

HDBUZZ

2017 - 2

HDBuzz 2017 Huntington's Disease Therapeutics Conference - 2



Dr Jeff Carroll 2017 07 10 () Dr Jeff Carroll

Prof Wooseok Im 2017 04 29 ()

2 가
“Huntingtin lowering”



Biomarkers Ed Wild

Ed Wild (University College London) trials (biomarkers) sensitive

가. Wild
가.
.

1 (SCA1)
Harry Orr (U. Minnesota) . SCA1 "C-A-G"가
DNA . SCA1 가
"Ataxin-1"
huntingtin
Orr
SCA1
Orr SCA1
Orr Ataxin-1
(ASOs) . SCA1

Nicole Deglon (Lausanne U.) huntingtin
tool
가 . CRISPR / Cas9 "
" tool DNA . Deglon
가 tool . Deglon
가 DNA "가"
Deglon DNA
가
DNA 가

"
huntingtin ."

CHDI (Liz Doherty) .
" "
ASO tool
huntingtin
CHDI 130,000 가
가
(huntingtin) 4 가 huntingtin

ASO가
.

가

가 가
FAQ...



ASOs DNA
DNA
.

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