Luxna Biotech

Bringing light to patients through innovative antisense oligonucleotide technologies

September 2026

J-Startup

Company / Leadership

Established	December, 2017
Employees	18
Location	Osaka Suita, Japan
Fundraising	20 M USD in total

Hideaki Sato, M.Sc.
President & CEO, Co-founder

- Oligonucleotide CMC expert
- Clinical Trials
- Global Collaborations
  Ajinomoto-Gene Design

Satoshi Obika Ph.D. , Co-founder
Professor, Pharmaceutical Sciences
The University of Osaka, Technical advisor at Luxna

- Nucleic acid chemistry authority, **BNA / LNA originator**
- XNAs Inventor
- Regulatory Science

Neurological Science

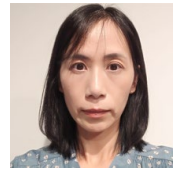
Hideaki Tomita Ph.D.
Biology Group Lead
Labs in US

Nucleic Acid Chemistry

Ajaya R. Shrestha Ph.D.
Chemistry Group Lead
Rena Therapeutics

ASO Drug Development

Kazuya Mori Ph.D.
Director, Research Planning
Nippon Shinyaku

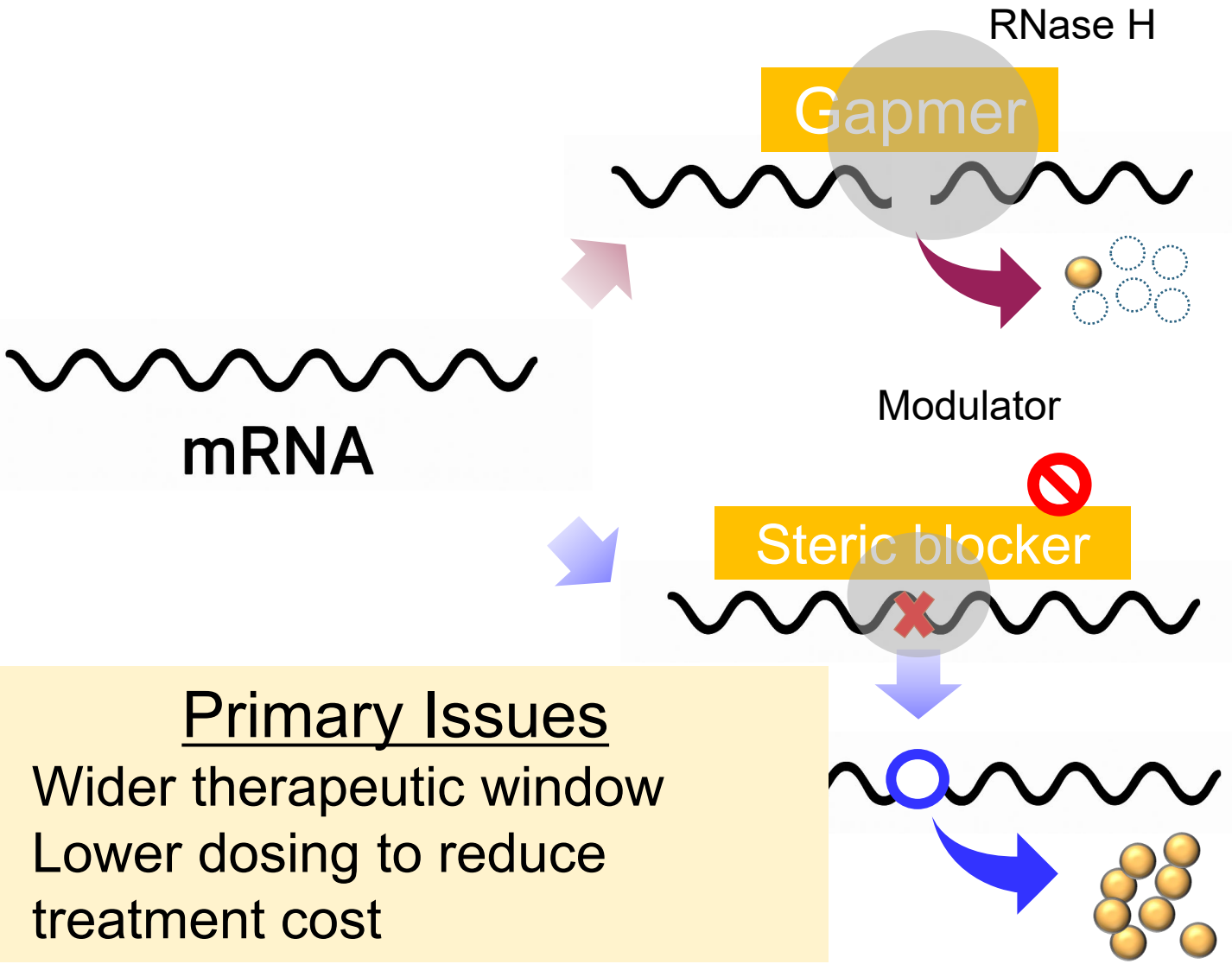


Alliance CNS expert

Hisako Sakai, M.Sc.
Director, Alliance
Mitsubishi Tanabe

Why ASO

Antisense (ASO) is the most established mRNA modulator



Reduction of disease-causing proteins to achieve therapeutic benefit

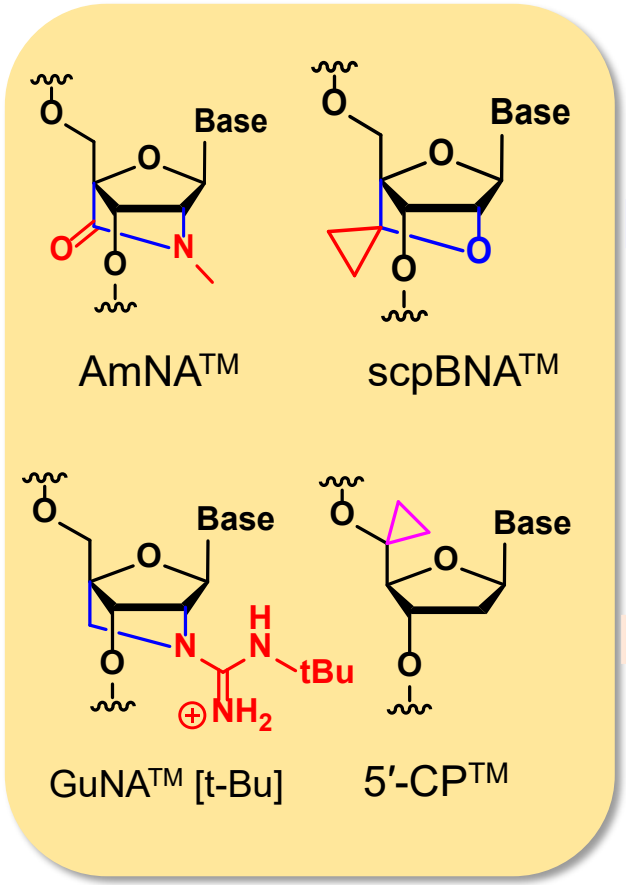
Increased therapeutic protein expression to achieve therapeutic benefit

Primary Issues

- Wider therapeutic window
- Lower dosing to reduce treatment cost

ASO Drug Discovery Platform LuxiAP™

- LuxiAP™ is an ASO discovery platform built on four modified nucleic acid chemistries, with a proven track record of advancing ASOs into clinical development.



Competitive edge

Improved Safety
- in Liver
- in CNS
- Reduced immune activation

Enhanced Activity
- Extended duration
- Improved potency
- upregulation

Identification of ASO drug candidate

End-to-End ASO Drug Discovery System

- From target selection to ASO candidate generation in as little as 18 months
- High-speed optimization cycle with in-house design, synthesis, and evaluation
- A drug discovery platform capable of simultaneously advancing multiple projects

2 weeks

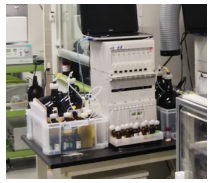
Sequence design

- RNA structure analysis
- Toxicity risk analysis
- Advanced off-target analysis

3 weeks

HTP system

- Completed in half the time of standard outsourcing using the HTP ASO synthesis system
- Support for in vivo synthesis



4 weeks

Cell-based evaluation

- In vitro Efficacy Assessment
- Comprehensive toxicity evaluation, including immunostimulatory effects

PBMC

4 weeks

Animal testing

- ASO Activity Assessment
- Safety evaluation
- Compatible with various mouse administration methods, including intraventricular administration

PK Evaluation

- Development of an evaluation system using HRLC-MS
- Development of an ELOSA assay system

Successful Optimization

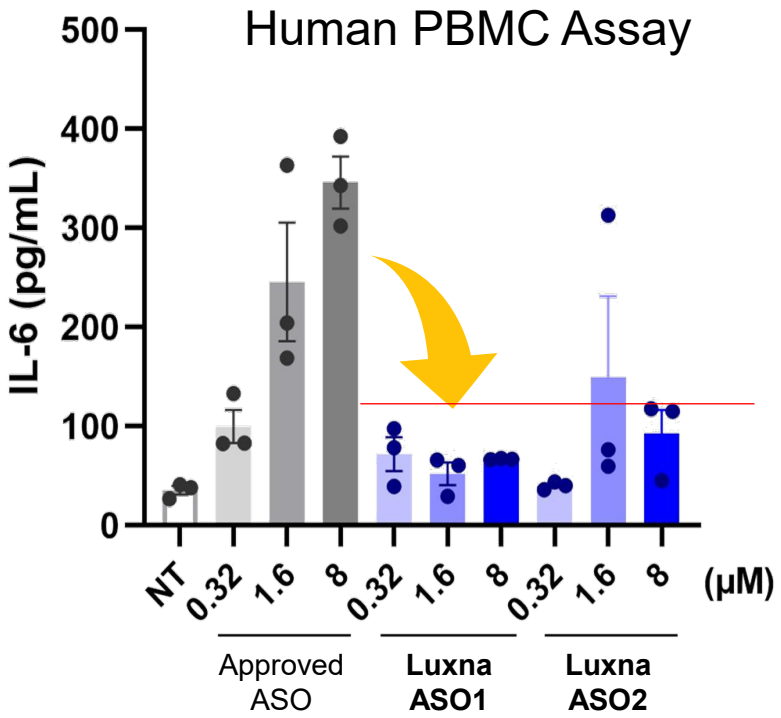
Science behind LuxiAP™

How our chemistry enables differentiated ASO optimization

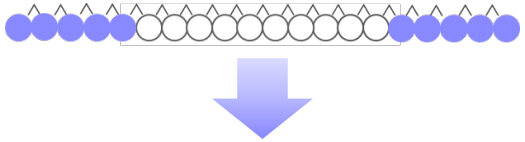
Reduced Immune Activation

Partial GuNA substitution mitigates ASO-induced immune activation

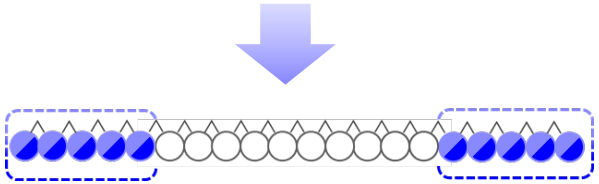
- We significantly reduced immune activation seen with an approved drug by partially replacing it with GuNA nucleic acids without changing the sequence.



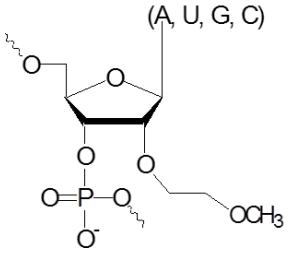
An approved ASO composed of MOE showed dose-dependent immune activation



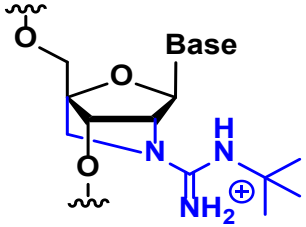
Partial conversion from MOE to GuNA



Toward an ASO with reduced immunostimulatory activity



2'-MOE



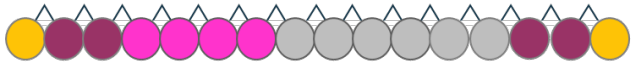
GuNA

Enhanced Potency with Improved Liver Tolerability

Our XNA chemistry improves activity while reducing ALT elevation

- Our proprietary modified nucleic acid chemistry showed ~3-fold higher activity than MOE and markedly lower ALT elevation than LNA under the tested conditions

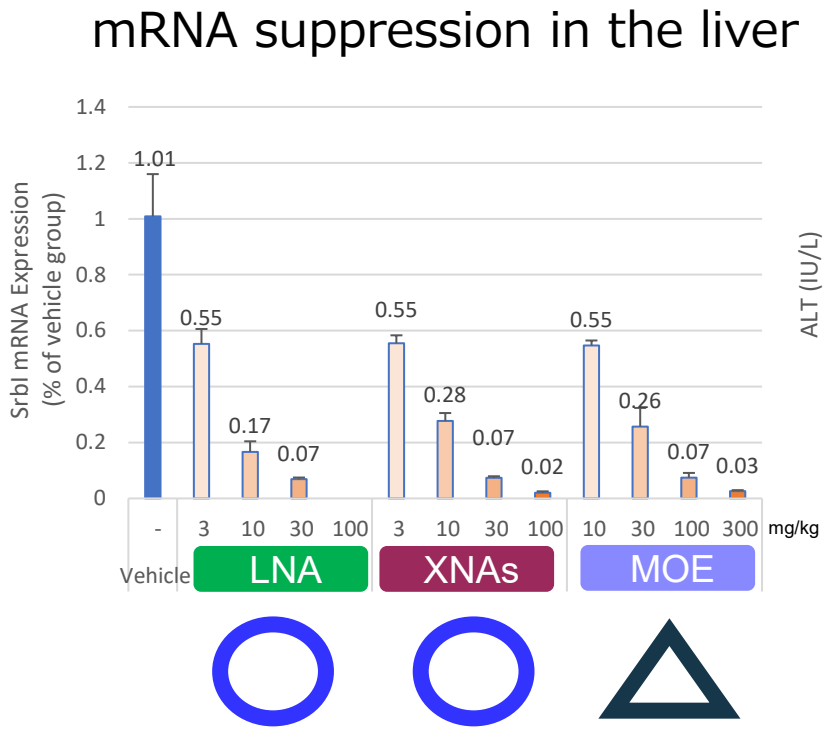
XNAs 3-10-3 all PS gapmer as followed modification

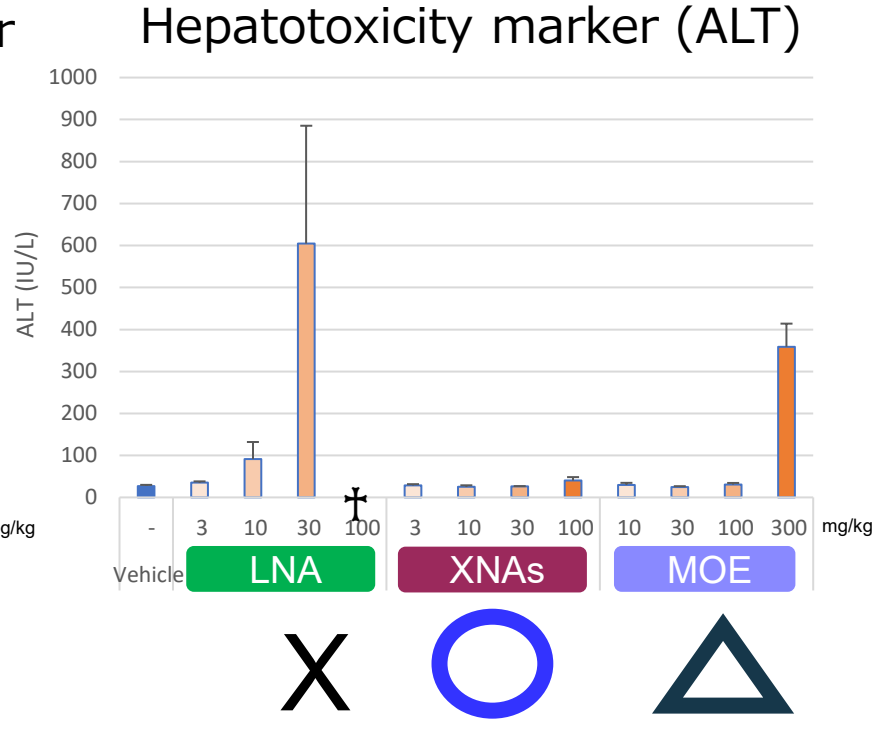


●:AmNA™, ●: scpBNA™, ●:5′-CP™, ○:DNA,

LNA 3-10-3 all PS LNA gapmer

MOE 5-10-5 all PS 2'-MOE gapmer





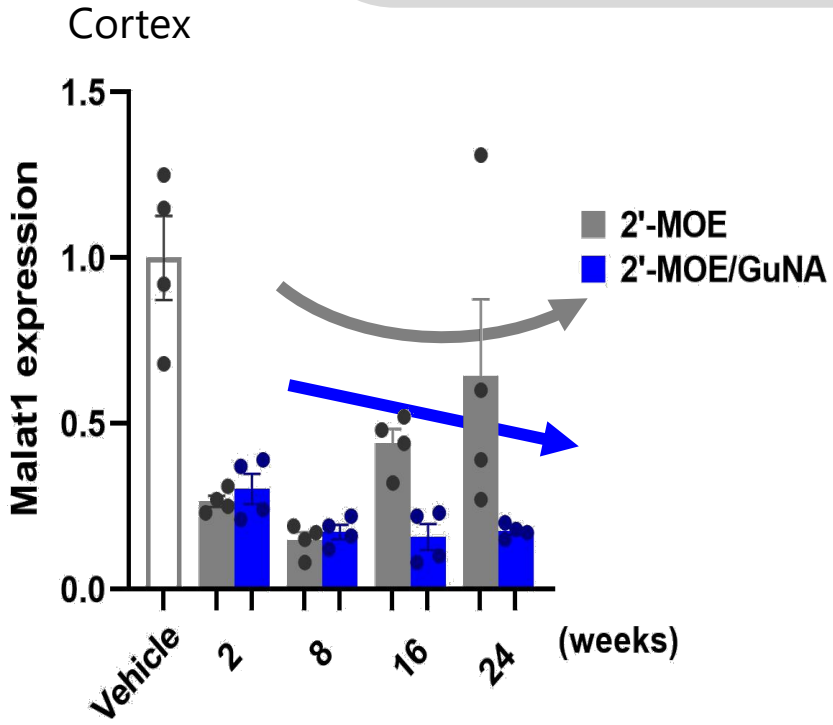
- Single dose in mice
- Analyzed 7 days after administration

Extended Duration in the CNS

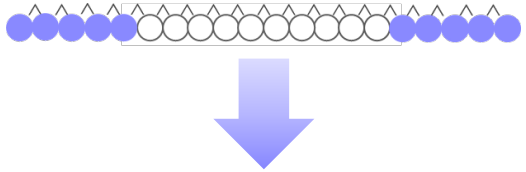
GuNA-containing ASOs sustain target knockdown for up to 6 months

- GuNA-containing ASOs maintained stronger target knockdown than MOE at later time points, with activity sustained for up to 6 months in mouse brain

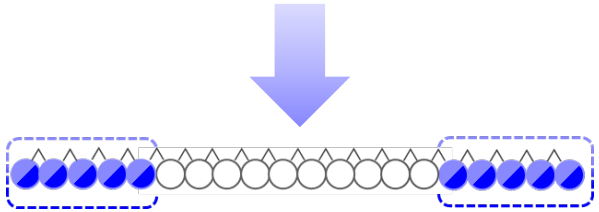
Single ICV administration in mice gives
6-month knockdown persistence



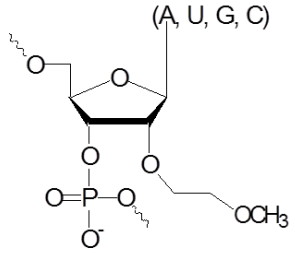
ASO composed of MOE



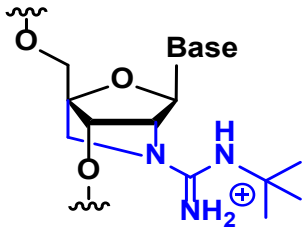
Partial conversion from MOE to GuNA



To ASO with long-term efficacy



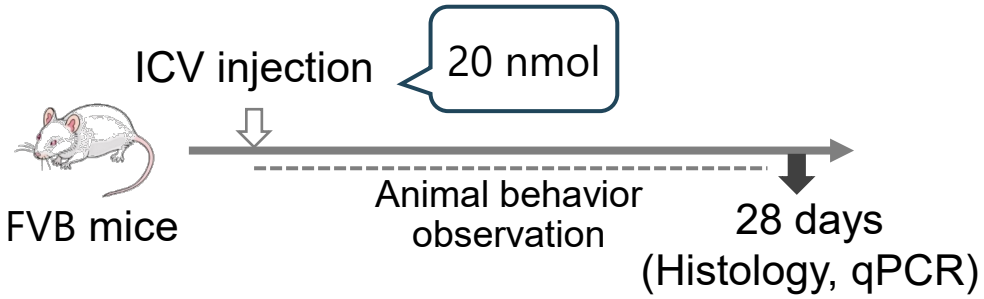
2'-MOE

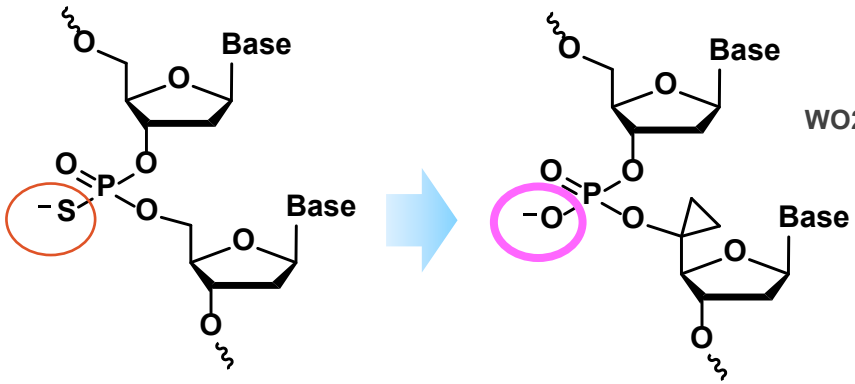


GuNA™

Mitigation of CNS Neurotoxicity

5′-CP/PO gap optimization reduces acute and delayed neurotoxicity



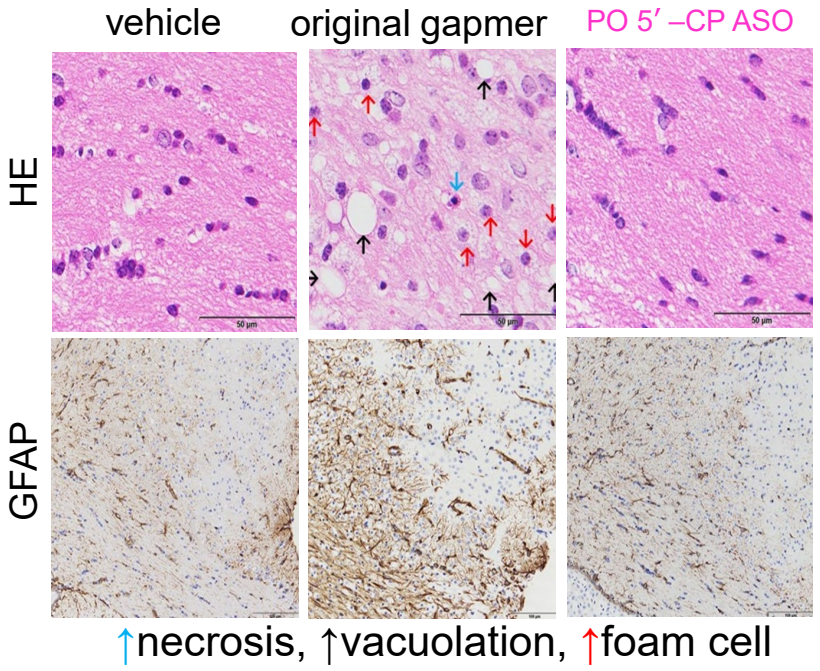


WO2024257848A1

Gene target:
Human UBE3A-NAT

Original: 4.4.PS.L (*Sci Transl Med* . 2023 Mar 22;15(688):eabf4077.)

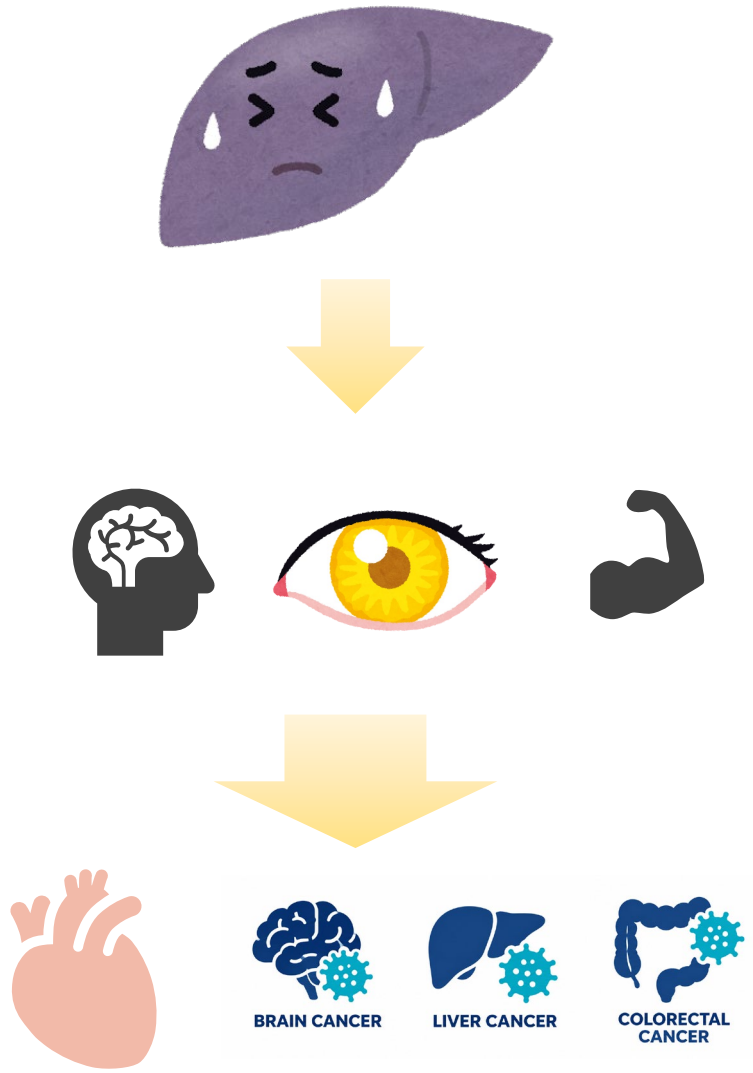
	~1h	3~5h	1~9d	10~17d	18d	19~28d
original gapmer	Hyperactive, Salivation, dyspnea	dyspnea	-	gait abnormality (paralysis)	dead (N=1)	gait abnormality (paralysis)
Modified 5′-CP PO ASO	Hyperactive, Salivation, dyspnea	dyspnea	-	-	-	-



Unique method of ASO gap optimization for CNS application

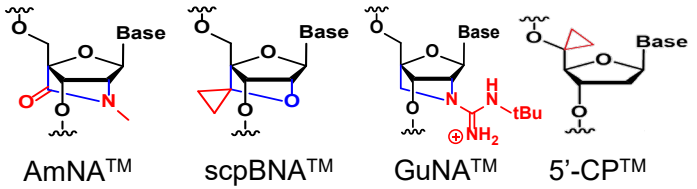
Therapeutic areas for LuxiAP™

- **Hepatology**
  - ✓ Our modified nucleic acid chemistries offer enhanced ASO activity with improved liver tolerability.
- **Creating High Value-Added Products Through Expanded Indications**
  - ✓ In **CNS, ophthalmology,** and **neuromuscular diseases**, we are advancing differentiated ASO candidates designed for improved activity, safety, and duration.
- **Prospects for Next-Generation Modalities**
  - ✓ We are also exploring selective delivery technologies, including DDS and antibody–oligonucleotide conjugates (AOCs), through external collaborations, with potential applications in oncology.



Business Model

Modified nucleic acid from Osaka University



Modified nucleic acid (XNA)

LuxiAP™

- Monomer
- Drugs Design
- Optimization Know-how

SPI INABATA & CO., LTD.

Supply of monomers

Supply to licensees

Collaborative ASO drug discovery

Licensing of XNAs technology

XNAs Oligo Research reagent supply

Internal programs for early out-licensing

SERVIER
BIOSPRING
KH NeoChem

Undisclosed pharmaceuticals

Takeda

BIOSPRING

Multiple programs achieved animal PoC with a safety margin

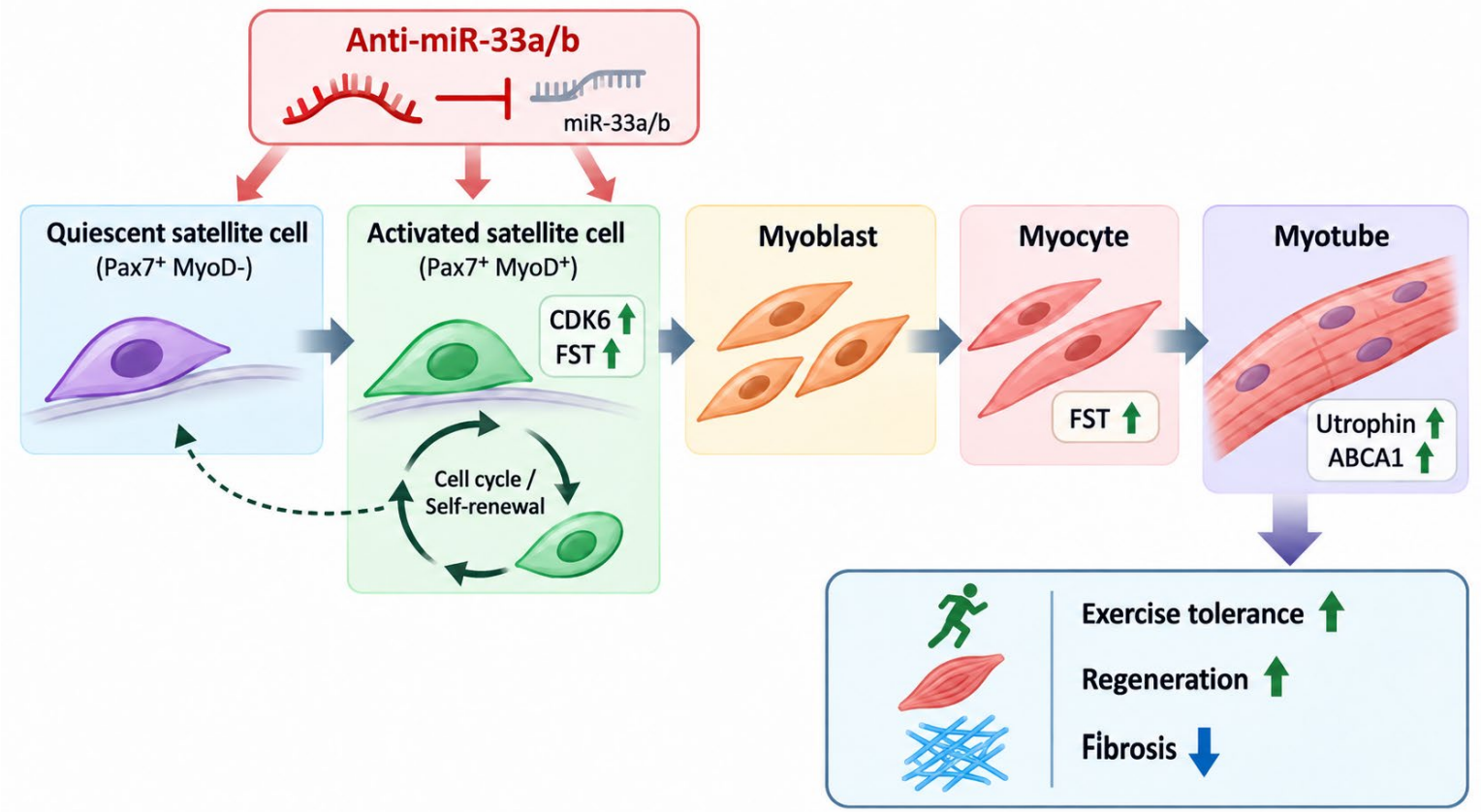
Internal Pipeline

- We are developing programs with academic partners in areas where our artificial nucleic acid technology can offer a clear advantage.
- For programs with animal PoC, we aim for early out-licensing and IND submission by 2029 together with a pharmaceutical partner.
- We are seeking a strategic pharmaceutical partner to advance these assets into clinical development.

Target Indications	ASO Type	Discovery	Lead Optimization	Animal POC	Non-GLP TOX	IND enabling	Phase 1	Partner
Muscular dystrophy (DMD, regardless of genetic mutation)	Anti-miR							Osaka Univ., Kyoto Univ., AMED
Glaucoma (optic nerve protection)	Gapmer							Luxna owned
Glaucoma (optic nerve protection)	Up-regulator							Luxna owned
Spinal cord injury	Gapmer							Fujita Health University
ALS / Repeat expansion diseases	Gapmer							Luxna owned
Multiple system atrophy (MSA)	Gapmer							Osaka Univ., AMED

Duchenne muscular dystrophy (DMD), regardless of genetic mutation by anti-miR 33 ASO program

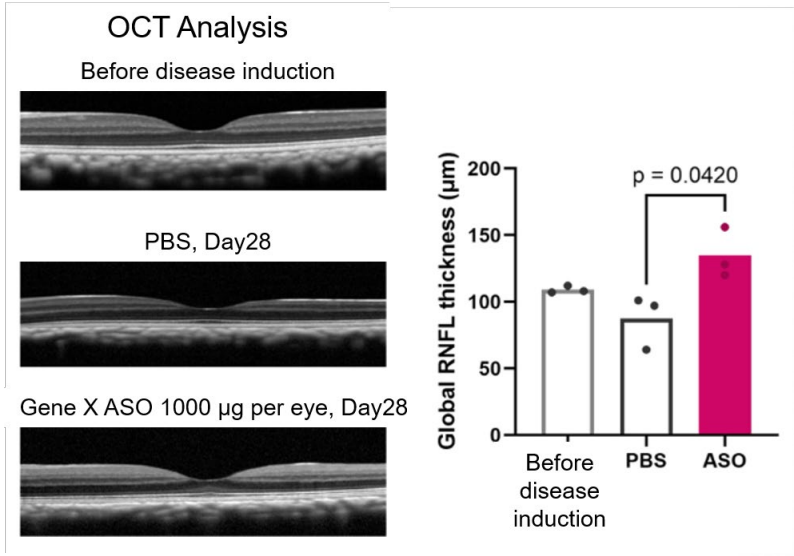
Skeletal muscle regeneration therapy via miR-33 inhibition ASO

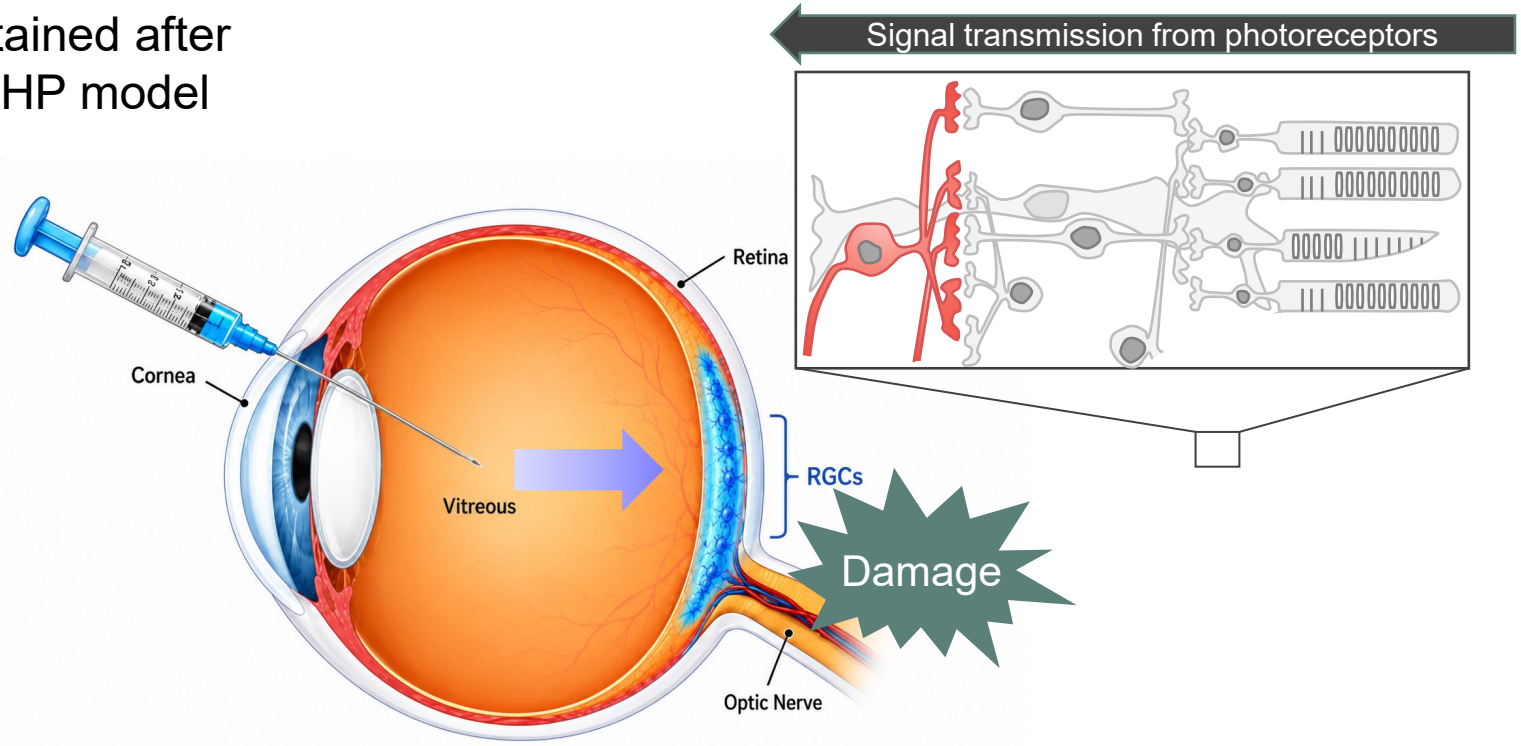


➡ In humanized miR-33a/b KI/mdx mice expressing miR-33a/b at human-comparable levels, optimized anti-miR ASO maintained motor function and improved blood biomarkers.

IOP-Independent Optic Nerve Neuroprotection for Progressive Glaucoma
by GeneX Gapmer ASO program

A structural protection signal was obtained after single IVT dosing in a translational NHP model





Partnership Opportunities

Strategic Partnership

Explore strategic partnership or corporate transaction to scale Luxna's ASO platform

Platform collaboration

Apply LuxiAP™ chemistries to partner-selected targets and programs

Access LuxiAP™ chemistry and know-how to accelerate your ASO development

Out-license / Co-development

Advance selected Luxna internal pipeline assets through licensing or co-development

Lead Optimization Collaboration

Improve potency, safety, and duration of existing ASO candidates

Partner with Luxna to build differentiated ASO therapeutics

Luxna Biotech