

Regulation of Human Cell Therapy and Gene Therapy Products in Taiwan

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Establishment of TFDA



2010

2010.01.01 TFDA Inauguration (食品藥物管理局)

Integration of 4 bureaus:

- Food Safety (食品處)
- Pharmaceutical Affairs (藥政處)
- Food & Drug Analysis (食品藥物檢驗局)
- Controlled Drugs (管制藥品管理局)

2013

2013.07.23 TFDA Elevation (食品藥物管理署)

The *Ministry of Health and Welfare (MOHW)* was restructured from the Department of Health (DOH).



Regulatory Change in Taiwan

Before TFDA Inauguration

- Cell therapy and gene therapy were regarded as a kind of “**New Medical Practice**”, for which doctors needed to apply to the *Bureau of Medical Affairs (BMA)* for permission to conduct human trials according to the Medical Affairs Act.
- After human trials, “new medical practice” would have the opportunity to turn into “**Routine Medical Practice**”, that means doctors could routinely executed by themselves in the hospitals.

Medical
Practice

2010

Medicinal
Products

After TFDA Inauguration

- Regulation of cell therapy and gene therapy was transferred to *TFDA* in 2010.
- The original “medical practice” management approach was changed to “**Medicinal Product**” management approach, in accordance with standards provided by the “Pharmaceutical Affairs Act”.
- Sponsors should submit marketing application for cell therapy and gene therapy products.

Reasons of Regulatory Change

- Cells or genes *manipulated in vitro* might be not the same with the original cells or genes. It is necessary to establish regulation to ensure manufacturing consistency.
- Many countries have considered cells and genes as one kind of *biologics, medical devices, advanced medicinal products or regenerative products*.

Current Status in Taiwan -1

- Currently, TFDA has not yet approved any human cell therapy or gene therapy products for marketing, but *some cell therapy (such as human cord blood to treat Thalassemia) has been regulated as routine medical practice.*
- Numerous clinical studies of cell therapy and gene therapy products are ongoing and the amount of application continues to grow at a fast rate.

Current Status in Taiwan -2

- TFDA has announced guidances to facilitate the development of cell therapy and gene therapy products.
 - ✓ Guidance on Investigational Cell Therapy Products
 - ✓ Guidance on Cell Therapy Products Application
 - ✓ Guidance on Donor Eligibility Determination
 - ✓ Guidance on Good Tissue Practice (GTP)
- TFDA has also established the consultation mechanism to provide scientific and regulatory advice to medical researchers and manufacturers in the area of novel product development.

Consultation Mechanism

- To facilitate cell therapy and gene therapy products approval, early scientific advice is important for successful outcome.
- Meeting Types
 - ✓ Kick-Off meeting (KO meeting)
 - ✓ Sponsor meeting
 - ✓ Pre-filing meeting
- What is needed at consultation?
 - Provide data on product characterization, specification, quality control & release, well developed & controlled manufacturing information
 - Provide preclinical studies to show safety and effect of products
 - Provide evidence to support human dosing and scientific rationale

Regulatory Framework in Taiwan

Regulatory Framework of Cell Therapy and Gene Ther

Law	Pharmaceutical Affairs Act
Regulation	Regulation for Registration of Medicinal Products Regulations on Human Trials Regulation on Good Clinical Practice (GCP) Regulation on Good Manufacture Practice (GMP)
Guidance	Guidance on Investigational Cell Therapy Products Guidance on Cell Therapy Products Application Guidance on Donor Eligibility Determination Guidance on Good Tissue Practice (GTP)

Guidance on Cell Therapy Products Application

2015.07.13 announcement

Human cell therapy products are the autologous or allogeneic cells used to treatment, prevent or diagnose diseases.

Content

- ◆ General rules
- ◆ Manufacturing and control
- ◆ Non-clinical studies
- ◆ Clinical studies
- ★ ◆ Pharmacovigilance

(Risk management & long-term traceability)



Quality, Safety, Efficacy

★ **GMP compliance**

- facility, environment, material, equipment, personnel, maintenance, SOP...

GTP v.s. GMP

◆ GTP standard

- cells processing at clinical trials stage
- to prevent the introduction, transmission or spreading of communicable diseases

◆ GMP standard

- medicinal products for marketing
- to ensure manufacturing consistency using a controlled, reproducible and auditable SOP

Future Prospects

To Enhance International, Regional and Cross-strait Regulatory Collaboration

To revise “Pharmaceutical Affair Act”

~To separate category and define the cell therapy product, gene therapy product

To Establish Training Programs and Scientific Workshop

~ GTP, GMP, and Specificity of cell production process, animal model, clinical trial design

**Better Products
Better Life**

To continue establishing specific guidance

~Risk management and long-term traceability system

To Improve Consultation Mechanism

Regulatory support and accelerate development of cell therapy products

~Incentives for small and medium sized enterprises

Triple-Win Situation



Consumer Protection



Win-Win-Win



Government
Smart Administration

Industry
Competences Enhancement





Thank You for Your Attention

For more information

Website is at: <http://www.fda.gov.tw>

