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GENE THERAPY

Rationale:

Genes are biological units of heredity. Genes determine evident traits like skin or eye color as well as less obvious characteristics like blood type, etc. A gene is part of deoxyribonucleic acid (DNA) molecule. Humans possess 50,000 to 100,000 genes. Genes carry instructions that permit the cells to produce specific proteins such as enzymes. When proteins are created, cells utilize another molecule, ribonucleic acid (RNA) to translate genetic information stored in DNA.

Only certain genes in a cell are active at any given time. As cells mature, many genes become permanently inactive. The kind of active and inactive genes in a cell and the resulting protein composition determine what type of cell it is, what this cell is capable of doing and what it cannot do. Defects in genes can result in disease.

Gene therapy is an experimental treatment that involves introducing genetic material (DNA or RNA) into a person's cells to treat or prevent disease. Gene therapy is being studied in clinical trials (research studies involving humans) for many different cancers and other diseases. Gene therapy is not available outside of these trials.

The primary function of BFAD, in the protection of health and welfare of Filipinos, is to ensure that drugs, medical devices and biological products are safe and effective before these are prescribed by doctors and used by patients. BFAD places under its authority the regulation of all gene therapy products and studies.

Scope of regulation:

Standards of safety, efficacy, purity and potency must be complied with:

List of requirements to be submitted for Investigational New Drug

1. For locally manufactured product, License to Operate as drug manufacturer
2. Certificate of Current Good Manufacturing Process local or country of origin
3. Summary of manufacturing process including quality control and assurance
4. General Information: