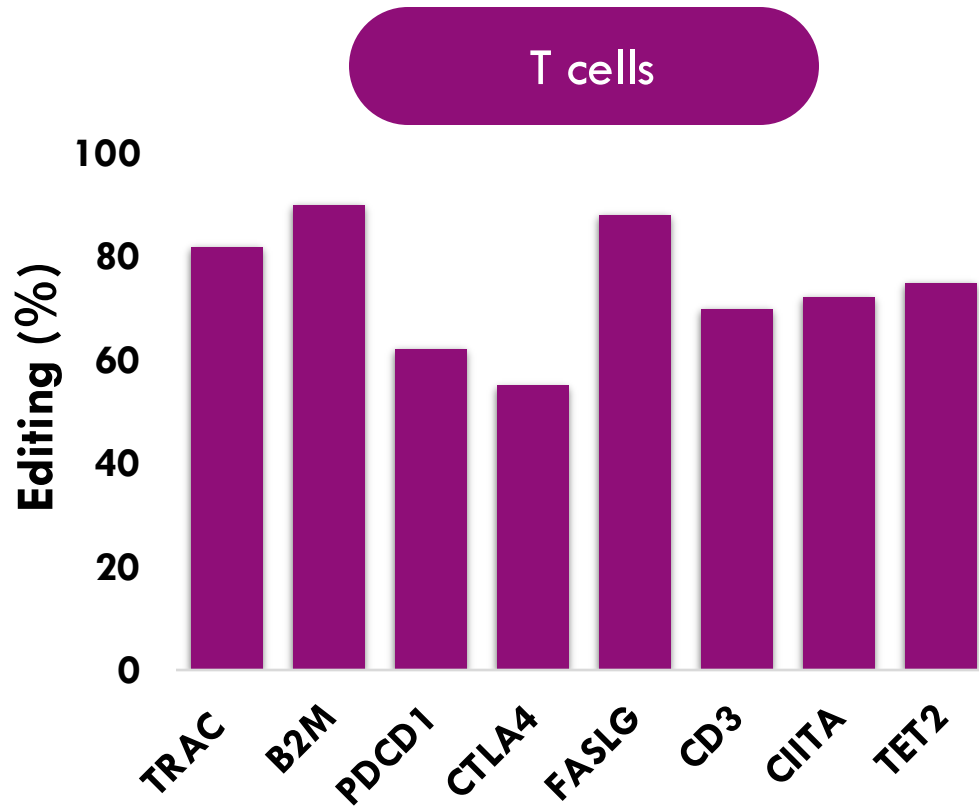
*Limited payload capacity*

## Editing by OMNI-A2-V20™

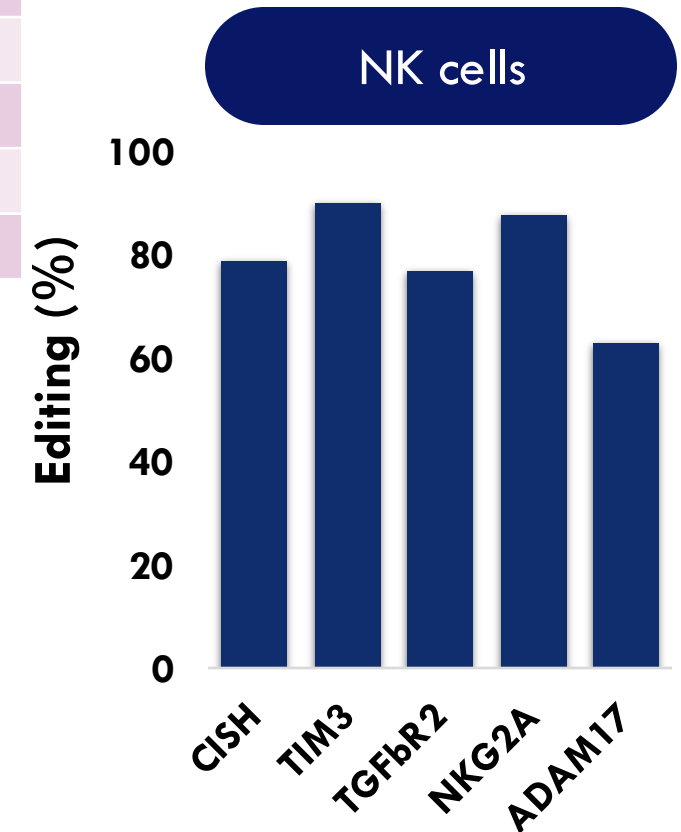


# OMNI-A4™ Presents High Activity and Specificity Profile

Non-NGG PAM nuclease compositions for major cell therapy and immuno-oncology targets



OMNI-A4™	
CRISPR type	II-A
Protein length	1,348 aa (161.9 Kda)
gRNA length	101 nt
PAM	NN <b>RACT</b>
hg38 coverage	0.77%



# A Portfolio of “Off-the-Shelf” Editing Solutions

## SAFE HARBOR

#	Target Gene	Computational	Cell Line	Target Cells
1	AAVS1	•	•	
2	ROSA26	•	•	
3	C3	•	•	
4	APLP2	•	•	•

## HEMATOPOETIC STEM CELLS

#	Target Gene	Disease	Computational	Cell Line	Target Cells
5	ELANE	Severe Congenital Neutropenia	•	•	•
6	SAMD9L	Myeloid malignancies	•	•	
7	GATA2	Myeloid malignancies	•	•	
8	SAMD9	Myeloid malignancies	•	•	
9	RPS19	Diamond Blackfan Anemia	•	•	

## IMMUNO-ONCOLOGY

#	Target Gene	Computational	Cell Line	Target Cells
10	PDCD1	•	•	•
11	TRAC	•	•	•
12	TRBC1	•	•	•
13	TRBC2	•	•	•
14	B2M	•	•	•
15	CTLA4	•	•	•
16	TET2	•	•	•
17	CD3E	•	•	•
18	LAG3	•	•	•
19	FAS	•	•	•
20	HAVCR2 (TIM3)	•	•	•
21	HLA-E	•	•	•
22	CIITA	•	•	•
23	FASLG	•	•	•
24	IL15	•	•	•
25	TIGIT	•	•	•
26	CISH	•	•	•

# A Portfolio of “Off-the-Shelf” Editing Solutions



## LIVER

#	Target Gene	Disease	Computational	Cell Line	Target Cells
27	SERPINA1	A1AD	•	•	•
28	ANGPTL3	Dyslipidemia including homozygous familial hypercholesterolemia	•	•	•
29	LDLR	Atherosclerotic cardiovascular disease	•	•	•
30	HBV	Hepatitis	•	•	



## CNS

#	Target Gene	Disease	Computational	Cell Line	Target Cells
31	LRRK2	Parkinson's disease	•	•	



## OPHTHALMOLOGY

#	Target Gene	Disease	Computational	Cell Line	Target Cells
32	TCF4	Fuchs Endothelial Corneal Dystrophy	•	•	
33	TGFBi	Corneal Dystrophies	•	•	
34	SARM1	Neuronal and macular degeneration	•	•	
35	RPE65	Retinitis Pigmentosa	•	•	
36	RHO	Retinitis Pigmentosa	•	•	
37	FLG	Ichthyosis vulgaris	•	•	
38	BEST1	Autosomal dominant vitreoretinopathopathy	•	•	
39	PRPH2	Retinitis Pigmentosa	•	•	



# OMNI™ Panel Genome Accessibility

## Nuclease Portfolio

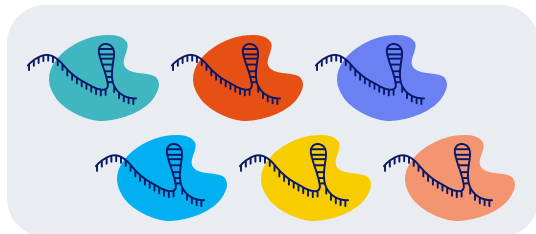
10,000 discovered nucleases

300 validated in vitro

80 shown active in cells

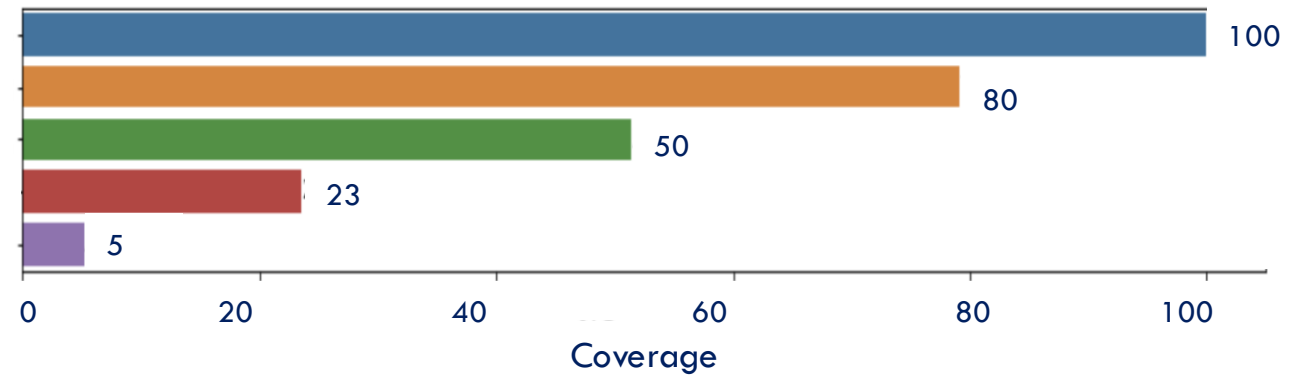
12 characterized

2 engineered



## OMNI™ Genomic PAM Coverage

Whole Genome  
Validated OMNIs  
Active OMNIs (cell)  
Characterized OMNIs  
NGG



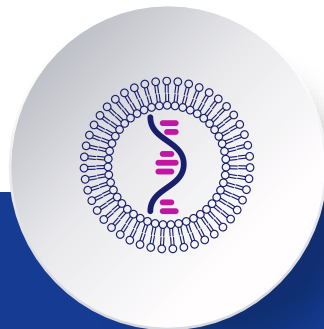
The diversity of PAM sites of the OMNI™ nucleases overcomes PAM constraints and significantly widens genome accessibility, making **any gene targetable**

# OMNI™-Generated Nucleases

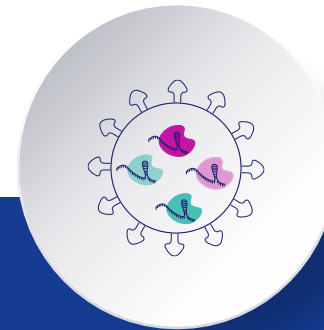
Compatible with all commonly used delivery platforms



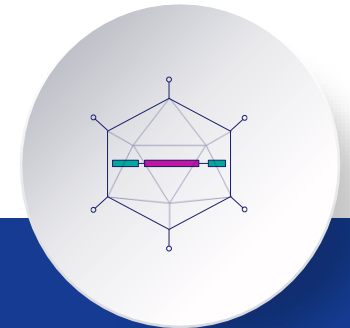
Ribonucleoprotein  
(RNPs)



Lipid nano particles  
(LNPs)



Lenti Virus Like Particles  
(LVLPs)



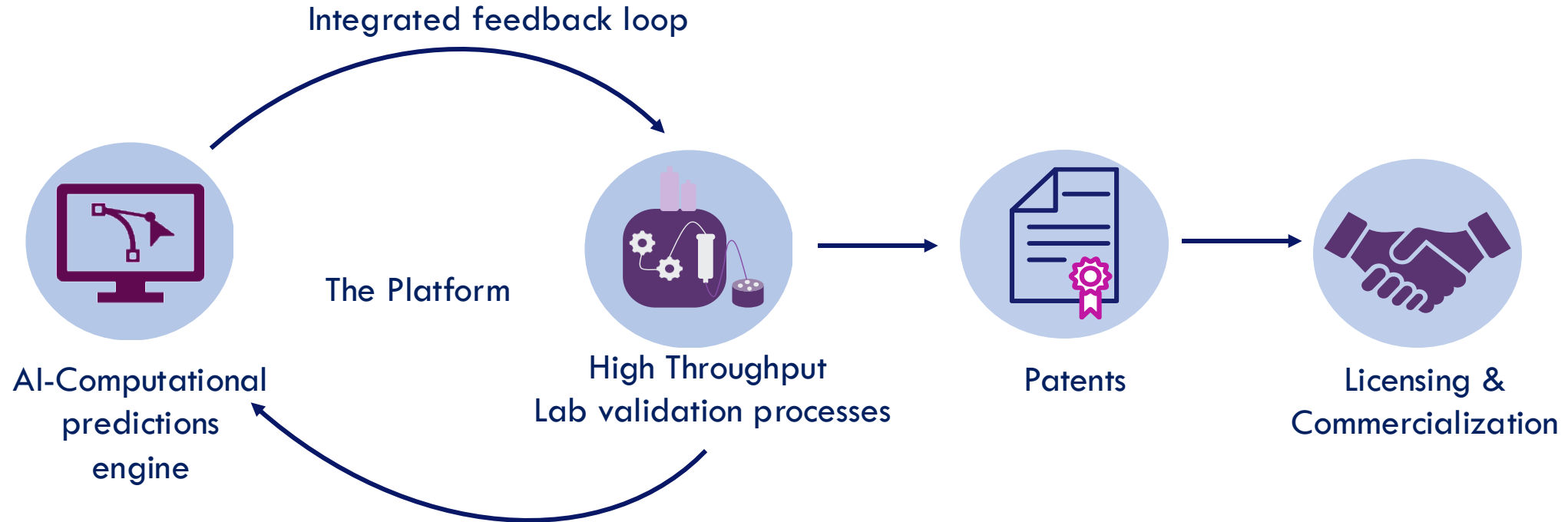
Adeno Associated Virus  
(AAV)

# Extensive Intellectual Property Portfolio

- Strong IP position – 191 patents/applications worldwide
- Coverage extending to 2041
- Gene Editing Techniques
- Compositions for gene editing
  - Knock-out and knock-in compositions
  - Allele-specific compositions
  - Numerous target genes & indications
- Novel CRISPR nucleases
  - OMNI™ Panel Nucleases
  - High-fidelity variants
  - Variants with increased activity, specificity



# EmendoBio's Business Model



## Collaboration Work Plan

Upon transfer of gene sequence:

- EmendoBio assesses licensee needs and optimizes OMNI™ nuclease
- EmendoBio provides nuclease and recommended guide RNA sequence

Time

2-4 weeks

6-8 weeks

# Summary

## EmendoBio's platform



AI-based nuclease discovery and engineering platform



Precision, diversity, efficiency and safety superior to conventional CRISPR



Compatible with all commonly used delivery platforms

## Strong IP position



Patent families covering all aspects of gene editing

## Custom-designed and off-the-shelf nucleases



Available for exclusive or nonexclusive licensing