

## WAVE PRECISION

Dr Michael Flower

2017 8 31

Dr Tamara Maiuri

Xi Cao

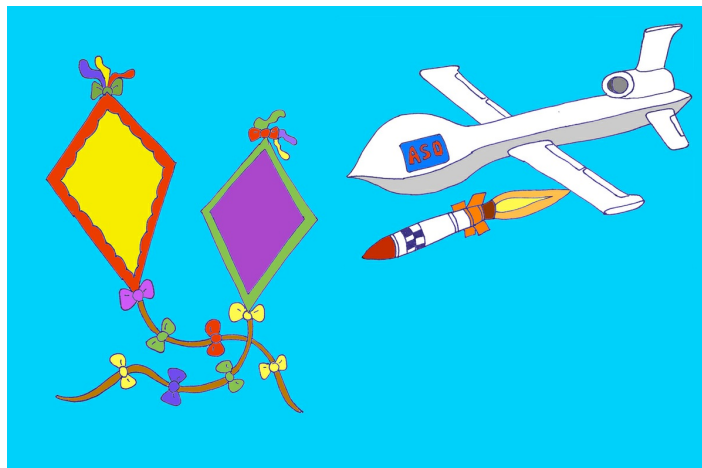
2017 8 25

- WAVE

PRECISION-HD1 2,

DNA

HD HTT



WAVE ASO

HD

Mike Flower

HTT

HTT

HD

HD

DNA

DNA  
RNA

DNA  
RNA DNA

DNA

DNA

DNA

RNA

DNA

RNA —  
DNA RNA

RNA mRNA

DNA DNA — DNA

DNA

DNA

-mRNA

HD

“ASOs” ASOs

RNA

ASOs

DNA

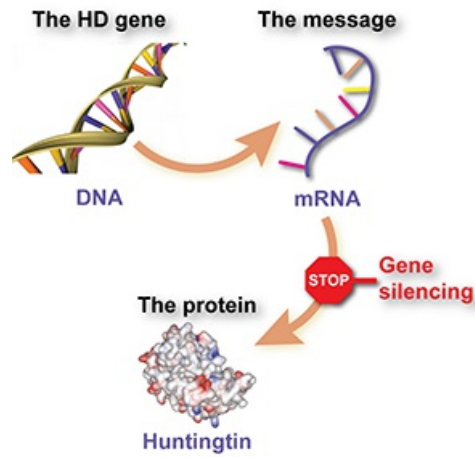
ASO

HD RNA

DNA DNA ASO RNA HD RNA  
 ASOs ASO CSF ASOS

# ASO

Ionis ASO Ionis ASO RNA RNA  
 50%  
 WAVE ASO SNPs SNP DNA SNPs  
 HD " " ASO  
 WAVE ASOs HTT SNPs SNPs HTT  
 SNP rs362307 HD SNP rs362331 40% HD  
 HD ASO



HD

HTT ASOs 1/3

WAVE HD Wave ASOs SNP

WAVE WAVE WAVE HD HD

- WAVE

WAVE 1b/ 2a 1 2 WAVE 1 2  
 1 HD 2 Ionis WAVE

WAVE 50 HD 18

HD SNPs

# HD

Ionis ASO

HD

HD

WAVE

RNA

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*HDbuzz*

*WAVE*

*Ionis*

*Jeff Carroll WAVE*

*Ionis ASO*

*Michael Flower Tamara Maiuri*

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