

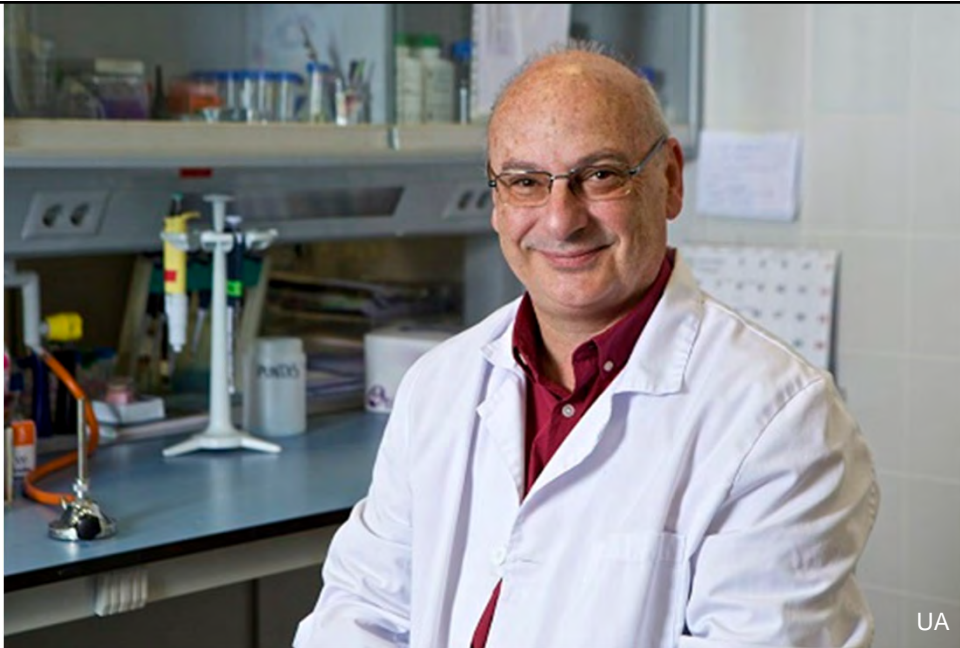
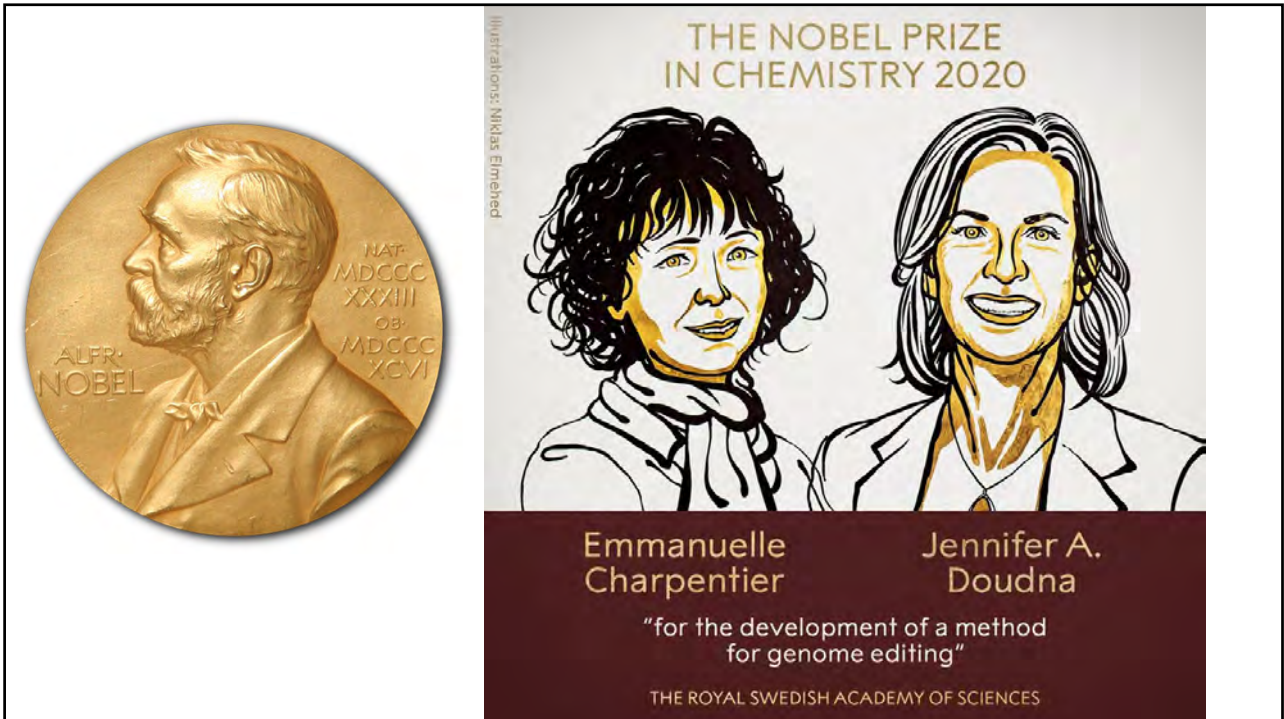
Medicina personalizada desde la edición genética

**@LluisMontoliu**

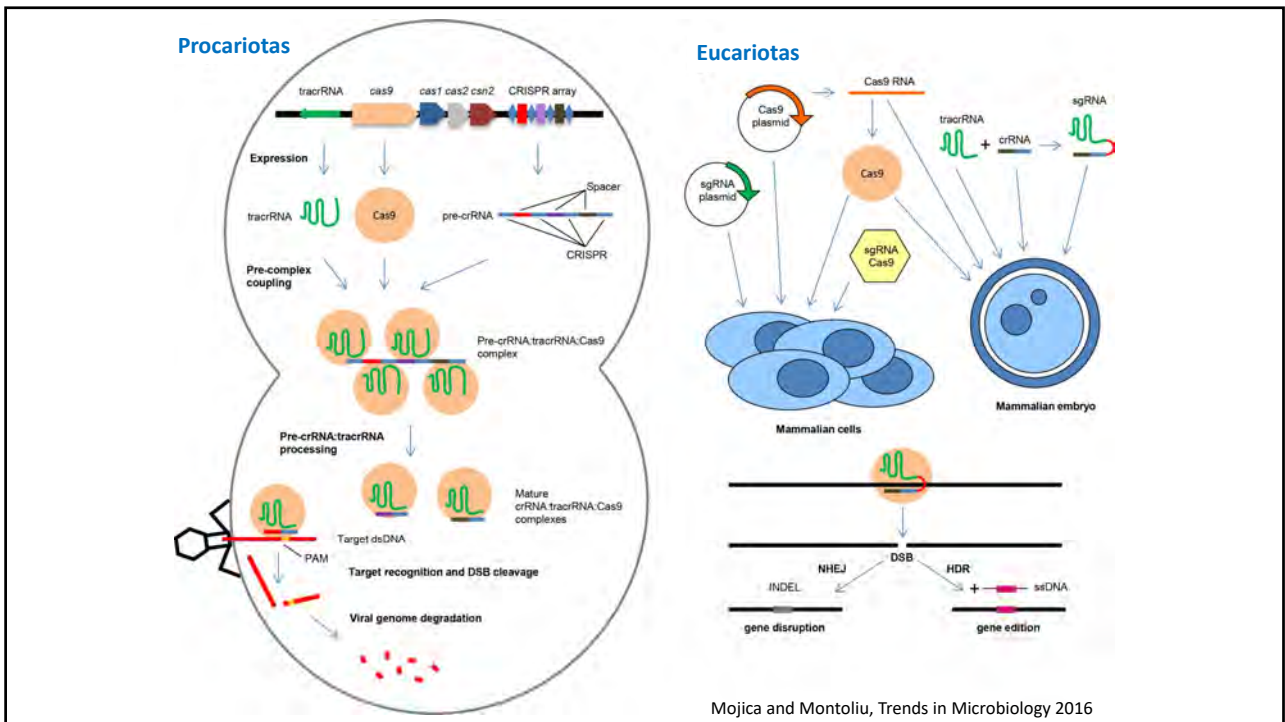
CNB-CSIC & CIBERER-ISCIII, Madrid



SINC






**Francisco Juan Martínez Mojica**



1993

↓

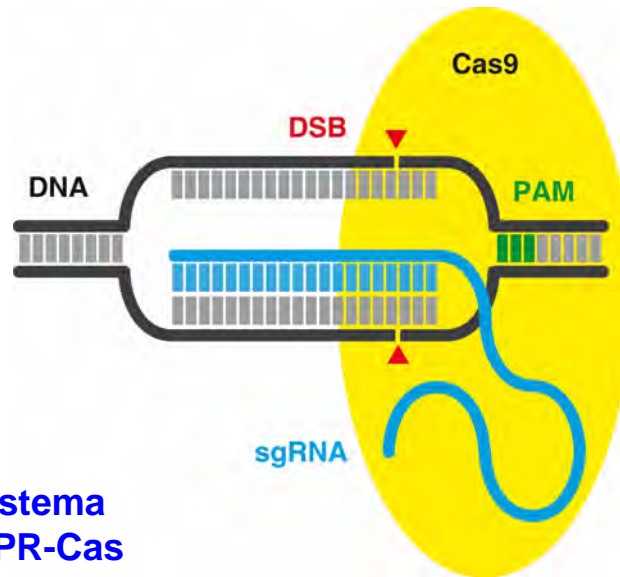
2013

 Francisco Mojica University of Alicante, Spain	 Rodolphe Barrangou North Carolina State Univ, Raleigh, USA	 Philippe Horvath Duke University Medical Center, Durham, USA	 Luciano Marraffini The Rockefeller Univ, New York, USA
 John van der Oost Radboud University, The Netherlands	 Emmanuelle Charpentier Inst für Infekt. Biol, Berlin, Germany	 Jennifer Doudna Univ California Berkeley, CA, USA	 Virgílius Sikorskýs Vilnius University, Lithuania
 Feng Zhang Broad/MIT, Cambridge, MA, USA	 George Church Harvard Med School, Boston, MA, USA	 Rudolf Jaenisch Wellcome Trust, Cambridge, MA, USA	 J. Keith Joung Mass General, Charlestown, MA, USA

  
David R. Liu  
BROAD-MIT, USA



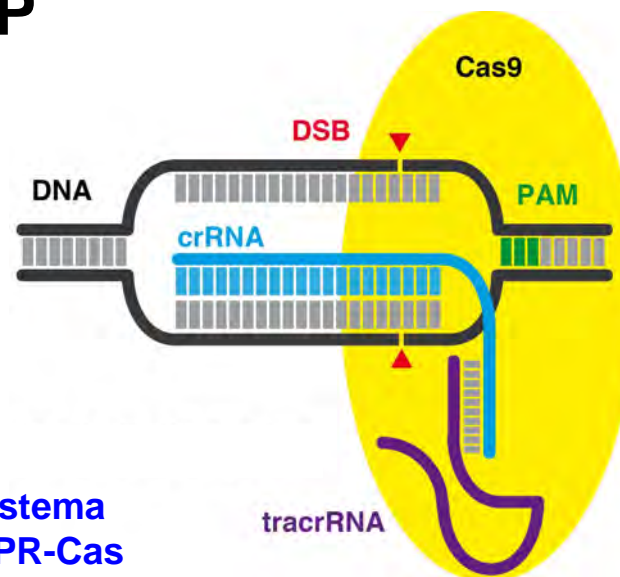
2012



El sistema  
CRISPR-Cas  
original

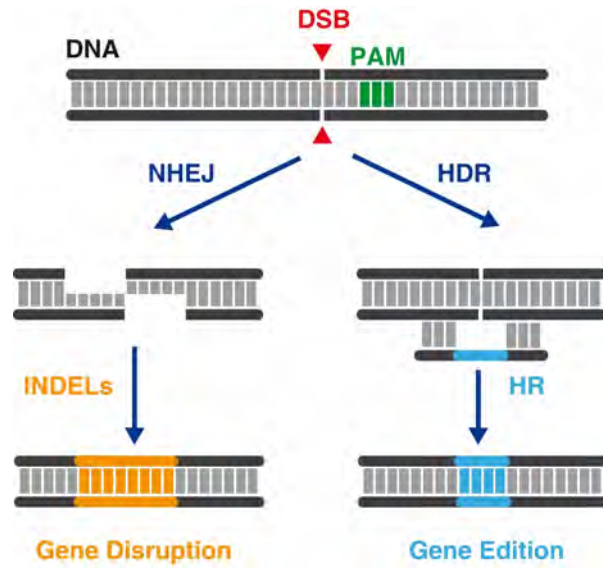
2021

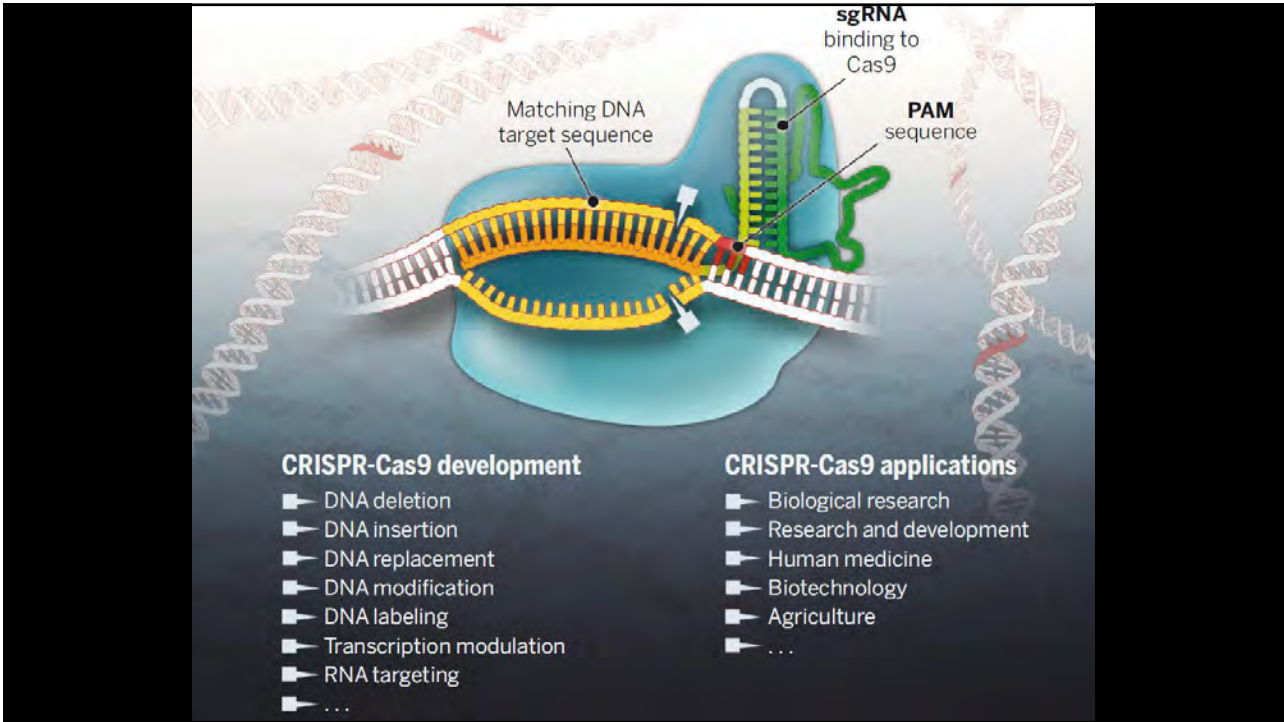
## RNP



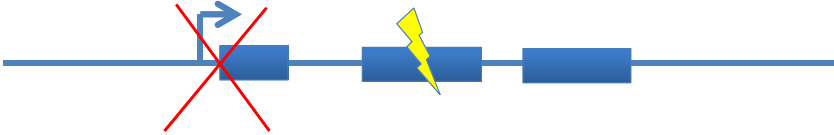
El sistema  
CRISPR-Cas  
hoy en día

### Fixing the DSB: NHEJ vs HDR

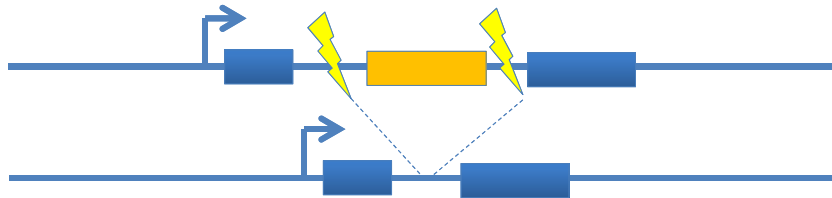




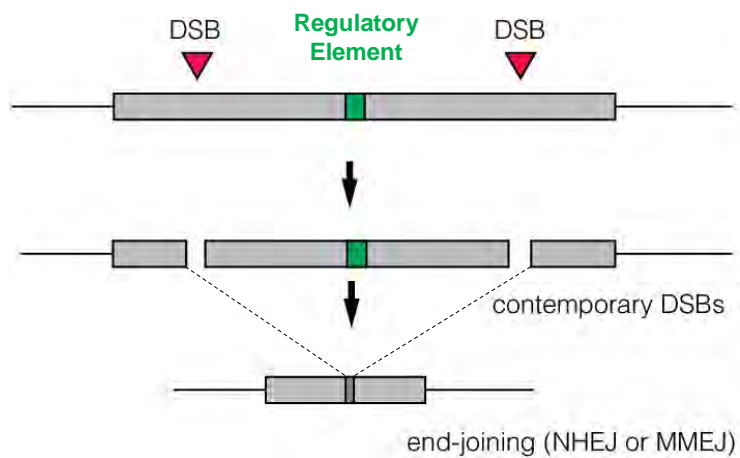
# Disrupting a gene: KO



## Deletions



### Using CRISPR-Cas9 genome editing to target *Tyr* regulatory elements

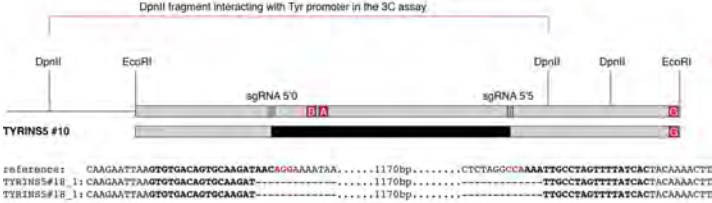


### CRISPR-Cas9 genome editing

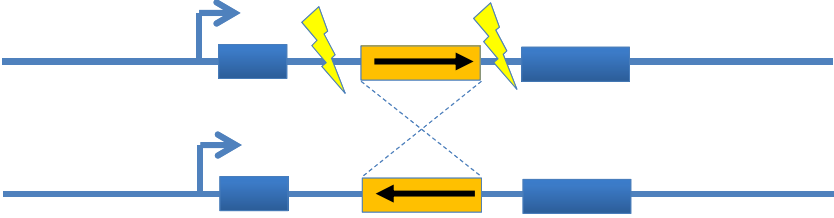
# Deleting 5' Tyr regulatory elements with CRISPRs *in vivo* results in hypopigmentation



Seruggia et al. 2015 Nucleic Acids Research  
 Seruggia et al. 2020 Scientific Reports  
 Seruggia et al. 2021 Pigment Cell & Melanoma Res.

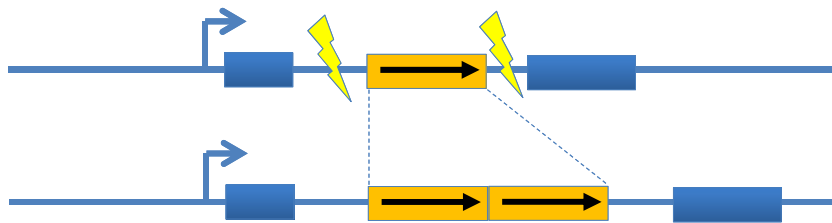


# Inversions

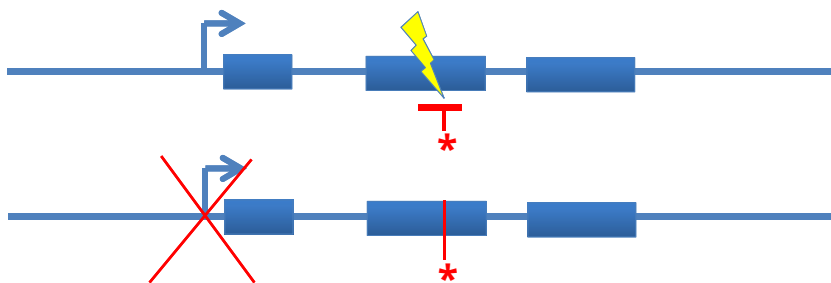




## Duplications



## Point mutations



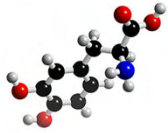
## AVATAR CRISPR mice

- Easier approach to reproduce human mutations in animal models



OCA4 (*SLC45A2*) c.986delC

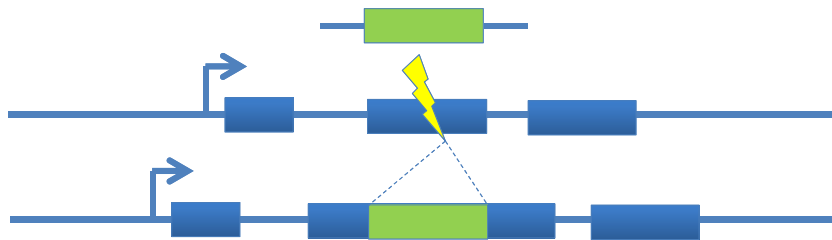
Patty

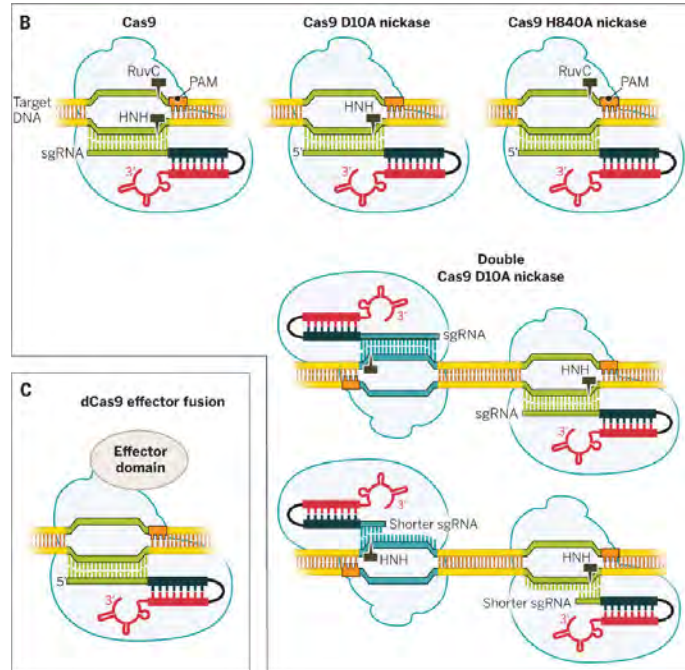


## Validating treatments



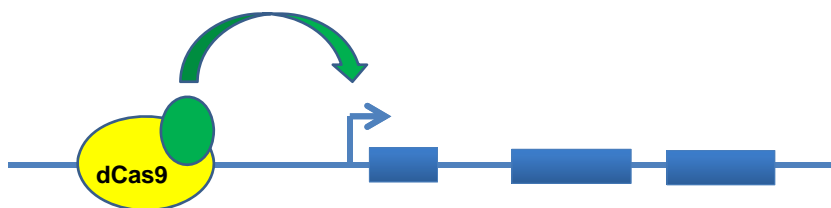
## Knock-ins



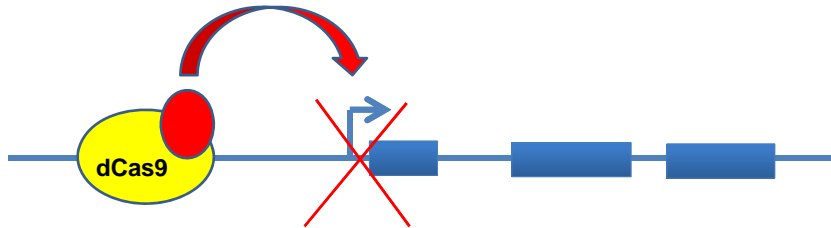


Doudna & Charpentier (2014) Science

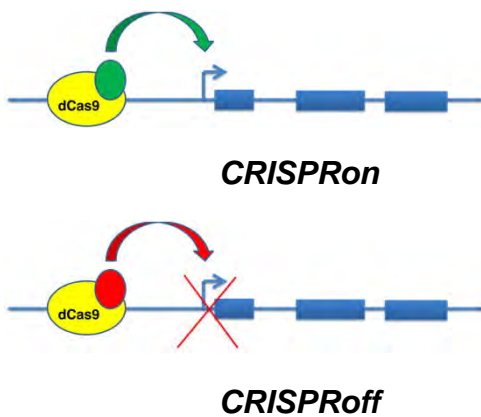
## Activating a gene



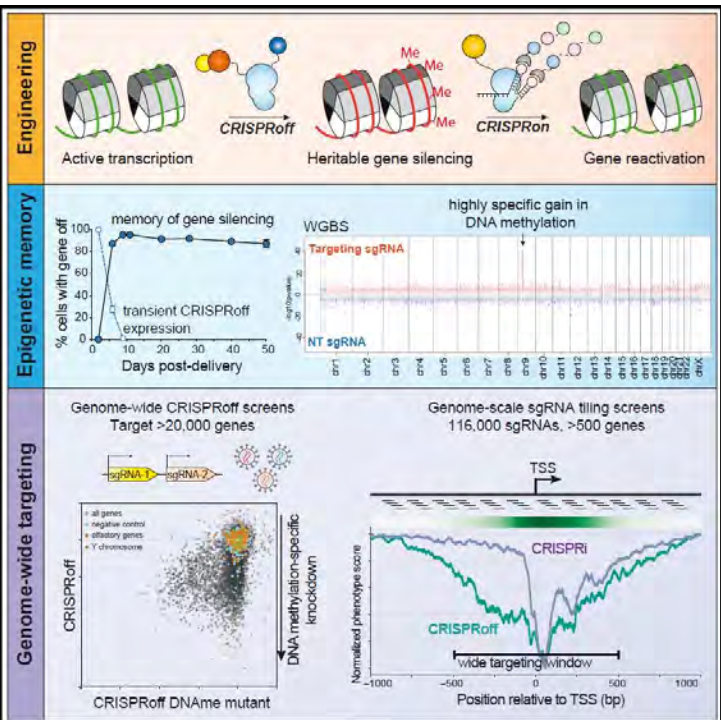
# Inactivating a gene



## Switching ON-OFF



Núñez *et al.* Cell (2021)

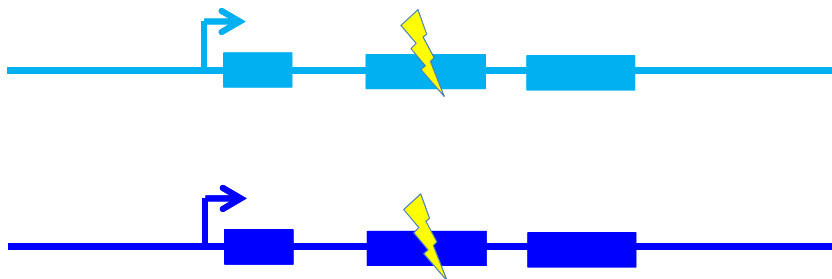




## Current limitations of CRISPR-Cas9 tools



## Inactivating similar genes



**off target effects**

## >100 programs available for designing sgRNA

**WeReview: CRISPR Tools**

**Summary:**  
This is a systematic review that anyone can edit. It is focused on bioinformatic tools for CRISPR/Cas experiments. Participate by proposing any modifications to the table! Just double-click on any row, modify the desired fields and click "Submit" (all submissions are manually checked by our staff).  
If you are the author of any CRISPR tool already in the table, please consider using this page to keep always updated its most relevant features.  
The information provided on this website has been compiled by a number of collaborators and has been curated by settings that to the best of our knowledge and belief, it is provided "as is" without express or implied warranty for the accuracy or usefulness of such content. Feedback: [weare@pilot.csi.csi.ac.nz](mailto:weare@pilot.csi.csi.ac.nz)

**Instructions:**  
- Click any header to filter rows by the corresponding field. For example, in Citations filter box, write "> 40" and click "Apply" to get all tools with more than 40 citations in PubMed.  
- Sort rows by clicking ▲ or ▼ arrows next to any header field. Depending on the column, rows will become sorted alphabetically, numerically or by date.  
- Double-click any row to start editing its content. A detailed view of the contents of the element will appear, with easy-to-follow indicators to modify and submit any field(s).  
- Have you found any new tool? Click [Add New CRISPR Tool](#).  
- Additional help: [help](#)

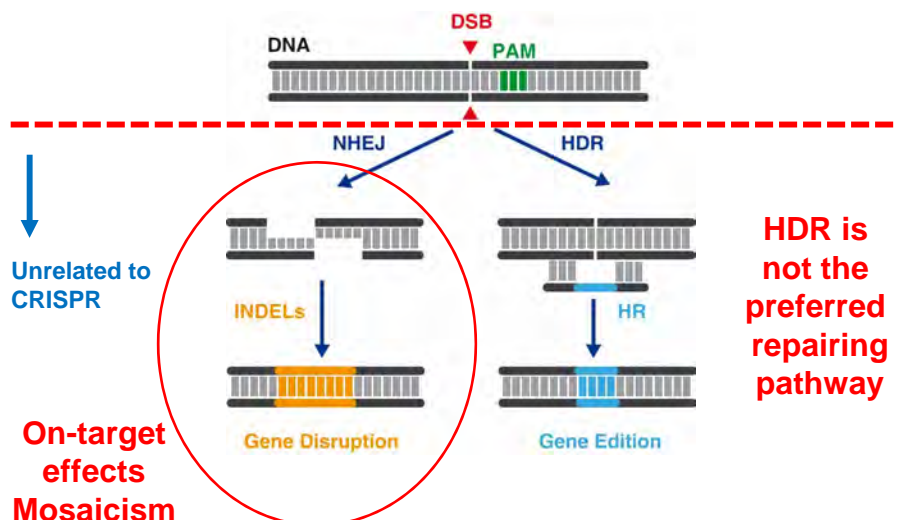
Showing 100 of 100 elements

Tool	Assessment	Platform	Access	Open Access	Search by	Encylope	PAM	Operations	Downloads	Citations
sgRNAcas9	?	Oligo Designer	Command-line	X	✓	Sequence	Custom	Custom	Provided by user	Xie S, Shen B, Zhang designing CRISPR sg Cite: 2014 Jan 23;95 (PMID: 2455336)
CRISPRdirect	✓	Oligo Designer	Web	✓	X	GeneID Coordinate Sequence	Custom	Custom	Amber Chen (University of Arizona) mma@uic.edu Submitted to CRISPR list Admin team (Vigna, Angarini) through the CRISPR	Miao Y, Luo K, Song guide RNA with reduced to 100 bioinformatic
CRISPR Design Tool	✓	Oligo Designer	Web	✓	X	GeneID Coordinate Sequence	Custom	Custom	American Algal (M&M) All Alpha (M&M) M&M R Car (M&M) R&M R&M Chosen (M&M) R&M R&M	

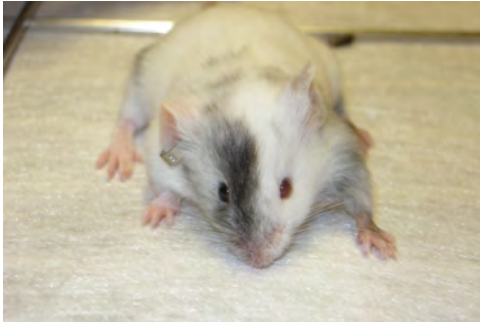
## WeReview: CRISPR Tools

Torres-Perez *et al.* Bioengineering 2019

## on target effects - mosaicism



# On-targets: the real problem



- Founder animals are nearly always complex mosaic
- Many different alleles can be present
- Not all of them might transmit through germline



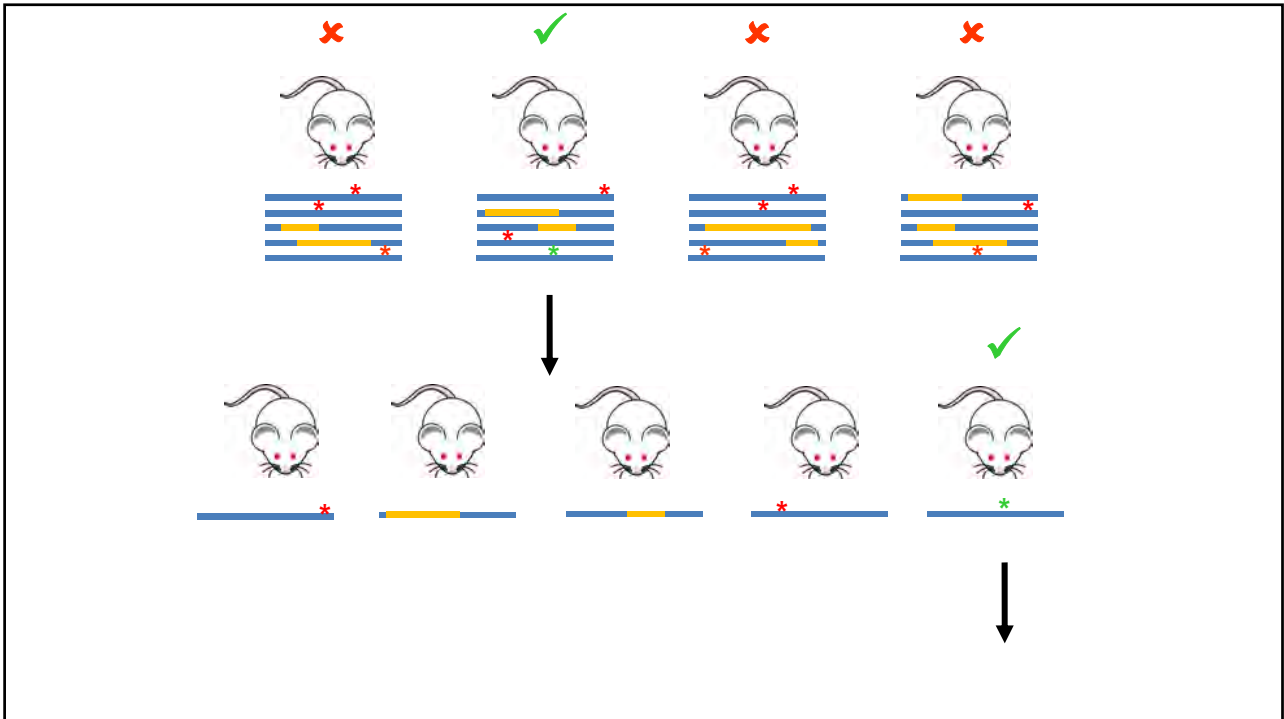
One 8-cell embryo = 16 possible alleles

## ON-TARGET genetic variations are the main challenge

Diagram showing a DNA sequence with a red arrow labeled "sgRNA-A4/6" and a green line labeled "ssDNA" below it.

```

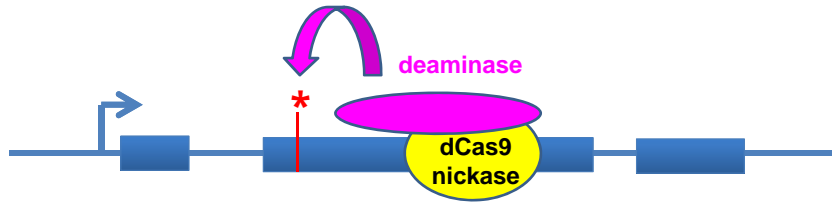
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B9040.3 AACATTGGAGGAGCTGCCACTGCTATTGGGGACCCACCAAATGTTATCATTTGTTCCAAATCAGGAGTTGAGAAAAATGGTAG-----GTAGGGTTGATTTTCAGGAAATGTAA
B9040.4 AACATTGGAGGAGCTGCCACTGCTATTGGGGACCCACCAAATGTTATCATTTGTTCCAAATCAGGAGTTGAGAAAAATGGTAGTTAACAGC-----AGGGTTGATTTTCAGGAAATGTAA
B9040.5 CAAGCTCTGCCCACTTTCAAAGCTGTAAGCTGCAAGTTCCTTCCACCACAGATTCCTGCAAGACTTGCACCGGG----- (437bp) -----
B9040.6 ----- (561bp) -----
B9041.1 AACATTGGAGGAGCTGCCACTGCTATTGGGGACCCACCAAATGTTATCATTTGTTCCAAATCAGGAGTTGAGAAAAATGGTAGTTAACAGCTGTTACCTAGGGTTGATTTTCAGGAAATGTAA
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## Solutions to the limitations of CRISPR-Cas9 genome editing tools



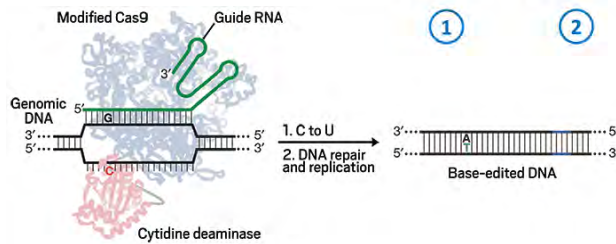
# Base editing a gene



David Liu Lab

## CRISPR-derived BASE EDITORS (BE3) CBE and ABE

**CBE:** C → U → T  
**ABE:** A → I → G



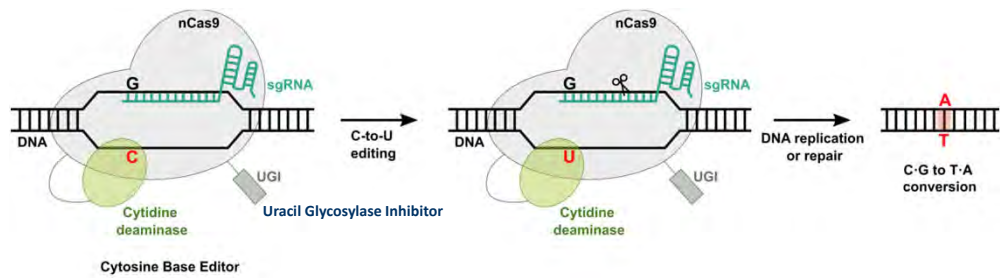
Komor et al. Nature 2016



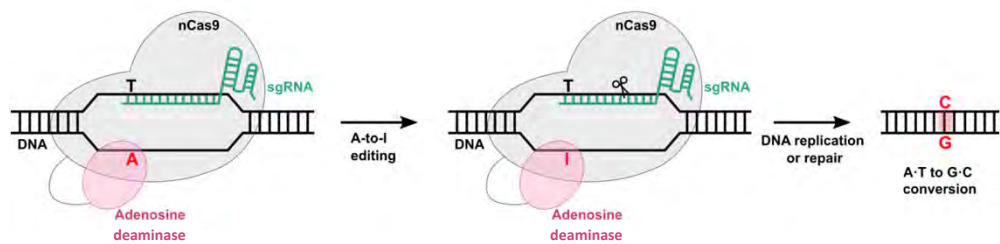


AGGATTAGAGC **C** GATAGCATACGATCAGTACGAT

**CBE**



**ABE**



<https://biotech.ucdavis.edu/news/dna-base-editors-genome-editing>

# New base editors: CGBE C→G

nature  
biotechnology

ARTICLES

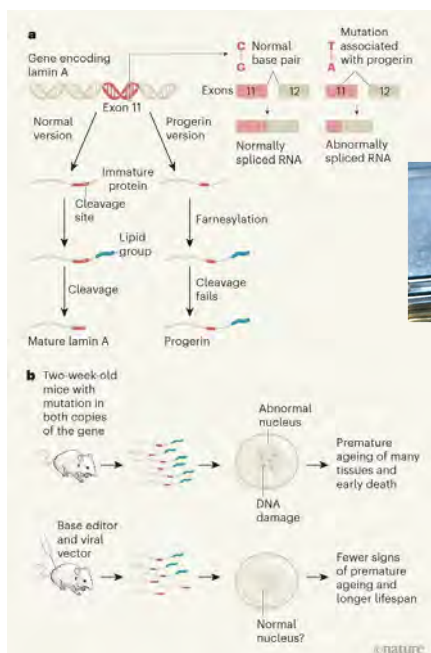
<https://doi.org/10.1038/s41587-021-00938-z>

Check for updates

## Efficient C→G-to-G→C base editors developed using CRISPRi screens, target-library analysis, and machine learning

Luke W. Koblan<sup>1,2,3,15</sup>, Mandana Arbab<sup>1,2,3,15</sup>, Max W. Shen<sup>1,2,3,4,15</sup>, Jeffrey A. Hussmann<sup>5,6,7,13,14</sup>, Andrew V. Anzalone<sup>1,2,3</sup>, Jordan L. Doman<sup>1,2,3</sup>, Gregory A. Newby<sup>1,2,3</sup>, Dian Yang<sup>5,7,13,14</sup>, Beverly Mok<sup>1,2,3</sup>, Joseph M. Replogle<sup>5,7,8,9,13,14</sup>, Albert Xu<sup>5,6,8,10</sup>, Tyler A. Sisley<sup>2</sup>, Jonathan S. Weissman<sup>5,7,8,13,14</sup>, Britt Adamson<sup>5,7,11,12</sup> and David R. Liu<sup>1,2,3</sup>

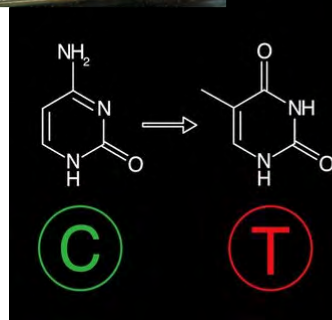
Koblan et al. Nat. Biotech. (2021)



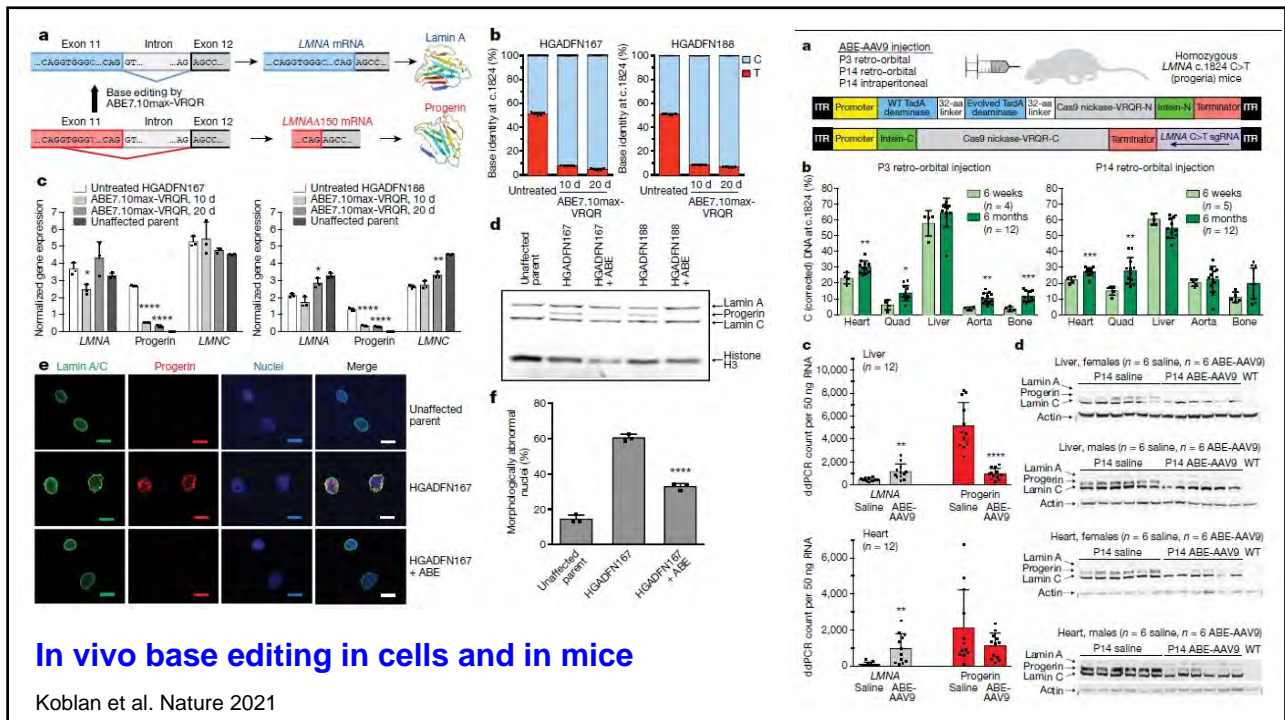
Article

## In vivo base editing rescues Hutchinson-Gilford progeria syndrome in mice

Luke W. Koblan<sup>1,2,3,15</sup>, Michael R. Erdos<sup>15</sup>, Christopher Wilson<sup>1,13</sup>, Wayne A. Cabral<sup>4</sup>, Jonathan M. Levy<sup>1,2,3</sup>, Zheng-Mei Xiong<sup>1</sup>, Urraca L. Tavares<sup>2</sup>, Lindsay M. Davison<sup>2</sup>, Yantao G. Ge<sup>2,4</sup>, Xiaojing Mao<sup>2</sup>, Gregory A. Newby<sup>1,2,3</sup>, Sean P. Doherty<sup>2</sup>, Nariisu Narisu<sup>4</sup>, Qianhu Sheng<sup>2</sup>, Chad Krilow<sup>4</sup>, Charles Y. Liu<sup>1,13</sup>, Leslie B. Gordon<sup>10,11</sup>, Kan Cao<sup>2</sup>, Francis S. Collins<sup>12</sup>, Jonathan D. Brown<sup>12</sup> & David R. Liu<sup>1,2,3,15</sup>



Koblan et al. Nature 2021



Article

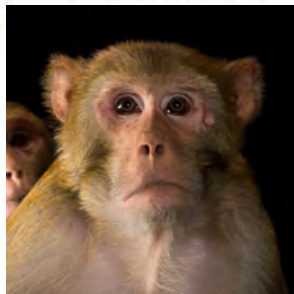
# In vivo CRISPR base editing of *PCSK9* durably lowers cholesterol in primates

<https://doi.org/10.1038/s41586-021-03534-y>

Received: 6 December 2020

Accepted: 11 April 2021

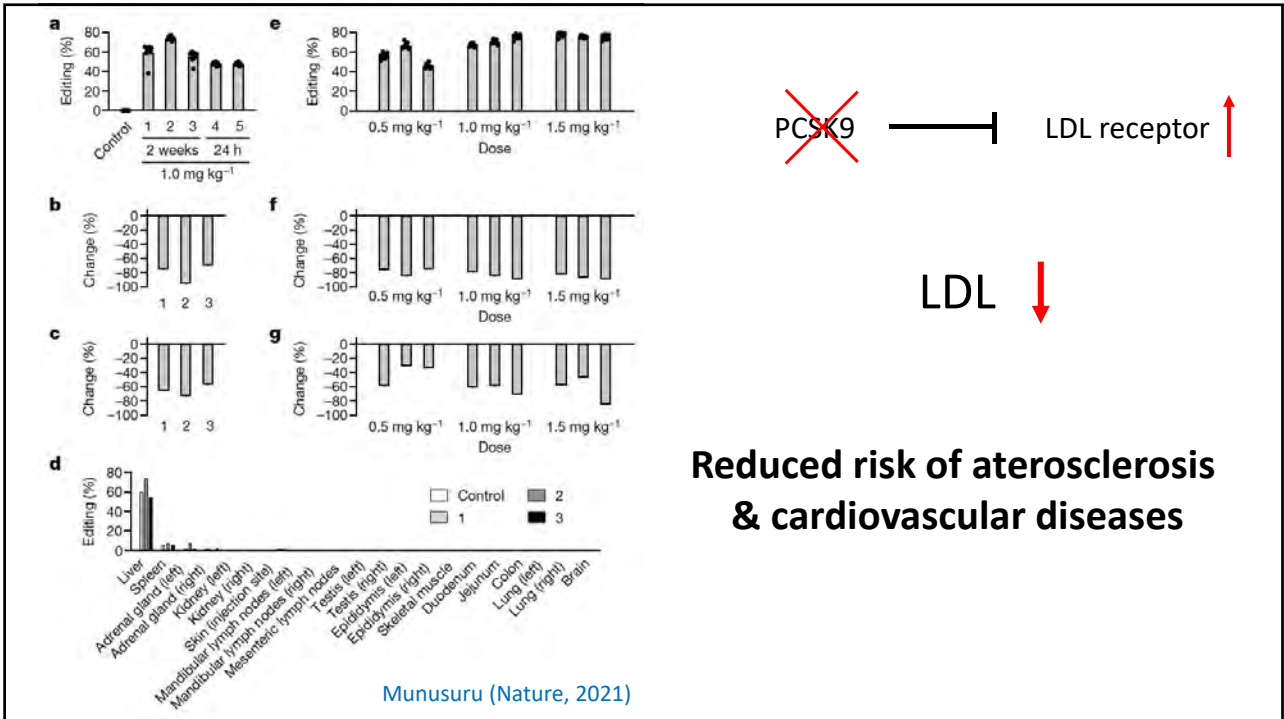
Published online: 19 May 2021



Kiran Musunuru<sup>1,2,3</sup>, Alexandra C. Chadwick<sup>4</sup>, Taiji Mizoguchi<sup>4</sup>, Sara P. Garcia<sup>4</sup>, Jamie E. DeNizio<sup>4</sup>, Caroline W. Reiss<sup>4</sup>, Kui Wang<sup>4</sup>, Sowmya Iyer<sup>4</sup>, Chaitali Dutta<sup>4</sup>, Victoria Clendaniel<sup>4</sup>, Michael Amaonye<sup>4</sup>, Aaron Beach<sup>4</sup>, Kathleen Berth<sup>4</sup>, Souvik Biswas<sup>4</sup>, Maurine C. Braun<sup>4</sup>, Huei-Mei Chen<sup>4</sup>, Thomas V. Colace<sup>4</sup>, John D. Ganey<sup>4</sup>, Soumyashree A. Gangopadhyay<sup>4</sup>, Ryan Garrity<sup>4</sup>, Lisa N. Kasiewicz<sup>4</sup>, Jennifer Lavoie<sup>4</sup>, James A. Madsen<sup>4</sup>, Yuri Matsumoto<sup>4</sup>, Anne Marie Mazzola<sup>4</sup>, Yusuf S. Nasrullah<sup>4</sup>, Joseph Nneji<sup>4</sup>, Huilan Ren<sup>4</sup>, Athul Sanjeev<sup>4</sup>, Madeleine Shay<sup>4</sup>, Mary R. Stahley<sup>4</sup>, Steven H. Y. Fan<sup>5</sup>, Ying K. Tam<sup>5</sup>, Nicole M. Gaudelli<sup>6</sup>, Giuseppe Ciarrella<sup>6</sup>, Leslie E. Stolz<sup>4</sup>, Padma Malysala<sup>4</sup>, Christopher J. Cheng<sup>4</sup>, Kallanthottathil G. Rajeev<sup>4</sup>, Ellen Rohde<sup>4</sup>, Andrew M. Bellinger<sup>4</sup> & Sekar Kathiresan<sup>4</sup>✉

Nature (2021)





### CRISPR genome editing and mitochondria



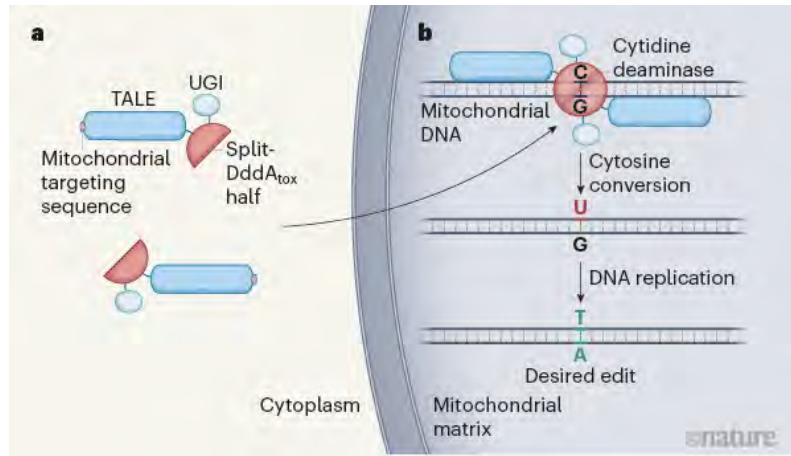
Article | Published: 08 July 2020

## A bacterial cytidine deaminase toxin enables CRISPR-free mitochondrial base editing

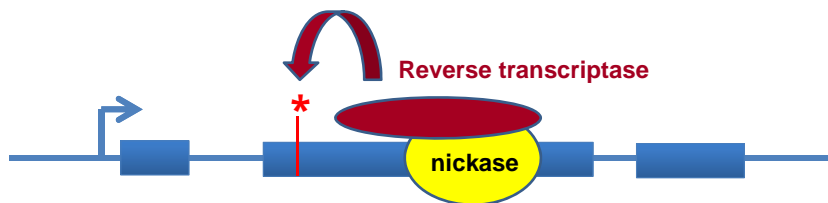
Beverly Y. Mok, Marcos H. de Moraes, Jun Zeng, Dustin E. Bosch, Anna V. Kotrys, Aditya Raguram, FoSheng Hsu, Matthew C. Radey, S. Brook Peterson, Vamsi K. Mootha, Joseph D. Mougous & David R. Liu

Nature 583, 631–637(2020) | Cite this article

July 2020

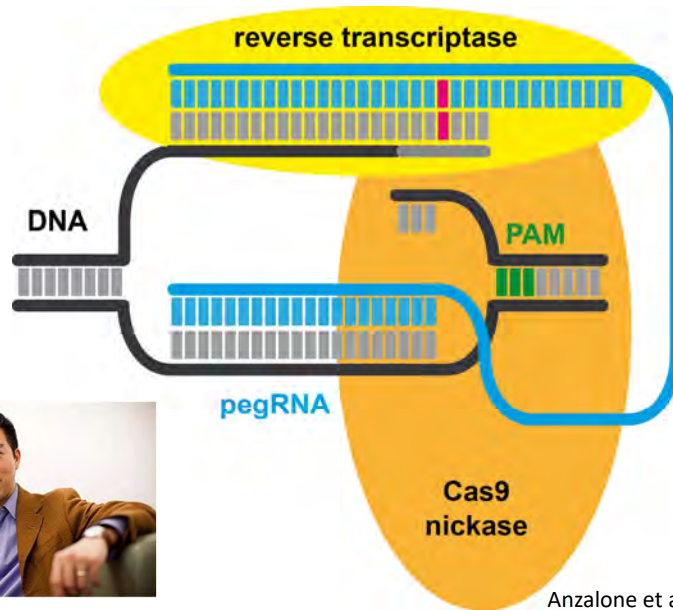


## Prime editing a gene





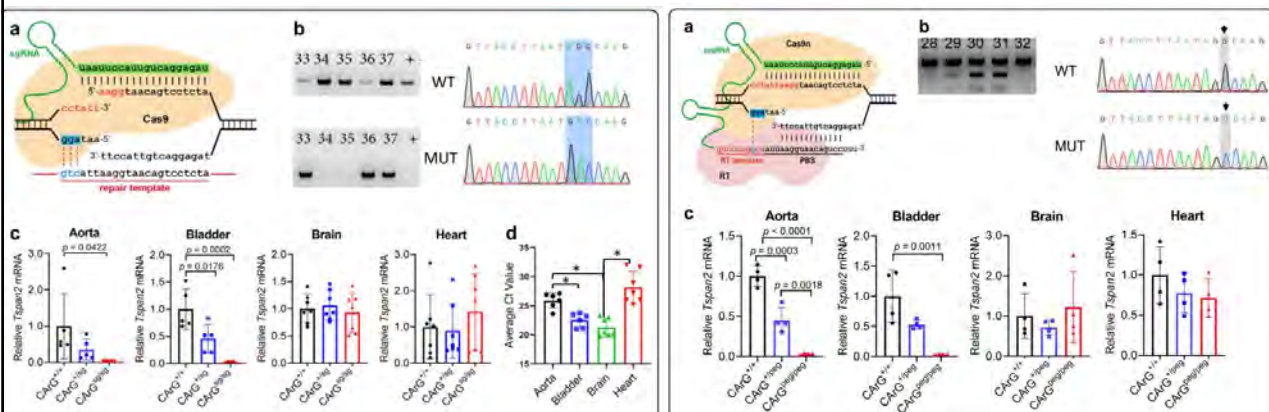
# Prime editing a gene



David Liu Lab

Anzalone et al. Nature 2019

## CRISPR-Cas9 mediated gene editing vs Prime-mediated gene editing



**56% correct on-targeting**  
**40% INDELS (mosaicism)**

**21% correct on-targeting**  
**0% INDELS (mosaicism)**

Gao et al. 2021 Genome Biol

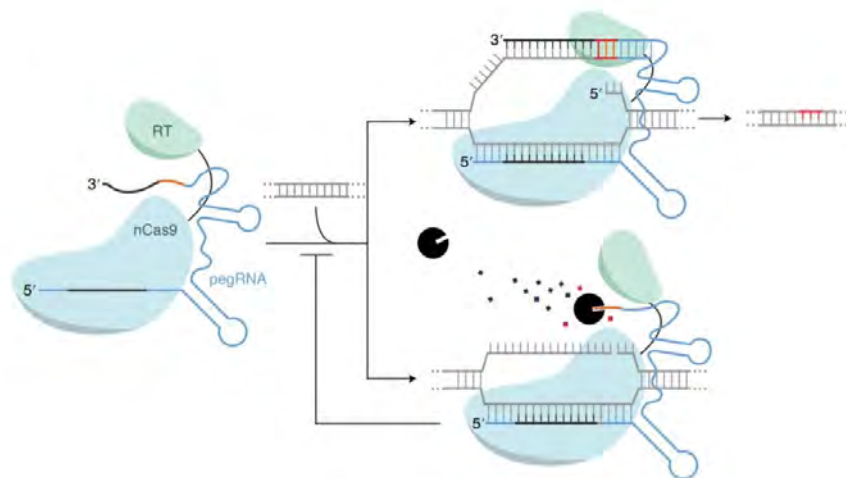
# Engineered pegRNAs improve prime editing efficiency

James W. Nelson<sup>1,2,3,4</sup>, Peyton B. Randolph<sup>1,2,3,4</sup>, Simon P. Shen<sup>1,2,3</sup>, Kelcee A. Everette<sup>1,2,3</sup>, Peter J. Chen<sup>1,2,3</sup>, Andrew V. Anzalone<sup>1,2,3</sup>, Meirui An<sup>1,2,3</sup>, Gregory A. Newby<sup>1,2,3</sup>, Jonathan C. Chen<sup>1,2,3</sup>, Alvin Hsu<sup>1,2,3</sup> and David R. Liu<sup>1,2,3</sup> 

Prime editing enables the installation of virtually any combination of point mutations, small insertions or small deletions in the DNA of living cells. A prime editing guide RNA (pegRNA) directs the prime editor protein to the targeted locus and also encodes the desired edit. Here we show that degradation of the 3' region of the pegRNA that contains the reverse transcriptase template and the primer binding site can poison the activity of prime editing systems, impeding editing efficiency. We incorporated structured RNA motifs to the 3' terminus of pegRNAs that enhance their stability and prevent degradation of the 3' extension. The resulting engineered pegRNAs (epegRNAs) improve prime editing efficiency 3–4-fold in HeLa, U2OS and K562 cells and in primary human fibroblasts without increasing off-target editing activity. We optimized the choice of 3' structural motif and developed pegLIT, a computational tool to identify non-interfering nucleotide linkers between pegRNAs and 3' motifs. Finally, we showed that epegRNAs enhance the efficiency of the installation or correction of disease-relevant mutations.

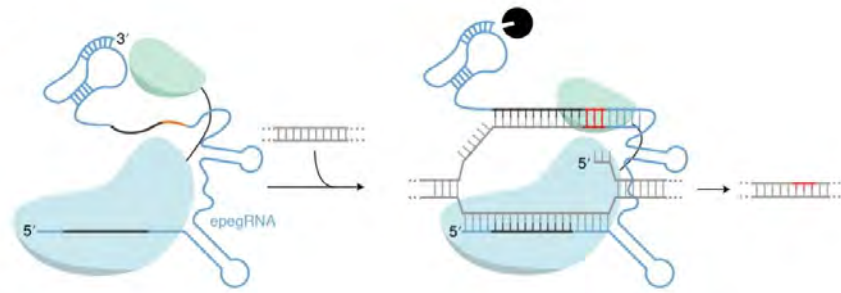
4 October 2021

## 3' degradation of pegRNA inhibits prime editing



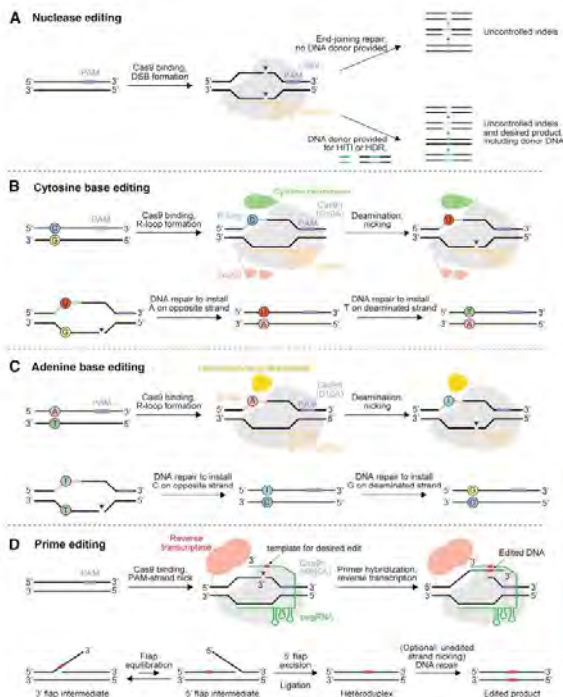
Nelson et al. (2021) Nature Biotechnology

## 3' structure protection of pegRNA enhances prime editing



### epegRNA “enhanced pegRNA”

Nelson et al. (2021) Nature Biotechnology

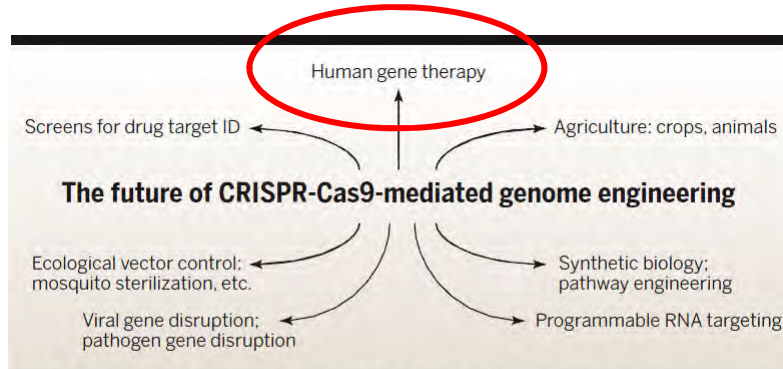


### Current CRISPR variants to be used in gene therapy approaches

- CRISPR-Cas9
- Base editors
- Prime editors

Newby & Liu, Mol. Therapy (2021)

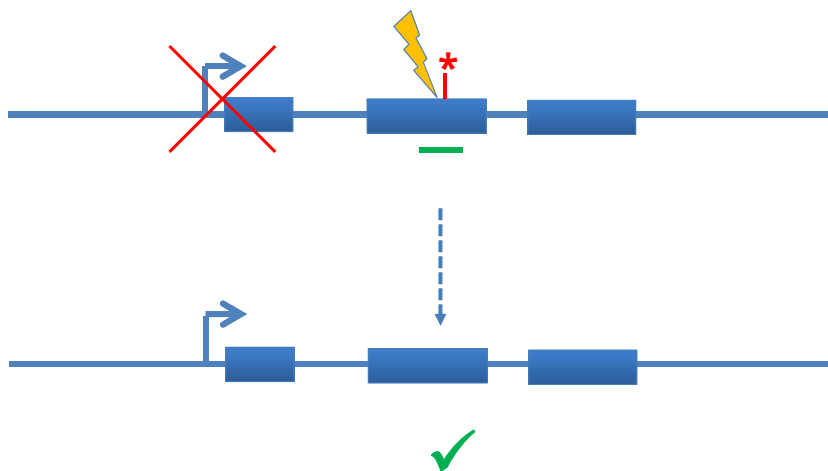
## CRISPR-Cas is the future

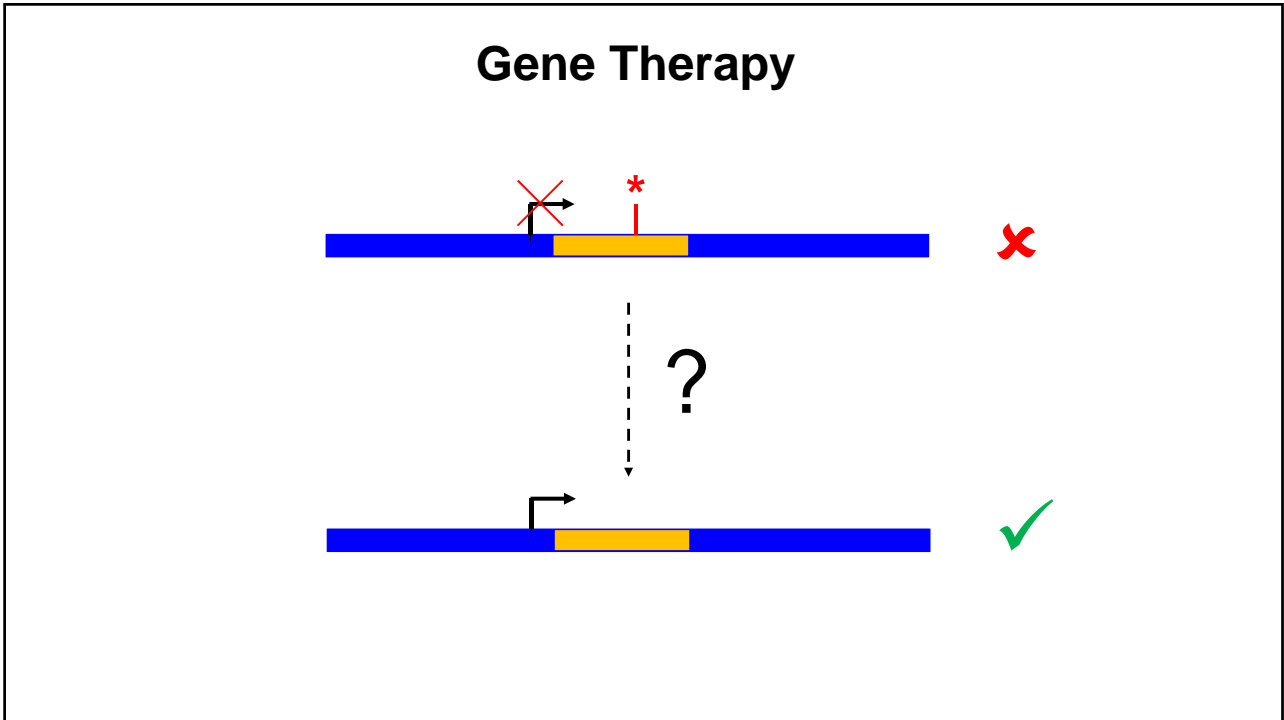
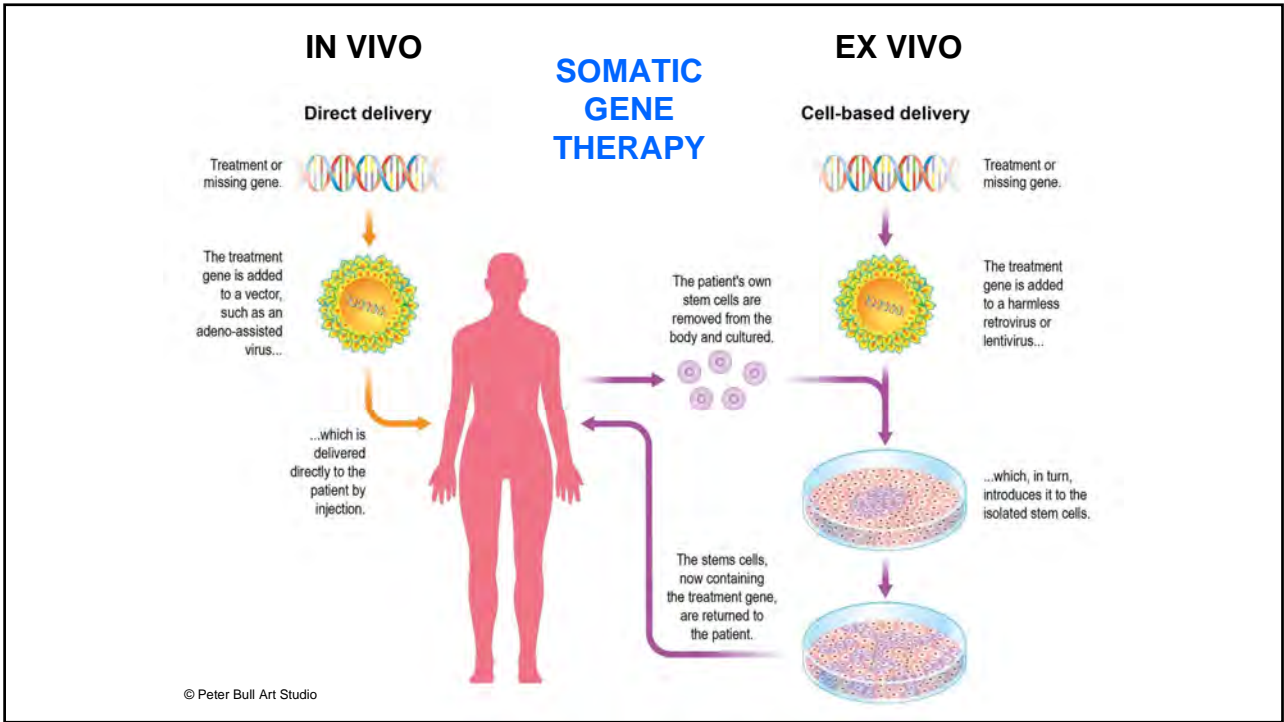


**Fig. 6. Future applications in biomedicine and biotechnology.** Potential developments include establishment of screens for target identification, human gene therapy by gene repair and gene disruption, gene disruption of viral sequences, and programmable RNA targeting.

Doudna & Charpentier (2014) *Science*

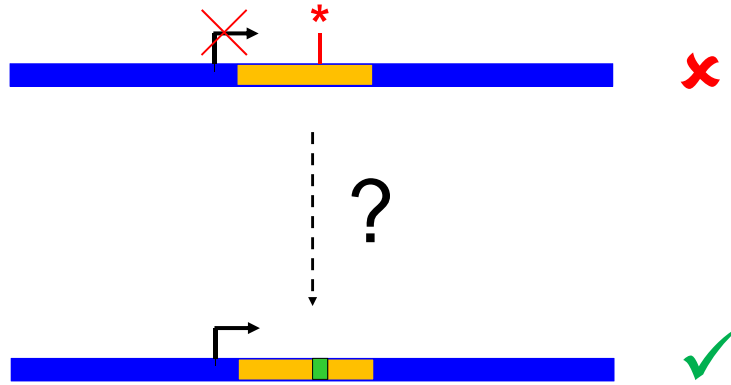
## Gene Therapy with CRISPR



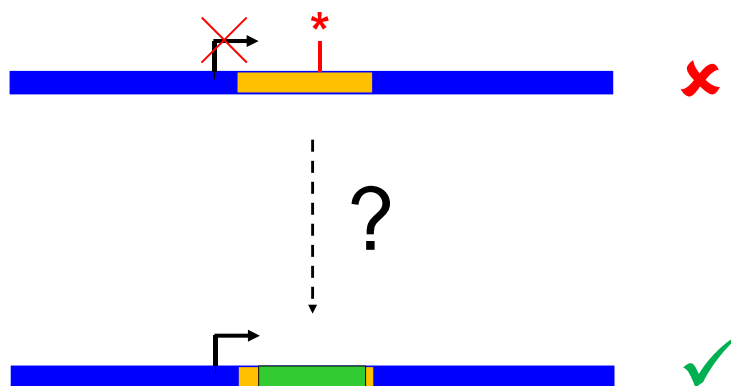




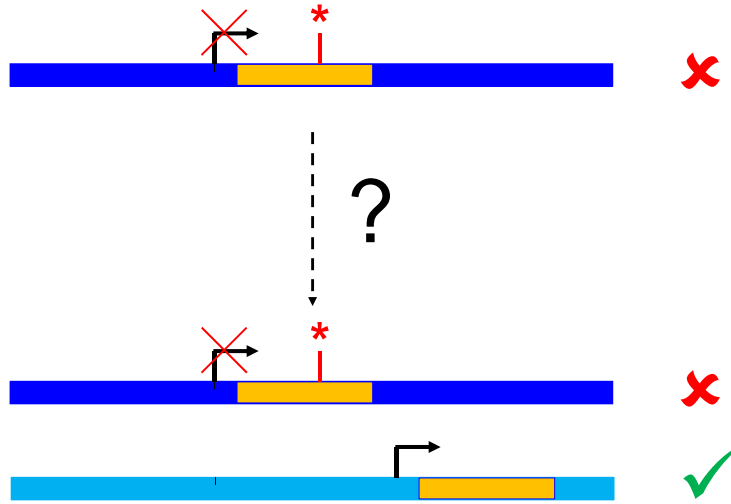
## Gene Therapy



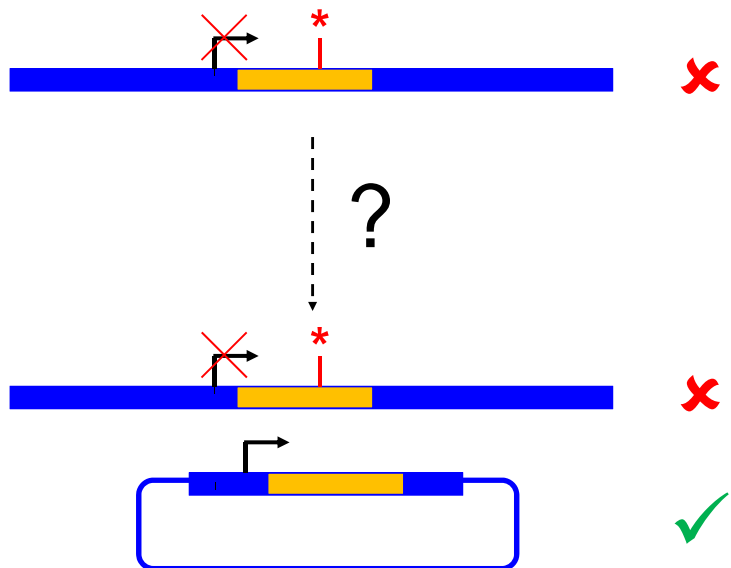
## Gene Therapy



## Gene Therapy



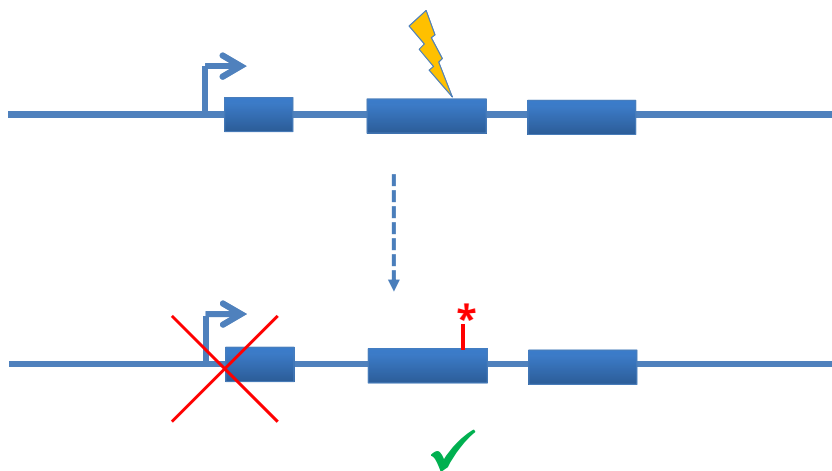
## Gene Therapy



**Luxturna (Spark Therapeutics, Inc.)**  
**Treatment of retinal degenerative diseases (RPE65 gene)**



**CRISPR gene therapy (currently)**



# 51 clinical trials today with CRISPR

U.S. National Library of Medicine  
**ClinicalTrials.gov**

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Home > Search Results

Modify Search Start Over

51 Studies found for: CRISPR

List By Topic On Map Search Details

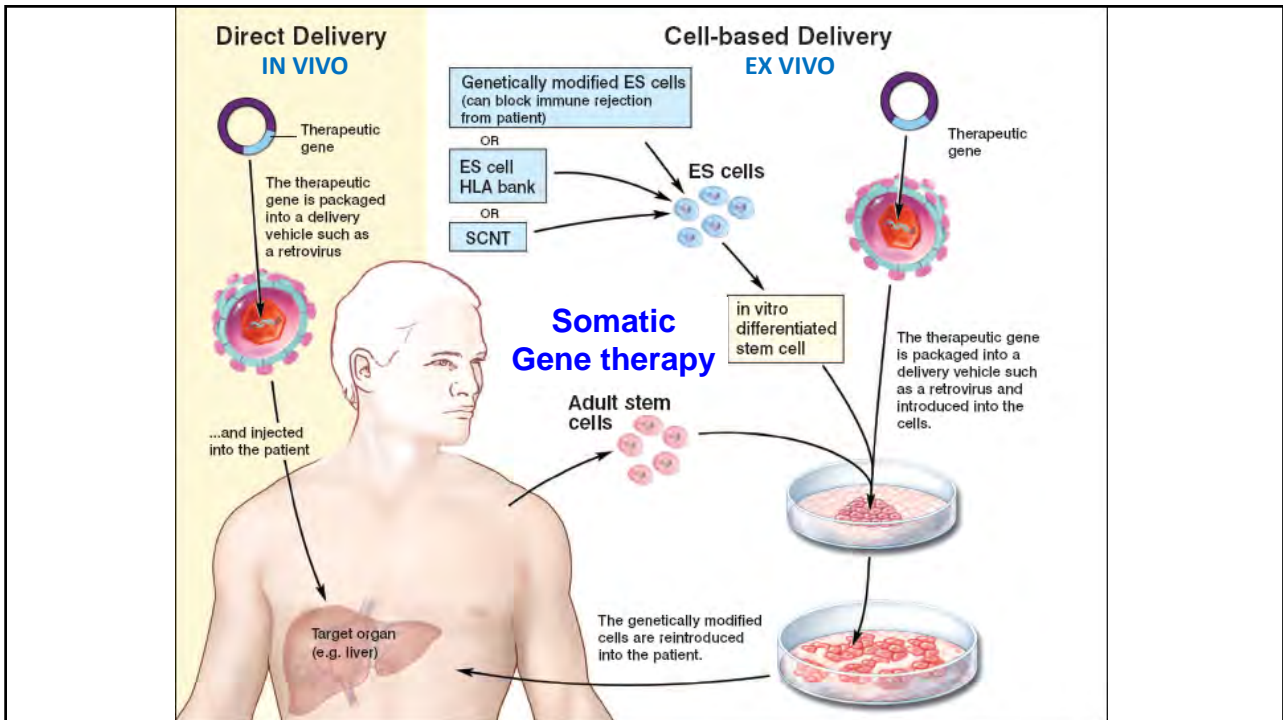
Hide Filters Download Subscribe to RSS

Showing: 1-10 of 51 studies 10 studies per page Show/Hide Columns

Row	Saved	Status	Study Title	Conditions	Interventions	Locations
1	<input type="checkbox"/>	Not yet recruiting	Transplantation of Clustered Regularly Interspaced Short Palindromic Repeats Modified Hematopoietic Progenitor Stem Cells (CRISPR_SCD001) in Patients With Severe Sickle Cell Disease	Sickle Cell Disease	Drug: CRISPR_SCD001	<ul style="list-style-type: none"> <li>University of California, Los Angeles, Los Angeles, California, United States</li> <li>UCSF Benioff Children's Hospital Oakland, California, United States</li> </ul>

Recruitment:  Not yet recruiting  Recruiting  Enrolling by invitation

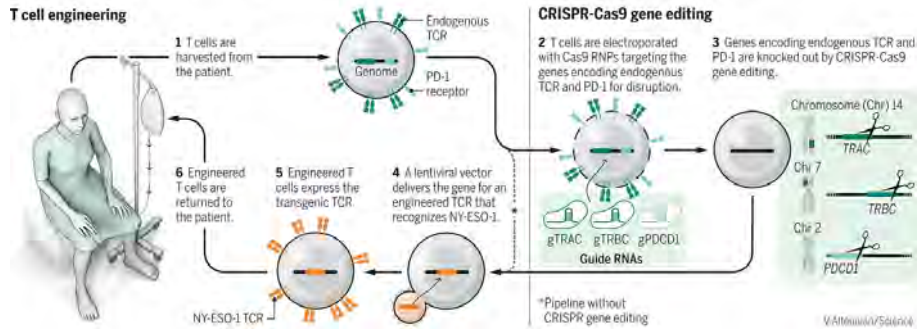
Most of them are EX-VIVO



# Cancer Immunotherapy with CRISPR

## Modifying engineered T cells with CRISPR-Cas9 gene editing

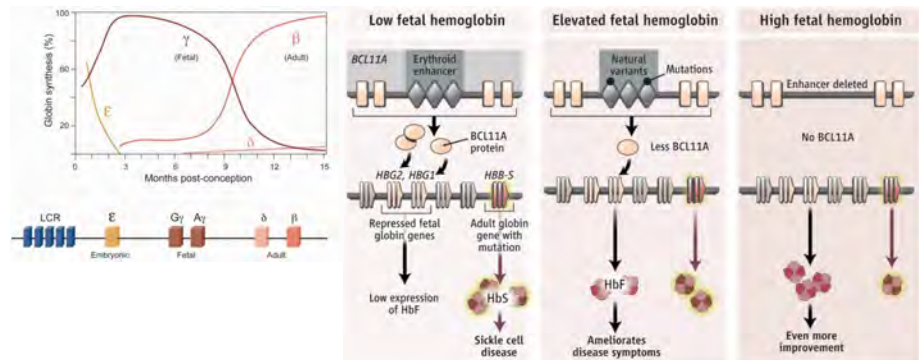
Engineered T cells with improved anticancer activity can be generated through the targeted disruption of immunomodulatory genes, such as programmed cell death protein 1 (*PDCD1*, which encodes PD-1), and T cell receptor (TCR) genes (*TRAC* and *TRBC*), using CRISPR-Cas9 delivered as preformed ribonucleoproteins (RNPs). These cells are then modified to express an engineered TCR that recognizes cancer-testis antigen 1 (NY-ESO-1) expressed by cancer cells.



6 Feb 2020

Hamilton & Doudna (Science, 2020)  
Stadtmauer et al. (Carl June Lab, Science 2020)

# Treating sickle-cell anemia and beta-thalassemia with CRISPR



First **ex-vivo** CRISPR therapy approved in Europe

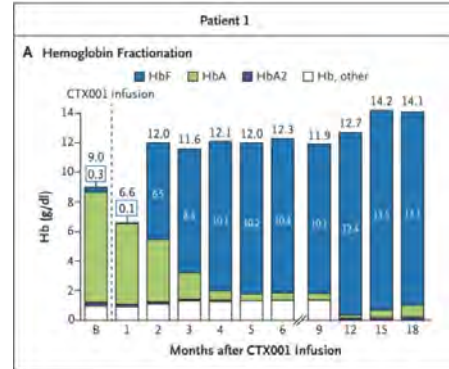
November 2019



## First CRISPR-treated Sickle Cell Anemia patients cured (2020)



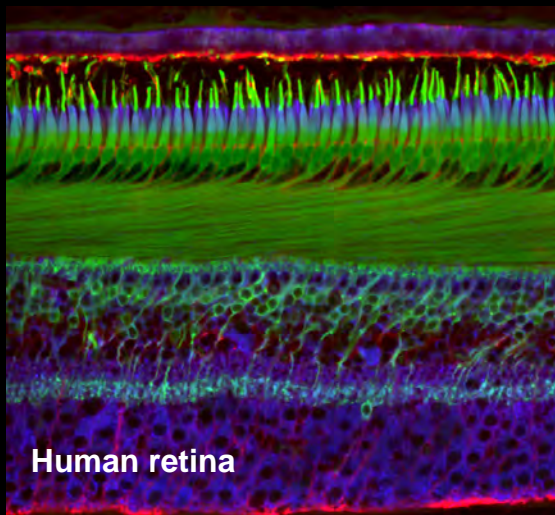
Victoria Gray (treated July 2, 2019)



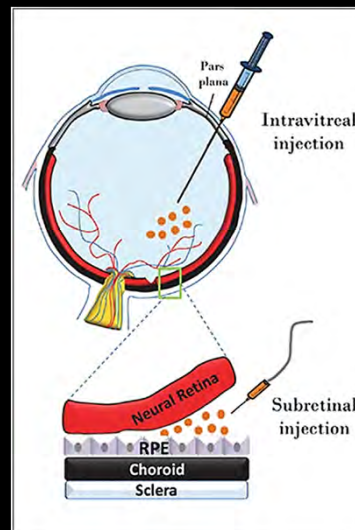
Frangoul *et al.* NEJM (5 December 2020)

NPR

## Correcting mutations in CEP290 gene with CRISPR Leber Congenital Amaurosis type 10



Human retina



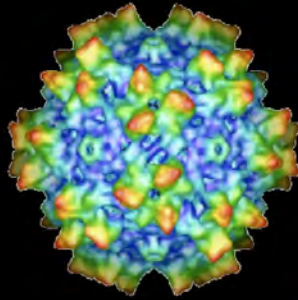
In vivo

Dec 2018

editas  
MEDICINE

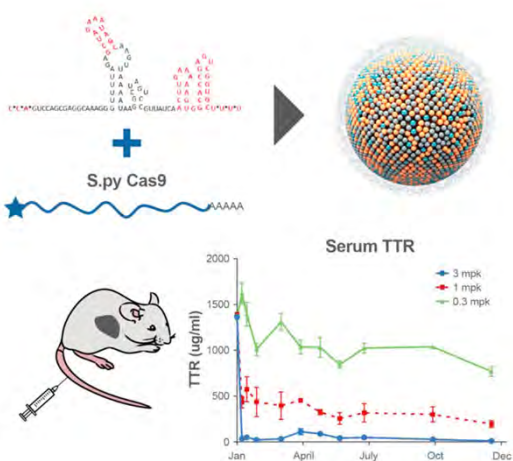
Allergan

# Adenoassociated virus (AAV) to bring CRISPR to cells



AAV

## Transthyretine Amyloidosis congenital (ATTR) NANOTECHNOLOGY - Nanoparticles



Investors & Media  
Press Releases  
Events & Presentations  
Corporate Governance

Oct 19, 2020  
NTLA-2001: First single-course therapy that potentially halts and reverses ATTR  
On track to dose first patient by year-end with a systemically delivered CRISPR/Cas9-based therapy



Finn et al. *Cell Reports* 2018 22, 2227-2235 DOI: (10.1016/j.celrep.2018.02.014)  
Copyright © 2018 Intellia Therapeutics, Inc.

October 2020

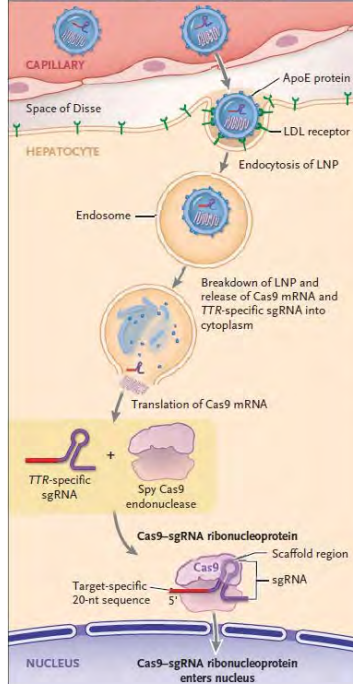
ORIGINAL ARTICLE

### CRISPR-Cas9 In Vivo Gene Editing for Transthyretin Amyloidosis

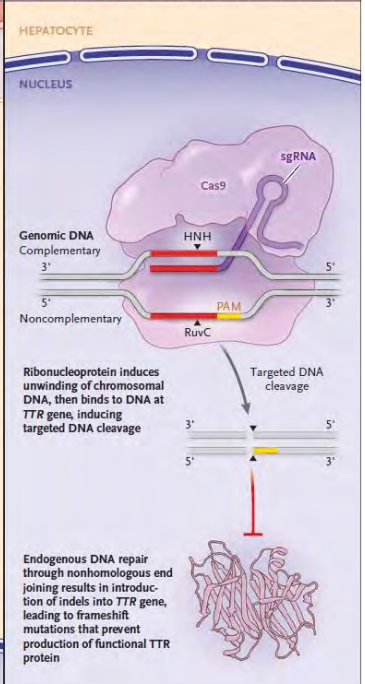
Julian D. Gillmore, M.D., Ph.D., Ed Gane, M.B., Ch.B., Jorg Taubel, M.D., Justin Kao, M.B., Ch.B., Marianna Fontana, M.D., Ph.D., Michael L. Maitland, M.D., Ph.D., Jessica Seitzer, B.S., Daniel O'Connell, Ph.D., Kathryn R. Walsh, Ph.D., Kristy Wood, Ph.D., Jonathan Phillips, Ph.D., Yuanxin Xu, M.D., Ph.D., Adam Amaral, B.A., Adam P. Boyd, Ph.D., Jeffrey E. Cehelsky, M.B.A., Mark D. McKee, M.D., Andrew Schiermeier, Ph.D., Olivier Harari, M.B., B.Chir., Ph.D., Andrew Murphy, Ph.D., Christos A. Kyriatsos, Ph.D., Brian Zambrowicz, Ph.D., Randy Soltys, Ph.D., David E. Gutstein, M.D., John Leonard, M.D., Laura Sepp-Lorenzino, Ph.D., and David Lebowitz, M.D.

26 June 2021

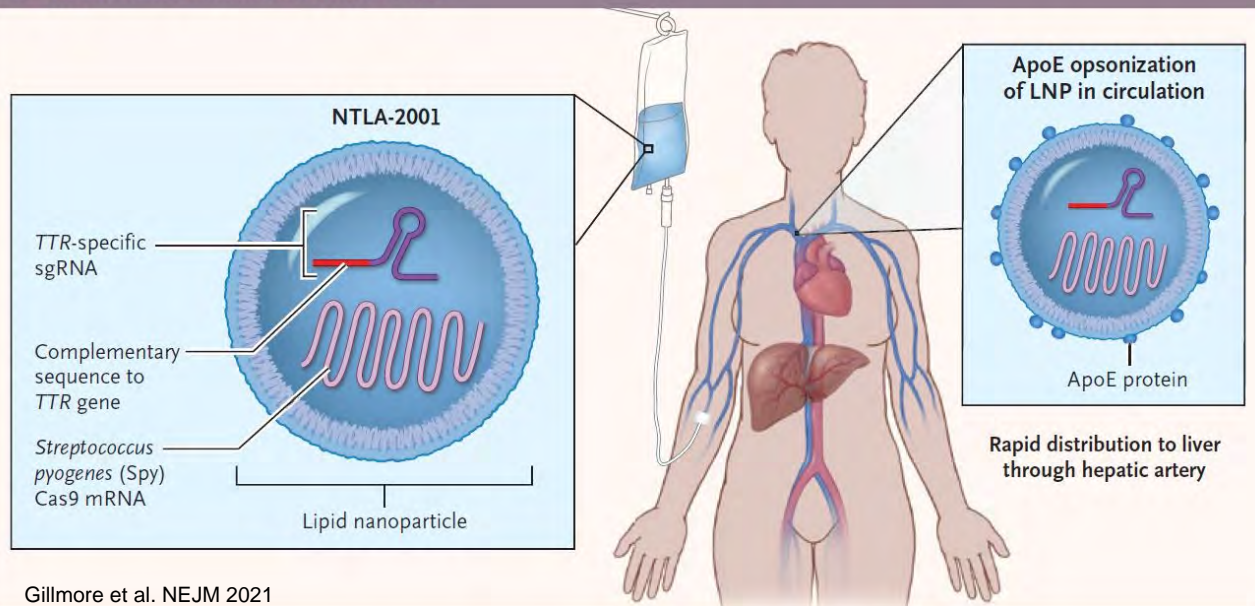
#### B NTLA-2001 LNP Uptake in Hepatocytes



#### C Cleavage of DNA at TTR Gene Sequence by Cas9



#### A Intravenous Infusion of NTLA-2001

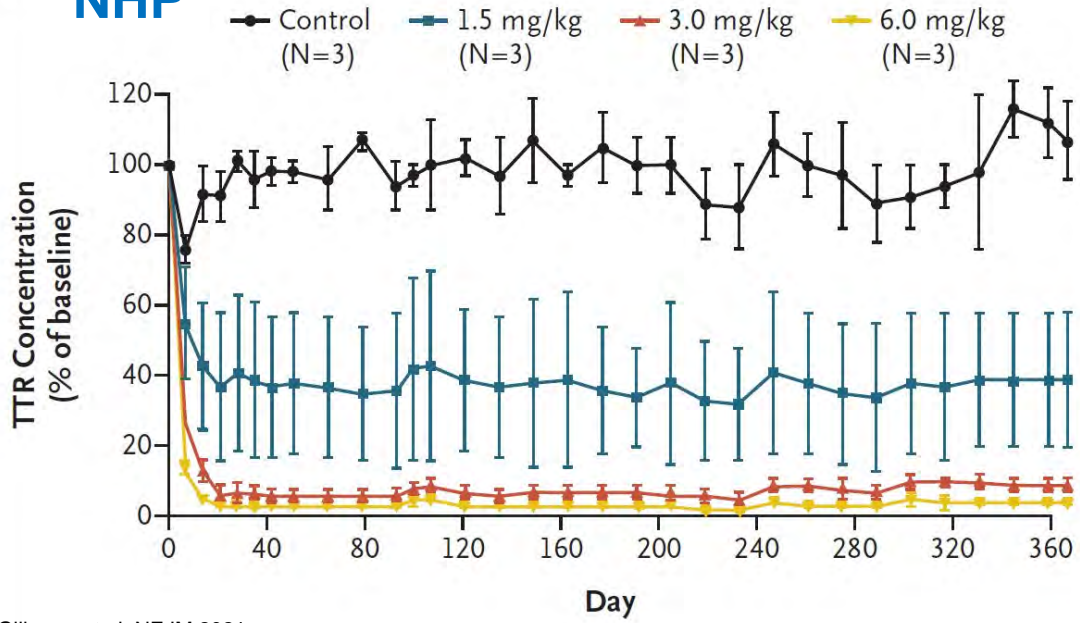


Gillmore et al. NEJM 2021



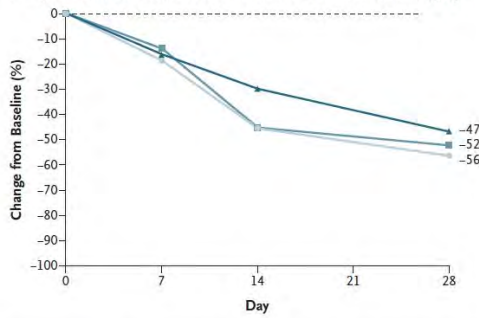
A

NHP

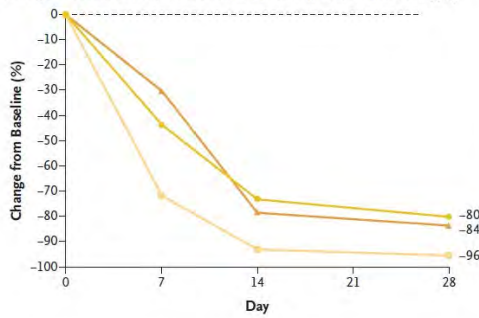


Gillmore et al. NEJM 2021

A Change in Serum TTR Concentration in Patients Who Received 0.1 mg/kg



B Change in Serum TTR Concentration in Patients Who Received 0.3 mg/kg

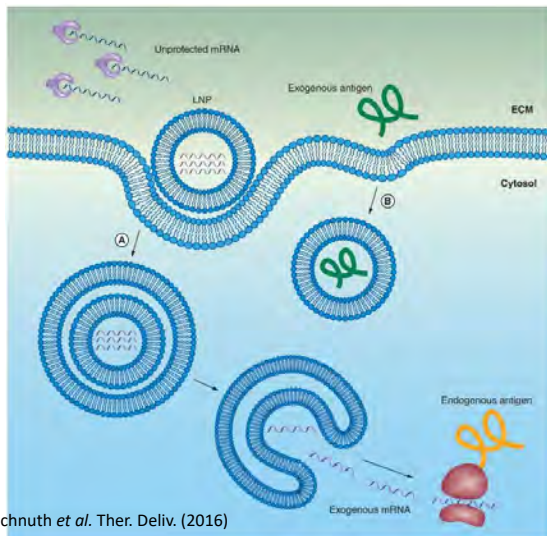


Gillmore et al. NEJM 2021



**COVID-19 first vaccines approved**

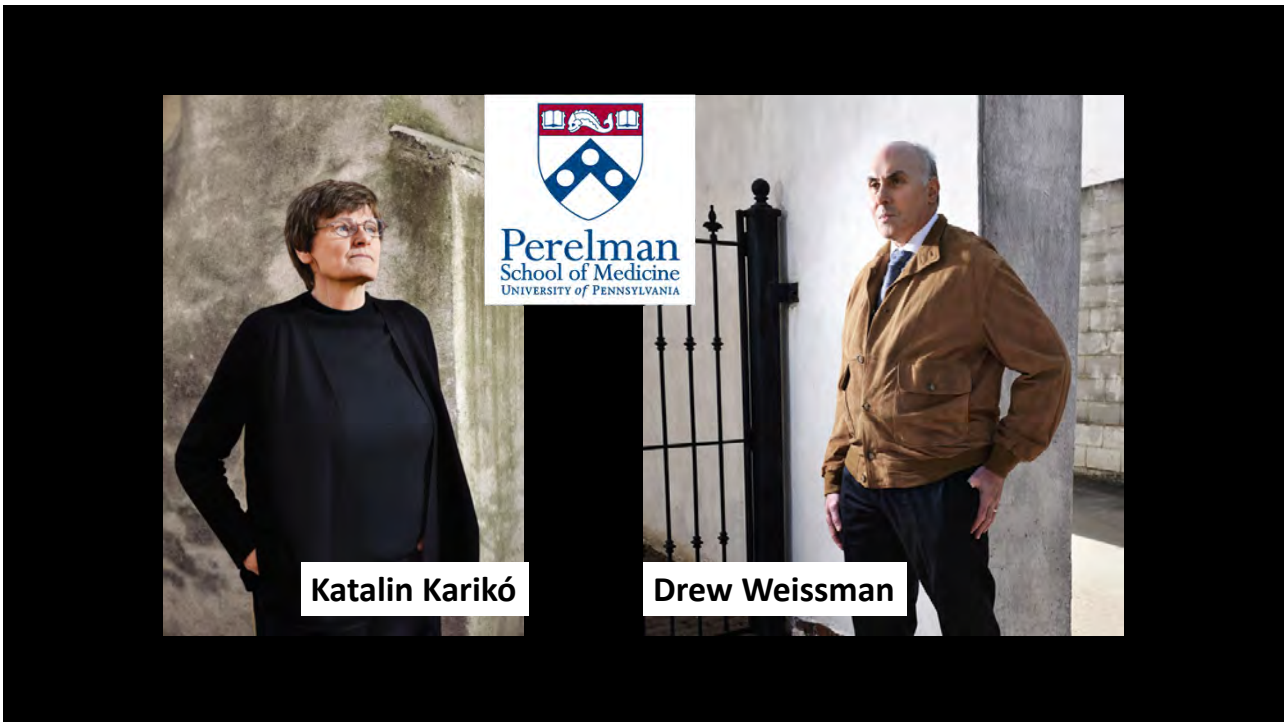
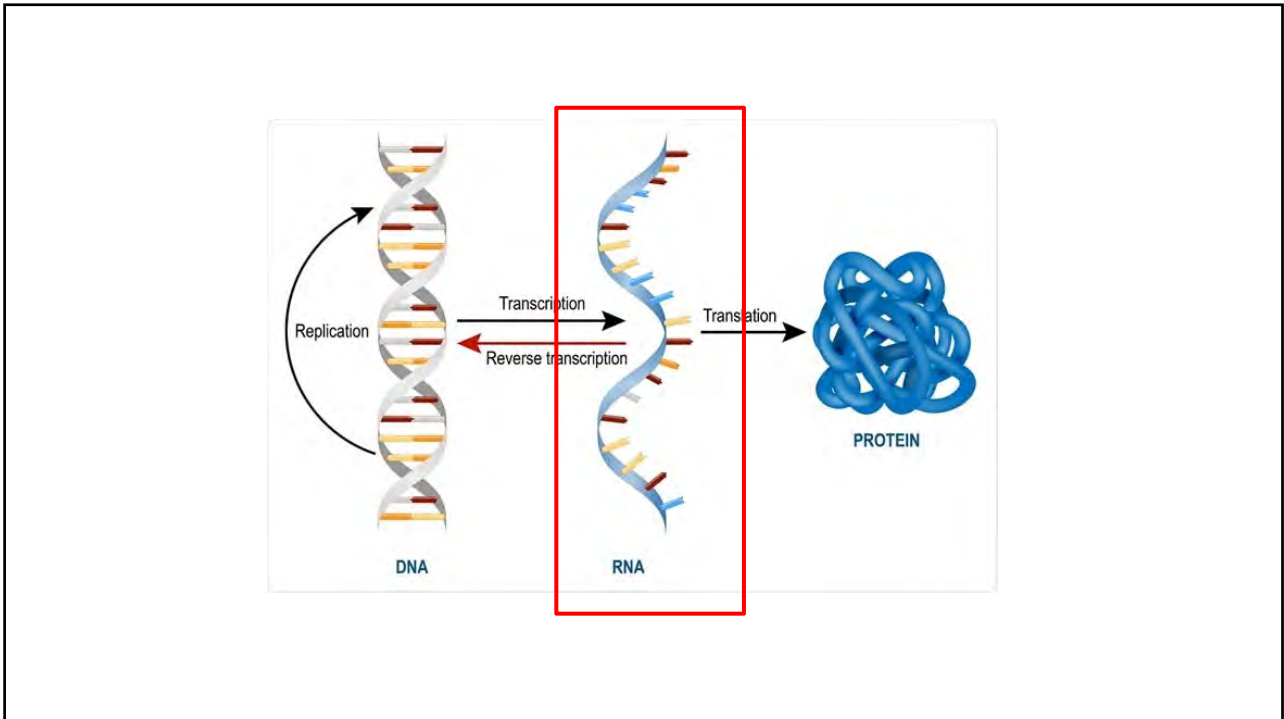
Pfizer-BioNTech	FDA	11 Dec 2020
Moderna	FDA	18 Dec 2020



Reichnuth *et al.* Ther. Deliv. (2016)







## Suppression of RNA Recognition by Toll-like Receptors: The Impact of Nucleoside Modification and the Evolutionary Origin of RNA

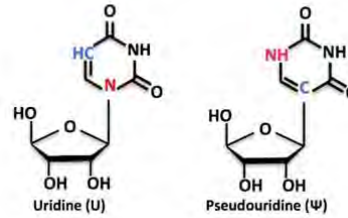
Katalin Karikó,<sup>1,\*</sup> Michael Buckstein,<sup>2</sup> Houping Ni,<sup>2</sup> and Drew Weissman<sup>2</sup>

<sup>1</sup>Department of Neurosurgery

<sup>2</sup>Department of Medicine

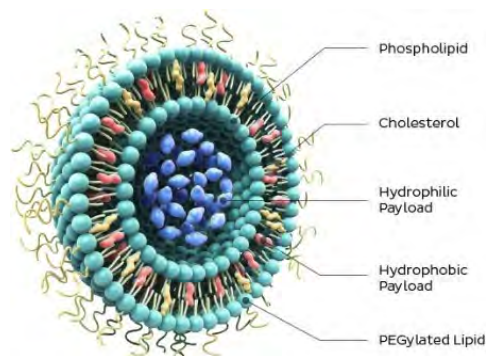
University of Pennsylvania School of Medicine

Philadelphia, Pennsylvania 19104



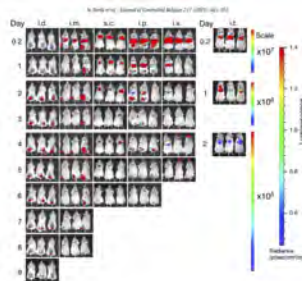
Modified nucleosides does not trigger an immune response

## Lipid NanoParticles (LNP)



RNA

2015



Journal of Controlled Release 217 (2015) 345–353

Contents lists available at ScienceDirect

Journal of Controlled Release

Journal homepage: www.elsevier.com/locate/jconrel

Expression kinetics of nucleoside-modified mRNA delivered in lipid nanoparticles to mice by various routes

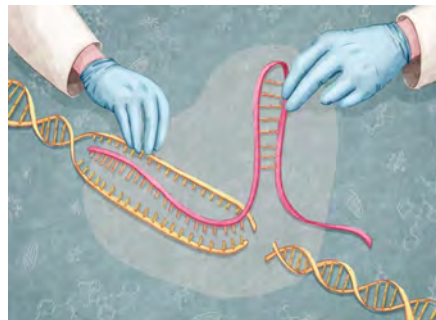
Norbert Pardi<sup>a</sup>, Steven Tsuchihashi<sup>a</sup>, Hiromi Muramatsu<sup>a</sup>, Katalin Kariko<sup>a</sup>, Barbara L. Mui<sup>b</sup>, Ying K. Tam<sup>b</sup>, Thomas D. Madden<sup>b</sup>, Michael J. Hope<sup>b</sup>, Drew Weissman<sup>a,b</sup>

<sup>a</sup> Department of Medicine, University of Pennsylvania, Philadelphia, PA 19104, USA  
<sup>b</sup> Astor, Thompson, University, VET 122 BC, Canada

Encapsulating modified mRNA in LNPs extends the translation period

## CRISPR & gene therapy (today) – clinical trials

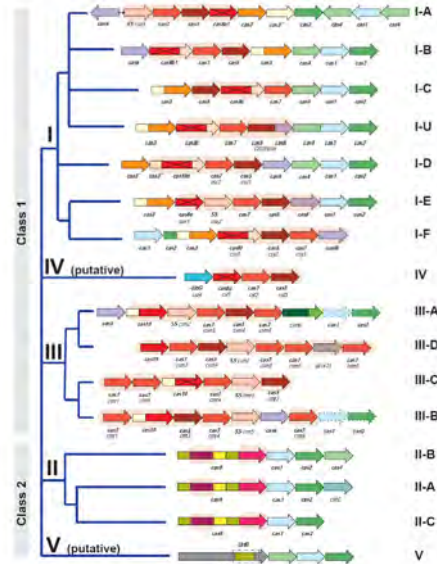
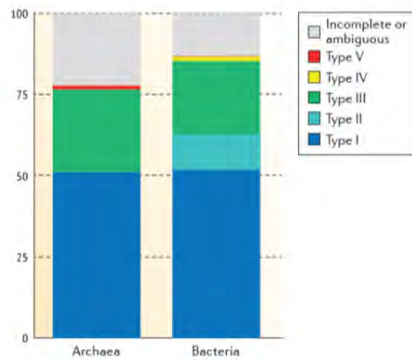
- **Inactivating genes** → CRISPR-Cas9 or base editors
- **Correcting genes** → Base editors
- **Technologies used** → mRNA + lipid nanoparticles (LNPs)



# Diversity of CRISPR-Cas systems

## An updated evolutionary classification of CRISPR-Cas systems

Makarova *et al.* Nature Rev. Microbiol. 2015

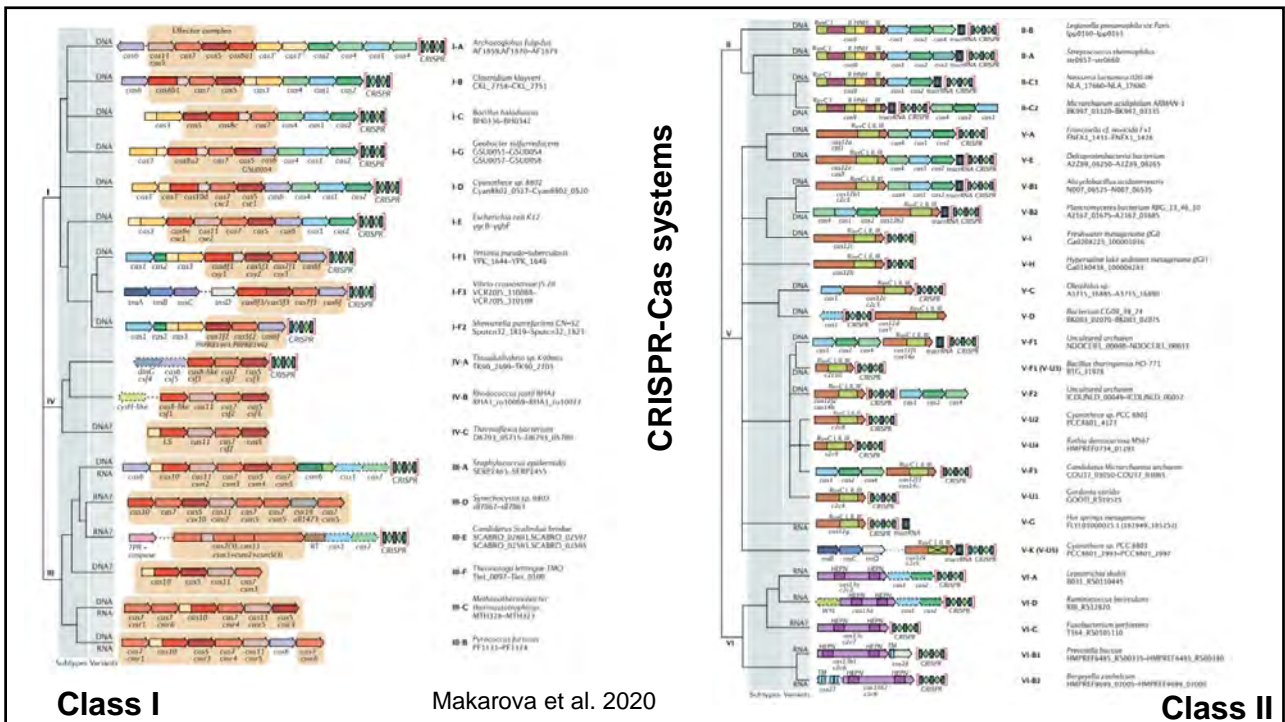


# REVIEWS

## Evolutionary classification of CRISPR-Cas systems: a burst of class 2 and derived variants

Kira S. Makarova<sup>1</sup>, Yuri I. Wolf<sup>1</sup>, Jaime Iranzo<sup>1</sup>, Sergey A. Shmakov<sup>1</sup>, Omer S. Alkhnbashi<sup>2</sup>, Stan J. J. Brouns<sup>3</sup>, Emmanuelle Charpentier<sup>4</sup>, David Cheng<sup>5</sup>, Daniel H. Haft<sup>1</sup>, Philippe Horvath<sup>6</sup>, Sylvain Moineau<sup>7</sup>, Francisco J. M. Mojica<sup>8</sup>, David Scott<sup>5</sup>, Shiraz A. Shah<sup>9</sup>, Virginijus Siksnys<sup>10</sup>, Michael P. Terns<sup>11</sup>, Česlovas Venclovas<sup>10</sup>, Malcolm F. White<sup>12</sup>, Alexander F. Yakunin<sup>13,14</sup>, Winston Yan<sup>5</sup>, Feng Zhang<sup>15,16,17,18</sup>, Roger A. Garrett<sup>19</sup>, Rolf Backofen<sup>2,20</sup>, John van der Oost<sup>21</sup>, Rodolphe Barrangou<sup>22</sup> and Eugene V. Koonin<sup>1</sup>\*





# The widespread IS200/605 transposon family encodes diverse programmable RNA-guided endonucleases

Han Altae-Tran<sup>1,2,3,4,5,†</sup>, Soumya Kannan<sup>1,2,3,4,5,†</sup>, F. Esra Demircioglu<sup>1,2,3,4,5</sup>, Rachel Oshiro<sup>1,2,3,4,5</sup>, Suchita P. Nety<sup>1,2,3,4,5</sup>, Luke J. McKay<sup>6,7,8</sup>, Mensur Dlakic<sup>9</sup>, William P. Inskeep<sup>6,7</sup>, Kira S. Makarova<sup>10</sup>, Rhiannon K. Macrae<sup>1,2,3,4,5</sup>, Eugene V. Koonin<sup>10</sup>, Feng Zhang<sup>1,2,3,4,5\*</sup>

Science

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CRISPR

## Las 'tatarabuelas' de las tijeras moleculares CRISPR harán más sencilla la edición genética

Investigadores de EE UU liderados por Feng Zhang, uno de los pioneros del corta-pegar genético, han descubierto una clase de nucleasas que podrían ser los ancestros de las más utilizadas, Cas9 y Cas12. Su pequeña longitud permitiría facilitar el proceso.

📄 📧 📱 📺 📺 📺

Analizando 9/9/2021 20:00 CEST





@LluisMontoliu

[www.user.cnb.csic.es/montoliu](http://www.user.cnb.csic.es/montoliu)



FUNDACIÓN RAMÓN ARECES

