



Accelerate Drug Development
using Oligonucleotides



Olix
Pharmaceuticals

Investor Relations 2021



Forward-looking Statements

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Contents

Prologue

Chapter 1. Company Overview

Chapter 2. Core Technology

Chapter 3. Growth Potential

Chapter 4. Core Programs

Appendix



01 | Company Overview

1. Company Overview
2. History
3. Management Team
4. Scientific Advisory Board

1. Company Overview

Developing novel therapeutics based on proprietary RNAi platform technology

■ Status

Company Name	OliX Pharmaceuticals, Inc.
CEO & Founder	Dong Ki Lee
Date of Establishment	Feb. 26, 2010
Headquarters	Ace Gwanggyo Tower1, Suite 1014, 17, Daehak 4-ro, Yeongtong-gu, Suwon-si, Gyeonggi-do, 16226, Republic of Korea
Number of Employees	87 (62 in R&D, 16 in OliX US) (Doctorate holders: 29, Master's degree holders: 29)
Major Business	Development of next-generation nucleic acid therapeutics based on RNAi technology

■ Shareholders

(Dec. 2020)

Shareholder name	No. of shares (common stock)	Share (%)
Major shareholder etc.	4,002,514	29.56%
Hugel, Inc.	355,592	2.62%
Etc.	9,183,954	67.82%
Total	13,542,060	100.00%

■ Corporate Philosophy

Our Mission

Contribute to the Health and Happiness of Mankind
Using Cutting-Edge Technology

Our Vision

- Become one of the world's top three nucleic acid companies
- Become a world-class biomedical technology research center



■ Subsidiaries

OliX US Boston	Preclinical development & pharmacology, CMC, clinical, regulatory, QA
OliX US San Diego	RNAi research through chemical modification of siRNA & development of analytical method for siRNA
mCureX Therapeutics, Inc.	Development of nucleic acid therapeutics and vaccines based on mRNA technology

2. History

Establishing Platform Technology

- ▶ **2010**
 - 02 BMT, Inc. is established
 - 03 Exclusive license on asymmetric siRNA is obtained
 - 09 Certified as a venture company (Kibo Technology Fund)
- ▶ **2011**
 - 06 Exclusive license for asymmetric lasiRNA is obtained
 - 10 Company affiliated research center is certified
 - 12 cp-asiRNA platform development is initiated
- ▶ **2012**
 - 11 Company moves to Gasan Digital Complex
- ▶ **2013**
 - 05 A patent application for cp-asiRNA platform is filed
 - 11 Out-License Agreement for OLX101A with Hugel is made (Asia)**

Expanding Pipelines

- ▶ **2014**
 - 08 Series A funding is completed
 - 09 OLX201A is selected as 'Korea-Singapore R&D project' (Ministry of Health and Welfare)
 - 10 Renamed to OliX, Inc.
 - 11 OLX101A is selected as a 'KOREA Drug Development Fund' project (preclinical)
- ▶ **2015**
 - 04 OLX103 is selected as a Technology Development Support Project (SME Business Administration)
 - 06 Hugel Inc. makes a strategic investment in OliX**
- ▶ **2016**
 - 07 Hypertrophic scar therapeutics data published in Journal of Investigative Dermatology
 - 12 CEO receives the Minister of Health & Welfare Award

Entering into Clinical Stage

- ▶ **2017**
 - 01 Company moves to Suwon
 - 01 Clinical trials for OLX101A approved by MFDS
 - 05 Asymmetric lasiRNA platform is patented in the US
 - 10 OliX passes technology evaluation (A,A) for IPO
- ▶ **2018**
 - 05 Phase 1 trial for OLX101A is completed in Korea
 - 05 Phase 1 trial for OLX101A is approved in UK**
 - 07 Listed on the KOSDAQ**
 - 10 OLX101A is selected as a 'KOREA Drug Development Fund' project (IND for Phase 2, FDA)
 - 10 Established OliX US, Inc. in Cambridge, US**
 - 11 Phase 2 trial for OLX101A is approved in KOREA**

Initiating a Global Clinical Trial

- ▶ **2019**
 - 01 Established R&D Lab in San Diego
 - 03 Out-Licensing Agreement for OLX301A with Théa (EU, MEA, Africa)**
 - 11 Phase 1 trial for OLX101A is successfully completed in the UK**
- ▶ **2020**
 - 03 GalNAC Conjugation Technology from AM Chemicals is introduced
 - 06 R&D Supply Contract on GalNAC-siRNA platform is signed**
 - 10 Expanded Out-Licensing Agreement for OLX301A,301D, two optional pipeline with Théa (Worldwide excl. Asia-Pacific)**
 - 12 Certified as a Family-friendly and an Innovative pharmaceutical company**
- ▶ **2021**
 - 01 Established mCureX Therapeutics, Inc.**
 - 02 Received KNDA (Korea New Drug Award)**



- 2015** Cell-Penetrating asymmetric siRNA (cp-asiRNA) is patented
- 2013** Long Asymmetric siRNA (lasiRNA) is patented
- 2010** Asymmetric siRNA (asiRNA) structure technology is patented
- 2009** Paper on asymmetric siRNA structure technology is published (Molecular Therapy)
- 2004** Research on RNAi technology is conducted (POSTECH, Sungkyunkwan Univ.)

3. Management Team



In-house expertise covering from R&D, clinical trials to commercialization



Development
Shin Young Park
EVP

- Ph.D. in Pharmacy, Seoul Nat'l Univ.
- Preclinical toxicology expert
- Nonclinical toxicology project manager for Ionis, KIT
- Toxicologist, DABT



CEO
Dong Ki Lee

**Makes strategic decisions
for the overall R&D process**

- B.S. in Chemistry, KAIST
- Ph.D. in Biochemistry, Cornell University
- Assistant Professor, Chemistry Dept., POSTECH
- Professor, Chemistry Dept., Sungkyunkwan Univ.



Management
Chung Gil Kang
Vice President

- B.S. & M.S. in Management Science, KAIST
- Director, Kumho, Powerlogics
- Director, L&S Venture Capital



Chemistry
Dongwon Shin
Senior Director (OLIX US)

- Ph.D. in Organic Chemistry, University of California, Riverside
- Senior Staff Scientist, TriLink Biotechnologies, LLC
- Synthesis specialist



Research
Sun Woo Hong
Vice President

- Ph.D. in Chemistry, POSTECH
- Research Professor, Dongguk Univ.
- Improves platform technology & supervises research
- CEO of mCureX



Legal Affairs
Young Hye Baek
Senior Director

- B.S. & M.S. in Biology, KAIST
- Patent lawyer
- FirstLaw P.C.
- Y.P. Lee, Mock & Partners
- Legal Dept., LG Household & Health Care

4. Scientific Advisory Board



Scientific Advisory Board advising on R&D, clinical trial development, and technology commercialization.

■ Platform Technology



John Lis

Gene Expression, Oligotherapeutics

- Distinguished Professor, Cornell University
- Member, National Academy of Sciences

■ Hepatology



Yury V. Popov

Hepatology

- Assistant Professor of Medicine, Beth Israel Deaconess Medical Center, Harvard Medical School

■ Ophthalmology



Demetrios Vavvas

Ophthalmology (AMD, Retina, Diabetic, Glaucoma)

- Associate Professor of Ophthalmology at Harvard Medical School
- Monte J Wallace Ophthalmology Chair in Retina at Mass Eye & Ear Infirmary (MEEI)



Gordon Jiang

Hepatology

- Transplant hepatologist and physician-scientist at Beth Israel Deaconess Medical Center, Harvard Medical School



Hye-Won Chung

Ophthalmology (AMD, Retina, Retinitis pigmentosa)

- Associate Professor of Ophthalmology at Konkuk University
- Member, The Macular Society



Aaron Hakim

Hepatology

- Clinical Fellow in Medicine (EXT) Beth Israel Deaconess Medical Center, Harvard Medical School



02

Core Technology

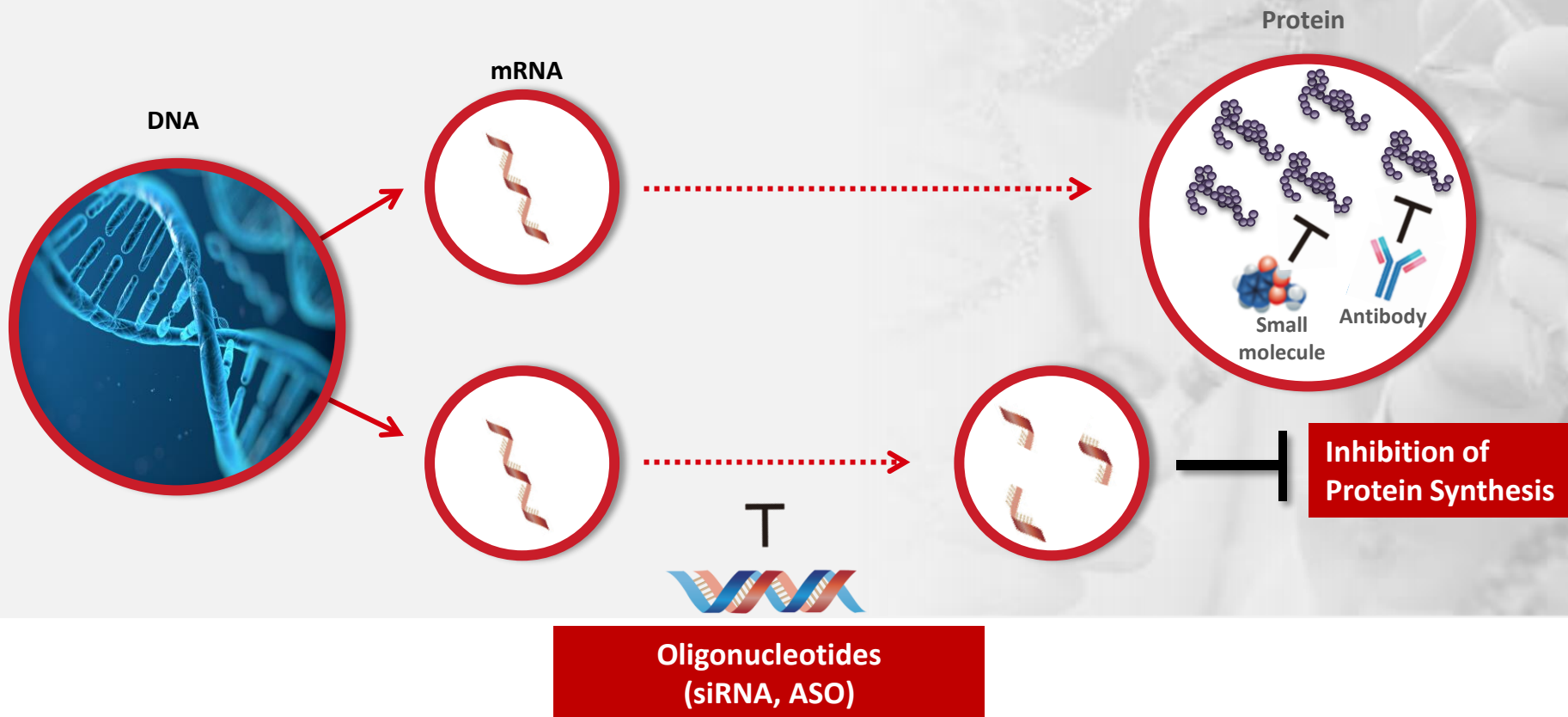
1. What is RNA interference (RNAi) ?
2. Limitations of Existing RNAi Technology
3. asiRNA
4. cp-asiRNA
5. GalNAc-asiRNA
6. Status of Global siRNA Deals

1. What is RNA interference (RNAi)?



Small molecule (1st generation) & antibody (2nd generation) drugs inhibit protein activities

Oligonucleotide (3rd generation) drugs inhibit protein synthesis



1. What is RNA interference (RNAi)?



RNAi therapeutics is the third-generation drug development platform that can efficiently target and theoretically silence all disease-causing genes

1st and 2nd Generation Drug Development Platform

85%
Undruggable Proteins¹

- ▶ Act on disease-related proteins that are already produced
- ▶ Unable to target the majority of cellular proteins due to their shape or location
- ▶ Average time needed to identify potential drug candidates: **3-5 years**

Note 1) Source: Pharmacol Ther. 2017 Jun;174:138-144

Undruggable Target: Disease sites undruggable with existing or new drugs

Note 2) mRNA (Messenger RNA): It is created using DNA as a template and produces a protein through translation based on sequencing.

3rd Drug Development Platform - Oligonucleotides

- ▶ Use oligonucleotides (such as chemically synthesized DNA or RNA) as drugs
- ▶ Act on mRNA² before protein synthesis
- ▶ Can target genes known to be “undruggable” by small molecules or antibodies
- ▶ One platform technology can quickly develop novel therapeutics against a variety of diseases (3-5 months)

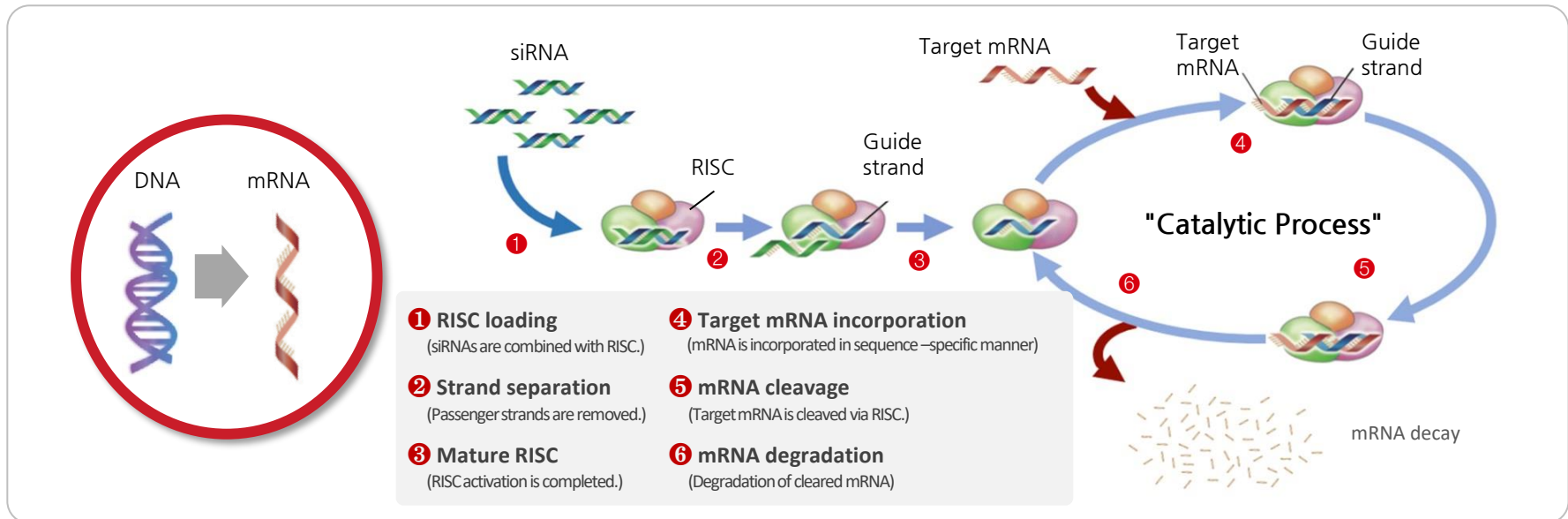
RNAi Technology

siRNA
(RNAi-triggering molecule)
(double-stranded small interfering RNA)

- ▶ Silence target genes more efficiently than other oligonucleotide technologies
- ▶ Develop RNAi-based therapeutics with patented delivery technology
- ▶ The FDA approves the first RNAi therapeutics in Aug. 2018

1. What is RNA interference (RNAi)?

RNA interference technology: The most powerful oligonucleotide technology



First RNA interference mechanism discovered in 1998
Wins a Nobel Prize in Physiology or Medicine in 2006



Opens the door to drug development for
“undruggable targets”



RNAi therapeutics
in market

(Alnylam | ONPATTRO, Aug. 2018, FDA)
(Alnylam | GIVLAARI, Nov. 2019, FDA)
(Alnylam | OXLUMO, Nov. 2020, FDA)
(Novartis | Leqvio, Dec. 2020 EMA)



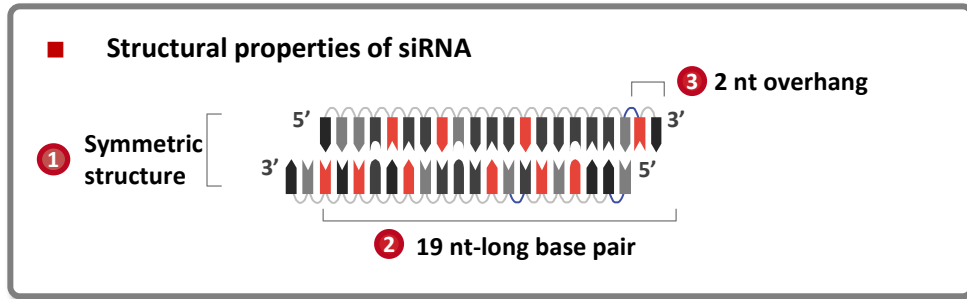
Rapid production of new siRNA
drug candidates is possible

Note 1) The RNA-induced silencing complex or RISC is a multiprotein complex which incorporates double-stranded small interfering RNA (siRNA) to recognize mRNA and cleaves the mRNA.

2. Limitations of Existing RNAi Technology



The structure of siRNA causes adverse events & delivery problems



Unintended gene repression

On target: 50%
Off target: 50%

- Passenger strand can be incorporated into RISC due to symmetric siRNA structure.
- Off-target gene silencing can occur.

Saturation of endogenous RNAi machinery

(Saturation of RNAi machinery)

- Endogenous RNAi machinery can be saturated when an excess amount of siRNA is introduced.
- Normal gene expression regulated by endogenous RNAi pathways can be compromised.

Retinal degeneration by Immune toxicity

Normal retinal tissue Degenerated retina

※ Source: Mol Ther. 2009 Apr;17(4):725-32 Mol Cells. 2011 Dec;32(6):543-8

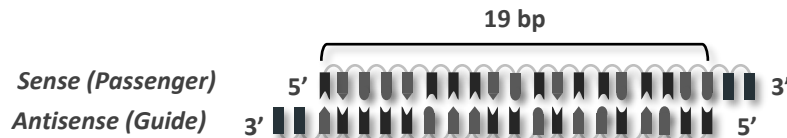
※ Source: Biochem Biophys Res Commun.'08 Feb 29;367(1):78-83 Biochem Biophys Res Commun.2010 Jul 16;398(1):92-7

※ Source: Nature. 2008 Apr 3;452(7187):591-7 Mol Ther. 2012 Jan;20(1):101-8

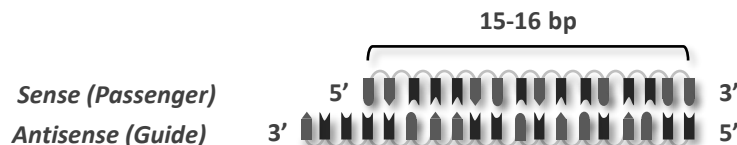
3. Asymmetric siRNA (asiRNA)

Asymmetric siRNA (asiRNA) is a unique gene silencing technology developed by Olix

Conventional siRNA



Olix's Asymmetric siRNA (asiRNA)



Secured 'Freedom to Operate'

Asymmetric siRNA patent registered

- "Novel siRNA structure for minimizing off-target effects and relaxing saturation of RNAi machinery and the use thereof" (Priority Claimed, 2007-12-18)
- Patent granted in Korea, China, Japan, Europe, Australia

original article

Asymmetric Shorter-duplex siRNA Structures Trigger Efficient Gene Silencing With Reduced Nonspecific Effects

Chan Il Chang¹, Jae Wook Yoo², Sun Woo Hong², Shi Eun Lee², Hy Harry A Rogoff¹, Changill Ban¹, Soyoun Kim¹, Chiang J Li³ and Dong-ki Lee^{1*}

in this issue

Efficient gene silencing with reduced nonspecific effects

Small interfering RNAs (siRNAs) are short, double-stranded RNAs that mediate gene silencing in human cell lines. In this issue, Chang *et al.* report an asymmetric siRNA (asiRNA) backbone structure with duplex regions shorter than 19 bp that can efficiently trigger gene silencing in human cell lines. This structure reduces off-target gene silencing triggered by conventional siRNA scaffolds, such as dilated off-target gene silencing of the RNAi pathway.

Selection and Optimization of Asymmetric siRNA Targeting the Human c-MET Gene

Seul-gi Jo^{1,3}, Sun-ki Lee^{1,3}, and Dong-ki Lee^{1,4}

Biochem J (2014) 461, 427–434 (Printed in Great Britain) doi:10.1042/BJ20140407

Effect of the guide strand 3'-end structure on the gene-silencing potency of asymmetric siRNA

Sun Woo HONG^{1*}, June Hyun PARK^{1*}, Soyeong YUN¹, Chang Han LEE¹, Chanseok SHIN^{1,2} and Dong-ki LEE^{1,2}

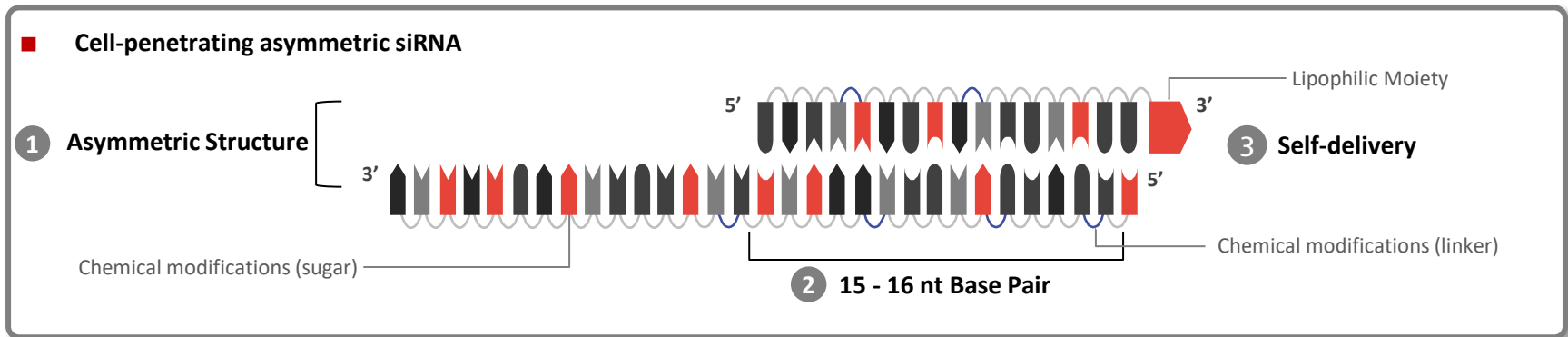
¹Global Research Laboratory for RNAi Medicine, Department of Chemistry, Sungkyunkwan University, Suwon 440-746, Republic of Korea
²Department of Agricultural Biotechnology, Seoul National University, Seoul 151-921, Republic of Korea
³Plant Genomics and Breeding Institute, Seoul National University, Seoul 151-921, Republic of Korea

427

4. Cell-penetrating Asymmetric siRNA (cp-asiRNA)



Solves delivery problems and reduces adverse events of existing siRNA technology



Asymmetric, short base pair (15-16nt)

Reduces the side effects of conventional siRNA

Self-delivering

Eliminates the risk of toxicity caused by delivery vehicles

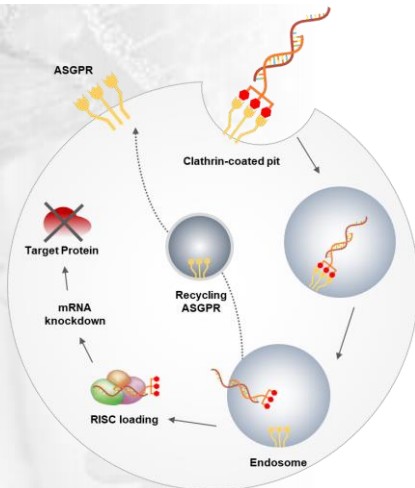
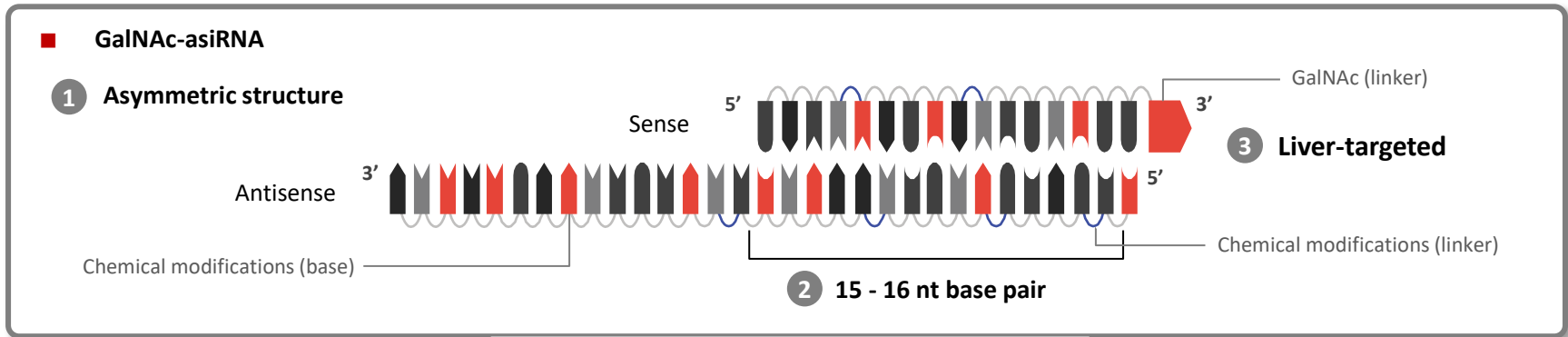
Simple chemical modification

Amenable to large-scale synthesis and analysis

5. GalNAc-asiRNA



Secured asymmetric GalNAc platform for liver-targeted delivery



1. Specifically & effectively targeting liver hepatocytes

2. Long duration of action

3. Patient-friendly via subcutaneous injection

4. Clinically validated technology

6. Status of Global siRNA Deals

Licensing deals driving the RNAi industry

[Major licensing deals from global RNA therapeutics companies for the past four years]

Licensor	Licensee	Year	Deal Size (USD)	License Target	Stage
Olix	Théa	2020 /2019	0.81B	Ophthalmic Diseases (4 targets)	R&D
	Undisclosed	2020	Single-digit m research funding ► Mega deal	Liver Diseases (GalNAc)	Discovery and R&D
Alnylam	Regeneron	2019	>1B	Ophthalmic, CNS Diseases	R&D
	Sanofi	2018	>1B	Hemophilia	Phase 2
	Vir	2017	>1B	HBV, Infectious Diseases	Phase 2
Dicerna	Roche	2019	>1.7B	HBV	Phase 1
	Novo Nordisk	2019	0.36B/target ► Mega deal	Liver Disease, NASH, etc. (30 targets)	R&D
	Eli Lilly	2018	0.35B/target ► Mega deal	Cardiometabolic Disease, etc. (10 targets)	Phase 1
	Alexion	2018	>0.64B	Complement-mediated Diseases	R&D
	Boehringer Ingelheim	2017	>0.2B	NASH	R&D
Arrowhead	Takeda	2020	>1B	Liver Disease (AATLD)	Phase 2
	Janssen	2018	>3.7B	HBV	Phase 1
Silence	Takeda	2020	Single-digit m research funding ► Mega deal	Undisclosed	Discovery and R&D
	AstraZeneca	2020	>4.2B	Cardiovascular, Metabolic Diseases, etc.	R&D
	Mallinckrodt	2019	>2.1B	Complement-mediated Diseases	Phase 1



03

Growth Potential

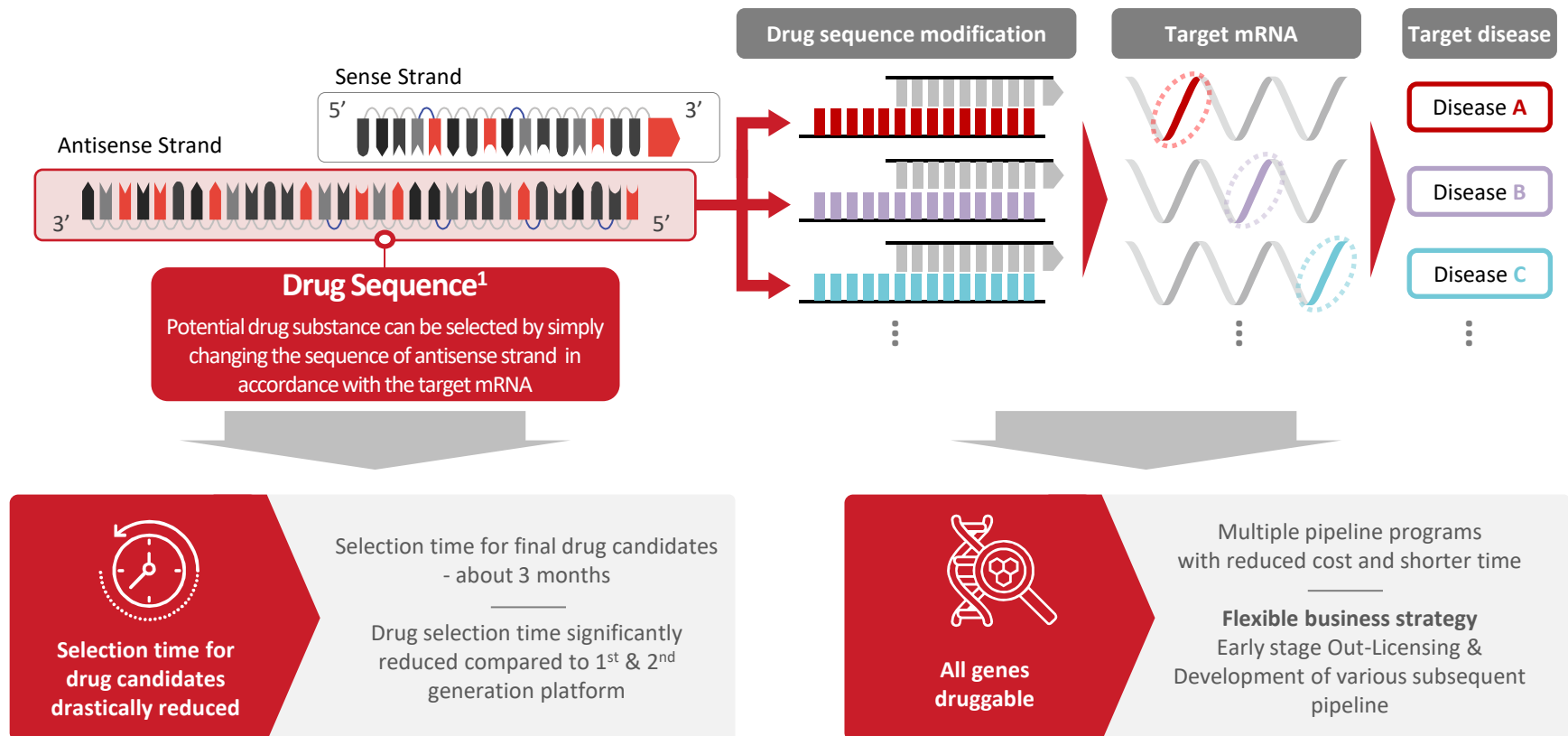
1. Generality and Expandability of OliX Platform
2. Development Strategy

1. Generality and Expandability of OliX Platform



Rapid selection of drug candidates based on OliX platform technology
Theoretically, all genes can be inhibited at high efficiency


















■ Expandability of the cp-asiRNA platform



Note 1) Drug sequence: binds the complementary sequence of the target mRNA to induce degradation of the target. (= Antisense RNA, Guide strand)

2. Development Strategy

RNAi-based therapeutics targeting incurable diseases

Indication Area	Program	Indication	R&D	Animal POC	Preclinical	Clinical	Remarks
 SKIN	OLX101A	Hypertrophic Scar					Hugel (Asia)
	OLX104C	Androgenic Alopecia					(Korea) Currently in Phase 2 (US) Currently in Phase 2
 EYE	OLX301A	Dry & Wet AMD					Théa (Worldwide Excl. Asia-Pacific)
	OLX301D	Subretinal Fibrosis					Théa (Worldwide Excl. Asia-Pacific)
	OLX301E	Wet AMD					
	OLX304A	Retinitis Pigmentosa					
 LIVER	OLX701	Liver Fibrosis					
	OLX702	Liver Disease (NASH, Diabetes etc.)					
	OLX703	HBV					
 LUNG	OLX201A	Idiopathic Pulmonary Fibrosis					
	OLX204A	COVID-19					
CNS & Oncology	OLX401A	Neuropathic Pain					
	OLX801A	Cancer Immunotherapy					



04 | Core Programs

1. Hypertrophic Scar (OLX101A)
2. Androgenic Alopecia (OLX104C)
3. Age-related Macular Degeneration (AMD)
 - Wet & Dry AMD (OLX301A)
 - Subretinal Fibrosis & Wet AMD (OLX301D)
4. Respiratory Diseases (OLX20X)
5. Liver Diseases (OLX70X)
6. License and Collaboration Status

1-1. OLX101A: Hypertrophic Scar



High rate of hypertrophic scar formation due to surgery or accident
 - Unmet medical needs due to limitations of existing therapy

What is hypertrophic scar?



Hypertrophic Scar

- Skin abnormalities that are characterized by excessive deposition of collagen in the dermis after surgery or injury
- Caused mainly due to the imbalance of synthesis and degradation of collagen
- Occurs in 39-68% of patients after surgery



Keloid Scar

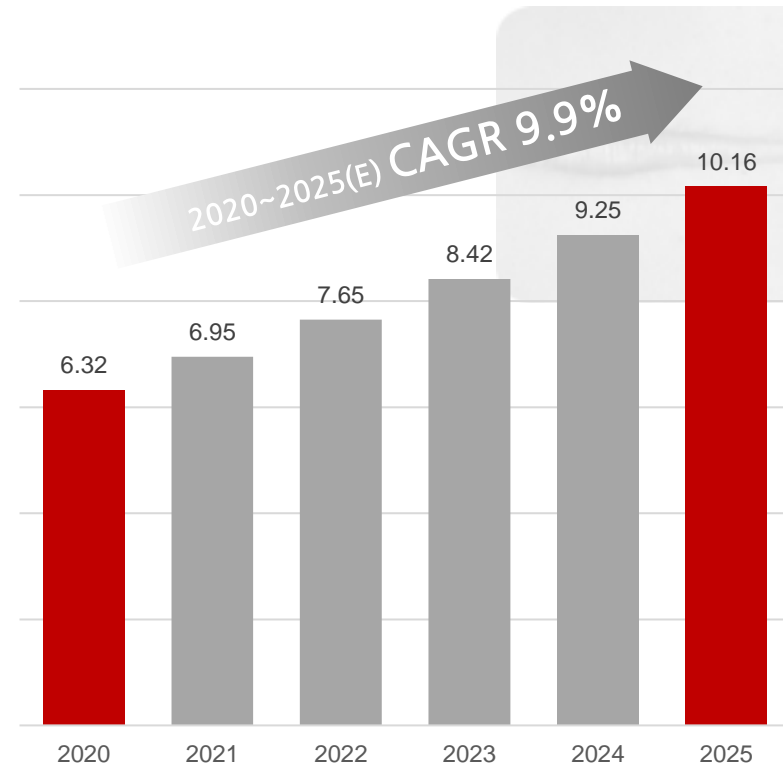
- Abnormal proliferation of scar tissue
- Caused mainly due to the imbalance of synthesis and degradation of collagen

Limitations of current treatment

Existing drugs	Limitations and Unmet Needs
Silicone sheets	<ul style="list-style-type: none"> • Unclear efficacy and compliance issue • Long-term treatment (6 months to 1 year) necessary
Physical compression therapy	<ul style="list-style-type: none"> • Unclear mechanism of action and efficacy • Long-term treatment (6 months to 1 year) necessary
Steroid injection	<ul style="list-style-type: none"> • High risk of recurrence (9~50%), risk of whole-body toxicity
Surgery	<ul style="list-style-type: none"> • High risk of recurrence (>50%), accompanied by pain

Prospect for global hypertrophic / keloid scar market

(USD Billion)



※ Source: Grand View Research 2020

1-2. OLX101A: Anti-fibrotic Effect in Clinical Studies



Effective anti-fibrotic activity through OliX RNAi platform acting on a validated target gene

■ Overview

Target protein	Administration route	Status
CTGF ¹⁾	Intradermal injection	Phase 2 clinical trial in progress (Korea) Phase 2 clinical trial in progress (US)

Selection criteria

► Patients with hypertrophic scars from surgery such as C-section or plastic surgery, or trauma

Note 1) Connective Tissue Growth Factor (CTGF): A major factor that promotes development of fibrosis

■ Phase 2 in Korea (Hugel)

Summary of Design

- Sponsor: Hugel Inc.
- Design: Independent Evaluator-Blind, Dose-Escalation, Untreated -Controlled, Within-Subject, Phase 2a Therapeutic Exploratory Clinical Trial
- Purpose: Effectiveness and safety
- No. of participants: 30
- Status: Technology out-licensed to Hugel

■ Development Status

- Verified effective fibrosis suppression in animal models
- Published in the Journal of Investigative Dermatology
- Nonclinical study and Phase 1 clinical trial in the UK: Supported by 'KOREA Drug Development Fund' Project
- Phase 2 clinical trial in progress

■ Phase 2 in US (oliX)

Summary of Design

- Sponsor: OliX Pharmaceuticals, Inc.
- Design: Prospective, Randomized, Double-blind, Intra-subject, Placebo-controlled, Proof of Concept Study
- Purpose: Preliminary efficacy and safety
- No. of participants: 20~30
- Status: Phase 2 clinical trial in progress

2. OLX104C: Androgenic Alopecia (AGA)



Novel hair loss treatment minimizing side effects with local administration

Overview

Target protein	Administration Route	Status
AR	Intradermal Injection	Pharmacology Study

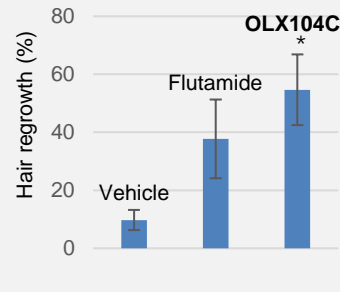
- ▶ Global Market Forecast ¹⁾ : 8.4B USD (2018) → 13.6B USD (2027), CAGR 5.51%
- ▶ For patients suffering from side effects due to systemic drug therapy
- ▶ Minimizes medical risks for female AGA patients
- ▶ Alleviates inconvenience caused by frequent administration

Nonclinical Pharmacology Data

Development Status

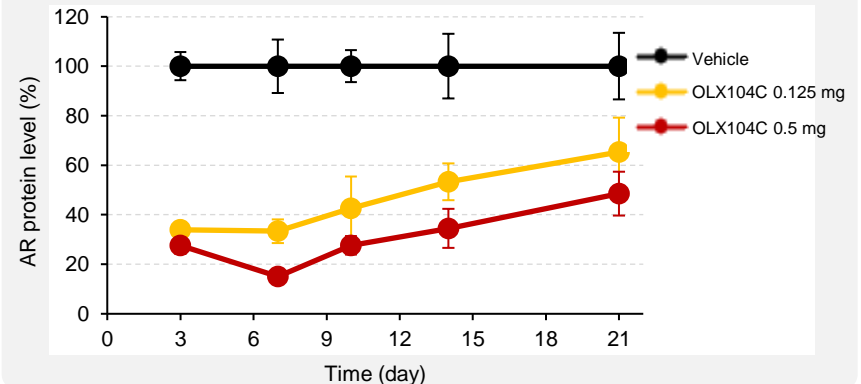
- Verified hair regrowth in alopecia mouse model
- Verified inhibition of telogen transition in AGA patient's hair follicle (ex vivo)
- Three-week duration of AR knock-down efficacy with a single injection
→ Solution to unmet needs for systemic side effects and inconvenience of daily administration
- Plan to enter clinical trial by 2022

Hair regrowth in alopecia mouse model



Vehicle Flutamide (200 mpk) OLX104C (0.125 mg)

Duration of AR knock-down with single injection



¹⁾ Inkwood Research (2019), End-use and Sales Channel Outlook (Homecare, clinics, prescriptions, OTC, etc)

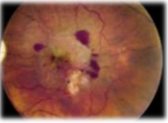
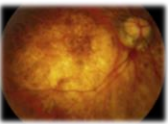
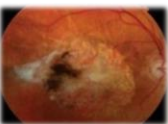
3-1. OLX301A/D: Age-related Macular Degeneration



No treatment available for subretinal fibrosis and dry AMD (GA) → high unmet medical needs

What is Age-related Macular Degeneration?

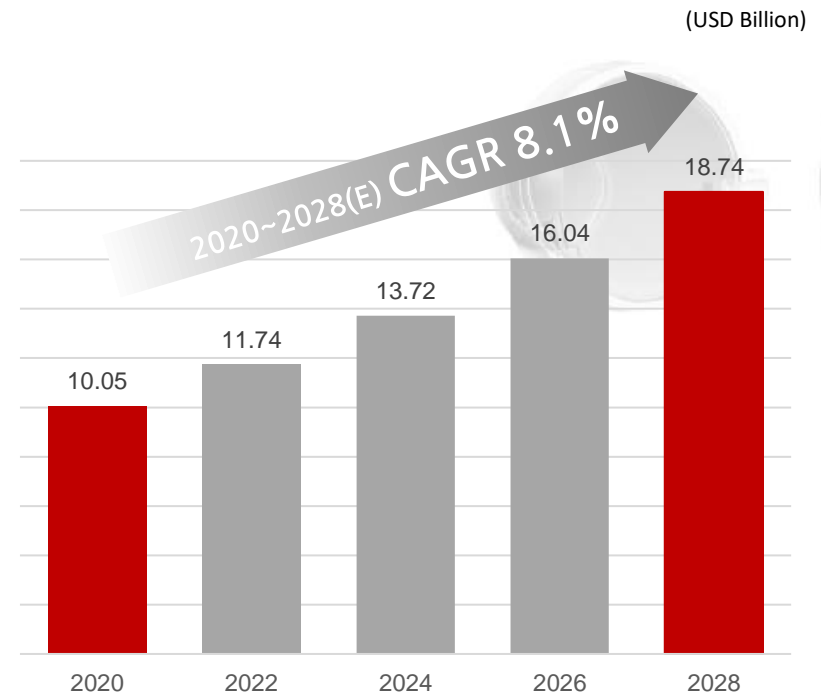
- ▶ Leading cause of severe vision loss in elderly persons (> age 50)
- ▶ One-third of cases of untreatable vision loss

	<p>Wet AMD</p> <ul style="list-style-type: none"> • 66% of late-stage AMD patients • Can lead to dry AMD / Subretinal Fibrosis
	<p>Dry AMD (GA)</p> <ul style="list-style-type: none"> • 34% of late-stage AMD patients • 15% of the patients develop wet AMD
	<p>Subretinal Fibrosis</p> <ul style="list-style-type: none"> • An excessive wound healing response to CNV in wet AMD • Can destroy the eye morphology, leading to permanent dysfunction of the macular visual system.

Limitations of current treatment

Type	Existing drugs	Limitations and Unmet Needs
Wet AMD	Eylea	<ul style="list-style-type: none"> • More than 30% of AMD patients have poor or no response to the treatment. • Develops advanced macular scarring and subretinal fibrosis within 2 years. • Ineffective to subretinal fibrosis and dry AMD
	Lucentis	
	Beovu	
Dry AMD (GA)	No treatment	<div style="background-color: #e91e63; color: white; padding: 5px; text-align: center; border-radius: 10px;">Unmet Medical Needs</div>
Subretinal fibrosis		

Prospects for the global AMD drug market



Full Year 2020 Product Sales (Net Sales):
 Lucentis: \$1,933m + Eylea: \$7,908m = \$9,841m

※ Source : GlobalData 2020

3-2. OLX301A: Efficacy in Animal Model



First-in-class drug for both wet and dry AMD

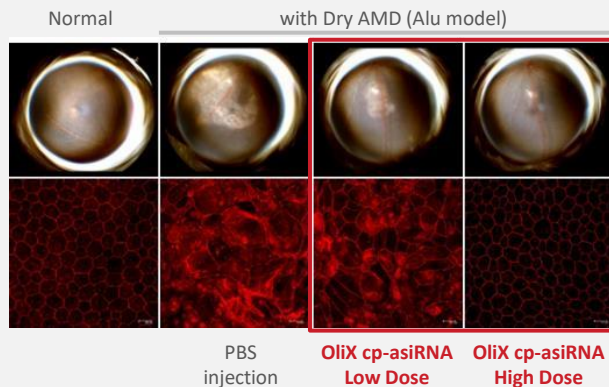
Overview

Target protein	Administration Route	Status
Undisclosed	Intravitreal Injection	Nonclinical Tox Study

- ▶ Special target indication: Geographic atrophy (GA), late-stage of dry AMD
- ▶ Available for the VEGF therapy-resistant wet AMD patients
- ▶ A potential first-in-class drug that can treat patients with both wet and dry AMD

Efficacy in animal models

Effective in Dry AMD (GA) mouse

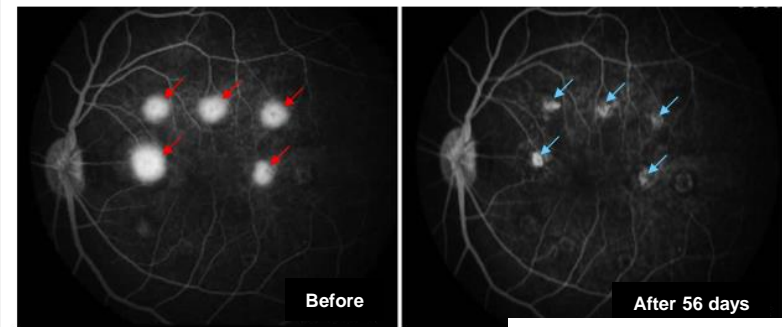


Development Status

- A novel therapeutic for advanced dry AMD (GA)
- Undruggable target gene discovered by Prof. J Ambati and his team: first-in-class
- Excellent effectiveness verified in multiple animal models with wet AMD (CNV) and dry AMD (GA)
→ Works on both wet AMD and dry AMD (GA)
- Nonclinical toxicity study in progress with global CRO

Effective in Wet AMD NHP

CNV(Choroidal Neovascularization) Model, Fundus Fluorescein Angiography (FFA)



→Fluorescein leakage from CNV

3-3. OLiX301D: Efficacy in Animal Model



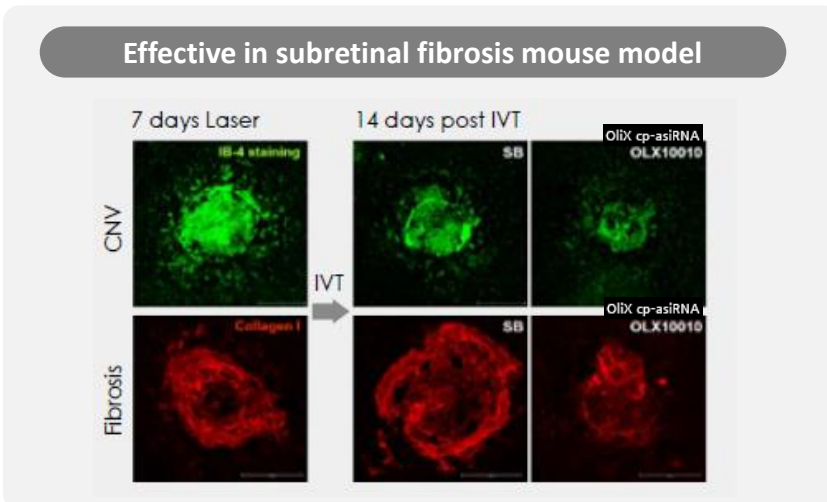
First-in-class drug for both subretinal fibrosis and wet AMD

Overview

Target protein	Administration Route	Status
CTGF	Intravitreal Injection	Nonclinical Tox Study

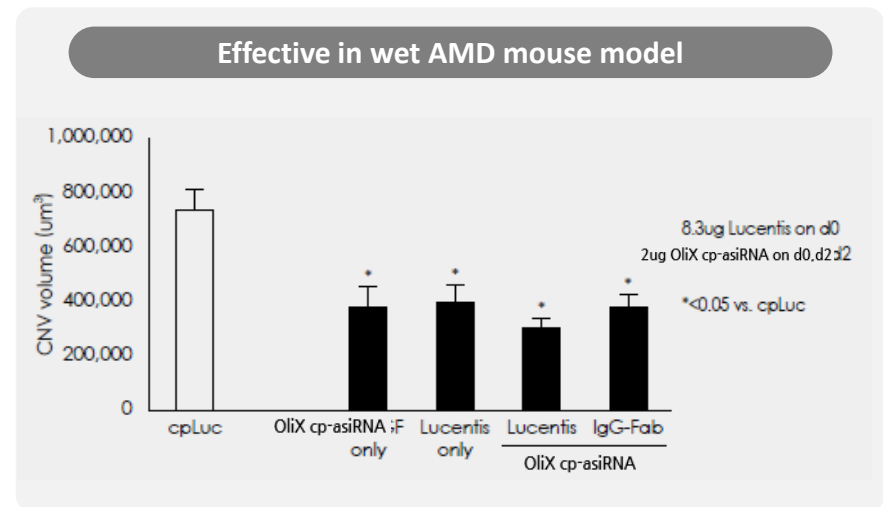
- ▶ More than 60% of patients have poor or no response to the standard anti-VEGF therapy and can develop subretinal fibrosis within 2 year-treatment, leading to vision loss
- ▶ A potential first-in-class drug which can treat patients with both wet AMD and subretinal fibrosis

Efficacy in animal model



Development Status

- Verified effectiveness in animal models with subretinal fibrosis
- Verified effectiveness in animal models with wet AMD
- Signed contract for active pharmaceutical ingredient (API) production for nonclinical and clinical trials: LGC Biosearch Technologies
- Nonclinical toxicity study in progress with global CRO



3-4. OLX301A/D: License and Collaboration Agreement

License & Collaboration Agreement signed in 2019, followed by an Expanded Agreement in Oct. 2020



Agreement Overview

1	OLX301A	Expanded territory from earlier agreement (EU, Middle East, Africa) → Worldwide patent excl. Asia-Pacific)
2	OLX301D	Out-license agreement signed (Worldwide patent excl. Asia-Pacific)
3	Two ophthalmic pipeline programs	Upon exercise of option w/i 2 years (Same term as OLX301A/D)

Total Volume : Undisclosed

Upfront	€8,800,000
Milestone	Undisclosed
Royalty	Undisclosed
Territory	Worldwide excluding Asia-Pacific

4. OLX20X: Respiratory Diseases



Expanding respiratory pipeline using OliX cp-asiRNA platform technology

Overview

Active pharmaceutical ingredient (API)	Indication	Administration Route	Status
cp-asiRNA / Target undisclosed	Respiratory diseases including IPF and COVID-19	Inhalation	Looking for global partners

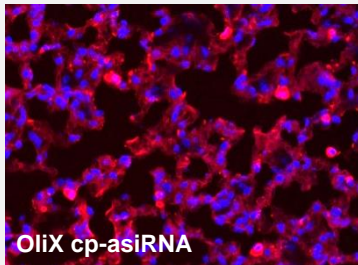
Development Status

- Animal proof of concept (POC) studies completed
- Established cp-asiRNA platform with improved efficacy and safety
- Pipeline development and drug candidate screening in progress

Nonclinical Study Results

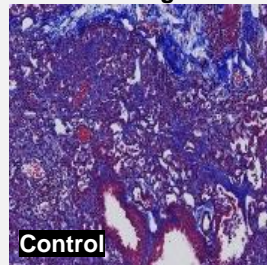
Delivery to mouse lungs

Broad distribution of siRNA



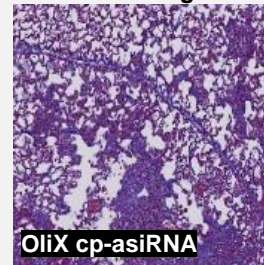
Efficacy in lung fibrosis mouse model

Fibrotic lung tissue



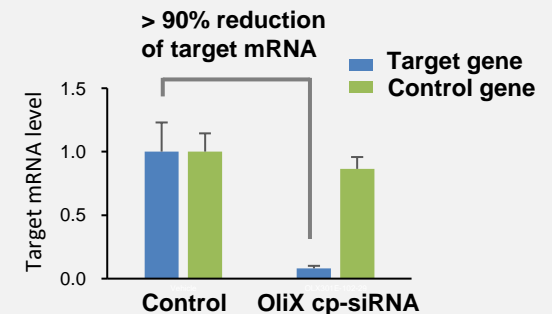
Control

Recovered lung tissue



OliX cp-asiRNA

Target knockdown in mouse lungs



5-1. OLX70X: Liver Diseases



Expanding liver pipeline using OliX GalNAc platform technology

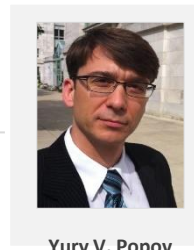
■ Overview

Active pharmaceutical ingredient (API)	Indication	Administration Route	Status
GalNAc-asiRNA / Target undisclosed	Undisclosed	Subcutaneous Injection	Looking for global partners

■ Development Status

- Developed OliX's proprietary GalNAc platform technology
- Lead compound discovery is ongoing for targets suggested by liver disease experts
- Nonclinical studies planned in 2021

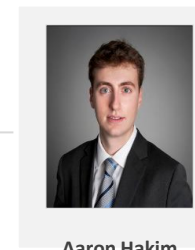
■ Scientific Advisory Board (Hepatology Experts)



Yury V. Popov



Gordon Jiang



Aaron Hakim

5-2. Effective Delivery of GalNAc-asiRNA



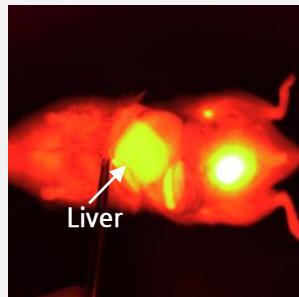
■ Nonclinical Delivery Test Results

- 10mg/kg of GalNAc-asiRNA subcutaneously injected into mouse model

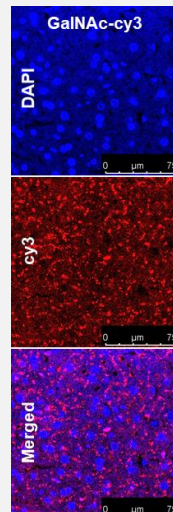
(A) Whole body, (B) Liver tissue distribution verified

(C) Effective hepatic delivery of Olix GalNAc-asiRNA both *in vitro* and *in vivo*

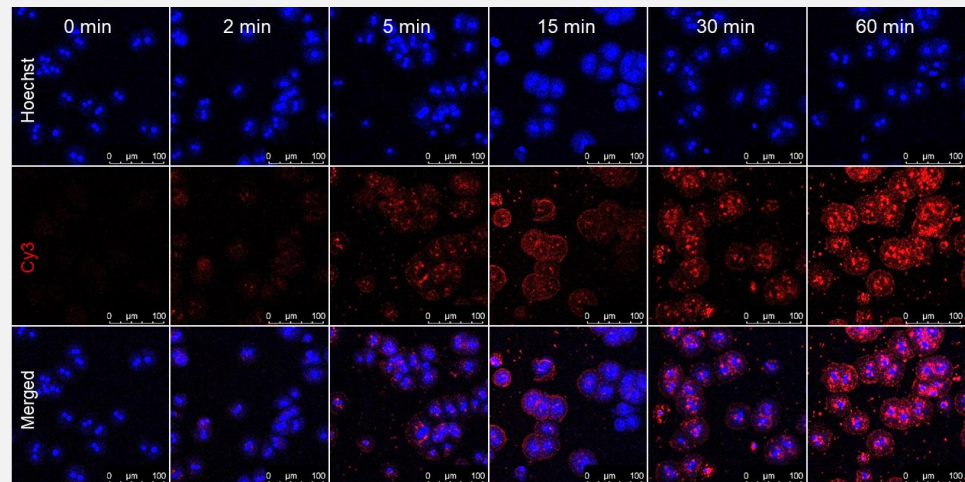
(A) Whole Body Distribution



(B) Liver Distribution

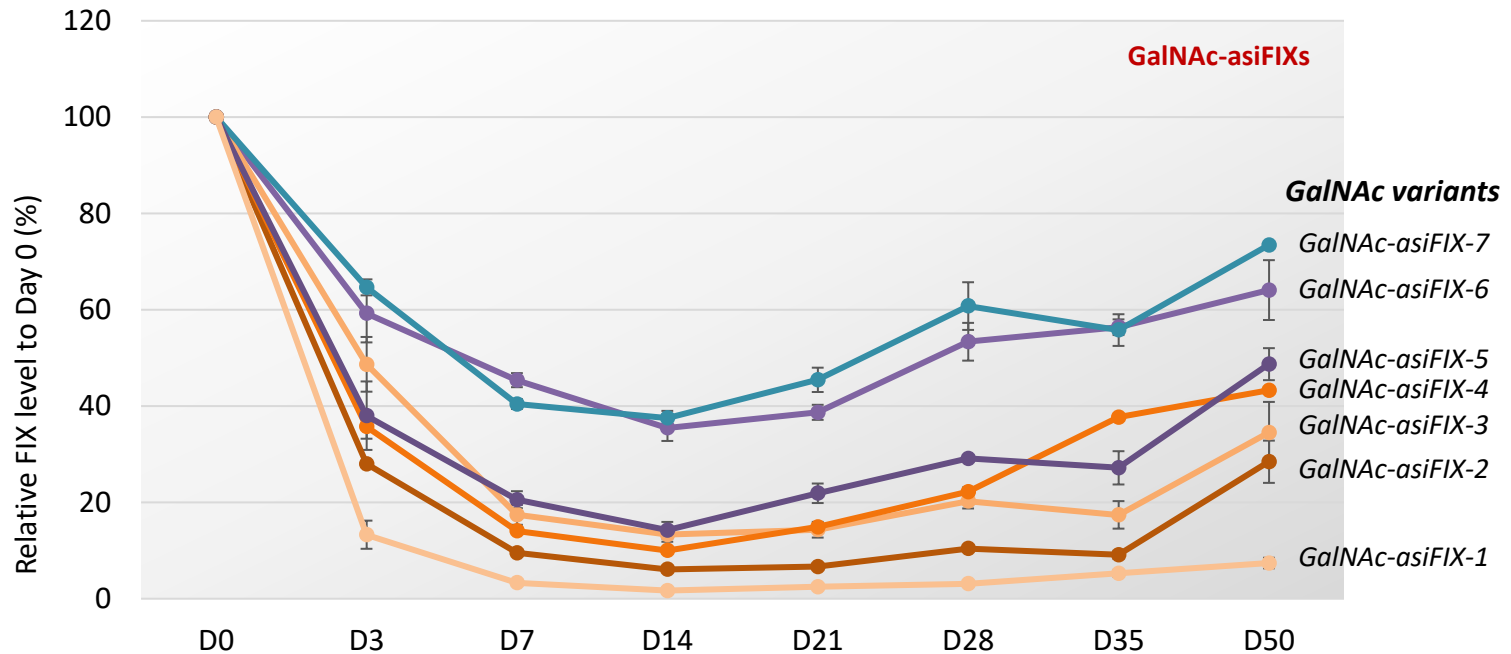


(C) Cellular uptake in mouse hepatocytes



5-3. Efficacy and Duration of Action

Competitive Potency and Duration of Action



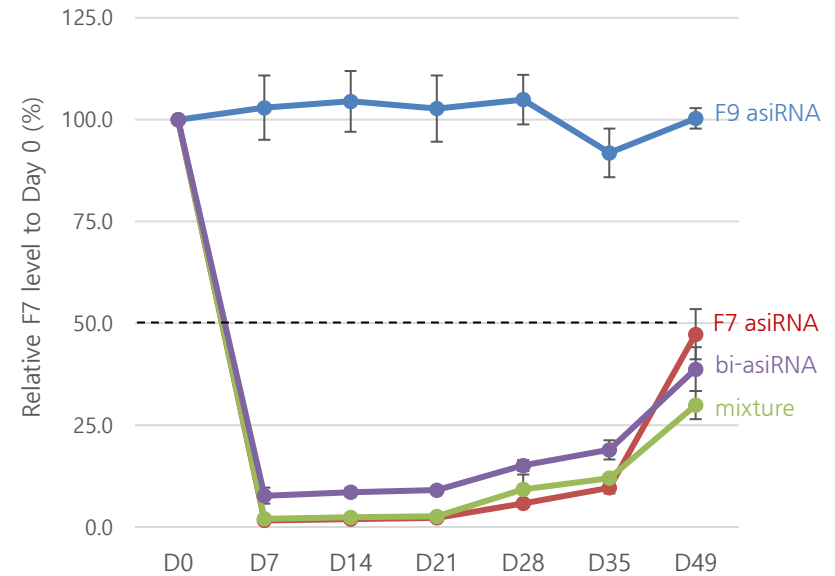
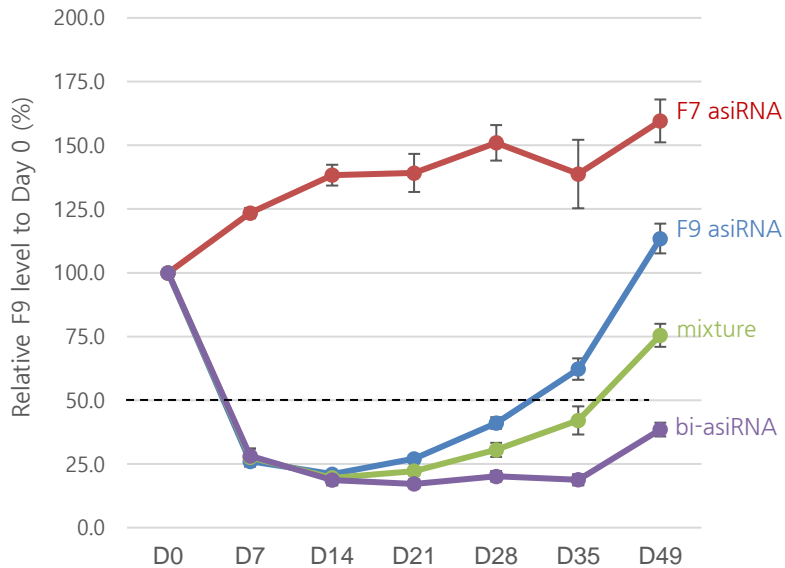
Development Status

- Chemical modification optimized for efficacy and metabolic stability
- Potent and durable silencing (>90%) achieved after a single dose (up to 50 days)
- Comparable to competitors' GalNac platforms

5-4. Dual Targeting GalNAc Platform



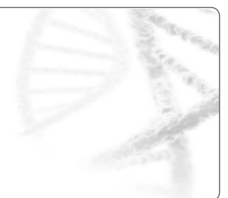
Developed GalNAc platform targeting two genes simultaneously



*bi-asiRNA : Dual targeting GalNAc-asiRNA by linking F7-F9 asiRNA with linker

■ Development Status

- Optimized chemistry for stabilization & developed bi-asiRNA for linking two asiRNAs
- Confirmed comparable efficacy to the mixture of two GalNAc substances



5-5. Liver Programs Development Status



Indication	Program	Stage of the Program		
		Discovery	Preclinical	Clinical
Liver Fibrosis	OLX701C			
	OLX701D			
	OLX702A			
Obesity / Type 2 Diabetes	OLX702B			
	OLX702C			
	OLX702D			
Nonalcoholic Steatohepatitis (NASH)	OLX702E			
	OLX702F			
	OLX702G			
	OLX702H			
Hepatitis B (HBV)	OLX703A			

5-6. GalNAc-siRNA R&D Supply Contract



First in Asia to sign GalNAc-siRNA platform R&D supply contract (2020.06.24)

(Confidential upon the
counterparty's request)



A Biotech Company Headquartered in Europe

OliX will apply GalNAc-siRNA platform technology to test and develop lead candidates for 4 different liver targets suggested by the contracting party.

* In March 2020, OliX obtained an exclusive license on GalNAc conjugation technology from AM Chemicals, located in San Diego.

Total Volume: USD 1,500,000

Contracting Party	Biotech Company in Europe
Subject	GalNAc-siRNA: R&D Supply Contract
Contract Period	2020.06.24 – 2021.06.23
Sales / Supply Method	In-house Production

6. License and Collaboration Status

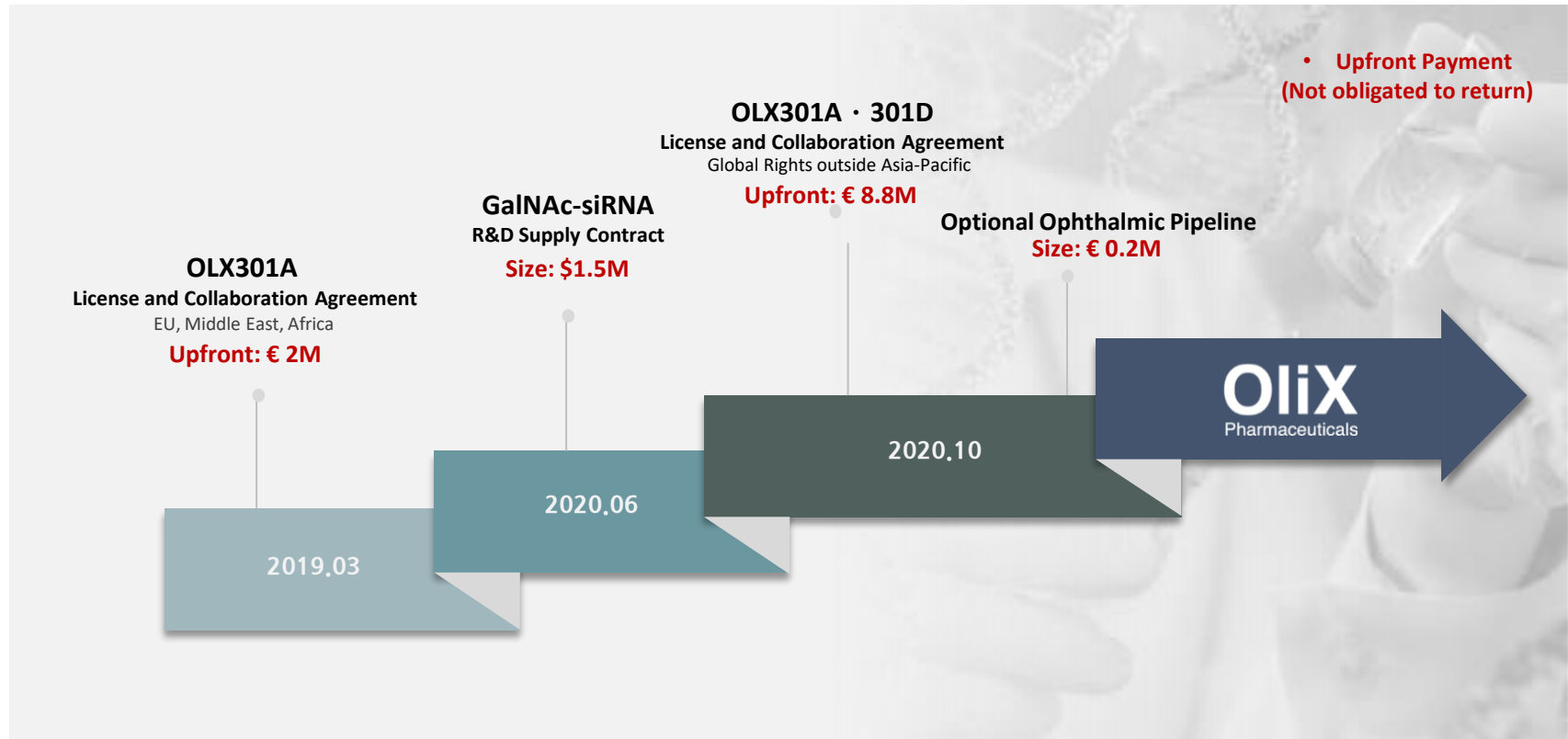


Flexible collaboration & partnership structure

(1) Partnership on **OliX's internal programs**

(2) **Providing OliX's RNAi platform** to identify siRNA lead compounds against genes of interest nominated by partnering company

■ License and Collaboration Agreement Status (For the past 2 years)





Appendix

1. Summary of Financial Statements
2. Subsidiary Overview
3. Global RNA Therapeutics Development Status

1. Summary of Financial Statements

■ Balance Sheet Summary

(Unit: million won)

Type	2018	2019	2020
Current assets	48,698	37,440	69,047
Fixed assets	6,939	7,767	9,530
Total assets	55,637	45,207	78,557
Current liabilities	314	2,754	4,488
Long-term liabilities	385	645	28,738
Total liabilities	699	3,399	33,226
Paid-in capital	3,252	3,265	6,771
Additional paid-in capital	71,168	71,540	81,099
Other capital components	766	1,801	11,859
Retained earnings	(20,248)	(34,799)	(54,379)
Total shareholders' equity	54,938	41,807	45,350

■ Income Statement Summary

(Unit: million won)

Type	2018	2019	2020
Operating income	302	1,130	2,474
Operating expense	8,536	16,247	18,715
Operating profit	(8,234)	(15,117)	(16,241)
Before tax Net profit	(7,742)	(14,244)	(20,511)
Tax expense	-	109	(1,126)
Net income	(7,742)	(14,353)	(19,385)

2-1. Subsidiary Overview (mCureX Therapeutics, Inc)

■ Status

Company Name	mCureX Therapeutics, Inc.
CEO	Sun Woo Hong
Date of Establishment	Jan. 20, 2021
Headquarters	6F, 225-15, Pangyoyeok-ro, Bundang-gu, Seongnam-si, Gyeonggi-do, Republic of Korea
Major Business	Development of nucleic acid therapeutics and vaccines based on mRNA technology

■ CEO



Sun Woo Hong Ph.D.

- 2021 - Present, CEO of mCureX
- 2010 - Present, Head of R&D, OliX
- 2010 - 2013, Research Professor, Dongguk Univ.
- 2005 - 2008, Ph.D. in Chemistry, POSTECH

■ Key Personnel



Dongwon Shin | Head of Research

- Ph.D. in Organic Chemistry, University of California, Riverside
- Postdoc, University of California, San Diego
- Senior Staff Scientist, TriLink Biotechnologies, LLC
- Director of Chemistry, OliX US
- Developed 5'-Capping technology used in COVID-19 mRNA vaccines

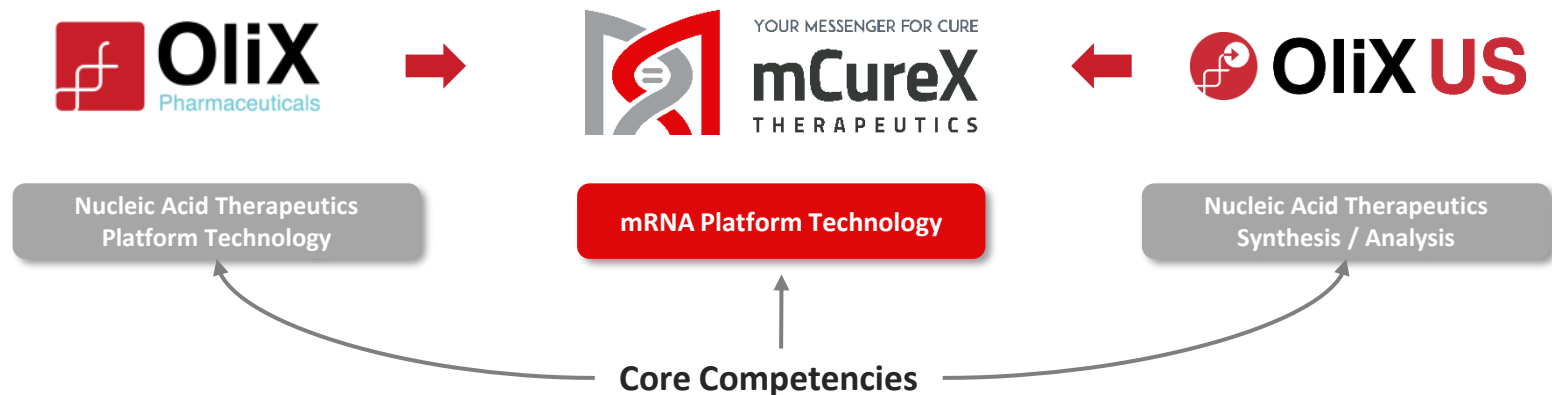


Anton McCaffrey | Scientific Advisory Board

- Ph.D. in Biochemistry, University of Colorado Boulder
- Postdoc, Stanford University School of Medicine
- Assistant Professor, University of Iowa
- Senior Director, R&D Biology, TriLink Biotechnologies, LLC
- 30 years of experience in nucleic acid therapeutics (mRNA, mRNA vaccines, RNAi, antisense, etc)

“mRNA + Cure + X[Acce]lerating” mCureX

- Based on messenger RNA (mRNA) technology
- Rapid development of vaccines and therapeutics **using proprietary mRNA platform technology**
- **In full cooperation with** Olix and Olix US for research and development



2-2. mRNA Technology



mRNA Therapeutics Technology

- Technology for ‘**Next Generation Therapeutics**’
- Produce disease-related proteins through in-vivo mechanism
- All proteins and antigens (vaccines) can be produced via mRNA administration



Chemically synthesized mRNA for vaccines/therapeutics



Rapid identification of drug candidate (3~5 months)
Amenable to large-scale synthesis

Can target various diseases with a single platform



Can produce all proteins with known amino acid sequence



First mRNA vaccine approved in 2020 (EUA)

Pfizer/BioNTech | BNT162b2 | Dec. 2020 by FDA
Moderna | mRNA-1273 | Dec. 2020 by FDA



Specifically induce production of disease-related proteins



Cooperate with companies with IP and intracellular delivery technology

2-3. mRNA Vaccine Development

mCureX-Samyang sign MOU for COVID19 Vaccine Development (2021.04.21)

 samyang



mCureX
THERAPEUTICS

mCureX-Samyang to co-develop mRNA vaccine

In April 2021, mCureX signed a memorandum of understanding (MOU) with Samyang Holdings Biopharm to develop a **local mRNA COVID19 vaccine** with **excellent efficacy and shelf life**.

With mCureX's proprietary mRNA technology and Samyang's delivery technology (DDS), rapid **development of vaccine** and **solutions to potential virus variants** are expected.



▲ Sun Woo Hong (CEO) of mCureX and Hye-Ryeon Jo (Director of Biopharmaceuticals R&D Center) of Samyang Holdings

3. Global RNA Therapeutics Development Status



Global RNA therapeutics development for various incurable diseases

[Representative programs from global RNA therapeutics companies]

Company	Product/Pipeline	Stage	Target
Alnylam	Patisiran (ONPATTRO)	Commercial	Hereditary ATTR Amyloidosis
	Givosiran (GIVLAARI)	Commercial	Acute Hepatic Porphyria
	Lumasiran (OXLUMO)	Commercial	Primary Hyperoxaluria Type 1
	Inclisiran (Leqvio)	Commercial	Hypercholesterolemia
	Fitusiran	Late Stage	Hemophilia & Rare Bleeding Disorders
	Vutrisiran	Late Stage	ATTR Amyloidosis
Arrowhead	ARO-AAT	Late Stage	Alpha-1 Liver Disease
	JNJ-1989	Late Stage	HBV
	AMG 890	Late Stage	Cardiovascular Disease
	ARO-ENaC	Early Stage	Cystic Fibrosis
Dicerna	Nedosiran	Late Stage	Primary Hyperoxaluria
	RG6346	Early Stage	HBV
	Belcesiran (DCR-A1AT)	Early Stage	AAT Liver Disease
Silence	SLN360	Early Stage	Cardiovascular Disease
	SLN124	Early Stage	Beta Thalassemia / Myelodysplastic Syndrome

Early Stage : IND or phase 2 | **Late Stage** : Phase 2b – Phase 3 | **Approved** : Phase 3 Completed