PMDA’s Achievements and Future Perspectives (Summary)

Tatsuya Kondo, M.D. Ph.D.
Chief Executive
Pharmaceuticals and Medical Devices Agency (PMDA), Japan
The views and opinions expressed in the following PowerPoint slides are those of the individual presenter and should not be attributed to Drug Information Association, Inc. (“DIA”), its directors, officers, employees, volunteers, members, chapters, councils, Special Interest Area Communities or affiliates, or any organization with which the presenter is employed or affiliated.

These PowerPoint slides are the intellectual property of the individual presenter and are protected under the copyright laws of the United States of America and other countries. Used by permission. All rights reserved. Drug Information Association, DIA and DIA logo are registered trademarks or trademarks of Drug Information Association Inc. All other trademarks are the property of their respective owners.
Pharmaceuticals and Medical Devices Agency

Date of Establishment: April 2004

PMDA’s Safety Triangle
Unique Three-piller System Securing Nation’s Safety

- Review
  - Reduction in risk

- Safety
  - Continuous risk mitigation efforts

- Japanese Citizens

- Relief
  - Relief measures for health damage caused by risk factors

Kansai Branch
PMDA Forum as the 10th Anniversary

2014. 2. 8. Tokyo

Guest speakers: Prof. Guido Rasi (EMA); Mr. Jüng H. Schnetzer (Swissmedic); A/Prof. John C W Lim (HSA); Dr. Chung Seung (MFDS); Dr. M. Hayatie Amal (NADFC); Mr. Kees de Joncheere (WHO); Dr. Margaret A. Hamburg (FDA, video presentation)
### Strategies and Measures for PMDA Innovation

<table>
<thead>
<tr>
<th>Issues with PMDA (past 6 years)</th>
<th>Basic policies to address the issues</th>
<th>Efforts made so far</th>
</tr>
</thead>
<tbody>
<tr>
<td>◆ Shorten review time • Reduce drug lag • Reduce device lag</td>
<td>◆ Philosophy (Mission Statement) ◆ Regulatory science ◆ Global partnership (Win-Win Relationship)</td>
<td>◆ Increase staffs ◆ Enhance training program ◆ Academic cooperation ➢ Science Board ➢ Joint Graduate School Program ➢ Human resource exchange program ◆ Industry-Government-Academia collaboration ◆ Pharmaceutical affairs consultation ◆ Cross-sectional project within PMDA ◆ IT-based safety measures ➢ MIHARI Project ➢ Project for developing medical information database infrastructure ◆ Risk Manager (RM) ◆ Risk Management Plan (RMP) ◆ GLP, GCP, GMP, QMS inspection programs ◆ Adverse health effect relief system ◆ International strategic plan ◆ International liaison officers to US and EU ◆ Global partnership with US, EU and Asian countries (ICH, IMDRF, PIC/S, etc.)</td>
</tr>
</tbody>
</table>

Pharmaceutical affairs are the ultimate medical ethics, and regulatory science is the underlying science.
PMDA Philosophy (September, 2008)

PMDA continues to improve the public health and safety of our nation by reviewing applications for marketing approval of pharmaceuticals and medical devices, conducting safety measures, and providing relief to people who have suffered from adverse drug reactions.

We conduct our mission in accordance with the following principles:

• We pursue the development of medical science while performing our duty with greater transparency based on our mission to protect public health and the lives of our citizens.

• We will be the bridge between the patients and their wishes for faster access to safer and more effective drugs and medical devices.

• We make science-based judgments on quality, safety, and efficacy of medical products by training personnel to have the latest technical knowledge and wisdom in their field of expertise.

• We play an active role within the international community by promoting international harmonization.

• We conduct services in a way that is trusted by the public based on our experiences from the past.
Shortening Review Period

With the increase of increase personnel and development of ability, PMDA has shortened review period while applications increasing.

In terms of 2013, data is accumulated until the end of October.
Improvement of Safety Measures

- Collection of Information
- Analysis
- Hypothesis
- Evaluation of hypothesis
- Planning and Implementation of Safety measures
- New Risk management system
- Assessment of Safety measure effects
- Crisis management

Goal:
- Prevention of serious drug safety-related crisis from Japan
- Effective encouragement of proper drug use.
- Ensuring credibility to post-market safety management system.
## Relief Services

PMDA shortened almost a month of review period for judgment of payment/non-payment.

<table>
<thead>
<tr>
<th>Fiscal Year</th>
<th>2009</th>
<th>2010</th>
<th>2011</th>
<th>2012</th>
<th>2013 (Apr-Sep)*</th>
</tr>
</thead>
<tbody>
<tr>
<td>Accepted Claims</td>
<td>1,052</td>
<td>1,018</td>
<td>1,075</td>
<td>1,280</td>
<td>664</td>
</tr>
<tr>
<td>Judgment</td>
<td>990</td>
<td>1,021</td>
<td>1,103</td>
<td>1,216</td>
<td>625</td>
</tr>
<tr>
<td>Payment</td>
<td>861</td>
<td>897</td>
<td>959</td>
<td>997</td>
<td>501</td>
</tr>
<tr>
<td>Non-payment</td>
<td>127</td>
<td>122</td>
<td>143</td>
<td>215</td>
<td>123</td>
</tr>
<tr>
<td>Rejection</td>
<td>2</td>
<td>2</td>
<td>1</td>
<td>4</td>
<td>1</td>
</tr>
<tr>
<td>Amount of Payment (thousand JPY)</td>
<td>1,783,783</td>
<td>1,867,190</td>
<td>2,058,389</td>
<td>1,920,771</td>
<td>865,404</td>
</tr>
<tr>
<td>% of Cases &lt; 8 months judgment*2</td>
<td>733</td>
<td>765</td>
<td>809</td>
<td>923</td>
<td>539</td>
</tr>
<tr>
<td></td>
<td>74.0%</td>
<td>74.9%</td>
<td>73.3%</td>
<td>75.9%</td>
<td>86.2%</td>
</tr>
<tr>
<td>% of Cases &lt; 6 months judgment*3</td>
<td>360</td>
<td>434</td>
<td>534</td>
<td>553</td>
<td>395</td>
</tr>
<tr>
<td></td>
<td>36.4%</td>
<td>42.5%</td>
<td>48.4%</td>
<td>45.5%</td>
<td>63.2%</td>
</tr>
<tr>
<td>Review period (median)</td>
<td>6.8 months</td>
<td>6.4 months</td>
<td>6.1 months</td>
<td>6.2 months</td>
<td>5.7 months</td>
</tr>
</tbody>
</table>

*1 Cases are publication basis  
*2 Percentile of cases with less than 8 month review period for judgment in the given year  
*3 Percentile of cases with less than 8 month review period for judgment in the given year
Facilitation of Consultation for Relief Services

By PMDA’s efforts of outreach and improvement of telephone consultation system, the numbers of consultation and access to relief service website has increased.

### Numbers of Consultation and Website Access

<table>
<thead>
<tr>
<th>Fiscal Year</th>
<th>2009</th>
<th>2010</th>
<th>2011</th>
<th>2012</th>
<th>2013 (Apr-Sep)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Consultation</td>
<td>34,586</td>
<td>16,123</td>
<td>21,577</td>
<td>22,324</td>
<td>11,275</td>
</tr>
<tr>
<td>Web Access</td>
<td>87,109</td>
<td>89,500</td>
<td>72,688</td>
<td>113,182</td>
<td>86,915</td>
</tr>
<tr>
<td>Special Site Access</td>
<td>397,583</td>
<td>29,375</td>
<td></td>
<td></td>
<td>16,165</td>
</tr>
</tbody>
</table>
Promotion of Regulatory Sciences

Basic Research: Seeds originated in Japan

Practical Use: Innovative Drugs and Medical Devices

- Quality Study
- Non-Clinical Trial
- Clinical Trial
- Review
- Approval
- Post-marketing

Correspond better to the characteristics of innovative new medicines in all phases from seeds to practical use

- Science Board
- Pharmaceutical Affairs Consultation on R&D Strategy
- Personnel Exchanges
- Advanced Review and Consultation with e-Submitted Study Data (ex. Modeling & Simulation)
- Improvement of Safety Measures
Achievement of Bilateral Activities

Confidentiality Arrangement
Memorandum of Understanding (MOU)
Resident Staff
Joint Symposium

* MOU between the Chinese SFDA (present CFDA) and the Japanese MHLW, under which PMDA supports cooperative activities
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)
- Drastic improvement of consultation service
  - Improvement of pharmaceutical affairs consultation service on R&D strategy
  - Improvement of clinical trial consultation service
- Readiness for introduction of risk management plan
- Utilization of medical information database
- Introduction of approval system with condition/period for Regenerative Medicines
- Advanced Review/Consultation System

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

Human Resources with excellent skills 751 staffs → 1065 staffs
To Improve Public Health

Review
Safety
Relief

REGULATORY SCIENCE
INTERNATIONAL COOPERATION

Philosophy
Thank you, and please enjoy our presentations!