



# CRISPR: Rewriting DNA and the Future of Humanity

Megan Hochstrasser, PhD

Mid-Atl. Senior Phys · 1.27.21



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Genomics  
Institute

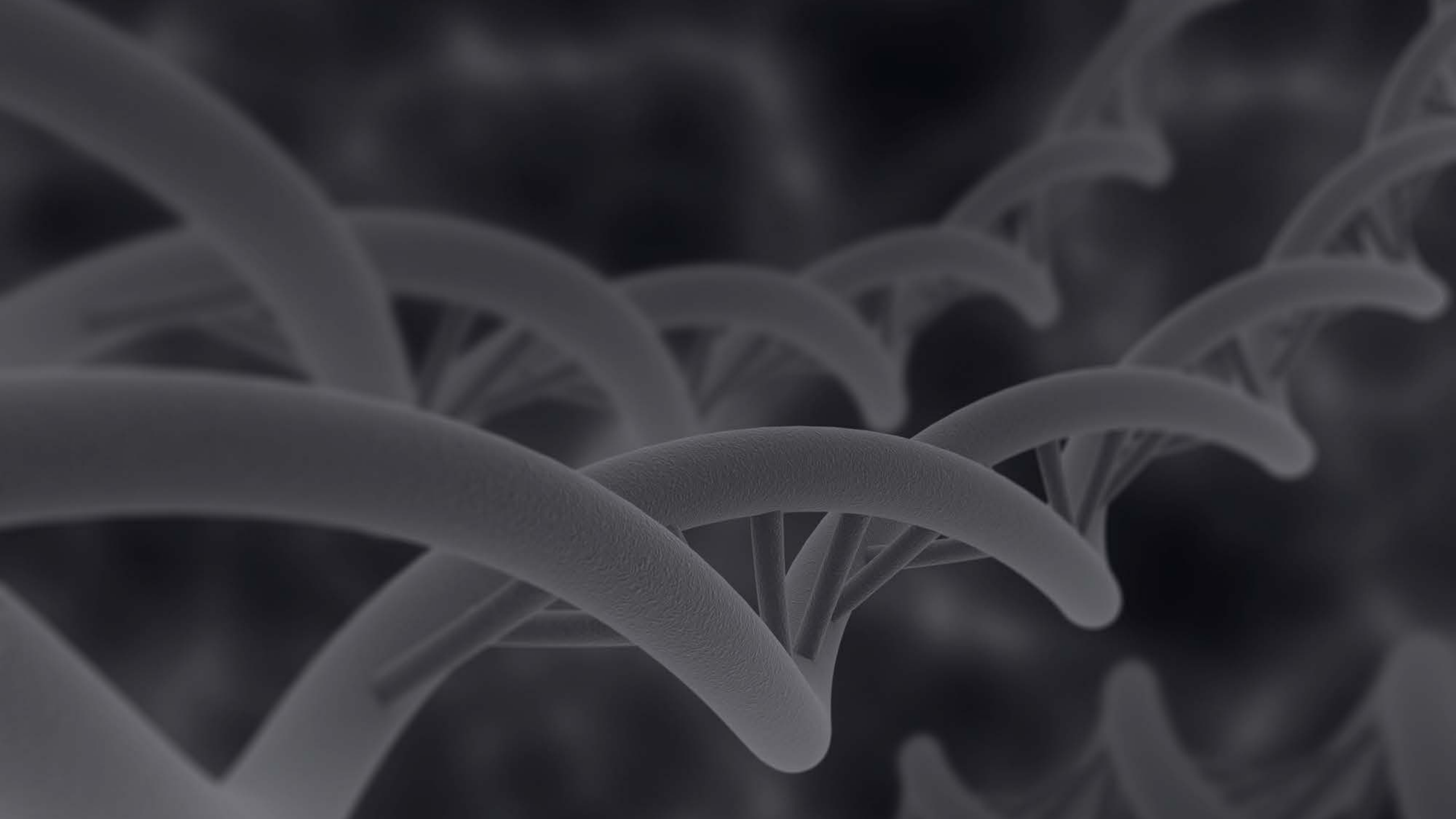


## Education & Outreach

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We aim to excite, engage, and empower diverse audiences with consistently clear and reliable resources.







# DNA controls traits

Cheek Dimples

Cilantro Taste Aversion

Cleft Chin

Dandruff

Earlobe Type

Early Hair Loss

Earwax Type

Eye Color

Fear of Heights

Fear of Public Speaking

Finger Length Ratio

Flat Feet

Freckles



Cystic Fibrosis

29 variants in the CFTR gene; relevant for Ashkenazi Jewish, European, Hispanic/Latino descent

D-Bifunctional Protein Deficiency

2 variants in the HSD17B4 gene

Dihydrolipoamide Dehydrogenase Deficiency

1 variant in the DLD gene; relevant for Ashkenazi Jewish descent

Familial Dysautonomia

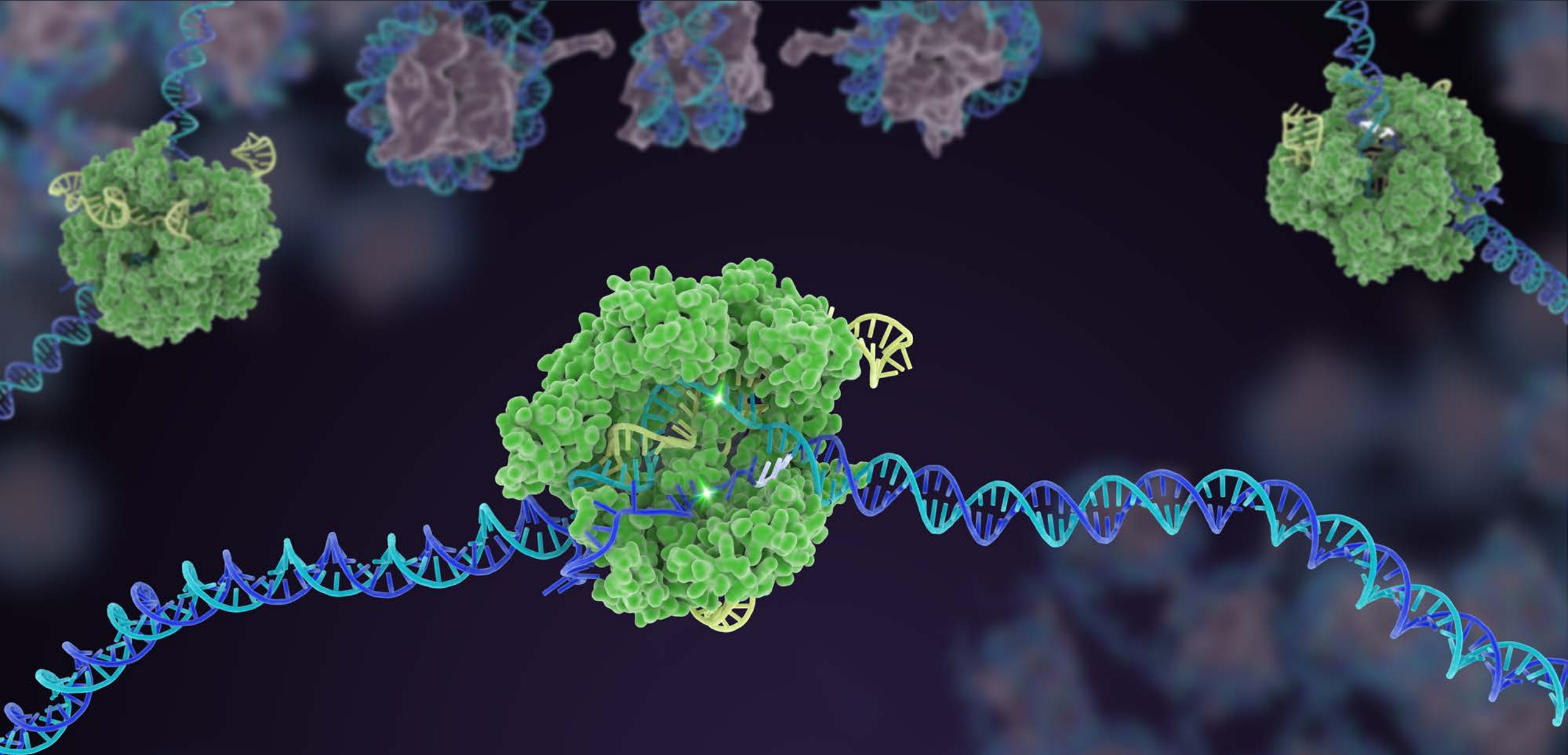
1 variant in the IKBKAP gene; relevant for Ashkenazi Jewish descent

Familial Hyperinsulinism (ABCC8-Related)

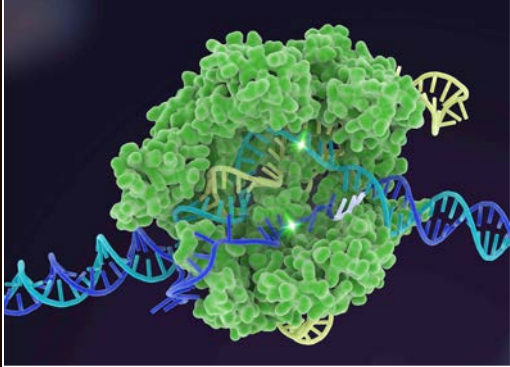
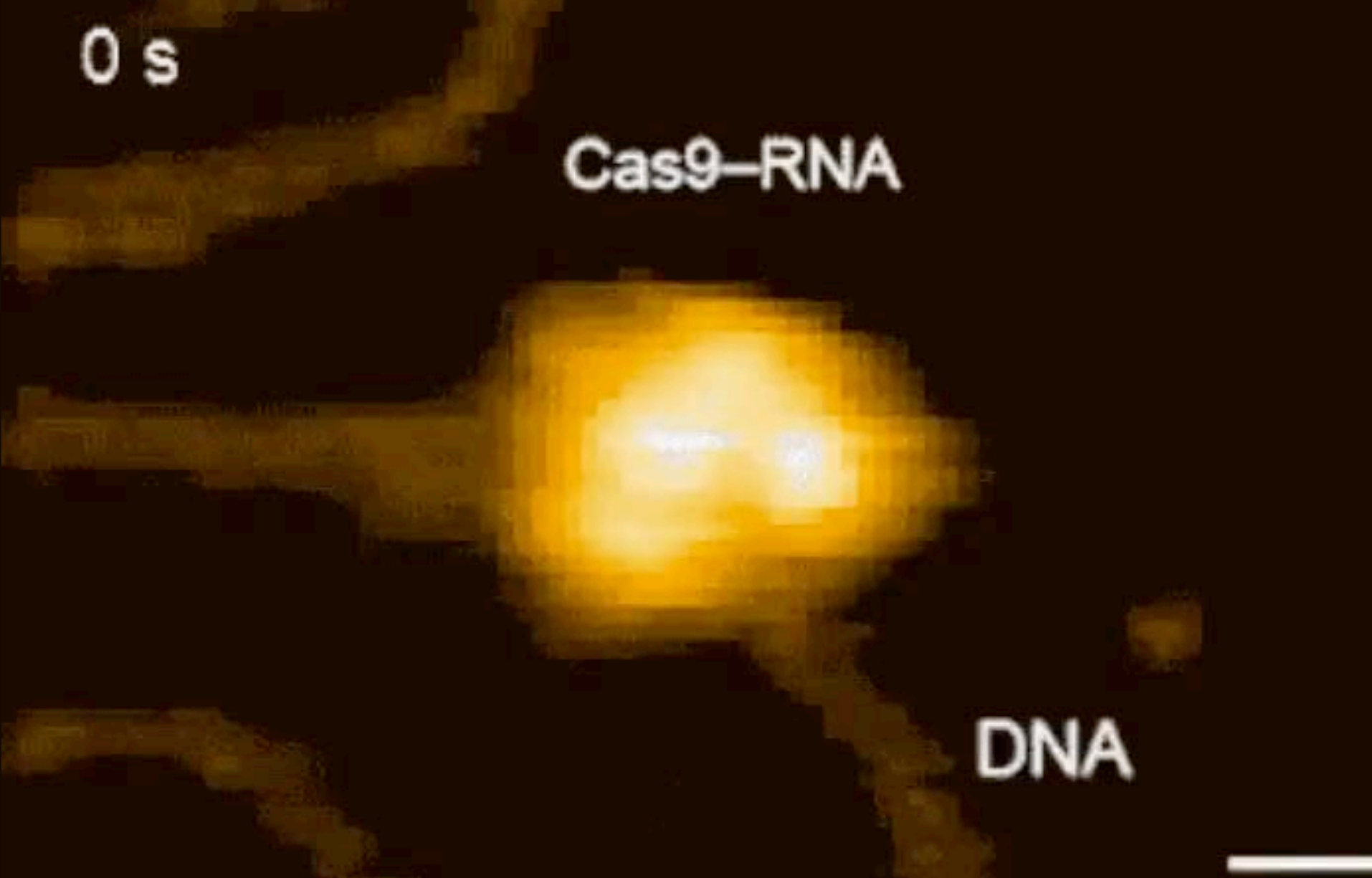
3 variants in the ABCC8 gene; relevant for Ashkenazi Jewish descent



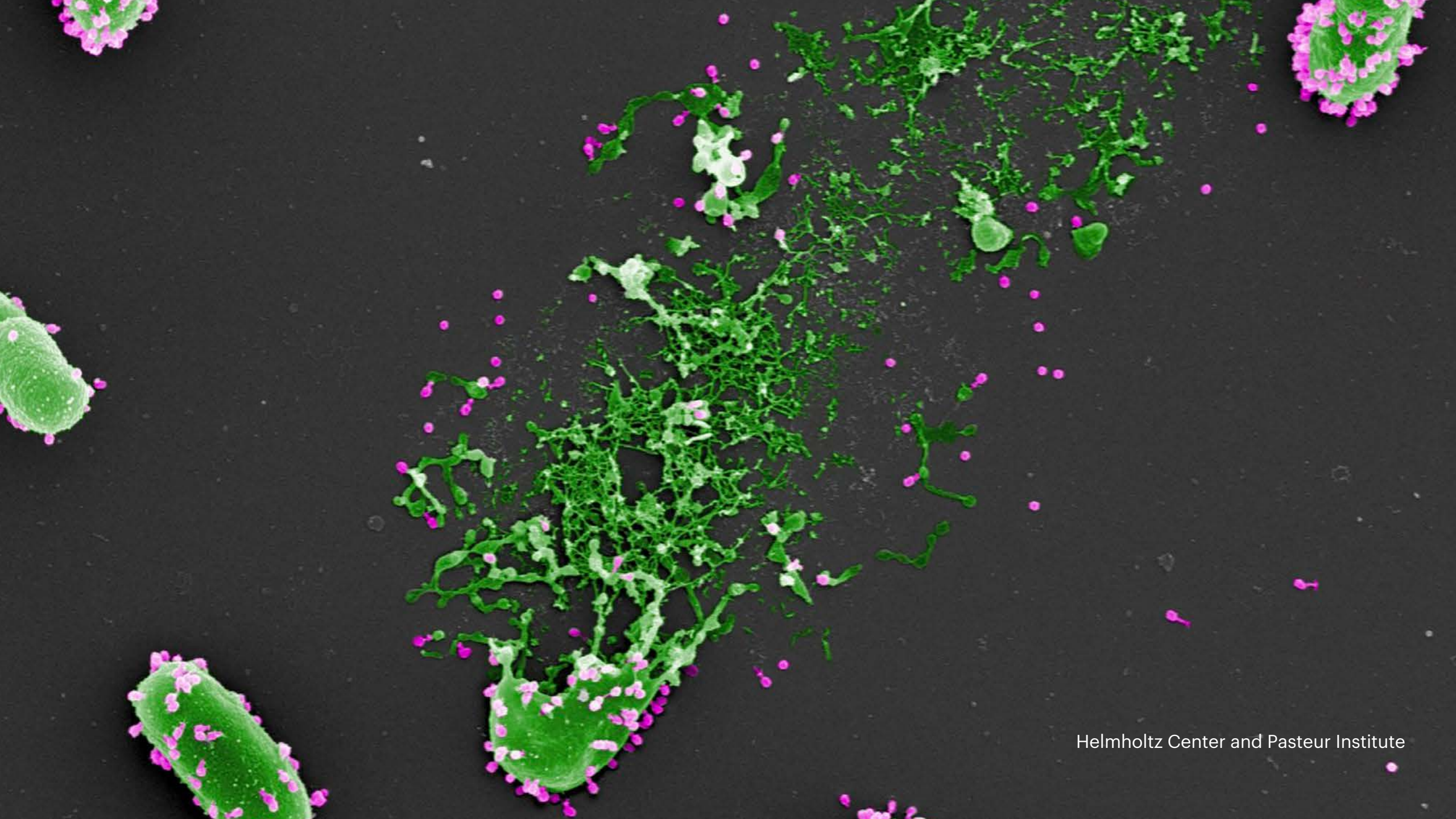
# CRISPR lets us change DNA



# Seeing is believing...









# Phage Invaders: A CRISPR Game



**15** escapes before cell death

**Phase 5**

**0** destroyed

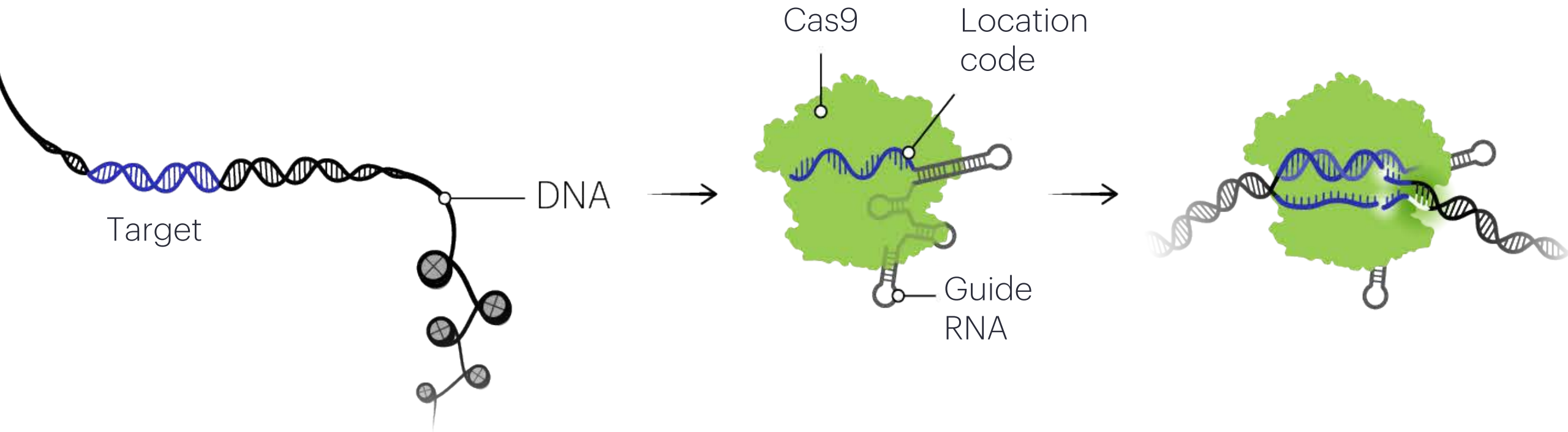
[tinyurl.com/  
phage-invaders](https://tinyurl.com/phage-invaders)

gRNA: 1 2 3 4

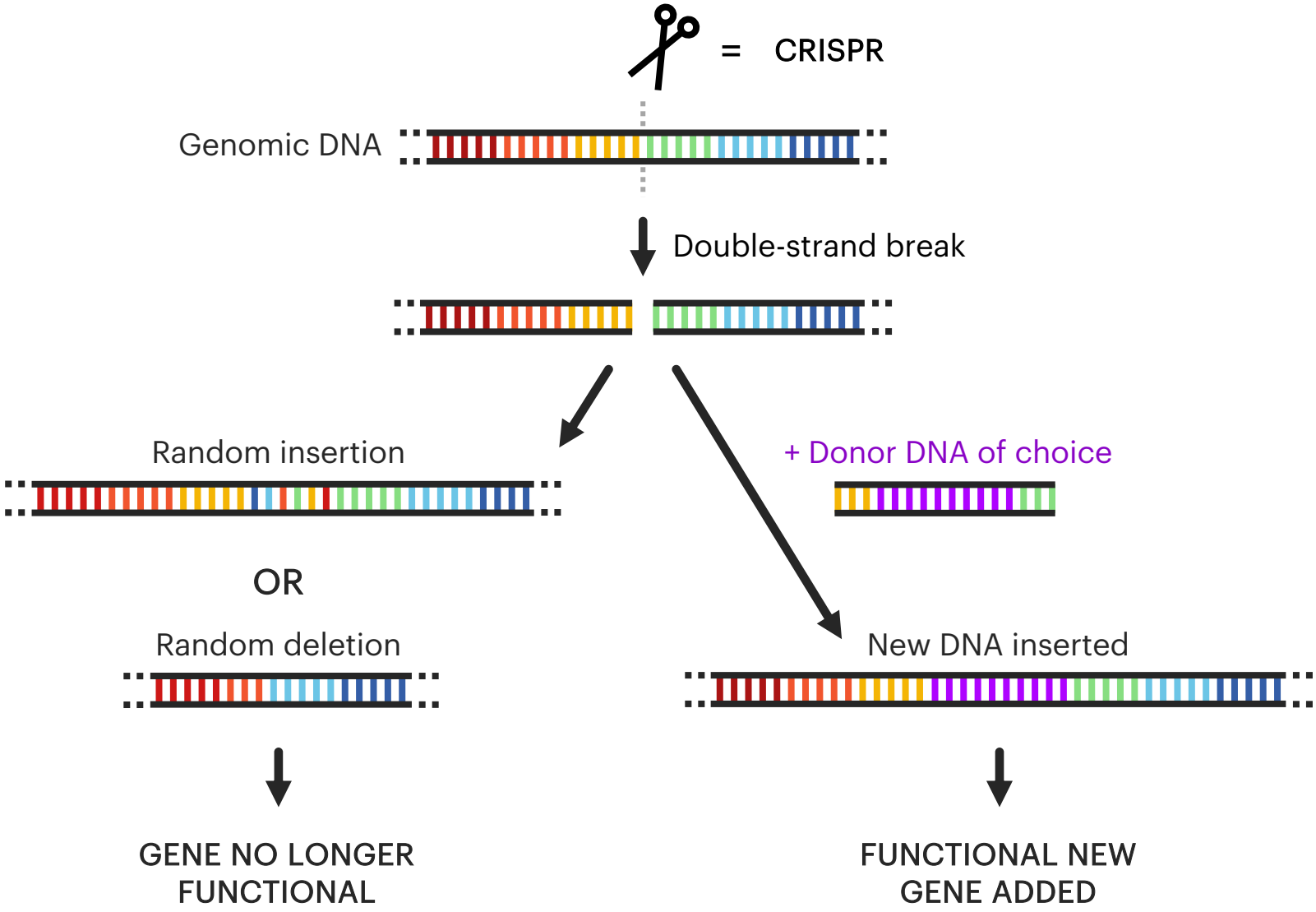
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# How to program Cas9

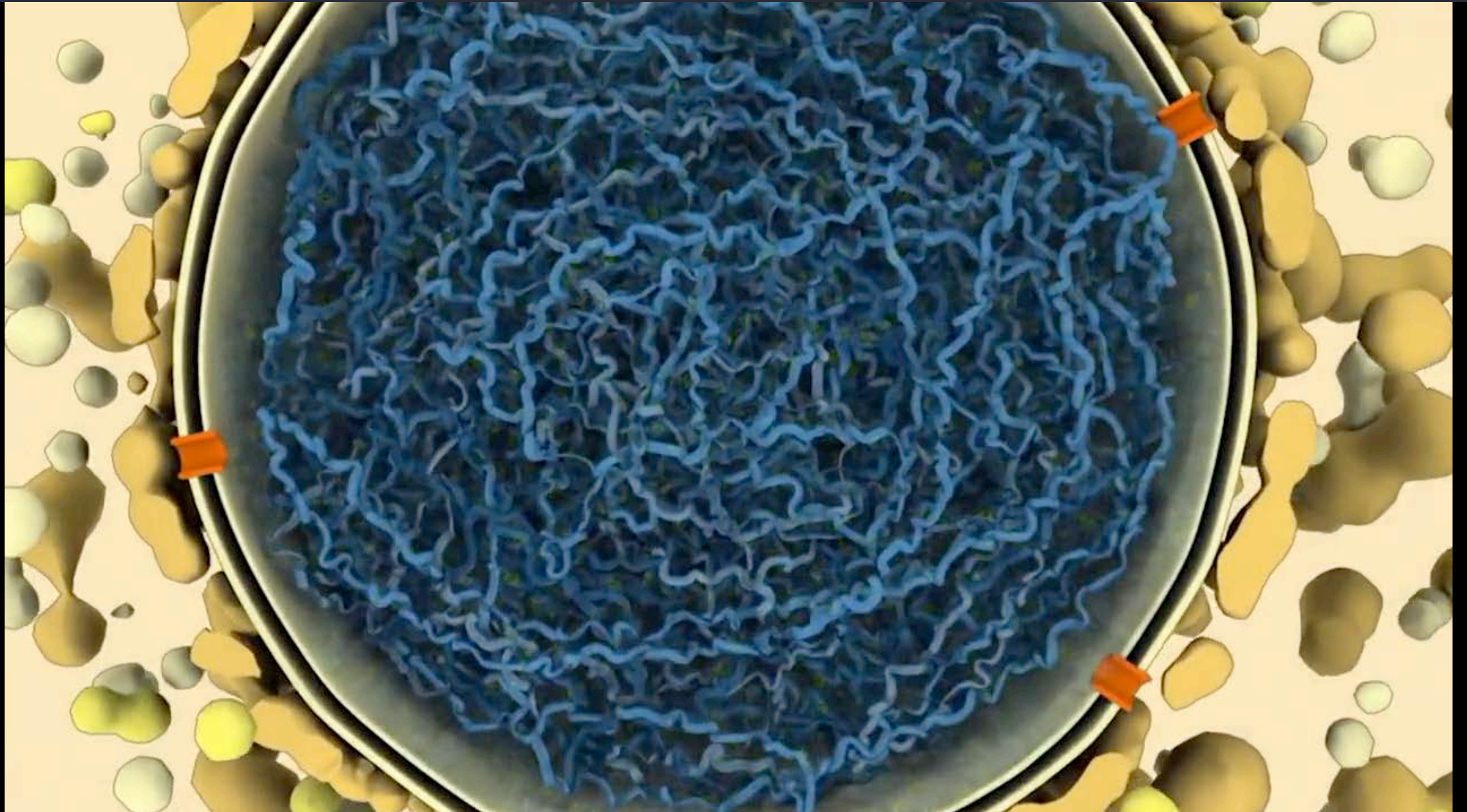


# Genome editing begins with a cut



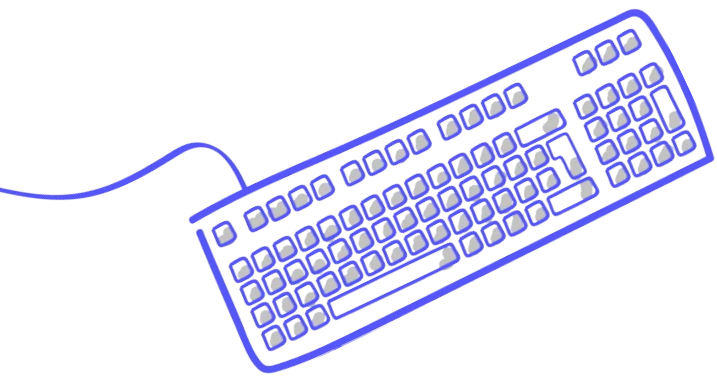


# Genome editing in action

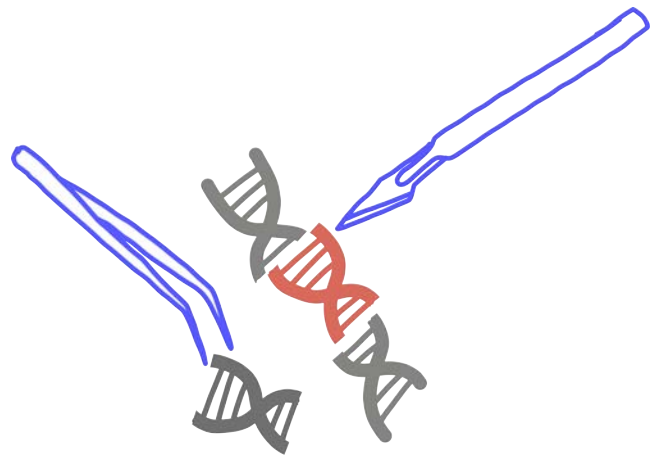


# CRISPR lets us change DNA in living cells

Twinkle, twinkle, **b**attle star



CRISPR



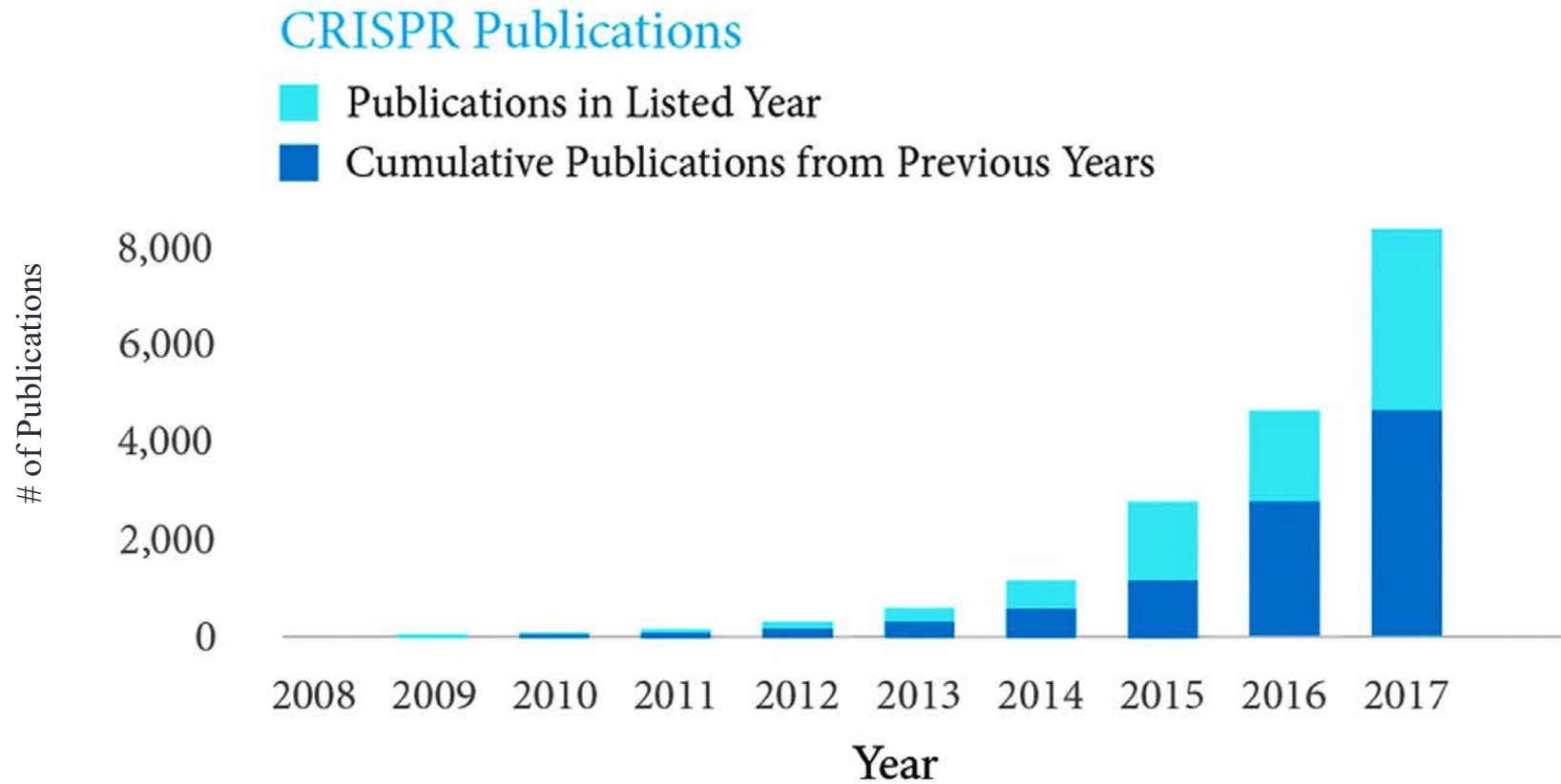
Twinkle, twinkle, little star







# The power of CRISPR





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Genomics  
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## OUR VISION:

A world in which genome engineering innovations benefit humanity and are accessible to all.

Berkeley & UCSF  
UNIVERSITY OF CALIFORNIA



# Innovative Genomics Institute

## OUR VISION:

A world in which genome engineering innovations benefit humanity and are accessible to all.



Human  
Health



Sustainable  
Agriculture



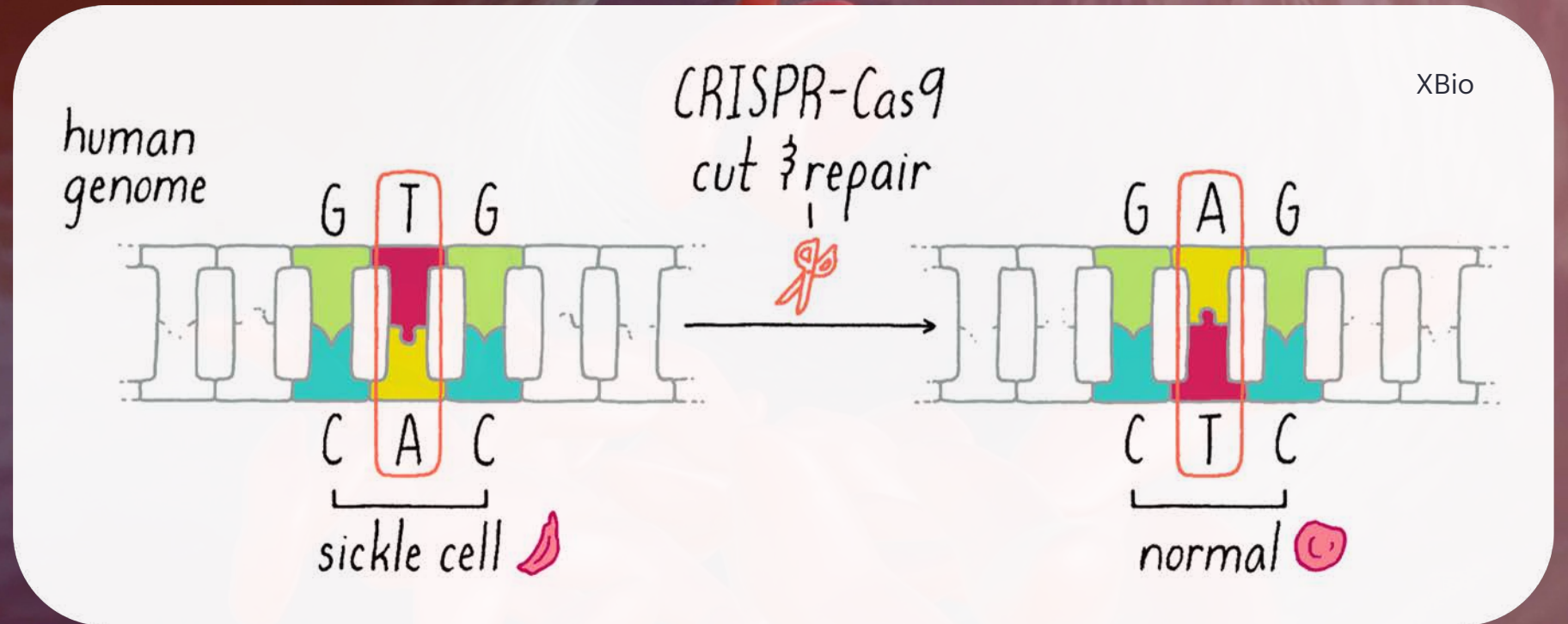
Advancing  
Genome  
Engineering



Public  
Impact



# Editing to treat genetic disease: Sickle cell



# Editing to treat genetic disease: Sickle cell

## CRISPR For Sickle Cell Disease Shows Promise In Early Test

November 19, 2019 · 5:14 PM ET  
Heard on All Things Considered



ROB STEIN



Image:

Meredith Rizzo/NPR



# Editing to treat genetic disease: Blindness (LCA10)

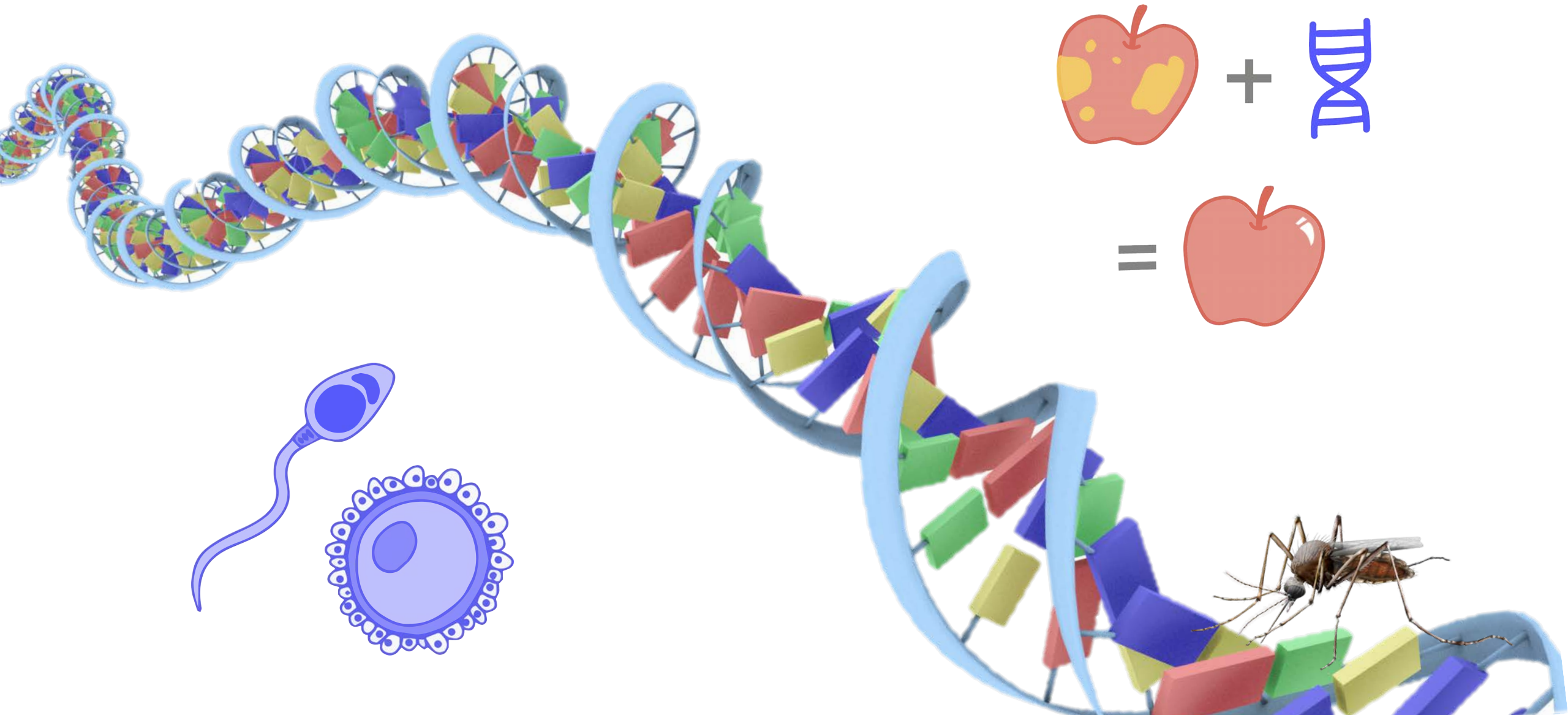


**AP**

**Doctors try 1st CRISPR editing in the body for blindness**

By MARILYNN MARCHIONE March 4, 2020

Now we can edit DNA, so what *should* we edit?

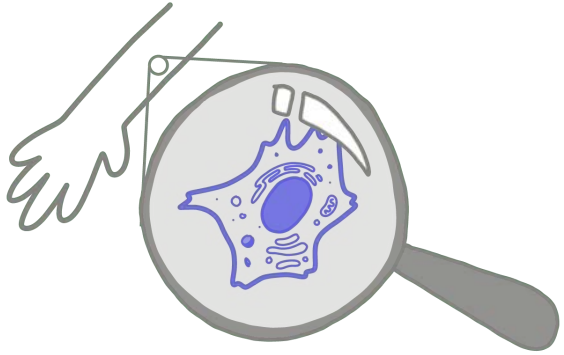




# Human germline editing



# A world of difference

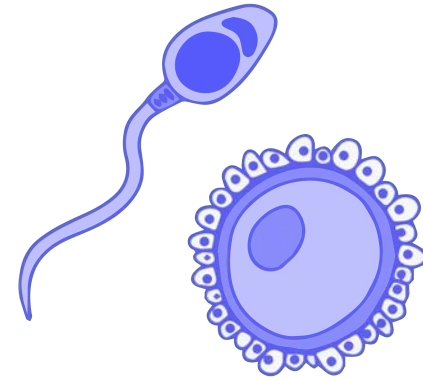


## Somatic Cells

Non-heritable

One Individual  
Affected

vs



## Germ Cells

Heritable

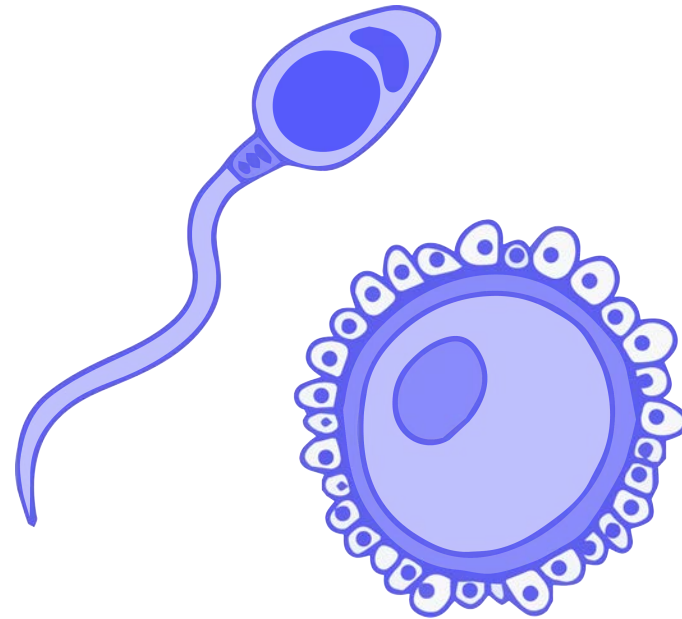
Individual and Offspring  
Affected

# Ethical questions about the germline

Technical risks

Philosophical risks

Social risks



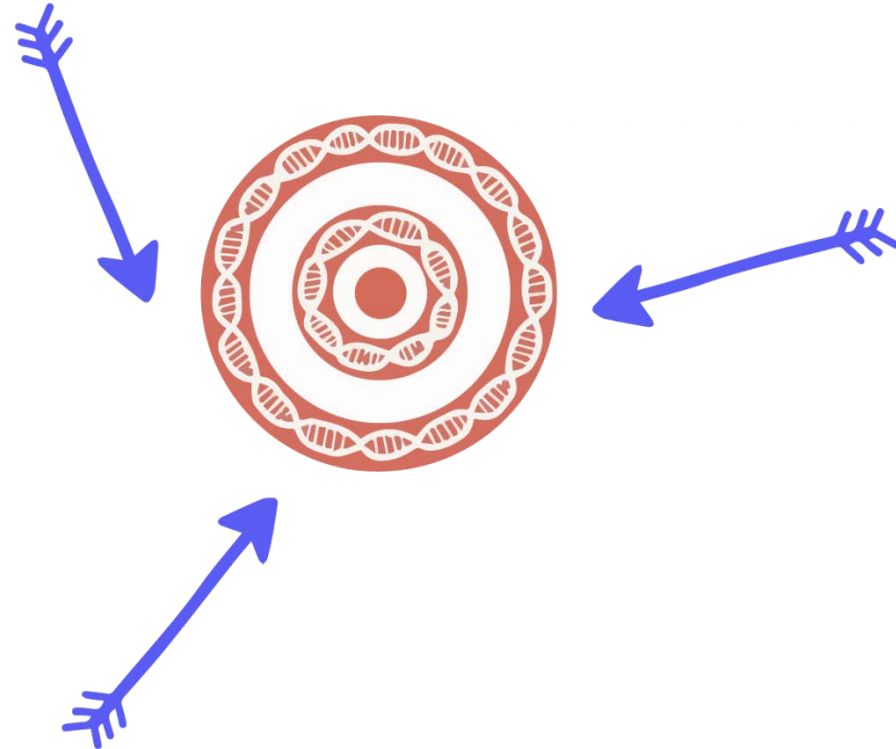
# Ethical questions about the germline

**Technical risks**

Philosophical risks

Social risks

Off-target effects



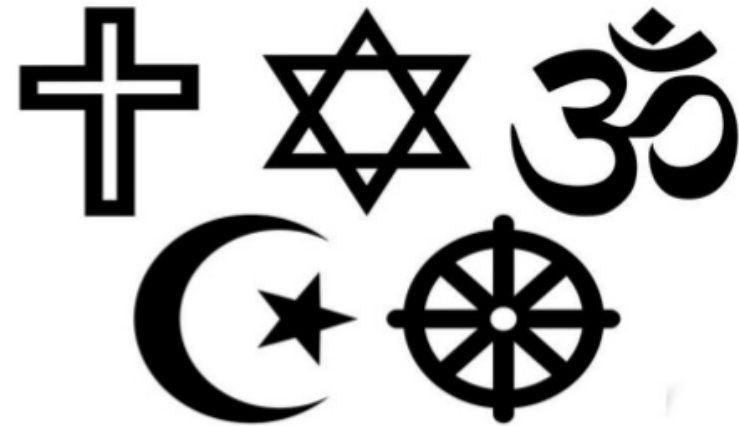


# Ethical questions about the germline

Technical risks

Philosophical risks

Social risks



# Ethical questions about the germline

Technical risks

**Philosophical risks**

Social risks



Treatment

vs.



Enhancement

# Ethical questions about the germline

Technical risks

Philosophical risks

**Social risks**





Human Genome Editing: Science, Ethics, and Governance

# Human Genome Editing

**SCIENCE, ETHICS, AND GOVERNANCE**

Committee on Human Gene Editing:  
Scientific, Medical, and Ethical Considerations

A Report of

The National Academies of  
SCIENCES • ENGINEERING • MEDICINE

THE NATIONAL ACADEMIES PRESS  
Washington, DC  
[www.nap.edu](http://www.nap.edu)

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February 2017

# Designer babies?



## 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

*The Atlantic*



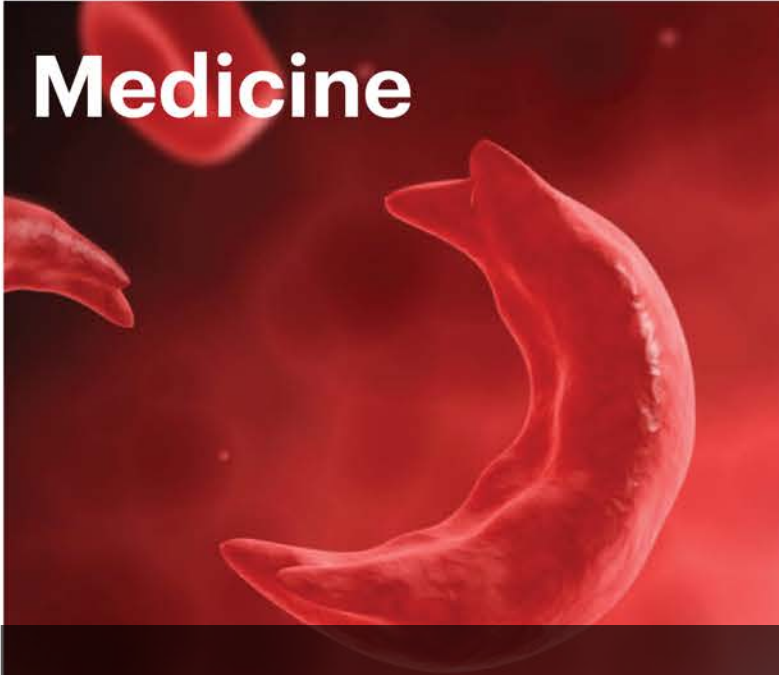
# The possibilities are endless

## Basic Research



Fundamental discoveries in  
cell function, development

## Medicine



New therapies, antibiotics,  
drug targets, diagnostics

## Agriculture

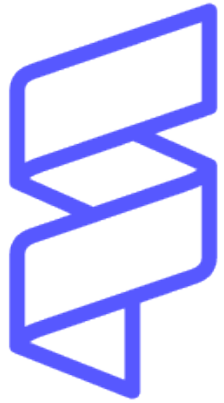


Nutritious, disease-resistant,  
climate-tolerant crops





Learn more!



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[www.innovativegenomics.org](http://www.innovativegenomics.org)

