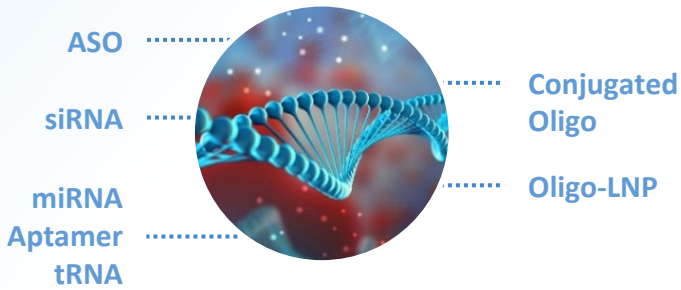


Advancing Discovery of Oligonucleotide Drugs



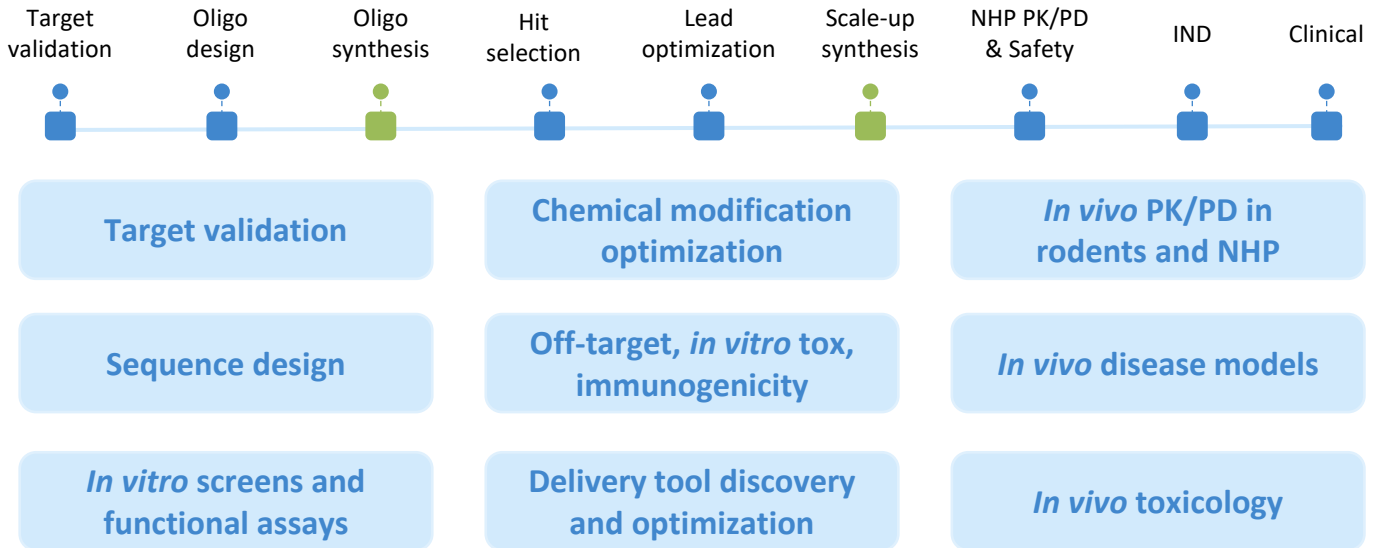
High potential new modalities

250+ Clients

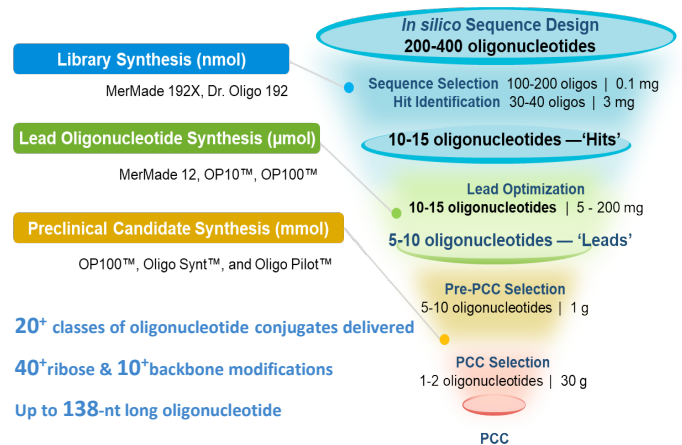
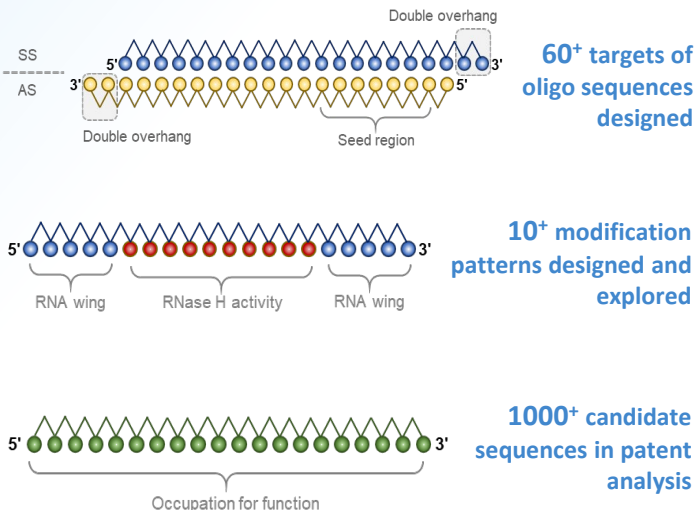
250+ Targets

1,000+ Projects

50+ IND projects



Sequence Design and Chemical Synthesis



In Vitro Evaluation of Efficacy and Delivery

Cell lines

1500+ cell lines, patient-derived fibroblasts, engineered cell lines, etc.

Primary cells

Primary hepatocytes from human, mouse, rat, dog and monkey. Other primary cells such as neurons, etc.

Reporter cells

psiCHECK2 reporter, splicing reporter

iPSCs

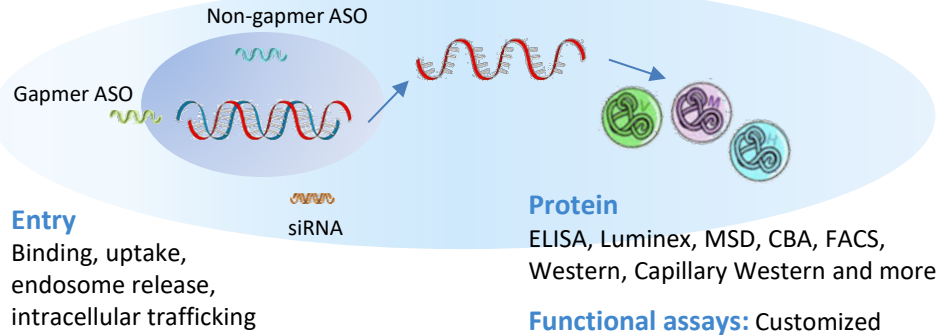
Neurons, cardiomyocytes, etc.

Stability

Plasma, tissue homogenate (liver, brain), RNase, EMSA, LC-MS/MS, HPLC, SL-PCR

mRNA expression

RT-qPCR, ddPCR, bDNA, capillary electrophoresis, transcriptome profiling, RISC loading



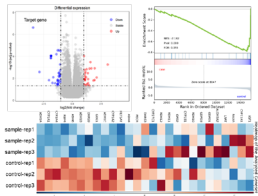
Off-Target and Safety Assessments

Off-Target Profiling

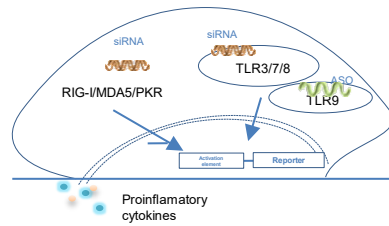
Computational Simulation analysis: Prediction of potential off-targets

3'-tag-seq, bulk RNA profiling: *In vitro* or *ex vivo* evaluation of potential off-targets

psiCHECK reporter: *In vitro* validation of potential off-target



In Vitro Immunogenicity Assays



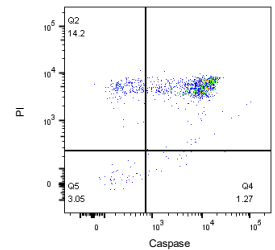
Cytokine release in PBMC: Multiple species and comprehensive cytokine profiling methods, including MSD and Luminex

High-throughput BJAB assay: RT-qPCR readout

Cytotoxicity Assays

Abundant cell resources: Primary cells, cell lines, engineered cells, etc.

Multiple readouts: CellTiter-Glo, LDH assay, caspase-Glo3/7 and more



In Vivo PK/PD Assessments

Efficacy

PK and PD assessments in mouse

- Hydrodynamic injection (HDI) mouse model
- AAV mouse model
- Transgenic mouse model
- Cell transplant model



Disease models for functional assessment in mouse and NHPs

- Neurodegenerative disease
- Metabolism & liver disease
- Ophthalmic disease
- Ear disease
- Infectious disease



Delivery

In vivo sampling

- Blood, CSF (+/- cannula), aqueous & vitreous humor, biopsy

In vivo imaging

Organ collection

- Bulk analysis and single cell analysis for PK and PD assessment, DNA/RNA profiling

Delivery tool development

- LNP, peptide conjugate and more

