Luxna Biotech

Bring the “light” to patients with our novel antisense (ASO) technologies

Drug discovery platform and Drug asset driven

President CEO, Hideaki Sato M.Sc.

Osaka University spin-out



J-Startup

Executive summary

- LuxiAP™ is a proprietary modified nucleic acid platform that solves key challenges in nucleic acid therapeutics: safety and manufacturing cost.

- Preclinical studies showed longer efficacy and better safety than standard-modified ASOs, directly enhancing pipeline value.

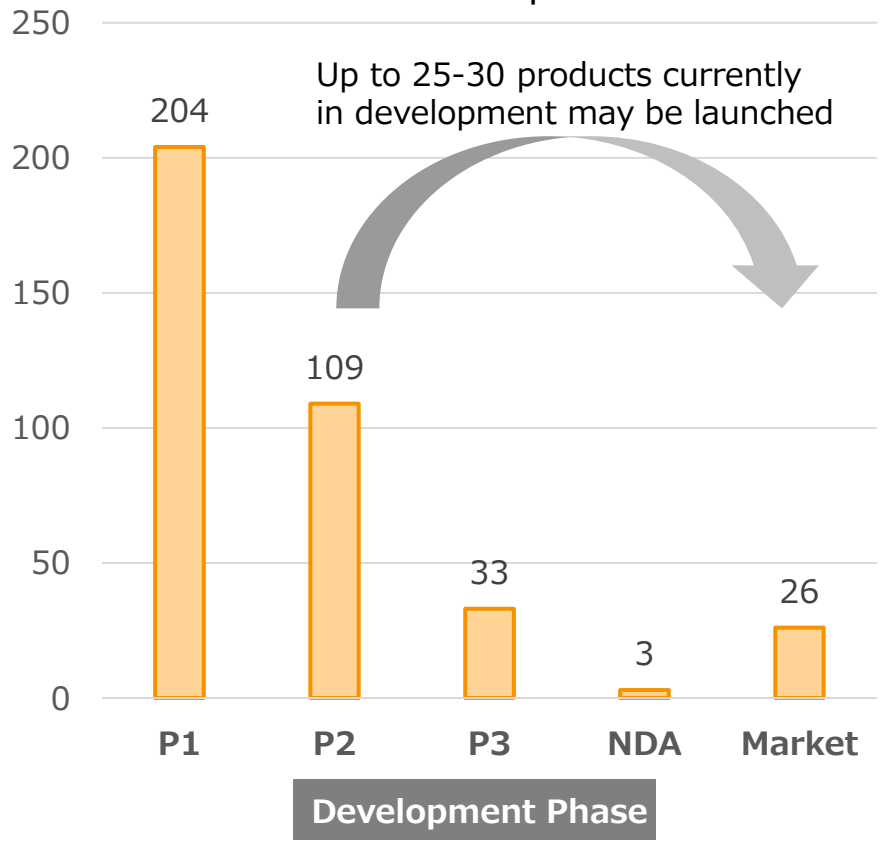
- By integrating this platform, your company can quickly secure proprietary nucleic acid modification technology and a differentiated position in a fast-emerging market.

- To further expand this technology platform, we are seeking broader collaborations to supply oligonucleotides as research reagents on a global scale.

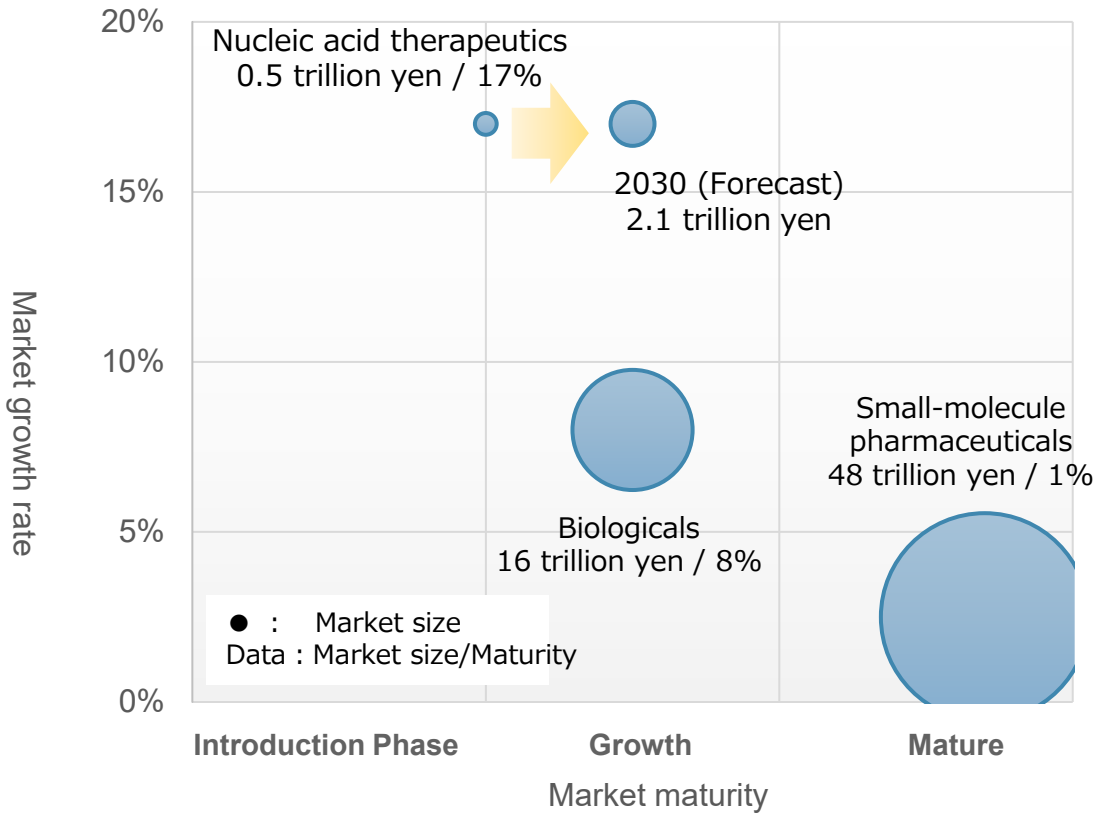
The Third Drug Category: Competitive Advantages Offered by Nucleic Acid Therapeutics

- Nucleic acid therapeutics are expected to double within the next 10 years, with growth on par with that of antibody drugs

Number of commercialized and development-stage for nucleic acid therapeutics



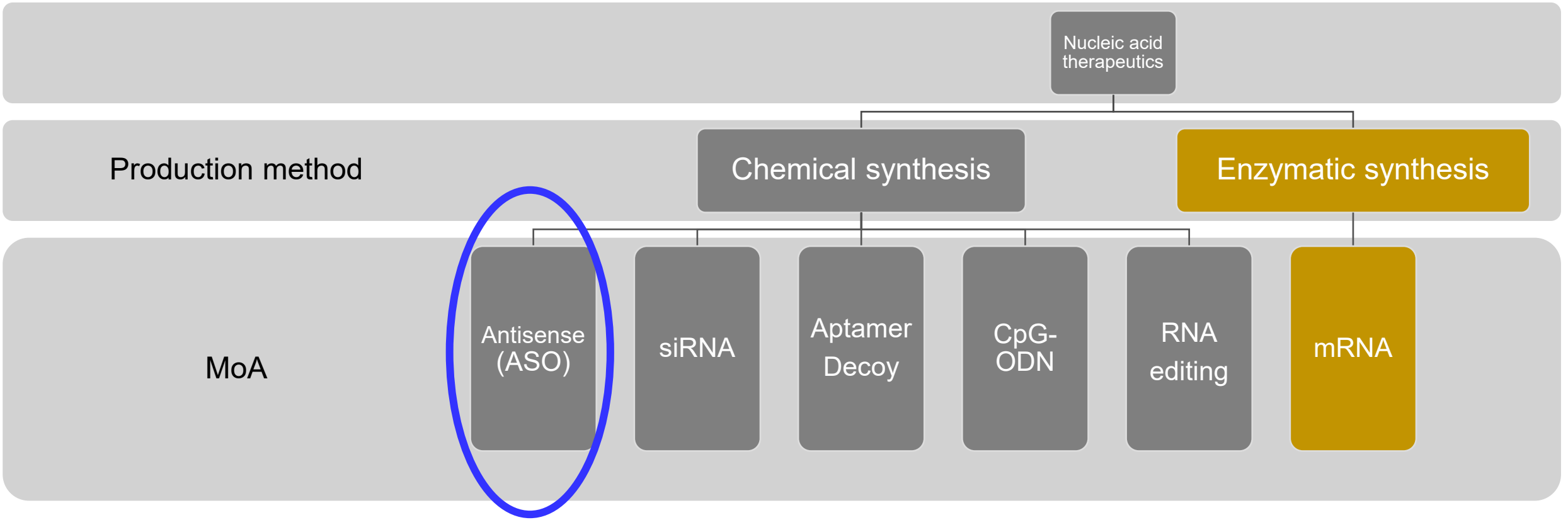
Market size by modality (2020) and growth rate (2020–2030)



Chen L et al., The Innovation Drug Discovery, 2026 (compiled by our company); success rates sourced from the Pharmaceutical Industry Policy Research Institute website

Compiled by our company based on data from the Development Bank of Japan (March 2022)

Classification of nucleic acid therapeutics and Luxna focuses



Our proprietary modified nucleic acid is suitable for ASO, Luxna focuses on ASO drug discovery

Classification of nucleic acid therapeutics and Luxna focuses



SPINRAZA® (nusinersen) injection 12 mg/5 mL

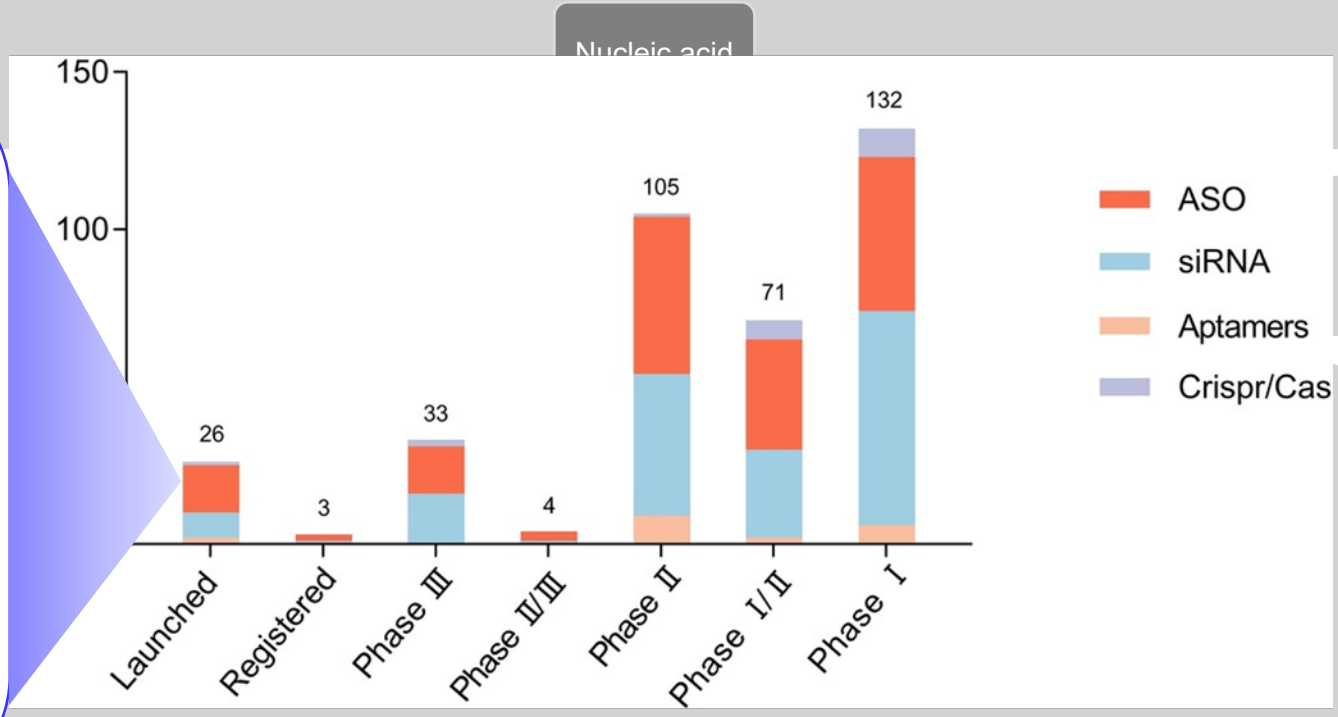
SMA

QALSODY® (tofersen) 100 mg/15 mL injection

SOD1 mut ALS

Viltepso® (viltolarsen) injection

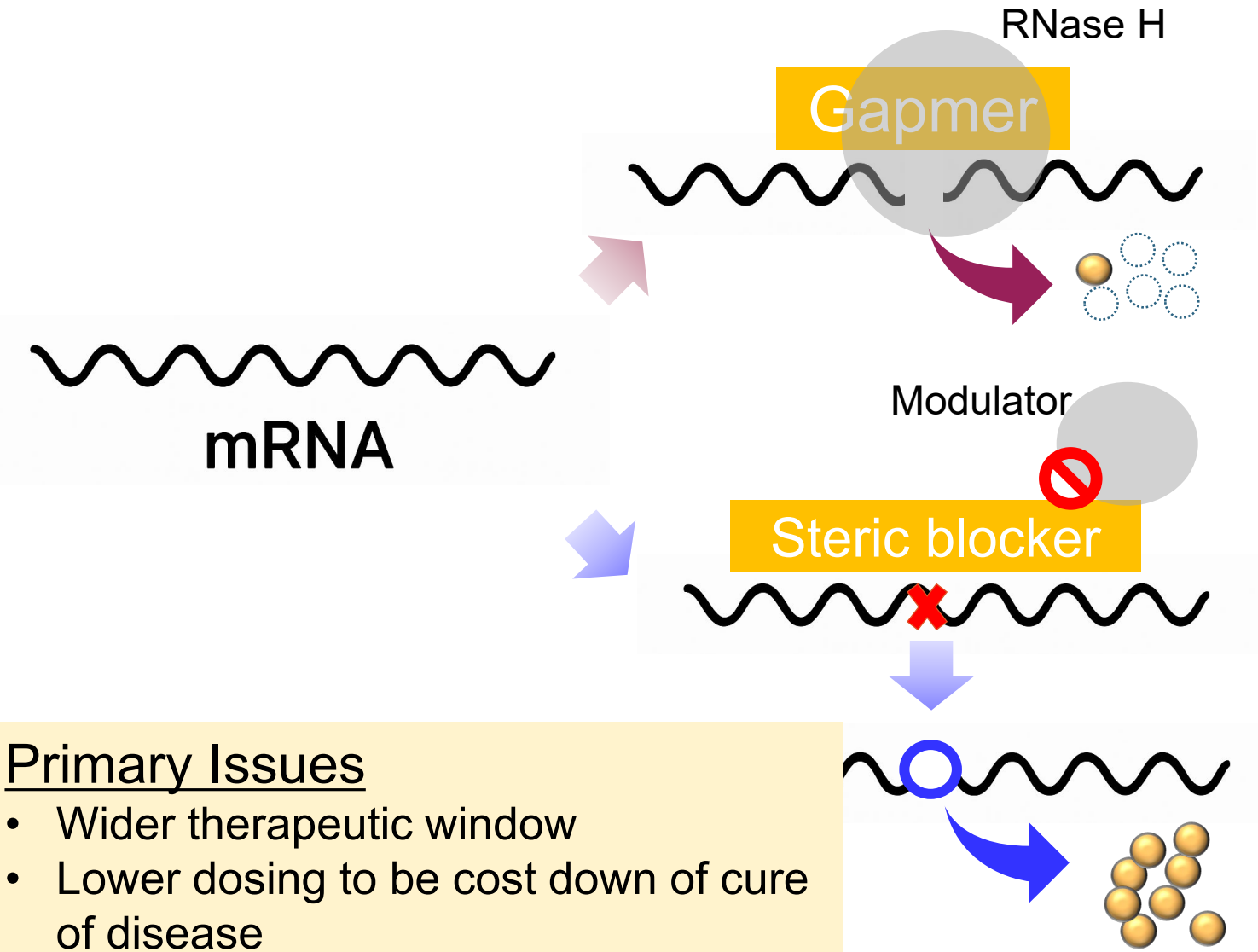
Exon53 mut DMD



Chen L., Xu J., Li J., et al. (2026). New trends of oligonucleotide-based therapeutics. The Innovation Drug Discovery 1:100007.

Our proprietary modified nucleic acid is suitable for ASO, Luxna focuses on ASO drug discovery

Antisense (ASO) is the most established mRNA modulator



Reduction of unnecessary protein ● induces cure of diseases

Increased protein ● promotes cure of diseases

Primary Issues

- Wider therapeutic window
- Lower dosing to be cost down of cure of disease

Bottlenecks in the expansion of the nucleic acid therapeutics market

- Market growth is expected to accelerate further as safety margins improve and manufacturing cost structures are optimized.

Business constraints		Market Impact
Safety margin	Immune activation and Inflammation Induction risks	Restrictions on repeated dosing, limitations on chronic disease indications, and lower probability of successful development
	Narrow safety margin	Upper dose limit constraints, difficulty in expanding indications, increased post-approval risks
Cost down of cure of disease	High-dose-dependent design	Increased API usage, rising COGS, reduced price competitiveness

Company Overview

Establishment	December 17, 2017
Number of Faculty Members	18, including 6 Ph.D. holders (including 3 non-regular employees)
Headquarters	9th Floor, Techno Alliance Building, Osaka University Suita Campus
Research Facilities	Headquarters Research Facility, Saito Bioincubator 203
Capital	90 million JPY
Funding	Equity: 2.36 billion JPY; Loans: 610 million JPY
Major Shareholders	VC: Osaka University Venture Capital, Shinsei Capital Partners, and 10 other funds Corporate investor: Nissan Chemical, UBE, Summit Pharmaceuticals International

Founder Background



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

- Oligonucleotide CMC
- Clinical development
- Experience global collaboration
- Business development at Ajinomoto-Gene Design

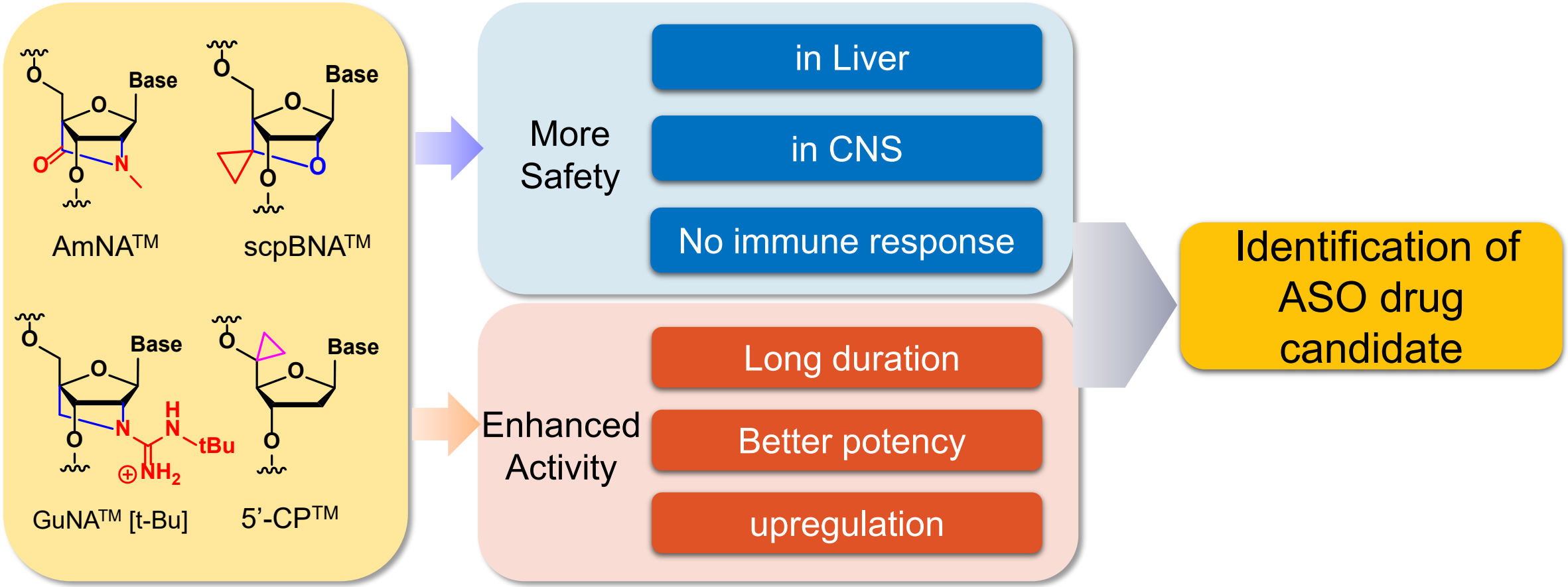


Satoshi Obika, Ph.D.
Professor, Graduate School of Pharmaceutical Sciences, Osaka University
Technical Advisor and Co-founder

- Leading expert in nucleic acid chemistry
- Inventor of Luxna modified nucleic acid chemistries
- Contributor to the regulatory science of nucleic acid therapeutics

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.



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

高速塩基配列検索 GGGenome

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

Technological differentiation

- Minimization of toxicity
  - ✓ Achieves biostability independent of PS modification, fundamentally reducing PS-derived toxicity
  - ✓ Established proprietary application technology to control immune activation

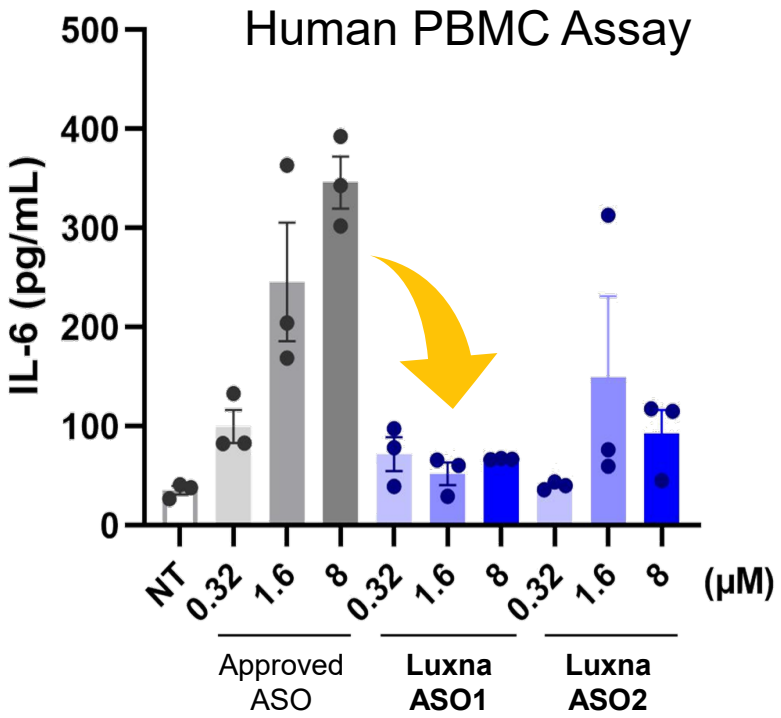
- Maximizing activity
  - ✓ Demonstrates high therapeutic efficacy at low doses due to high base specificity and strong RNA binding affinity
  - ✓ Achieves Long-lasting effectiveness through excellent in vivo stability

- Strong IP position
  - ✓ Holds exclusive rights to implement substance patents for each modified nucleic acids
  - ✓ Secured manufacturing technology licenses for modified nucleic acids and proprietary intellectual property
  - ✓ Holds utility patents for applications such as neurotoxicity reduction, as well as co-created IP with pharmaceutical companies, chemical firms, and universities

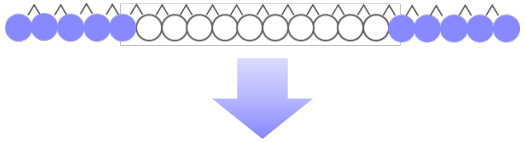
- Competitive advantages
  - ✓ Leading companies (Ionis, Alnylam) have internalized artificial nucleic acid technologies
  - ✓ We create co-creation value by offering a wide range of technologies
  - ✓ A highly scalable strategy that treats all ASO development companies as potential partners

Nonclinical data:
Conversion to our chemistry mitigates immune activation

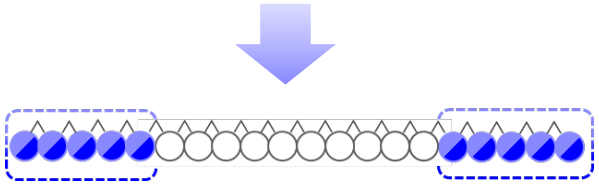
- We significantly reduced immune activation seen with an approved drug by partially replacing it with GuNA nucleic acids without changing the sequence.



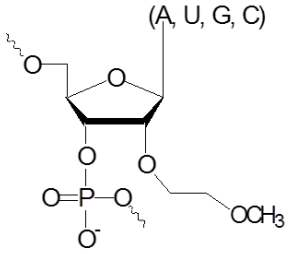
An approved ASO composed of MOE showed dose response immune activation



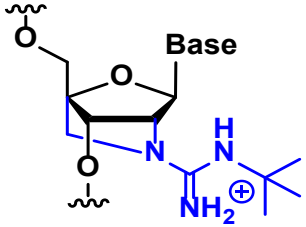
Partial conversion from MOE to GuNA



To be better ASO without Immunostimulatory properties



2'-MOE



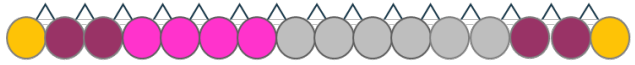
GuNA

Non-clinical data: Achieved a threefold increase in activity in the liver and a 90% suppression in hepatotoxicity

- Our proprietary modified nucleic acid outperformed standard ASO chemistry, showing ~3-fold higher activity than MOE and less than one-tenth the liver toxicity of LNA.

Successfully achieved both enhanced activity and reduced hepatotoxicity in the liver

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



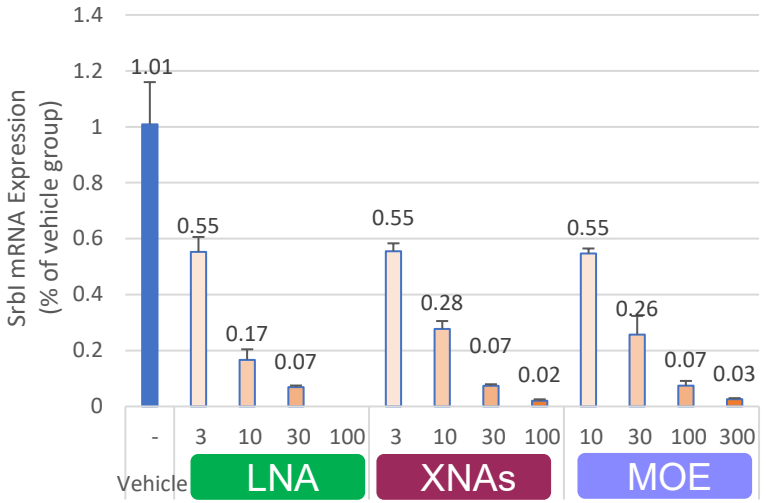
● :AmNA™, ● : scpBNA™, ● :5'-CP™, ○ :DNA,

LNA 3-10-3 all PS LNA gapmer

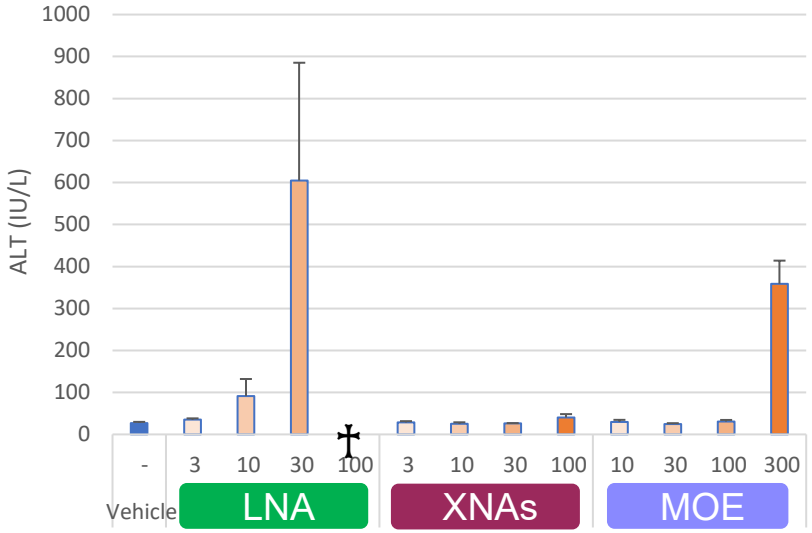
MOE 5-10-5 all PS 2'-MOE gapmer



mRNA suppression in the liver



Hepatotoxicity marker (ALT)



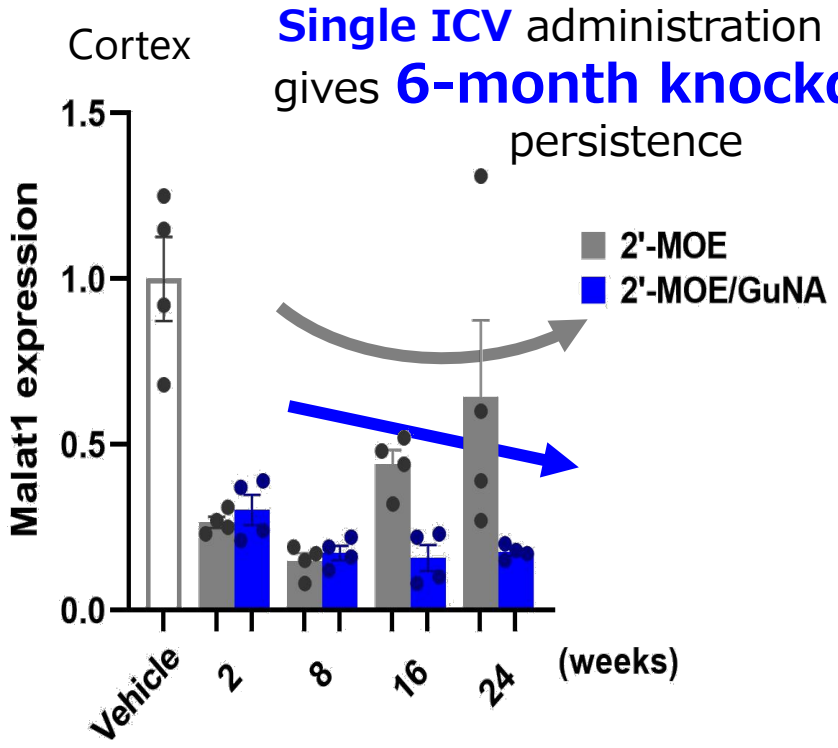
- Single dose in mice
- Analyzed 7 days after administration

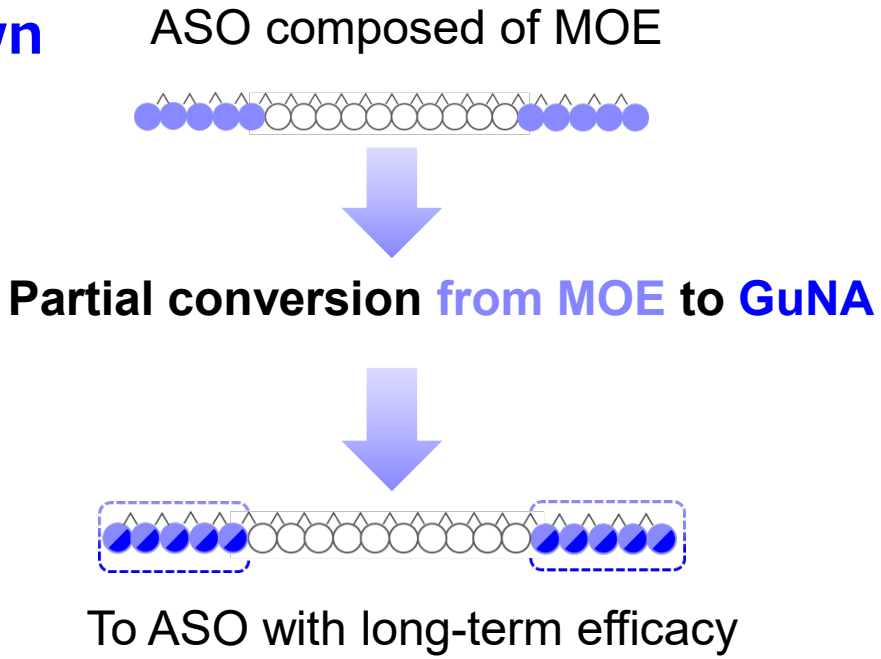
Nonclinical data:
Achieved 3-fold longer-lasting efficacy in the brain

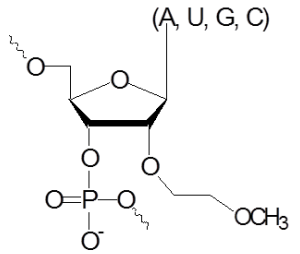
- Our proprietary modified nucleic acid “GuNA” achieved three times the long-term efficacy compared to the MOE of the global standard ASO sequence.

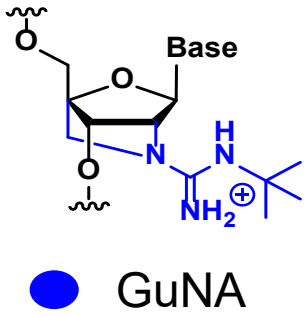


Improved long duration in the Brain







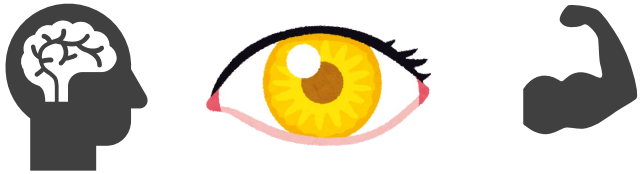


Expansion of Indications for LuxiAP™

- **Hepatology**
  - ✓ Our modified nucleic acid chemistries show higher liver ASO activity and lower hepatotoxicity than existing modified ASOs.



- **Creating High Value-Added Products Through Expanded Indications**
  - ✓ In **CNS, Ophthalmology, Neuromuscular**, we are advancing ASO candidates with enhanced activity and reduced toxicity to deliver differentiated, high-value solutions.

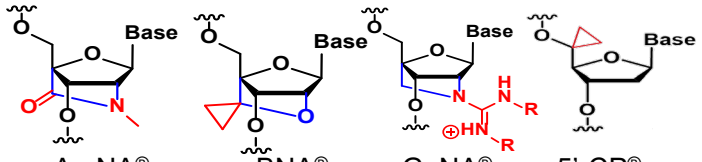


- **Prospects for Next-Generation Modalities**
  - ✓ Looking ahead, we see potential in oncology, particularly in combination with immunotherapy, through DDS integration and antibody –ASO conjugation (AOC) for specific delivery external collaboration.



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

Stable revenue

Supply to licensees

Collaborative ASO drug discovery

Licensing of XNAs technology

XNAs Oligo Research reagent supply (CRO works)

Internal pipeline, go for early out-license

SERVIER

JP-Pharmaceutical A

BIOSPRING

KH NeoChem

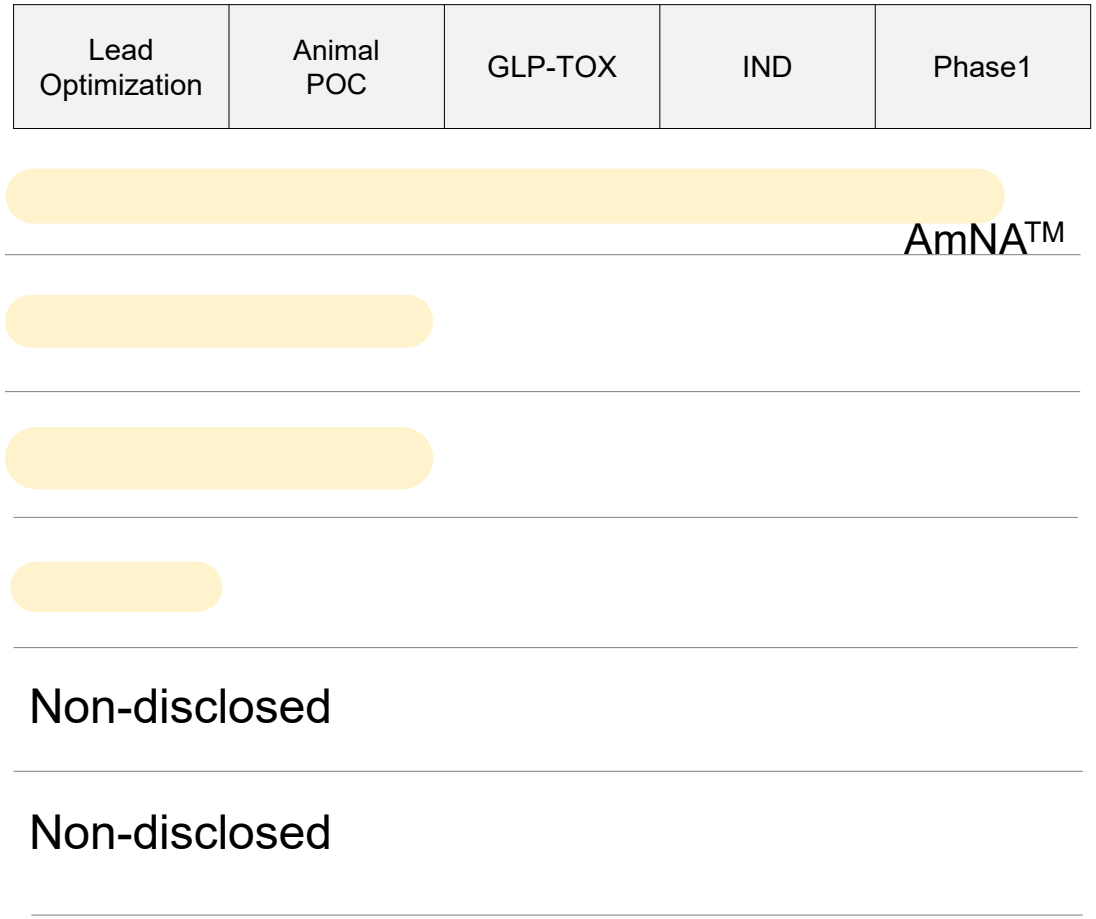
Takeda

BIOSPRING

Multiple programs achieved animal PoC with a safety margin

Collaboration Pipelines

Target Indications	Collaboration scheme	Partner
Gastric cancer with peritoneal metastasis	XNAs tech-out license	Nagoya University Investigator-initiated clinical trial
Metabolic liver disorder	Co-development	Undisclosed JP-Pharma A
Neurological disease	Co-development	SERVIER
Novel functional oligonucleotide	Co-development	BIOSPRING
Novel functional oligonucleotide	Co-development	KH NeoChem
Neurological disease, multiple target genes	XNAs tech-out license	Takeda



Internal Pipeline

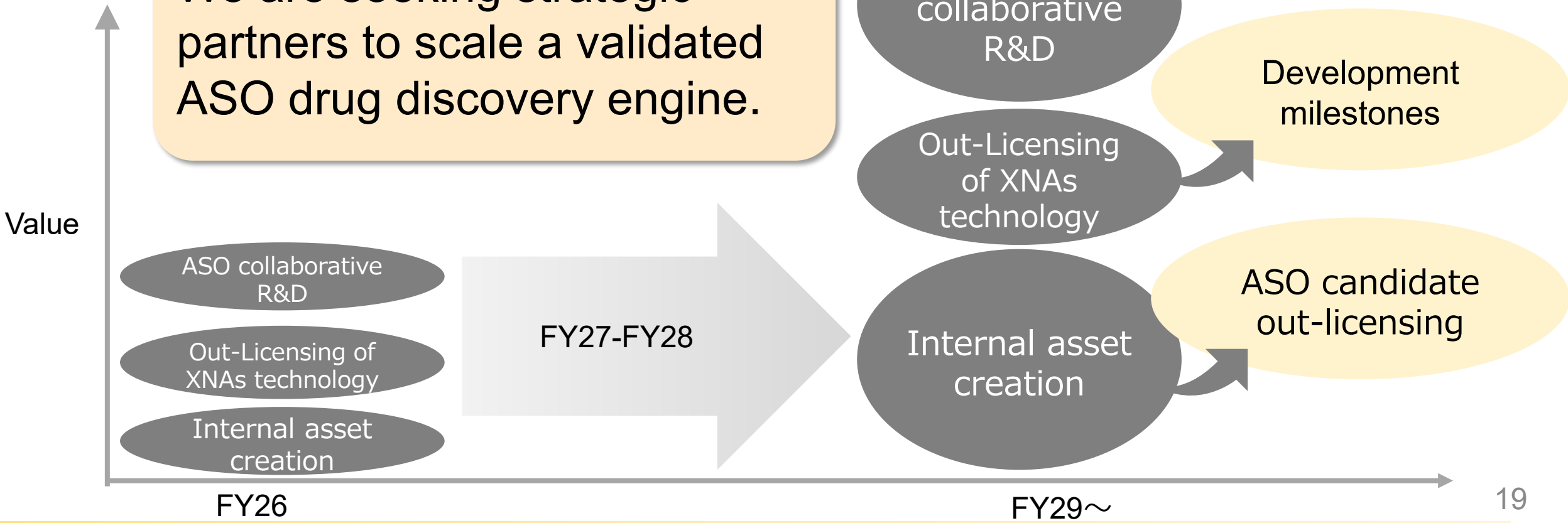
- We are developing programs with academic partners in areas where our artificial nucleic acid technology can offer a clear advantage.
- For programs with animal PoC, we aim for early out-licensing and IND submission by 2029 together with a pharmaceutical partner.
- We are seeking a strategic pharmaceutical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

Target Indications	ASO Type	Discovery	Lead Optimization	Animal POC	Non-GLP TOX	IND Enable	Phase1	Partner
Muscular dystrophy (DMD, regardless of genetic mutation)	Anti-miR						2029	Osaka Univ. Kyoto Univ、AMED
Glaucoma (optic nerve protection)	Gapmer						2029	Luxna own
Glaucoma (optic nerve protection)	Up-regulator							Luxna own
Spinal cord injury	Gapmer							Fujita Health University
ALS / Repeat expansion diseases	Gapmer							Luxna own
Multiple system atrophy (MSA)	Gapmer							Osaka Univ. AMED

Growth strategy / Ask

Accelerate Co-Development, Build Value, Advance to the Clinic

We are seeking strategic partners to scale a validated ASO drug discovery engine.





Confidential