National Guidelines for Gene Therapy

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Context: 'National Guidelines for Gene Therapy Product Development and Clinical Trials' document has been released by ICMR

- Gene therapy is a technique that uses genetic modifications to treat or prevent ailments. As a part of the procedure, clinicians treat a disorder by inserting a gene into the cells of patients instead of using drugs or performing surgery.
- For a variety of debilitating diseases caused by genetic mutations, gene therapy offers a treatment modality that cannot be provided by conventional therapy including small molecule drugs or alternative medicines.
- The aim of the document is to ensure that gene therapies can be introduced in India and clinical trials for gene therapies can be performed in an ethical, scientific and safe manner
- These national guidelines provide the general principles for developing Gene Therapy Products (GTPs) for any human ailment and provides the framework for human clinical trials which must follow the established general principles of biomedical research for any human applications
- The guidelines cover all areas of GTP production, pre-clinical testing and clinical administration as well as long term follow up.
- These national guidelines apply to all stakeholders in the field of gene therapy including researchers, clinicians, oversight/regulatory committees, industry, patient support groups and any others involved in GTP development or their application in humans and their derivatives

Inherited Genetic diseases

- Inherited genetic diseases or “rare diseases” (RDs) refer to medical conditions that affect a small percentage of the population but has vast, debilitating and often life threatening effects of the patients, many of whom are in the paediatric age group
- These include haemophilia, thalassemia, sickle-cell anaemia certain forms of muscular dystrophies, retinal dystrophies such as retinitis pigmentosa, corneal dystrophies, primary immunodeficiency (PID) in children, lysosomal storage disorders such as Pompe disease, Gaucher’s disease, haemangioma, cystic fibrosis etc.
- Treatments for such diseases have long been neglected by the traditional pharma industry because of the notion that it will have uncertain or poor commercial outcomes given the smaller affected population size.