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# Emendo Biotherapeutics

October 2022

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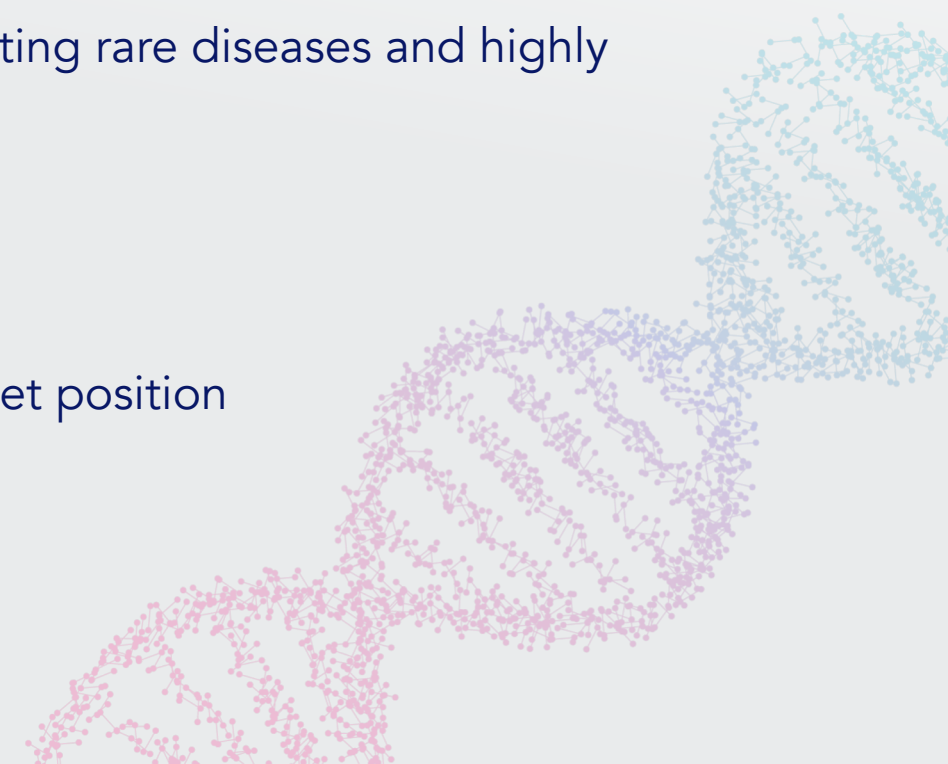
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# Value Proposition

- Most advanced privately held CRISPR gene-editing company
- 100 employees with 80 scientists and product developers
- Strong IP position – 70 patent families covering all aspects of CRISPR Gene Editing
- Forefront of AI based Nuclease discovery and optimization
- Strong pipeline covering ExVivo and InVivo indications, targeting rare diseases and highly prevalent ones
- Significant Licensing-out deals under negotiations
- Significant value inflection points along the coming year
- Equivalent companies with high valuations and a strong market position
- Clear exit strategy



# Comparable Companies and Deals





## Pre-clinical stage editing companies

Beam Therapeutics (BEAM)	\$5.6B
Generation Bio (GBIO)	\$322m
Omega Therapeutics (OMGA).	\$267m

## Clinical stage editing companies

Verve Therapeutics (VERV)	\$2.1B
Intellia Therapeutics Inc (NTLA)	\$4.5B
CRISPR Therapeutics Ltd (CRSP)	\$5.2B
Editas Medicine (EDIT)	\$921M
Caribou Biosciences (CRBU)	\$669M

## Licensing deals - benchmarks

Date	Comapnies	Agreement details	# of inidcations	Type	Upfront	Milestones
2022	Novartis - Precision 	In-vivo gene editing of HSCs including hemoglobinopathies, insertion into safe harbor site	ND	HSC	\$75M	\$1.4B
2021	Biogen-Scribe 	Discover and develop CRISPR-based genetic medicines for neurological diseases	2	ALS, Neuro	\$15M	\$400M
2022	Pfizer - Beam 	Discover and develop in vivo base-editing therapies	3	Liver, Muscle, CNS	\$300M	\$1.05B
2022	Bayer - Mammoth 	Discover and develop in vivo CRISPR-based gene editing therapies	5	Liver	\$40M	\$1B

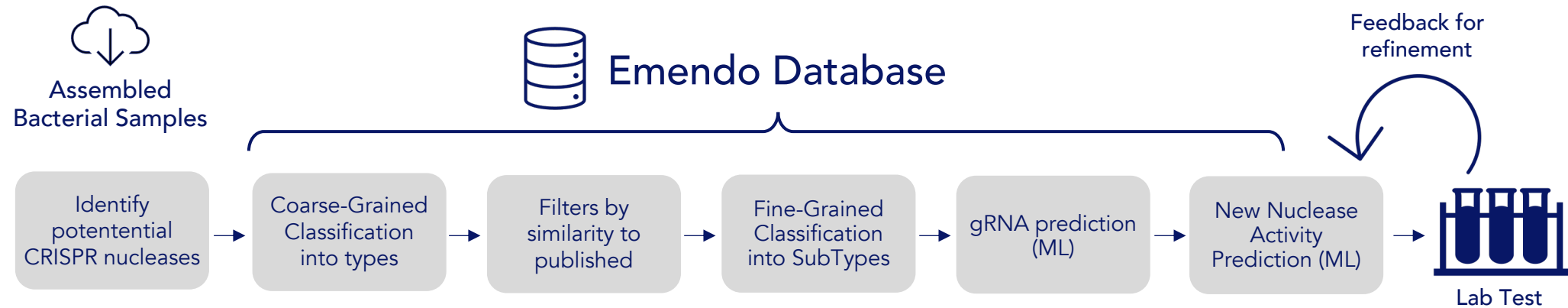
Valuations are of Sep. 30th 2022

Source: Licensing deals data is taken from the IR section of the respective companies

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# The OMNI Platform

## Data science, Machine Learning (ML) and Engineering



### Machine Learning Tools

#### gRNA Prioritization

Predict the best composition

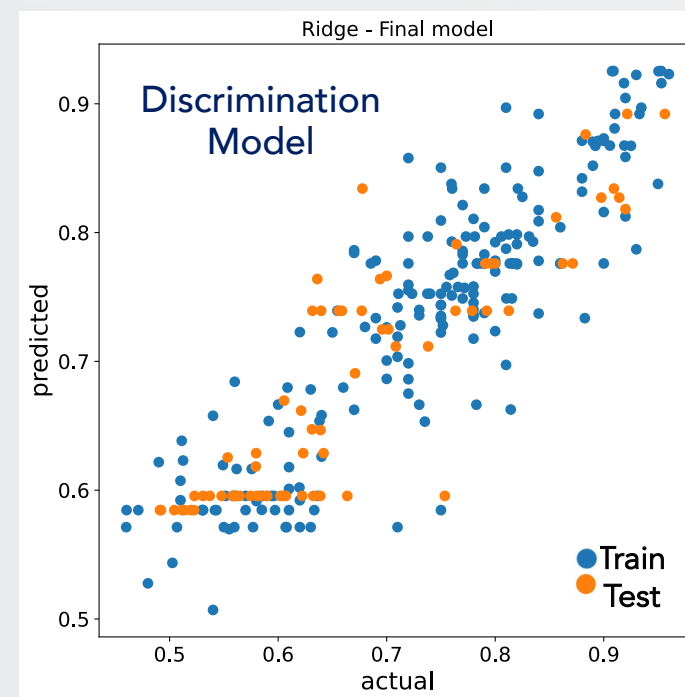
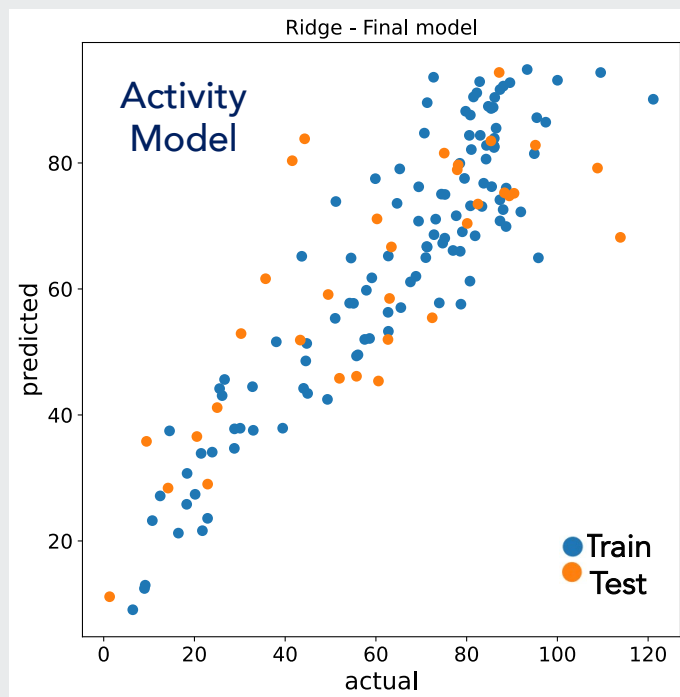
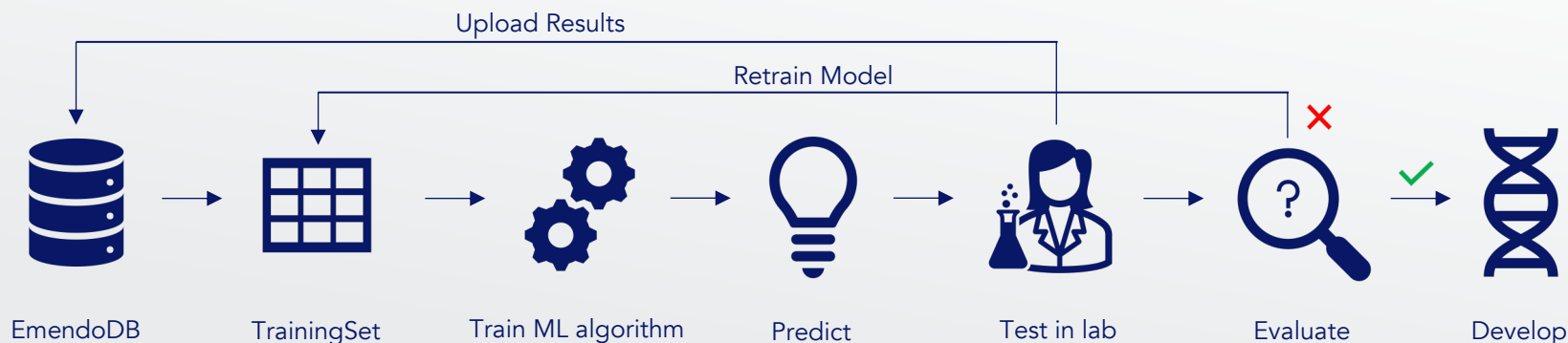
#### Nuclease Eng.

Predict the optimal nuclease variant

Optimization per indication

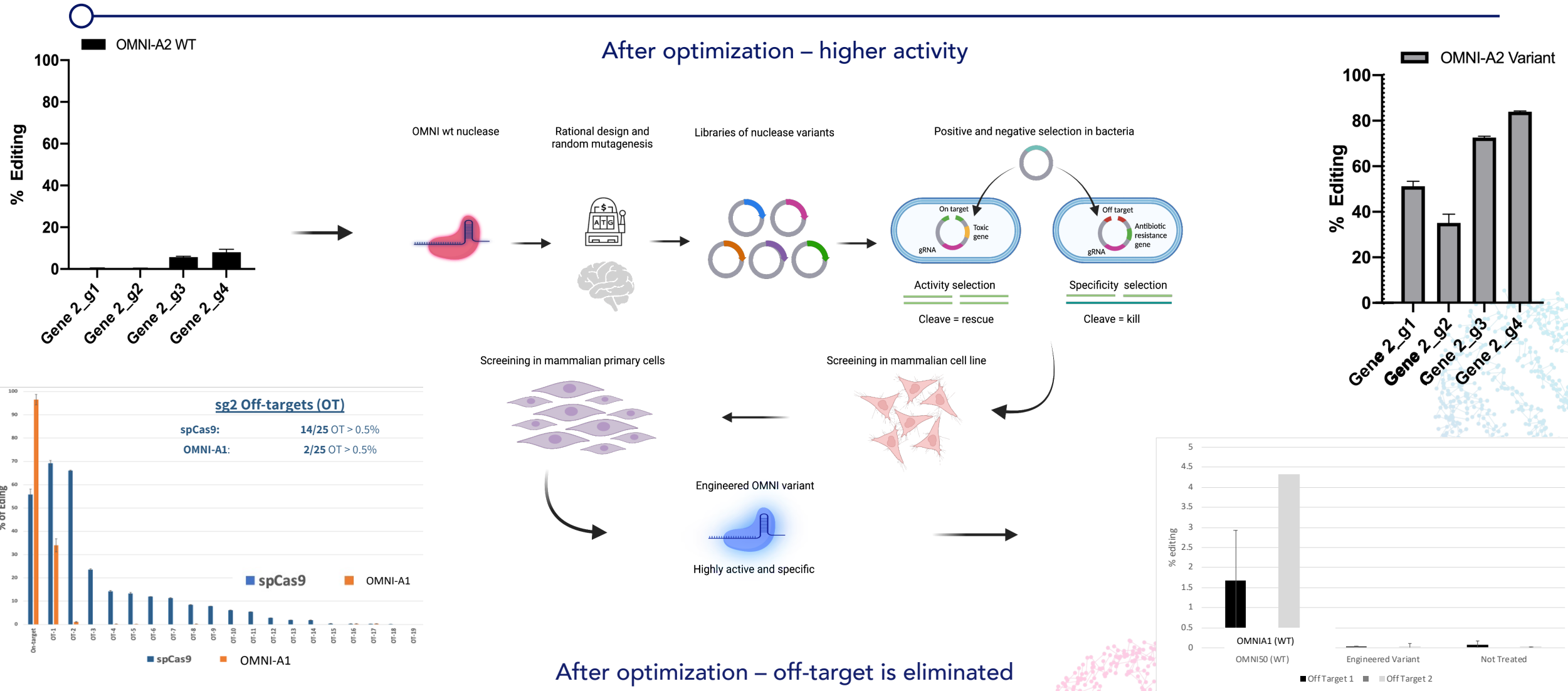
# Platform- Computational

## We use ML to predict better CRISPR drug products





# Platform in Action: Engineering Increasing Nuclease Activity and Specificity



# The OMNI Platform Allows to Target more Diseases in a Safer Way



- SNP based Allele specific editing - ELANE
- Upregulating genes – LDLR
- Covering 86% of genomic sites
- Allowing FTO around guides –Immuno-oncology
- Eliminating off-targets

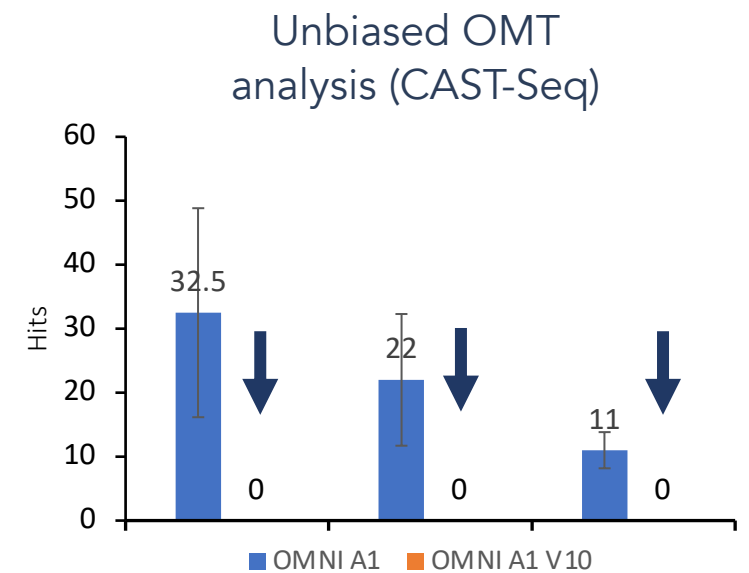
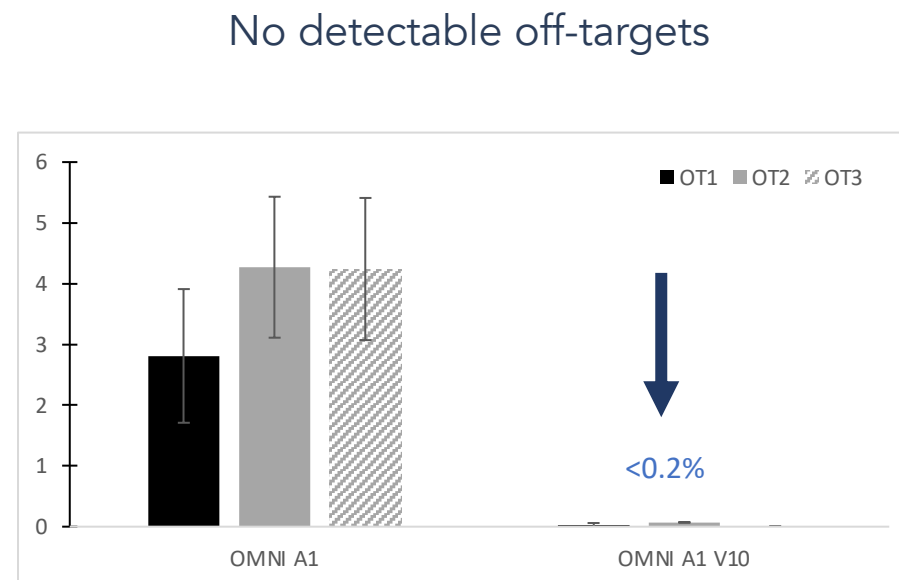
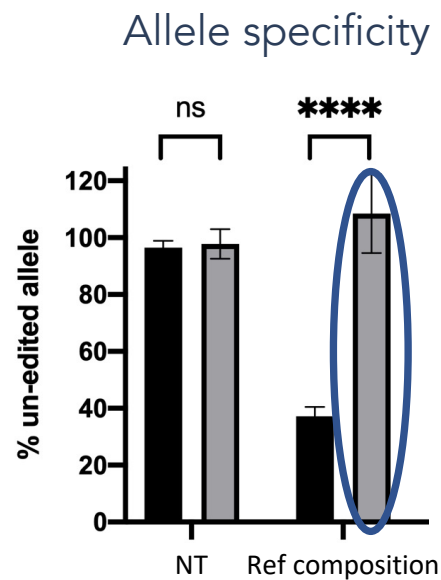




# ELANE Severe Congenital Neutropenia

- Neutrophil maturation disorder resulting in severe and recurrent infections
- Over 200 *ELANE* heterozygous dominant mutations
- High Unmet Need
  - Lifelong daily injection of G-CSF: Severe side effects, increased risk for AML/MDS, not curative
  - Allo-transplants: Graft failure and acute GvHD

## OMNI A1 V10: A Novel, optimized nuclease is fully discriminatory and target specific

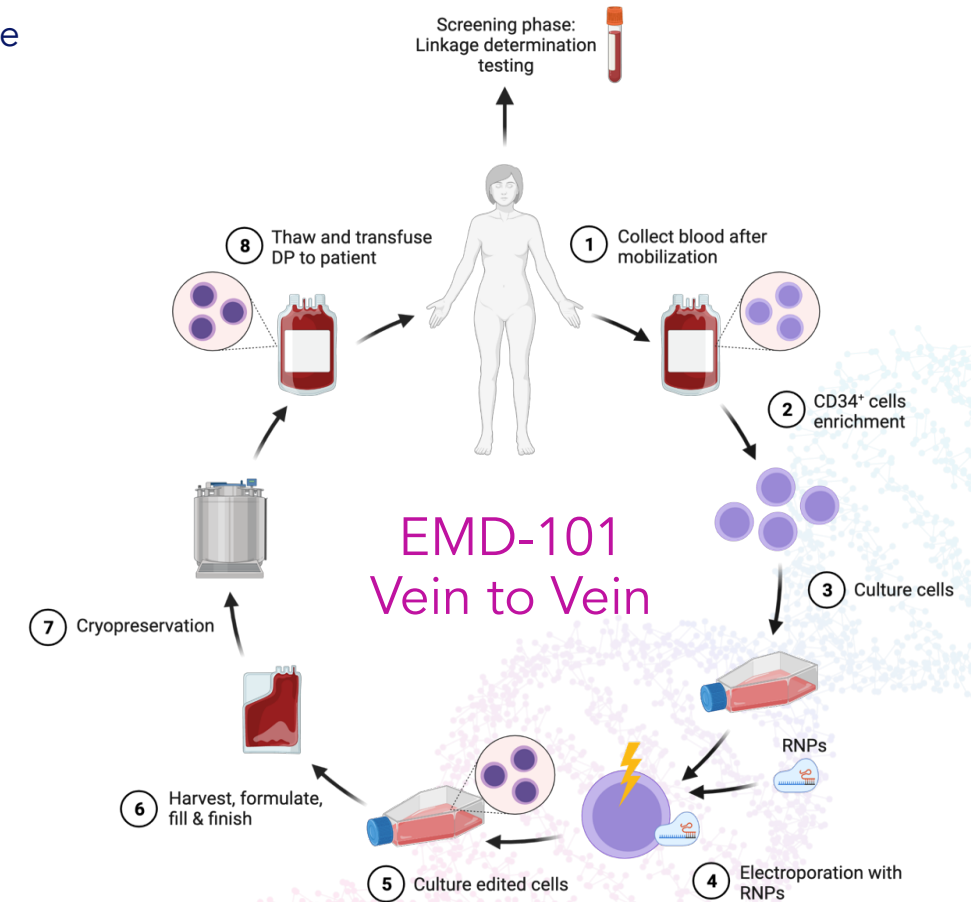


Source: "Mutant Allele Knock-out with Novel CRISPR Nuclease Promotes Myelopoiesis in ELANE Neutropenia." *Molecular Therapy-Methods & Clinical Development* (2022).

# The Road to the Clinic- ELANE Dependent Severe Congenital Neutropenia (SCN)

- POC on stem cells (HSPC) from patients' bone marrow show significant increase in neutrophils maturation
- A scaled-up process for drug-product manufacturing was developed.
- Trusted CMOs were contracted for the manufacturing of raw materials (sgRNA and OMNI)
- Patients' bone-marrow mobilization study is underway with Seattle Children's Hospital (SCRI)
- Adaptive Clinical study (FIH + Pivotal) is planned at SCRI

Milestone	Timeline
Pre- IND Meeting	Nov 2022
Safety and CMC studies	Q4 2022- Q2 2023
IND	Q3 2023



# Familial Hypercholesterolemia (LDLR): Therapeutic Strategy Overview

- HeFH: Affects 1:220
- >90% individuals remain undiagnosed

## FDA approved lipid-lowering drug classes

Mipomersen: Antisense oligonucleotide to ApoB

Lomitapide: Microsomal triglyceride transfer protein

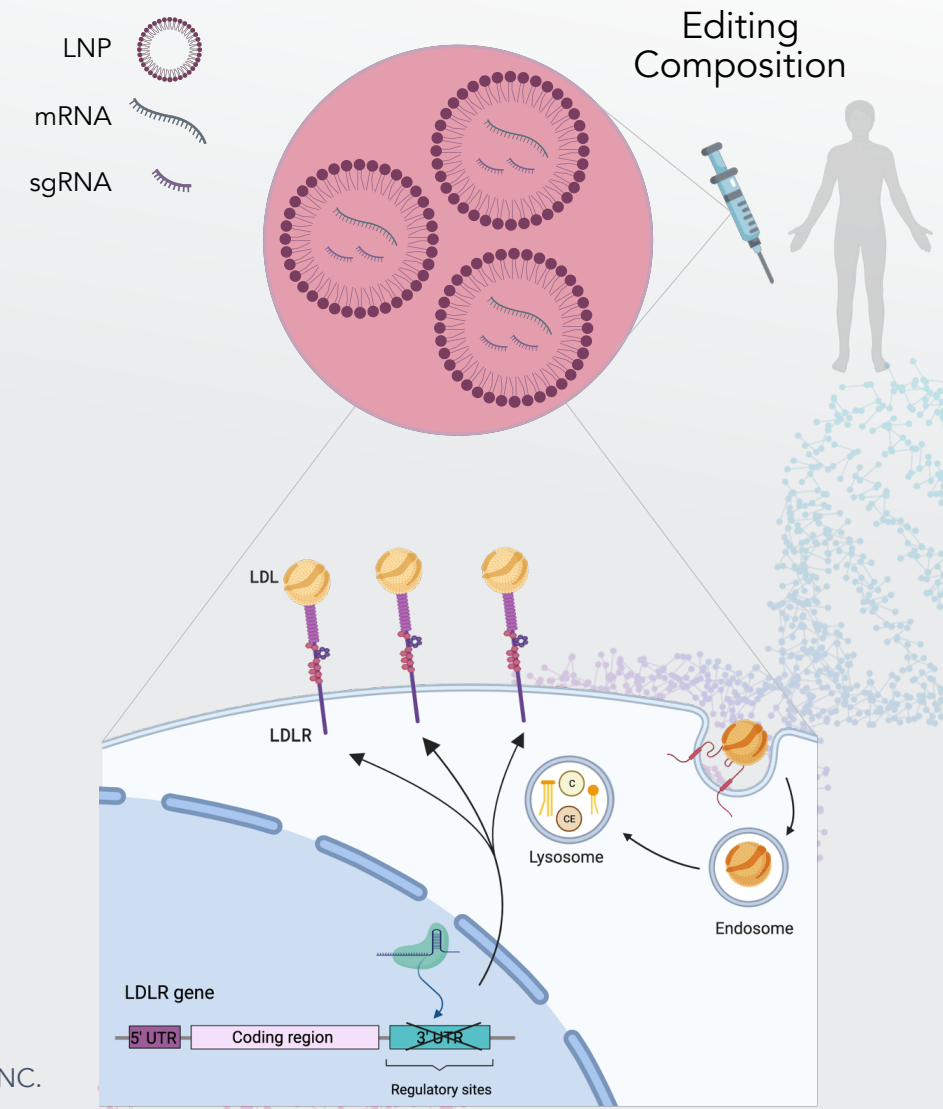
Alirocumab, Evolocumab: Monoclonal antibodies to PCSK9

Novartis Leqvio® (inclisiran): first-in-class siRNA

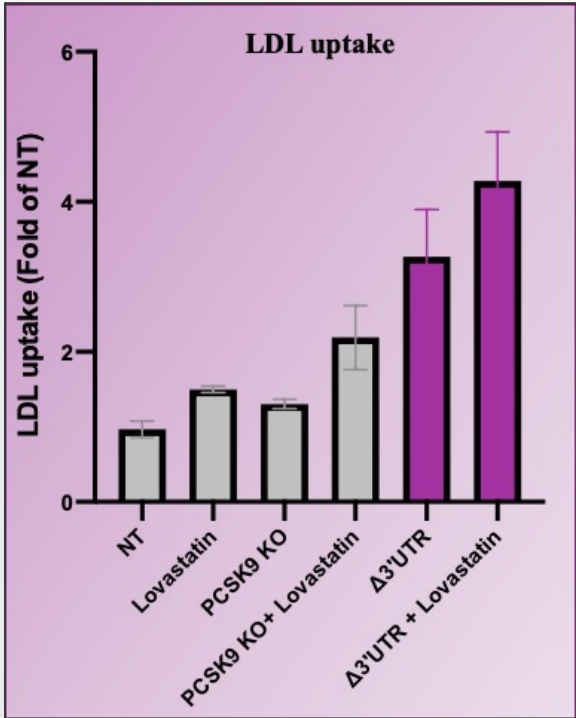
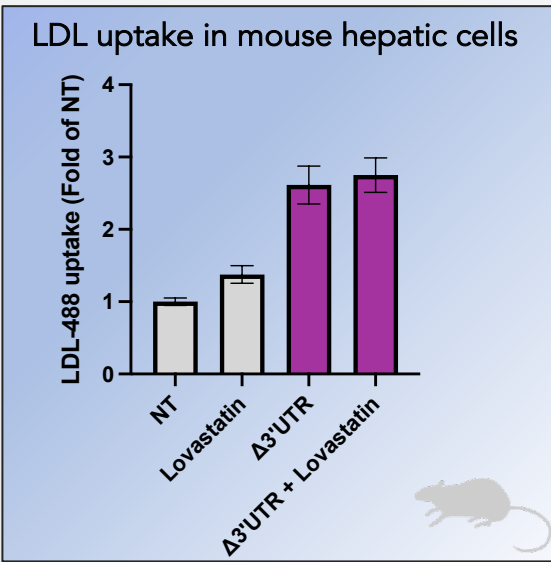
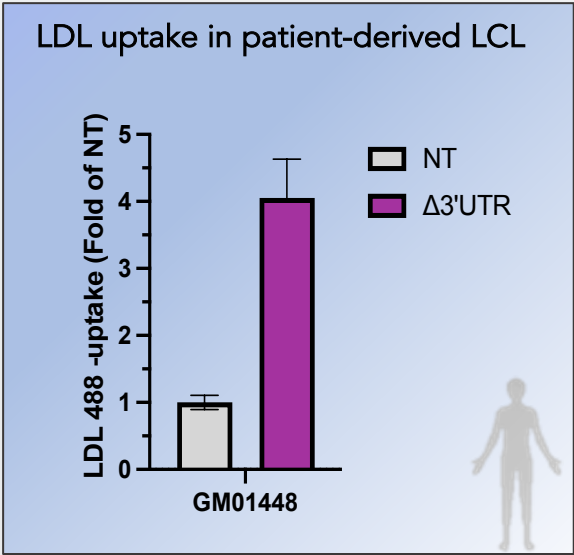
## Market revenue of anti-PCSK9:

Repatha (Evolocumab): \$654M in the 6 months from Jan 2022 to June 2022

Praluent (Alirocumab): \$220.3M in the 6 months from Jan 2022 to June 2022



# Emendo's Novel Gene Editing Solution: Upregulate surface LDLR expression using our OMNI-A2 nuclease



LDL uptake by 3'UTR excised HepG2 cells, PCSK9 KO cells and non-treated cells with and without Statins (Flow cytometry)

3'UTR excision of *LDLR* is superior to PCSK9 depletion and Statins treatment

Milestone	Date
POC editing Composition	Q4 -2022
In-Vivo efficacy POC AAV delivery	Q4 - 2022
In-Vivo efficacy POC LNP delivery	Q2 - 2023
Start DP GMP production	Q3 - 2023
Pre IND	Q3 - 2024

# SARM1: Targeting not Only Genetic Disease

- A unique and generalized gene editing approach, to address axonal degeneration in multiple diseases, at the source
- Although taking a gene editing approach, it is not addressing monogenic genetic diseases, but rather address large indications, regardless of specific gene or mutation

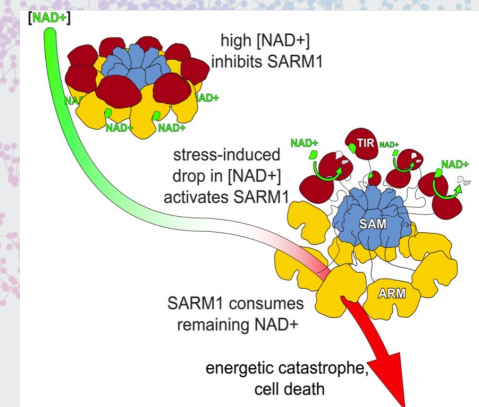
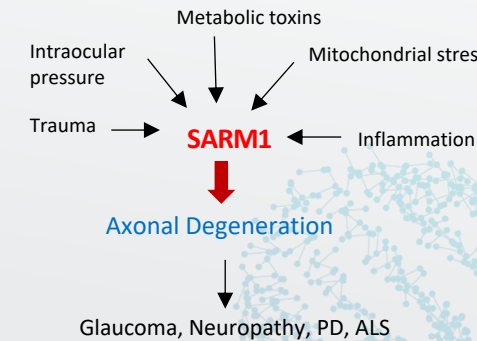
## SARM1 inhibitors – a promising new class of therapeutics

a promising new class of potential therapeutics called SARM1 inhibitors that target axonal degeneration as a treatment in:

- The CNS - Multiple Sclerosis; Amyotrophic Lateral Sclerosis; Parkinson's Disease, Ischemic and other injuries
- Neuro-ophthalmology - Glaucoma; Leber's Hereditary and sporadic Optic Neuropathy; Optic Neuritis
- Peripheral Nervous System - Charcot-Marie-Tooth Disease; Chemotherapy-induced Peripheral Neuropathy; Diabetic Neuropathy;

Eli Lilly bought DiSARM for \$135M upfront and \$1.225 billion in milestones, on October 2020.



Milestone	Date
In-Vivo POC	FEB - 2023
INTERACT	APR - 2023
Pre-IND	Q1 2024





# Wide Variety of Clinical Applications



## Ex-Vivo

PROGRAM	COLLABORATOR	LEAD OPTIMIZATION	PRE-CLINICAL	IND-ENABLING	PHASE 1-3
<div> HEMATOLOGY</div>					
EMD-101 Severe Congenital Neutropenia	University of Washington	<div><div></div></div>			
<div> IMMUNO-ONCOLOGY</div>					
CAR-T Cells	Confidential	<div><div></div></div>			
NK Cells	Proprietary	<div><div></div></div>			

## In-Vivo

PROGRAM	COLLABORATOR	LEAD OPTIMIZATION	PRE-CLINICAL	IND-ENABLING	PHASE 1-3
<div> LIVER</div>					
EMD-301 Familial Hypercholesterolemia	Proprietary	<div><div></div></div>			
EMD- 302 Inborn Errors of Metabolism	Proprietary	<div><div></div></div>			
<div> OPHTHALMOLOGY</div>					
EMD-201 Retinitis Pigmentosa	Columbia University	<div><div></div></div>			
EMD-202 Cone-Rod Dystrophy	Proprietary	<div><div></div></div>			
EMD-203 Macular Dystrophy	Proprietary	<div><div></div></div>			





## Summary

- ❖ Emendo is the most advanced privately held CRISPR GE company
- ❖ Emendo presents a hidden source of significant value for AnGes that is not yet appreciated by the markets
- ❖ Significant value inflection points to unfold over the coming 18 months
- ❖ Due to more newsflow and outreach efforts we anticipate more public attention
- ❖ Clear exit strategies IPO/M&A



Thank You!

