

Gene Editing

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Method to change selected DNA
sequence in living cell

Gene Editing Tools

- Zinc Finger Nucleases (ZFNs)
- Transcription Activator-Like Effector Nuclease (TALENs)
- **CRISPR-Cas9**

Gene Editing Tools

- Zinc Finger Nucleases (ZFNs)
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- **CRISPR-Cas9**
 - Efficient, accurate, low-cost**
 - Allow edits of multiple sites in a genome through one procedure**

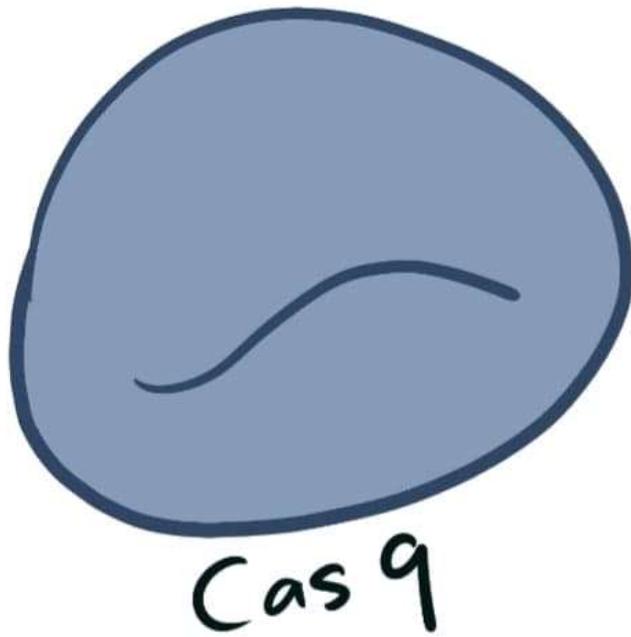
CRISPR-Cas9

Clusters of Regularly Interspaced Short
Palindromic Repeats

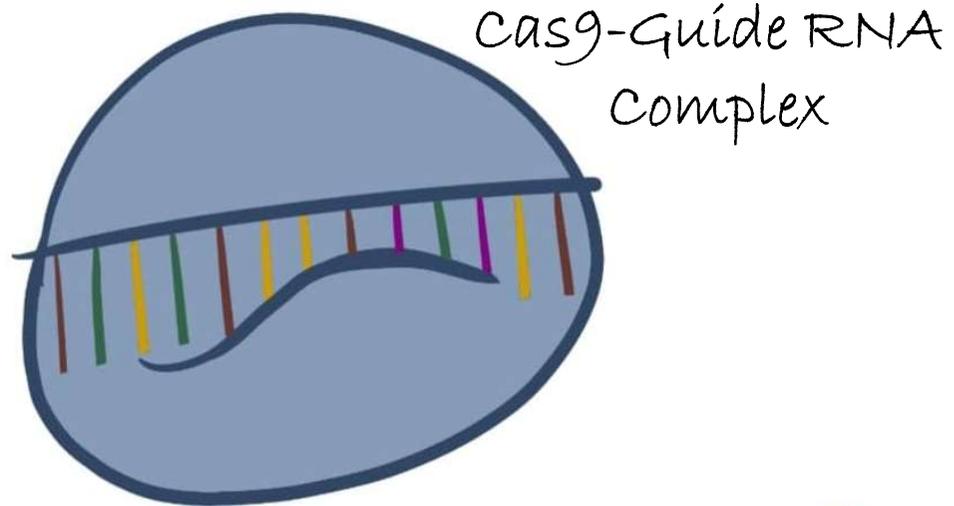
Adapted from naturally occurring genome editing
system in bacteria

To protect bacteria from viruses infection

Components

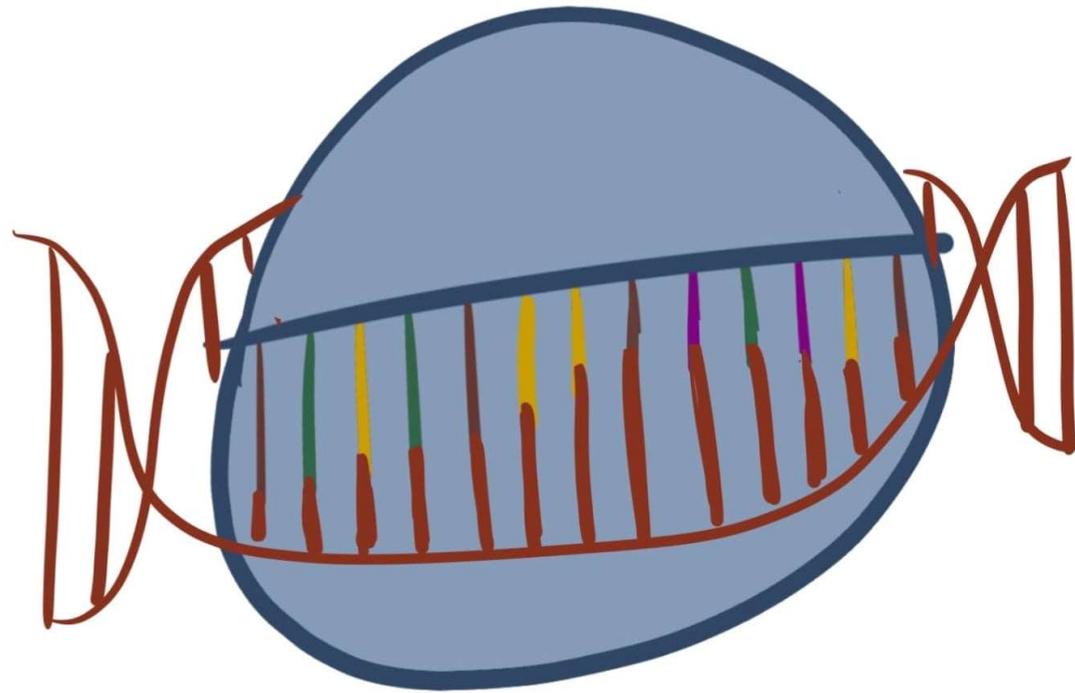


Mechanism



Virus DNA

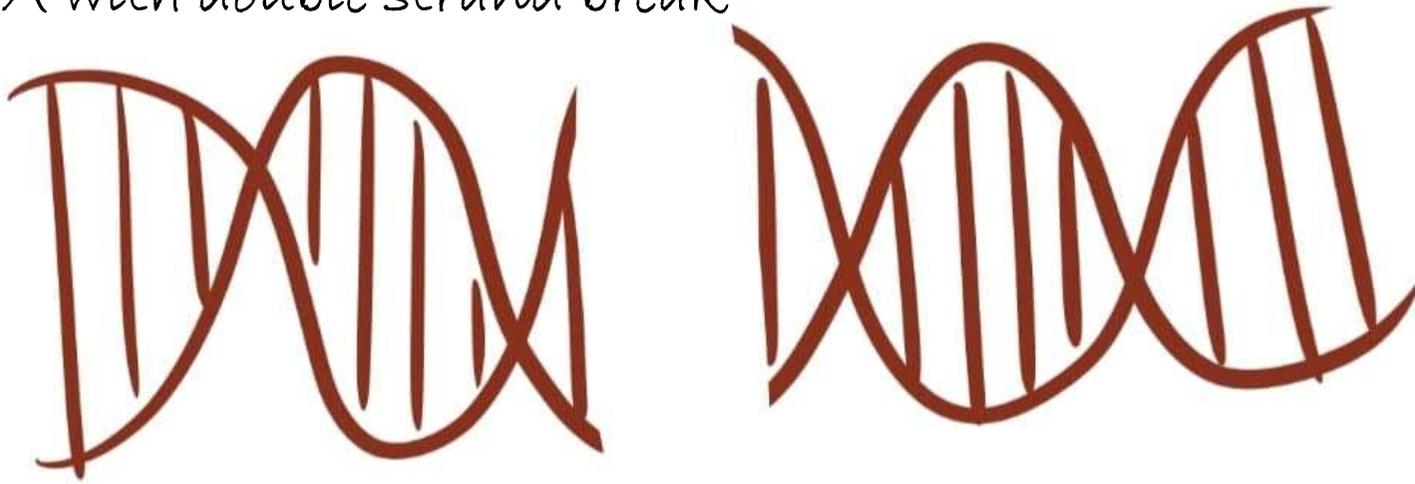
Mechanism



- Guide RNA find the target within viral genome
- Cas9 cut the virus DNA

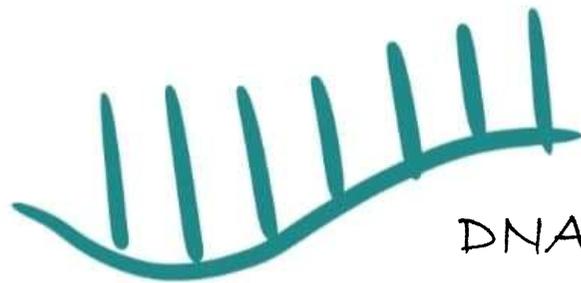
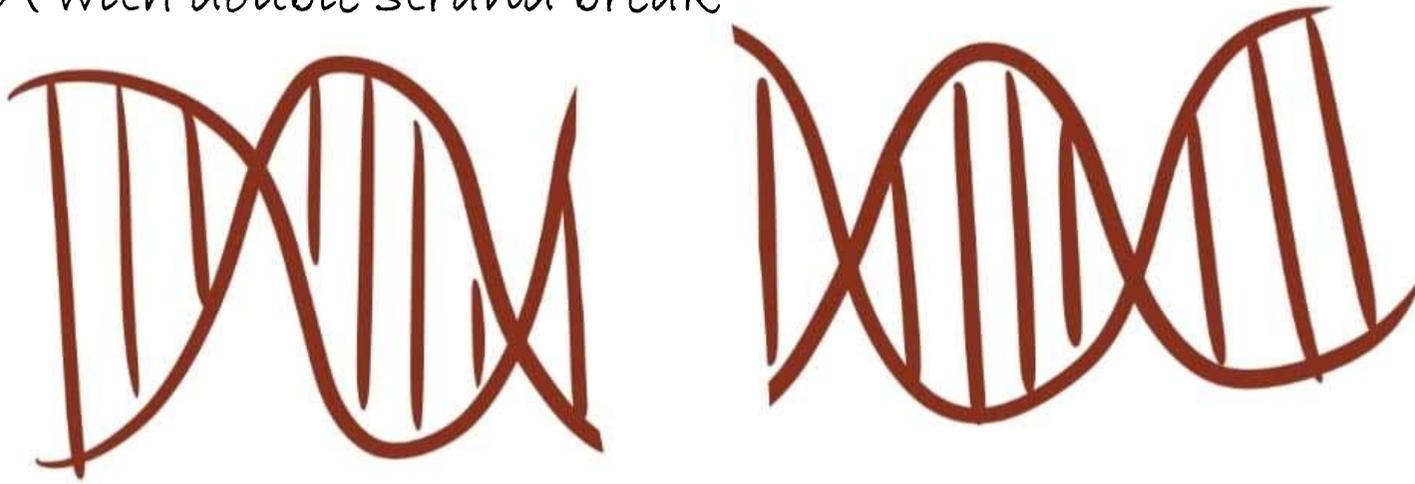
Mechanism

DNA with double strand break



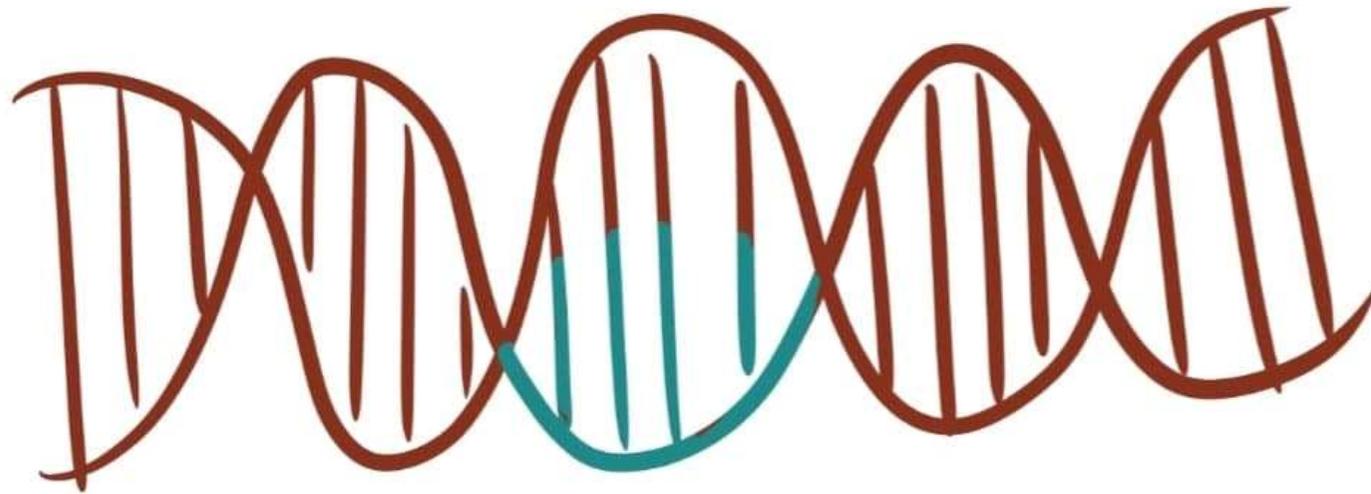
Mechanism

DNA with double strand break



DNA copy with
desired sequence

Mechanism



Edited DNA

In short:

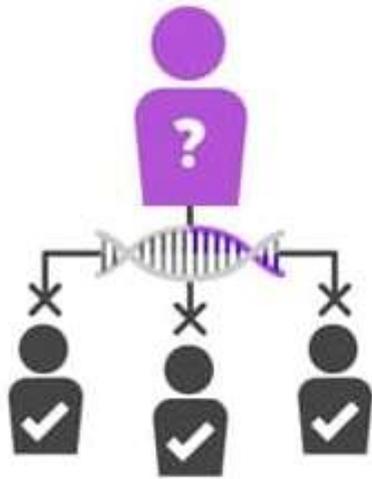
- Introduce double-strand break to a specific position
- DNA is modified during repair process

Current Applications:

- Scientific research
- Creating model organism

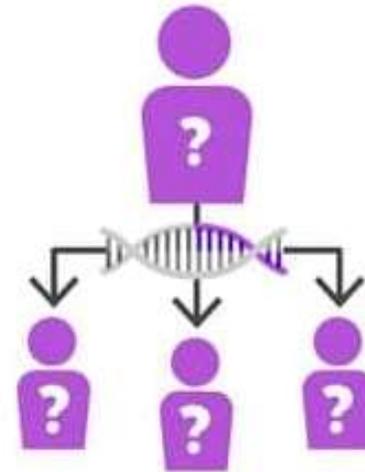
Gene Editing

Somatic Cells



Not passed to future generation

Germline Cells



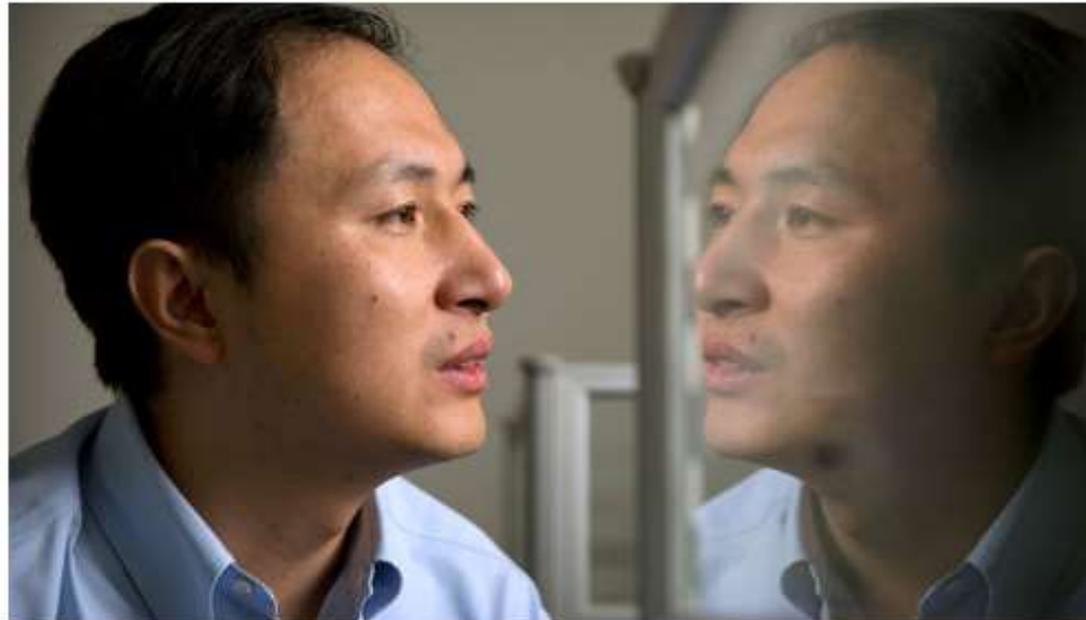
Passed to future generation



6K



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He Jiankui told The Associated Press that he carried out his experiment to protect the twin sisters from HIV infection later in life. MARK SCHIEFELBEIN/AP PHOTO

CRISPR bombshell: Chinese researcher claims to have created gene-edited twins

By **Dennis Normile** | Nov. 26, 2018, 1:10 PM

HONG KONG, CHINA—On the eve of an international summit here on genome editing, a Chinese researcher has shocked many by claiming to have altered the genomes of twin baby girls born this month in a way that will pass the modification on to future generations. The alteration is intended to make the children's cells resistant to infection by HIV, says the scientist, He Jiankui of the Southern University of Science and Technology in Shenzhen, China.

<https://www.sciencemag.org/news/2018/11/crispr-bombshell-chinese-researcher-claims-have-created-gene-edited-twins> 2019/6/24

CRISPR: Repurposed

- Promote or inhibit certain gene expressions
- Fluorescent tag for specific gene

References

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- NuffieldBioethics. 2016. Genome editing: an ethical review. <http://nuffieldbioethics.org/project/genome-editing>

Thank You!