Introduction of
Third 5-Year Mid-Term Plan of PMDA

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Background

The Japanese Government issued the important policies which require PMDA to strengthen the organization both in size and in quality.

- Japan Revitalization Strategy - JAPAN is BACK - (Cabinet Decision on June 14, 2013)

- Health and Medical Strategy (Agreed by related Ministers on June 14, 2013)
Japan Revitalization Strategy - JAPAN is BACK -
(Cabinet Decision on June 14, 2013)

Extended national “Healthy life expectancy”

<Future vision of the society>
The society where people can receive necessary healthcare services at the most advanced level in the world

Foster the industry specialized in extended healthy life, by developing innovative pharmaceuticals, medical devices and regenerative medicines first in the world, and by introducing these products to the market through speedy review process.

Measures

- Strengthen PMDA organization both in size and in quality
  - While maintaining a keen attention to post marketing product quality and safety, further reduction of review time (achieve “0” review lag ) and improved quality will be pursued.
  - Enhancement of Pharmaceutical Affairs Consultation on R&D Strategy
  - Establishment of the Medical Information Databases

*Reform of regulation and system to accelerate generative medicine research and environmental improvement for practical use of regenerative medicine products are required.
Health and Medical Strategy
(Agreed by Chief Cabinet Secretary, Minister of Health, Labour and Welfare and other related Ministers on June 14, 2013)

Basic philosophy (Three philosophies)
- Realize extended healthy life society
- Contribute to economic growth
- Contribute to the world

Measures
- Establish organization to promote research and development
  ~ Prepare ”All Japan” support system ~
  - Establish control tower function (New organization)

- Strengthen PMDA
  - Further reduction of review time (achieve “0” review lag )
  - Expand and enhance the pharmaceutical affairs consultation on R&D strategy program
  - Establishment of the medical information databases
  - Promote PMDA’s own analysis and study of clinical data
  - Utilization of Science Board, Global harmonization etc.

※ Development of the security evaluation system using iPS cells is required for better new drug development
Amendment of Pharmaceutical Affairs Act

◆ **Regulation considering Regenerative Medicines Character**
  - Creation for Regenerative Medicines regulations
  - Introduction of approval system with condition/period

◆ **Regulation considering Medical Devices Character**
  - Independent Chapter for “Medical Devices”
  - Third party certification system
  - Quality Management System (QMS)
  - Other revisions related to medical devices
  - Regenerative and Cellular Therapy Products, and Gene Therapy Products

◆ **Strengthen Safety Measures regarding Drugs, Medical Devices**
  - Specify relevant party’s obligation to ensure quality, safety, and efficacy of drugs and medical devices.
  - MAH’s obligation to notify revised Package insert reflecting the latest findings
Outline of 3rd mid-term plan

In the 5-year plan, we are planning to

- Improve both review and safety measures implemented by PMDA  
  【Operational matter】

- Increase the number of staff  
  ( 751 in FY2013→1065 in FY2018 )  
  【Organizational matter】
3rd 5-year mid-term plan of PMDA (FY2014-2018)

Major challenges

- Shortening the time to approval & High quality review/consultation services
- Enhancing safety measures
- Globalization

Specific measures

- Accelerated review process (Improvement of approval predictability)
- Improvement of prior assessment (substantial acceleration of approval review process)
- Readiness for introduction of risk management plan
- Improvement of consultation service
  - Improvement of pharmaceutical affairs consultation service on R&D strategy
  - Improvement of clinical trial consultation service
- Drastic improvement of consultation service
- Utilization of medical information database

Goal

- Development of Japan’s original innovative drugs and medical devices
- Marketing of cellular and tissue-based products
- Activation of the industry
- Extending health and life span of Japanese people
- Contribution to global medicine

Advanced Review/Consultation System

Human Resources with excellent skills【751 staffs →1065 staffs】
3rd 5-year mid-term plan of PMDA (FY2014-2018)

Major challenges

Shortening the time to approval & High quality review/consultation services

Specific measures

- Accelerated review process (Improvement of approval predictability)
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- Drastic improvement of consultation service
  - Improvement of pharmaceutical affairs consultation service on R&D strategy
  - Improvement of clinical trial consultation service
- Introduction of approval system with condition/period for Regenerative Medicines
- Advanced Review/Consultation System

Enhancing safety measures

Globalization

Goal

- Development of Japan’s original innovative drugs and medical devices
- Marketing of cellular and tissue-based products

- Activation of the industry
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Human Resources with excellent skills

- 751 staffs → 1065 staffs

Introduction of approval system for Regenerative Medicines

Utilization of medical information database

Readiness for introduction of risk management plan

Goal

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### New Target of Review Time

#### New Drugs (Priority)

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<tr>
<th>Fiscal Year</th>
<th>Percentile</th>
<th>Review Time</th>
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<td>2013</td>
<td>50% (median)</td>
<td>9 months</td>
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<tr>
<td>2014</td>
<td>60%</td>
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<tr>
<td>2018</td>
<td>80%</td>
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#### New Drugs (Standard)

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<tbody>
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- **3rd mid-term plan**
- **7.2 months (Result)**
- **11.3 months (Result)**
To achieve new target

In order to achieve new target, we will take these measures.

• Reinforcement of Prior Assessment Consultations (substantial acceleration of approval review process)

• New approval system with condition/period for Regenerative Medicines

• Improvement of consultation service

→ We can shorten review time of new drugs which were not covered in target time before.

→ We need more reviewers.
Reinforcement of Prior Assessment Consultations

Quality
(Drug substance, product specification, stability, etc.)

Non-Clinical
(Toxicology, Pharmacology, and ADME)

Clinical P1
Clinical P2
Clinical P3
Application preparation

Stability of Drug Product
Carcinogenicity

Gradual evaluation based on each test result
More smooth reaction by applicant

Front-loaded review in practice by prior assessment

(Start without waiting until whole test results are ready.)
Clinical Trial
(confirmation of efficacy and safety)

Clinical Trial
(confirmation of probable benefit* and safety**)

Provisional Approval with condition

Marketing
(further confirmation of efficacy and safety)

Approval or Expiration of provisional approval

Informed Consent and Post Market Safety Measures

※Earlier Patient Access!

* Probable benefit: Confirmation of efficacy with small patient population.
** Safety: Earlier detection and evaluation of adverse events.
Pharmaceutical Affairs Consultation on R&D Strategy

Valley of Death
- Shortage of funds, Knowledge on Regulation and development strategy

Strategic Consultation

Basic Research
Pharmaceutical and Medical Devices candidates

Quality Study

Non-Clinical Study

Clinical Trial
Up to the level of POC studies*

* Further studies are handled by the Regular Consultation

Introductory Consultation
- Explain procedure
- No Charge
657 Consultations

Pre-Consultation
- Sort out issues
- 30 min, No Charge, Not binding
753 Consultations

Face-to-Face Consultation
- Scientific discussion
- 2 hours, Charged, Binding, Minutes
193 Consultations

Practical Use
Innovative Products originated from Japan

7/1/2011 – 3/31/2014

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50TH ANNUAL MEETING
**3rd 5-year mid-term plan of PMDA (FY2014-2018)**

### Major challenges

- Shortening the time to approval & High quality review/consultation services
- Enhancing safety measures
- Globalization

### Specific measures

- **Accelerated review process** (Improvement of approval predictability)
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  - Improvement of pharmaceutical affairs consultation service on R&D strategy
  - Improvement of clinical trial consultation service
- **Advanced Review/Consultation System**
- **Utilization of medical information database**
- **Readiness for introduction of risk management plan**

### Goal

- **Activation of the industry**
- **Extending health and life span of Japanese people**
- **Contribution to global medicine**
- **Development of Japan’s original innovative drugs and medical devices**
- **Marketing of cellular and tissue-based products**

### Human Resources with excellent skills

- From 751 staffs to 1065 staffs
### Priority Issues to be Consolidated for Post-Marketing Safety Measures

1. Strengthening of information gathering on adverse drug reactions and malfunctions
2. Organization of information on adverse drug reactions and systemization of evaluation and analysis
3. Establishment of the medical information databases
4. Establishment of a post-marketing safety system through information feedback
5. Fulfilling information distributed to general public related to Pharmaceuticals and Medical Devices Safety
6. Appropriate safety measures based on the Risk Management Plan
7. Reinforcement of safety measures adapted to new review system as well as consistently monitoring the safety of drugs from the clinical trial stage to post-marketing stage
8. Strengthening and improvement of follow-up on implemented safety measures
9. Organizing, evaluating, and analyzing information gathered from Vaccine Adverse Reaction Reporting System

> We need more staff with excellent skills.
3rd 5-year mid-term plan of PMDA (FY2014-2018)

Major challenges

Shortening the time to approval & High quality review/consultation services

Globalization

Enhancing safety measures

Specific measures

Accelerated review process (Improvement of approval predictability)

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Introduction of approval system with condition/period for Regenerative Medicines

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Human Resources with excellent skills [751 staffs → 1065 staffs]

Goal

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Improvement of prior assessment

Improvement of clinical trial consultation service

Improvement of pharmaceutical affairs consultation service on R&D strategy
Advanced Review/Consultation System

Analysis by PMDA
- Giving additional scientific value to submitted data

Cooperation with Academia

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

Regulatory Science

NDA etc.
- e-Submission of study data

Data Accumulation
- Database

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

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

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Introduction of approval system with condition/period for Regenerative Medicines

Human Resources with excellent skills

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Roadmap for the PMDA International Vision

Five Important Areas Where RMs are needed

1) Response to advanced science and technology
   ・ Proactively provide information about the policies for review and scientific consultation of cutting-edge products and recommendation for relevant guideline developments.
   ・ Introduce progressive analyzing and predictive methods.

2) Improvement of international operation basis
   ・ Improve the organizational structure enabling wide range international activities and cultivate new internationally minded personnel* in a prompt manner.
   *A personnel who has 1) good command of foreign languages, 2) an international human network, 3) abundant knowledge of his or her related area of expertise, 4) ability to make appropriate decisions under the given circumstances domestically and internationally, and 5) trustworthy international relations.

3) Dissemination of English information on regulatory review of medicinal products, especially publication of review reports in English
   ・ Increase the number of English version of review reports (aiming to cover all the necessary review reports in English in the future).

4) Dissemination of information and international cooperation on safety measures
   ・ Enhance exchanging information and establish a system to share evaluation reports with our overseas counterparts.
   ・ Enrich the contents related to safety information in the English website.

5) Increase of the leverage of Japanese Pharmacopoeia (JP)
   ・ Publish the newest JP version simultaneously in English and Japanese.
   ・ Enhance cooperative relationship with the USP, EP, WHO and each Asian pharmacopeia.

Note) As we have been committed to emphasize the activities with ICH, IMDRF and other foreign regulatory agencies, the effort should continue for the future development.

We need more staff with excellent skills.
Staff Size of PMDA

常勤役職員数（人）

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Thank you

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