Emendo Biotherapeutics

January 2023

Value Proposition

- Most advanced privately held CRISPR gene-editing company
- 120 employees with over 100 scientists and product developers
- Strong IP position – 56 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)	$2.90B
Generation Bio (GBIO)	$301m
Omega Therapeutics (OMGA).	$296m

Clinical stage editing companies

Verve Therapeutics (VERV)	$1.25B
Intellia Therapeutics Inc (NTLA)	$3.25B
CRISPR Therapeutics Ltd (CRSP)	$3.52B
Editas Medicine (EDIT)	$635m
Caribou Biosciences (CRBU)	$434m

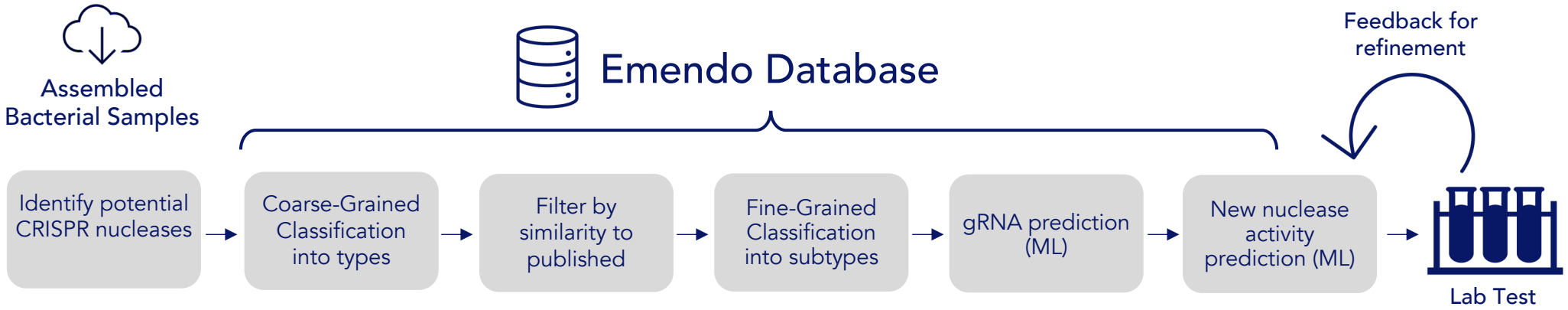
Licensing deals - benchmarks

Date	Companies	Agreement details	# of indications	Type	Upfront	Milestones
Nov 2022	Ionis - Metagenomi	Develop genetic targets and therapies via a collaboration of the technologies	4-8	Liver	$80M	ND
June 2022	Novartis - Precision	In-vivo gene editing of HSCs, insertion into safe harbor site	ND	HSC	$75M	$1.4B
Jan 2022	Pfizer - Beam	Discover and develop in vivo base-editing therapies	3	Liver, muscle, CNS	$300M	$1.05B
Jan 2022	Bayer - Mammoth	Discover and develop in vivo CRISPR- based gene editing therapies	5	Liver	$40M	$1B

Valuations are of Jan. 5th 2023

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

The OMNI Platform
Data Science, Machine Learning (ML) and Engineering



Machine Learning Tools

gRNA Prioritization

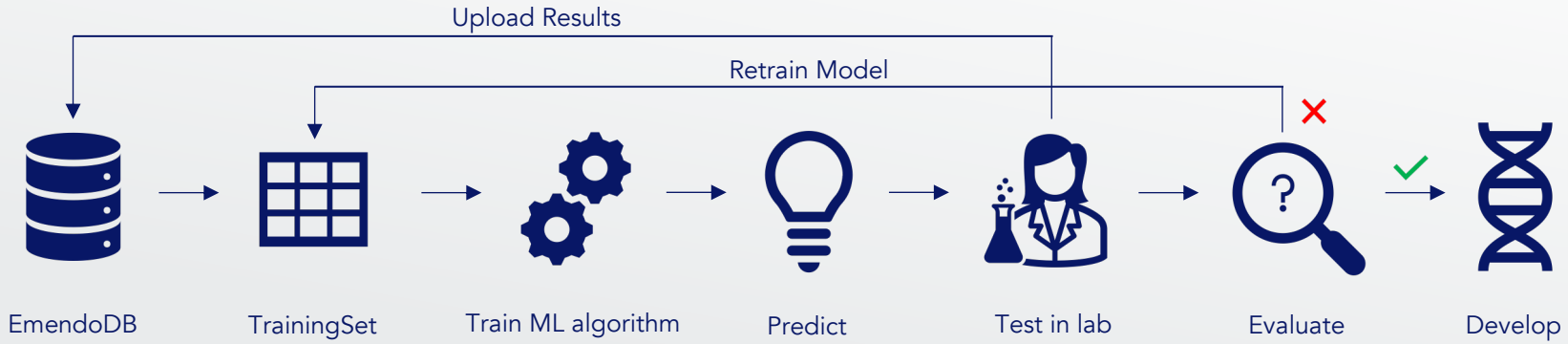
Predict the best composition

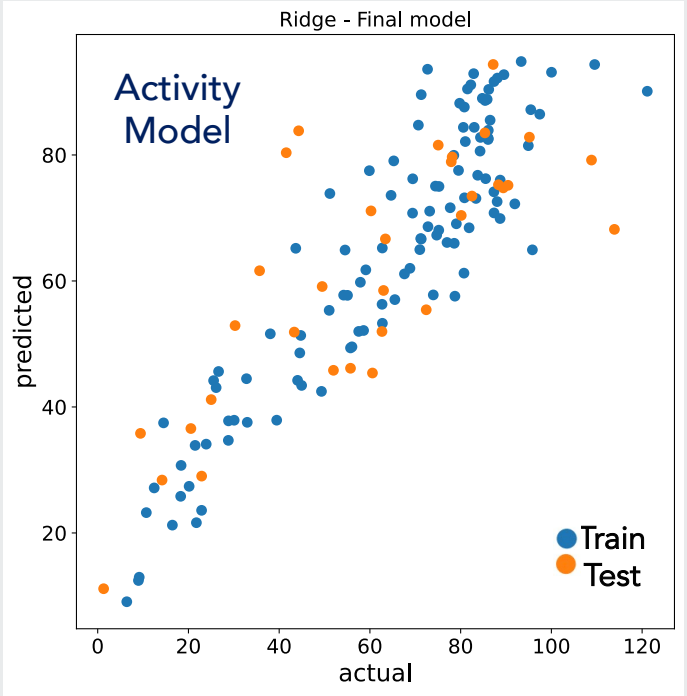
Nuclease Eng.

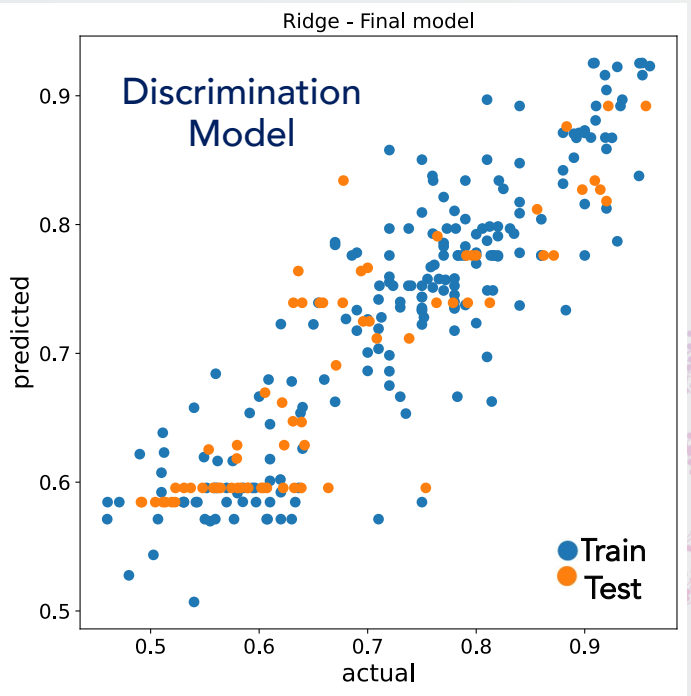
Predict the optimal nuclease variant

Optimization per indication

Platform- Computational
We use ML to predict better CRISPR drug products

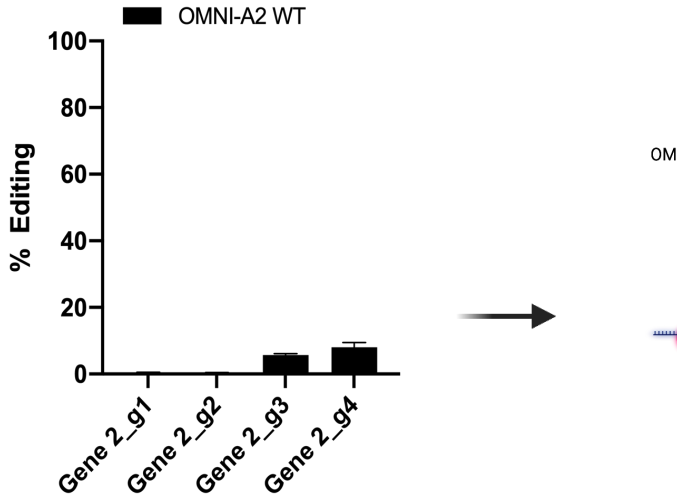




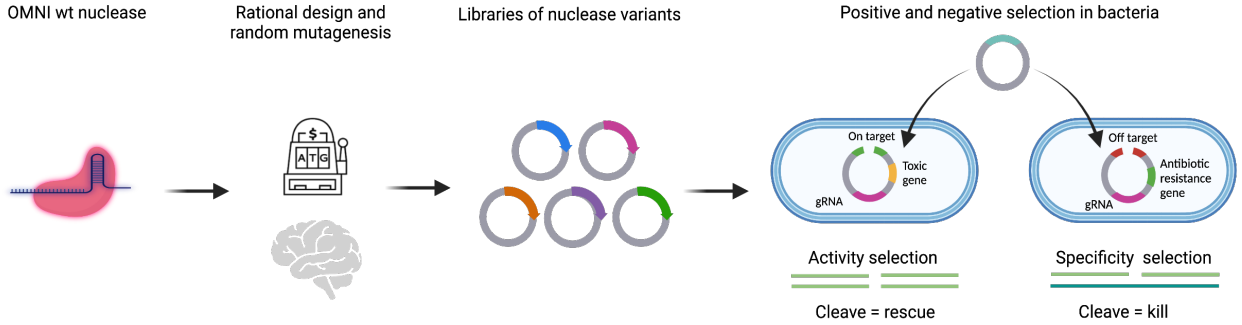


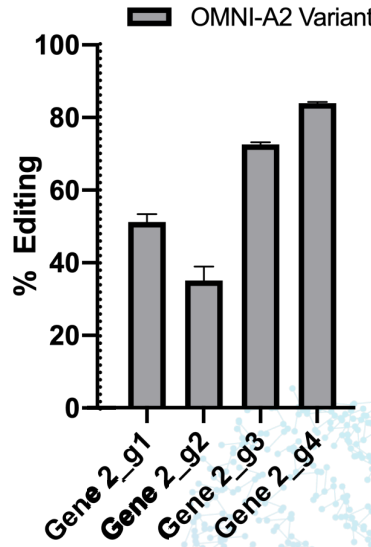
Source: Visualization of data collected in-house

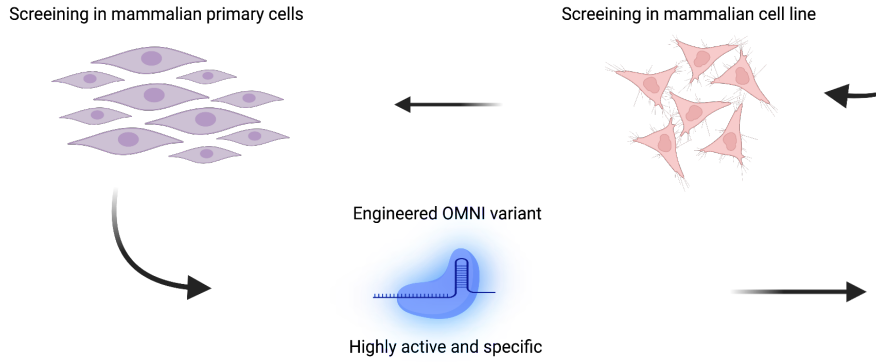
Platform in Action: Engineering
Increasing Nuclease Activity and Specificity

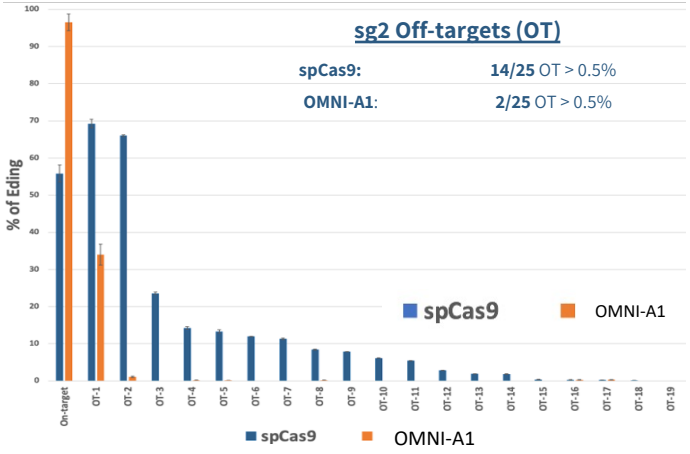


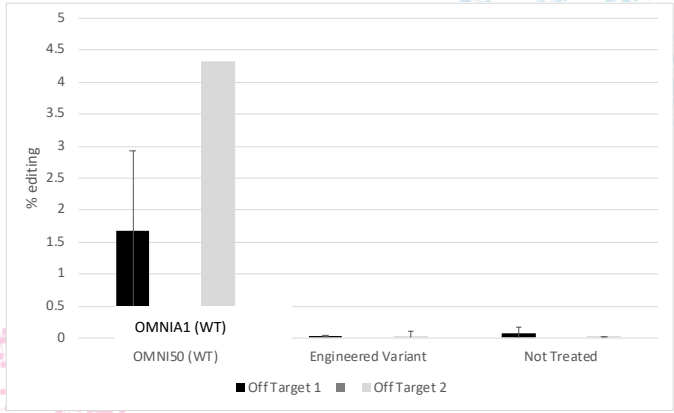
After optimization – higher activity











After optimization – off-target is eliminated

Source: Visualization of data collected in-house

The OMNI Platform Enables Targeting of more Diseases in a Safer Way

Panel of Nucleases + Optimization = More Available Gene Targets + High Activity + Extreme Specificity

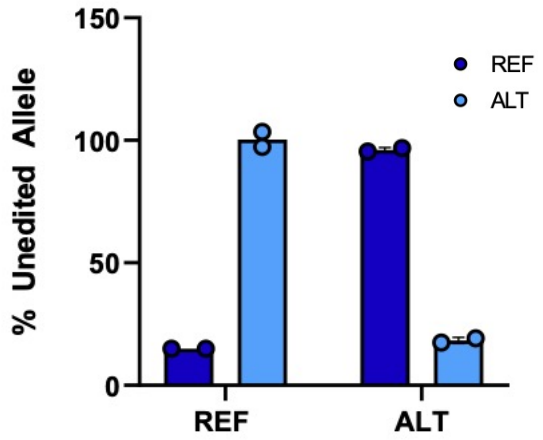
- SNP based allele specific editing - ELANE
- Upregulating genes – LDLR
- Covering 86% of genomic sites
- Allowing FTO around guides –Immuno-oncology
- Eliminating off-targets

ELANE Severe Congenital Neutropenia

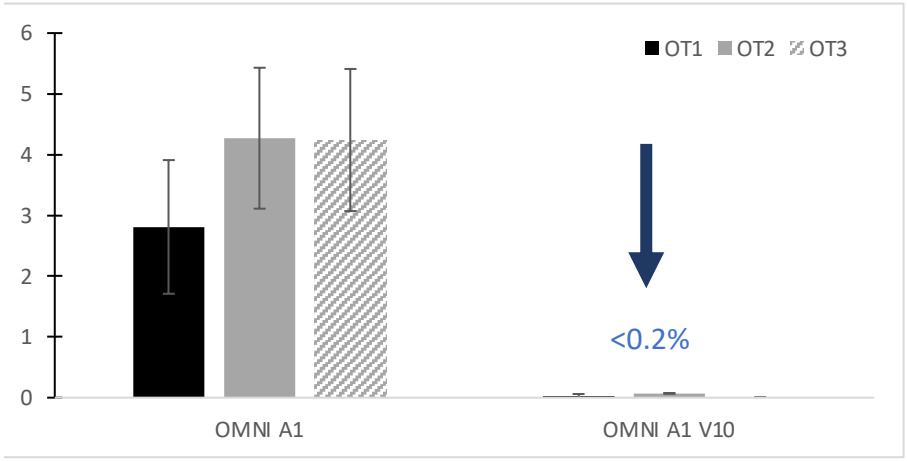
- Neutrophil maturation disorder resulting in severe and recurrent infections
- Over 200 *ELANE* heterozygous dominant mutations
- High Unmet Need
  - Lifelong daily injection of G-CSF: severe side effects, increased risk for AML/MDS, not curative
  - Allo-transplants: graft failure and acute GvhD

OMNI A1 V10: a novel, optimized nuclease is fully discriminatory and target specific

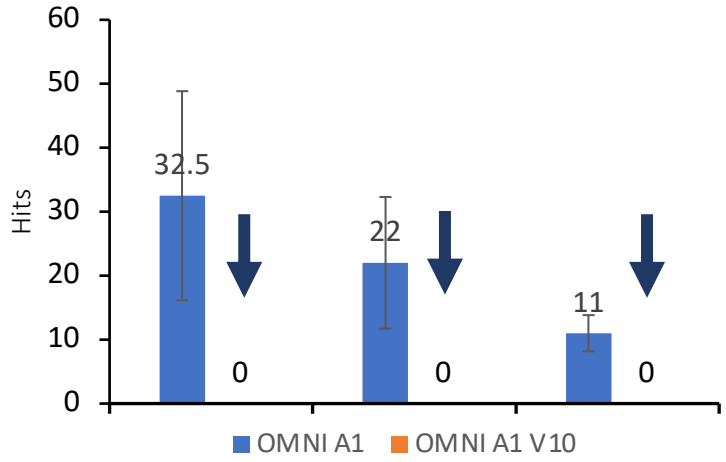
Allele specificity



No detectable off-targets



Unbiased OMT analysis (CAST-Seq)

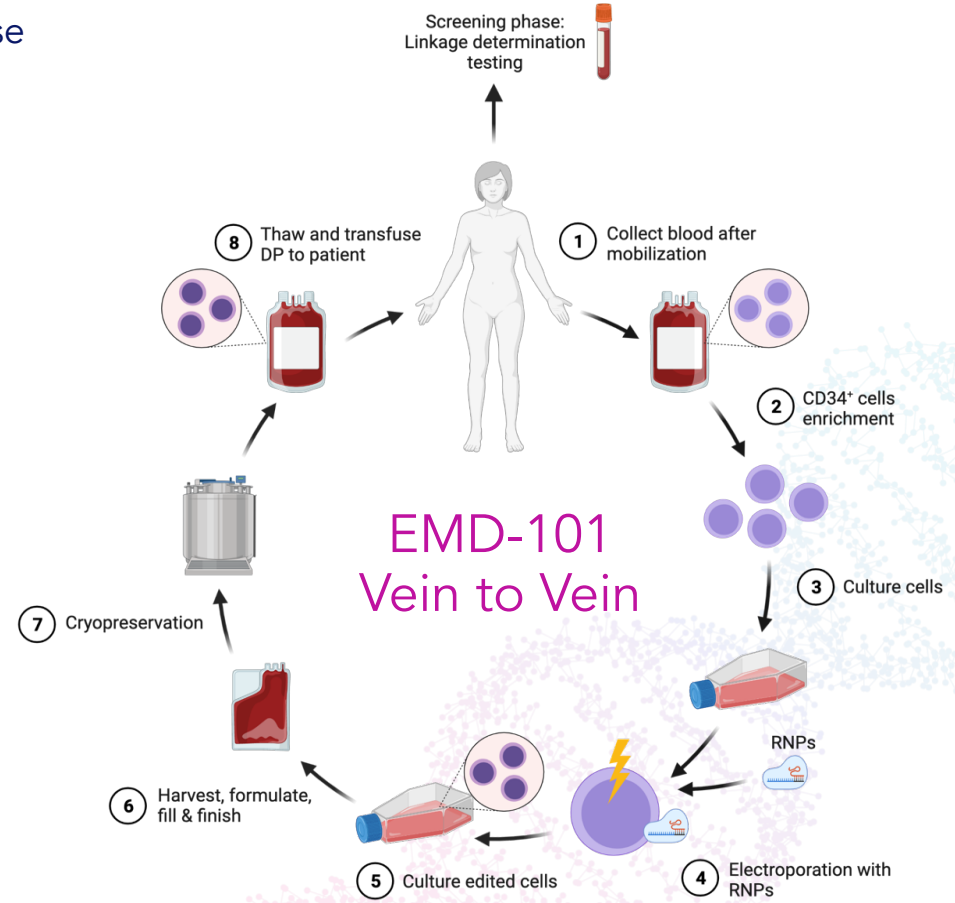


Source: "Mutant Allele Knock-out with Novel CRISPR Nuclease Promotes Myelopoiesis in ELANE Neutropenia." *Molecular Therapy-Methods & Clinical Development* (2022).

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

- POC on stem cells (HSPC) from patients' bone marrow shows significant increase in neutrophil 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 Research Institute (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

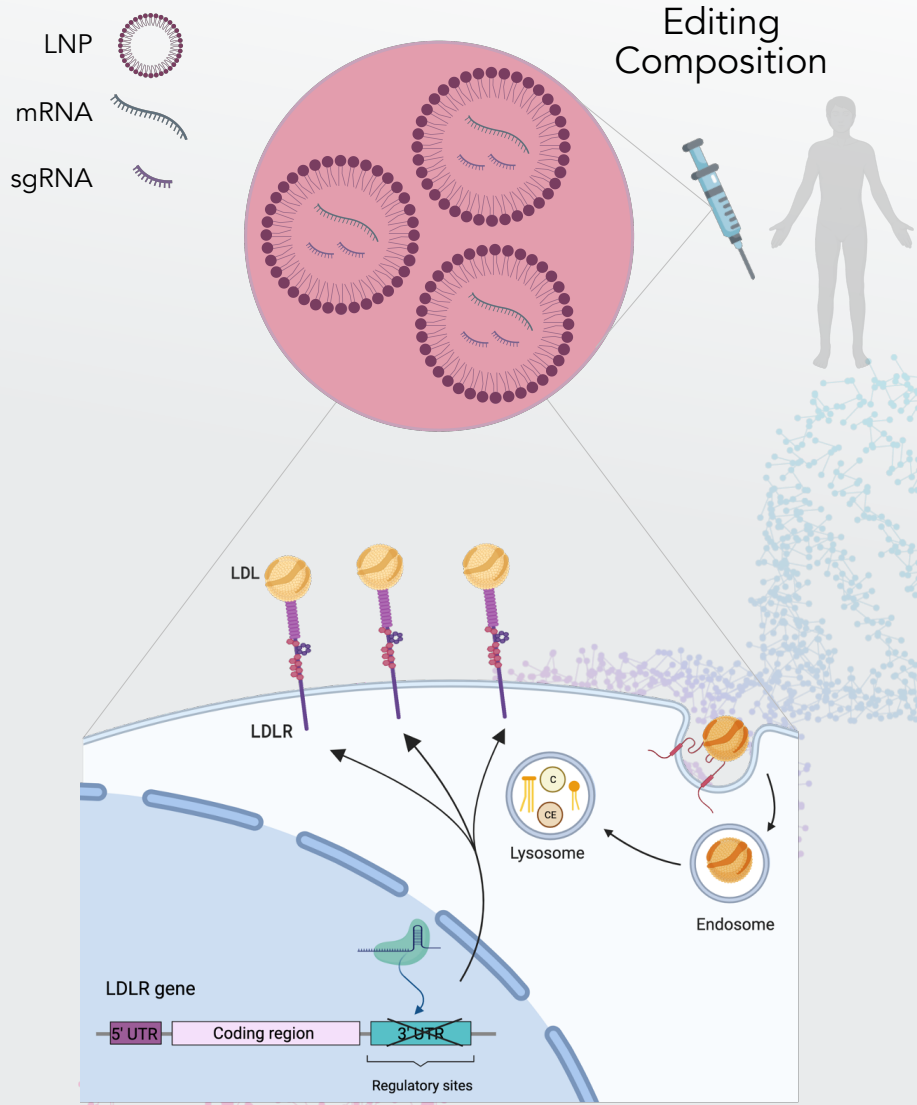
Alirocumab, Evolocumab: Monoclonal antibodies to PCSK9

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

Market revenue of anti-PCSK9:

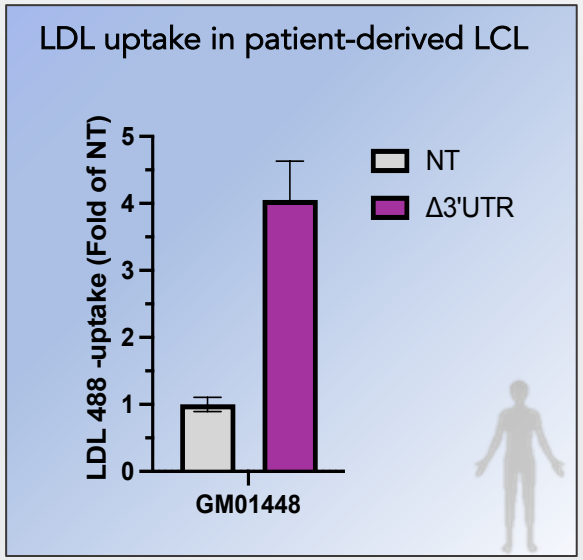
Repatha (Evolocumab): $654M in the 6 months from Jan 2022 to June 2022

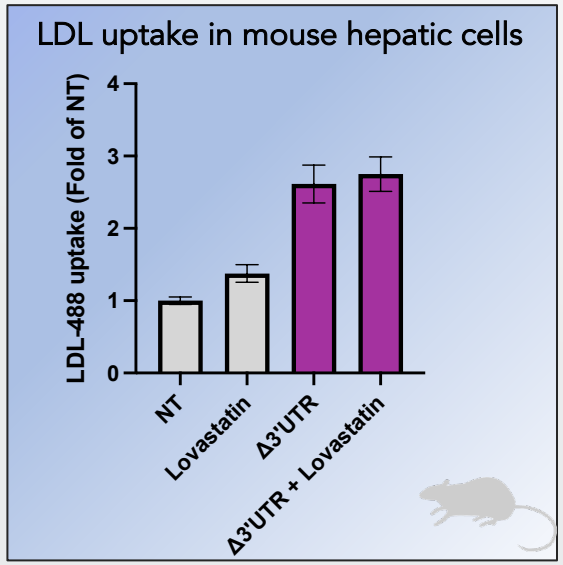
Praluent (Alirocumab): $220.3M in the 6 months from Jan 2022 to June 2022

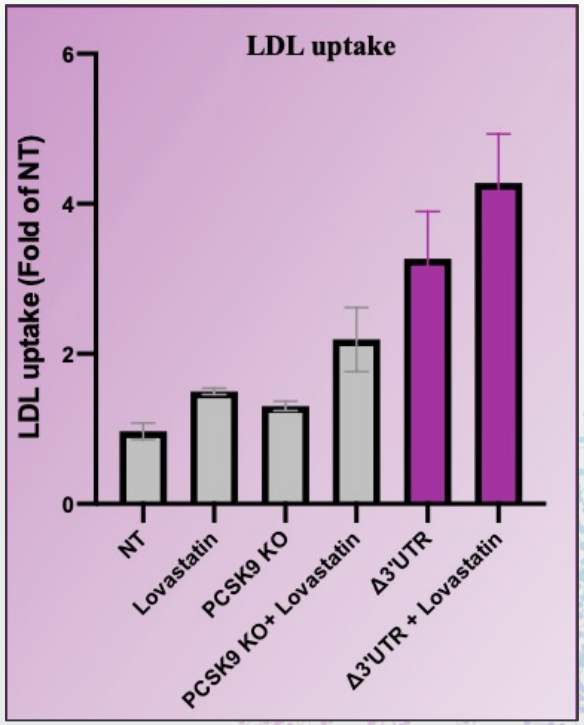


Market revenue of anti-PCSK9 treatments are according to Quarterly reports provided by Amgen (Aug 2022) and Regeneron (Aug 2022)

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







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

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

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

Source: Visualization of data collected in-house

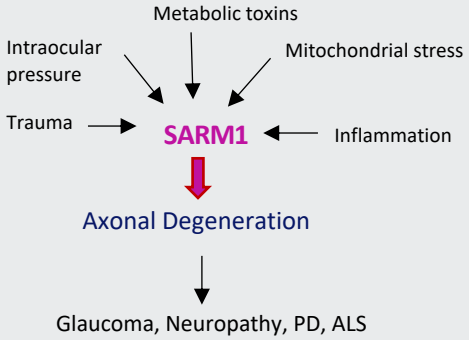
SARM1: Targeting not Only Genetic Diseases

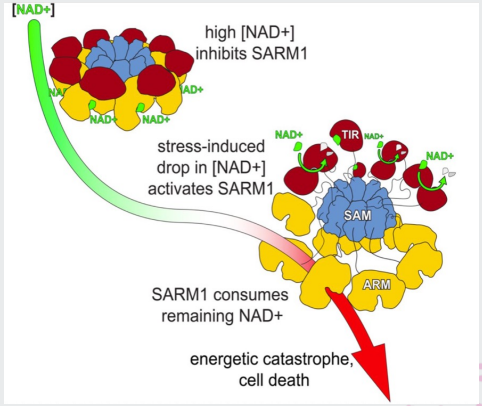
- A unique and generalized gene editing approach, to address axonal degeneration in multiple diseases, at the source
- Even though a gene-editing approach is taken, it's not addressing monogeneic genetic diseases, but rather addresses large indications, regardless of a specific gene or mutation

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

Source: Eli Lilly's IR press released dated October 15th, 2020

SARM1: Potential "Rescue" of Retinal Ganglion Cells (RGC) Degeneration in Glaucoma

Indication: Glaucoma is a complex neurodegenerative disease that causes progressive RGC death and optic nerve damage which results in irreversible vision loss

Prevalence: Glaucoma is the world's second-leading cause of blindness after cataracts. By 2040 it is expected to affect 110 million people

Therapy: Lowering intraocular pressure (IOP) either via drugs or surgeries

Unmet need: Current treatment strategies outcome is not very satisfactory since RGCs continue to die even after IOP management

Rational Behind SARM1 KO Development for Glaucoma

- High unmet need
- Target cell population: SARM1 contribution is well established in neurons; RGCs are the neurons that transmit visual information from the retina to the brain
- Less invasive administration- Intravitrial injection
- Delivery: LVLP (VSVG), AAV

Milestone	Date
POC editing composition	Q4 - 2022
In-vivo efficacy POC AAV delivery	Q1 - 2023
In-vivo POC	Q4 - 2023
Pre-IND	Q4 – 2024
GMP Production	Q1 - 2025
IND	Q4 - 2025

Wide Variety of Clinical Applications

PROGRAM	COLLABORATOR	LEAD OPTIMIZATION	PRE-CLINICAL	IND-ENABLING	PHASE 1-3
HEMATOLOGY					
EMD-101 **Severe Congenital Neutropenia**	University of Washington				
LIVER					
EMD-301 **Familial Hypercholesterolemia**	Proprietary				
EMD- 302 **Inborn Errors of Metabolism**	Proprietary				
OPHTHALMOLOGY					
EMD-203 **Macular Dystrophy**	Proprietary				
EMD-201 **Retinitis Pigmentosa**	Columbia University				
EMD-202 **Cone-Rod Dystrophy**	Proprietary				
IMMUNO-ONCOLOGY					
CAR-T Cells	Confidential				
NK Cells	Proprietary				

Summary

- Emendo is the most advanced privately held CRISPR Gene Editing 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!