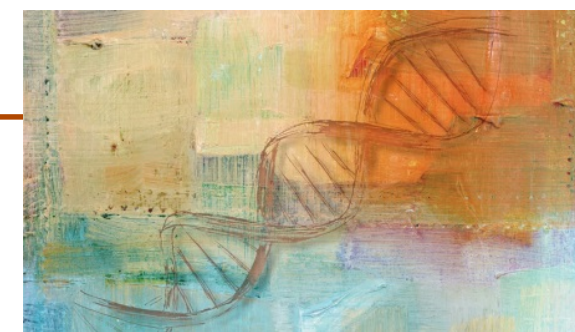


The Sciences of Human Genome Editing



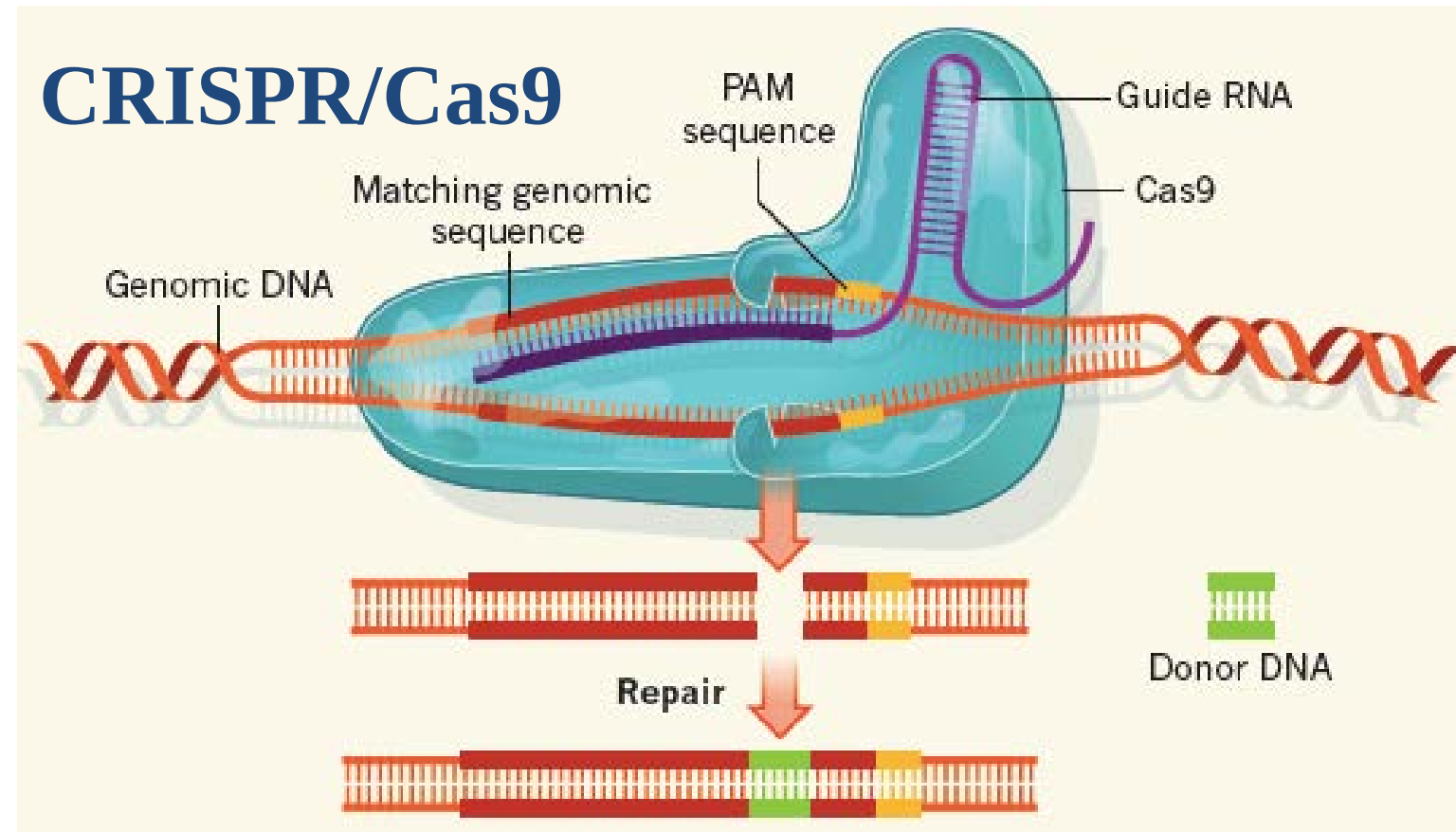
International Summit on Human Gene Editing
by National Academy of Sciences

NATIONAL ACADEMY OF SCIENCES AND
NATIONAL ACADEMY OF MEDICINE



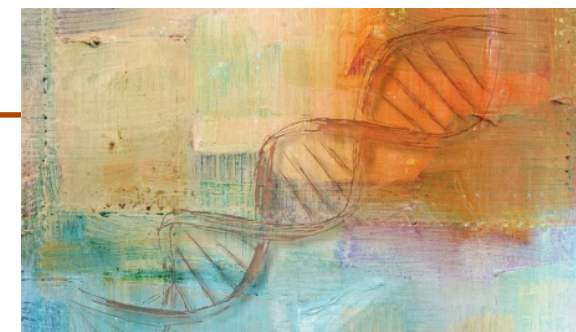
Genome Editing & CRISPR/Cas9

- Genome editing tools can add, delete or inactivate a gene, or make targeted alterations
- Not a new concept; in use for a number of years
- Specific DNA recognition precisely targets DNA cutting
- Cellular repair mechanisms used to introduce changes

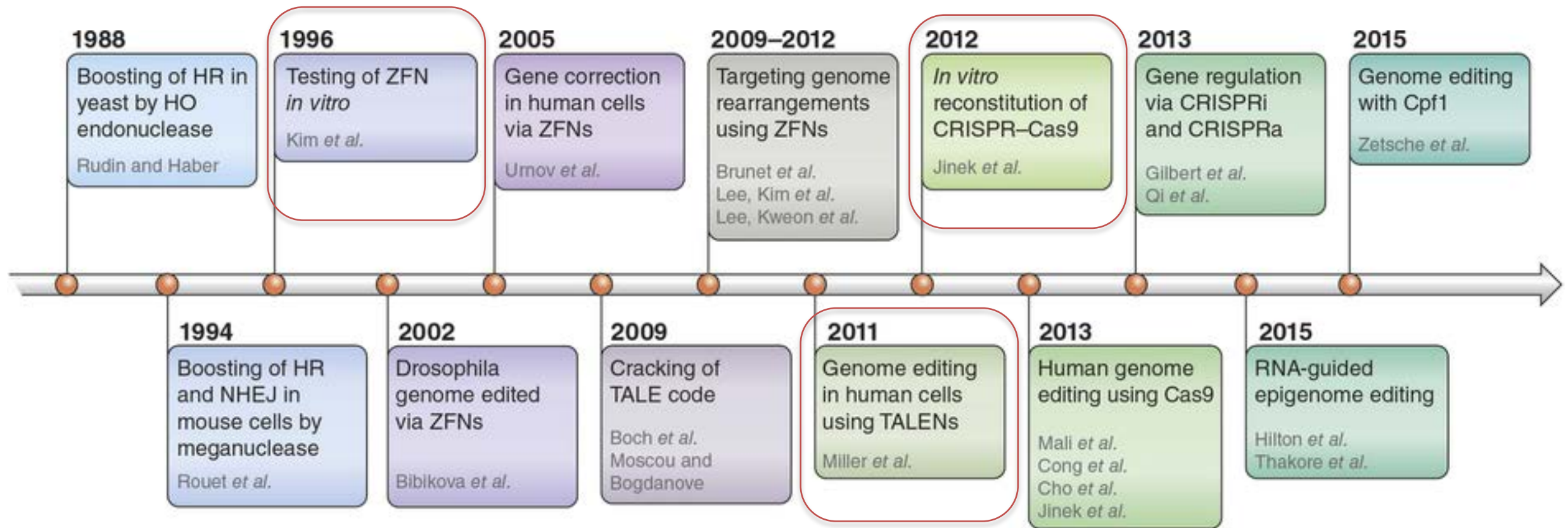


(Charpentier & Doudna, 2013)

- **RNA-guided, rather than protein-guided like the earlier editing tools**
- More efficient, less costly, more versatile, guide RNAs easily synthesised
- Can also be used to make single base edits, turn genes on and off, etc.
- Explosion of use in basic research demonstrates rapid advances possible



History of genome editing



Nature Protocols, 11, 1573–1578 (2016)

Main tools:

- ZFN, Sangamo
- TALEN, faster and more accurate than ZFN
- CRISPR-Cas9 easy and widely available

The Science of Human Genome Editing

- Moderator: Duanqing PEI, GIBH, CAS
- **Speakers:**
 - **Feng Zheng, Broad Institute**
 - **David R. Liu, Broad Institute**
 - **Angelo Lombardo, San Raffaele University**
 - **Jennifer Doudna, University of California, Berkeley**