REPUBLIC OF THE PHILIPPINES
DEPARTMENT OF HEALTH
BUREAU OF FOOD AND DRUGS
Civic Drive, Filinvest Corporate City
Alabang, Muntinlupa City

MALAYA AT MALUSOG NA PAMAYANAN

6 July 2005

BUREAU CIRCULAR
No. 14 s. 2005

Re: 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:
    - Name of product
    - Active Ingredient(s)
        - Official Chemical Name (if applicable)

- Molecular Chemical Formula
- Amount per unit dose

- Dosage Form
- Recommended dosage and frequency of administration
- Indication/s or recommended use/s
- Route of administration

5. Laboratory analysis and tests demonstrating safety of gene therapy products
6. Animal Study Protocol and results of tests
7. If clinical trials (study of gene therapy in humans) are yet to be conducted, company must secure permit to conduct appropriate (Phases 1,2 or 3) study. The study product shall be considered an Investigational New Drug (IND).
8. Study protocol must be submitted. The protocol explains how the study will be conducted, what patients to include and exclude how the drug will be administered, the benefits that may be gained, determination of efficacy, possible risks that maybe encountered and measures by which patients are to be protected. The protocol should also provide additional data in support of study. Such study should adhere to principles of Declaration of Helsinki, Good Clinical Practice (GCP, 1997) and applicable Philippine laws and regulations.
9. Informed Consent should be obtained from persons participating in the study. Part of the information provided should include the potential risks and benefits.

IND approval by BFAD shall be based on careful review by its advisor-consultants before a study can be started. The consultants shall include microbiologists, molecular biologists, pharmacologists, toxicologists and immunologists

Duties of this panel of experts:

1. determine if gene therapy is appropriate for the disease
2. determine if such gene therapy is effective and safe, taking into account the protection of human subjects in the study

Manufacturer must secure approval of study protocol from the Hospital Institutional Ethics Review Board, a committee consisting of scientific, legal and medical advisors, religious and consumers. Focus is on the protection of persons participating in the study.

In keeping with the mandate of BFAD, the safety of patients is a primary concern. BFAD requires that manufacturers and medical researchers to be committed to the protection of patient and consumer welfare, to use highest quality experimental products, practice good clinical practice and accurately communicate information to BFAD and patients concerned.

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Director