

Q. What do you know about gene therapy?
 Analyze why is gene therapy considered the game changer in treating Sickle cell anaemia and beta thalassemia? (250 W)

- Gene Therapy is a technique to modify person's gene to treat or cure disease. (Cancer, Aids... etc)
- Basically it's divided into 3 types -
 - i) Ex vivo
 - ii) In vivo
 - iii) In situ.
- Somatic & Germ-line therapy are two prominent types.
- Applications :-
 - ✓ Replacement of ill-causing gene.
 - ✓ Helps and Empowers body for protection.
 - ✓ Treat Hereditary Disease, like Sickle cell ... etc.
- U.K. approved Casgevry and Lyfgenia, the gene therapy to treat Sickle cell and beta thalassemia.
- Sickle cell Anaemia
 - ✓ A Hereditary disease where RBCs Contort into a Sickle Shape.

• Beta Thalassaemia

- ✓ An Inherited blood disorder in which the blood makes meagre beta globin

• Challenges :-

- ✓ Expensive
- ✓ Need Special Equipments
- ✓ Need to serve Large Population.
- ✓ Limiting number of beneficiaries due to numerous factors.
- ✓ Effective monitoring for it's safety & efficacy.
- ✓ Need for Real World data.
- ✓ Possibility of Side-effects.

• Conclusive :-

- ✓ With research & Development, the welfare goes to cures even ^{the} incurable diseases. But one should caution about it's Inclusivity.