



WAVE PRECISION

Dr Michael Flower 2017 8 31

Dr Tamara Maiuri ; Xi Cao

2017 8 25

- WAVE

PRECISION-HD1 2,

DNA HTT HD HTT  
 HTT HD  
 DNA DNA DNA DNA DNA  
 DNA RNA DNA RNA RNA  
 DNA  
 RNA — DNA DNA DNA DNA  
 — DNA DNA RNA mRNA  
 DNA -mRNA  
 HD "ASOs" ASOs RNA ASOs DNA ASO  
 HD RNA  
 DNA DNA ASO RNA HD RNA  
 ASOs ASO CSF ASOS



**ASO**

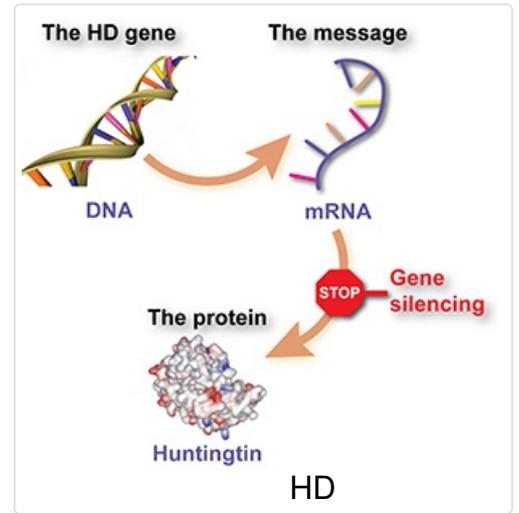
Ionis ASO Ionis ASO RNA RNA  
 50%  
 WAVE ASO SNPs SNP DNA SNPs  
 HD " " ASO

WAVE ASOs HTT SNPs SNPs HTT  
 SNP rs362307 HD SNP rs362331 40% HD  
 HD ASO

ASOs 1/3 HTT

WAVE Wave ASOs SNP HD

WAVE HD WAVE HD WAVE  
 - 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*