PMDA Town Hall
- New Regulation in Japan and Future Direction of PMDA

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Today’s content

1. Introduction - PMDA

2. Enhancement of Cooperative Activity with key partner

3. Advanced Review and Consultation

4. Promotion of Regulatory Science to the Global Level

5. Summary
1. Introduction - PMDA
“Pharmaceuticals and Medical Devices Agency”

Major Services
- Scientific Review for Drugs & Medical Devices
- GCP, GMP Inspection
- Scientific Advice on Clinical Trials
- Safety Measures
- Relief Services

Unique Three-pillar System Securing Nation’s Safety

Headquarter
Established in 2004

Kansai Branch
Launched on Oct. 1, 2013

Hokuriku Branch
Launched on June 9, 2016
PMDA Staff Size

- **Administrative part**
- **Safety Department**
- **Review Department**
- **Planned**

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Current Missions for PMDA

- **Shortening the time from early development to approval**
  Measures: improvement in consultation system, accelerated review process, etc.

- **High quality review/consultation services**
  Measures: promotion of regulatory science research, etc.

- **Enhancing safety measures**
  Measures: utilization of medical information database

- **Globalization**
  Measures: information transfer with the world
2. Enhancement of Cooperative Activity with key partners
Enhancement of Pharmaceutical Affairs Consultation on R&D Strategy

① Facilitate development by academia by increasing reliability of development ROADMAP.
② Contribute to promotion of clinical trials led by academia.

Advice on ROADMAP

Advice on protocol of each study

Quality study
Non-clinical study
Clinical study

Exploratory trial
Confirmatory trial

Bridge between seeds and products

* In collaboration with the Japan Agency for Medical Research and Development (AMED), PMDA will be proactively supporting the establishment of an exit strategy via Pharmaceutical Affairs Consultation on R&D Strategy.

Practical Use
Innovative drugs, medical devices, regenerative medical products

Basic Research
Promising seeds
Japan Agency for Medical Research and Development (AMED)
Established on 1 April, 2015

1. Research and Development on Medical issues
   - Strategic and focused Assignment of Research and Development Budget, and Strengthening management function
   - Initiation of Initiative on Rare and Undiagnosed Diseases (IRUD)
     (note) IRUD: Social Reduction Research and Development Program that direct to create comprehensive medical system of nation-wide rare and undiagnosed diseases

2. Foundation of such as Clinical Study
   - Promotion of site project to create innovative medical technology
     - Biobank

3. Support towards commercialization
   - Promotion of ALL JAPAN Support under the “Drug Discovery Support Network” (start Drug-discovery Innovation and Screening Consortium(DISIC) from Dec., 2015)
   - Support function to the research institutes towards obtaining intellectual property

4. Promotion of International Strategy
   - To join International Rare Diseases Research Consortium (IRDiRC) in Jul., 2015
   - To conclude Memorandum of Understanding with National Institutes of Health (NIH) on cooperation in Jan., 2016
1. Utilizing Pharmaceutical Affairs Consultations on R&D Strategy

2. Support AMED to evaluate projects

3. Mutual cooperation to improve clinical research infrastructures

4. Sharing information
Collaboration with other Organizations

- Joint Graduate School Agreement (December 2009-)
  with 19 graduate schools in medicine: Personnel Exchanges

  Expansion and Improvement

- Comprehensive Partnership Agreements with

  NCC (National Cancer Center) (February 2, 2016)
  Hiroshima University (March 4, 2016)
  Keio University (March 11, 2016)
  University of Tsukuba (March 30, 2016)

  • Joint Research
  • Human Resources Development
  • Information Dissemination
3. Advanced Review and Consultation
Advanced Review/Consultation System

Analysis by PMDA
- Giving additional scientific value to submitted data

NDA etc.
- e-Submission of study data
- Data Accumulation
- Database

Regulatory Science
- Sophisticated NDA review
  - Each reviewer utilizes innovative assessment techniques
- Cross-Products Analysis
  - Innovative evaluation methods
  - Active utilization of Modeling & Simulation
    - Disease model
    - Objective B/R assessment
    - Identifying AE-related factors etc.
- Sophisticated Consultation
  - More evidence-based consultation

Cooperation with Academia

Practical use of Innovative Medical Products
- A rational & effective evaluation process for regulatory decision

Effective and High Quality Review
- More predictable efficacy/safety after approval
- Reduction of applicant’s work load
- More scientific regulatory decision

Effective and Successful Development
- Epoch-making proposal leading the world
- Proactive publication of guideline
Prospect of Advanced Review and Consultation

Set up e-data management and utilization scheme

FY2016

Standardization of utilization of e-data in product review

FY2018

Start full-fledged cross-product analysis

FY2019 - 2021

Publication of guidelines to contribution to drug development

FY2022 - 2023

First-class review authority

We are here
4. Promote Regulatory Science to the Global Level
Regulatory Science

Science → Technology

RS Macroscopia
Science for Evaluation Method
(Comprehensive Judgment)

RS Microscopia
Science for Evaluation Method
(Quality, Efficacy, Safety)

Knowledge Accumulation

Deductive Approach

Technology for regulatory adaptation

Achievements from RS Engineering (e.g.)
- Establish evaluation method for cutting-edge technology
- Respond to translational research
- Modelling & Simulation
- Establish guidelines
- Establish review standards
- Draft legislation etc.

Ethical Science and Technology for the People and Society/
The Science Board was established in May 2012 to discuss how PMDA can better cope with products with advanced science & technology, in each developmental stage (basic research, development support, product review, and PMS).
Support for Innovation Implementation via Science Board

Universities/institutes/medical institutions
Researchers with superior knowledge, experiences in drugs/medical devices, and with superior research achievements, who are taking active part in the front line.

Collaboration with academia

Rotation of Personnel

Science Board

Exchange opinions between top-class researchers in Japan and PMDA reviewers on assessment methods of cutting-edge technologies

Take initiative in putting cutting-edge technologies into practical use based on regulatory science
Outcome of the Science Board

1st term (FY2012 - 2013)
- Summary of discussion on the assessment of the current status of personalized medicine related to development and regulatory review (2014)
- Summary of discussion on non-clinical pharmacological studies on anticancer drugs (2013)
- Current perspective on evaluation of tumorigenicity of cellular and tissue-based products derived from induced pluripotent stem cells (iPSCs) and iPSCs as their starting materials (2013)

2nd term (FY2014 - 2015)
- Discussion on Evaluation of Medical Devices in Pediatric Use (2015)
- Report on the use of non-clinical studies in the regulatory evaluation of oncology drugs (2016)
- Current Status and Perspectives of Placebo-Controlled Studies (2016)
The Report of the Science Board was Published in Peer-Reviewed Journal

5. Summary
Future Direction - Infrastructure

Strengthen the infrastructure toward Globalization

- Human Resource Development
- Communication
- Dissemination of information
To Improve Public Health

- Review
- Safety
- Relief

REGULATORY SCIENCE
INTERNATIONAL COOPERATION

Philosophy
Thank You

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