

The National Academies of  
SCIENCES • ENGINEERING • MEDICINE

REPORT

# Human Genome Editing

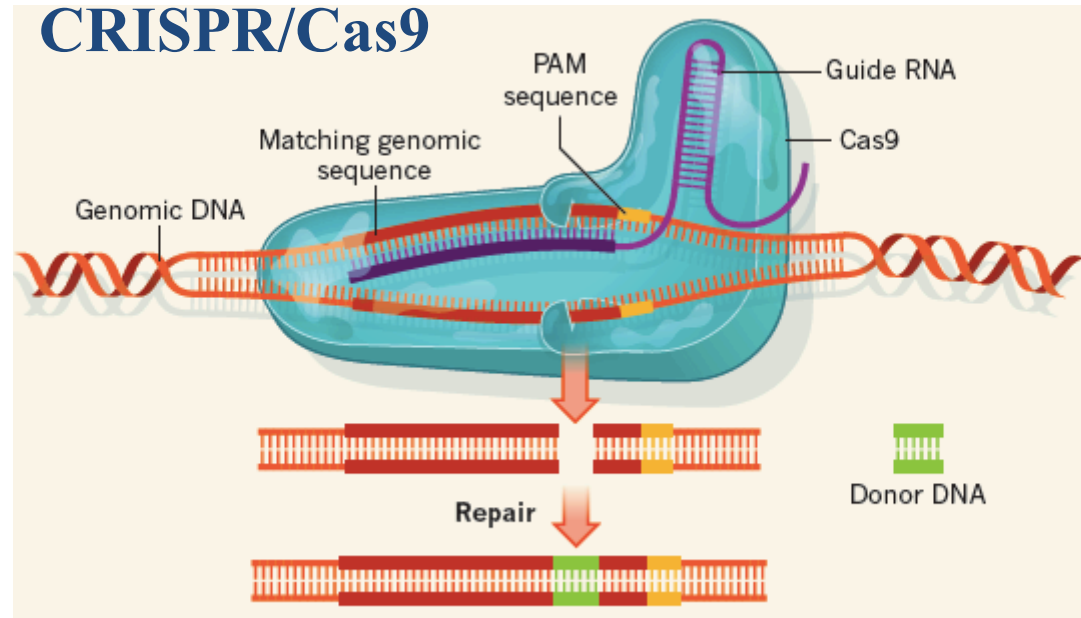
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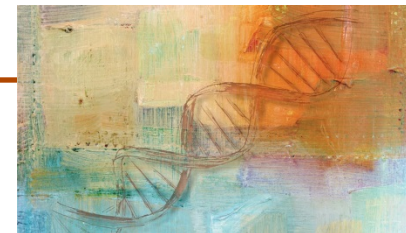
**Report, Handouts, and Archived Video available online:**  
[www.nationalacademies.org/gene-editing/consensus-study](http://www.nationalacademies.org/gene-editing/consensus-study)

# Genome Editing & CRISPR/Cas9

- Genome editing tools can add, delete or inactivate a gene, or make targeted alterations
- Not a new concept; already in use
- Specific DNA recognition precisely targets DNA cutting
- Cellular repair mechanisms introduce changes
- RNA-guided, rather than protein-guided like the earlier editing tools
- More efficient, less costly, more versatile
- Explosion of use in basic research demonstrates rapid advances possible



(Charpentier & Doudna, 2013)



# Consensus Study Charge

- Assess scientific aspects of human genome editing:
  - Current state of the science
  - Potential clinical applications
  - Efficacy and potential risks to humans
  - Standards for quantifying potential “off target events”
- Do current ethical and legal standards adequately address human genome editing?
- What are the prospects for harmonizing policies?
- Are there overarching principles or frameworks for oversight?



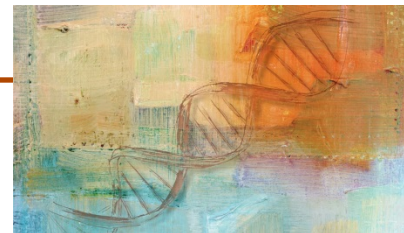
# 3 Major Applications of Genome Editing

## RESEARCH

- Basic research (purely laboratory) work on cells and tissues

## CLINICAL

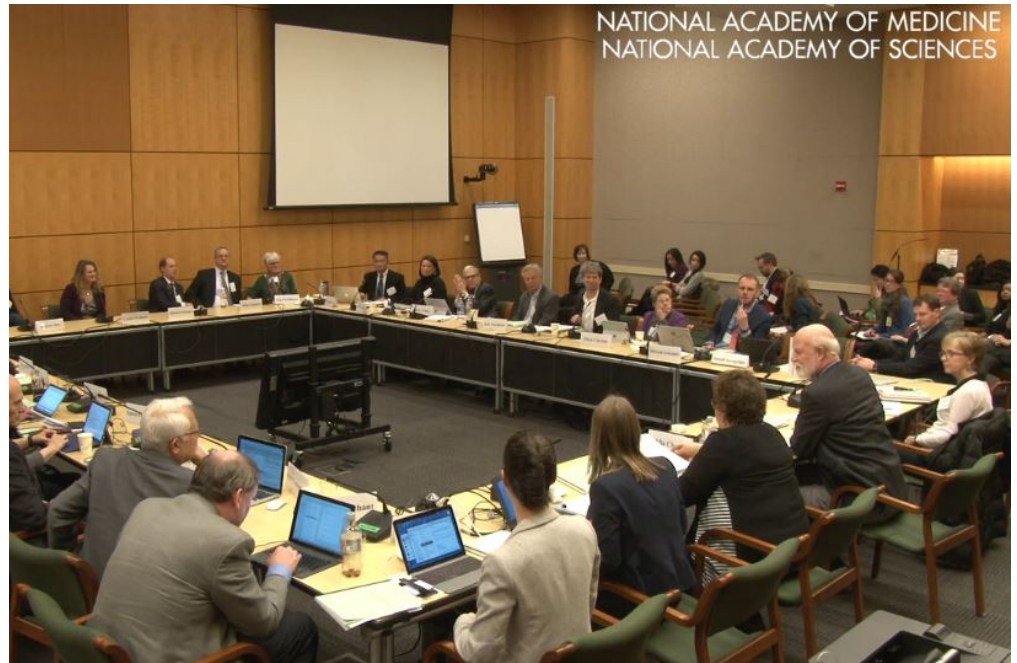
- Somatic (non-heritable) interventions in patients to treat or prevent disease
- Germline (heritable) interventions to treat or prevent disease



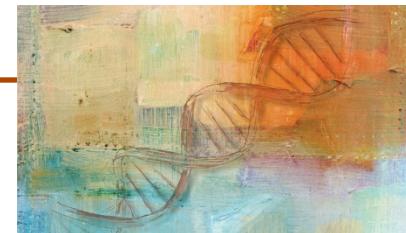
# Committee Process

- Examined the relevant literature
- Held meetings to gather input from:

- Patient advocacy groups
- Clinicians
- Researchers
- Ethicists
- Policymakers
- Public engagement experts
- Industry representatives
- The public



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