**SPE**
Summit Pharmaceuticals Europe

# Modified phosphoramidites

## Business model and track records

### Collaborative ASO drug discovery

**SERVIER**     **Sumitomo Pharma**
Innovation today, healthier tomorrows     **And so on...**

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### Licensing of XNAs technology

**Takeda**     **ALIGOS**
THERAPEUTICS

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### Own drug development, go for asset license
**ALS / triplet repeat disease**
**Spinal cord injury**
**Ophthalmological disease**

**S P E**
Summit Pharmaceuticals Europe

# Contribution to your ASO drug development

## 1.
## Licensing of XNAs technology on a gene-by-gene basis

You have in-house resources for oligonucleotide drug development, but there are issues that you cannot solve on your own...

**Luxna offers XNAs technology out-licensing opportunity**,start withanMTA forevaluationoranexclusive technology licensing agreement.

## 2.
## Collaborative drug discovery

You have good candidate antisense but you need better tox and efficacy profile...

**Collaborative research for "Lead Optimization".**

You have good target gene of genetic disorder or other specific overexpression for treatment...

**Collaborative discovery from "Screening".**

## 3.
## In-house drug development -> Asset licensing
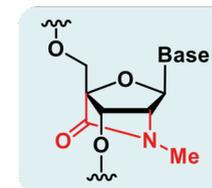
You are looking for drug candidate...

**Collaborative development or asset licensing of our in-house developed pipelines.**

# New phosphoramidites for oligonucleotide synthesis

Amido-bridged nucleic acid
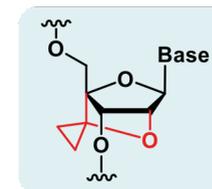
## AmNA™

ASO  SSO



• High binding affinity towards
• complementary ssRNA (+4ºC/mod).
• Improves metabolic stability. Reduces hepatotoxicity.

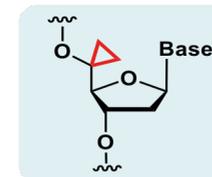Spirocyclopropylene-bridged nucleic acid

## scpBNA™

ASO  SSO



• High binding affinity towards complementary ssRNA (+5ºC/mod).
• Improves metabolic stability.
• Introduce hydrophobic moiety to oligonucleotide.
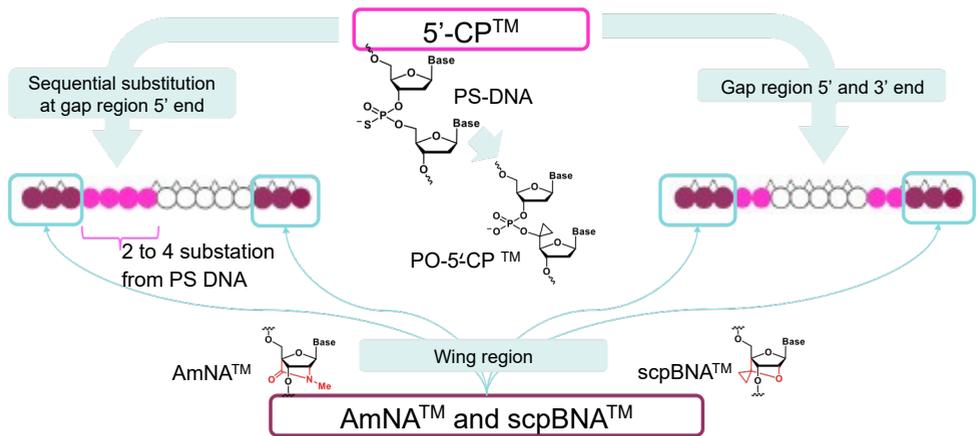
5'-cyclopropylene nucleic acid

## 5'-CP™

ASO  SSO  siRNA



• Sugar conformation similar to DNA.
• Reduces binding affinity towards complementary ssRNA (-3 ºC/mod).
• Improves enzymatic stability enabling PS modification reduction.
• Reduces toxicity of an oligonucleotide.
• Reduces off-target effects.

**Luxna XNAs are available from its collaboration partner, Summit Pharmaceuticals International Corporation (SPI).**

**S P I**
Summit Pharmaceuticals International

**S P E**
Summit Pharmaceuticals Europe

# Recommended incorporation
# of XNAs [gapmer]



5'-CP[TM]

Sequential substitution
at gap region 5' end

PS-DNA

Gap region 5' and 3' end

2 to 4 substation
from PS DNA

PO-5'CP [TM]

AmNA[TM]

Wing region

scpBNA[TM]

AmNA[TM] and scpBNA[TM]

**Combined incorporation of Luxna XMAs enables
to produce suitable ASO gapmer.**