Gene Editing

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Advancing knowledge, awareness, and education of gene and cell therapy

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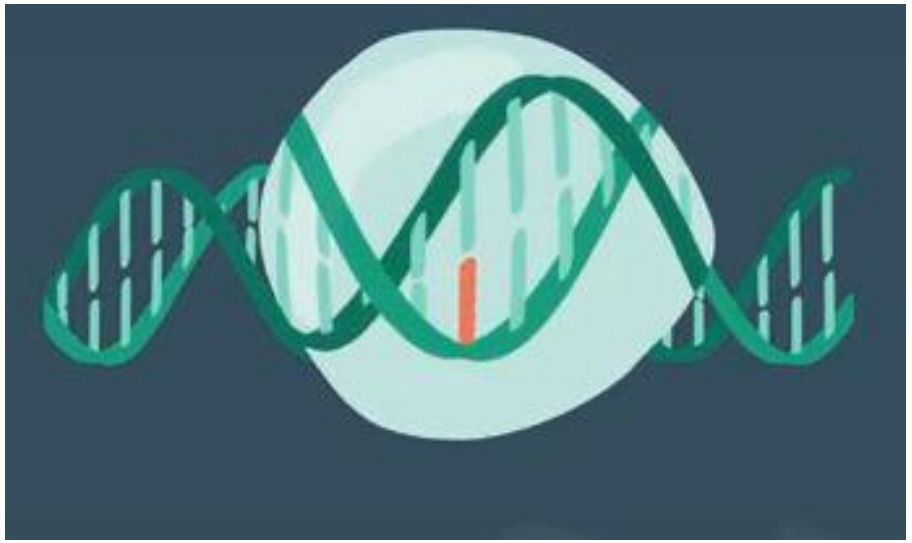
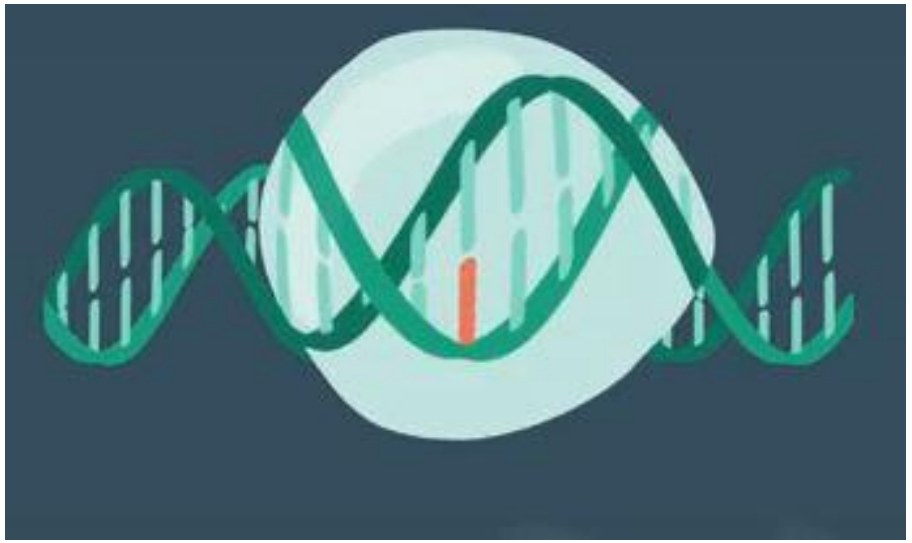
Gene editing

- Removes, disrupts, or corrects faulty elements of DNA within a gene
- An enzyme cuts DNA at one location
- The specific DNA cut allows one to change the sequence with high precision



Two Basic Genome Editing Strategies for Sickle Cell Disease

- Re-activate protective fetal hemoglobin
  - Multiple academic labs around the world
  - Two open clinical trials run by different biotechnology companies
- Directly correct variant that causes the disease (“gene correction”)
  - Several different programs moving towards clinical trials in next 6-18 months.



Example of Approach 1: Increasing Protective Fetal Hemoglobin

- Hematopoietic and progenitor cells are removed from the body
- CRISPR/Cas9 technology is used to edit a portion of the BCL11A gene
- The edited cells are then infused back into the patient as part of an autologous stem cell transplant
- The edited cells produce high levels of fetal hemoglobin (HbF) in red blood cells

Example of Approach 2: Direct Correction of the Sickle Cell Disease Gene

- Hematopoietic and progenitor cells are removed from the body
- CRISPR/Cas9 technology is used to correct the sickle cell disease gene
- The edited cells are then infused back into the patient as part of an autologous stem cell transplant
- The edited cells produce the non-sickling hemoglobin instead of the sickling hemoglobin

Preliminary Data

- Upregulation of Protective Fetal Hemoglobin
  - In pre-clinical studies achieve 40-60% expression of fetal hemoglobin
    - Higher than 20% likely provide benefit to patients
  - Patient enrolled in the United States (no data reported, too early to tell)

- Gene Correction
  - In pre-clinical studies, achieve 20-60% gene correction
    - Higher than 5-20% correction frequency that is predicted to change lives of patients
    - Moving towards clinical trials

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