



PRIME TIME

for patients with metabolic disease

Sabine Fuchs

Professor Metabolic Diseases and Innovative Therapies



Jelte celebrates his “monthday”



> 1800 different metabolic diseases



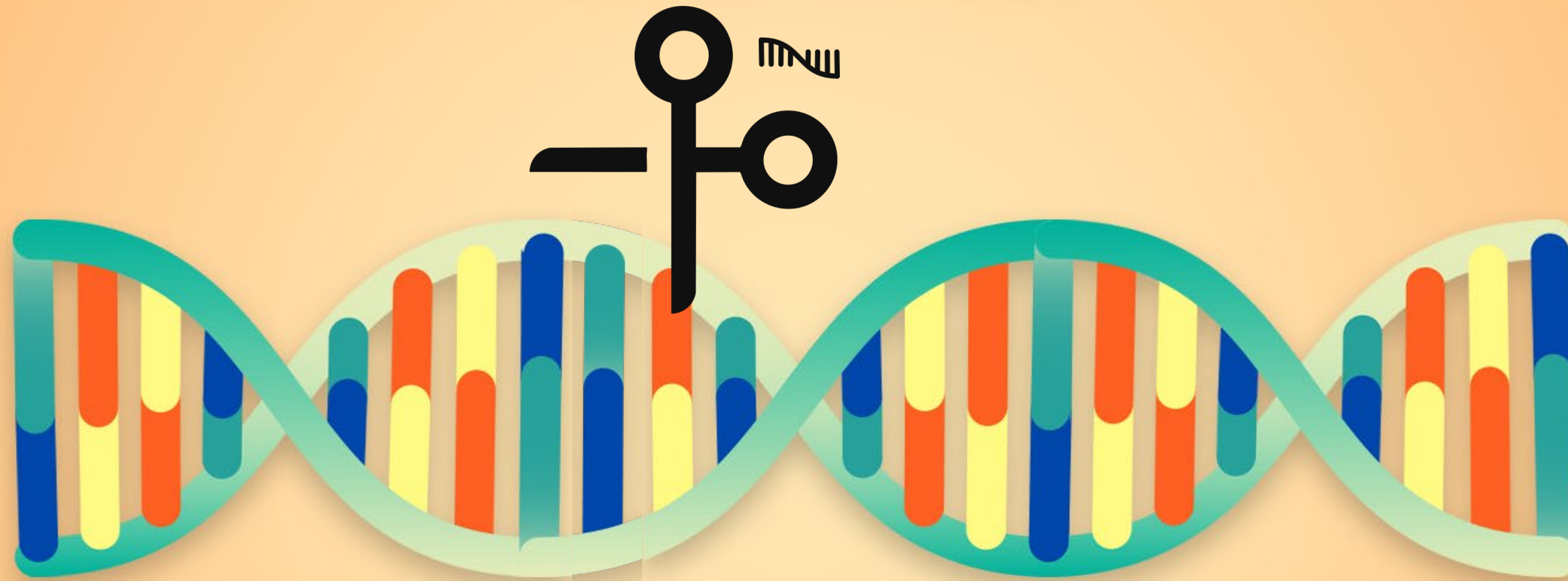
CRISPR | Cas



CRISPR | Cas



CRISPR | Cas



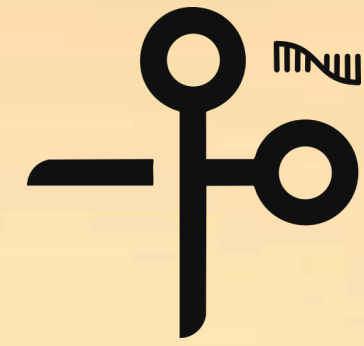
CRISPR | Cas



Nobel prize
Chemistry 2020



BASE EDITING



David Liu

BASE EDITING



David Liu

Only A>G or C>T
Bystander editing

PRIME EDITING



David Liu

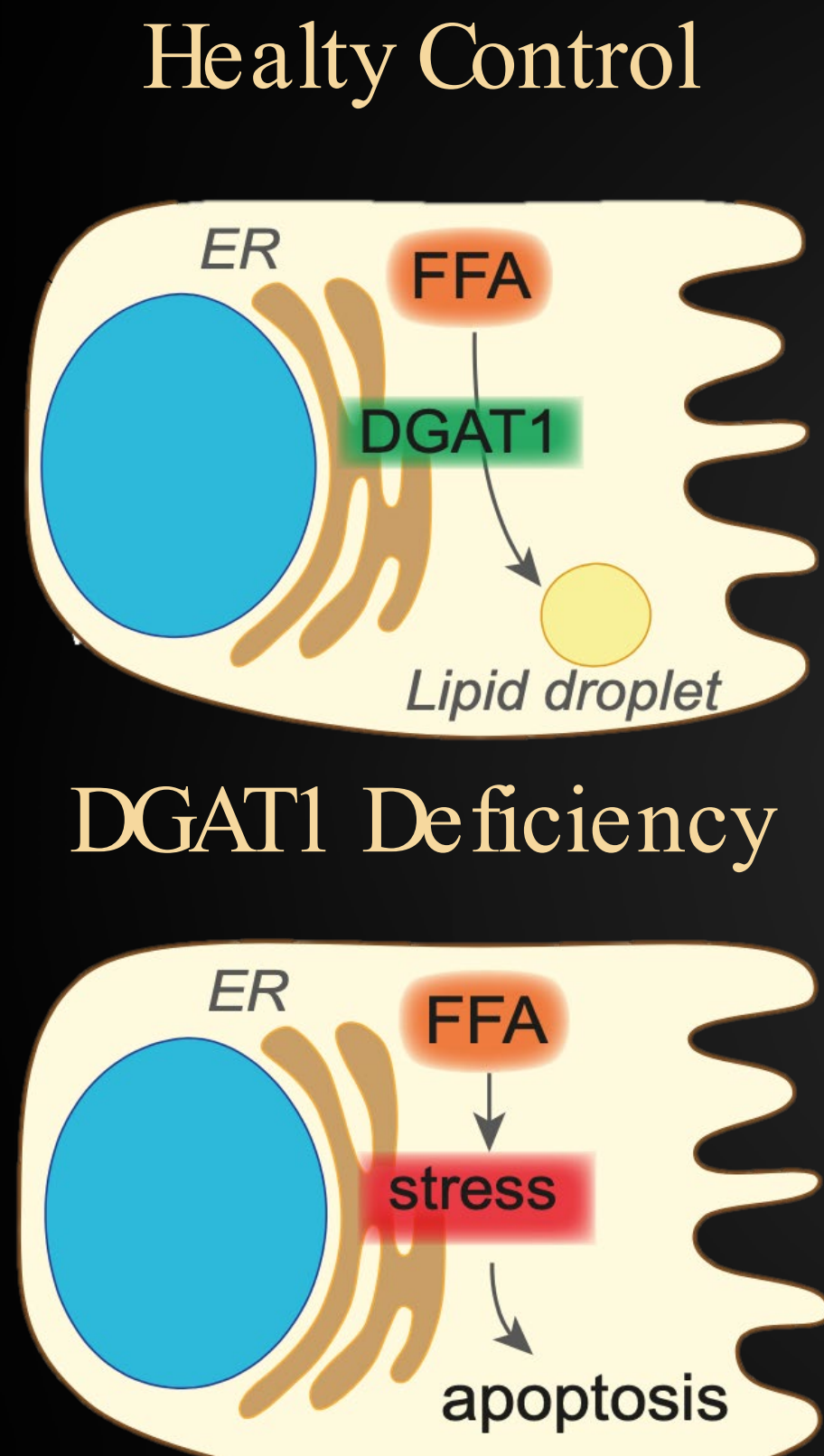
PRIME EDITING



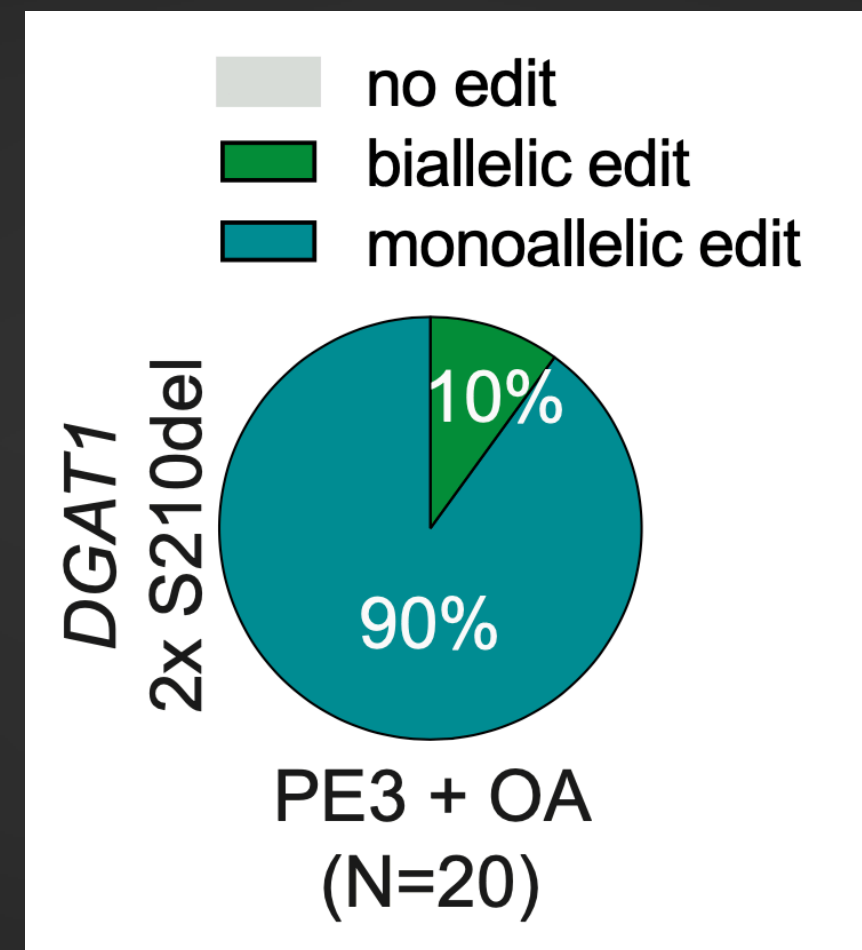
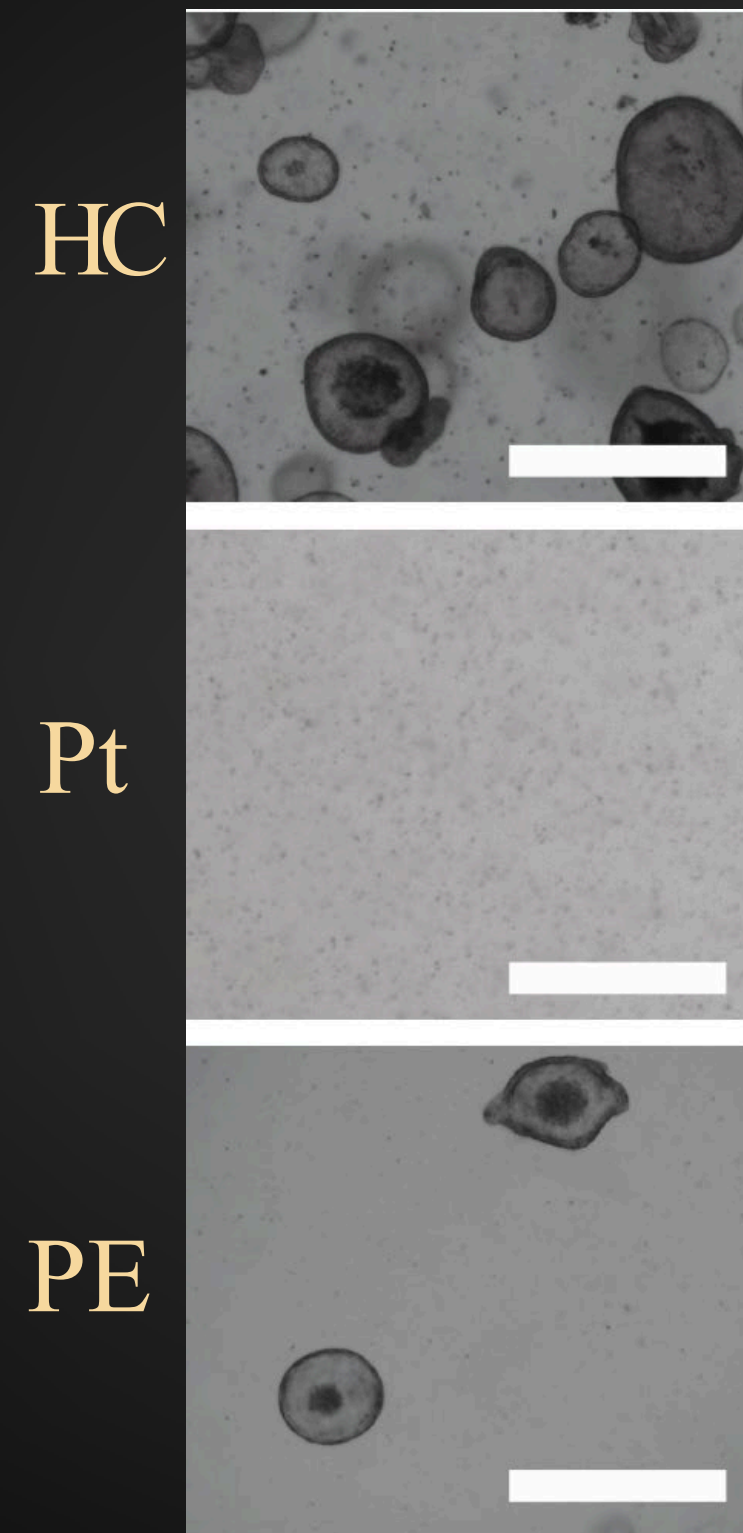
David Liu

For all mutations

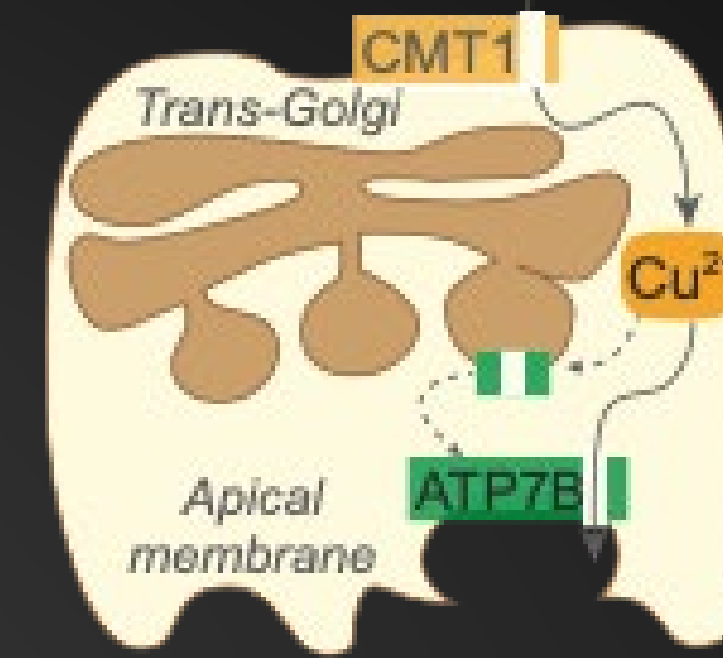
Does this work in human cells?



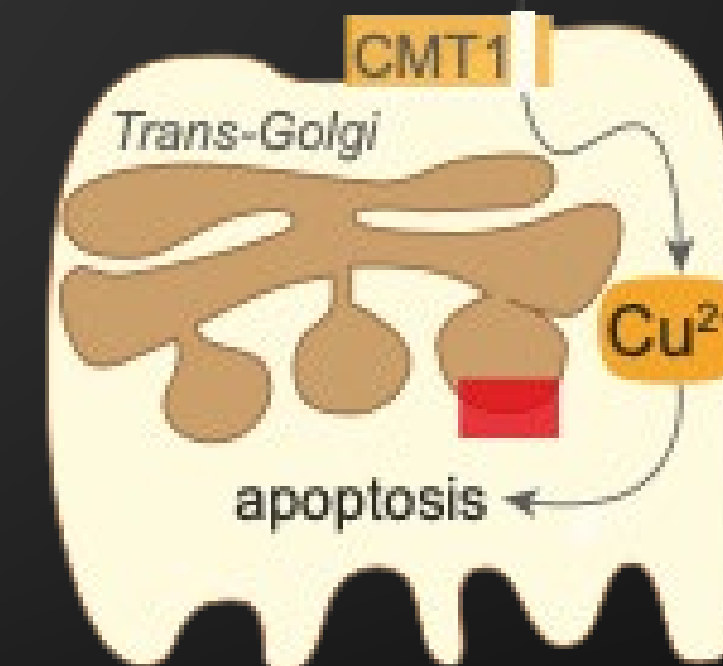
Fatty Acids



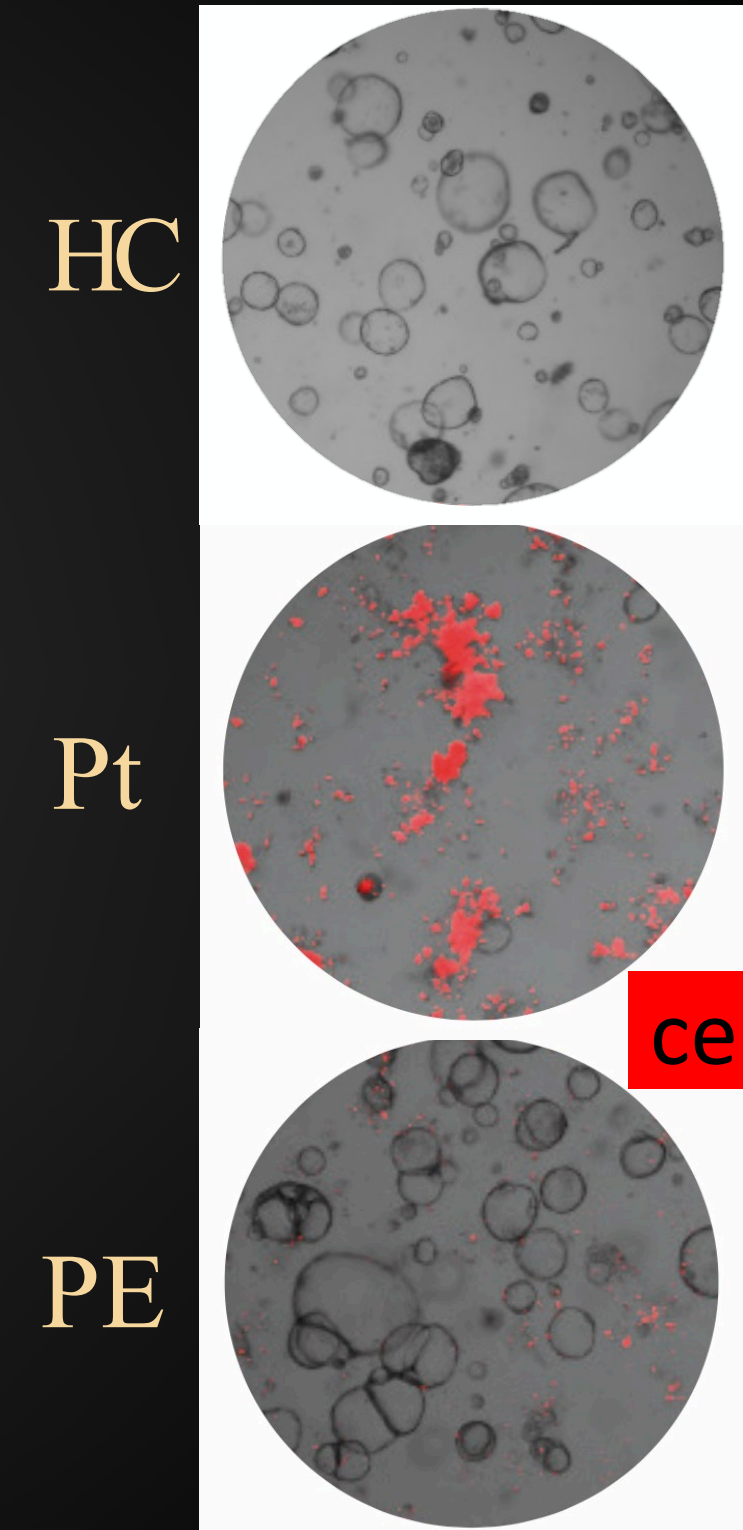
Healthy Control



Wilson Disease



Copper



PI = cell death

WGS: genetically safe

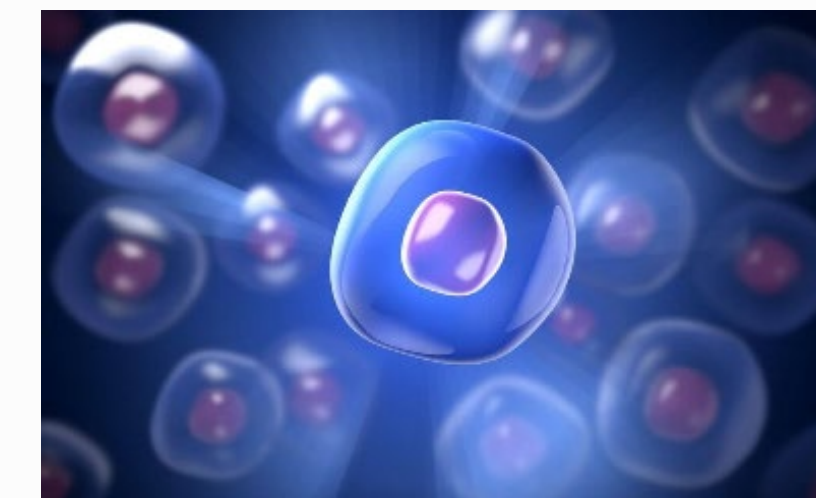
Schene et al. Nature Commun 2020

In vitro editing

FORBES > INNOVATION > HEALTHCARE

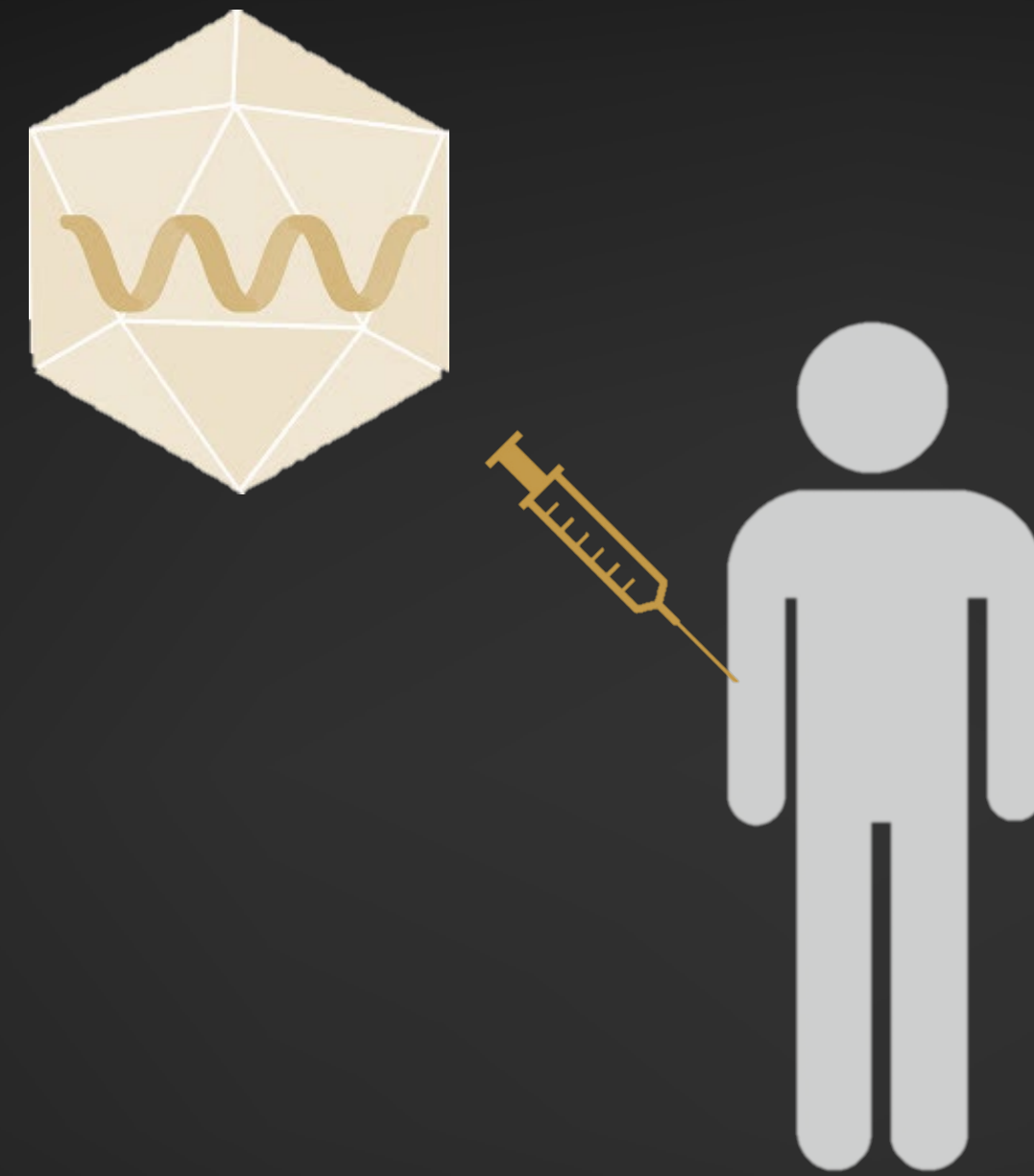
UK Approves Groundbreaking CRISPR-Based Gene Therapy For Sickle Cell Disease And Thalassaemia

William A. Haseltine Contributor ©



Nov 18, 2023, 09:00am EST

What about in vivo editing?



Current gene therapies: viral delivery

Immune reactions

Small cargo capacity

Long-term expression

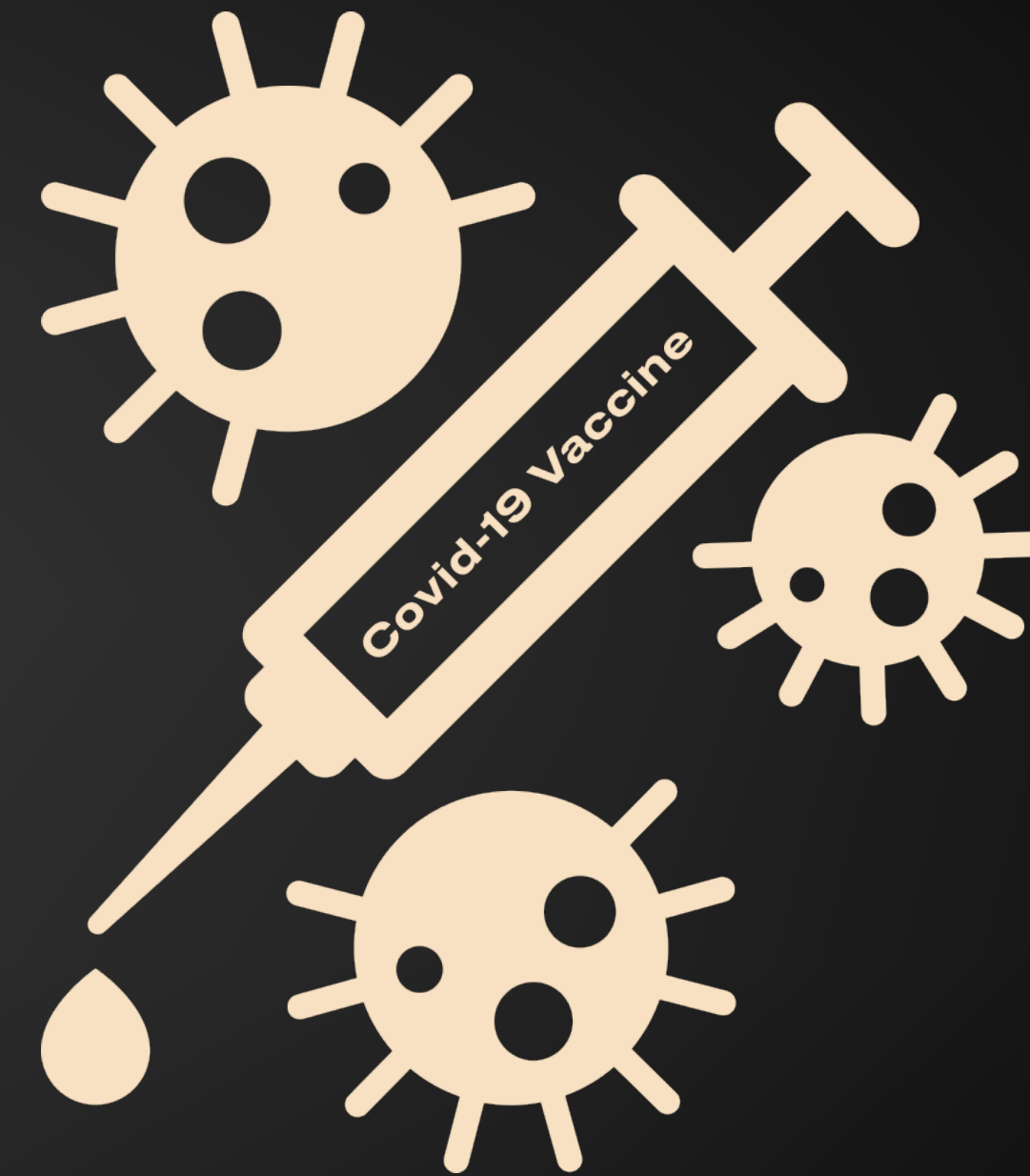


RNA

Nobel prize

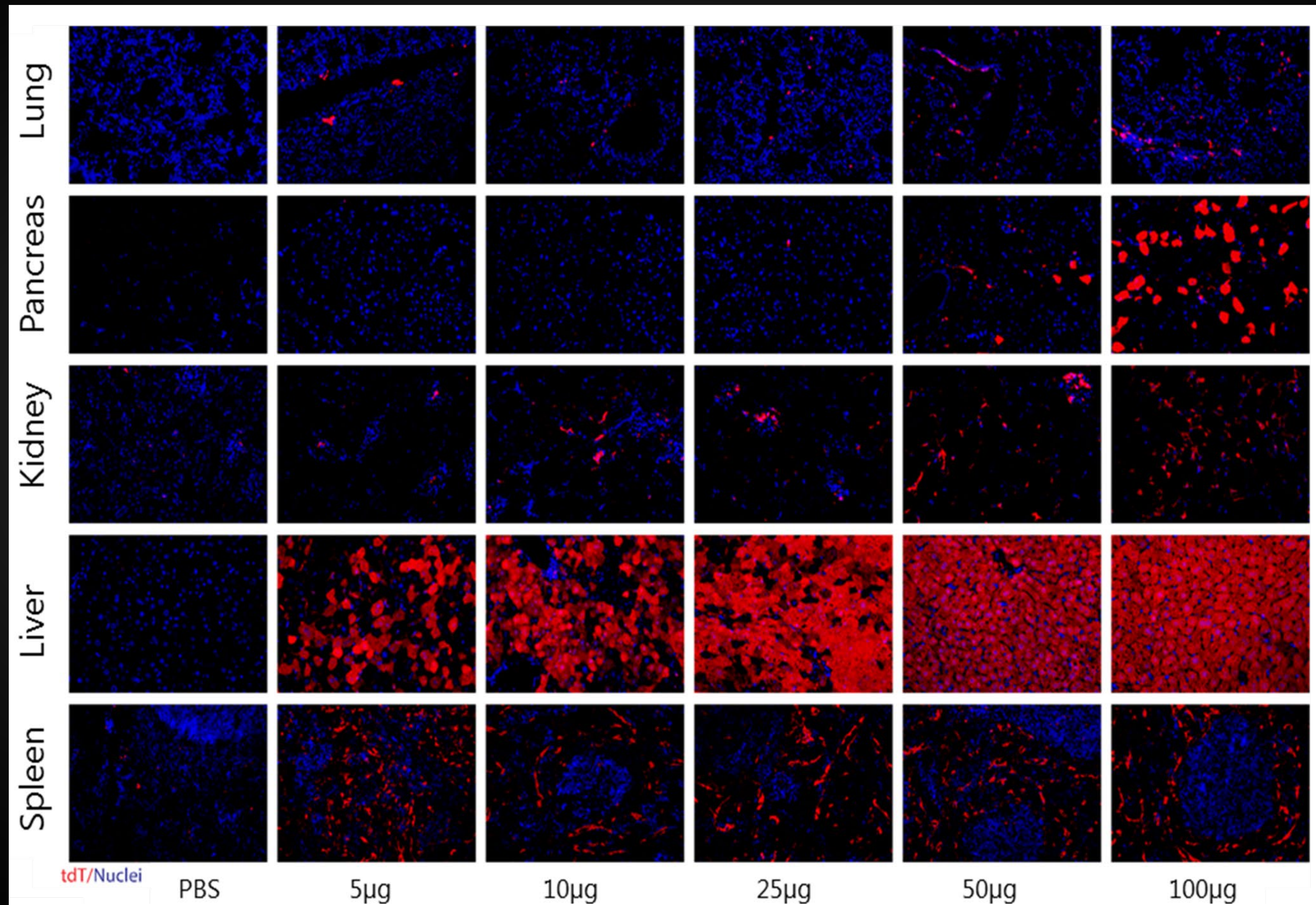
Medicine

2023



**KATALIN
KARIKO**

Intravenous mRNA in LNP



Intravenous mRNA of MMA enzyme

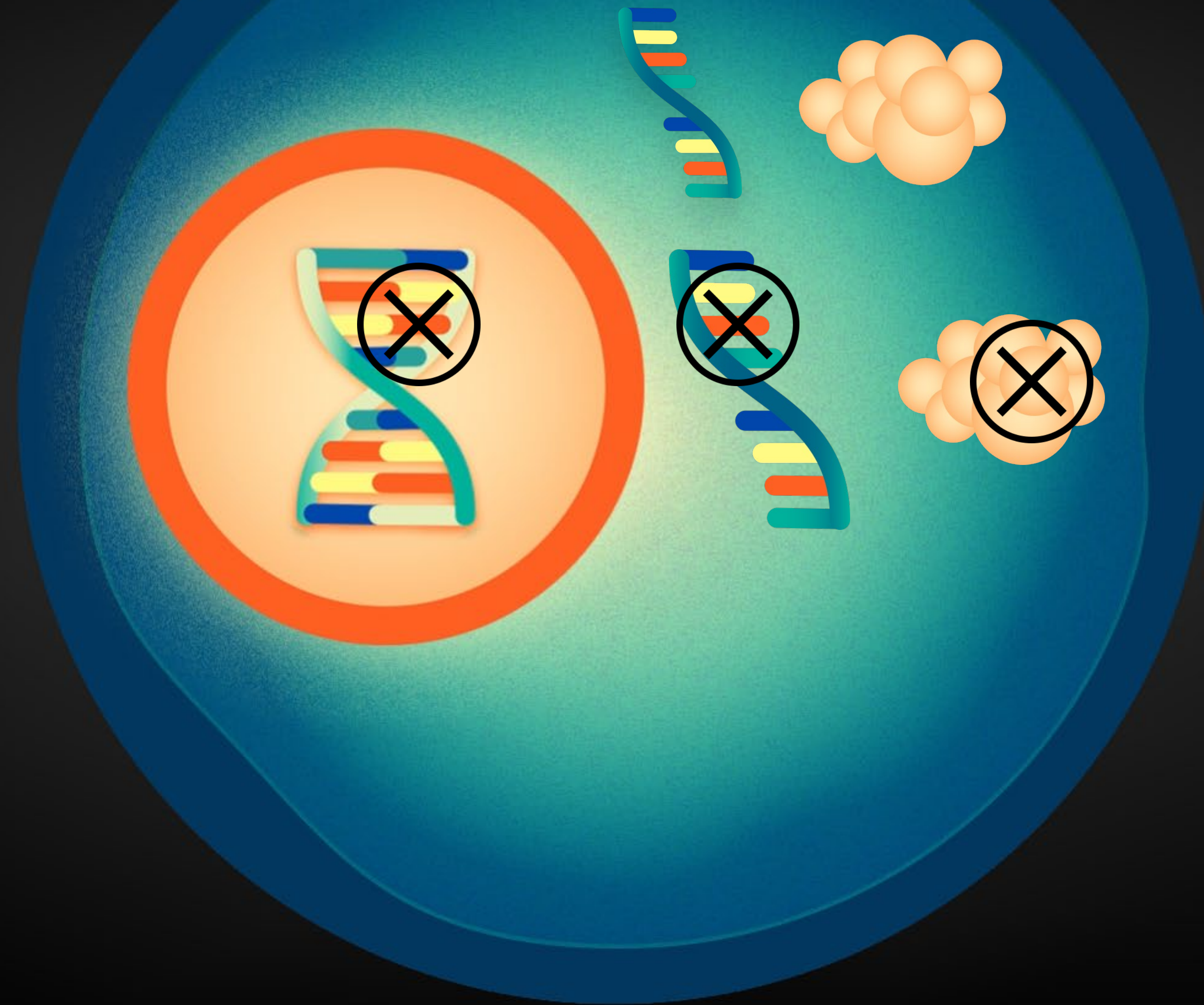


moderna®

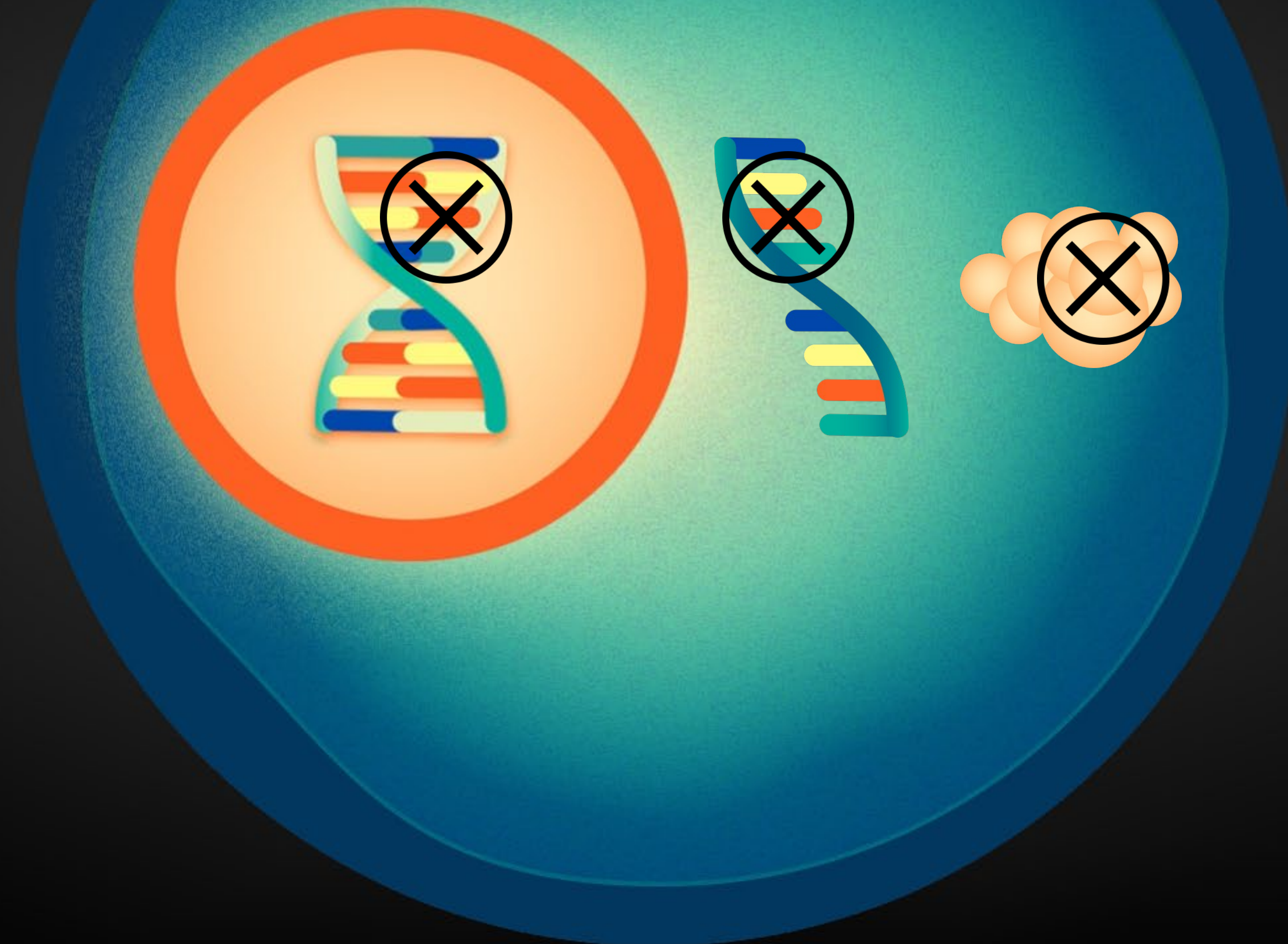


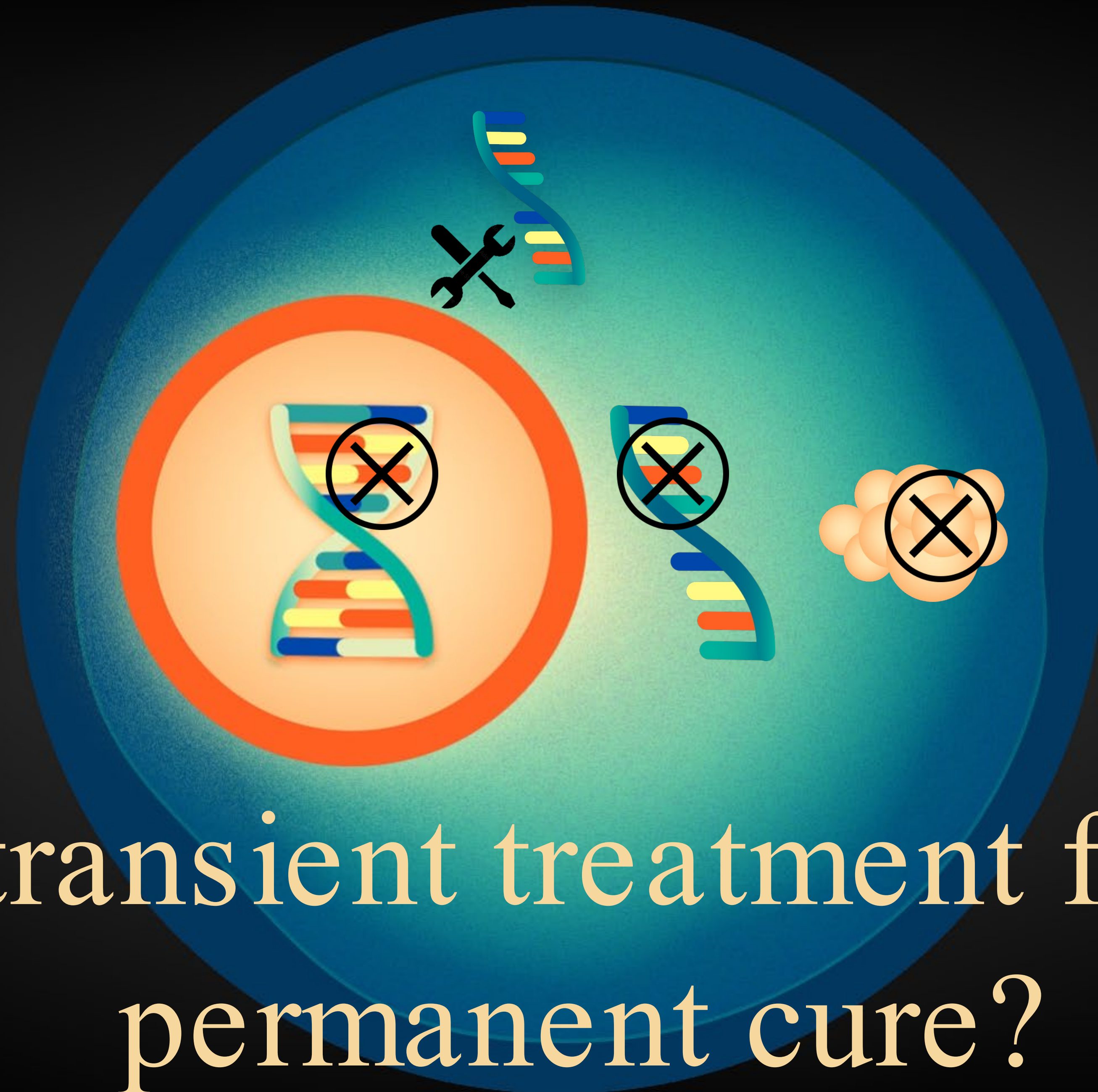
But effects are transient

mRNA of the deficient enzyme



Transient effects





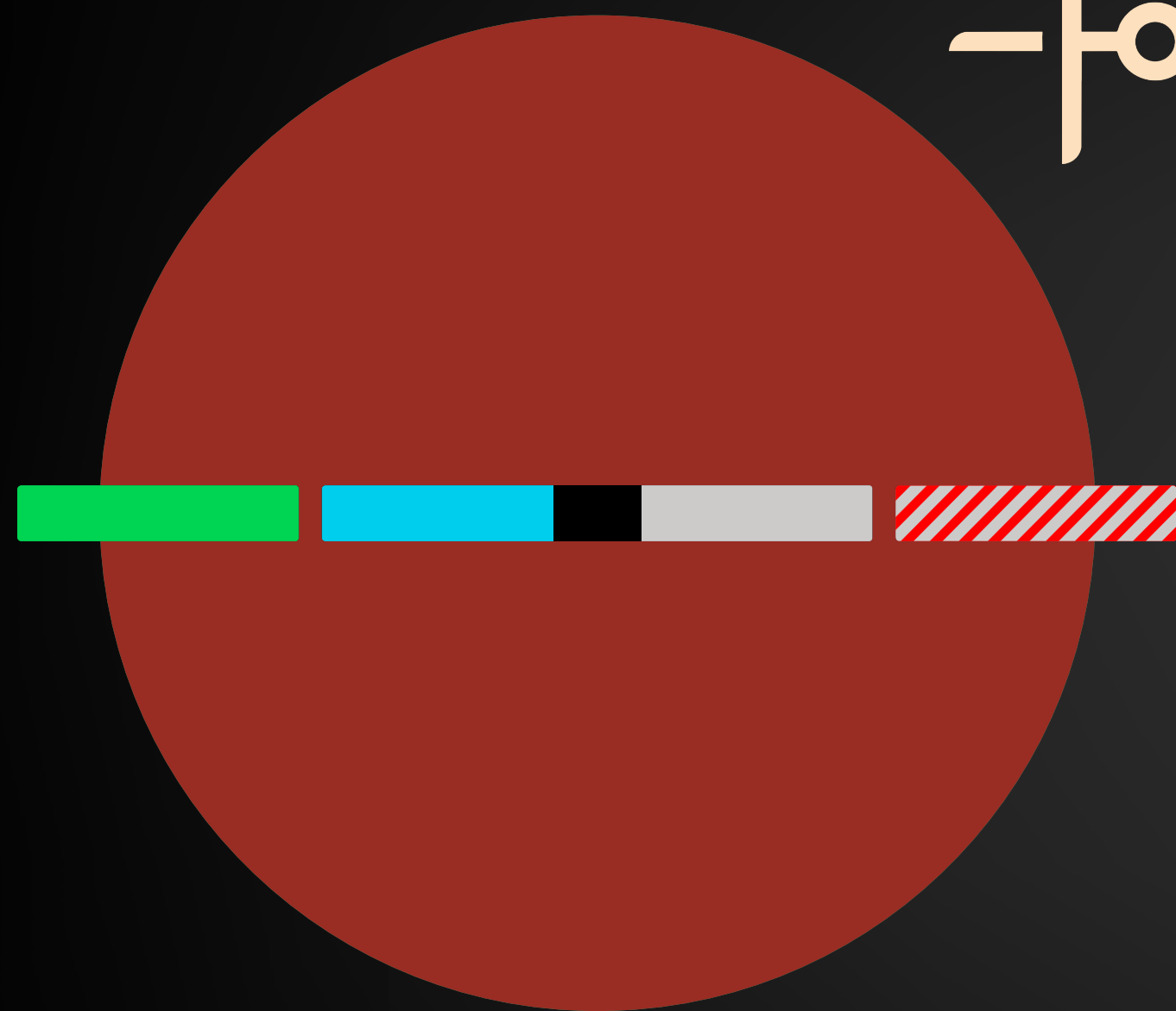
A transient treatment for a
permanent cure?



European Research Council

Gene correction program

AMMODO
SCIENCE
AWARD



Reporter

DNA- and mutation specific



Organoids

Patient and organ specific



NANOSPRESSO NL

Personalized therapy



When does this become a
clinical reality?



NEWS | HEALTH

Base editing, a new form of gene therapy, sharply lowers bad cholesterol in clinical trial

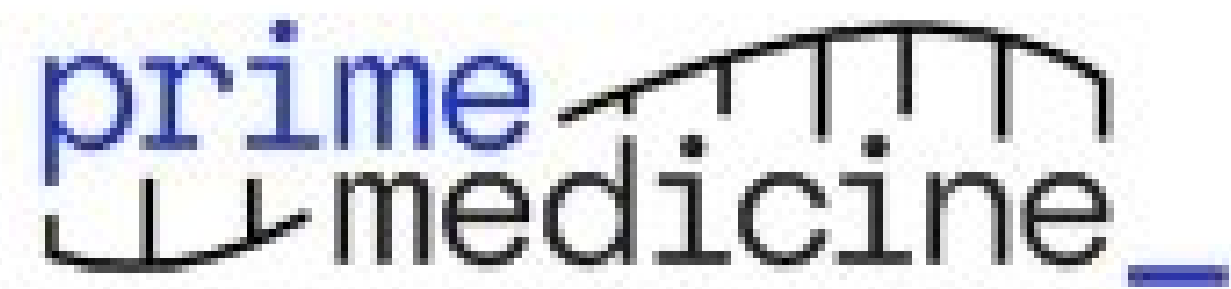
Verve Therapeutics says its twist on CRISPR could with one treatment help prevent heart disease, a major killer, in many people

12 NOV 2023 • 3:30 PM ET • BY [JOCELYN KAISER](#)



Beam Therapeutics Announces Positive Initial Data for BEAM-302 in the Phase 1/2 Trial in Alpha-1 Antitrypsin Deficiency (AATD), Demonstrating First Ever Clinical Genetic Correction of a Disease-causing Mutation

March 10, 2025



Delivering on the promise
of Prime Editing

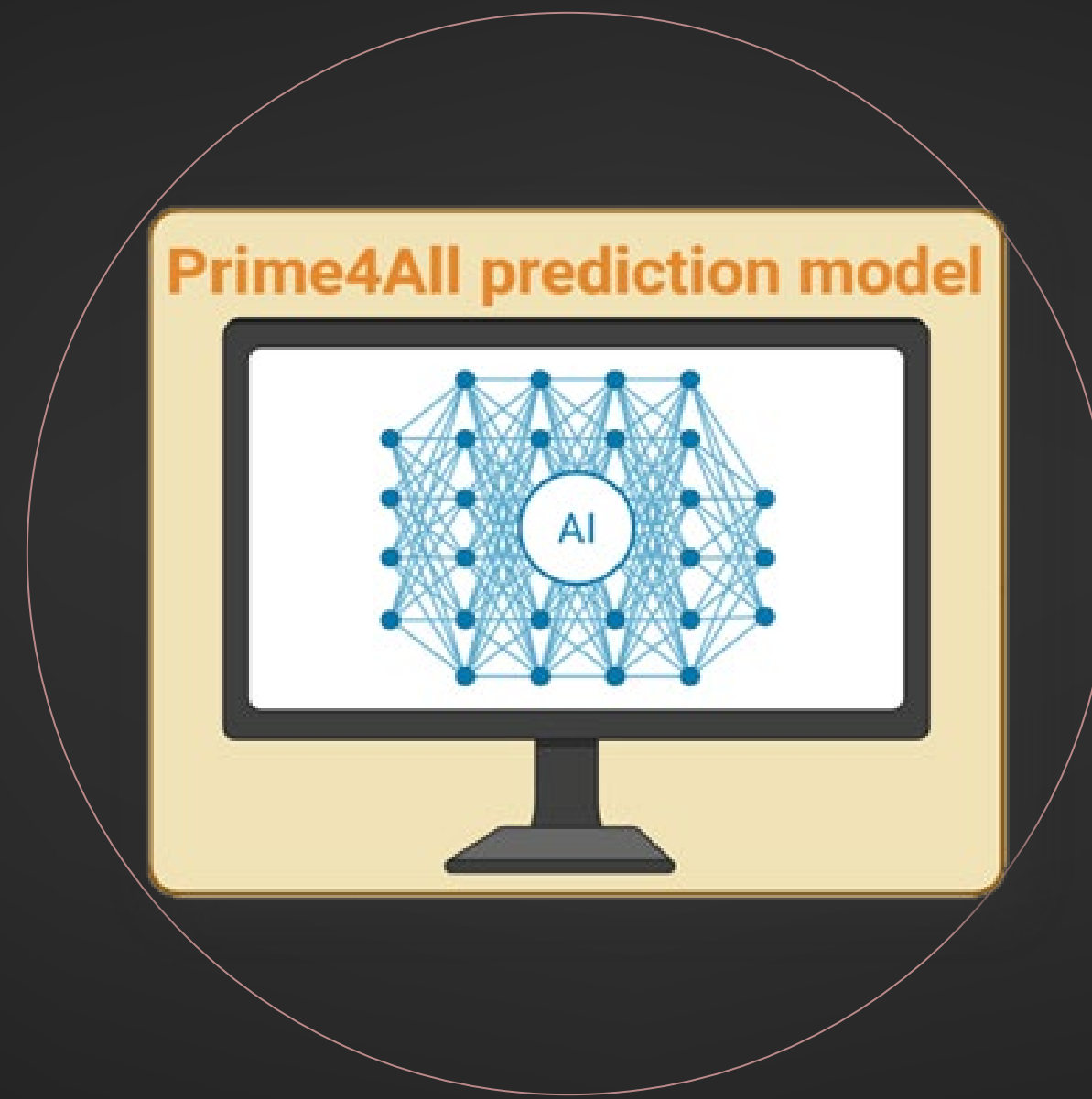
Restoring Copper Homeostasis: Prime Medicine's Path to Developing Transformative Therapies for Wilson Disease

November 12, 2025

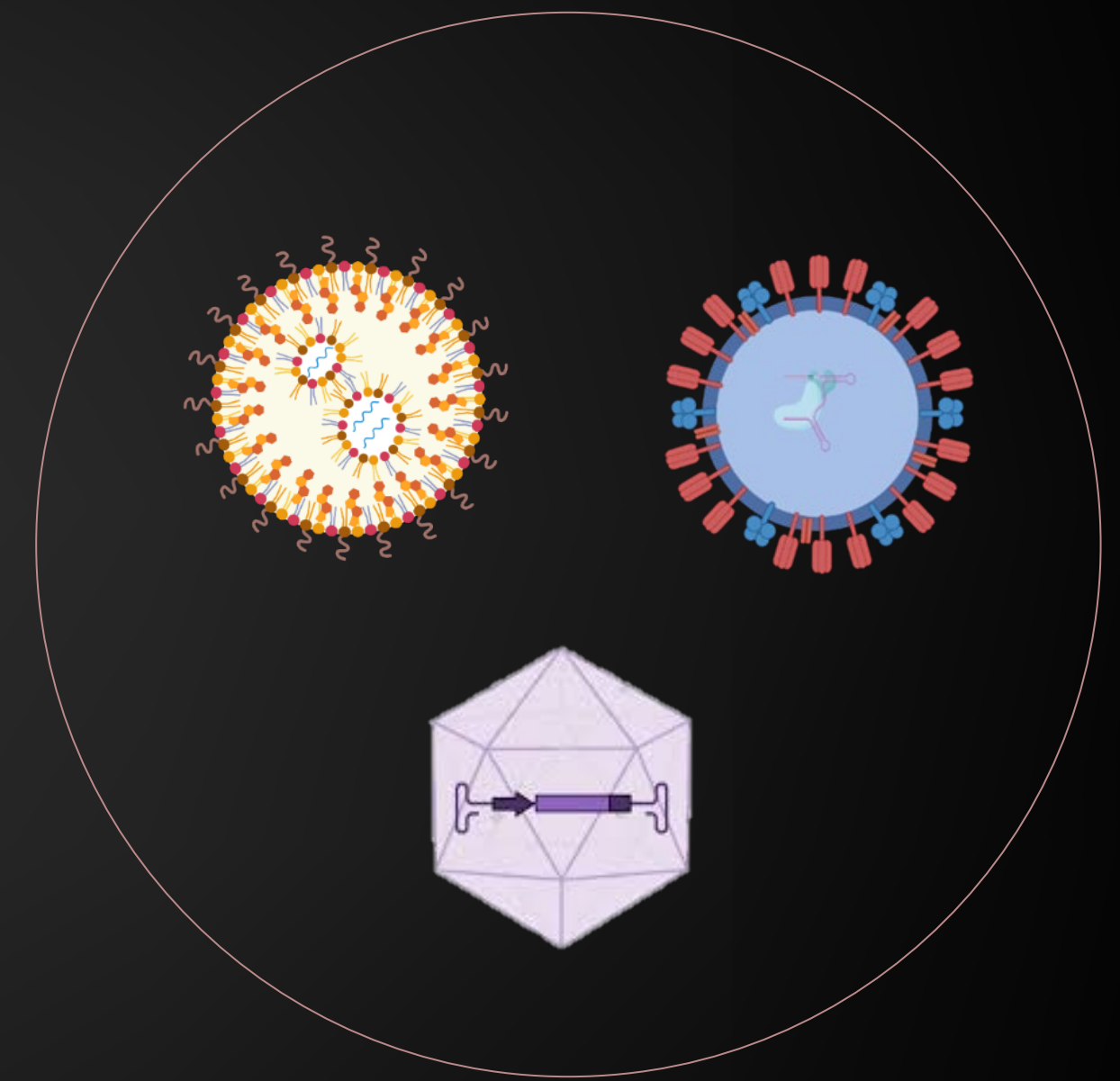
What are we working on now?



The Dutch KJ
Metakids: GREAT
NWA-ORC ARREST



Prime4All
Abeln group
ERC-Consolidator?



Organ targeting
ZonMW VICI



And how to develop therapies sustainably



Preclinical platform



For whom? Affordable?

Home > Topics > About MEB > Our Work > Science

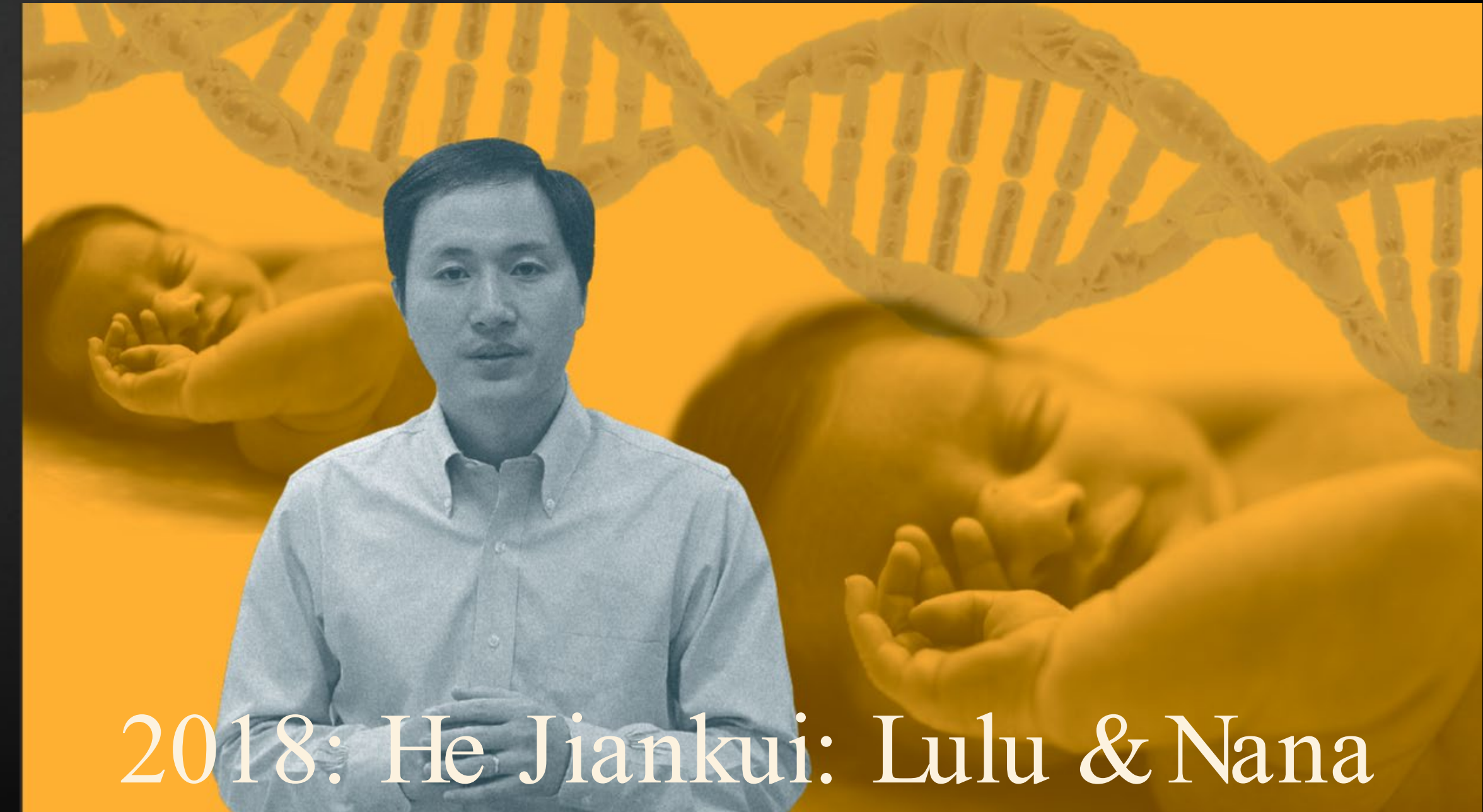
MEB-CCMO Science Day 2026 - Theme and programme



Towards a Framework for N-of-1 Individualized Therapies in the Netherlands

Recent developments in individualized medicine have challenged traditional approaches to the development, evaluation, and regulation of therapies. High-profile examples such as milasen and other N-of-1 treatments, including the case of Baby KJ, illustrate both the potential of highly tailored therapies and the complexity of bringing them responsibly to patients within existing regulatory and healthcare frameworks.

Regulatory platform



2018: He Jiankui: Lulu & Nana

How far?

How to balance diligence versus speed?





PRIME TIME



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European Research Council
Established by the European Commission



METAKIDS

