

GENOME EDITING and the CRISPR CRAZE



Kevin Davies

Executive Editor, *The CRISPR Journal*

Author, *Editing Humanity*

Lexington KY | 02.25.2022

Conflicts of Interest

Nothing to declare.

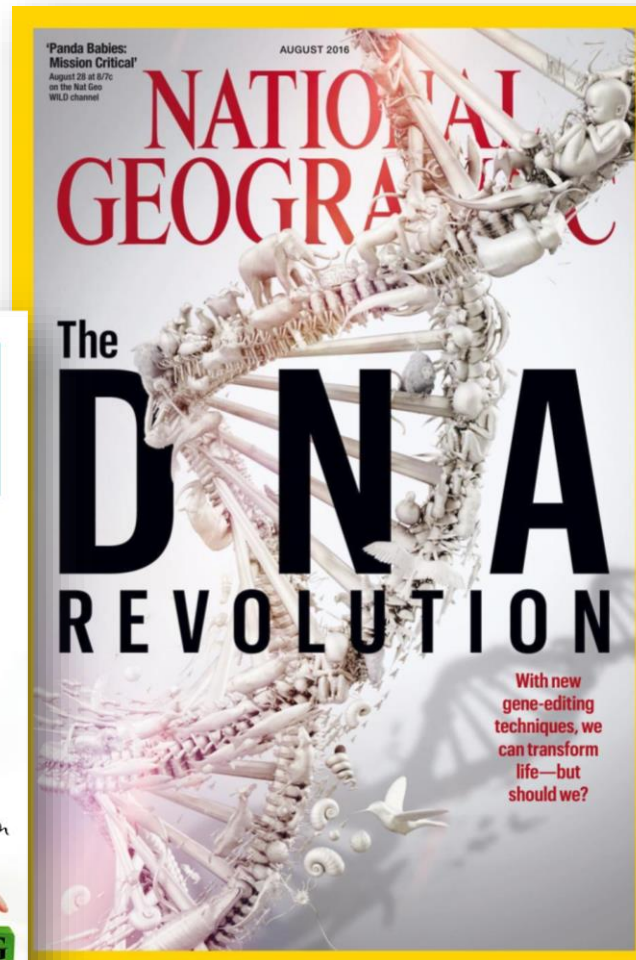
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CRISPR





BIG
MEETS
BIGGER

In 1993, a breakthrough new technology, known as CRISPR, gave scientists a path to treat incurable diseases through genetic editing.

In 2016, due to its potential for misuse, the U.S. Intelligence Community designated genetic editing a 'Weapon of Mass Destruction and Proliferation.'

*“Are you familiar
with CRISPR?”*

DWAYNE JOHNSON
RAMPAGE

SEE IT IN REALD 3D AND IMAX

APRIL 20



ADD TO CALENDAR

Jeopardy! November 29, 2019

**JENNIFER DOUDNA &
EMMANUELLE
CHARPENTIER
ARE CO-INVENTORS OF
THE REVOLUTIONARY
TOOL CRISPR TO EDIT
THESE IN THE BODY**



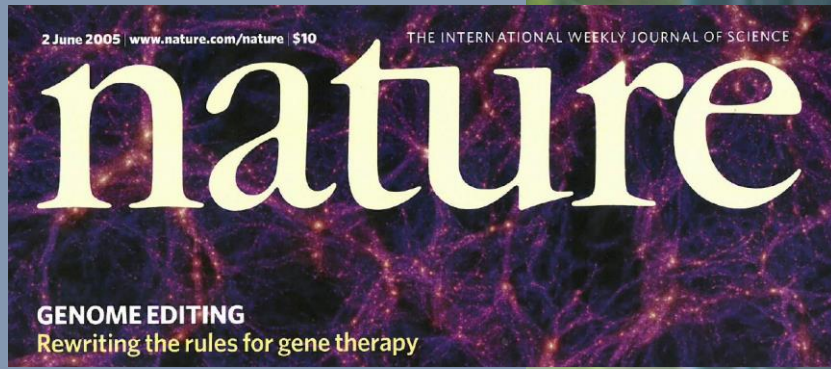
The Nobel Prize
in Chemistry 2020
awarded jointly to

**Emmanuelle
Charpentier
and
Jennifer A.
Doudna**

"for the development
of a method for
genome editing."

October 7, 2020





ARTICLES

Highly efficient endogenous human gene correction using designed zinc-finger nucleases

Fyodor D. Urnov¹, Jeffrey C. Miller¹, Ya-Li Lee¹, Christian M. Beausejour¹, Jeremy M. Rock¹, Sheldon Augustus¹, Andrew C. Jamieson¹, Matthew H. Porteus², Philip D. Gregory¹ & Michael C. Holmes¹

Permanent modification of the human genome *in vivo* is impractical owing to the low frequency of homologous recombination in human cells, a fact that hampers biomedical research and progress towards safe and effective gene therapy. Here we report a general solution using two fundamental biological processes: DNA recognition by C₂H₂ zinc-finger proteins and homology-directed repair of DNA double-strand breaks. Zinc-finger proteins engineered to recognize a unique chromosomal site can be fused to a nuclease domain, and a double-strand break induced by the resulting zinc-finger nuclease can create specific sequence alterations by stimulating homologous recombination between the chromosome and an extrachromosomal DNA donor. We show that zinc-finger nucleases designed against an X-linked severe combined immune deficiency (SCID) mutation in the *IL2R γ* gene yielded more than 18% gene-modified human cells without selection. Remarkably, about 7% of the cells acquired the desired genetic modification on both X chromosomes, with cell genotype accurately reflected at the messenger RNA and protein levels. We observe comparably high frequencies in human T cells, raising the possibility of strategies based on zinc-finger nucleases for the treatment of disease.

Courtesy:
HUMAN NATURE

Fyodor Urnov
Innovative
Genomics Institute





Francisco Mojica
(University of Alicante)
Salterns of Santa Pola, Spain



Asunto: Re: Acronym

Fecha: Wed, 21 Nov 2001 16:39:06 +0100

De: "Ruud Jansen" <R.Jansen@vet.uu.nl>

Empresa: Diergeneeskunde

A: "Francisco J. Martínez Mojica" <fmojica@ua.es>

Dear Francis

What a great acronym is CRISPR.

I feel that every letter that was removed in the alternatives made it less crispy so I prefer the snappy CRISPR over SRSR and SPIDR. Also not unimportant is the fact that in MedLine CRISPR is a unique entry, which is not true for some of the other shorter acronyms.

Intervening Sequences of Regularly Spaced Prokaryotic Repeats Derive from Foreign Genetic Elements

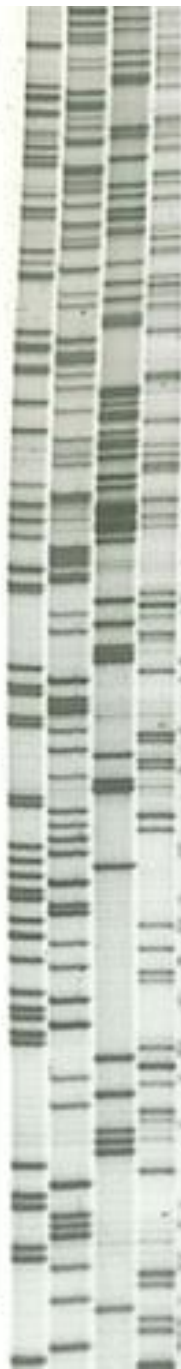
Francisco J.M. Mojica, César Díez-Villaseñor, Jesús García-Martínez, Elena Soria

División de Microbiología, Departamento de Fisiología, Genética y Microbiología, Universidad de Alicante, Campus de San Vicente, E-03080, Spain

Received: 6 February 2004 / Accepted: 1 October 2004 [*Reviewing Editor:* Dr. John Huelsenbeck]

JOURNAL OF **MOLECULAR
EVOLUTION**

© Springer Science+Business Media, Inc. 2005



CRISPR Provides Acquired Resistance Against Viruses in Prokaryotes

Rodolphe Barrangou,¹ Christophe Fremaux,² H el ene Deveau,³ Melissa Richards,¹
Patrick Boyaval,² Sylvain Moineau,³ Dennis A. Romero,¹ Philippe Horvath^{2*}

Science 2007



A woman with long, wavy brown hair and blue eyes is speaking. She is wearing a dark blue, long-sleeved top. She is sitting in a wooden chair. Behind her is a metal fence with a diamond pattern. The background is slightly out of focus, showing some greenery.

Jill Banfield
UC Berkeley

“One thing they’ll write on
my tombstone is:

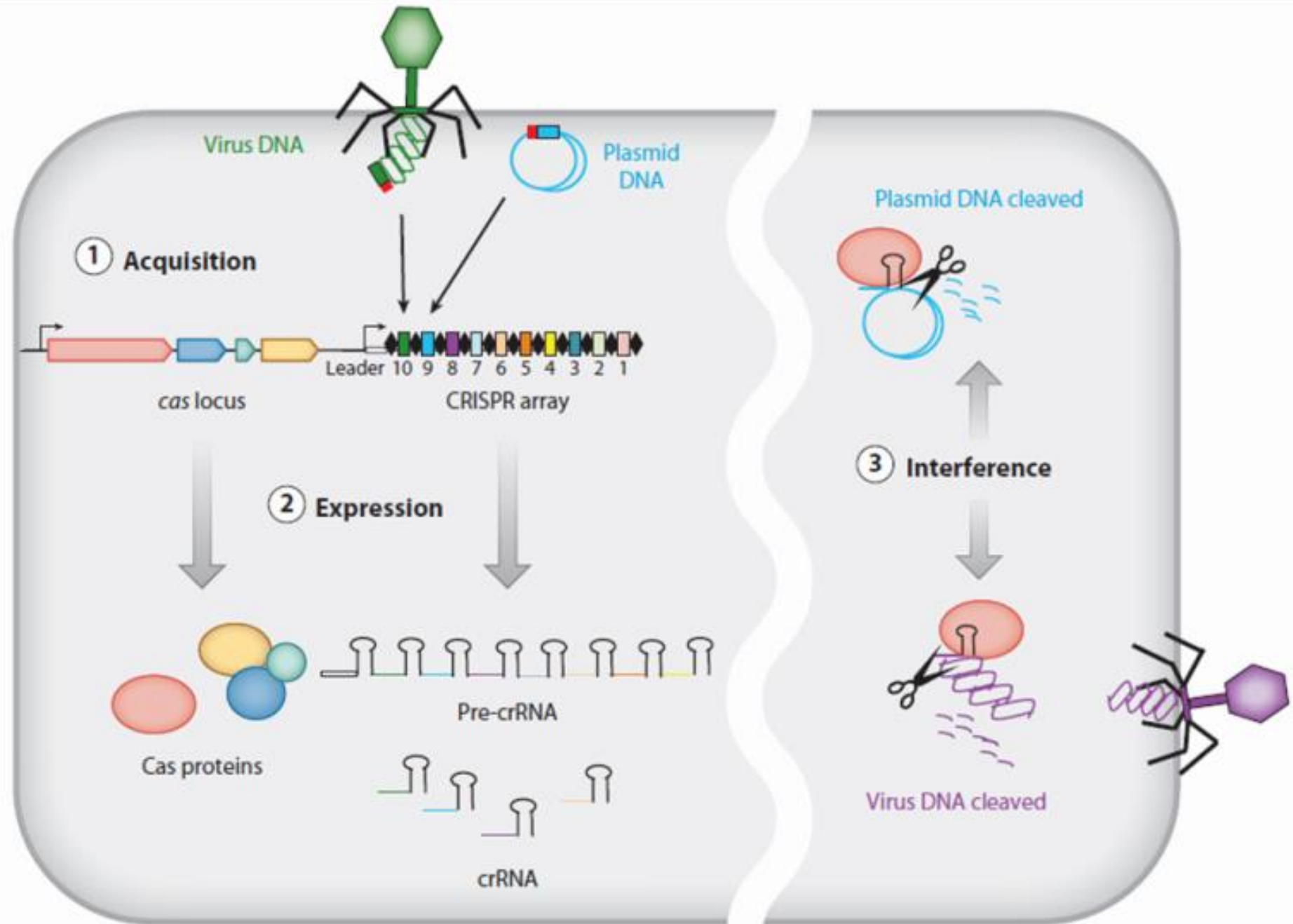
*Told Jennifer Doudna
about CRISPR-Cas.*

Like, that will be the sum of
my life!”

Courtesy:
HUMAN NATURE

CRISPR

... is a natural bacterial immune defense system that provides a means to recognize, remember and destroy viral invaders.



Team Doudna/Charpentier (2012)

Emmanuelle
Charpentier

Jennifer
Doudna

Martin
Jinek

Krzysztof
Chylinski

Ines
Fonfara

Stanley Hall
UC Berkeley
2012

Kavli Prize
Oslo, Norway
Sept. 2018



“... for the invention of CRISPR-Cas9, a precise nanotool for editing DNA”



RNA-Guided Human Genome Engineering via Cas9

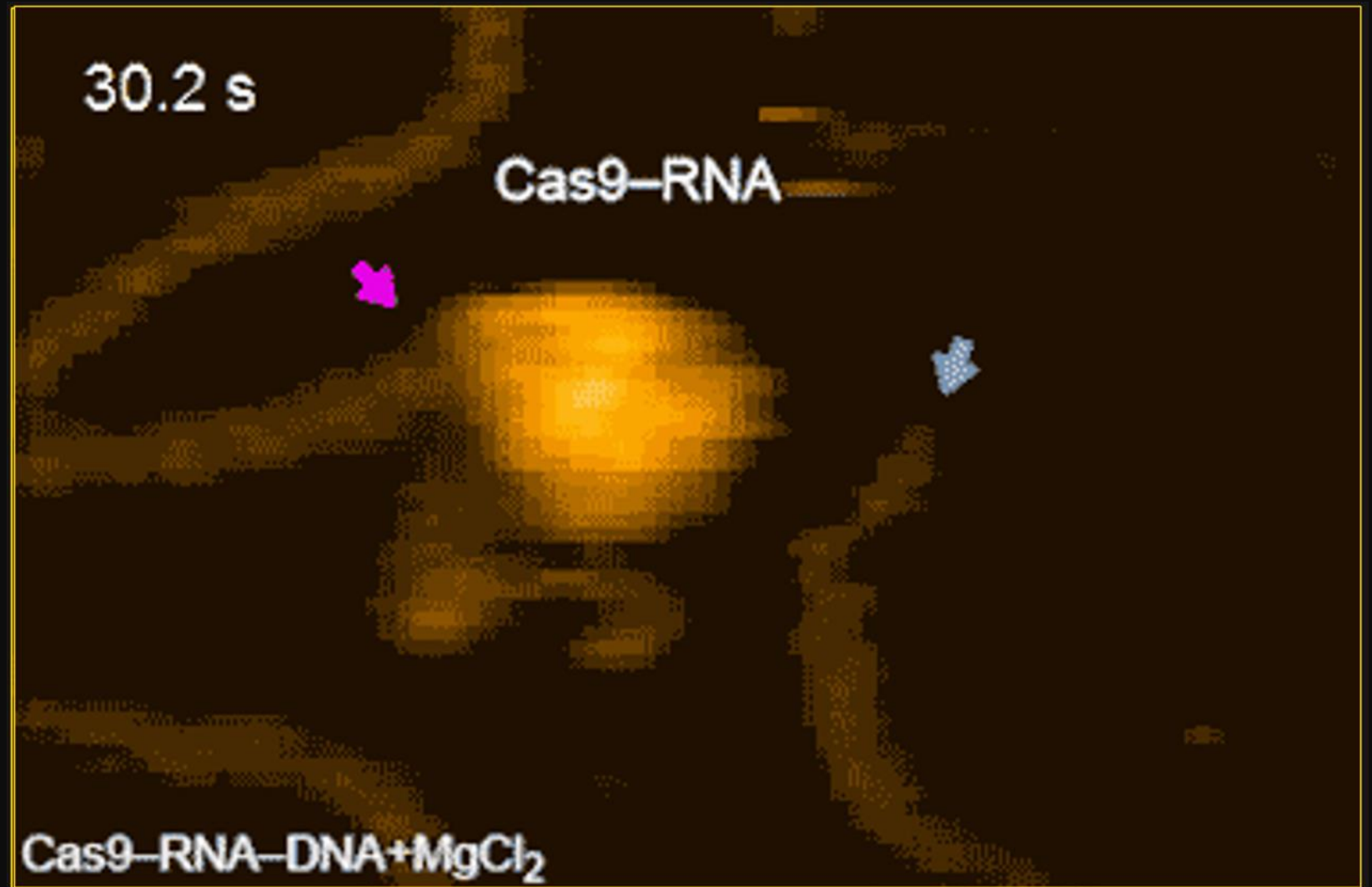
Prashant Mali,^{1*} Luhan Yang,^{1,3*} Kevin M. Esvelt,² John Aach,¹ Marc Guell,¹ James E. DiCarlo,⁴ Julie E. Norville,¹ George M. Church^{1,2†}

Bacteria and archaea have evolved adaptive immune defenses, termed clustered regularly interspaced short palindromic repeats (CRISPR)/CRISPR-associated (Cas) systems, that use short RNA to direct degradation of foreign nucleic acids. Here, we engineer the type II bacterial CRISPR system to function with custom guide RNA (gRNA) in human cells. For the endogenous AAVS1 locus, we obtained targeting rates of 10 to 25% in 293T cells, 13 to 8% in K562 cells, and 2 to 4% in induced pluripotent stem cells. We show that this process relies on CRISPR components; is sequence-specific; and, upon simultaneous introduction of multiple gRNAs, can effect multiplex editing of target loci. We also compute a genome-wide resource of ~190 K unique gRNAs targeting ~40.5% of human exons. Our results establish an RNA-guided editing tool for facile, robust, and multiplexable human genome engineering.



Lights. Camera. Action... **CUT!**

CRISPR-Cas9
visualized by high-
speed atomic force
microscopy



M. Shibata, H. Nishimasu *et al.*
Nature Communications 8, 1430 (2017)

Hiroshi Nishimasu (Univ Tokyo)





Victoria Gray

Forest, Mississippi

First patient to receive CRISPR gene therapy for sickle-cell disease in the USA

- 46% total HbF
- 99.7% red blood cells contain some of it.

ORIGINAL ARTICLE BRIEF REPORT

CRISPR-Cas9 Gene Editing for Sickle Cell Disease and β -Thalassemia

Haydar Frangoul, M.D., David Altshuler, M.D., Ph.D., M. Domenica Cappellini, M.D., Yi-Shan Chen, Ph.D., Jennifer Domm, M.D., Brenda K. Eustace, Ph.D., Juergen Foell, M.D., Josu de la Fuente, M.D., Ph.D., Stephan Grupp, M.D., Ph.D., Rupert Handgretinger, M.D., Tony W. Ho, M.D., Antonis Kattamis, M.D., Andrew Kernysky, Ph.D., Julie Lekstrom-Himes, M.D., Amanda M. Li, M.D., Franco Locatelli, M.D., Markus Y. Mapara, M.D., Ph.D., Mariane de Montalembert, M.D., Damiano Rondelli, M.D., Akshay Sharma, M.B., B.S., Sujit Sheth, M.D., Sandeep Soni, M.D., Martin H. Steinberg, M.D., Donna Wall, M.D., Angela Yen, Ph.D., and Selim Corbacioglu, M.D.

In vivo Genome Editing

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. Kyrtasous, 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 Lebwohl, M.D.



Genome Editing News Rare and Neglected Diseases

“New Era of Medicine”: Researchers Publish First Positive Clinical Data for In Vivo Genome Editing in Humans

Intellia, Regeneron candidate NTLA-2001 shows sustained reduction in protein-causing transthyretin (ATTR) amyloidosis after a single dose in six patients

By Alex Philippidis · June 28, 2021 · 0

TTR exon 1

Leu Leu Leu Cys Leu Ala Gly Leu Val Phe Val Ser Glu Ala Gly
...|C T C|C T C|C T C|T G|C C T|T G C T|G G A|C T G|G T A|T T T|G T G|T C T|G A G|G C T|G G C|...

↓ CRISPR-Cas9 editing

Leu Leu Leu Cys Leu Ala Trp Thr Gly Ile Cys Val **STOP**
...|C T C|C T C|C T C|T G C|C T T|G C T|T G G A|C T G|G T A|T T T|G T G|T G T C|T G A|G G C|T G G C|...

↑ frequent 1-bp insertion

Intellia
THERAPEUTICS

NEJM June 2021

An epic clash of cultures in
ancient Mesoamerica p. 968

Music is another
language pp. 974 & 1043

A primordial body in the
Kuiper Belt pp. 980 & 998-1000

Science

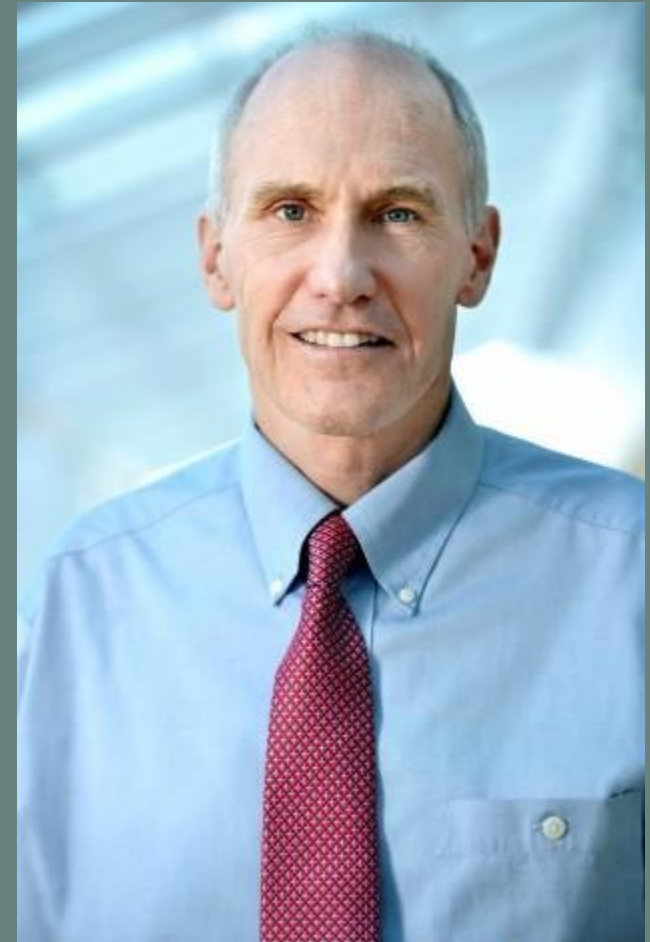
\$15
28 FEBRUARY 2020
sciencemag.org

AAAS

HUMAN CRISPR

Gene editing meets
cancer immunotherapy

pp. 976 & 1001






Carlene Knight


STAT+

BIOTECH

Editas CRISPR treatment improved vision for one patient, but not others, early data show

Reprints

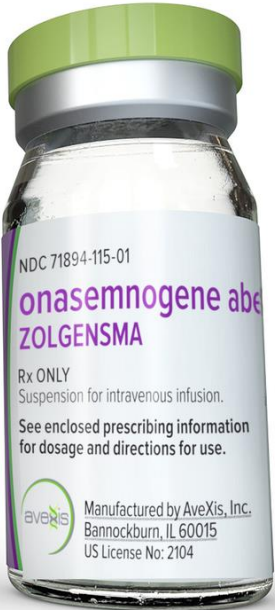
By Adam Feuerstein  Sept. 29, 2021



The illustration depicts a stylized DNA double helix with blue and red strands. A person in a blue suit is running alongside the helix, holding a red marker. Several blue and red markers are scattered around the helix, suggesting a process of editing or analysis.

The world's most expensive medicine could save toddlers' lives. But getting it is complicated

By Jonathan Saltzman -- *Boston Globe* 8.8.19



Estelle Lemieux



YOU WERE SO PREOCCUPIED WITH WHETHER OR NOT YOU COULD



YOU DIDN'T STOP TO THINK IF YOU SHOULD



2011

2007

2013

He Jiankui's Journey

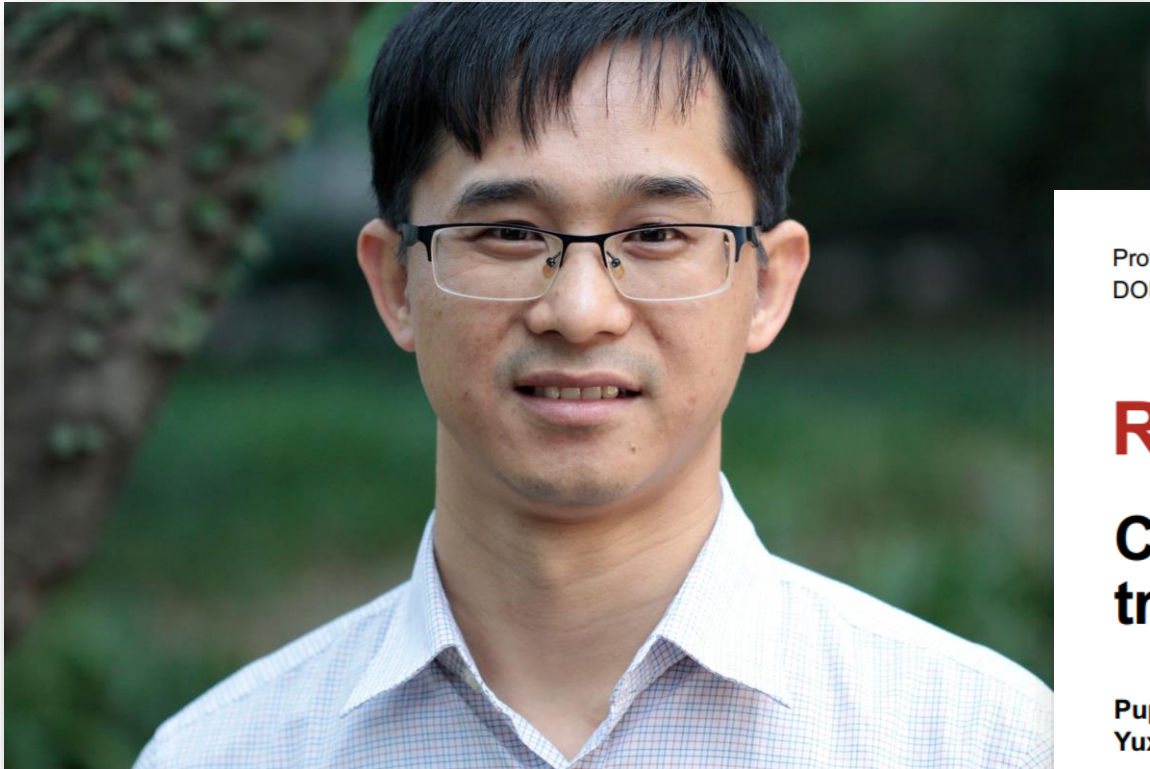
Heterogeneous Diversity of Spacers within CRISPR (Clustered Regularly Interspaced Short Palindromic Repeats)

Jiankui He & Michael W. Deem

Phys. Rev. Lett. **105**, 128102 – Published 14 September 2010

Chinese Scientists Edit Genes of Human Embryos, Raising Concerns

-- *New York Times* (2015)



Huang Junjiu
(Sun Yat-sen University)

Protein Cell 2015, 6(5):363–372
DOI 10.1007/s13238-015-0153-5



Protein & Cell

RESEARCH ARTICLE

CRISPR/Cas9-mediated gene editing in human tripronuclear zygotes

Puping Liang, Yanwen Xu, Xiya Zhang, Chenhui Ding, Rui Huang, Zhen Zhang, Jie Lv, Xiaowei Xie, Yuxi Chen, Yujing Li, Ying Sun, Yaofu Bai, Zhou Songyang, Wenbin Ma, Canquan Zhou[✉], Junjiu Huang[✉]

Guangdong Province Key Laboratory of Reproductive Medicine, the First Affiliated Hospital, and Key Laboratory of Gene Engineering of the Ministry of Education, School of Life Sciences, Sun Yat-sen University, Guangzhou 510275, China

✉ Correspondence: hjunjiu@mail.sysu.edu.cn (J. Huang), zhoucanquan@hotmail.com (C. Zhou)

Received March 30, 2015 Accepted April 1, 2015



ARTICLE

doi:10.1038/nature23305

Correction of a pathogenic gene mutation in human embryos

Hong Ma^{1*}, Nuria Marti-Gutierrez^{1*}, Sang-Wook Park^{2*}, Jun Wu^{3*}, Yeonmi Lee¹, Keiichiro Suzuki³, Amy Koski¹, Dongmei Ji¹, Tomonari Hayama¹, Riffat Ahmed¹, Hayley Darby¹, Crystal Van Dyken¹, Ying Li¹, Eunju Kang¹, A.-Reum Park², Daesik Kim⁴, Sang-Tae Kim², Jianhui Gong^{5,6,7,8}, Ying Gu^{5,6,7}, Xun Xu^{5,6,7}, David Battaglia^{1,9}, Sacha A. Krieg⁹, David M. Lee⁹, Diana H. Wu⁹, Don P. Wolf¹, Stephen B. Heitner¹⁰, Juan Carlos Izpisua Belmonte^{3§}, Paula Amato^{1,9§}, Jin-Soo Kim^{2,4§}, Sanjiv Kaul^{10§} & Shoukrat Mitalipov^{1,10§}

Genome editing of the heterozygous mutation improved accuracy and response. In the presence of homologous recombination, the DSB was repaired in human embryos carrying the mutation. The safety of the approach was confirmed by the successful birth of embryos by clinical applications.



Shoukrat Mitalipov (OHSU)

50p

THE I PAPER - BRITAIN'S FIRST AND ONLY CONCISE QUALITY TITLE

i world exclusive

One giant step for designer babies

The essential daily briefing

THURSDAY
27 JULY 2017
40p per issue

News.co.uk

Twin Peaty

Swimming hero wins second gold



» **Revealed** Era of genetically modified babies moves closer, as scientists prove they can safely alter human embryos

» Inherited diseases caused by defective genes can be corrected in the earliest stage of life, revolutionary technique shows

» Same technology could be used to select stronger muscles or better eyesight, prompting fierce ethical debate

» 'They've done it. The quality of the work is high,' top scientist tells **i**

» Religious organisations likely to oppose groundbreaking research

SPECIAL REPORT BY STEVE CONNOR, PAGES 6-7

INSIDE CAR BAN BACKLASH **120** | SCIENCE **130** | TV & RADIO **138** | GAMES **144**




Robert Edwards / Louise Brown



Daily Mail THURSDAY, JULY 21, 1978 **WORLD EXCLUSIVE**

And here she is...

THE LOVELY LOUISE



LOUISE BROWN, bright-eyed at 18 hours old: The test tube baby in hospital yesterday
Daily Mail World Exclusive Picture by Bill Cross © World Copyright Associated Newspapers Group Ltd., 1978. Full story and more pictures inside



The “AIDS Village”

Wenlou Village

Henan Province, China



Lu Guang



“The Baby is Born”

Hi Stephen,

Great news! the baby is born (please keep it in confidential). I am coming to San Francisco this week, will you be available for a meeting? I can come to Stanford In Wednesday, Thursday or Friday. I want get help from you on how to announce the result, PR and ethics.

Regards,
JK

Jiankui He
Associate Professor
Southern University of Science and Technology (SUSTech)
Lab website: www.sustc-genome.org.cn

贺建奎
南方科技大学生物系副教授



New York Times
April 14, 2019

code

OF THE WILD

Antonio Regalado
MIT Technology Review



COURTESY: HE HE LAB, YOUTUBE



HE JIANKUI
BIOPHYSICS RESEARCHER

BREAKING NEWS

PRESIDENT PUTIN BRIEFED ON THE TRAGEDY

在露露和娜娜还是早细胞的时候



HKU November 28, 2018



Embryo 1
(Lulu)
+1 bp / - 4 bp

Embryo 2
(Nana)
-15 bp / WT



**SECOND INTERNATIONAL SUMMIT ON
HUMAN GENOME EDITING**

Convened by

The Academy of Sciences of Hong Kong 港科院

THE ROYAL SOCIETY

NATIONAL ACADEMY OF SCIENCES

NATIONAL ACADEMY OF MEDICINE



15 Reasons Why

SCIENCE


The CRISPR Baby Scandal Gets Worse by the Day

The alleged creation of the world's first gene-edited infants was full of technical errors and ethical blunders. Here are the 15 most damning details.

ED YONG DEC 3, 2018

7. A few people knew about He's intentions but failed to stop him.
8. He acted in contravention of global consensus.
9. He acted in contravention of his own stated ethical views.
10. He sought ethical advice and ignored it.
11. There is no way to tell whether He's work did any good.
12. He has doubled down.
13. Scientific academies have prevaricated.
14. A leading geneticist came to He's defense.
15. This could easily happen again.

Ed Yong, *The Atlantic*
Dec 3 2018



“ How could Dr. He and [his] team change the gene pool of the human species without considering the need to consult other parts of the human species?”

-- Qiu Renzong

Birth Announcement

Birth of Twins After Genome Editing for HIV Resistance

Jinzhou Qin^{1,2,#}, Yanran Chen^{1,#}, Xiaoqing Zhou^{1,#}, Shuo Song^{1,#},
Kaijing Chen¹, Rui Chen¹, Yuanlin Chen¹, Hua Bai³, Michael W.
Deem⁴, Jiankui He^{1,*}

¹ Department of Biology, Southern University of Science and Technology, Shenzhen, 518055, China.

² Department of Human Reproductive Medicine Center, Third Affiliated Hospital of Shenzhen University, Shenzhen 518001, China.

³ BaiHuaLin China People Living With HIV/AIDS Alliance, Cuipingli 10-132, Tongzhou, Beijing, China

⁴ Departments of Bioengineering and Physics & Astronomy, Center for Theoretical Biological Physics, and Graduate Program in Systems, Synthetic, and Physical Biology, Rice University, Houston, TX 77005, USA.

*Corresponding Author. Email: hejk@sustc.edu.cn

#Equal contribution

Abstract

Millions of children are born annually with inherited genetic diseases or infectious diseases acquired from parents. The recently developed CRISPR-Cas9 genome editing technique may provide an efficient and cost-effective therapeutic strategy to cure diseases with a genetic component. Genome editing at the embryonic stage has potential to permanently cure disease and confer resistance to pathogenic infections. Here, we report the first birth from human gene editing: twin girls who had undergone CCR5 gene editing as embryos were born normal and healthy in November 2018. Their father was an HIV carrier. We used CRISPR-Cas9 to reproduce a prevalent genetic variant of the CCR5 gene in fertilized oocytes during in vitro fertilization procedure, with the aim of helping the twins to be born with their own natural protection against HIV infection. Genome sequencing during pre-implantation genetic testing and after birth confirmed that the twins' CCR5 genes were edited successfully and are thus expected to confer either complete or partial HIV resistance. No off-targets, large deletions or pathogenic cancer gene mutations was observed. We here bring a novel therapy to enable acquired immunity to HIV and to control the HIV epidemic. We anticipate that human embryo genome editing will bring new hope to millions of families seeking healthy babies free from inherited or acquired life-threatening diseases.

Residential Surveillance



Elsie Chen
New York Times

HE JIANKUI

By Jennifer Doudna



Anthony Kwan—Bloomberg/Getty Images

“Going against the consensus... that CRISPR-Cas9 technology is still too experimental and dangerous to use in human embryos, he applied it to forever change the genomes of twin girls.... His reckless experimentation on the girls in China not only shattered scientific, medical and ethical norms, it was also medically unnecessary...

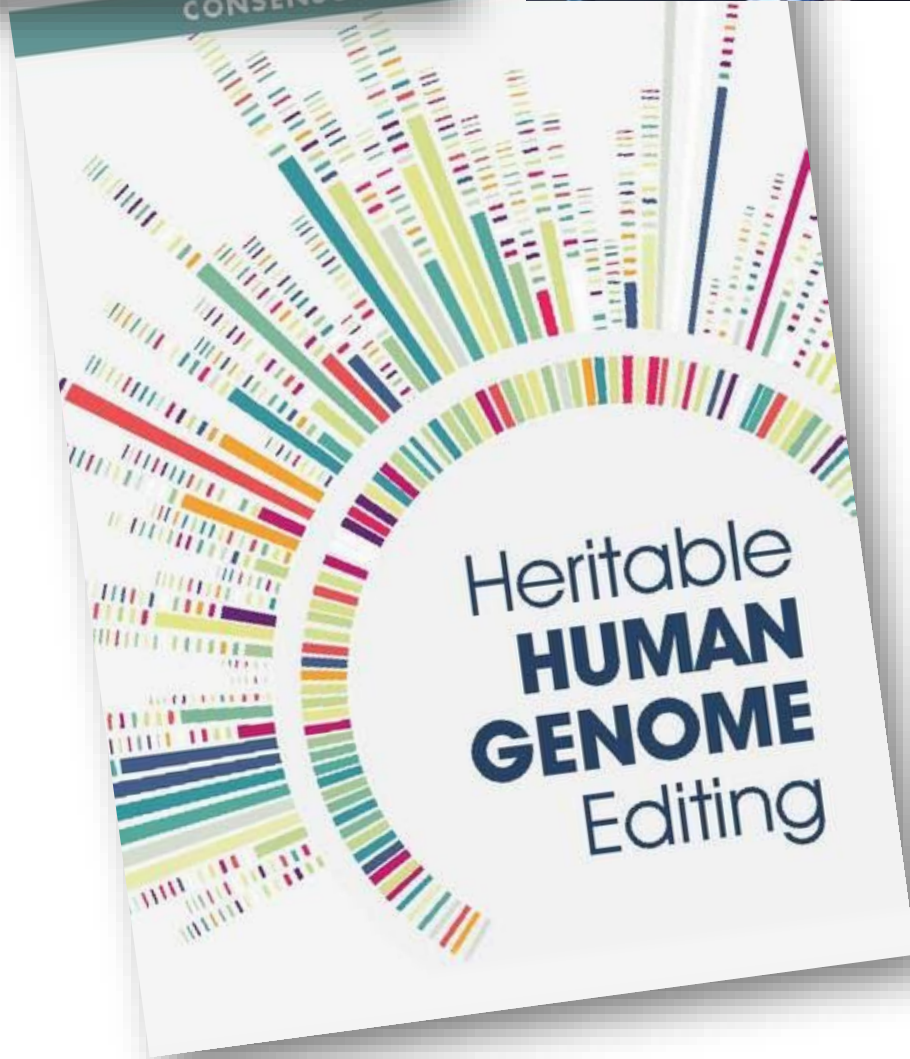
He’s fateful decision to ignore the basic medical mantra of “do no harm” and risk the unintended consequences will likely be remembered as one of the most shocking misapplications of any scientific tool in our history.”

HHGE Criteria

... should a country decide to permit them, are:

1. Limited to serious monogenic diseases (severe morbidity or premature death)
2. Limited to changing a pathogenic gene variant to a sequence common in the population and non-disease-causing
3. No healthy embryos to be subjected to HHGE
4. Limited to cases where prospective parents have no (or very poor) options for having a genetically related child.

NAS/Royal Society
Sept 2020



Denis Rebrikov

Pirogov Medical University

Moscow

«Вчера было рано, завтра будет поздно. Власть надо брать сегодня».

“Yesterday was early, tomorrow will be late.

Power must be taken today.”

-- LENIN

Andrey Rudakov /
Bloomberg

Who Wants a CRISPR Clinic?

From: "xxxxxxx" <xxxxxxx>;
Date: Wed, Dec 5, 2018 01:18 PM
To: "hejk" <xxxxxxxx >;
Cc: "xxxxxxx xxxx" < >;
Subject: CRISPR Gene Editing Embryology Lab Application Course

Dear He Jiankui,
Congratulations on your recent achievement of the first gene editing baby delivered by your application!
My name is XXXXXXX, and I am the Business Director's Assistant at XXXXXXX Fertility & Gynaecology Center, in Dubai.
Our Embryologist is interested in partaking in a course regarding CRISPR gene editing for Embryology Lab Application.
Does your facility offer this type of course?

Kind regards,
XXXXXXXXXXXX
Business Director's Assistant
XXXXXX

New hope for China's
left-behind kids p. 1226

How pesticides should
be regulated p. 1232

A twist on photoemission
delay pp. 1239 & 1274

Science

\$15
22 SEPTEMBER 2017
sciencemag.org

AAAS

CRISPR PIGS

Eliminating endogenous
retrovirus in a step toward
xenotransplantation



Luhan Yang (eGenesis)

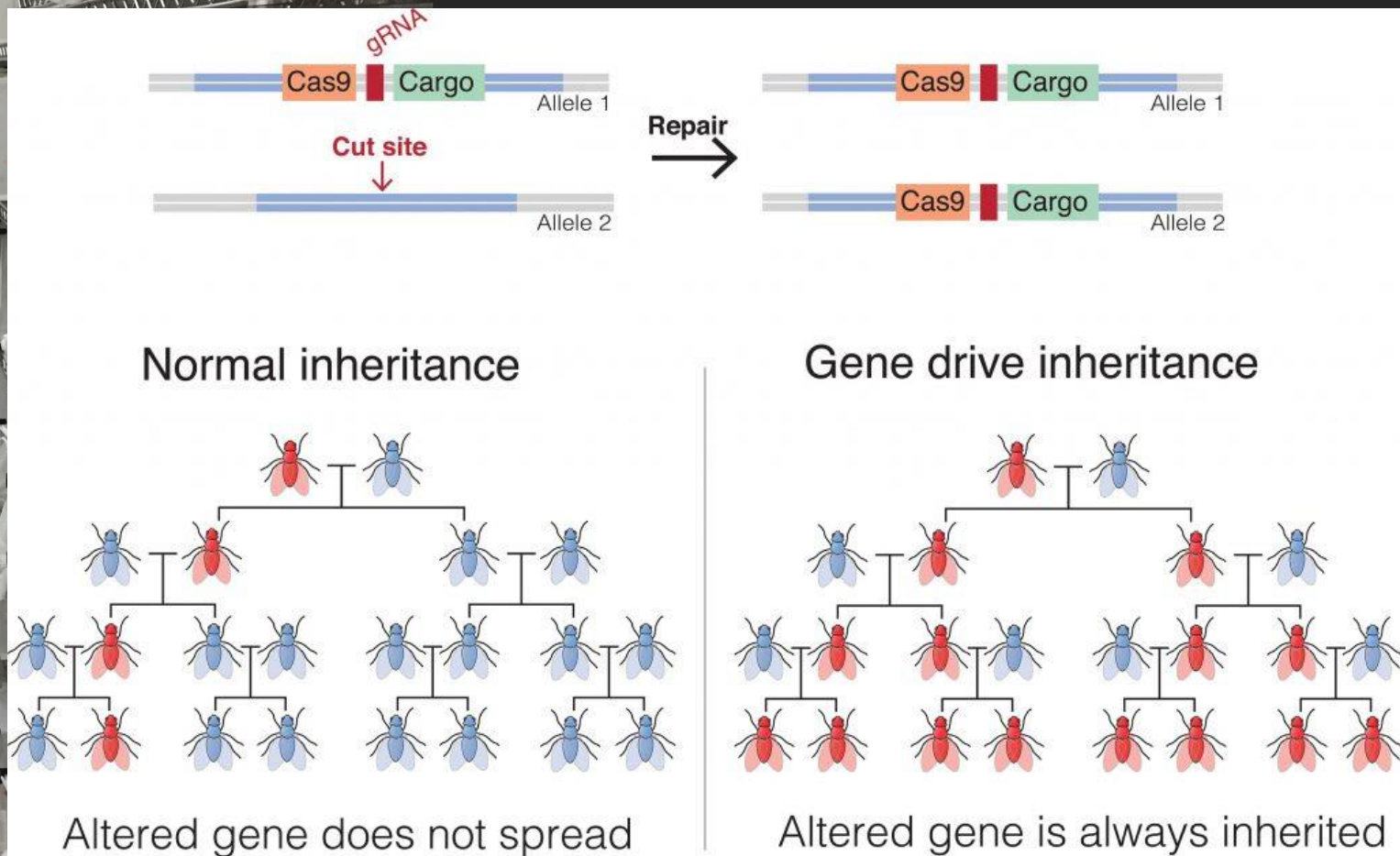
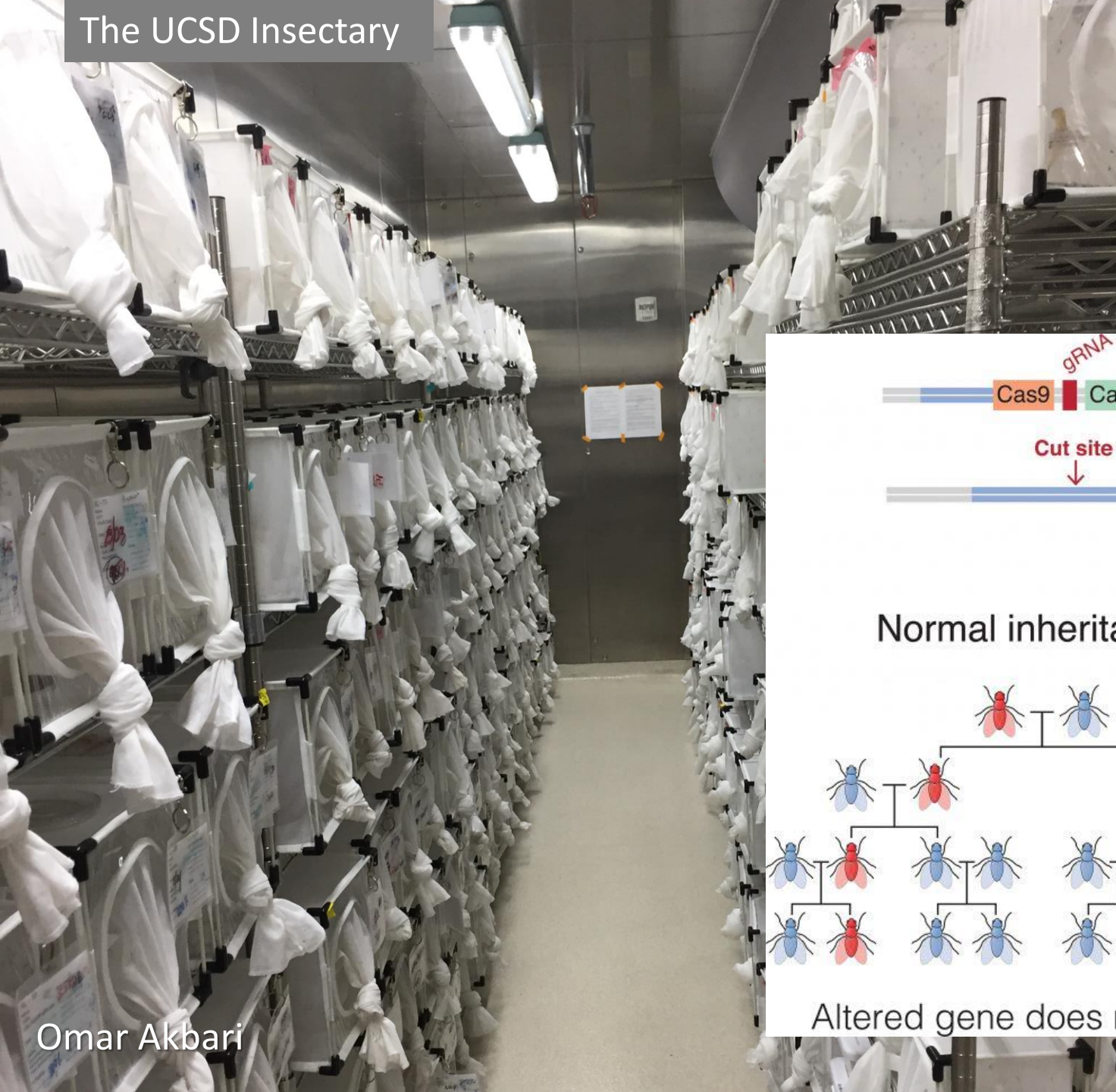
REPORT

Inactivation of porcine endogenous retrovirus in pigs using CRISPR-Cas9

Dong Niu^{1,2,*}, Hong-Jiang Wei^{3,4,*}, Lin Lin^{5,*}, Haydy George^{1,*}, Tao Wang^{1,*}, I-Hsiu Lee^{1,*}, Hong-Ye Zhao³, Yong Wang⁶, Yanan Kan¹, Ellen Shrock⁷, Emal Lasha¹, Gang Wang¹, Yonglun Luo⁵, Yubo Qing^{3,4}, Deling Jiao^{3,4}, Heng Zhao^{3,4}, Xiaoyang Zhou⁶, Shouqi Wang⁸, Hong Wei⁶, Marc Güell^{1,†}, George M. Church^{1,7,9,†}, Luhan Yang^{1,†,‡}

¹eGenesis, Inc., Cambridge, MA 02139, USA.

GENE DRIVES



Beef Barn,
UC Davis



Christie Hemm Klok
Washington Post



Genome Edited Plants



Caixia Gao (Chinese Academy Sciences)



Zach Lippman (CSHL/HHMI)

Tomato is first CRISPR-edited food to go on sale in the world



ENVIRONMENT 24 September 2021

By [Michael Le Page](#)



Tomatoes with genes edited by CRISPR technology are now on sale in Japan
Courtesy of Sanatech Seed

For the first time ever, you can now buy a food altered by [CRISPR gene editing](#) – at least, if you live in Japan, where the Sicilian Rouge High GABA tomato has just gone on sale.

“We started shipping the tomatoes on September 17,” says Minako Sumiyoshi at Japanese start-up Sanatech Seed, which is selling the tomatoes directly to consumers. She says demand for the tomatoes is “not too bad”.

“It is a very significant milestone for CRISPR foods,” says ...

The first CRISPR gene-edited food has gone on sale in Japan recently, in the form of a tomato packed with an alleged increase in nutritional content.

The Sicilian Rouge High GABA tomato, created by start-up [Sanatech Seed](#), sold gene-edited seedlings to any farmers that wanted them earlier in the year, and 4,200 farmers took up the offer. Now, the tomatoes are ripe for sale.





Eriona Hysolli
Medium December 2018



The UN has declared that



1,000,000

ANIMAL AND PLANT SPECIES ARE THREATENED WITH EXTINCTION.



According to Leading Scientists;



30,000

species per year on average are being driven to extinction.

6

Per hour

150

Per day

Up To

55,000

Per year



50%

The World Animal Foundation predicts that up to

ONE-HALF OF ALL SPECIES

could become extinct by 2050.



David Liu
Broad Institute/HHMI




Behold: Base Editing



Programmable editing of a target base in genomic DNA without double-stranded DNA cleavage

Alexis C. Komor^{1,2}, Yongjoo B. Kim^{1,2}, Michael S. Packer^{1,2}, John A. Zuris^{1,2} & David R. Liu^{1,2}



Alexis Komor
(UCSD)

Programmable base editing of A·T to G·C in genomic DNA without DNA cleavage

Nicole M. Gaudelli^{1,2,3}, Alexis C. Komor^{1,2,3}†, Holly A. Rees^{1,2,3}, Michael S. Packer^{1,2,3}†, Ahmed H. Badran^{1,2,3}, David I. Bryson^{1,2,3}† & David R. Liu^{1,2,3}

Nicole Gaudelli

(Beam Therapeutics)

MEGAN MOLTENI

SCIENCE 10.21.2019 11:00 AM

A New Crispr Technique Could Fix Almost All Genetic Diseases

A less error-prone DNA editing method could correct many more harmful mutations than was previously possible.

Beam Therapeutics Cofounder And Crispr Scientist Publishes Research On New Sickle Cell Treatment In Mice



Leah Rosenbaum Forbes Staff

Innovation

I write about the business of healthcare.



David Liu inside his office at the Broad Institute in Cambridge, MA. BOSTON GLOBE VIA

Science

Contents ▾

News ▾

Careers ▾

Journals ▾

SHARE



A 4-year-old with progeria, a syndrome with features of premature aging that stems from a mutated gene. MARTIN ZABALA XINHUA/EYEVIN/REDUX

'Incredible' gene-editing result in mice inspires plans to treat premature-aging syndrome in children

By Jocelyn Kaiser | Jan. 6, 2021, 11:00 AM

GENE THERAPY

One-time CRISPR hit lowers cholesterol in monkeys

Verve Therapeutics demonstrates long-term LDL reduction for base editor therapy

by Alla Katsnelson, special to C&EN

May 19, 2021 | A version of this story appeared in Volume 99, Issue 19



HUMAN NATURE

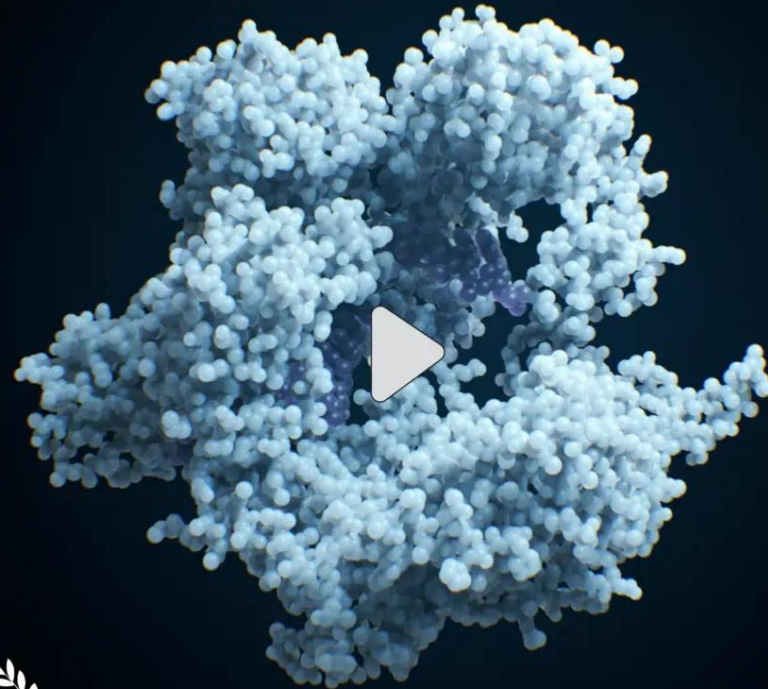
OUR FILMS

WHO WE ARE

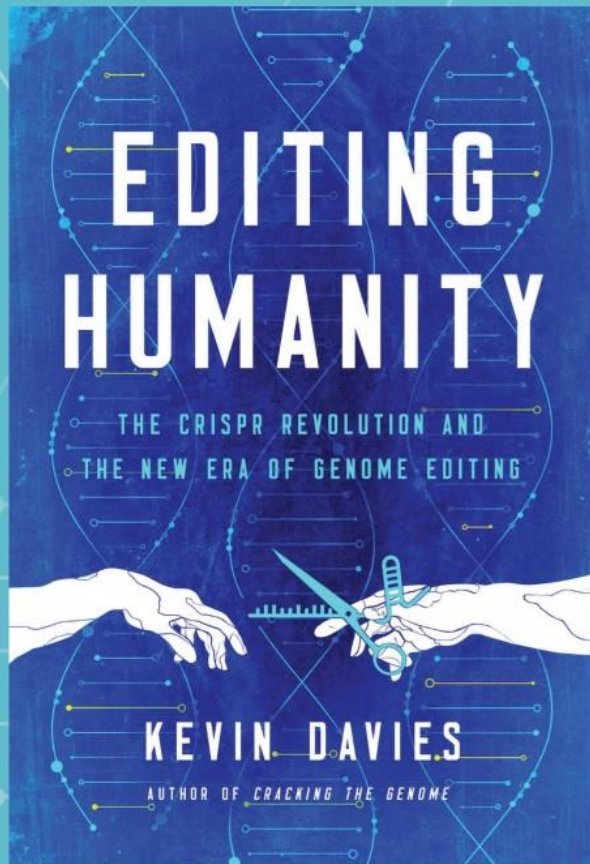
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