

## HD

HD

Mr. Shawn Minnig

2016 11 07

Dr Jeff Carroll

Xi Cao

2016 10 31

zinc finger nucleases CRISPR/Cas9

ASOs



DNA

-mRNA

ASO

CRISPR

DNA

DNA

DNA

-

DNA

HD

...

DNA

RNA mRNA

DNA

DNA

mRNA

DNA -> mRNA ->

### ASO

### CRISPR

ASO CRISPR

DNA HD

mRNA HD

ASO mRNA ASO mRNA mRNA DNA ASO mRNA  
ASO mRNA ASO ASO " " HD DNA HD DNA ASO  
**CRISPR/Cas9** HD DNA DNA  
mRNA HD HD mRNA ASO ASO  
**CRISPR/Cas9** CRISPR/Cas9 DNA DNA  
mRNA CRISPR DNA CRISPR DNA CRISPR HD HD

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CRISPR

DNA

**HD**

HDbuzz

Mark Isalan

AAV

Isalan

HD

HD

**CRISPR HD**

CRISPR

CRISPR HD Jong Min Lee  
HD HD

CRISPR HD CRISPR

CRISPR – **1000** HD

CRISPR      DNA      ASO ASO      ASO  
                 HD      HD      HD

CRISPR

HD

CRISPR

HD

—Sangamo

Shire

HD

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