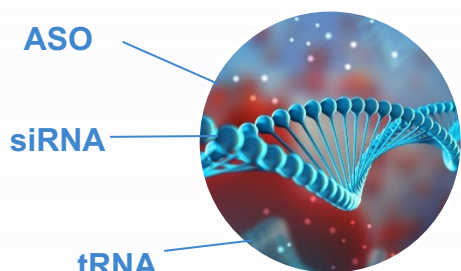


# Supporting Discovery of Oligonucleotide Drugs



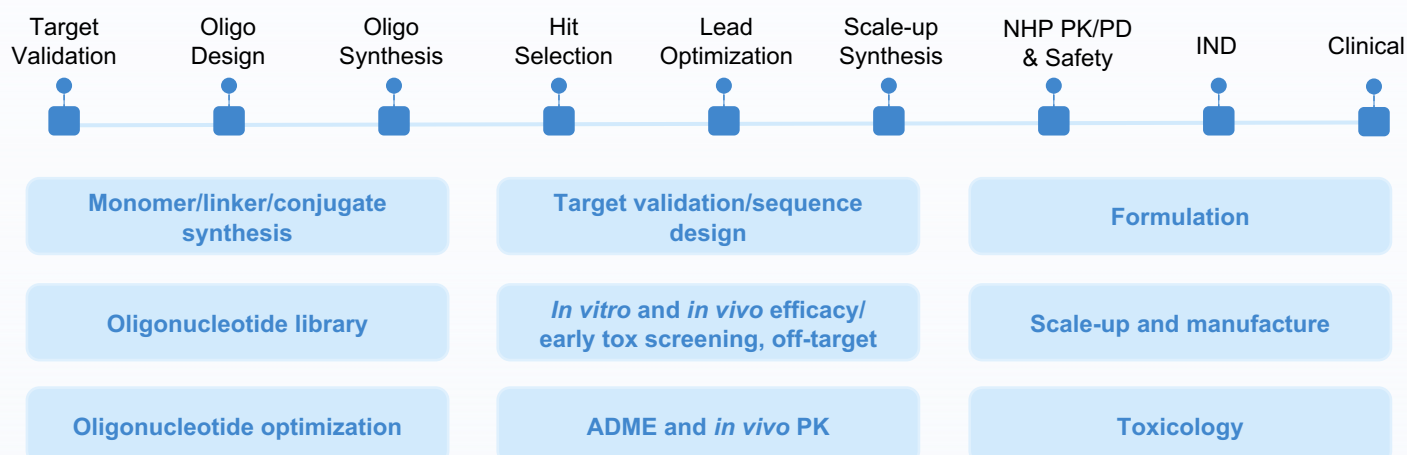
High potential new modality

**110+**  
Clients

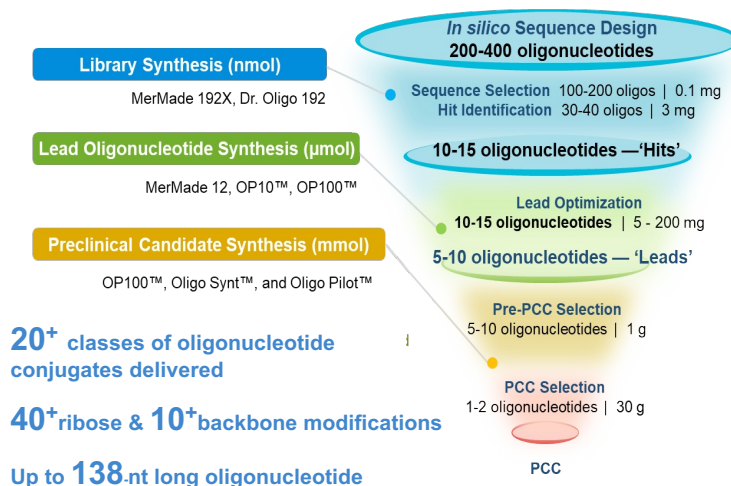
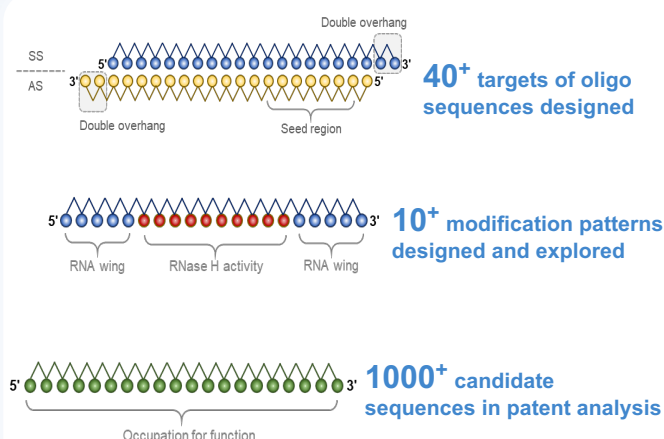
**150+**  
Targets

**500+**  
Projects

**20+**  
IND Projects



## Sequence Design and Chemical Synthesis



## In Vitro Evaluation of Stability and Efficacy

### Cell lines

**1500+** cell lines and patient-derived fibroblasts. Customized stable cell lines

### Primary cells

Primary hepatocytes of human, mouse, rat, dog and monkey. Customized other primary cells

### Reporter cell lines

ASO splicing reporter, psiCHECK

### Methods

Free uptake and transfection

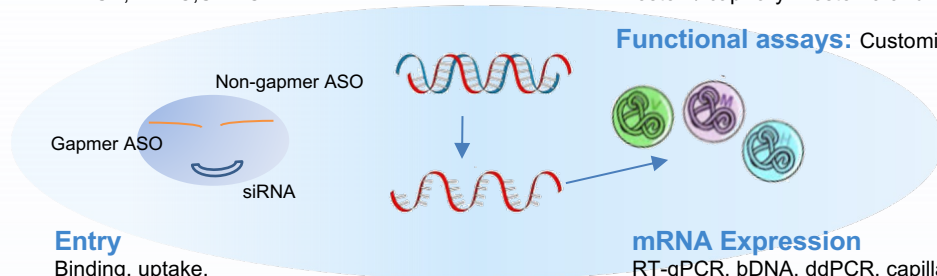
### Stability

Plasma, tissue homogenate, RNase, EMSA, HPLC, SL-PCR

### Protein

ELISA, Luminex, MSD, CBA, FACS, Western/ capillary Western and more

**Functional assays:** Customized



### Entry

Binding, uptake, intracellular trafficking, endosome release

### mRNA Expression

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



## 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 assessments, sequencing

#### Delivery tool development

- LNP, peptide conjugate and more

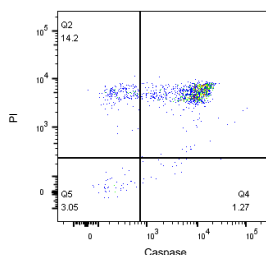


## Off-target and Safety Assessments

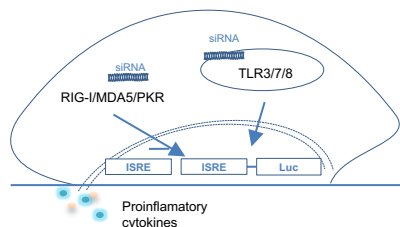
### Cytotoxicity assays

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

**Multiple readouts:** CellTiter-Glo, LDH assay, caspaseGlo3/7 and more



### In vitro immunogenicity assays



#### Comprehensive PRR reporter cell lines

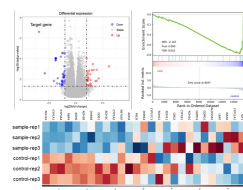
**Cytokine release in PBMC:** Multiple species and comprehensive cytokine profiling methods, including MSD and Luminex

### Off-target profiling

**In silico analysis:** Prediction of potential off-target

**RNA-seq:** In vitro or ex vivo evaluation of potential off-target

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



## Contact Us

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