

Prime Editing voor patiënten met metabole ziekten



Symposium Innovatieve Therapieën
Prof. dr. Sabine A. Fuchs



Metabole ziekten



>1400 individueel zeer zeldzame ziekten

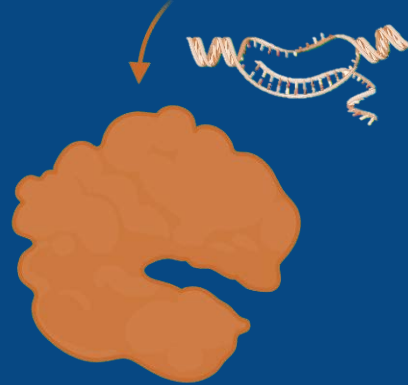
Metabole ziekten



DNA

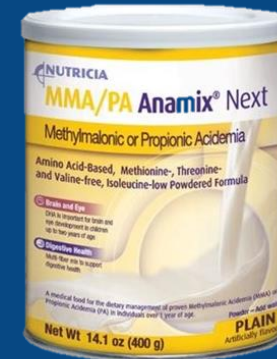
RNA

Enzym



A

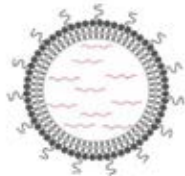
B



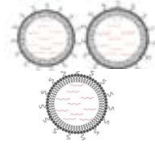
RNA



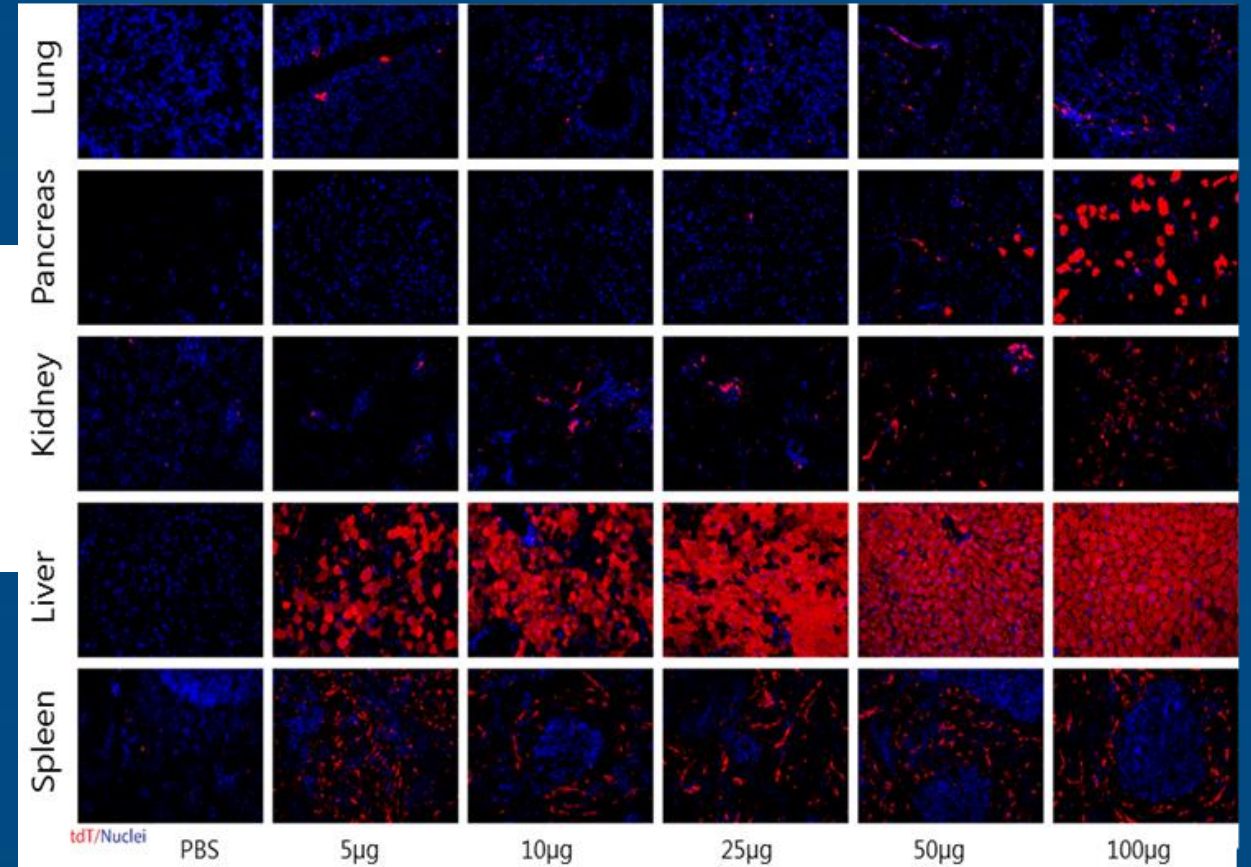
RNA



vetbolletjes

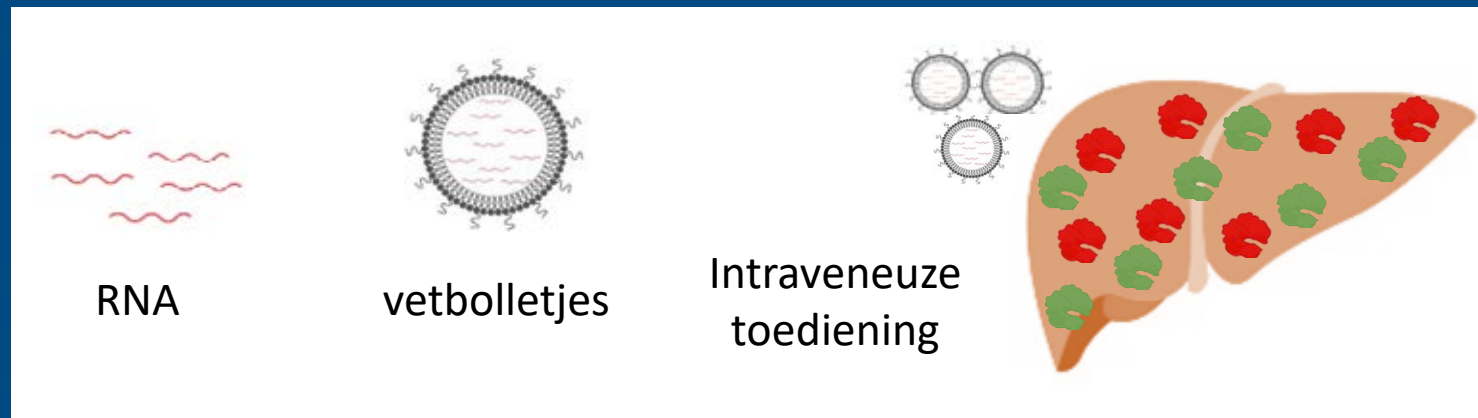


Intraveneuze
toediening



Evers et al. *JCR* 2022

Intraveneus RNA als behandeling?



Eerste 3 patiënten in NL



Effectief, maar tijdelijk

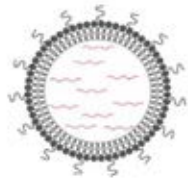
RNA voor permanent effect?



Gen correctie
gereedschap



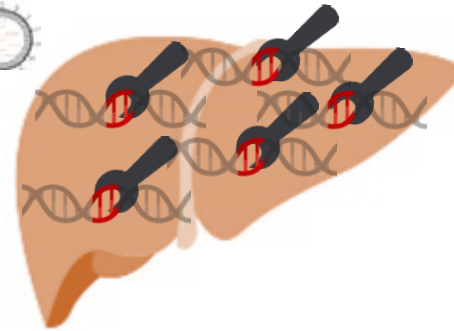
RNA



vetbolletjes



Intraveneuze
toediening



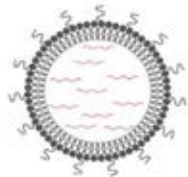
RNA voor permanent effect?



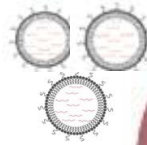
Gen correctie
gereedschap



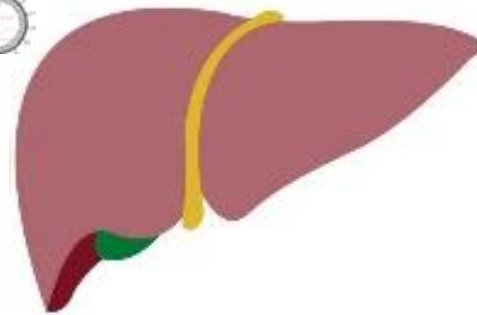
RNA



vetbolletjes



Intraveneuze
toediening

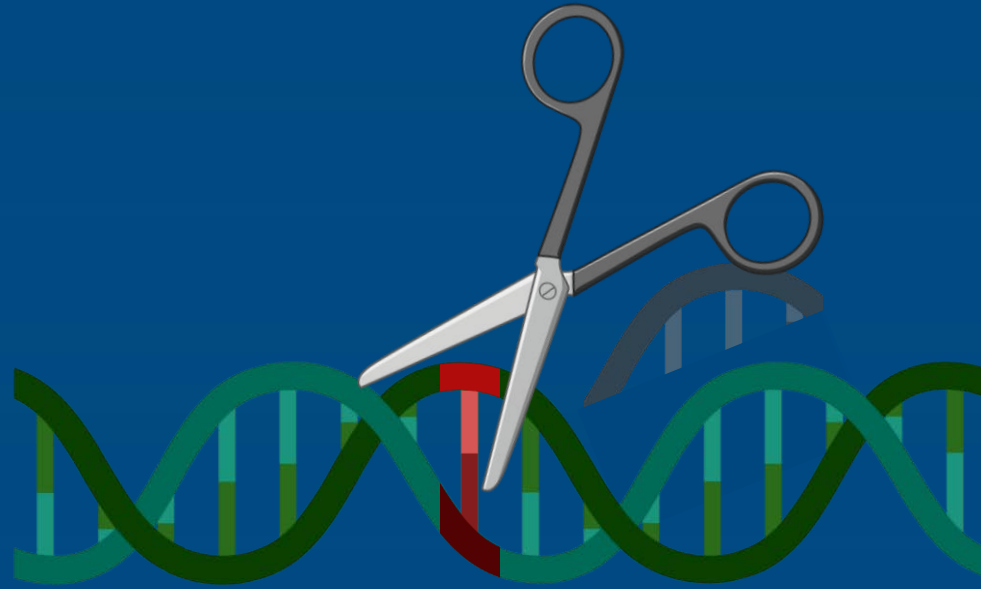


**Eenmalige
Behandeling**

**Permanente
Genezing**

Maar dan moeten we goed gereedschap hebben

CRISPR-Cas: moleculaire schaar



CRISPR-Cas: moleculaire schaar



CRISPR-Cas: moleculaire schaar



Alleen in delende cellen (max 30%)

CRISPR-Cas: moleculaire schaar



CRISPR-Cas: moleculaire schaar



Schakelt genen uit
Eenvoudig



Nobelprijs voor Scheikunde
2020

Gen correctie: prime editing



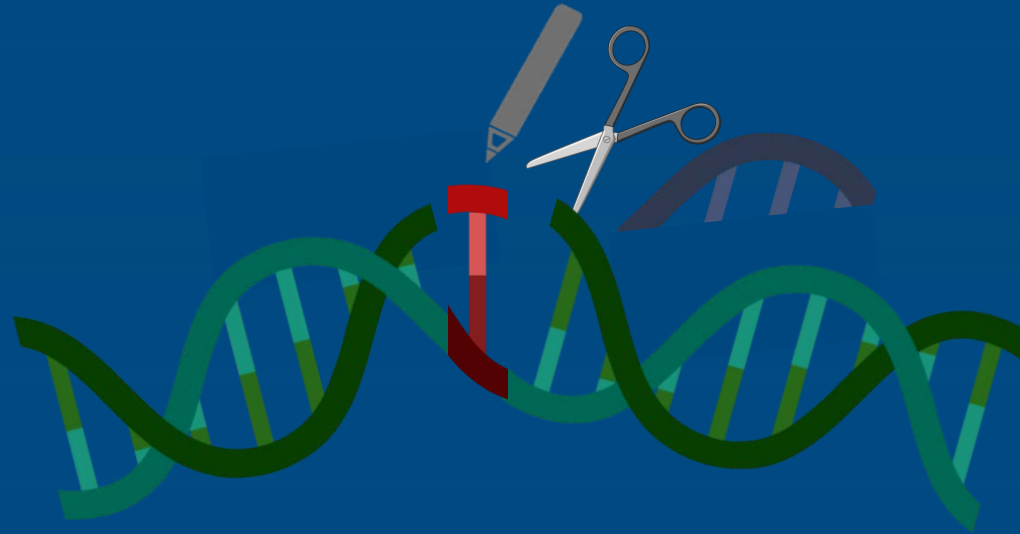
David Liu



Gen correctie: prime editing



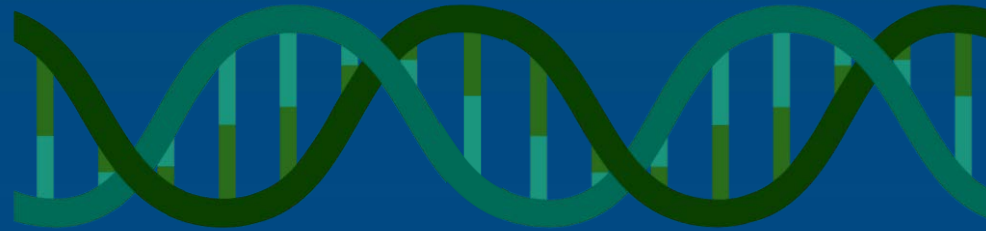
David Liu



Gen correctie: prime editing



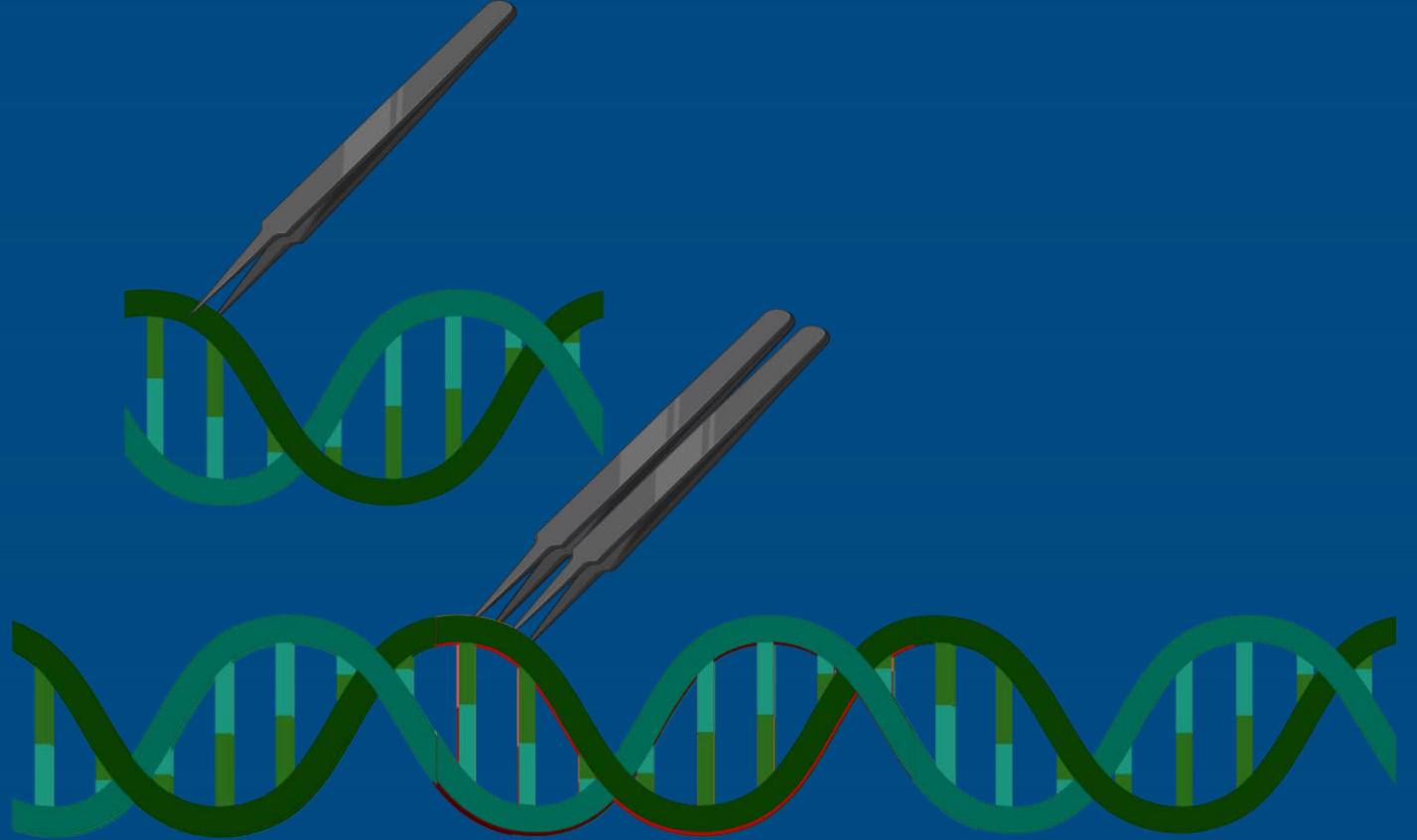
David Liu



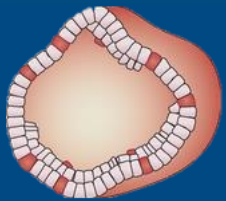
Gen correctie: prime editing



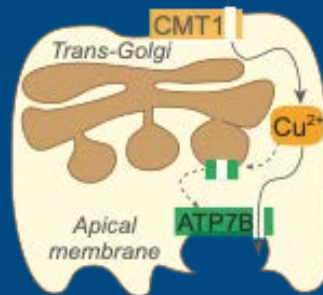
David Liu



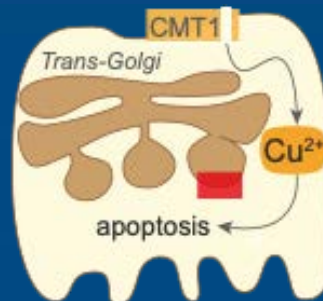
Gen correctie in mini orgaantjes van patiënten



Gezonde controle



Ziekte van Wilson

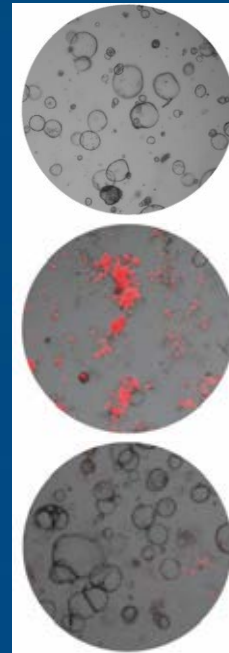


Gezonde
controle

Wilson
patiënt

Wilson
prime
editing

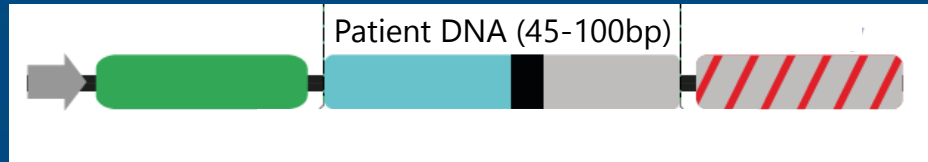
Koper



PI = cel dood

100% Editing
Geen ongewenste effecten

Gen correctie programma



Mutatie- en context-specifieke
Reporter



Patiënt- en orgaan-specifiek
in vitro model



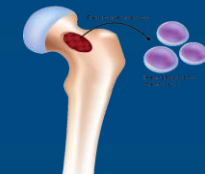
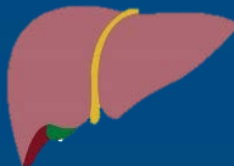
Gepersonaliseerde
gen correctie therapie

Schene et al. *Nature Commun* 2022

Schene et al. *Nature Commun* 2020



StG
POC



Prime Time

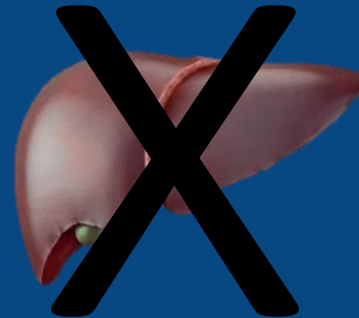
Prime editing comes of age as FDA clears human trials



Prime Medicine has been given the go-ahead by the FDA for the first human trial of its prime editing technology, which promises to deliver one-shot therapies for a range of severe genetic diseases.



Wat betekent dit voor Bjorn?



A

B



Prime time voor metabole ziekten



Afleveringen



Voor de volgende Jelte



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