CRISPR CAS 9 - GS III MAINS

Q. CRISPR-Cas9 marks the unique transformation in the treatment of diseases. Critically analyse the significance of the CRISPR-Cas9 technology in the Indian scenario. (10 marks, 150 words)

News: *CRISPR-Cas9 gene-editing tool repairs defective T cells to treat rare hereditary disease*

What's in the news?

- Some hereditary genetic defects cause an exaggerated immune response that can be fatal. Using the CRISPR-Cas9 gene-editing tool, such defects can be corrected, thus normalizing the immune response, as researchers led by Klaus Rajewsky from the Max Delbrück Center now report in "Science Immunology."

Key takeaways:

Familial hemophagocytic Lymphohistiocytosis (FHL):

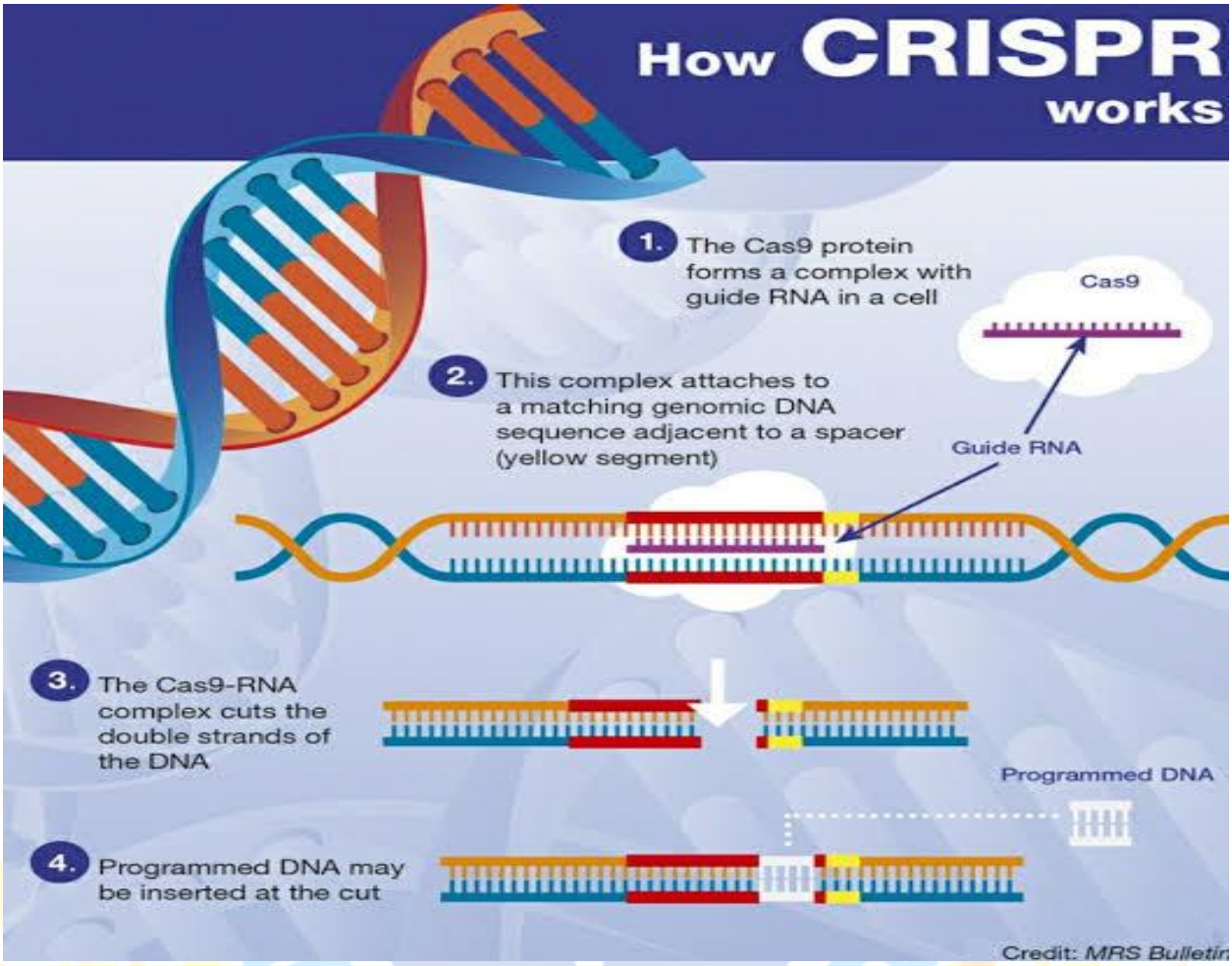
- It is a rare disease of the immune system that usually occurs in infants and young children under the age of 18 months.
- The condition is severe and has a high mortality rate.
- It is caused by various gene mutations that prevent cytotoxic T cells from functioning normally.

CRISPR-Cas9 Genome Editing:

- CRISPR-Cas9 is a unique technology that enables scientists to edit parts of the genome by removing, adding or altering sections of the DNA sequence.
- **CRISPR full form - Clustered Regularly Interspaced Palindromic Repeats.**
- CRISPRs are specialized stretches of DNA, and the **protein Cas9** ("CRISPR-associated") is an enzyme that acts like a pair of molecular scissors, capable of cutting strands of DNA.
- CRISPR is a dynamic, versatile tool that allows us to target nearly any genomic location and potentially repair broken genes.
- It can remove, add or alter specific DNA sequences in the genome of higher organisms.

How does it work?

- DNA sequence is cut at a particular spot and then with help of a guide RNA (gRNA) that place is re-made with desired base pairing. This way genes can be altered.
- It allows scientists to selectively edit genome parts and replace them with new DNA stretches
- Cas9 is the enzyme which acts as a "molecular scissors" and helps in cutting the DNA sequence.
- **CRISPR is a collection of DNA sequences that direct Cas9 where to cut and paste.**



Applications of CRISPR-Cas9 genome editing:

1. Embryonic stem cell and transgenic animals - CRISPR-Cas systems can be used to rapidly and efficiently engineer one or multiple genetic changes to murine embryonic stem cells for the generation of genetically modified mice.

2. Disease modelling - With the help of genome editing technologies, many applicable models with specific mutations which could mimic clinical phenotypes have been generated.

3. Cancer models - With the help of genome editing tools, numerous studies have been carried out through modifying key genes for generating accurate and specific cancer models.

4. Genome editing based therapy - Genome editing technologies are not only used for generating disease animal models but also destined to enter the therapeutic area through

- inactivation or correction of harmful mutations
- introduction of protective mutations
- insertion of therapeutic exogenous genes
- destruction of viral DNA.

5. Productivity improvement - Genome editing helps in enhancing crop productivity to overcome the shortcomings of traditional transgenic methods like irregular breeding cycles, lack of precision in intended trait selection and uncertainty in getting desirable mutations.

6. Allergy-free food - With CRISPR, it could be possible to make milk, eggs or peanuts that are safe for everyone to eat.

7. Greener fuels - Gene editing could improve the production of biofuels by algae.

8. Eradicating pests - CRISPR could help us control the numbers of animal species that transmit infectious diseases or that are invasive in a particular ecosystem. The gene-editing technology can be used to create 'gene drives' that ensure a genetic modification will be inherited by all the offspring, spreading throughout an animal population over several generations.

Challenges of CRISPR-Cas9 genome editing:

1. Ethical concerns - unnaturalness or against nature.

2. Safety concerns - slight changes in the results will lead to enormous consequences.

3. Against diversity - detrimental effect on our genetic diversity, a key to evolution on earth.

4. High cost - affordable issues.